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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2018

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission file number: 001-38520

MEIRAGTX HOLDINGS PLC
(Exact name of registrant as specified in its charter)

Cayman Islands	**Not applicable**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
430 East 29th Street, 10th Floor New York, NY	**10016**
(Address of principal executive offices)	**(Zip Code)**

(646) 490 2965
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of exchange on which registered**
Ordinary Shares, $0.00003881 Nominal value	**Nasdaq**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Exchange Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of June 30, 2018, the aggregate market value of the registrant's ordinary shares held by non-affiliates of the registrant was approximately $154.6 million (based upon the closing sale price of the registrant's ordinary shares on that date on the Nasdaq Global Select Market).

As of March 15, 2019, the registrant had 33,183,734 ordinary shares outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement relating to its 2019 annual shareholder meeting to be filed with the SEC within 120 days after the end of the fiscal year ended December 31, 2018 are incorporated herein by reference in Part III.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K (the "Form 10-K") contains forward-looking statements that can involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Form 10-K, including statements regarding our future results of operations and financial position, business strategy, prospective products, product approvals, research and development costs, future revenue, timing and likelihood of success, plans and objectives of management for future operations, future results of anticipated products and prospects, plans and objectives of management are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential," "would" or "continue" or the negative of these terms or other similar expressions. The forward-looking statements in this Form 10-K are only predictions. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements speak only as of the date of this Form 10-K and are subject to a number of risks, uncertainties and assumptions described under the sections in this Form 10-K entitled "Item 1A. Risk Factors" and "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Form 10-K. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

You should read this Form 10-K and the documents that we reference in this Form 10-K and have filed as exhibits to this Form 10-K, completely and with the understanding that our actual future results may be materially different from what we expect.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Form 10-K, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

BASIS OF PRESENTATION

On June 7, 2018, in connection with its initial public offering (the "IPO"), MeiraGTx Holdings plc, an exempted company incorporated under the laws of the Cayman Islands, acquired all the issued and outstanding ordinary shares of MeiraGTx Limited pursuant to a series of reorganization transactions. We refer to these events in this Form 10-K as the "Reorganization Transactions." Prior to the Reorganization Transactions, MeiraGTx Holdings plc had not conducted any operations and had nominal assets and liabilities.

Unless the context otherwise requires, references in this Form 10-K to "Meira," "we," "us", "our" or "the Company" refer to (i) MeiraGTx Limited and its subsidiaries prior to the Reorganization Transactions and (ii) MeiraGTx Holdings plc and its subsidiaries upon completion of the Reorganization Transactions, as applicable.

We have proprietary rights to trademarks, trade names and service marks appearing in this Form 10-K that are important to our business. Solely for convenience, the trademarks, trade names and service marks may appear in this Form 10-K without the ® and TM symbols, but any such references are not intended to indicate, in any way, that we forgo or will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensors to these trademarks, trade names and service marks. All trademarks, trade names and service marks appearing in this Form 10-K are the property of their respective owners.

INDUSTRY AND OTHER DATA

We obtained the industry, market and competitive position data in this Form 10-K from our own internal estimates and research as well as from industry and general publications and research, surveys and studies conducted by third parties. Industry publications, studies and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that each of these studies and publications is reliable, we have not independently verified market and industry data from third-party sources. While we believe our internal company research as to such matters is reliable and the market definitions are appropriate, neither such research nor these definitions have been verified by any independent source.

PART I

ITEM 1. BUSINESS

Overview

We are a vertically integrated, clinical stage gene therapy company with five programs in clinical development and a broad pipeline of preclinical and research programs. We have core capabilities in viral vector design and optimization and gene therapy manufacturing, as well as a potentially transformative gene regulation technology. Led by an experienced management team, we have taken a portfolio approach by licensing, acquiring and developing technologies that give us depth across both product candidates and indications. Though initially focusing on the eye, salivary gland and central nervous system, we intend to expand our focus in the future to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

We own and operate a flexible and scalable viral vector manufacturing facility that we expect can supply our current clinical and preclinical programs through regulatory approval and, should they be approved, provide sufficient capacity for commercial production. Completed in early 2018 and designed to meet global regulatory requirements, including the current good manufacturing practices, or cGMP, required by the U.S. Food and Drug Administration, or FDA, our 29,000 square foot facility has two cell production suites, three independent viral vector production suites providing multi-product and multi-viral vector manufacturing capabilities and an integrated, flexible fill-and-finish suite. In May 2018, we were granted a license to manufacture gene therapy product candidates in our cGMP compliant manufacturing facility by the UK Medicines and Healthcare products Regulatory Agency. On December 14, 2018, we acquired from Moorfields Eye Hospital NHS Foundation Trust of Moorfields Eye Hospital a long leasehold interest, (the “Head Lease”), in the site of our manufacturing facility, 92 Britannia Walk, London N1 7LU, for a purchase price of £5,250,000 (approximately $6,615,000 assuming a rate of $1.26 per GBP on the date of the acquisition. As a result of this transaction, we are now the tenant under the Head Lease, which has a remaining term of 108 years, with no facility rent due

We have also established a comprehensive platform for the efficient clinical development of the next generation of gene therapies and manufacturing in accordance with cGMP. Our deep understanding of disease models informs our development of potency assays for the cGMP production of our product candidates, and our teams experienced in viral vector design and optimization work closely with our process development team to design viral vectors and develop proprietary production cell lines for efficient scaling of manufacturing processes.

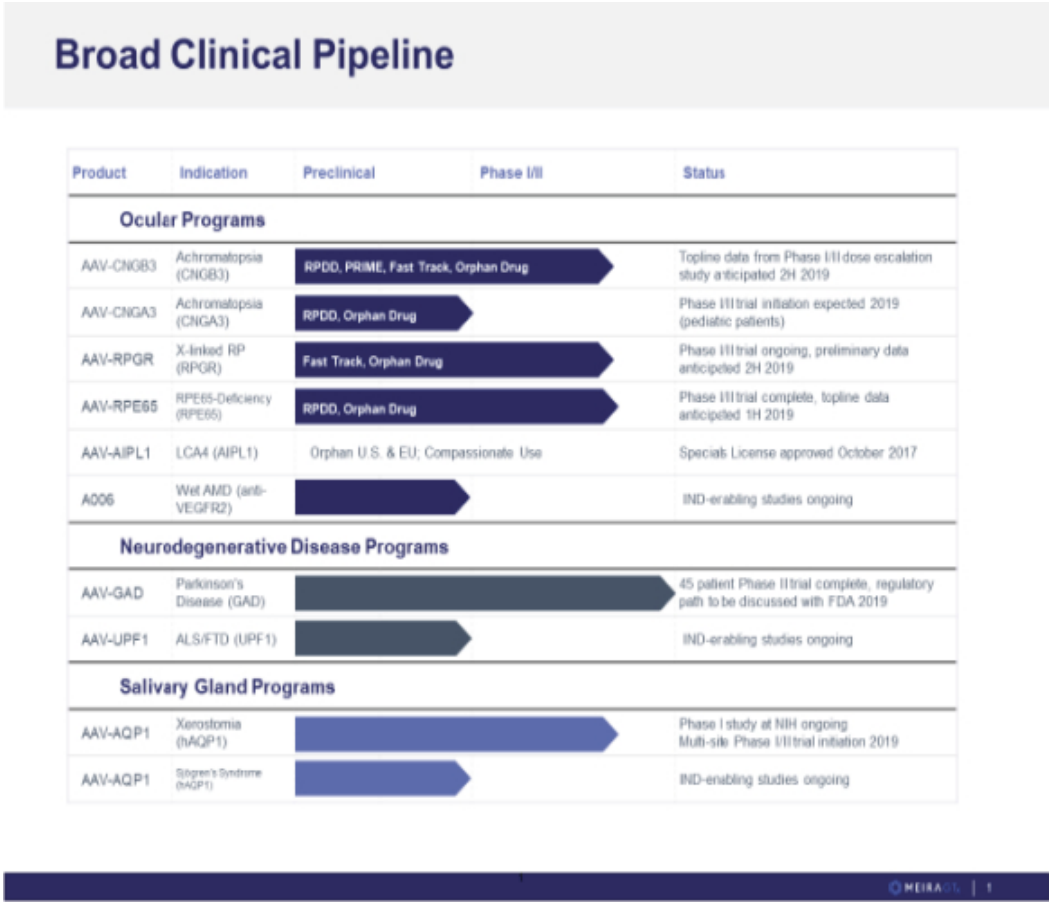
We are also developing a potentially transformative technology to enable the use of small molecules to turn gene therapy product candidates on and off. The aim of this gene regulation platform is to convert gene therapy into a generalizable delivery mechanism for biologic drugs using a small molecule “switch” for temporal control. We believe the capacity for temporal control of gene therapy products has the potential to transform the gene therapy landscape by opening up new treatment possibilities.

Our Pipeline

Our initial focus is on three distinct areas of unmet medical need: inherited retinal diseases, or IRDs, severe forms of xerostomia and neurodegenerative diseases. Utilizing our product development platform, we have assembled a pipeline of gene therapies to treat these serious diseases. Our criteria for selecting our initial product candidates included:

- unmet medical need;
- high potential for meaningful clinical benefit;
- promising preclinical data using multiple animal models as well as human stem cell derived organoids;
- compartmentalized anatomy of target tissue and the partially immune protected nature of target tissue; and
- understanding of the disease state from natural history studies and detailed long-term characterization of patients prior to entry into gene therapy treatment studies.

A summary of our product candidates and the status of such product candidates as of March 15, 2019 is described below. We retain worldwide development and commercialization rights to all of our product candidates, with the exception of AAV-CNGB3, AAV-CNGA3 and AAV-RPGR, which are subject to a strategic Collaboration, Option and License Agreement (the “Collaboration Agreement”) that we executed with Janssen Pharmaceuticals, Inc. (“Janssen”), one of the Janssen Pharmaceutical Companies of Johnson & Johnson that was executed on January 30, 2019.



In addition to these clinical and preclinical programs, we have preclinical and research programs in other indications and novel molecular technologies that we aim to advance into clinical development, including:

- neovascular age related macular degeneration, or wet AMD – use of a gene therapy product to deliver an antibody targeting the vascular endothelial growth factor receptor 2, or anti-VEGFR2, with the aim of blocking disease related vascular formation in the eye;
- geographic atrophy age related macular degeneration, or dry AMD – use of gene therapy technology to introduce light sensitive molecules into rod photoreceptors in order to restore some aspects of vision lost in this disease;
- amyotrophic lateral sclerosis, or ALS—targeting dysregulation of neuronal RNA, processing, which we believe may lead to the degeneration of motor neurons that occurs in ALS;
- Alzheimer's disease—targeting endosomal trafficking, which is a central mechanism that we believe underlies Alzheimer's disease; and
- gene regulation—use of our proprietary RNA shape regulation cassette to switch gene therapy product candidates on and off with small molecules, potentially transforming gene therapy technology into a delivery mechanism for a broad array of biologic drugs.

Our Ophthalmology Programs

Eye diseases are our first area of clinical focus and we aim to provide treatments with durable, long-term clinical benefit that will halt vision loss in patients. We currently have three ongoing Phase 1/2 clinical programs in IRDs, with an additional program expected to initiate a Phase 1/2 clinical trial in 2019. The targets of our three ongoing Phase 1/2 ophthalmology programs include achromatopsia, or ACHM, related to mutations in *CNGB3*, X-linked retinitis pigmentosa related to mutations in *RPGR*, or XLRP-RPGR, and inherited retinal dystrophy caused by mutations in *RPE65*, or *RPE65*-deficiency. We also have a product candidate that was manufactured and released for compassionate use under a specials license in the United Kingdom to treat patients with Leber congenital amaurosis 4, or LCA4, caused by mutations in *AIPL1*. For each of our Phase 1/2 clinical programs we have a prospectively designed natural history study ongoing, which includes the same endpoints as our corresponding gene therapy treatment trial. We believe use of these natural history studies differentiates our programs by providing patient populations to facilitate the efficient execution of our clinical trials and offering insight into the appropriate endpoints for regulatory approval of our gene therapy product candidates. In addition to these clinical programs in IRDs, we have preclinical programs that apply novel approaches to both wet and dry AMD.

The FDA and EMA have granted orphan drug designation to each product candidate in our ongoing clinical programs, including those treating mutations in *CNGB3*, *RPGR* and *RPE65*, as well our product candidates for the treatment of achromatopsia due to autosomal-recessive *CNGA3* gene mutations and to treat mutations in *AIPL1*. The FDA has also granted rare pediatric disease designation for our clinical programs treating mutations in *RPE65* and *CNGB3* and our product candidate for the treatment of autosomal-recessive *CNGA3* gene mutations, and Fast Track designation to our clinical programs for the treatment of achromatopsia caused by *CNGB3* gene mutations to improve visual function and for the treatment of XLRP caused by mutations in *RPGR*. We have also received PRIME designation from the EMA for our clinical program treating mutations in *CNGB3*.

The deep scientific and clinical understanding of IRDs driving our approach to gene therapy development helps us to optimize our product candidates for each specific genetic mutation and phenotype. We develop our viral vectors by selecting and modifying proprietary cell specific promoters, selecting appropriate capsids for transfection of target cells and refining the vector for efficient production and scalable manufacturing. Not only does this allow us to synergistically target a portfolio of inherited eye conditions, we also believe it has potential to be applied to the development of gene therapies for other diseases.

Our longstanding relationships with leading institutions in retinal disease treatment, including the Moorfields Eye Hospital in London, the University of Michigan Kellogg Eye Center, Massachusetts Eye and Ear, the Medical College of Wisconsin & Froedtert Hospital and the Casey Eye Institute at the Oregon Health & Science University, provide us with access to experts whose guidance and insight informs our development strategy, as well potential patients for our clinical trials.

Our Salivary Gland Programs

Our second area of clinical focus is xerostomia, a chronic and debilitating disorder of the salivary glands in which saliva production is impaired. Xerostomia may be caused by a number of different insults to the salivary glands, including radiation therapy for head and neck cancer and certain autoimmune diseases. A Phase 1 clinical trial of our gene therapy product candidate, AAV-AQP1, is ongoing in patients who have survived cancer free for five or more years following treatment for head and neck cancer and are suffering from grade 2 or 3 radiation induced late xerostomia, or RIX. There are approximately 170,000 grade 2 or 3 RIX patients who have survived two or more years after radiation treatment for head and neck cancer in the United States, with approximately 10,000 new cases each year. We also intend to initiate a Phase 1/2 clinical trial of AAV-AQP1 for the treatment of patients with chronic xerostomia caused by Sjogren's syndrome, an autoimmune disease affecting more than two million people in the United States.

The FDA has granted orphan drug designation to AAV-AQP1.

Our Neurodegenerative Disease Programs

Neurodegenerative diseases are our third area of focus. Our first target indication is Parkinson's disease, where we have Phase 2 clinical data from a successful randomized, double-blind, sham-controlled trial. Our product candidate targeting Parkinson's disease, AAV-GAD, is designed to deliver the glutamic acid decarboxylase, or *GAD*, gene to the subthalamic nucleus in order to increase production of GABA, the primary inhibitory neurotransmitter in the human brain. GAD is the rate-limiting enzyme in the synthesis of GABA, therefore we believe that increasing subthalamic nucleus GAD expression through gene therapy has the potential to result in normalization of motor circuits and improve symptoms in Parkinson's disease patients without affecting other brain regions, which can be responsible for complications of existing therapies. To date, we have not had any formal meetings with regulatory agencies nor determined the regulatory pathway and any potential related development costs for our recently acquired AAV-GAD gene therapy program for Parkinson's disease.

Our second target indication is ALS, where we currently have IND-enabling studies ongoing. We believe our approach to treating ALS patients is differentiated because, rather than targeting a specific genetic defect that defines a small subset of ALS patients, we aim to target the underlying cell biology driving motor neuron death in ALS, potentially enabling us to treat a broader patient population that includes both sporadic and inherited forms of the disease. Increasing evidence suggests a critical role of RNA metabolism in neuronal cells, in particular in motor neurons that are specifically affected in ALS. We believe that dysregulation of neuronal RNA processes results in the degeneration of motor neurons that leads to ALS. Using our viral vector product candidate, AAV-UPF1, we target the central quality control system regulating RNA in motor neurons with the aim of enhancing motor neuron survival in ALS patients.

We have an Alzheimer's disease program that is likewise directed towards the underlying cell biology of the disease, in this case endosomal trafficking, a mechanism cells use to cycle proteins to the cell surface. Over the past decade, evidence has emerged supporting endosomal trafficking dysfunction in neurons as a central process in the early etiology of Alzheimer's disease. In particular, a master regulator of trafficking out of the endosomes, called retromer, has been implicated. We are in the process of identifying what we believe to be the optimal approach to restoring normal endosomal function to the neurons that are the first to be affected in Alzheimer's. In parallel, we are developing and validating biomarkers of endosomal dysfunction and pre-symptomatic Alzheimer's disease. We believe this approach may also provide a framework for treating certain forms of Parkinson's disease that are also associated with endosomal dysfunction.

Our Strengths

In addition to our four ongoing clinical programs, we have a broad pipeline of preclinical programs, core capabilities in viral vector design and optimization, gene therapy manufacturing and a potentially transformative gene regulation technology. Utilizing the following key strengths, we aim to develop, commercialize and expand our portfolio of product candidates.

- **Deep Expertise in Gene Therapy Development:** We believe our expertise in viral vector design, optimization and process development allows us to efficiently advance gene therapy products candidates from preclinical development to cGMP manufacturing and clinical development through commercialization.
- **Potentially Transformative Gene Regulation Technology Platform:** We are developing proprietary technology to enable innovative gene therapy treatments whose expression can be turned on and off with an easily administered small molecule. We believe the capacity for temporal control of gene therapy products has the potential to transform the gene therapy landscape by opening up new treatment possibilities.
- **Manufacturing Capabilities and Capacity:** We have a flexible and scalable cGMP manufacturing facility and production process, which we expect can supply all of our current clinical and preclinical programs through regulatory approval and, should they be approved, provide sufficient capacity for their commercial production.

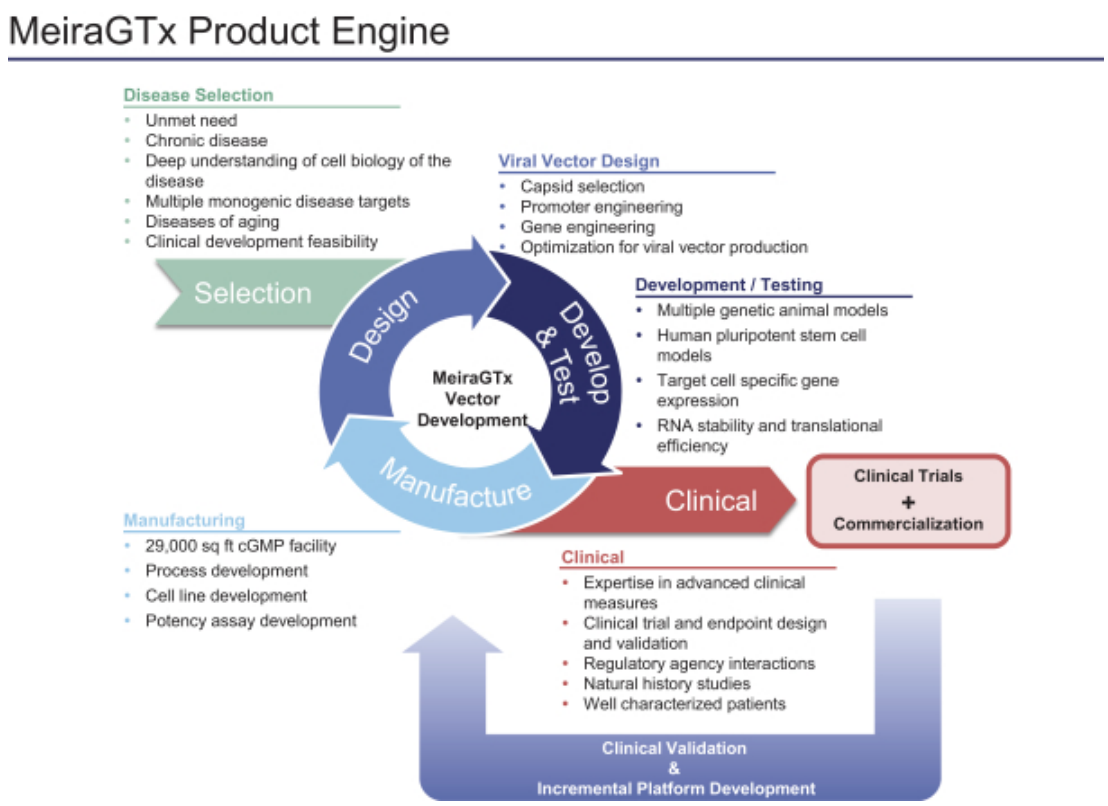
- **Robust and Diverse Clinical and Preclinical Pipeline:** Applying our portfolio approach to gene therapy product development, our initial focus is on treatments for IRDs, salivary glands disorders and neurodegenerative diseases with potential for accelerated approval and has produced five programs in clinical development and multiple preclinical development programs.
- **Relationships with Leading Institutions:** Our longstanding relationships with leading institutions and experts provides us with guidance on development strategy and access to potential patients for our clinical trials.
- **Natural History Study Data:** We sponsor ongoing prospective long-term natural history studies in IRDs that facilitate our ability to efficiently enroll our treatment studies, potentially reducing clinical trial timelines and providing insight into the appropriate endpoints for regulatory approval.

Our Strategy

Our goal is to develop and commercialize innovative gene therapy products to treat serious disorders and broaden the scope of indications that may be treatable by our gene therapies. Our strategy to achieve this goal is to:

- successfully complete clinical development, obtain regulatory approval and commercialize our pipeline of gene therapy product candidates to treat disorders of the eye and salivary gland;
- continue to advance the development of our pipeline of product candidates for the treatment of neurodegenerative disorders;
- utilize our viral vector design and optimization capabilities to identify and develop new gene therapies for other serious diseases;
- advance the development of our potentially transformative proprietary technology for regulating the activity of gene therapy products using small molecules and initiate clinical trials of new product candidates; and
- continue to pursue and evaluate further strategic collaborations with additional biotechnology and pharmaceutical companies to leverage our capabilities, manufacturing capacity and proprietary gene regulation technology.

The figure below depicts the steps in our product engine, including disease selection, design, development/testing, clinical and manufacturing:



Gene Therapy Overview

Gene therapy uses a delivery vehicle, referred to as a vector, to insert a functionally active gene into cells in the body. The gene encodes a therapeutic protein that may block disease pathways or may enhance a deficient pathway. Gene therapy has been studied for over 50 years, with a variety of different viral vectors employed to deliver therapeutic genes. Since the first clinical study of therapeutic gene transfer in humans in 1990, more than 2,300 gene therapy studies covering a broad range of disease targets have been initiated. Recently, the first gene therapies have received regulatory approval, including approval by the FDA of Luxturna for an ophthalmology condition, resulting in a growing acceptance of gene therapy technology as a potentially safe and effective therapeutic approach.

Our current programs use the adeno-associated virus, or AAV, as the vector for delivering gene sequences into a patient's cells. The key components of an AAV vector include: (i) the capsid, or the outer viral protein shell that encloses the target DNA, which is responsible for binding to the cell surface and allowing the therapeutic gene that it is carrying to enter the cell; (ii) the therapeutic gene, or transgene, that encodes the therapeutic protein; and (iii) the promoter, or the DNA sequence that drives the expression of the transgene. AAV is a good vector for gene therapy delivery because of its relative safety and broad applicability. AAV is less immunogenic, or less prone to causing an immune reaction, than previous generations of gene therapy vectors, such as adenoviral vectors and AAV does not readily integrate into the genome of the target cell, reducing the potential for oncogenesis, or the induction of cancer. AAV vectors can transfer a therapeutic gene into, or transduce, numerous cell types. Slight differences in capsid proteins can modulate the efficiency with which

different capsids deliver genes to different cells, thus allowing different AAV capsids to be selected to most effectively target particular cell types.

The therapeutic gene sequence that enters the targeted cell includes both the protein coding region and an engineered promoter sequence that is used to drive functional gene expression. These engineered promoters may be designed to drive different levels of gene expression or to limit gene expression to specific cell types. Additional aspects of the transgene sequence may be engineered for optimal gene expression, such as codon usage and synthetic introns, which may enhance levels of therapeutic protein expression.

Gene therapy can be used to address monogenic diseases, which result mutations in a single gene in a patient's genome. In such cases, the viral vector is used to deliver a normal copy of the gene to the cells that are defective due to the lack of the gene function. The normal gene then drives production of the missing protein and offers a therapeutic benefit in patients with the disease. This gene replacement approach underlies all of our clinical IRD programs.

Rather than replacing a gene that is defective or missing in a monogenic disease, gene therapy can also provide a therapeutic impact by adding a particular new gene function to cells and thereby change cell behavior and function. This is the aim of our salivary gland programs, where our treatment is designed to promote water to flow through otherwise impermeable cells in damaged salivary glands and increase saliva flow into the mouth. Additionally, gene therapy may be used to deliver a therapeutic protein that may block a disease pathway or enhance a deficient cellular pathway in multifactorial diseases such as wet AMD and neurodegenerative diseases, including ALS and Alzheimer's disease.

Importantly, AAV vectors enable targeting of therapeutic genes to non-dividing cells, in which they are thought to remain for the rest of the cell's life. This means that a single treatment may offer patients a durable effect and long-term benefit. The specific cells of the eye, salivary gland and the neurons that we target in our current gene therapy programs are largely non-dividing cells and preclinical evidence has shown that they can be effectively targeted by the specific AAV capsids that we use, enabling us to potentially achieve a durable impact on each of the diseases that we treat.

Our Ophthalmology Programs

Overview and Strategy

We currently have three ongoing Phase 1/2 clinical programs targeting IRDs, including ACHM related to mutations in *CNGB3*, XLRP-RPGR and *RPE65*-deficiency, with an additional program expected to enter a Phase 1/2 clinical trial in 2019 in patients with ACHM related to mutations in *CNGA3*. We also have a product candidate that was manufactured and released for compassionate use under a specials license in the UK to treat LCA4 patients. We chose diseases of the eye as our first area of clinical focus because we believe the eye is ideally suited for gene therapy for the following reasons.

- The eye is easily accessible and has highly compartmentalized anatomy, which allows for accurate delivery of vectors to specific tissues using direct visualization and microsurgical techniques.
- The structure of the eye allows for efficient delivery to specific cell subtypes with small volumes of vector, making the dose per patient much lower than for systemic treatment.
- Anatomical barriers and unique structure of the eye make the immune response to the intraocular administration of vectors more attenuated than systemic administration.
- Largely non-dividing cell populations in the eye make good targets for potentially stable, long-term gene delivery and expression.
- The retina, a structure in the back of the eye, is visible and there are many well validated structural and functional readouts allowing the detailed assessment of the therapeutic impact of the gene therapy treatment.

Our strategy for developing gene therapies targeting eye diseases is to begin with a number of monogenic IRDs that are good candidates for gene replacement therapies and expand to more common eye diseases over time. We have taken a portfolio approach to the development of IRDs because, while some of these genetic defects are rare, IRDs as a class are one of the most common causes of blindness in working age adults and there are multiple synergies at the clinical, regulatory and commercial levels between many of these diseases caused by different gene mutations.

Two of our clinical-stage product candidates are targeting IRD indications in achromatopsia caused by mutations in the *CNGB3* gene and inherited retinal dystrophy caused by mutations in the *RPE65* gene. The primary defect in these particular genetic disorders is the absence of function of one of the two different types of photoreceptors that constitute the light sensing part of the retina, rods or cones. In achromatopsia, the cones completely lack function, while in *RPE65*-deficiency it is the rods that lack function. In these indications, we anticipate that restoring a normal copy of the mutant gene to the retina of patients with these genetic disorders may restore function to the particular type of photoreceptor that is defective in each of these indications. Although cone photoreceptors are generally preserved during childhood in *RPE65*-deficient patients, the lack of healthy active rods eventually affects the cones that they support and over time both rods and cones die, the retina degenerates and *RPE65*-deficient patients become totally blind.

We also have a clinical-stage product candidate for XLRP-RPGR, in which all photoreceptors, both rods and cones, function poorly, leading to degeneration of the retina and total blindness. We have used data and learnings from our *RPE65* studies to inform the design and techniques used in our XLRP-RPGR program. The aim of our XLRP-RPGR treatment is to slow the degeneration of the retina, which we measure using validated surrogate structural endpoints and changes in the detailed structural maps of these patients' retinas over time.

In order to expand our gene therapy pipeline for retinal diseases, we are also developing treatments for certain multifactorial eye diseases, which are diseases caused by multiple genetic or environmental factors. We have a preclinical program using an anti-VEGFR2 antibody targeting wet AMD and we are currently conducting IND-enabling studites for this program. We are also working to integrate our gene regulation technology with our wet AMD program. Our ultimate aim is to activate the anti-VEGFR2 antibody gene that we have installed in the eye with a small molecule applied topically in an eye drop. This could potentially allow for intermittent dosing of the antibody using an eye drop rather than injection. Additionally, we are developing a novel approach to treat advanced dry AMD patients who have lost central vision through our innovative "rod-to-cone" technology. By genetically engineering rods with molecules that will improve their speed of response to light, we aim to effectively transform a patch of rod photoreceptors in the outer part of the retina to behave more like cone photoreceptors, thus improving vision.

We intend to leverage our platform to take advantage of the many synergies across our ophthalmology programs, including identification, diagnosis and characterization of patients, specialized surgical techniques, clinical and regulatory process, vector production and cGMP manufacturing, as well as commercial synergies, if these product candidates are approved by the FDA and other regulatory authorities.

Our Competitive Advantage: Natural History Studies, Relationships with Leading Institutions and Our cGMP Manufacturing Facility

IRDs as a class are the most common cause of blindness in the working age population worldwide and a leading cause of impaired vision in children in developed countries. There are approximately 200,000 people in each of the United States and European Union affected by IRDs. However, IRDs may be caused by mutations in over 200 identified genes, and in many cases each genetically defined IRD may be a small patient population. Meaningful clinical trials for these sorts of rare indications are especially challenging because they require access to sufficient patients and baseline data on each patient in order to secure clear indicators of efficacy as a result of intervention. We seek to address this problem by sponsoring prospectively designed natural history studies in each of the indications that we are treating in our Phase 1/2 trials.

The lead investigator for the natural history studies is Michel Michaelides, Professor of Ophthalmology at Moorfields Eye Hospital in London. For each of the natural history studies, baseline assessments are made upon enrollment, with follow up assessments at six-month intervals for two years and annual assessments for up to an additional three years. A broad range of assessments are used, including functional tests, retinal imaging and electrophysiological assessments. The same assessments used for each natural history study are used in our corresponding clinical trial targeting the same indication, allowing us to compare the impact of our product candidates on the progression of these diseases on a population, as well as individual patient basis.

We expect the natural history studies will enhance our understanding of disease progression for each indication that we are targeting and allow us to identify optimal windows for intervention, provide specific functional and structural parameters to quantify treatment effects and define clinical endpoints. These studies also provide us with a source of potential patients for our treatment studies and have facilitated efficient enrollment of these studies. These patients are not only genotyped, but also have up to five years of detailed functional and structural assessment data prior to enrollment into an appropriate treatment study.

We also have longstanding active relationships and clinical site agreements with leading institutions in retinal disorder treatments, including Moorfields Eye Hospital in London, the University of Michigan Kellogg Eye Center, Massachusetts Eye and Ear, the Medical College of Wisconsin & Froedtert Hospital and the Casey Eye Institute at the Oregon Health & Science University. Our relationships with these institutions, in most cases, precede the date on which we entered into a clinical site agreement with the applicable institution. Professor Robin Ali, Ph.D., our Head of Preclinical Ophthalmology and one of our founders, is Professor of Human Molecular Genetics at UCL Institute of Ophthalmology and Theme Leader for Gene Therapy at NIHR Biomedical Research Centre for Opthalmology, Moorfields Eye Hospital in London, and a Visiting Professor at the University of Michigan Kellogg Eye Center. These institutions are among the premier treatment centers for the indications that we are pursuing and provide us with access to potential patients for our clinical trials and experts in IRDs who offer strategic guidance and expertise for our development strategy. These institutions provide services with respect to our preclinical and clinical studies. For example, all of our IRD clinical trials are being conducted at Moorfields Eye Hospital in London, University of Michigan Kellogg Eye Center and Massachusetts Eye and Ear Hospital in Boston. Participants enrolled at the University of Michigan Kellogg Eye Center and Massachusetts Eye and Ear Hospital may travel to the Medical College of Wisconsin & Froedtert Hospital for adaptive optic assessments. The Casey Eye Institute at the Oregon Health & Science University provides certain reading center and other clinical services with respect to our clinical trials. Achromatopsia

Disease Background and Market Opportunity

Achromatopsia, or ACHM, is an IRD that specifically prevents cone photoreceptors from functioning. ACHM patients are legally blind from birth and usually suffer from severely reduced visual acuity of 20/200 or worse, a disabling sensitivity to light, or photoaversion, total color blindness and involuntary back and forth eye movements, or nystagmus.

Cones and rods are the two kinds of photoreceptors in the human eye. Photoreceptors are light-sensitive cells that absorb light and convert it into an electrical signal that is transmitted to the brain for the perception of light, or vision. Rods can detect very low levels of light and are quickly inactivated in higher light levels, enabling vision in dim light. Cones, on the other hand, remain active in high light levels and support daytime vision and the perception of color. Even though only 5% of the photoreceptors in the eye are cones, they are concentrated in the central part of the retina, the macula, where they are tightly packed, thus enabling high-acuity daytime vision. The highest cone density is in the centermost spot of the macula, called the fovea. Since cones are essential for central visual acuity, color vision, and most daily visual activities, ACHM patients suffer significant vision loss due to the complete lack of cone function.

ACHM occurs in approximately one in 30,000 people in the United States. To date, mutations of any one of six genes encoding components of the light sensing machinery of cone photoreceptors have been

identified as causing ACHM. The *CNGB3* and *CNGA3* genes are the two most common of these genes, together accounting for up to 92% of ACHM cases, with *CNGB3* slightly more common than *CNGA3* in most geographic territories. Together, the proteins encoded by the *CNGB3* and *CNGA3* genes make up the cone-specific cyclic nucleotide gated, or CNG, channel, which is essential for cones to produce an electrical signal in response to light. Mutations in either of these genes prevent the formation of the CNG channel.

There are estimated to be approximately 12,000 patients with ACHM caused by mutations in *CNGB3* in the United States, Japan and Germany, France, Spain, Italy and the United Kingdom, or the EU5, with about 25% of those patients being under the age of 18 and approximately 125 new cases being diagnosed annually. We believe the availability of a therapeutic option may increase patient identification and the estimated prevalence of ACHM.

Our Gene Therapy Program

We have designed specifically optimized gene therapy viral vector candidates to treat ACHM caused by mutations in each of *CNGB3* and *CNGA3*, with which we aim to address the majority of patients suffering from ACHM. Our product candidates are delivered via subretinal injection covering the central macula region of the eye, where most of the cones in the retina are located.

ACHM is predominantly a stationary disease, which means that ACHM patients' retinas contain non-functioning cones that survive intact for many decades. This is in contrast to many IRDs in which the entire retina slowly degenerates over a patient's life. This extended survival of cones with their potential for light sensitivity presents a wide window of opportunity to introduce a normal copy of the mutated gene via a gene therapy product candidate and thereby restore cone function. While the stationary nature of ACHM means that cones remain present for decades, the functional connections between active cones and the visual cortex in the brain are thought to become fixed in teenage years. Therefore, we believe that younger individuals are likely to benefit most from gene therapy treatment for ACHM because of their greater visual plasticity. Another debilitating symptom of ACHM, which lasts throughout life, is photoaversion. A disabling and ubiquitous symptom of ACHM, photoaversion is the avoidance of light due to discomfort in the presence of levels of light equivalent to a normally lit room or daylight. ACHM patients often avoid light and wear dark glasses, which further diminishes their already very poor vision. We believe it is possible that restoration of cone function in adult patients might have an impact on photoaversion even if brain plasticity is limited.

We believe that gene therapy treatment for ACHM in which we aim to restore cone function via a gene replacement strategy may offer benefits across a range of ages, which we aim to define in our clinical development programs.

ACHM Caused by Mutations in CNGB3

With our collaborators at the University College of London's Institute of Ophthalmology, or UCL IO, led by Professor Robin Ali, we have developed a product candidate to treat ACHM caused by mutations in the *CNGB3* gene. Mutations in the *CNGB3* gene prevent cone photoreceptors from functioning because *CNGB3*'s gene product is integral to the formation of a specific membrane channel that enables cones' electrical response to light. *CNGB3* is a gene exclusively expressed in cones and our aim is to replace the absent function of the mutant *CNGB3* gene with a normal copy of the gene in cones of IRD patients and thereby restore cone function. In order to drive expression of the functional gene specifically in cones and not in other cells of the retina, we use the cone specific human cone arrestin, or CAR, promoter to drive the expression of a codon optimized *CNGB3* cDNA. Codon optimization improves protein expression by increasing translation efficiency. To transfect cone photoreceptors, we use the AAV8 capsid, which enables the efficient delivery of the *CNGB3* gene cargo to those photoreceptors. As the vast majority of the cones in the eye are located centrally and concentrated in the macula, we treat this central region of the retina through subretinal injection to deliver the viral vector product candidate to the photoreceptors in which its activity is required.

Preclinical studies in mice lacking the *Cngb3* gene were carried out by our collaborators at UCL IO and led by Professor Robin Ali in 2010 and 2011 and the data was published in *Human Molecular Genetics* in 2011. In these studies, it was observed that delivery of our *CNGB3* product candidate, AAV-CNGB3, via subretinal injection at doses between $6E^9$ and $8E^9$ viral genomes per eye, or vg/eye, was associated with a restoration of cone function to near normal levels.

In one of these studies, retinal response to light was measured in three groups of mice: *Cngb3* mutant mice treated with AAV-CNGB3, untreated *Cngb3* mutant mice and normal mice. Mice were treated with AAV-CNGB3 vector dosed at $2E^{12}$ vg/mL ($8E^9$ vg/eye) via subretinal injection. One eye was treated per mouse. Vector was administered when mice were 30 days old, and electrical responses, or ERGs, of the retina were measured 90 days following administration of the vector.

Figure 1 shows representative ERG responses of the retina to different light stimuli: a single flash of bright light (1A; 1B; 1C) and repeated pulses, or flickers, of bright light at different frequencies to assess the refresh rate of the photoreceptors (1A'; 1B'; 1C'). Retinas from three different groups of mice were tested: retinas of normal mice (Figure 1A), retinas of mutant mice lacking the *Cngb3* gene (Figure 1B) and retinas of mutant mice lacking the *Cngb3* gene, but treated with AAV-CNGB3 (Figure 1C). It was observed that the response to a bright light pulse was largely absent in the retinas of *Cngb3* mutant mice, as this response is largely mediated by the cones and is therefore severely impacted by the *Cngb3* mutation (Figure 1B). It was also observed that treatment of *Cngb3* mutant mice with AAV-CNGB3 was associated with a high-degree of restored cone function in these mutant mouse retinas in response to a single flash of bright light (Figure 1C). In addition, we tested the response of cones to rapid flickers of bright light (Figure $1A^1$) because only cones can register multiple sequential signals from rapid flickers. It was observed that registering rapid flickers was also impacted by the *Cngb3* mutation (Figure $1B^1$) and the cone flicker response was nearly completely restored in the retinas of *Cngb3* mutant mice following treatment with AAV-CNGB3 (Figure $1C^1$).

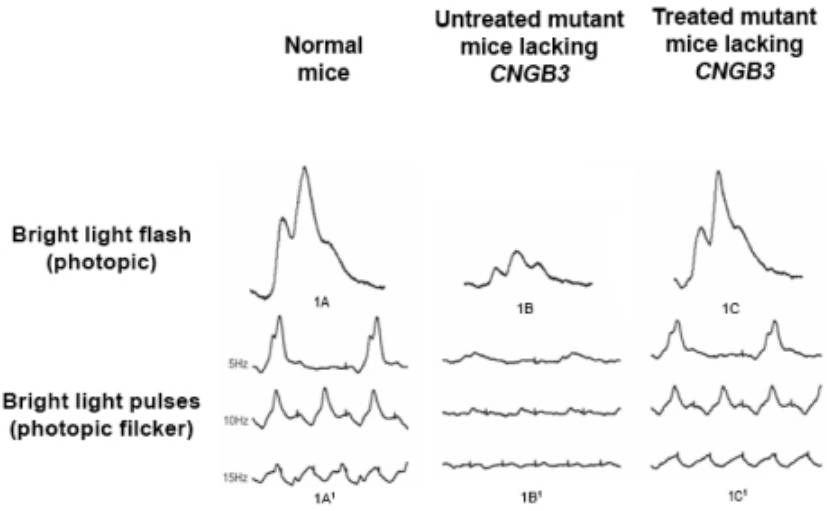


Figure 1. Electrical responses of the retina reacting to two different light stimuli: a single flash of bright light and repeat pulses of bright light at increasing frequency, or flickers.

Figure 1A and $1A^1$. The electrical responses of the retina from a normal mouse reacting to a flash of bright light and flickers.

Figure 1B and $1B^1$. The electrical responses of the retina from a mutant mouse lacking *Cngb3* reacting to a flash of bright light and flickers.

Figure 1C and 1C1. The electrical responses of the retina from a mutant mouse lacking *Cngb3* treated with AAV-CNGB3 reacting to a flash of bright light and flickers. Treatment with AAV-CNGB3 was associated with a high degree of restoration of cone function, with the electrical response to a bright flash of light and rapid flickers nearly matching the response observed in the normal mouse retina.

In another one of the preclinical studies carried out by our collaborators at UCL IO, it was assessed whether functional improvement at the retinal level of *Cngb3* mutant mice was impacted by the age of the mice at the time treatment with the gene therapy product candidate. In this study, *Cngb3*-mutant mice were treated with AAV-CNGB3 at 30 days old, the P30 group, or 180 days old, the P180 group. One eye was treated in each mouse via subretinal injection dosed at $2E^{12}$ vg/mL ($8E^{9}$ vg/eye). Optomotor measurements were taken 60 days after treatment of the P30 group of the *Cngb3* mutant mice treated with AAV-CNGB3 (n=11), untreated *Cngb3* mutant mice (n=11) and normal, age-matched untreated mice (n=5). Optomotor measurements were also taken 60 days after the treatment of the P180 group of the *Cngb3* mutant mice treated with AAV-CNGB3 (n=6), untreated *Cngb3* mutant mice (n=6) and normal, age-matched untreated mice (n=5). Optomoter assessments were made by placing each mouse on a pedestal located in the center of four inward facing computer screens, that projected a rotating image. The head-tracking responses of each mouse was observed using an overhead infrared video camera. Visual acuity was measured by involuntary reflex head-tracking responses driven by the left (clockwise rotations, black arrow) and right (counter-clockwise rotations, white arrow) eyes, respectively (Figure 2A).

Figure 2B is a graph comparing the visual acuity as measured by the average optomotor assessment of the normal eyes, eyes from *Cngb3* mutant mice treated with AAV-CNGB3 and eyes from *Cngb3*-mutant mice that were untreated. It was observed that *Cngb3* mutant mice had a significantly lower visual acuity than normal mice. However, after treatment with AAV-CNGB3, visual acuity in the *Cngb3* mutant mice eyes treated at 30 days old was observed to be restored to a similar level as that observed in normal mice. In contrast, no significant difference in visual acuity between the treated and untreated eyes was observed when treatment was given at 180 days old. Data from this study suggests that treatment received at an older age is less capable of restoring visual acuity.

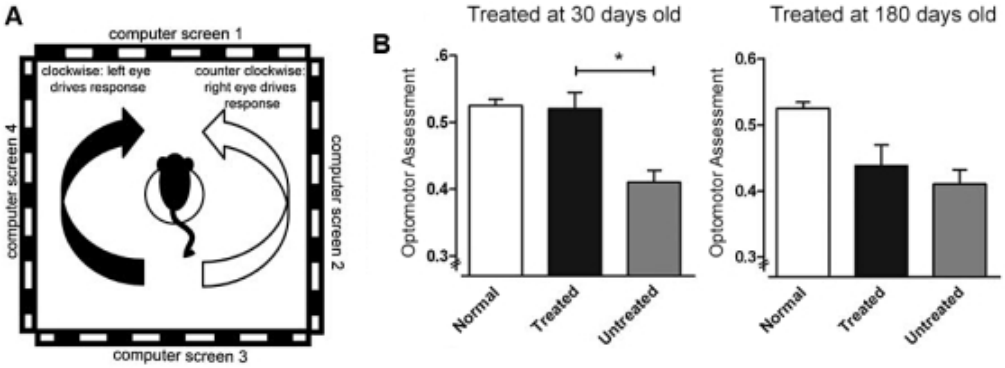


Figure 2A. Depiction of how optomotor assessments were taken. Each mouse was placed on a pedestal located in the center of four inward facing screens and was observed by an overhead infrared video camera. The assessments were made 60 days following treatment of the *Cngb3* mutant mice with AAV-CNGB3 at 30 days old and 180 days old.

Figure 2B. Graph showing averaged optomotor assessment of each group. Visual acuity, as measured by the optomotor assessment, was observed to be restored to normal levels in the mouse treated at 30 days old (left panel) but not in the mouse treated at 180 days old (right panel).

Between October 2016 and December 2016, we carried out a dose-ranging preclinical study at UCL IO to determine the efficacy of different doses of AAV-CNGB3 in rescuing cone response to a single pulse of bright

light in *Cngb3* mutant mice as measured by electrical responses across the retina, or ERG. The study was conducted to support a planned Phase 1/2 clinical trial in patients with *CNGB3* mutations following the FDA's request for additional rescue data at a range of different doses. While long-term functional rescue was observed in prior tests, there was no dosage titration tested in those studies. The data from this study has not been published.

We assessed cone-mediated ERG responses in *Cngb3* mutant mice at three doses and compared these responses to baseline recordings of untreated eyes at two different time-points following treatment. *Cngb3* mutant mice were administered AAV-CNGB3 via subretinal injection of 4uL of vector at each dose. Mice were treated at four weeks of age and ERG responses were measured in these mice at four weeks and eight weeks following treatment. Three eyes were treated at each of the three doses, and three eyes were untreated. The reviewer analyzing the ERG traces was masked to which eye received treatment.

Figure 3 shows the photopic ERG amplitudes measured to assess cone photoreceptor function in *Cngb3* mutant mice treated with AAV-CNGB3 at $1E^{11}$ vg/mL ($4E^{8}$ vg/eye; n=3), $3E^{11}$ vg/mL ($1.2E^{9}$ vg/eye; n=3) and $1E^{12}$ vg/mL ($4E^{9}$ vg/eye; n=3), and untreated eyes (n=3) at four and eight weeks post administration. A photopic ERG is the electrical response of the retina corresponding to cone electrical activity following a single pulse of bright light (single ERG recordings are shown in Figure 1). The higher the ERG amplitude, the greater the response of the retina to bright light. These data show a statistically significant increase in cone ERG responses for all three doses of AAV-CNGB3 and for both time-points following vector administration except for low dose at four weeks post injection (p=0.051). These data indicate that AAV-CNGB3 is associated with the rescue of the cone electrical response in the retina of *Cngb3* mutant mice in a dose-responsive fashion. While initial responses were observed in mice four weeks after subretinal injection of the vector, at all three doses significantly stronger ERG responses were observed eight weeks after vector administration, with the maximum ERG response observed at the highest dose eight weeks after administration.

In this study, a dose response in ERG amplitudes from treated eyes was observed, suggesting that increasing amounts of AAV-CNGB3 is associated with greater rescue of responses in *Cngb3* mutant mice. The three doses tested in this study are the same as those doses administered in our AAV-CNGB3 Phase 1/2 clinical trial on a vg/mL of viral vector titer basis.

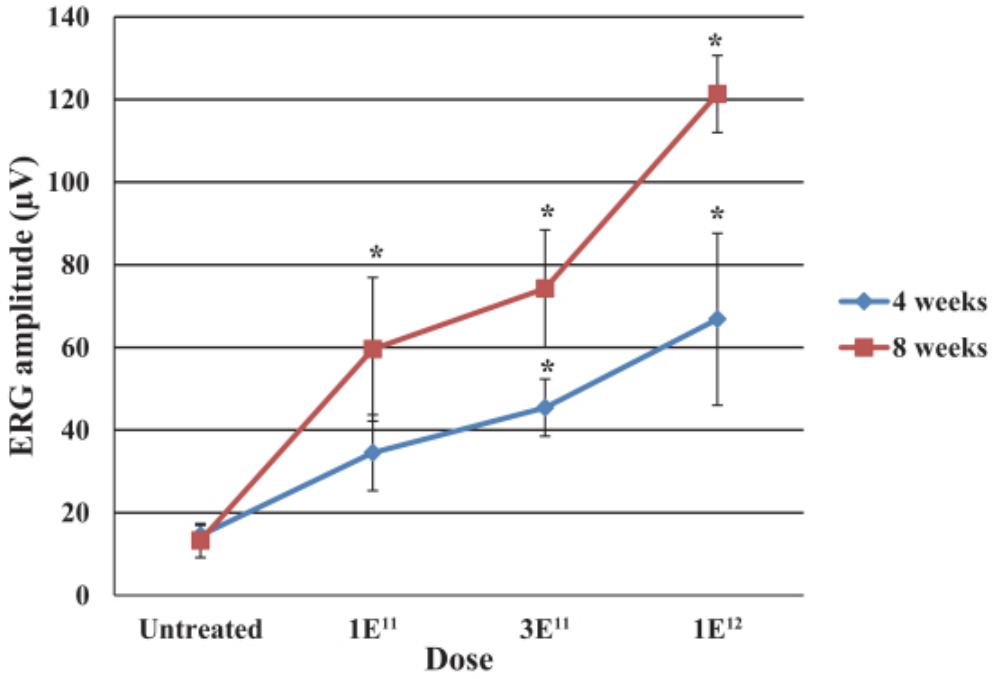


Figure 3. Graph of averaged photopic ERG amplitudes at four and eight week time-points post administration of AAV-CNGB in *Cngb3* mutant mice treated at four weeks of age. The blue line indicates the electrical response amplitudes observed following administration of different doses of AAV-CNGB3 measured at four weeks after treatment. The red line shows the increased level of electrical response observed in the same mice treated with AAV-CNGB3 four weeks later, at eight weeks following treatment. This is compared to the ERG response in untreated eyes, which remained unchanged at both time points.

During 2016, we conducted three preclinical toxicology studies of AAV-CNGB3 at UCL IO. We conducted a nine-month study in normal mice in which four eyes were dosed with saline as a control, nine eyes were dosed at $2E^9$ vg/eye, and nine eyes were dosed at $4E^9$ vg/eye, with a toxicology assessment at one, three, six and nine months post-administration. We also conducted an eight week study in normal mice, in which 15 mice were dosed with saline as a control, 15 mice were dosed at $2E^9$ vg/eye, and 15 mice were dosed at $4E^9$ vg/eye, providing five mice from each group for studying local and systemic effects at each time-point of one, four and eight weeks after treatment. We also conducted an eight week rabbit study in which the right eyes of nine animals were dosed with saline as a control, the right eyes of nine animals were dosed at $0.8E^{11}$ vg/eye and the right eyes of nine animals were dosed at $2.4E^{11}$ vg/eye, providing three animals from each dose group for studying local and systemic effects at each time-point of one, four and eight weeks after treatment. Biodistribution was examined in the eight-week mouse and rabbit studies. No harmful effects on the retina or systemically were observed at the time-points listed after treatment. The data from these studies have not been published. We used these data to support our clinical trial application, or CTA, and IND for treatment of patients with ACHM related to *CNGB3* mutations with AAV-CNGB3.

Clinical Development of AAV-CNGB3

We have an ongoing natural history study in ACHM including over 90 patients that allows us to collect structural and functional data for up to five years on prospectively defined endpoints, including functional tests (visual acuity, contrast sensitivity, mobility maze and photoaversion assessments), retinal imaging (color fundus photography, fundus autofluoresence imaging, spectral domain optical coherence tomography and visual field

testing) and electrophysiological assessments. The study center is the Moorfields Eye Hospital in London. We believe access to these ACHM patients has enabled us to efficiently enroll the most appropriate patients into our *CNGB3* Phase 1/2 clinical trial. We intend to present this natural history data to regulatory agencies for discussion of our pivotal trial design and path to regulatory approval. In addition to giving us access to patients and potentially accelerated enrollment in our treatment studies, we believe the prospective natural history data on each treated patient allow us to gather robust data from our Phase 1/2 clinical trial in a condensed timeframe.

We are conducting a Phase 1/2 clinical trial of AAV-CNGB3 in both adult and pediatric patients. In the dose escalation phase of the trial, up to 18 adult patients (18 years or older) may be administered one of three different doses of vector in dose escalating cohorts of three to six patients at a time. We have treated patients in all three dose cohorts. We have also treated one pediatric patient in the expansion cohort of the trial. We will close out this trial six months after the last pediatric patient has been treated. Six months following treatment, each patient moves onto a long term follow up study in which they are followed for safety and indication of benefit for an additional four and a half years.

The primary endpoint of this open-label, dose-escalation clinical trial is the safety of a one-time subretinal administration of AAV-CNGB3 in patients with ACHM caused by *CNGB3* mutations. Secondary endpoints include the outcomes of a range of functional assessments and detailed structural analysis of the retina, including imaging of individual photoreceptors.

This trial is open in the United Kingdom and the United States. We have treated 11 adult patients, including three patients at the highest proposed dose, and nine pediatric patients.

We submitted our IND for AAV-CNGB3 to the FDA in the fourth quarter of 2017. The IND was initially not opened because we received a question from the FDA regarding our injection device compatibility assay, thus putting the IND on clinical hold until we satisfactorily answered the FDA's question and the clinical hold was lifted by the FDA. In the device compatibility assay the FDA noted a disparity between the target titer for the intended low dose dilution and the actual titer obtained on polymerase chain reaction, or PCR, analysis. The FDA requested clarification on whether this was an imprecise dilution scheme for the low dose or a PCR assay issue. We submitted a response to the FDA on May 2, 2018 providing data that identified the issue as a PCR assay artifact and also showing data that we believe supports that this has now been addressed. On May 31, 2018 the FDA released the clinical hold on our IND for AAV-CNGB3.

In this trial, AAV-CNGB3 viral vector is delivered via subretinal injection of up to 0.5mL targeting the central region of the retina, including the macula and fovea, where most of the cones are located. One eye is treated in each patient, which is the eye assessed to be the worst eye at baseline. We completed the dose escalation phase of our Phase 1/2 clinical trial, with 11 adult patients treated. These 11 patients were dosed in three cohorts with escalating doses of $1E^{11}$ vg/mL (cohort one), $3E^{11}$ vg/mL (cohort two) and $1E^{12}$ vg/mL (cohort three) of AAV-CNGB3. In addition, nine pediatric patients have now been treated in the extension phase of the study.

In each of the 11 adult patients treated and nine pediatric patients treated, the macula, including the fovea, was covered by the subretinal injection. Subretinal injections have been administered by three different surgeons at the Moorfields Eye Hospital in London and the University of Michigan Kellogg Eye Center in the United States.

In adult and pediatric *CNGB3* patients, treatment of the central retina with our AAV-CNGB3 product candidate via subretinal injection has been generally well tolerated. Data from the first adult treatment cohort along with our preclinical data of AAV-CNGB3 was used to support our PRIME designation that was awarded by the EMA in February 2018.

As of December 31, 2018, we had completed dosing of eleven adult patients and nine pediatric patients. We may treat up to four additional patients in the United States during the first quarter of 2019. We

expect to release data from the adult dose escalation cohorts along with preliminary six-month data from the pediatric patients in the second half of 2019.

Our *CNGB3* gene therapy product candidate, AAV-CNGB3, was granted orphan drug designation by the FDA and EMA, rare pediatric disease designation by the FDA for the treatment of achromatopsia caused by mutations in the *CNGB3* gene, and Fast Track designation by the FDA for the treatment of achromatopsia caused by *CNGB3* mutations to improve visual function. We have also been granted PRIME designation by the EMA.

ACHM Caused by Mutations in CNGA3

We are also developing AAV-CNGA3 to treat ACHM caused by mutations in the *CNGA3* gene. While the mechanism for treating *CNGA3* related ACHM is similar to that for *CNGB3*, we believe a larger amount of CNGA3 protein is required to restore cone function compared to CNGB3. We believe this may reflect the 1:3 ratio in which CNGB3: CNGA3 subunits associate to form the cone CNG channel. We have designed a synthetic promoter to drive high levels of CNGA3 expression specifically in cones. In preclinical studies, our novel cone specific promoter has been associated with strong gene expression in all human cone subtypes in human retinas grown *in vitro* using human pluripotent stem cells, or hPSC. AAV-CNGA3 utilizes this proprietary pan cone promoter to drive a codon optimized *CNGA3* gene sequence. We believe this novel promoter can drive sufficient expression of *CNGA3* in cones to restore light sensitivity to these cones in CNGA3 deficient patients. We use the AAV8 capsid to transfect cone photoreceptors in the back of the eye and we target the cones concentrated in the central region of the retina via a subretinal injection that covers the macula.

A key aspect of developing an effective treatment for ACHM caused by *CNGA3* mutations is a promoter that drives robust gene expression in all of the three different cone subtypes, S/blue, L/red and M/green. In particular, it is important to evaluate the activity of the promoter in human photoreceptors, which can be achieved prior to treating patients using human stem cell derived retinas. Our relationship with UCL provides access to what we believe is one of the few centers in the world that can generate human “mini retinas,” or HMRs, which are human retinas grown *in vitro* from hPSCs. These HMRs develop from stem cells over several months to form primary photoreceptors arrayed in a spherical structure that resembles many aspects of the human retina and includes all the rod and cone subtypes, allowing us to assess and predict the utility of many aspects of gene therapy vectors for human clinical application.

In vitro studies to measure the gene expression driven by our cone specific promoter in human cones using HMRs were conducted at UCL IO during 2017. Figure 4A shows a fully formed HMR that has been transfected with a construct in which our cone specific promoter was associated with the expression of green fluorescent protein, or GFP, a green marker gene. The expression of GFP is seen specifically in cone photoreceptors throughout this HMR, indicating that our promoter is driving robust gene expression in human cones. To support that this promoter drives gene expression in all cone subtypes, GFP expression driven by our promoter was overlaid with markers specific to each of the cone types. Figure 4B is an image of an S/blue cone and Figure 4C is an image of an L/red cone and an M/green cone, with the cone-specific marker labeled in red in each case. The multiple colors in Figures 4B’ and 4C’ show the overlay of these cone-specific markers with cone specific GFP expression, indicating that our promoter drives gene expression in all cone subtypes in this HMR. The data from these studies have not been published.

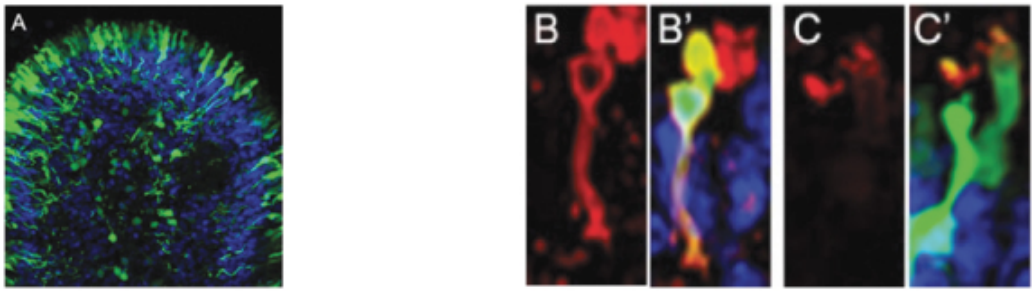


Figure 4A. An hPSC derived HMR showing GFP expression (green) driven by our promoter in human cones throughout the HMR.

Figure 4B. The red marker identifies this cone as an S/blue cone.

Figure 4B'. An overlay of GFP expression from our promotor on top of the S/blue cone specific marker. The overlap between the cone marker and GFP expression indicates that our promoter drives gene expression in S/blue cones.

Figure 4C. The red marker identifies M/red and L/green cones.

Figure 4C'. An overlay of GFP expression from our promotor on top of the M/Red and L/green cone specific marker. The overlap between the cone marker and GFP expression indicates that our promoter drives gene expression in M/red and L/green cones.

In preclinical studies of mutant mice lacking the *Cnga3* gene conducted at UCL IO during 2017 and 2018, treatment of retinas of *Cnga3* mutant mice with AAV-CNGA3 was associated with restoration of the cone electrical response following subretinal delivery. Figure 5 and Figure 6 show the rescue of cone function that was observed in two of these studies with doses spanning those proposed in the clinical trial and in a dose dependent manner. We believe this supports the evaluation of AAV-CNGA3 to treat patients with ACHM caused by mutations in *CNGA3* in a Phase 1/2 clinical trial. The data from these studies have not been published.

Figure 5 shows the electrical response of retinas to a single flash of bright light. A retina from three different mice were tested: a normal mouse (Figure 5A), a mutant mouse lacking the *Cnga3* gene (Figure 5B) and a mutant mouse lacking the *Cnga3* gene, but treated with 3uL of $1E^{12}$ vg/mL AAV-CNGA3 (Figure 5C). A high degree of restoration of function of mutant cones was observed in the retina from the *Cnga3* mutant mouse treated with AAV-CNGA3, with approximately 60% of the electrical response to a bright flash of light of the normal retina observed in the retina from the *Cnga3* mutant mouse that were treated with AAV-CNGA3. The mice were injected with AAV-CNGA3 at four weeks of age and assessed using ERG four weeks post injection.

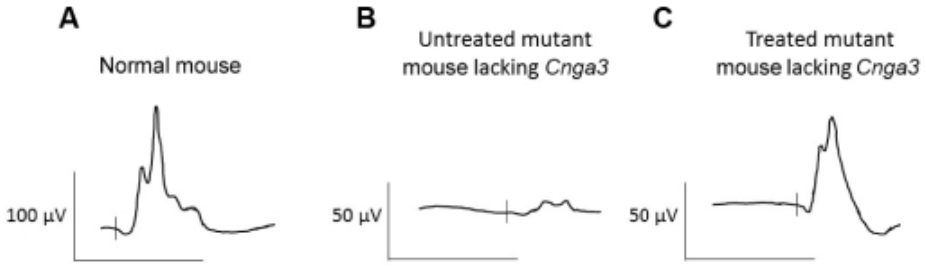


Figure 5. Cone electrical response in mice retinas reacting to a single flash of bright light, measured by ERG.

Figure 5A. The electrical response of the retina from a normal mouse reacting to a flash of bright light.

Figure 5B. The electrical response of the retina from a mutant mouse lacking *Cnga3* reacting to a flash of bright light.

Figure 5C. The electrical response of the retina from a mutant mouse lacking *Cnga3* treated with AAV-CNGA3 reacting to a flash of bright light.

Figure 6 shows the cone-mediated ERG responses measured in *Cnga3* mutant mice treated with three different doses of AAV-CNGA3. 3uL of AAV-CNGA3 was administered via subretinal injection in each eye at $1E^{11}$ vg/mL (n=5), $3E^{11}$ vg/mL (n=5) and $1E^{12}$ vg/mL (n=5). The mice were treated at 15 days old and the ERG was measured four weeks after treatment. The reviewer analyzing the ERG responses was masked to which eye received treatment. Doses tested in this study span the doses of AAV-CNGA3 proposed for our Phase 1/2 clinical trial. Figure 6 shows the ERG responses that were observed at each of the three doses.

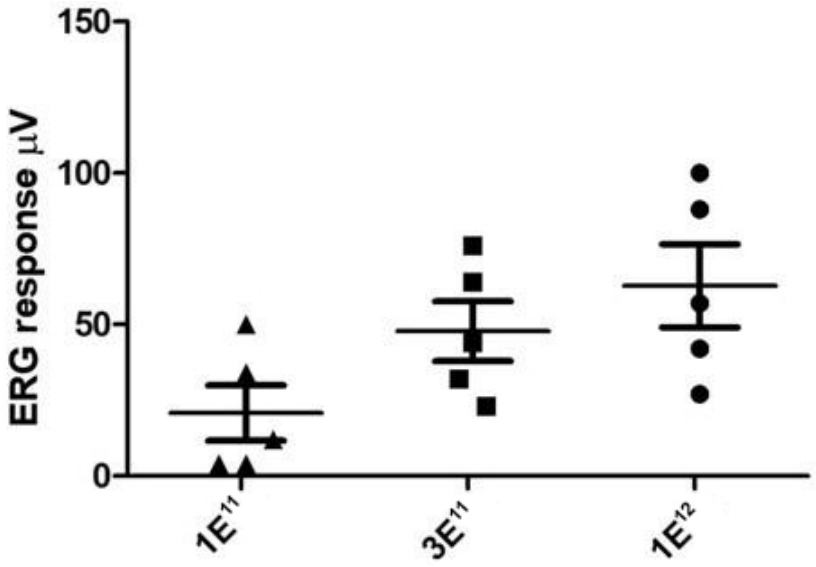


Figure 6. The photopic ERG amplitudes measured to assess cone photoreceptor function in *Cnga3* mutant mice treated with AAV-CNGA3 at doses of $1E^{11}$ vg/mL (n=5), $3E^{11}$ vg/mL (n=5) and $1E^{12}$ vg/mL (n=5). Mice were treated at 15 days post-natal and ERGs were assessed four weeks following treatment. The retinal ERGs are the electrical recordings corresponding to cone activity following a single pulse of bright light. A dose response was observed in retinal sensitivity obtained using the three tested doses and a treatment effect was also observed in the lowest dose tested.

In 2018, we completed a toxicology package similar to that used with the AAV-CNGB3 and AAV-RPGR programs using cGMP AAV-CNGA3 material manufactured at our cGMP facility. We conducted an approximately six-month toxicology study in mice and two, eight-week acute toxicology and biodistribution studies in mice and rabbits.

Clinical development of AAV-CNGA3

We have an ongoing natural history study in ACHM including over 90 patients that allows us to collect structural and functional data for up to five years on prospectively defined endpoints, including functional tests

(visual acuity, contrast sensitivity, mobility maze and photoaversion assessments), retinal imaging (color fundus photography, fundus autofluoresence imaging, spectral domain optical coherence tomography and visual field testing) and electrophysiological assessments. The study center is the Moorfields Eye Hospital in London. We believe access to these ACHM patients will enable us to efficiently enroll appropriate patients into our *CNGA3* Phase 1/2 clinical trial. In addition to giving us access to patients and potentially accelerated enrollment in our treatment study, we believe the prospective natural history data on each treated patient will allow us to gather robust data from our Phase 1/2 clinical trial in a condensed timeframe.

We aim to initiate a Phase 1/2 dose escalating trial of AAV-CNGA3 in ACHM patients with mutations in *CNGA3*. AAV-CNGA3 clinical material is currently being manufactured at our cGMP facility and we expect to release material for this trial in the first half of 2019 and open a Phase 1/2 dose escalation trial in pediatric patients shortly thereafter.

We will present pre-clinical data describing our AAV-CNGA3 vector optimization work in a scientific forum in the first half of 2019.

X-Linked Retinitis Pigmentosa

Disease Background and Market Opportunity

Retinitis pigmentosa, or RP, is a group of IRDs which represent the most common genetic cause of blindness. The condition is characterized by progressive retinal degeneration and vision loss that ends in complete blindness. There are currently no approved treatments for RP. We believe gene therapy is a promising therapeutic approach. RP initially presents as nighttime blindness during childhood or early adulthood, progressing to peripheral visual field loss and “tunnel vision,” central visual impairment, reduced visual acuity and, ultimately, complete blindness.

RP may be caused by mutations in any of over 100 different genes. The most severe forms of RP are X-linked, or XLRP, with onset in early childhood and rapid progression to blindness generally by the time patients reach 30 to 40 years old. RP has an estimated prevalence of one in 3,500 in the United States, and between 10% and 15% of RP patients are estimated to suffer from XLRP. The most frequent mutation causing XLRP is in the retinitis pigmentosa GTPase regulator gene, or *RPGR*. XLRP associated with a mutation in RPGR, or XLRP-RPGR, accounts for more than 70% of cases of XLRP. There are estimated to be approximately 20,000 XLRP-RPGR patients in the United States, Japan and EU5, with a little less than 50% those patients being under the age of 40 and approximately 200 new cases being diagnosed annually. We believe the availability of a therapeutic option may increase patient identification and the estimated prevalence of XLRP-RPGR.

The RPGR protein has an essential role in the maintenance of the structure and function of both rod and cone photoreceptors. The RPGR protein is tightly localized in the photoreceptors at the junction of the inner and outer segments, or cilium, of the photoreceptors. Correct localization of the RPGR protein to this site is important for the transport of light sensing proteins to the outer part of the photoreceptors, where these proteins convert light signals into electrical signals. In the absence of the RPGR protein the movement of light sensing protein within both rods and cones is impaired, leading to dysfunction and death of photoreceptors, resulting in retinal degeneration and ultimately complete blindness.

Our Gene Therapy Program

Our product candidate for the treatment of XLRP, AAV-RPGR, is designed to treat XLRP caused by mutations in *RPGR*, the most common form of XLRP. The eye specific form of RPGR is encoded by a nucleotide sequence called RPGR open reading frame 15, or *RPGR-ORF 15*. Both rods and cones photoreceptors require *RPGR-ORF 15* to function. The protein coding message *RPGR-ORF 15* contains a region of repeated

sequences that make the *RPGR-ORF 15* nucleotide sequence unstable in the cell. We have engineered a small deletion in *RPGR-ORF 15* that we observed was associated with a stable sequence that rescued RPGR protein levels, localization and function in mouse and human photoreceptors in preclinical studies. Our novel AAV-RPGR viral vector utilizes the human rhodopsin kinase, or RK, promoter to specifically drive the expression of our stabilized *RPGR-ORF 15* in both rods and cones. We selected the AAV5 capsid because of its efficient transfection into both of these types of photoreceptors.

In preclinical studies, performed at UCL IO between 2009 and 2015 and discussed in *Gene Therapy* in 2016, it was observed that treatment with AAV-RPGR containing our stabilized *RPGR-ORF15* was associated with rescue of the *Rpgr* mutant phenotype in mice that completely lacked the RPGR protein as measured by ERG responses of retinas from *Rpgr* mutant mice and assessments of RPGR protein. Restored levels, localization and function of RPGR protein in mutant mice lacking the *Rpgr* gene were observed, along with the restoration and localization of photosensitive proteins in both rod and cone photoreceptors, and reduction of long-term retinal degeneration. In addition to these experiments in *Rpgr* mutant mice, we have observed that our *RPGR-ORF 15* construct also rescued levels, localization and function of RPGR protein in human photoreceptors, in HMRs derived from patients with *RPGR* mutations. We believe that AAV-RPGR may have the potential to positively impact human photoreceptor function and survival in XLRP-RPGR patients, slowing or halting the degeneration that leads to blindness in these patients.

Figure 7 and Figure 8 show data from these preclinical studies regarding the effect of subretinal delivery of AAV-RPGR containing our stabilized *RPGR-ORF 15* in mice lacking the *Rpgr* gene. In these studies, different color stains were used to investigate the restoration of (i) RPGR protein expression, (ii) correct localization of RPGR protein and (iii) RPGR function. Figure 7 and Figure 8 show a retina from a normal mouse (7A and 8A), a retina from a mutant mouse strain lacking the gene for *Rpgr* (7B and 8B) and a retina from the same mutant mouse strain that lacks the gene for *Rpgr*, but treated with AAV-RPGR containing our stabilized *RPGR-ORF 15* (7C and 8C).

The red stain in Figure 7 marks a photoreceptor protein, rootletin, within the cilium, or middle section, of every photoreceptor, while the green stain reveals the localization of RPGR protein. The green stain in Figure 7A shows the localization of RPGR protein within the photoreceptor at the end of the cilium in a normal mouse retina. Figure 7B shows a retina from a mouse lacking the *Rpgr* gene without any green staining, indicating the absence of the RPGR protein. Figure 7C shows a retina from a mouse lacking the *Rpgr* gene that was treated with AAV-RPGR containing our stabilized *RPGR-ORF 15*. The green staining at the end of the photoreceptor cilium is similar to the normal mouse retina. Treatment with AAV-RPGR containing our stabilized *RPGR-ORF 15* was associated with the restoration of RPGR protein expression and localization within the individual photoreceptor cells consistent with normal RPGR expression.

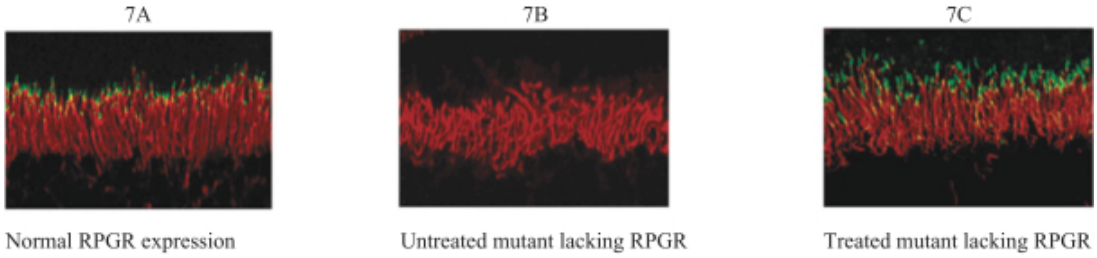


Figure 7. In these preclinical mouse models, the red staining marks a normal protein in the photoreceptor cilia, rootletin, while the green stain reveals localization of RPGR protein within the photoreceptor.

Figure 7A. The retina from a normal mouse.

Figure 7B. The retina from a mouse completely lacking the *Rpgr* gene.

Figure 7C. The retina from a mouse completely lacking the *Rpgr* gene and treated with AAV-RPGR containing our stabilized *RPGR-ORF 15*. The green stain indicates that RPGR protein expression was restored and was similarly localized within the individual photoreceptor consistent with normal RPGR expression.

Figure 8 shows a different analysis of retinas from the same mutant mouse strain that completely lacks the gene for *RPGR* expression. This analysis uses a yellow tag to reveal the location of photoreceptor light pigment, or opsins. The top row shows the rod opsin (8A; 8B; 8C), rhodopsin, and the bottom row shows cone opsin (8A1; 8B1; 8C1). Fully functional RPGR is critical for the correct localization of opsins and Figures 8B and 8B1 show that in a *Rpgr* mutant mouse retina the opsins are incorrectly localized. Figures 8C and 8C1 show that treatment of these mutant animals with subretinal injection of AAV-RPGR containing our stabilized *RPGR-ORF 15* enabled the correct location of the opsins within the photoreceptor as seen in the similarity to the normal mouse, indicating that RPGR is functionally active in supporting correct localization of key photoreceptor proteins.

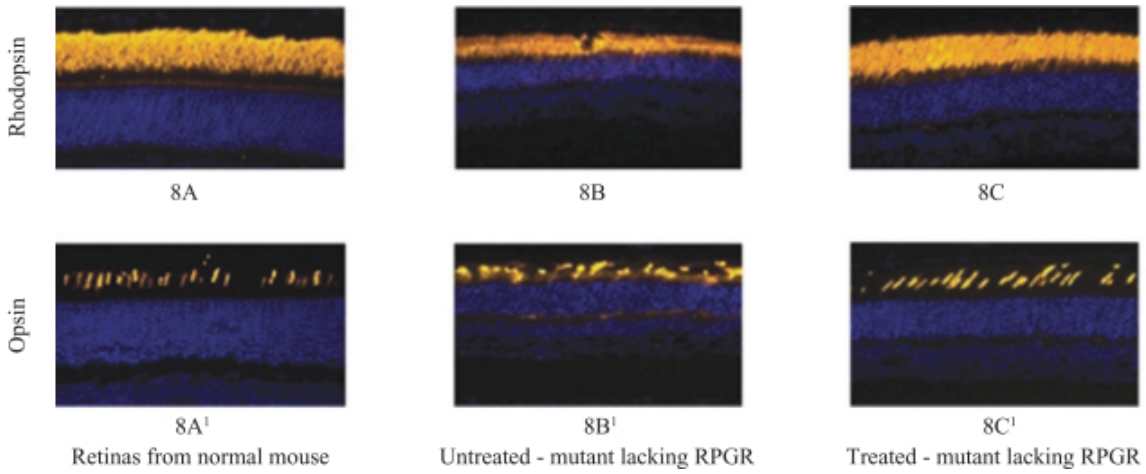


Figure 8. In these preclinical mouse experiments, a yellow tag reveals the location of the opsins.

Figures 8A and 8A1. Normal mouse retina showing the localization of rhodopsin and opsin.

Figures 8B and 8B1. *Rpgr* mutant mouse retina showing the localization of rhodopsin and opsin.

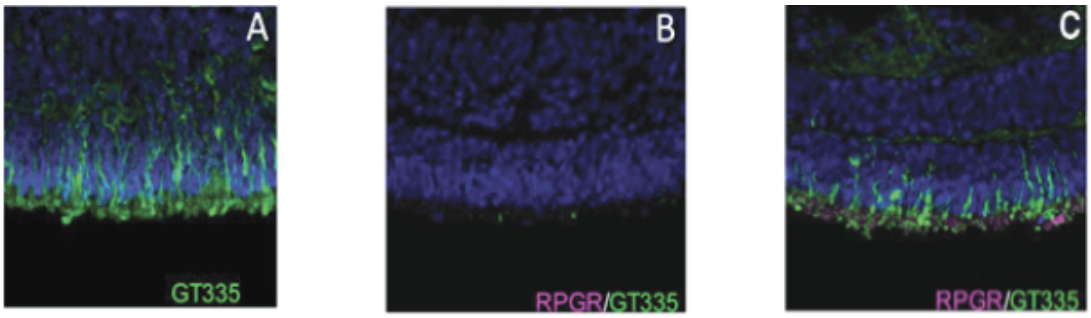
Figures 8C and 8C1. *Rpgr* mutant mouse retina that was treated with AAV-RPGR containing our stabilized *RPGR-ORF 15* shows the localization of rhodopsin and opsin. The similar localization between the normal mouse retina and the *Rpgr* mutant mouse retina that was treated with AAV-RPGR containing our stabilized *RPGR-ORF 15* indicates that the rhodopsin and opsin are correctly localized and can perform their key function in the visual cycle.

We are further advancing the HMR system to evaluate the potential for our product candidates to functionally restore mutant photoreceptor cells in a human retina.

One of the many tasks that *RPGR* performs in the photoreceptor is to enable a process called glutamylation of a key cilia protein called tubulin, which is required for photoreceptor function. We conducted an *in vitro* study at UCL IO in 2016 using HMRs derived from stem cells from XLRP-RPGR patients to determine the effect of AAV-RPGR containing our stabilized *RPGR-ORF 15* on human photoreceptors. The data from this

study has not been published. An HMR grown from hPSC from a normal individual is shown in Figure 9A, in which glutamylation of tubulin in normal human photoreceptors is stained in green. Figure 9B shows a HMR derived from a XLRP-RPGR patient and cultured over several months to form many of the anatomic layers of the retina and consisting of all photoreceptor types, while Figure 9C shows the impact of AAV-RPGR containing our stabilized *RPGR-ORF 15* treatment on a similarly cultured HMR derived from a XLRP-RPGR patient.

The HMR derived from a XLRP-RPGR patient lacks RPGR protein, making the photoreceptors dysfunctional with no glutamylation of tubulin present (Figure 9B). When HMRs from a XLRP-RPGR patient were treated with AAV-RPGR containing our stabilized *RPGR-ORF 15* we observed that glutamylation of tubulin returned (Figure 9C). We also stained HMRs derived from an XLRP-RPGR patient with a pink marker of RPGR protein. In Figure 9B no pink staining was observed, confirming the lack of RPGR protein. In Figure 9C, when the HMR from a XLRP-RPGR patient was treated with AAV-RPGR containing our stabilized *RPGR-ORF 15*, pink staining can be seen at the correct localization in the base of the cilium of the photoreceptors that are now stained with green glutamylation of tubulin marker. This restoration of RPGR protein expression and localization, and restoration of markers of photoreceptor function in human photoreceptors in HMRs derived from XLRP-RPGR patients, indicates potential clinical utility of AAV-RPGR containing our stabilized *RPGR-ORF 15*.



Derived from normal patient

Untreated - Derived from XLRP-RPGR patient

Treated - Derived from XLRP-RPGR patient

Figure 9. A HMR derived from hPSCs from a normal individual (9A) or a XLRP-RPGR patient (9B and 9C) cultured over several months to form many of the anatomic layers of the retina and consisting of all photoreceptor types with the glutamylation of tubulin having a green fluorescent stain.

Figure 9A. A HMR derived from normal hPSCs.

Figure 9B. A HMR derived from a XLRP-RPGR patient.

Figure 9C. A HMR derived from a XLRP-RPGR patient that was treated with AAV-RPGR containing our stabilized *RPGR-ORF 15*. The appearance of pink RPGR protein at the base of the photoreceptor cilium and glutamylation of tubulin returns, demonstrating the restoration of RPGR protein expression, localization and function.

To support the advancement of AAV-RPGR into clinical development, we conducted three single-dose toxicology studies from January 2016 to August 2017 at UCL IO. We performed a six-month toxicology study in normal mice delivering doses of saline in both eyes for the control (n=5), and doses of AAV-RPGR in both eyes of $2E^9$ vg/eye (n=5) and $4E^9$ vg/eye (n=5), with a toxicology assessment at one, three and six months post-administration. Two further toxicology studies were performed. An eight week mouse study in which groups of mice were dosed with saline in both eyes as a control (n=15), AAV-RPGR in both eyes at $1E^9$ vg/eye (n=15) or AAV-RPGR in both eyes at $4E^9$ vg/eye (n=15), providing five mice from each group for studying local and

systemic effects at each time-point of one, four and eight weeks after treatment. An eight-week rabbit study was also conducted, in which nine right eyes were dosed per group, with doses of saline as a control, and AAV-RPGR doses of $0.8E^{11}$ vg/eye and $2.4E^{11}$ vg/eye, providing three rabbits for studying local and systemic effects at each time-point of one, four and eight weeks after treatment. Biodistribution was examined in the eight-week mouse and rabbit studies. No harmful effects on the retina or systemically were observed. We used these data to support our CTA and IND for treatment of XLRP-RPGR patients with AAV-RPGR.

Clinical Development of AAV-RPGR

We have an ongoing natural history study in XLRP-RPGR including approximately 100 patients, which allows us to collect structural and functional data for up to five years on prospectively defined endpoints, including functional tests (visual acuity and contrast sensitivity), retinal imaging (color fundus photography, fundus autofluoresence imaging, spectral domain optical coherence tomography, adaptive optics and visual field testing) and electrophysiological assessments. The study centers are the Moorfields Eye Hospital in London, the Kellogg Eye Center at the University of Michigan, the Medical College of Wisconsin & Froedtert Hospital and Massachusetts Eye and Ear. We believe access to this large population of XLRP-RPGR patients has enabled us to efficiently enroll appropriate patients into our XLRP-RPGR Phase 1/2 clinical trial. In addition to giving us access to patients and potentially accelerated enrollment in our treatment studies, we believe the prospective natural history data on each treated patient will potentially allow us to enroll a more heterogenous population into our treatment studies and may allow us to gather robust data from our Phase 1/2 clinical trial in a condensed timeframe.

RP initially presents as nighttime blindness during childhood or early adulthood, progressing to peripheral visual field loss and “tunnel vision,” central visual impairment, reduced visual acuity and, ultimately, complete blindness. XLRP-RPGR is a progressive disease in which the retina gradually degenerates starting in the outer, or peripheral, regions of the retina and initially causing “tunnel vision” with final degeneration of the central retina resulting in the complete loss of visual acuity and blindness that generally occurs by the time patients are 30 to 40 years old. We believe that to prevent this ultimate degeneration to blindness and to retain visual acuity, the central region of the retina, including the macula and fovea, must be preserved. To this end, we aim to deliver AAV-RPGR to this central region of the retina. In our Phase 1/2 clinical trial, AAV-RPGR viral vector is delivered via subretinal injection of up to 1mL with the potential for the surgeon to use multiple retinotomies targeting the region of the central retina, including the macula and fovea. One eye is treated in each patient, which is the eye assessed to be the worst eye at baseline.

We are conducting a Phase ½ clinical trial of AAV-RPGR in both adult and pediatric XLRP-RPGR patients. This trial is open in the United Kingdom under our CTA and in the United States under our IND.

We have treated ten young adult patients (aged 18 to 30 years old) in the Phase 1/2 XLRP-RPGR clinical trial. We have treated one pediatric patient in the pediatric extension arm of this trial. We expect to treat up to 40 additional patients in the extension cohorts of this study in 2019. In all patients, subretinal injection covered the central part of the retina, including the macula and fovea.

We expect to report preliminary safety and efficacy data from the dose escalation cohorts in the second half of 2019.

The FDA and EMA have granted orphan status to AAV-RPGR for the treatment of retinitis pigmentosa. The FDA has also granted Fast Track designation to AAV-RPGR for the treatment of XLRP caused by defects in *RPGR*.

***RPE65*-Deficiency**

Disease Background and Market Opportunity

RPE65-deficiency causes rod photoreceptor dysfunction and impaired vision from birth. The *RPE65* gene encodes a protein that is produced in the retinal pigment epithelium, or RPE, a thin layer of cells at the back of the eye that support photoreceptors and nourish the retina. The RPE65 protein is essential for rod function because it recycles the light sensing machinery in rod photoreceptors. Absence of RPE65 results in severe dysfunction of rods and causes impaired vision in dim lighting conditions. Although cone photoreceptors are generally preserved during childhood in *RPE65*-deficient patients, the lack of function and degeneration of the rods eventually results in the loss of cones and degeneration of the whole retina over time. Consequently, most *RPE65*-deficient patients experience central vision loss progressing to complete blindness by early adulthood.

RPE65-deficiency is often characterized as a specific subtype of Leber congenital amaurosis or RP that is caused by mutations in the *RPE65* gene, called Leber congenital amaurosis 2 and RP20, respectively. However, we believe a more precise approach to genetic diseases is to focus on the underlying genetic defect, rather than the phenotype, and have therefore identified *RPE65*-deficiency as the target of our product candidate AAV-RPE65.

Based on an estimated prevalence of approximately one in 500,000 people in the United States suffering from Leber congenital amaurosis, or LCA, related to mutations in the *RPE65* gene, and approximately one in 70,000 people in the United States having RP due to mutations in the *RPE65* gene, *RPE65*-deficiency occurs in approximately one in 125,000 people in the United States. There are estimated to be approximately 6,000 *RPE65*-deficiency patients in the United States, Japan and EU5, with almost 30% of those patients being under the age of 30 and approximately 50 new cases being diagnosed annually. We believe the availability of a therapeutic option may increase patient identification and estimated prevalence of *RPE65*-deficiency.

Our Gene Therapy Program

Our gene therapy candidate for the treatment of *RPE65*-deficiency, AAV-RPE65, is an AAV2/5 viral vector, in which a codon optimized *RPE65* gene is driven by a novel synthetic RPE cell specific promoter.

RPE65-deficiency is a well validated condition for gene replacement therapy. In multiple clinical trials, including one conducted by our collaborators at UCL, replacing the mutant *RPE65* gene with a normal copy of the gene resulted in improved nighttime vision in affected children and young adults, suggesting the potential impact of gene therapy on rod function in this disease. The FDA recently approved the first gene treatment for *RPE65*-deficiency, Luxturna. While *RPE65*-deficiency primarily causes a loss of rod function initially leading to impaired vision in dim light, these patients ultimately experience complete blindness because of degeneration of the cone rich fovea. To prevent blindness, therefore, we believe it is critical to treat the central retina in order to maintain structural integrity in this region and save central vision. We aim to treat as extensive an area of the central retina as possible, including the cone rich fovea. Thus, in addition to improving rod function, we aim to provide sufficient RPE65 protein to the cells in the central retina to prevent the degeneration of both rods and cones in this region, and thereby prevent the progression to complete blindness.

We focus on detailed structural and functional data in our treatment studies with the aim of demonstrating an impact on the degeneration of the central retina.

Our novel AAV2/5 vector, AAV-RPE65, has been optimized for both transduction of RPE cells and RPE65 protein production and our surgical approach targets the central area of retina whose preservation is most critical for long term maintenance of visual function. Building on the work of Professor Ali and in collaboration with the team at UCL, we have developed AAV-RPE65. AAV-RPE65 is a second generation viral vector that has compared favorably to our first generation AAV2/2 vector in a number of ways, including being two to three logs, or 100 to 1,000 times, more potent on a particle for particle basis than our original AAV2/2 vector in a head

to head *Rpe65* null animal model rescue experiment. On a logarithmic scale, each "log" represents a 10-fold change. We believe this increased potency will improve transgene expression and RPE65 protein production in the back of the eye. The table below summarizes elements of the optimization of AAV-RPE65 compared to our original AAV2/2 vector. Vector optimization studies were performed at UCL IO and were carried out from 2010 to 2015. The data summarized here was referred to in *Gene Therapy* in 2016. We used these data to support our IND and CTA for treatment of *RPE65*-deficient patients with AAV-RPE65.

Optimization of AAV2/5 compared to AAV2/2	
Transfection Efficiency	Changing the capsid from AAV2/2 to AAV2/5 was associated with an improvement of the transfection efficiency of RPE by four times.
Protein Expression (Promoter)	AAV2/5's optimized promoter was associated with a 20 times increase in protein expression.
Protein Expression (Codon)	AAV2/5's optimized codon usage was associated with a seven times increase in protein expression in human cells.
mRNA Stability	Utilizing SV40 intron increases mRNA stability and was associated with a two and one half times increase in protein production.
Overall Efficacy	The optimized construct was associated with a 300 to 1,000 times increase in efficacy in mouse functional rescue and is expected to be two to three logs more potent on a particle for particle basis in humans.

We conducted four single-dose toxicology and biodistribution studies of AAV-RPE65 from January 2015 to December 2016. We performed a long-term toxicology study in normal mice in which four eyes were dosed with saline as a control and nine eyes were dosed with AAV-RPE65 at $4E^9$ vg/eye, with a toxicology assessment at one, three, six and nine months post-administration. We also conducted an eight-week mouse study, an eight-week rabbit study and an eight-week minipig study. In the mouse study, 15 mice were dosed in both eyes with saline as a control, 15 mice were dosed in both eyes with AAV-RPE65 at $1.2E^9$ vg/eye and 15 mice were dosed in both eyes with AAV-RPE65 at $3.7E^9$ vg/eye, providing five mice from each group for studying local and systemic effects at each time-point of one, four and eight weeks after treatment. In the rabbit study, nine rabbit's right eyes were dosed for each group with a saline control group, a $0.6E^{11}$ vg/eye group and a $1.9E^{11}$ vg/eye group, providing three animals from each group for studying local and systemic effects at each time-point of one, four and eight weeks after treatment. The minipig study was a two-week single dose injection study in minipigs (n=2, each dosed in one eye) at a dose of $1.84E^{10}11$ vg/eye. The minipig study was performed by Charles River Laboratories, while the other three studies were performed at UCL IO. No harmful effects on the retina were observed during these studies. We used these data in support of our CTA and IND for treatment of *RPE65*-deficient patients with AAV-RPE65.

Clinical development of AAV-RPE65

We have an ongoing natural history study in patients with *RPE65*-deficiency with approximately 30 patients enrolled that allows us to collect structural and functional data on prospectively defined endpoints, including functional tests (mobility maze, color vision, visual acuity and quality of life measures), retinal imaging (color fundus photography, fundus autofluoresence imaging, visual field testing, spectral domain optical coherence tomography and adaptive optics) and electrophysiological assessments. The study is global, with study centers including at the Moorfields Eye Hospital in London, University of Michigan Kellogg Eye Center and the Medical College of Wisconsin & Froedtert Hospital. We plan to present this natural history data to regulatory agencies in our meetings following completion of the Phase 1/2 clinical trial for discussion of our pivotal trial design and path to regulatory approval.

This clinical trial enrolled patients in the United Kingdom under our CTA, as well as the United States under our IND. Dosing in the Phase 1/2 clinical trial was completed June of 2018. The primary endpoint of this

open-label, dose-escalation clinical trial is the safety of delivering AAV-RPE65 through subretinal injection in patients with *RPE65*-deficiency. Secondary endpoints include the outcomes of a range of functional tests, detailed structural analysis of the retina and quality of life measures. A total of 15 patients were treated in this clinical trial, including nine adult patients in three dose escalating cohorts and six pediatric patients in the pediatric extension arm of the trial. Surgeries in this trial have been carried out in the United States at the University of Michigan Kellogg Eye Center and in the United Kingdom at the Moorfields Eye Hospital by three different surgeons. No differences in outcomes have been observed between the different surgeons.

In cohort one, three adults (aged 19, 20 and 20 years old) were administered up to 1mL of $1E^{11}$ vg/mL of AAV-RPE65 vector. In each patient, the central region of the retina was targeted. No serious adverse events or dose limiting events have been reported in this cohort as of the 18-month time point following vector administration.

In each patient treated in cohort one, we observed improvement or stability in central visual function following treatment compared to baseline and compared to the untreated fellow eye. Retinal sensitivity was measured with threshold static perimetry using Octopus 900 perimetry and visual field modelling and analysis, or VFMA. Observations at 18 months following vector administration in the first cohort have demonstrated a potential increase in retinal sensitivity by up to 40% from baseline. In contrast, we observed that the untreated eyes of patients in this cohort suffered a reduction in retinal sensitivity of up to 40%, which is in consistent with data from our parallel prospective natural history study of *RPE65*-deficient patients. In addition to the perimetry and VFMA data, improvements in time and error rate in mobility maze navigation have been observed in these patients at six and 12 months following treatment. However, this is preliminary data and needs to be supported by data collected at later time-points and in larger numbers of patients.

In addition to the functional data discussed above, we are collecting detailed structural data on all patients in our Phase 1/2 clinical trial. This includes data to visualize the mosaic of individual cones in the central part of the retina over time in each patient. As of May 4, 2018, we had sequential cone map data for more than 18 months following subretinal injections that covered the macula and fovea in the patients in cohort one and we had not observed retinal thinning or loss of cones over time following treatment. We are continuing to monitor the detailed structure of both treated and untreated fellow eye in the five-year follow on study to assess if AAV-RPE65 treatment has an impact on degeneration in the treated area of the eye compared to the untreated fellow eye.

In cohort two, three adults (aged 16, 20 and 22) were administered vector peripherally via subretinal injection into the outer region of the retina at a higher dose of up to 1mL of $3E^{11}$ vg/mL. Following completion of the four-week steroid taper, in two patients inflammatory responses were noted by the investigator as being probably related to vector administration. Both responded to a further course of steroids. Improvements in visual mobility in study eye over non-study eye were observed at 12 months at the four lux lighting level in this cohort. Similar increases in retinal sensitivity to cohort one have not been observed at this dose level. We believe data from cohort two is suggestive that peripheral administration of the vector into more degenerating areas of the retina may result in an increased chance of an immune response with a decreased potential benefit compared central administration of the vector. Following the review of the safety data for each adult cohort treated in this study, the IDMC recommended escalation to the higher dose in the next cohort.

Based on the safety and activity data from the patients treated at the $1E^{11}$ vg/mL dose level (cohort one), the decision was made to treat pediatric patients at this dose prior to receiving the full safety data from adult cohort three, and to target the central part of the retina in all pediatric patients. Treatment of pediatric patients in the United Kingdom and United States has been completed and six pediatric patients have been treated.

We carry out detailed assessments of retinal sensitivity across the entire retina, with the majority of tested retinal locations covering the central retina, at baseline and at prospectively defined time-points following

treatment using the Octopus 900 perimeter. The Octopus 900 perimeter, unlike other standard clinical devices, enables full-field static perimetry using testing algorithms specifically designed for retinal conditions, affords the use of custom-developed testing grids, and allows the full dataset to be extracted and modelled for more accurate measurement of change over time.

Discreet small spots of light are shone onto multiple predetermined points (the test grid) covering the central retinal field and peripheral retina, with the patient responding each time they perceive a spot of light. Each point on the retina is tested multiple times with different intensities of light, thereby allowing the determination of both the reliability and reproducibility of the patients' response to each light level at each spot and an accurate measurement of the exact retinal sensitivity at topographically determined points. The data is read out as a 'heat map' of sensitivity across the retinal field. The reliability of the patient and the data produced is also calculated and provided by the machine. In addition to the heat map of retinal sensitivity and conventional mean sensitivity metric produced by Octopus 900 assessment, we also conduct advanced VFMA. This produces a volumetric measure of retinal sensitivity presented both numerically as well as in a topographical retinal sensitivity map, which better represents the entire dataset across the visual field. We compare the VFMA data from the retinal map at each timepoint following treatment to the baseline data to measure the extent and position of changes in retinal sensitivity over time in treated and untreated eyes.

We completed dosing of the final pediatric patients in June of 2018, and we closed out the study six months after the last pediatric patient was treated. Patients will then move into a long term follow up study in which patients will be followed for safety and an indication of benefit for an additional four and a half years.

We expect to report topline six-month follow up safety and efficacy data in the first half of 2019, with full data expected to be presented in a scientific forum in the second half of 2019.

The FDA and EMA each granted orphan status to AAV-RPE65 for the treatment of LCA caused by mutations in *RPE65*. The FDA also granted AAV-RPE65 rare pediatric disease designation for the treatment of inherited retinal dystrophy due to biallelic *RPE65* mutations.

LCA4

Disease Background

LCA4 is an IRD that causes complete blindness before age five. AIPL1 is a central protein for the maintenance of photoreceptor structure and function. Deletion of the *AIPL1* gene causes the most severe form of early retinal dystrophy, LCA4, in which the retinal structure is destroyed with complete vision loss. LCA4 is rare, representing approximately 8% of all LCA cases.

There are currently no approved treatments for LCA4, and we believe an effective intervention will require introducing a normal functional copy of the *AIPL1* gene into rod and cone photoreceptors early in a patient's life while some retinal structure remains in order to activate function and survival of the photoreceptors that are still present. We believe gene therapy has the potential to be the only effective way to address the disease's root cause.

Our Gene Therapy Program

LCA4's extremely rapid progression, rarity and early age of onset make the standard process of seeking regulatory approval through clinical development challenging because adult safety trials would not yield meaningful data given the early onset of the disease. We believe we are well placed to initiate the first clinical intervention in this indication through our relationships with UCL and Moorfields Eye Hospital, whose expertise and large IRD patient population enables such an aggressive and uncommon IRD to be treated.

To address LCA4, we developed a viral vector to replace the *AIPL1* gene in all photoreceptors by using the *AIPL1* cDNA driven by the rhodopsin kinase promoter, which is active in both rods and cones.

Much of the preclinical work to support the proof of concept for gene therapy in LCA4 was conducted by our collaborators at UCL IO, led by Professor Robin Ali, and was published in *Gene Therapy* in 2010.

Figure 10 and Figure 11 show histological sections revealing the structure of retinas from *Aipl1* mutant mice treated with AAV-AIPL1, compared to untreated retinas from *Aipl1* mutant mice, at six months and 23 months after treatment.

It was observed at both time-points that *Aipl1* mutant mice treated with AAV-AIPL1 suffered less photoreceptor loss than untreated *Aipl1* mutant mice, with the retinas of untreated mutant mice almost completely degenerated by 24 months of age. *Aipl1* mutant mice were treated with.5uL of AAV-AIPL1 at $1E^{12}$ at four weeks post-natal and eyes were sectioned at six months and 23 months after treatment. These treated retinas were compared to retinas from untreated control *Aipl1* mice (n=17 eyes), and retinas from normal mice without the *Aipl1* mutation sectioned at the same post-natal ages as the treated *Aipl* mutant mice. The data indicates that subretinal administration of AAV-AIPL1 is associated with the protection of the retina from degradation that occurs in *Aipl1* mutant mice, and this effect was durable out to two years, the oldest age studied in these mice.

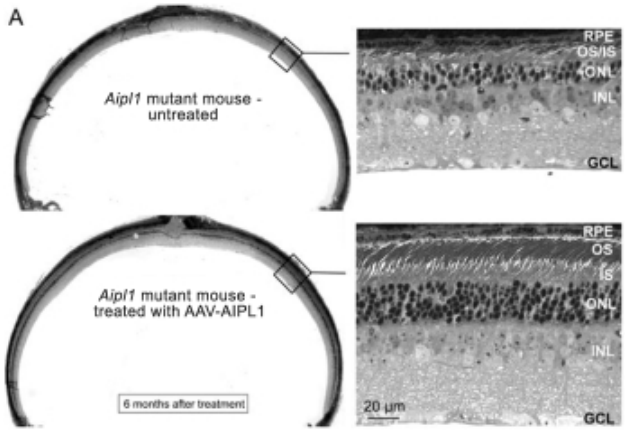


Figure 10. Histological sections of retinas of *Aipl1* mutant mice treated with AAV-AIPL1 six months after treatment with AAV-AIPL1, compared to untreated retina. The top image is the retina of an untreated *Aipl1* mutant mouse at seven months post-natal. The lower image is the retina of a *Aipl1* mutant mouse at seven months post-natal, and six months following treatment with AAV-AIPL1.

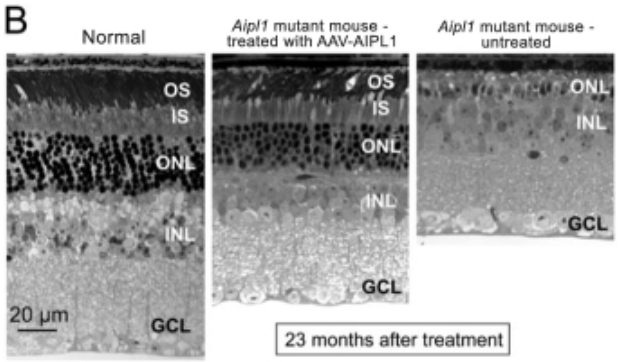


Figure 11. Histological sections of retinas of *Aipl1* mutant mice treated with AAV-AIPL1 23 months after treatment with AAV-AIPL1, compared to untreated retina and retina from a normal mouse. The left image

is the retina of an normal mouse at 24 months post-natal. The middle image is the retina of an *Aipl1* mutant mouse at 24 months post-natal, and 23 months following treatment with AAV-AIPL1. The right image is the retina of an untreated *Aipl1* mutant mouse at 24 months post-natal.

Clinical material was manufactured to cGMP standards under a specials license. Some of this clinical material was used in studies in 2017 at UCL to confirm potency in an AIPL1 deficient mouse and to perform toxicology testing in normal mice and rabbits. A potency study was conducted at a AAV-AIPL1 dose of $3E^9$ vg/eye with a group size of three eyes. Assessments were performed at three weeks and supported the potency of the cGMP clinical material through analysis of the ERG response.

A toxicology study was performed with normal mice and rabbits. In the mouse portion, five animals per group were evaluated for local and systemic effects at four weeks. In the rabbit portion, three animals per group were evaluated for local and systemic effects at four and eight weeks. Safety evaluation included functional assessments using ERG, fundoscopy, and structural assessments.

Our product candidate, AAV-AIPL1, was manufactured and released for compassionate use under a specials license in the UK to treat LCA4 patients at the Moorfields Eye Hospital. This allows physicians at the Moorfields Eye Hospital to prescribe a treatment of AAV-AIPL1 for LCA4 patients they deem appropriate. We play no role in the physician's treatment decision. We intend to use the data produced by the compassionate use treatment to inform any potential clinical development plan as well as any interactions with the regulatory agencies that would enable us to make this intervention more widely available to the LCA4 patient population. Although patients have been reviewed at the Moorfields Eye Hospital, so far none have been treated with AAV-AIPL1. As the manufacturer of AAV-AIPL1 under a specials license, we have a record retention requirement and a continuing obligation to inform the MHRA of any suspected adverse reaction to our medicinal product which is a serious adverse reaction.

The UK's Human Medicines Regulations 2012 allow for the manufacture and supply of medicinal products not authorized for marketing to patients with special needs at the request of the healthcare professional responsible for the patient's care (these products are referred to as "specials"). A special may only be supplied in: (i) response to an unsolicited order from a healthcare professional responsible for the care of the patient, (ii) if the product is manufactured and assembled in accordance with the specifications of that healthcare professional to fulfil the special needs of the individual patient that cannot be met by products already authorized for marketing and (iii) if the product is manufactured under a specials license granted by the UK's Medicines and Healthcare Products Regulatory Agency, or MHRA.

Manufacturing a special also imposes a five year record retention requirements subject to review by the MHRA, including details of any suspected adverse reaction to the product so sold or supplied of which the person is aware or subsequently becomes aware, as well as a continuing obligation to notify the MHRA of any suspected adverse reaction to the medicinal product which is a serious adverse reaction.

The FDA and EMA granted orphan designation to our product candidate, AAV-AIPL1, for treatment of inherited retina dystrophy due to defects in *AIPL1* gene.

Age-Related Macular Degeneration (AMD)

Disease Background and Market Opportunity

AMD is a chronic eye condition that causes gradual decline of central vision. It is the leading cause of vision loss in individuals more than fifty years old in the United States and it affects 6.2 million people globally. Although AMD does not usually lead to complete blindness because peripheral vision is generally maintained, losing central vision has a critical impact on visual acuity, which is achieved by the closely packed cones of the central retina. As a result, loss of central retinal function has a significant impact on a person's independence and quality of life.

AMD is a complex disease attributed to several different causes. A number of genetic and environmental risk factors have been associated with the development of AMD. Two late forms of AMD have been characterized: geographic atrophy, or dry AMD, and neovascular AMD, or wet AMD. Dry AMD is characterized by the progressive, irreversible loss of many of the cell types in the retina, including the RPE, photoreceptors, and underlying capillaries of the macula, and causes a decline in central visual function. Wet AMD is defined by the growth and invasion of immature blood vessels from the underlying choroid into the retina. Leakage from these fragile blood vessels causes build-up of blood and fluid under the retina, leading to detachment of the RPE or retina and long-term scarring.

Current therapeutic options for AMD are limited. There is no approved therapy that impacts the disease progression of dry AMD. The best available treatment for patients after they lose central vision and acuity is support and rehabilitation services to help them better utilize the remaining peripheral part of their retina.

Effective treatments for wet AMD are focused on blocking blood vessel growth, or angiogeneisis, that underlies the pathology of the disease. These wet AMD treatments specifically block the activity of vascular endothelial growth factor, or VEGF, one of the key drivers of angiogenesis. There are two approved anti-VEGF treatments on the market, ranibizumab, or Lucentis, and aflibercept, or Eyelea. These may be effective in some wet AMD patients, but require challenging dosing regimens that typically include intra-ocular injections every one to three months.

Our Gene Therapy Programs

Wet AMD

We aim to use a gene-based strategy to deliver an anti-angiogenic molecule to the retina and create a “local factory” that will deliver a continual supply of antiangiogenic molecules to the key parts of the eye. This would require a one-time injection procedure to deliver the gene therapy to the affected eye. We focus our blockade on the receptor involved in driving aberrant blood vessel growth and leakage, VEGF Receptor 2, or VEGFR2. Rather than depleting the VEGF ligand from the system we aim to block the receptor for that ligand preventing VEGF binding to the receptor and thereby preventing downstream pathology of wet AMD that is normally triggered by VEGF binding to VEGFR2. We use an anti-VEGFR2 antibody. In some rodent models the inhibition of the VEGFR2 with this antibody appears to have reduced long term toxicity as compared to the depletion of the VEGF ligand. Initial studies have identified a lead candidate that we have now moved from the research stage to formal preclinical development. We are also working to integrate our gene regulation technology with our wet AMD program. Our ultimate aim is to activate the anti-VEGFR2 antibody gene that we have installed in the eye with a small molecule applied topically with an eye drop. This could potentially allow for intermittent dosing of the antibody using an eye drop rather than an injection.

Dry AMD—Rod to Cone Program

To treat dry AMD, and specifically to treat patients with geographic atrophy in which the central part of the retina is irreversibly damaged, we are advancing a novel strategy to alter the very character of rod photoreceptors.

In advanced forms of dry AMD, central visual function is lost after the fovea and the larger central retina area has degenerated, and any residual vision is generally via the outer retina’s rods. A critical characteristic of cones that allows for response to high light levels is that they recover rapidly after they are stimulated by light. In contrast to cones, rods work in low light levels and require longer exposure to trigger an impulse and are then much slower to recover to a state when they can respond again. Therefore, seeing via rods is slow and imprecise, whereas cones mediate acute high-resolution vision. By genetically engineering rods with molecules that will improve their speed of recovery and response to light, we aim to effectively transform a patch of rod photoreceptors to behave more like cones. This rod-to-cone transformation would potentially be achieved

by using localized gene therapy vector placement in the rod-rich peripheral retina to create a small patch or “pseudo fovea,” where cone like behavior of rods would enable the patient’s brain to fixate on a functional part of the peripheral retina and recover a more cone like response to higher light levels.

The proof of concept has already been achieved in animal models, with first generation molecules designed to speed up the visual cycle recovery times in rods. We will continue to develop and optimize these molecules using our screening systems to allow selection for sensitivity to natural high light intensities and for the high refresh rate required.

Xerostomia

Disease Background and Market Opportunity—RIX

Radiation induced xerostomia, or RIX, is a severe and debilitating long-term side effect of radiation treatment for head and neck cancer. There is not currently any FDA approved treatment for RIX. Worldwide, there are approximately 500,000 new cases of head and neck cancer diagnosed each year, with approximately 50,000 cases in the United States alone, making it the fifth most common malignancy. Approximately 85% of patients who receive radiation treatment for head and neck cancer experience reduced saliva production during treatment, and approximately 40% of those patients who remain cancer free for two or more years after treatment continue to suffer from grade 2 or 3 RIX. There are approximately 170,000 such patients in the United States, with approximately 10,000 new cases each year. Severity of RIX is measured from grade 1 (mildest) to 4 (most severe). As these patients tend to be under the care of a physician and dentist and have access to some form of health insurance, we believe this is an accessible indication with much larger commercial potential than some genetic diseases that are currently targets of our gene therapy programs.

RIX is a frequent side effect of head and neck cancer treatment because the standard treatment of locally advanced head and neck cancer involves radiotherapy, which can cause irreversible damage to non-diseased tissues located near oral tumors, such as the salivary glands. The fluid secreting, or acinar cells, of the salivary glands are uniquely sensitive to radiation, are destroyed acutely on IR exposure and to a large extent do not regenerate, resulting in chronically reduced salivary output. Because saliva plays such a critical role in the physiology and protection of upper gastrointestinal, or GI, tract tissues, patients with chronic RIX suffer severe long-term complications of head and neck irradiation that has a significant impact on the patient’s daily living. Chronic RIX results in severe side effects, including difficulty swallowing, or dysphagia, oral discomfort, malnutrition, oral mucositis, changes in taste, increased oral infections and dental cavities.

Our Gene Therapy Program—RIX

Salivary glands are an attractive target organ for gene therapy treatments because they are self-contained, partially immune protected and easily accessible, allowing for non-invasive delivery of small vector doses.

We are developing AAV-AQP1 to treat RIX by increasing water conduction in the chronically damaged salivary glands by introducing a water conducting channel into the remaining epithelial cells of these damaged glands. Adequate water secretion by surviving epithelial cells has the potential to deliver the protective exocrine proteins produced by remaining gland cells into the mouth.

The key to our approach is that, unlike the water conducting acinar cells, the water impermeable duct cells of the glands appear to be resilient to IR exposure. As a consequence of this relative resilience to radiation treatment, salivary glands damaged by radiation treatment tend to contain mostly water impermeable ductal epithelial cells. To make these duct cells permeable to water, AAV-AQP1 introduces the gene for the human aquaporin water channel, or *hAQP1*. We have demonstrated that this has the potential to convey water permeability and causes ductal cells to generate an osmotic gradient, which causes them to secrete fluid into the lumen of the duct.

The proof of concept for this mechanism and its ability to increase the volume of saliva secreted by damaged salivary glands was observed in a Phase 1/2 clinical trial conducted by the NIH in patients with chronic grade 2 or 3 RIX. The trial was designed as a short-term dose escalation trial of doses of AAV-AQP1 between $4.8E^7$ and $5.8E^9$ pu/gland using adenovirus as the vector to deliver the *hAQP1* to the remaining epithelial cells in the parotid gland of 11 patients suffering from chronic RIX. There were no reported severe adverse events among the patients treated, two out of three patients in each of the first three cohorts in this clinical trial were observed to have objective increases in saliva volume produced by the treated parotid gland, with increases in parotid flow ranging from 60% to 540%, and all but one of these patients showed a decrease in symptoms of dry mouth as measured by subjective visual analog scales, validated in other forms of xerostomia. The results of this study were published in *Proceedings of the National Academy of Sciences* in 2012.

We are currently conducting a Phase 1 dose escalation clinical trial in patients with grade 2 or 3 RIX who remain cancer free for at least five years after receiving radiation treatment. In this trial we are using AAV2 to deliver the *hAQP1* gene, as we believe AAV2 efficiently transfects the salivary gland cells and does not spread beyond the target cells. Up to 18 adult patients may be administered doses of AAV-AQP1 between $3E^9$ and $6E^{11}$ viral particles per gland in dose escalation cohorts of three patients each. The aim of the trial is to determine the safety of inserting *hAQP1* locally into the salivary glands of RIX patients, as well as to measure changes in salivary flow resulting from the introduction of this channel. We have completed dosing in the first cohort and second cohort, having treated six patients. This clinical trial is being conducted in conjunction with the National Institute of Dental and Craniofacial Research at the NIH Dental Clinic.

We expect to initiate an additional clinical trial at Memorial Sloan Kettering Cancer Center and up to five additional clinical sites in the United States in 2019.

The FDA granted orphan drug designation for AAV-AQP1 to treat symptoms of grade 2 and grade 3 late xerostomia from parotid gland hypofunction caused by radiotherapy for cancer of the oral cavity.

Sjogren's Syndrome

The destruction of salivary tissue resulting in chronic xerostomia may also be caused by chronic autoimmune disease. Sjogren's syndrome is an autoimmune disease in which a patient's immune system may target the salivary glands. Chronic inflammation of the salivary glands results in long term damage and chronic xerostomia in many Sjogren's patients. Data from preclinical studies in animal models of Sjogren's syndrome and data from explants of minor salivary glands of Sjogren's patients suggest that Sjogren's syndrome may also be treatable with our AAV-AQP1 vector. Supported by data from our preclinical studies and our ongoing RIX clinical trials, we are currently conducting IND-enabling studies of AAV-AQP1 for xerostomia caused by Sjogren's syndrome.

Neurodegenerative Diseases

We also have clinical stage, research and preclinical programs targeting neurodegenerative diseases, with a clinical program in Parkinson's disease and a research focus in ALS and Alzheimer's disease. In ALS and Alzheimer's disease, we aim to target what we believe is a central factor in the underlying cell biology of the diseases - RNA metabolism in ALS and endosomal trafficking dysfunction in Alzheimer's disease. Relying on our expertise in viral vector design, delivery, production and manufacturing, we are aiming to develop and optimize vectors to effectively treat both genetic and sporadic forms of these diseases.

Parkinson's Disease

Disease Background and Market Opportunity

Affecting nearly one million Americans and 10 million worldwide, Parkinson's disease is the second-most common neurodegenerative disease after Alzheimer's disease and is the 14th-leading cause of death in the

United States. It is associated with a progressive loss of motor control (e.g., shaking or tremor at rest and lack of facial expression), as well as non-motor symptoms (e.g., depression and anxiety). There is no cure for Parkinson's disease and 60,000 new cases are diagnosed each year in the United States alone.

Our Gene Therapy Approach

AAV-GAD is an investigational gene therapy product candidate designed to deliver the glutamic acid decarboxylase, or *GAD*, gene to the subthalamic nucleus in order to increase production of GABA, the primary inhibitory neurotransmitter in the human brain. GAD is the rate-limiting enzyme in the synthesis of GABA, therefore it is believed that increasing subthalamic nucleus GAD expression through gene therapy has the potential to result in normalization of motor circuits and improve symptoms in Parkinson's disease patients without affecting other brain regions, which can be responsible for complications of existing therapies.

AAV-GAD Clinical Results

In a blinded Phase 2 clinical trial of AAV-GAD in patients with medically refractory Parkinson's disease, 45 patients were randomized 1:1 to receive either AAV-GAD gene therapy delivered by injection into the subthalamic nucleus on both sides of the brain or bilateral sham surgery. Subjects were followed for one year and all results remained blinded until the final treated patient reached the 6-month primary endpoint. The trial met the primary endpoint, with a significant improvement in the off-medication motor section of the Unified Parkinson's Disease Rating Scale, or UPDRS, part 3 observed in treated patients compared to baseline. There was also a significant difference in the degree of improvement observed compared with patients in the sham arm. The primary outcome measure was the six-month change from baseline in double-blind assessment of off-medication UPDRS motor scores. At the six-month endpoint, UPDRS score for the AAV-GAD group decreased by 8.1 points (SD 1.7, 23.1%; $p<0.0001$) and by 4.7 points in the sham group (1.5, 12.7%; $p=0.003$). The AAV-GAD group showed a significantly greater improvement from baseline in UPDRS scores compared with the sham group over the six-month course of the study (RMANOVA, $p=0.04$). Significant difference in the response rate between groups were observed, with responders being defined as patients achieving a 9-point or more improvement in UPDRS, which may be deemed clinically meaningful. At six months, 50% of AAV-GAD treated patients were responders compared with only 14% of patients in the sham arm. At 12 months, response rates were 63% and 24%, in AAV-GAD and sham arms, respectively. An improvement in complications of medical therapy as measured by the UPDRS part 4 was observed in the AAV-GAD group at both six and 12 months. A significant decline in duration of disabling dyskinesia was observed only in the AAV-GAD treated patients.

AAV-GAD was reported to be well-tolerated, with no significant adverse events related to the therapy and no speech or cognitive complications observed. The most commonly reported adverse events were transient mild or moderate headache (seven in treated arm vs. two in sham arm), nausea (six in treated arm vs. two in sham arm) and worsening of Parkinson's disease (zero in treated arm vs. four in sham arm). The results of the trial were published in the March 2011 issue of *The Lancet Neurology*, the August 2014 issue of the *Journal of Clinical Investigation* and the April 2017 issue of *JCI Insight*, building upon publications of the Phase 1 trial data in *The Lancet* and the *Proceedings of the National Academy of Sciences*. In addition, in research published in the November 28, 2018 issue of *Science Translational Medicine*, fifteen patients treated with AAV-GAD gene therapy were observed to have expressed a treatment-related reorganization of functional brain connectivity that was related to disease symptom improvement. This flurodeoxyglucose positron emission tomography analyses provided objective biological evidence of improvements in abnormal brain networks associated with Parkinson's disease following AAV-GAD gene therapy.

These results were observed in patients treated in both Phase 1 and Phase 2 studies. Blinded analyses showed significant improvements in abnormal thalamic metabolism, a key node in the movement circuitry, in the AAV-GAD treated patients. This pattern of brain network activity was not seen in untreated hemispheres or patients in the sham arm. Furthermore, a specific pattern of brain network activity was identified in those

subjects with clinical improvements in the sham arm, which was different from the pattern observed in AAV-GAD responders.

ALS

Disease Background and Market Opportunity

ALS is a devastating, progressive, neurodegenerative disease leading to the loss of motor neurons, which are the neurons that control the ability to move, speak, swallow and ultimately to breathe. The gradual paralysis in ALS invariably leads to death. While 10% of ALS cases are caused by inherited genetic mutations, most ALS occurs sporadically, with no known genetic cause. Mutations in over 20 genes have been identified that cause the inherited ALS cases. Characterization of these disease-causing genes have implicated several cellular pathways in the disease, with a prominent role emerging for genes involved in the cellular control of RNA. Many new regulatory roles are being discovered for RNA, particularly in neurons.

One gene linked to ALS is the transactive response DNA-binding protein 43, or *TDP-43*. The TDP-43 protein is one of a number of proteins that binds to cellular RNA and regulates its function and stability. When *TDP-43* does not function normally, RNA regulation in the neurons loses its tight control and the motor neurons die. While mutations in *TDP-43* account for only about 0.5% of ALS cases, aggregates of TDP-43 protein are observed in motor neurons of more than 95% of all ALS patients, including both inherited and sporadic, and TDP-43 aggregation is a central feature of diseased motor neurons in ALS patients. *TDP-43*, therefore, may be a critical therapeutic target for the treatment of both sporadic and inherited forms of ALS. TDP-43 aggregates are also seen in approximately 50% of patients with frontotemporal dementia, or FTD, suggesting a potentially similar underlying mechanism of this neurodegenerative disease.

Our Gene Therapy Approach

Genetic approaches using yeast as the primary model system have been used to identify ways of protecting motor neurons from death in ALS. A genetic screen was conducted to look for activated genes that rescue the toxicity of TDP-43 aggregation. A group of fives genes that reduce TDP-43 toxicity, was identified including members of the nonsense mediated decay, or NMD, machinery, and two genes in particular: up frameshift protein 1, or *UPF1*, and up frameshift protein 2, or *UPF2*.

NMD is the quality control system regulating RNA processing and activity in all cells, and has additional and broader functions specifically in regulating RNA in neurons.

Over the past decade, increasing evidence suggests that motor neurons may be particularly vulnerable to defects in RNA regulation, which may be caused by a number of defects, including *TDP-43* aggregation. We believe that increasing the master quality control machinery for RNA processing, NMD, in motor neurons may improve the ability of motor neurons to survive in ALS.

We have designed a viral vector product candidate, AAV-UPF1, with the aim of increasing *UPF1* expression in the motor neurons of ALS patients. In preclinical studies, we observed that administration of AAV-UPF1 reduced motor neuron death thought to be driven by the toxic effects of several different genetic causes of ALS including, TDP-43, FUS and C9*orf*72. Improvements in ALS-like symptoms related to limb strength and mobility in rodent models of ALS have also been observed following administration of AAV-UPF1.

We believe that gene therapy using AAV-UPF1 may increase *UPF1* levels in cells affected by ALS, and we intend to deliver our viral vector product candidate to the central nervous system via intrathecal injection, or injection into the spinal canal. We are currently conducting IND enabling studies in animal models and have observed the preservation of neurons and a positive impact on functional endpoints in Fus and TDP-43 neuronal cell cultures. Based on data from our non-human primate studies conducted in 2018, we have identified a

proprietary capsid for use in our AAV-UPF1 vector. We anticipate completing pre-IND activity in 2019 and aim to file an IND in the second half of 2019. We expect that AAV-UPF1 clinical material for the Phase 1/2 clinical trial will be manufactured at our cGMP manufacturing facility.

Alzheimer's Disease

Disease Background and Market Opportunity

With the world population aging, Alzheimer's disease has emerged as an extremely common and costly disease. While some treatments that have temporary effects on Alzheimer's disease symptoms are available, there is currently no approved treatment that halts the progression of the disease. Two biological pathways have been identified that are considered causes of Alzheimer's disease. They are identified as causative because mutations in genes that affect these pathways alone cause patients to develop the disease. The first causative pathway, identified in rare forms of early onset Alzheimer's disease, is the misprocessing of amyloid precursor protein, or APP, caused by genetic defects in *APP* itself and the APP processing proteins presenilin 1 and 2. The second causative pathway is related to the correct movement, or trafficking, of cellular protein which is controlled by a cell component called the endosome. Loss of function mutations in the endosomal protein sortilin-related receptor 1, or *SORL1*, have recently been shown to cause Alzheimer's disease.

The endosome is an organelle within the cell that is responsible for correctly directing protein trafficking to different sites within the cell as well as to the cell surface. In neurons, there is significant protein trafficking, particularly at dendrites or neuronal connections where the endosomal trafficking system regulates the movement of the cell signaling machinery that allows neurons to communicate with one another.

The molecular machine that drives endosomal protein trafficking is made up of a complex of proteins together called the retromer. The protein encoded by the Alzheimer's disease causing gene *SORL1* is a retromer component. *SORL1* and other components of the retromer complex are commonly deficient in Alzheimer's disease patients' brains. Defects in the endosomal trafficking pathway have also been implicated in other neurodegenerative diseases, including Parkinson's disease, ALS and frontotemporal dementia.

Our Gene Therapy Approach

Our Alzheimer's disease program focuses on the endosomal trafficking pathway. In preclinical studies, we observed that increasing levels of key retromer proteins may reverse endosomal trafficking defects. We are identifying suitable retromer targets for gene augmentation in pre-symptomatic Alzheimer's patients.

There are several reasons why gene therapy is, in principle, well suited for Alzheimer's disease and other neurodegenerative diseases. The first relates to the pathophysiology, time course, and anatomical spread of these disorders. Neurodegenerative diseases generally begin locally in selectively vulnerable regions with "cell sickness" years before rampant cell death and wide-spread anatomical distribution. To be most effective, we believe interventions should be administrated early and will benefit from local delivery. Even then, however, an intervention must maintain its efficacy for years because, unlike other cells in the body, neurons do not typically divide over the course of their life. We believe AAV-delivered gene therapy products may have a durable effect. In the best case scenario, one delivery successfully taken up by targeted neurons would be sufficient for years of efficacy.

An important component of our approach is the development and validation of surrogate markers of endosomal dysfunction and predictive markers of Alzheimer's disease. In particular, several well studied biomarkers linked to Alzheimer's disease, such as amyloid-beta and tau, have also been shown to be biomarkers of endosomal trafficking dysfunction in neurons. Such biomarkers could potentially be used to identify patients with Alzheimer's disease, as well as demonstrate potential product efficacy in the absence of Alzheimer's disease symptoms. By targeting endosomal trafficking dysregulation we aim to address the underlying cause of Alzheimer's disease as well as other neurodegenerative diseases, such as certain forms of Parkinson's disease.

Our Gene Regulation Platform

We are developing a potentially transformative technology designed to enable us to use small molecules to turn gene therapy product candidates on and off. The aim of this gene regulation platform is to transform gene therapy into a generalizable mechanism for the delivery of biologic drugs. The idea is that the gene encoding a particular biologic drug, for example epoetin alfa, or a therapeutic antibody, would be delivered to target cells in the body, but these genes would only be activated in the presence of a specific small molecule. The therapeutic protein would be manufactured by the body only in the presence of the small molecule so that intermittent production of the therapeutic protein would be achieved by dosing with the small molecule drug.

This temporal regulation of gene therapy products by exogenous small molecules has long been a goal of gene therapy researchers. The ability to regulate transgenes by introducing temporal control has the potential to transform the gene therapy landscape and the biologics industry as a whole. Our approach focuses on riboswitches to regulate gene expression rather than on the modulation of transcription factor activity, and this is the basis of our gene regulation platform.

Riboswitches are pieces of RNA that fold into alternative shapes depending on the binding of a specific small molecule to that RNA sequence. One RNA shape allows the gene containing the riboswitch to be active, while the alternative shape inactivates the gene. Riboswitches are used extensively by bacteria, but none have been identified in mammalian cells to date.

We designed *de-novo* mammalian riboswitches that we have observed respond to small molecules to switch genes on and off in mammalian cells and *in vivo* in mice. Our riboswitch contains a stretch of RNA sequence, called an aptamer, that binds to a specific small molecule. The riboswitch is inserted into the therapeutic transgene cDNA. In the absence of the specific small molecule, the unbound riboswitch folds into the shape that drives the destruction of the RNA message and no therapeutic protein is produced in the absence of the small molecule. However, when the small molecule is present and binds to the riboswitch it adopts the alternative RNA shape, causing stable messages to be formed and the therapeutic protein to be produced.

One of the features of our mammalian riboswitch is its range of regulation. Using a small molecule we were able to switch the riboswitch containing gene on to levels greater than 1,000x higher than in the absence of the small molecule. We believe this technology is viable for a therapeutic product and is also the first instance of a proprietary system for screening randomized aptamers and small molecules within mammalian cells for functional interactions.

Our Manufacturing Capabilities

We own and operate a cGMP manufacturing facility situated in London, United Kingdom. Supporting our global approach to clinical development and market supply, we designed the 29,000 square foot facility to meet multiple regulatory standards, including the Medicines and Health Products Regulatory Agency, or MHRA, in the UK, EMA and FDA standards. In the first quarter of 2018, we had our final MHRA certification inspection. In May 2018, we were granted a license to manufacture gene therapy product candidates in our cGMP compliant manufacturing facility by the UK Medicines and Healthcare products Regulatory Agency.

We believe our facility can supply all of our current clinical and preclinical programs through regulatory approval and, should they be approved, provide sufficient capacity, for commercial production. Strategically, we believe our facility will minimize our dependence on third-party CMOs, which we believe provides a significant strategic, clinical and commercial advantage.

Our facility is flexible and scalable, with eleven independent air handling units, two cell culture suites and three separate viral vector production suites, which allows us to produce multiple product candidates in parallel, as well as sequentially at different scales. This allows us to accommodate up to three independent parallel manufacturing streams of viral vector products that are isolated within dedicated production areas.

Our manufacturing facility includes an integrated analytical department and in-house analytical tool kit that allows for in-house release of clinical and commercial manufactured products. Equipped with dedicated areas for microbiology, molecular biology, and cell-based analytics. Our analytical department can perform product related assays, allowing us to retain and gain expertise that is normally lost to third parties. The close integration allows for rapid turnaround and flexibility in scheduling of key assays, reducing lead times for product candidate releases. Further, our dedicated product fill and finish suite allows us to manufacture a full range of clinical and commercial products under one roof and in our control.

We have more than 40 highly trained multidisciplinary staff on our manufacturing team with backgrounds in manufacturing, managing and delivering gene therapy products.

We have identified and licensed a proprietary HEK293 cell line that is well characterized and that we have banked in 400 vials. The specific cell line, size of the bank, culture media, and cryopreservation agents have been selected to facilitate bridging between process development platforms and targets. Our HEK-293 cells lack the T antigen component and are suitable for both the current adherent culture platform and the bioreactor process. We believe the ability to use the same cell line throughout the product and process development lifecycle will allow us to use a bracketed approach to process validation and comparability, which we believe may reduce the time and costs related to their implementation.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is true in the field of gene therapy generally, and in the treatments for our key disease areas. While we believe that the strength of our team, gene therapy expertise, scientific knowledge and intellectual property provide us with competitive advantages, we face competition from several sources, including large and small biopharmaceutical companies, academic research institutions, government agencies and public and private research institutions. Not only must we compete with other companies that are focused on gene therapy, but any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials and acquiring technologies complementary to, or necessary for, clinical programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

There are other organizations working to improve existing therapies or to develop new therapies for our initially selected disease indications. Depending on how successful these efforts are, it is possible they may increase the barriers to adoption and success for our product candidates, if approved. These efforts include two product candidates Applied Genetic Technologies Corporation, or AGTC, have in Phase 1/2 clinical trials to treat ACHM related to *CNGB3* and *CNGA3*, respectively, a product candidate in Phase 1/2 clinical trials by Nightstar Therapeutics plc and a program AGTC is running to treat XLRP, as well as Luxturna, which is marketed by Spark Therapeutics, Inc. and has been approved to treat *RPE65*-deficiency. We are not aware of any other gene therapy product candidates in clinical development targeting xerostomia. We are aware of other ALS gene therapies utilizing different treatment mechanisms to treat different genetically defined subsets of ALS patients, as well as gene therapy product candidates being developed for the treatment of Parkinson's disease, including those being developed by Voyager Therapeutics, Inc. and Axovant Sciences Ltd.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to

compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Intellectual Property

Our success depends in large part upon our ability to secure and maintain proprietary protection for our technologies and products and to operate without infringing the proprietary rights of others. Our policy is to protect our proprietary position by, among other methods, filing or collaborating with our licensors to file U.S. and foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also use other forms of protection, such as confidential information and trademark protection, particularly where we do not believe patent protection is appropriate or obtainable. Our patent portfolio consists of a combination of issued patents and pending patent applications that are owned or licensed from third parties.

As of December 31, 2018, we own, have an exclusive license or co-exclusive license under, or an exclusive option to license 38 United States and foreign issued or allowed patents and 176 patent applications, pending in the United States and internationally. For any individual patent, the term depends on the applicable law in the country in which the patent is granted. In most countries where we have filed patent applications or in-licensed patents and patent applications, patents have a term of 20 years from the application filing date or earliest claimed non-provisional priority date. In the United States, the patent term is 20 years but may be shortened if a patent is terminally disclaimed over another patent that expires earlier. The term of a U.S. patent may also be lengthened by a patent term adjustment, in order to address administrative delays by the United States Patent and Trademark Office in granting a patent. In the United States, the term of a patent that covers an FDA-approved drug or biologic may be eligible for patent term extension in order to restore the period of a patent term lost during the premarket FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the natural expiration of the patent. The patent term restoration period is generally equal to the regulatory review period for the approved product which period occurs after the date the patent issued, subject to certain exceptions. Only one patent may be extended for a regulatory review period for any product, and the application for the extension must be submitted prior to the expiration of the patent. In the future, we may decide to apply for restoration of patent term for one of our currently owned or licensed patents to extend its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

Company-Owned Intellectual Property

We own six patent families relating to gene regulation platform technologies developed by us. The first patent family includes 22 pending patent applications with claims directed to compositions of matter and methods of use in the United States, Europe, Australia, Canada, China, Hong Kong, India, Japan, Brazil, Egypt, Indonesia, Israel, Republic of Korea, Malaysia, Mexico, New Zealand, Vietnam, African Regional IPO, Philippines, Singapore, South Africa and Eurasia. Patents issued from this family are expected to expire February 2, 2036, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

The second patent family includes 20 pending patent applications with claims directed to compositions of matter and methods of use in the United States, Europe, Australia, China, India, Japan, Brazil, Egypt, Indonesia, Israel, Republic of Korea, Malaysia, Mexico, New Zealand, Vietnam, African Regional IPO, Philippines, Singapore, South Africa and Eurasia. Additional filings in Hong Kong and Canada will be made in due course. Patents issued from this family are expected to expire February 2, 2037, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

The third patent family includes 20 pending patent applications with claims directed to compositions of matter and methods of use in the United States, Europe, Australia, China, India, Japan, Brazil, Egypt, Indonesia, Israel, Republic of Korea, Malaysia, Mexico, New Zealand, Vietnam, African Regional IPO, Philippines, Singapore, South Africa and Eurasia. Additional filings in Hong Kong and Canada will be made in due course. Patents issued from this family are expected to expire February 2, 2037, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

The fourth patent family includes 19 pending patent applications with claims directed to compositions of matter and methods of use in the United States, Europe, Australia, India, Japan, Brazil, Egypt, Indonesia, Israel, Republic of Korea, Malaysia, Mexico, New Zealand, Vietnam, African Regional IPO, Philippines, Singapore, South Africa, and Eurasia. Additional filings in China, Hong Kong, and Canada will be made in due course. Patents issued from this family are expected to expire August 3, 2037, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

The other two families include two international applications relating to different gene regulations platform technologies with claims directed to compositions of matter and methods of use. We expect to convert each of these international applications to U.S. and international patent filings in due course. Patents issued from these two patent families are expected to expire in 2037 and 2038, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

We also own one patent relating to a vector technology developed by Vector Neurosciences Inc, acquired on October 5, 2018, with claims directed to compositions of matter. This patent is expected to expire October 21, 2025, not including any patent term adjustments that may extend the patent term in certain jurisdictions.

Licensed Intellectual Property

Certain of our issued patents and pending patent applications are exclusively licensed to us from UCL Business, Plc (“UCLB”), Brandeis University (“Brandeis”) and the National Institute of Dental and Craniofacial Research (“NIDCR”).

UCLB

The UCLB portfolio includes three licensed patent families relating to our *RPE65*, *CNGA3*, and *RPGR* gene therapy programs and one optioned patent family relating to our dry AMD gene therapy program with a combined 49 pending patent applications.

The first patent family, with claims directed to compositions of matter and methods of use relating to our *RPE65* program, and the AAV-RPE65 product candidate includes 18 pending patent applications in the United States, Europe, Australia, Canada, China, Hong Kong, India, Japan, Brazil, Egypt, Israel, Malaysia, Mexico, New Zealand, Nigeria, Philippines, Singapore, and Thailand. Patents issued from this family are expected to expire February 8, 2036, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

The second patent family, with claims directed to compositions of matter and methods of use relating to our achromatopsia program and the AAV-CNGA3 product candidate, includes one pending patent application, which we expect to convert to an international application and subsequent U.S. and international patent filings in due course. Patents issued from this family are expected to expire in January 2039, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

The third patent family, with claims directed to compositions of matter and methods of use relating to our retinitis pigmentosa program and the AAV-RPGR product candidate, includes one allowed patent application

in the United States and four pending applications in Europe, Canada, China, and Japan. Patents issued from this family are expected to expire in July 2035, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

The fourth patent family which we have optioned, with claims directed to compositions of matter and methods of use relating to our dry AMD gene therapy program, includes 25 pending applications in the United States, Europe, Australia, Canada, China, Hong Kong (two applications), India, Japan, Brazil, Egypt, Indonesia, Israel, Republic of Korea, Malaysia, Mexico, New Zealand, Nigeria, Vietnam, African Regional IPO, Philippines, Singapore, South Africa, Thailand and Eurasia. Patents issued from this family are expected to expire February 19, 2036, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

Brandeis

The licensed Brandeis portfolio includes one patent family with claims directed to compositions of matter and methods of use relating to our ALS gene therapy program and the AAV-UPF1 product candidate.

This patent family includes an issued patent in Australia and pending patent applications in the United States, Europe, Canada and Hong Kong. Patents issued from this family are expected to expire October 8, 2033, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

National Institute of Dental and Craniofacial Research

The exclusively licensed NIDCR portfolio includes one patent family with claims directed to compositions of matter and methods of use relating to our Sjogren's Syndrome gene therapy program. This patent family includes four issued or allowed patents in the United States, Europe, Canada and Australia, and one pending patent application in the United States. Patents issued from this family are expected to expire August 30, 2033, not including any patent term extensions or adjustments that may extend the patent term in certain jurisdictions.

License Agreements

License Agreements with UCLB

We previously entered into several license agreements with UCLB, covering *the following inherited retinal disease programs: (a) ACHM caused by mutations* in *CNGB3; (b) ACHM caused by mutations in CNGA3; (c) XLRP; and (d) RPE65-mediated IRD (together,* the "Licensed Gene Therapy Programs"). The terms of these license agreements were set forth in (i) the license agreement, dated February 4, 2015, as amended, between Athena Vision Ltd. and UCLB (the "First UCLB License Agreement"); (ii) the license agreements, dated July 29, 2017, as amended, between MeiraGTx UKII Limited and UCL Business, Plc (the "Second UCLB License Agreement"); and (iii) the license agreement, dated March 15, 2018, among MeiraGTx Limited, MeiraGTx UKII Limited and UCL Business Plc (the "Third UCLB License Agreement" and, collectively, the "prior UCLB license agreements"). In January and February 2019, we amended and restated the prior UCLB license agreements to establish new standalone license agreement (each, a "StandAlone UCLB Agreement") for each of the Licensed Gene Therapy Programs. We have removed from each of the Stand-alone Agreements our obligation to pay UCLB a share of certain sublicensing revenues as was provided under the First UCLB License Agreement and have aligned the material terms of the Stand-Alone Agreements to track those under the Third UCLB License Agreement as previously disclosed and a summary of which is set forth below as is now reflected in each of the Stand-Alone Agreements.

Under the terms of the Third UCLB License Agreement, we paid an initial upfront payment of £6,994, and issued to UCLB £100,000 of our ordinary shares. Under the Stand-Alone Agreement related to CNGB3, we paid UCLB an upfront payment of £1.5 million and issued £1.5 million of our ordinary shares.

Under each of the Stand-Alone UCLB Agreements, UCLB granted us an exclusive, worldwide, and sublicensable license under certain intellectual property rights controlled by UCLB relating to one of the Licensed Gene Therapy Programs to develop and commercialize licensed products in a relevant field of gene therapy. We must use diligent efforts to develop and commercialize the licensed products.

Under the terms of each Stand-Alone UCLB Agreement, we are required to pay UCLB sales milestone payments of up to a total of £39.75 million in the aggregate and an annual management fee of £50,000 until certain royalty payments have been paid. Additionally, pursuant to the Stand-Alone UCLB Agreement related to CNGB3, we agreed to pay UCLB an upfront payment of £1.5 million and issue £1.5 million of the Company's ordinary shares.

Commencing on the first commercial sale of licensed products under each Stand-Alone UCLB Agreement, we must make low single-digit percentage royalty payments to UCLB on net sales of such products. Our royalty obligations under each agreement continue on a licensed product-by-licensed product and country-by-country basis until the latest to occur of the expiration of the last valid claim of a patent claiming such licensed product in such country, the expiration of any regulatory exclusivity for all licensed products in such country, or the tenth anniversary of first commercial sale of such licensed product in such country.

Each Stand-Alone UCLB Agreement will remain in effect on a country-by-country basis until the expiration of the last payment obligation in such country. Each Stand-Alone UCLB Agreement may be terminated: (a) by either party in the event of the other party's material breach that remains uncured for 30 days (or for 14 days in the case of breaches related to payment obligations), (b) by either party for the other party's insolvency, (c) immediately by UCLB if we are in persistent breach of the agreement and the parties fail to agree upon a mechanism to remedy such persistent breach (or we do not comply with such agreed upon mechanism), or (d) immediately by UCLB if we undergo certain change of control events or if we enter into a sublicense with certain prohibited persons, which may adversely affect UCL's and/or UCLB's reputation. Each Stand-Alone UCLB Agreement may also be terminated or converted to a non-exclusive license by UCLB upon three months' notice if we, based on an independent expert determination, fail to use diligent efforts to develop and commercially exploit licensed products and do not cure such failure within a certain cure period.

License Agreement between Bri-Alzan Inc. and Brandeis

In May 2013, BRI-Alzan Inc., or BRI-Alzan, entered into a license agreement with Brandeis, or the Brandeis Agreement. On December 31, 2015, we entered into an Agreement and Plan of Merger, or the BRI-Alzan Merger Agreement, with BRI-Alzan, and the Brandeis Agreement was assigned to us as a result of such merger. Pursuant to the terms of the BRI-Alzan Merger Agreement, we agreed to make cash payments to BRI-Alzan upon the achievement of certain milestones, subject to an aggregate cap of $4,500,000. In addition, we agreed to make low single-digit percentage royalty payments to BRI-Alzan on net sales of any product for the therapeutic or prophylactic treatment of ALS that is covered by a valid claim of the patent rights licensed under the Brandeis Agreement. The BRI-Alzan Merger Agreement includes customary confidentiality, indemnification, non-competition and non-solicitation provisions.

Pursuant to the Brandeis Agreement, Brandeis granted us an exclusive, worldwide license under certain patent rights with claims directed to compositions of matter and methods of use relating to our ALS gene therapy program and the AAV-UPF1 product candidate to develop and commercialize licensed products.

We must use commercially reasonable efforts to develop and commercialize licensed products. We also acquired non-exclusive, worldwide licenses to certain know-how controlled by Brandeis' to exploit licensed products. We are required to pay Brandeis developmental and regulatory milestone payments of up to a total of $1.0 million in the aggregate. We are also required to pay Brandeis annual license maintenance fees ranging from $15,000 to $100,000 depending on the development stage of the licensed product. Commencing on the first commercial sale of licensed products, we must make low single-digit percentage royalty payments to Brandeis on net sales of such products. In addition, we must pay Brandeis mid-teen percentages of sublicensing revenues.

The Brandeis Agreement will remain in effect on a country-by-country basis until the earlier of: (a) 1 year after the date that we, our affiliates or sublicensees last sell any licensed product in such country or (b) until the expiration of the last–to-expire of the licensed patent rights in such country. The Brandeis Agreement may be terminated by Brandeis for our insolvency or for our material breach that remains uncured for 60 days (or for 30 days in the case of breaches related to payment obligations). Such material breach may be cured only once in any 12-month period. Brandeis may also terminate any license granted under the Brandeis Agreement if we fail to timely achieve certain regulatory milestone events.

Trade Secrets

We also rely on trade secrets, technical know-how and continuing innovation to develop and maintain our competitive advantage. Our policy requires inventors who are identified on any company-owned patent applications to assign rights to us. We also rely on confidentiality agreements with our employees, consultants and other advisors to protect our proprietary information. Our policy is to require third parties that receive material confidential information to enter into confidentiality agreements with us.

Trademarks

Our trademark MeiraGTx has been registered in the European Union and United States.

Government Regulation and Product Approval

Governmental authorities in the U.S., at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, labeling, packaging, promotion, storage, advertising, distribution, marketing, post-approval monitoring and reporting and export and import of products such as those we are developing. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, are extensive and require the expenditure of substantial time and financial resources.

FDA Approval Process

We expect our future product candidates to be regulated as biologics. Biological products, including gene therapy products, are subject to extensive regulation by the FDA under the Federal Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and other federal, state, local and foreign statutes and regulations. Both the FDCA and the PHSA and their corresponding regulations govern, among other things, the research, development, safety, testing, packaging, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of biological products. Before clinical testing of biological products in the United States may begin, we must submit an investigational new drug application, or IND, to the FDA, which reviews the clinical protocol, and the IND must become effective before clinical trials may begin.

Gene therapy products must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agencies before they may be legally marketed in foreign countries. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. The FDA has published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy clinical trials for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs.

To date, the FDA has approved three human gene therapy products for sale, including Kite Pharma's Yescarta, Novartis' Kymriah and Spark's Luxturna, and has provided general guidance regarding the development of gene therapy products. For example, the FDA has established the Office of Tissue and Advanced Therapies within CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee, to advise CBER on its reviews. In addition, the FDA has issued a growing body of clinical guidelines, chemical, manufacturing and control, or CMC, guidelines and other guidelines, all of which are intended to facilitate industry's development of gene therapy products.

Ethical, social and legal concerns about gene-editing technology, gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any product candidates. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

U.S. Biological Products Development Process

The FDA determined that more than minimally manipulated products must be approved by the FDA through the Biologics License Application, or BLA, process before they may be legally marketed in the United States. The process required by the FDA before a biologic may be marketed in the United States generally involves the following:

- completion of extensive nonclinical, sometimes referred to as preclinical laboratory tests, and preclinical studies and applicable requirements for the humane use of laboratory animals and formulation studies in accordance with applicable regulations, including good laboratory practices, or GLPs;
- submission to the FDA of an IND application, which must become effective before clinical trials may begin;
- approval by an independent Institutional Review Board, or IRB, or ethics committee at each clinical site before the trial is commenced;
- performance of adequate and well controlled human clinical trials according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity, potency and efficacy from results of nonclinical testing and clinical trials;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity;
- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including a gene therapy product, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements, including GLPs. The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing and controls, information about product chemistry, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing, such as reproductive toxicity tests and carcinogenicity in animals, may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, after which human clinical trials may begin unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such trials. In addition to the IND submission process, sponsors of certain human clinical trials of cells containing recombinant or synthetic nucleic acid molecules, including human gene transfer studies, had historically been subject to review by the Recombinant DNA Advisory Committee, or RAC, pursuant to the National Institutes of Health's Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines. On August 17, 2018, the NIH issued a notice in the Federal Register and issued a public statement proposing changes to the oversight framework for gene therapy trials, including changes to the applicable NIH Guidelines to modify the roles and responsibilities of the RAC with respect to human clinical trials of gene therapy products, and requesting public comment on its proposed modifications. During the public comment period, which closed October 16, 2018, the NIH has announced that it will no longer accept new human gene transfer protocols for review as a part of the protocol registration process or convene the RAC to review individual clinical protocols. These trials will remain subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level will continue as set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, the efficacy measurements to be evaluated and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance

with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. Clinical trials also must be reviewed by an IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1. The biological product candidate is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- Phase 2. The biological product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

In most cases, the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the safety and efficacy of a biological product. In rare instances, a single Phase 3 trial, together with other confirmatory evidence may be sufficient to support a BLA submission. Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other trials, tests in laboratory animals or *in vitro* testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend or permanently discontinue a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk or the clinical trial is not being conducted in accordance with FDA regulations. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients. The FDA and the IRB may also halt, terminate or impose other conditions if either believes the patients are subject to unacceptable risk.

There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at www.clinicaltrials.gov. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved.

Human gene therapy products based on gene-editing technology are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the study period, the number of patients the FDA will require to be enrolled in the trials in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these trials will be acceptable to the FDA to support marketing approval. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene transfer trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events in these trials.

Concurrent with clinical trials, companies usually complete additional animal trials and must also develop additional information about the physical characteristics of the biological product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

After the completion of clinical trials of a biological product candidate, FDA approval of a BLA must be obtained before commercial marketing and distribution of the biological product. The BLA must include results of product development, laboratory and animal trials, human trials, information on the manufacture, pharmacology, chemistry and controls of the product, proposed labeling and other relevant information. In addition, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective.

The Food and Drug Administration Safety and Innovation Act, or FDASIA, requires that a sponsor who is planning to submit a marketing application for a drug or biological product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within sixty days after an end-of-Phase 2 meeting or as may be agreed between the sponsor and FDA. The initial PSP must include, among other things, an outline of the pediatric study or studies that the sponsor plans to conduct, including to the extent practicable study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information, along with any other information specified in FDA regulations. The FDA and the sponsor must reach agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from nonclinical studies, early phase clinical trials, and/or other clinical development programs. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by

regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. PDUFA also imposes an annual program fee for products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first human drug application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. Under PDUFA, the FDA has agreed to certain performance goals to complete the review of BLAs. The FDA may give a priority review designation to biological products that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. Under the current PDUFA agreement, these six and ten month review periods are measured from the “filing” date rather than the receipt date for original BLAs, which typically adds approximately two months to the timeline for review and decision from the date of submission.

The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP requirements to assure and preserve the product’s identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product candidate. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements. To assure cGMP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified

may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter. Under the current PDUFA guidelines, the FDA has committed to reviewing such resubmissions in two or six months of receipt depending on the type of information included.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with REMS, to ensure the benefits of the product outweigh its potential risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. The requirement for a REMS can materially affect the potential market and profitability of the product.

Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. Changes to some of the conditions established in an approved BLA, including changes in indications, product labeling, manufacturing processes or facilities, require submission and FDA approval of a new BLA or BLA supplement before the change can be implemented. A BLA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing BLA supplements as it does in reviewing BLAs. The FDA may require one or more Phase 4 post-market studies or surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

Additionally, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could impact the timeline for regulatory approval or otherwise impact ongoing development programs. For example, in December 2016, the 21st Century Cures Act was signed into law. This act is intended, among other things, to modernize the regulation of drugs and biologics and to spur innovation, and contains provisions specific to the development of cell therapies.

One of the performance goals agreed to by the FDA under the PDUFA is to review 90% of standard BLAs in ten months from the filing date and 90% of priority BLAs in six months from the filing date, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Orphan Drug Designation

The FDA may grant Orphan drug designation to drugs or biologics intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and marketing the drug or biologic for this type of disease or condition will be recovered from its sales in the United States. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

In the United States, Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and BLA user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application, including a full BLA, to market the same drug or biologic for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer with orphan exclusivity is unable to assure sufficient quantities of the approved orphan-designated product. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new biological products that meet certain criteria. Specifically, new biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new biologic may request that the FDA designate the biologic as a Fast Track product at any time during the clinical development of the product. The FDA must determine if the biologic product candidate qualifies for Fast Track designation within 60 days of receipt of the sponsor's request. Unique to a Fast Track product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new biological product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval, which means that they may be approved on the basis of adequate and well controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a biological product subject to accelerated approval perform adequate and well-controlled post-marketing Phase 4 clinical trials. Failure to conduct required post-approval trials, or to confirm a clinical benefit during post-marketing trials, will allow the FDA to withdraw the approved biologic product from the market on an expedited basis. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast Track designation, priority review and

accelerated approval do not change the standards for approval but may expedite the development or approval process.

In addition, under the provisions of FDASIA, enacted in 2012, the FDA established a Breakthrough Therapy Designation which is intended to expedite the development and review of products that treat serious or life-threatening diseases or conditions. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the features of Fast Track designation, as well as more intensive FDA interaction and guidance. The Breakthrough Therapy Designation is a distinct status from both accelerated approval and priority review, but these can also be granted to the same product candidate if the relevant criteria are met. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy. All requests for breakthrough therapy designation will be reviewed within 60 days of receipt, and FDA will either grant or deny the request.

Furthermore, as part of its implementation of the 21st Century Cures Act, the FDA established the Regenerative Medicine Advanced Therapy, or RMAT, designation, to facilitate an efficient development program for, and expedite review of, certain drugs and biological products. A biological product is eligible for RMAT designation if it qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions, and is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and for which preliminary clinical evidence indicates that the biological product has the potential to address unmet medical needs for such a disease or condition. Like breakthrough therapy designation, RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT-designated products that receive accelerated approval may, as appropriate, fulfill their post-approval requirements through the submission of clinical evidence, clinical trials, patient registries, or other sources of real world evidence (such as electronic health records); through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy.

Fast Track designation, priority review, accelerated approval, breakthrough therapy designation and RMAT designation do not change the standards for approval but may expedite the development or approval process. Even if we receive one of these designations for our product candidates, the FDA may later decide that our product candidates no longer meets the conditions for qualification. In addition, receiving these designations may not provide us with a material commercial advantage.

Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to cGMP requirements. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products, include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements.

After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

To help reduce the increased risk of the introduction of adventitious agents, the PHSA Act emphasizes the importance of manufacturing controls for products whose attributes cannot be precisely defined. The PHSA Act also provides authority to the FDA to immediately suspend biologics licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases within the United States.

The FDA may require one or more Phase 4 post-market trials or surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies. We also must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the Internet. Biologics may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP requirements and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under Hatch-Waxman Act. The Hatch-Waxman Act permit a patent restoration term of up to five years as compensation for patent term lost

during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally equal to the regulatory review period for the approved product which period occurs after the date the patent issued, subject to certain exceptions. Only one patent may be extended for a regulatory review period for any product, and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to extend its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

For patents that might expire during the BLA review phase, the patent owner may request an interim patent term extension. If eligible, an interim patent term extension may be granted for a period of not more than one year. The patent owner may apply for not more than four subsequent interim extensions. Any interim extension granted will not be longer than the maximum period of extension allowed post-approval.

Biosimilars and Exclusivity

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. To date, only a handful of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical trial or trials. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, recent government proposals have sought to reduce the 12-year reference product exclusivity period. Other

aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and meaning of the BPCIA remains subject to significant uncertainty.

Other Healthcare Laws and Compliance Requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation: the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program; federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent; the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes which prohibit, among other things, executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters; HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, which imposes certain requirements on certain types of individuals and entities relating to the privacy, security and transmission of individually identifiable health information; the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to the federal government, information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and state and foreign law equivalents of each of the above federal laws, which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If their operations are found to be in violation of any of such laws or any other governmental regulations that apply, they may be subject to penalties, including, without limitation, civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, exclusion from participation in federal and state healthcare programs, integrity oversight and reporting obligations to resolve allegations of non-compliance and individual imprisonment.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement for such product by third-party payors. Decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies

in jurisdictions with existing controls and measures, could further limit sales of any product. Decreases in third-party reimbursement for any product or a decision by a third-party payor not to cover a product could reduce physician usage and patient demand for the product.

Healthcare Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

In March 2010, the ACA was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, the Tax Cuts and Jobs Act of 2017 was enacted, which, among other things, removes penalties for not complying with ACA's individual mandate to carry health insurance. On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Cuts and Jobs Act of 2017. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the law.There is still uncertainty with respect to the impact President Trump's administration and the U.S. Congress may have, if any, and any changes will likely take time to unfold, and could have an impact on coverage and reimbursement for healthcare items and services covered by plans that were authorized by the ACA.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers. Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level,CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. On January 31, 2019, the HHS Office of Inspector General proposed modifications to U.S. federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plans, the purpose of which is to further reduce the cost of drug products to consumers. Although some of these, and other proposals will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and

marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act of 1977, or FCPA, prohibits U.S. corporations and individuals from engaging in certain activities to obtain or retain business or secure any improper advantage, or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any employee or official of a foreign government or public international organization, or political party, political party official, or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA also includes employees and officials of state-owned or controlled enterprises, which may include healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval of a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application, or CTA, much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and the IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trial development may proceed.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki. If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Clinical Trials

Clinical trials of medicinal products in the European Union must be conducted in accordance with European Union and national regulations and the International Conference on Harmonization, or ICH, guidelines on GCPs. Additional GCP guidelines from the European Commission, focusing in particular on traceability, apply to clinical trials of advanced therapy medicinal products. If the sponsor of the clinical trial is not established within the European Union, it must appoint an entity within the European Union to act as its legal representative. The sponsor must take out a clinical trial insurance policy, and in most EU countries, the sponsor is liable to provide 'no fault' compensation to any study subject injured in the clinical trial.

Prior to commencing a clinical trial, the sponsor must obtain a clinical trial authorization from the competent authority, and a positive opinion from an independent ethics committee. The application for a clinical trial authorization must include, among other things, a copy of the trial protocol and an investigational medicinal product dossier containing information about the manufacture and quality of the medicinal product under investigation. Currently, clinical trial authorization applications must be submitted to the competent authority in each EU Member State in which the trial will be conducted. Under the new Regulation on Clinical Trials, which is currently expected to take effect in 2019, there will be a centralized application procedure where one national authority takes the lead in reviewing the application and the other national authorities have only a limited involvement. Any substantial changes to the trial protocol or other information submitted with the clinical trial applications must be notified to or approved by the relevant competent authorities and ethics committees. Medicines used in clinical trials must be manufactured in accordance with cGMP. Other national and European Union-wide regulatory requirements also apply.

During the development of a medicinal product, the EMA and national medicines regulators within the European Union provide the opportunity for dialogue and guidance on the development program. At the EMA level, this is usually done in the form of scientific advice, which is given by the Scientific Advice Working Party of the Committee for Medicinal Products for Human Use, or CHMP. A fee is incurred with each scientific advice procedure. Advice from the EMA is typically provided based on questions concerning, for example, quality (chemistry, manufacturing and controls testing), nonclinical testing and clinical trials, and pharmacovigilance plans and risk-management programs. Advice is not legally binding with regard to any future marketing authorization application of the product concerned.

Marketing Authorizations

To obtain regulatory approval of an investigational biological product under EU regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements. The process for doing this depends, among other things, on the nature of the medicinal product.

The centralized procedure results in a single marketing authorization, or MA, granted by the European Commission that is valid across the EEA (i.e., the European Union as well as Iceland, Liechtenstein and Norway). The centralized procedure is compulsory for human drugs that are: (i) derived from biotechnology processes, such as genetic engineering, (ii) contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative diseases, autoimmune and other immune dysfunctions and viral diseases, (iii) officially designated orphan medicines and (iv) advanced-therapy medicines, such as gene therapy, somatic cell therapy or tissue-engineered medicines. The centralized procedure may at the request of the applicant also be used in certain other cases. Therefore, the centralized procedure would be mandatory for the products we are developing.

The Committee for Advanced Therapies, or CAT, is responsible in conjunction with the CHMP for the evaluation of ATMPs. The CAT is primarily responsible for the scientific evaluation of ATMPs and prepares a draft opinion on the quality, safety and efficacy of each ATMP for which a marketing authorization application is submitted. The CAT's opinion is then taken into account by the CHMP when giving its final recommendation regarding the authorization of a product in view of the balance of benefits and risks identified. Although the CAT's draft opinion is submitted to the CHMP for final approval, the CHMP may depart from the draft opinion, if it provides detailed scientific justification. The CHMP and CAT are also responsible for providing guidelines on ATMPs and have published numerous guidelines, including specific guidelines on gene therapies and cell therapies. These guidelines provide additional guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs and include, among other things, the preclinical studies required to characterize ATMPs; the manufacturing and control information that should be submitted in a marketing authorization application; and post-approval measures required to monitor patients and evaluate the long term

efficacy and potential adverse reactions of ATMPs. Although these guidelines are not legally binding, we believe that our compliance with them is likely necessary to gain and maintain approval for any of our product candidates.

Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA by the EMA is 210 days. This excludes so-called clock stops, during which additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. At the end of the review period, the CHMP provides an opinion to the European Commission. If this is opinion favorable, the Commission may then adopt a decision to grant an MA. In exceptional cases, the CHMP might perform an accelerated review of an MAA in no more than 150 days (not including clock stops). This is usually when the product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation.

The European Commission may grant a so-called "conditional marketing authorization" prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional marketing authorizations may be granted for product candidates (including medicines designated as orphan medicinal products), if (i) the risk-benefit balance of the product candidate is positive, (ii) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (iii) the product fulfills an unmet medical need and (iv) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization.

The European Commission may also grant a so-called "marketing authorization under exceptional circumstances". Such authorization is intended for products for which the applicant can demonstrate that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use, because the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, or in the present state of scientific knowledge, comprehensive information cannot be provided, or it would be contrary to generally accepted principles of medical ethics to collect such information. Consequently, marketing authorization under exceptional circumstances may be granted subject to certain specific obligations, which may include the following:

- the applicant must complete an identified program of studies within a time period specified by the competent authority, the results of which form the basis of a reassessment of the benefit/risk profile;
- the medicinal product in question may be supplied on medical prescription only and may in certain cases be administered only under strict medical supervision, possibly in a hospital and in the case of a radio-pharmaceutical, by an authorized person; and

The package leaflet and any medical information must draw the attention of the medical practitioner to the fact that the particulars available concerning the medicinal product in question are as yet inadequate in certain specified respects.

A marketing authorization under exceptional circumstances is subject to annual review to reassess the risk-benefit balance in an annual reassessment procedure. Continuation of the authorization is linked to the annual reassessment and a negative assessment could potentially result in the marketing authorization being suspended or revoked. The renewal of a marketing authorization of a medicinal product under exceptional

circumstances, however, follows the same rules as a “normal” marketing authorization. Thus, a marketing authorization under exceptional circumstances is granted for an initial five years, after which the authorization will become valid indefinitely, unless the EMA decides that safety grounds merit one additional five-year renewal. A marketing authorization under exceptional circumstances should not be granted when a conditional marketing authorization is more appropriate.

The European Union medicines rules expressly permit the EU Member States to adopt national legislation prohibiting or restricting the sale, supply or use of any medicinal product containing, consisting of or derived from a specific type of human or animal cell, such as embryonic stem cells. While the products we have in development do not make use of embryonic stem cells, it is possible that the national laws in certain EU Member States may prohibit or restrict us from commercializing our products, even if they have been granted an MA.

Data and Marketing Exclusivity

The European Union also provides opportunities for market exclusivity. Marketing authorization applications for generic medicinal products do not need to include the results of preclinical and clinical trials, but instead can refer to the data included in the marketing authorization of a reference product for which regulatory data exclusivity has expired. In the European Union, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator’s data to assess a generic application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator’s data may be referenced, but no generic product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the EU regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity.

There is a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product, for example, because of differences in raw materials or manufacturing processes. For such products, the results of appropriate preclinical or clinical trials must be provided, and guidelines from the EMA detail the type of quantity of supplementary data to be provided for different types of biological product. There are no such guidelines for complex biological products, such as gene or cell therapy medicinal products, and so it is unlikely that biosimilars of those products will currently be approved in the European Union. However, guidance from the EMA states that they will be considered in the future in light of the scientific knowledge and regulatory experience gained at the time.

Orphan Medicinal Products

Products receiving orphan designation in the European Union can receive ten years of market exclusivity. During the ten-year market exclusivity period, the EMA cannot accept another application for a marketing authorization, or grant a marketing authorization or accept an application to extend an existing marketing authorization, for the same therapeutic indication, in respect of a similar medicinal product. An orphan product can also obtain an additional two years of market exclusivity in the European Union for pediatric studies. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The criteria for designating an “orphan medicinal product” in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the

European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers. The application for orphan drug designation must be submitted before the MAA. The applicant will receive a fee reduction for the MAA if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, an MA may be granted to a similar product for the same indication at any time if:

- the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior;
- the applicant consents to a second orphan medicinal product application; or
- the applicant cannot supply enough orphan medicinal product.

Pediatric Investigation Plan

In the EMA, MAAs for new medicinal products not authorized have to include the results of trials conducted in the pediatric population, in compliance with a pediatric investigation plan, or PIP, agreed with the EMA's Pediatric Committee, or PDCO. The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which an MA is being sought. The PDCO can grant a deferral of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults. Further, the obligation to provide pediatric clinical trial data can be waived by the PDCO when these data are not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Once the MA is obtained in all EU Member States and trial results are included in the product information, even when negative, the product is eligible for a six-months supplementary protection certificate extension.

Post-Approval Controls

The holder of an MA must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of periodic safety update reports, or PSURs.

All new MAAs must include a risk management plan, or RMP, describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. The regulatory authorities may also impose specific obligations as a condition of the marketing authorization. Such risk-minimization measures or post-authorization obligations may include additional safety monitoring, more frequent submission of PSURs, or the conduct of additional clinical trials or post-authorization safety studies. RMPs and PSURs are routinely available to third parties requesting access, subject to limited redactions.

All advertising and promotional activities for the product must be consistent with the approved summary of product characteristics, and therefore all off-label promotion is prohibited. Direct-to-consumer

advertising of prescription medicines is also prohibited in the European Union. Although general requirements for advertising and promotion of medicinal products are established under EU directives, the details are governed by regulations in each EU Member State and can differ from one country to another.

Pricing and Reimbursement

Governments influence the price of medicinal products in the European Union through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other EU Member States allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription medicines, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

PRIME Scheme

In July 2016 the EMA launched PRIME scheme. PRIME is a voluntary scheme aimed at enhancing the EMA's support for the development of medicines that target unmet medical needs. It is based on increased interaction and early dialogue with companies developing promising medicines, to optimize their product development plans and speed up their evaluation to help them reach patients earlier. Product developers that benefit from PRIME designation can expect to be eligible for accelerated assessment but this is however not guaranteed. The benefits of a PRIME designation includes the appointment of a rapporteur from the CHMP before submission of an MAA, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review earlier in the application process.

UK Specials Regulation

The UK's Human Medicines Regulations 2012 allow for the manufacture and supply of medicinal products not authorized for marketing to patients with special needs at the request of the healthcare professional responsible for the patient's care (these products are referred to as "specials"). A special may only be supplied in: (i) response to an unsolicited order from a healthcare professional responsible for the care of the patient, (ii) the product is manufactured and assembled in accordance with the specifications of that healthcare professional to fulfil the special needs of the individual patient which cannot be met by products already authorized for marketing, and (iii) the product is manufactured under a specials license granted by the UK's MHRA.

Manufacturing a special also imposes a five year record retention requirements subject to review by the MHRA, including details of any suspected adverse reaction to the product so sold or supplied of which the person is aware or subsequently becomes aware, as well as a continuing obligation to notify the MHRA of any suspected adverse reaction to the medicinal product which is a serious adverse reaction.

Privacy and Data Protection Laws

We are also subject to laws and regulations in non-U.S. countries covering data privacy and the protection of health-related and other personal information. EU member states and other jurisdictions have adopted data protection laws and regulations, which impose significant compliance obligations. Laws and regulations in these jurisdictions apply broadly to the collection, use, storage, disclosure, processing and security of personal information that identifies or may be used to identify an individual, such as names, contact information, and sensitive personal data such as health data. These laws and regulations are subject to frequent revisions and differing interpretations, and have generally become more stringent over time.

The General Data Protection Regulation, or GDPR, is a European framework law which imposes many requirements for controllers and processors of personal data, including, for example, higher standards for obtaining consent from individuals, if this is required, to process their personal data, more robust disclosures to individuals and a strengthened individual data rights regime, shortened timelines for data breach notifications, limitations on retention and secondary use of personal data, increased requirements pertaining to health data and pseudonymised (i.e., key-coded) data and additional obligations when we contract third-party processors in connection with the processing of t personal data. The GDPR allows EU member states to make additional laws and regulations further limiting the processing of genetic, biometric or health data. Failure to comply with the requirements of GDPR and the applicable national data protection laws of the EU member states may result in fines of up to €20,000,000 or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties.

The United Kingdom is expected to leave the European Union on March 29, 2019 further to the "Brexit" referendum. The UK's Data Protection Act 2018, or DPA2018, governs the UK's privacy regime and will continue to do so after the United Kingdom exits the EU. The DPA2018 incorporates the GDPR's text in full, with only minor amendments and further derogations including those pertaining to the processing of health data. Accordingly, the terms of the GDPR, and its significant penalties, will continue to apply after the UK exits the EU. At time of writing, it is still unclear whether the UK will be granted adequacy by the European Commission. In any event, for, at least, an interim period, export mechanisms, such as Model Clauses, will likely need to be put in place to govern the transfer of personal data from the EU to the UK.

Employees

As of December 31, 2018, we had 68 employees, all of which are full-time employees and 12 of which have M.D. or Ph.D. degrees. Of these full-time employees, 54 are engaged in research and development activities. None of our employees is subject to a collective bargaining agreement or represented by a trade or labor union. We consider our relationship with our employees to be good.

Corporate Information

MeiraGTx Holdings plc was formed on May 1, 2018 under the laws of the Cayman Islands. Our predecessor, MeiraGTx Limited, a limited company under the laws of England and Wales, was formed on March 20, 2015. In connection with our IPO, we reorganized whereby MeiraGTx Limited became a wholly owned subsidiary of MeiraGTx Holdings plc.

Available Information

We file annual, quarterly and current reports, proxy statements and other information with the U.S. Securities and Exchange Commission ("SEC"). Our SEC filings are available to the public over the Internet at the SEC's website at http://www.sec.gov. Our SEC filings are also available under the Investors and Media section of our website at *www.meiragtx.com*. Our website and the information contained on or connected to that site are not incorporated into this Form 10-K.

ITEM 1A. RISK FACTORS

You should carefully consider the risks and uncertainties described below and the other information in this Form 10-K before making an investment in our ordinary shares. Our business, financial condition, results of operations or prospects could be adversely affected if any of these risks occurs, and as a result, the market price of our ordinary shares could decline and you could lose all or part of your investment. This Form 10-K also contains forward-looking statements that involve risks and uncertainties. See "Special Note Regarding Forward-Looking Statements." Our actual results could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those set forth below.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future, and may never achieve or maintain profitability.

We are a clinical stage company with limited operating history. We were formed and began operations in 2015. We have never been profitable and do not expect to be profitable in the foreseeable future. We have incurred net losses since inception, including net losses of approximately $82.9 million and $31.0 million for the years ended December 31, 2018 and December 31, 2017, respectively. As of December 31, 2018, we had an accumulated deficit of approximately $148.3 million. Since our inception, we have devoted substantially all of our resources to developing our technology platform, establishing our viral vector manufacturing facility and developing manufacturing processes, advancing the product candidates in our ophthalmology, salivary gland and neurodegenerative disease programs, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, and providing general and administrative support for these operations. We have not yet demonstrated an ability to successfully complete a clinical program, including large-scale, pivotal clinical trials, obtain marketing approval, manufacture product at a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Typically, it takes about six to ten years to develop a new drug from the time it enters Phase 1 clinical trials to when it is approved for treating patients, but in many cases it may take longer. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing genetic medicine products.

We expect to continue to incur significant expenses and additional operating losses for the foreseeable future as we seek to advance product candidates through preclinical and clinical development, expand our research and development activities, develop new product candidates, complete clinical trials, seek regulatory approval and, if we receive regulatory approval, commercialize our products. Furthermore, the costs of advancing product candidates into each succeeding clinical phase tend to increase substantially over time. The total costs to advance any of our product candidates to marketing approval in even a single jurisdiction would be substantial. Because of the numerous risks and uncertainties associated with gene therapy product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to begin generating revenue from the commercialization of products or achieve or maintain profitability. Our expenses will also increase substantially as we operate as a public company and add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts, as well as to support our transition to a public reporting company.

Before we generate any revenue from product sales, each of our programs and product candidates will require additional preclinical and/or clinical development, potential regulatory approval in multiple jurisdictions, manufacturing, building of a commercial organization, substantial investment and significant marketing efforts. Our expenses could increase beyond expectations if we are required by the U.S. Food and Drug Administration, or FDA, European Medicines Agency, or EMA, or other regulatory authorities to perform preclinical studies and clinical trials in addition to those that we currently anticipate. These risks are further described under "—Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval" and "—Risks

Related to Commercialization." As a result, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our shareholders' equity and working capital.

As we continue to build our business, we expect our financial condition and operating results may fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any particular quarterly or annual period as indications of future operating performance. If we are unable to develop and commercialize one or more of our product candidates either alone or with collaborators, or if revenues from any product candidate that receives marketing approval are insufficient, we will not achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability. If we are unable to achieve and then maintain profitability, the value of our equity securities will be adversely affected.

We will require additional capital to fund our operations, which may not be available on acceptable terms, if at all.

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize our product candidates. We will require additional capital, which we may raise through equity offerings, debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable us to complete the development and potential commercialization of our product candidates. Furthermore, we expect to continue to incur costs associated with operating as a public company. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our operations have consumed significant amounts of cash since inception. As of December 31, 2018, our cash and cash equivalents were $68.1 million. Additionally, during the period from January 1, 2019 through the date of this filing, we have received additional funds in the aggregate amount of approximately $77.4 million and expect to receive an additional $100 million pursuant to the terms of the Collaboration Agreement. Therefore, based on our cash and cash equivalents after receiving the additional $177.4 million, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into 2022. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more than currently expected because of circumstances beyond our control. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the progress, timing, costs and results of our ongoing clinical development for our *CNGB3* gene therapy product candidate, AAV-CNGB3, for our *RPE65*-deficiency product candidate, AAV-RPE65, for our X-linked retinitis pigmentosa product candidate, AAV-RPGR, for our radiation induced xerostomia and Sjogren's syndrome-associated xerostomia or xerophthalmia product candidate, AAV-AQP1, and continue to conduct our ongoing natural history studies for inherited retinal diseases, or IRDs;
- the initiation of Phase 1/2 clinical trials for our *CNGA3* gene therapy product candidate, AAV-CNGA3, and for our product candidate for the treatment of xerostomia associated with Sjogren's syndrome, AAV-AQP1;

- future discussions with regulatory agencies and potential subsequent initiation of future clinical trials for our product candidate for the treatment of Parkinson's disease, AAV-GAD;
- continuing our current research programs, our preclinical development of product candidates from our current research programs and further developing our gene regulation technology;
- seeking to identify, assess, acquire and/or develop additional research programs and additional product candidates;
- the preclinical testing and clinical trials for any product candidates we identify and develop;
- establishing a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, EMA and other regulatory authorities;
- the cost of expanding and protecting our intellectual property portfolio, including filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or any of our product candidates;
- the effect of competing technological and market developments;
- the cost of further developing and scaling our manufacturing facility and processes;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the cost of making royalty, milestone or other payments under current and any future in-license agreements;
- the extent to which we in-license or acquire other products and technologies;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where we choose to commercialize our products; and
- the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or potentially discontinue operations.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common shareholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

We are heavily dependent on the success of our Most Advanced Product Candidates, which are still in development, and if none of them receive regulatory approval or are successfully commercialized, our business may be harmed.

To date, we have invested a significant portion of our efforts and financial resources in the development of AAV-CNGB3, AAV-CNGA3, AAV-RPGR, AAV-RPE65 and AAV-AQP1. Our future success and ability to generate product revenue is substantially dependent on our ability to successfully develop, obtain regulatory approval for and successfully commercialize these product candidates. We currently have no products that are approved for commercial sale and may never be able to develop marketable products. We expect to invest a meaningful portion of our efforts and expenditures over the next few years in AAV-GAD, AAV-CNGB3, AAV-CNGA3, AAV-RPGR, AAV-RPE65 and AAV-AQP1 (the "Most Advanced Product Candidates"), which will require additional clinical development, management of clinical and manufacturing activities, regulatory approval in multiple jurisdictions, manufacturing sufficient supply, building of a commercial organization, substantial investment and significant marketing efforts before we can generate any revenues from any commercial sales. Accordingly, our business currently depends heavily on the successful development, regulatory approval and commercialization of our Most Advanced Product Candidates, which may never occur. We cannot be certain that our Most Advanced Product Candidates will be successful in clinical trials, receive regulatory approval or be successfully commercialized even if we receive regulatory approval. Even if we receive approval to market our Most Advanced Product Candidates from the FDA, EMA or other regulatory bodies, we cannot be certain that our product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. Additionally, the research, testing, manufacturing, labeling, approval, sale, marketing and distribution of gene therapy products are and will remain subject to extensive and evolving regulation by the FDA, EMA and other regulatory authorities. We are not permitted to market our Most Advanced Product Candidates in the United States until they receive approval of a biologics license application, or BLA, from the FDA, we cannot market them in the European Union until we receive approval for a Marketing Authorization Application, or MAA, from the EMA, and we cannot market them in other countries until we receive any other required regulatory approval in those countries.

AAV-GAD, AAV-CNGB3, AAV-CNGA3, AAV-RPGR, AAV-RPE65 and AAV-AQP1 are our most advanced product candidates, and because some of our other product candidates are based on similar technology, if our Most Advanced Product Candidates show unexpected adverse events or a lack of efficacy in the indications we intend to treat, or if we experience other regulatory or developmental issues, our development plans and business could be significantly harmed. Further, competitors may be developing products with similar technology and may experience problems with their products that could identify problems that would potentially harm our business.

To date, we have not had any formal meetings with regulatory agencies nor determined the regulatory pathway and any potential related development costs for our recently acquired AAV-GAD gene therapy program for Parkinson's disease.

We may not be successful in our efforts to identify additional product candidates.

Part of our strategy involves identifying novel product candidates. The process by which we identify product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

- we may not be able to assemble sufficient resources to acquire or discover additional product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;

- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate may be too complex and difficult to navigate successfully or economically.

In addition, we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases that may later prove to have greater commercial potential, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights. If we are unable to identify additional suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval

We intend to identify and develop product candidates based on our novel gene therapy platform, which makes it difficult to predict the time and cost of product candidate development. Very few products that utilize transduction technology have been approved in the United States or in Europe, and there have only been a limited number of clinical trials involving a gene therapy product candidate.

We have concentrated a portion of our research and development efforts on our gene therapy platform, which uses both transduction and gene regulation technology. Our future success depends on the successful development of these novel therapeutic approaches. To date, very few products that utilize gene transfer have been approved in the United States or Europe. There have been a limited number of clinical trials of gene transduction technologies, with only one product candidate ever approved by the FDA.

Our gene therapy platform is based on a suite of viral vectors which we can deploy with gene therapy constructs, which relies on the ability of AAV to efficiently transmit a therapeutic gene to certain kinds of cells. The mechanism of action by which these vectors target particular tissues is still not completely understood. Therefore, it is difficult for us to determine that our vectors will be able to properly deliver gene transfer constructs to enough tissue cells to reach therapeutic levels. We cannot be certain that our viral vectors will be able to meet safety and efficacy levels needed to be therapeutic in humans or that they will not cause significant adverse events or toxicities. Furthermore, recent work conducted by a third party in non-human primates suggests that intravenous, or IV, delivery of certain AAV vectors at very high doses may result in severe toxicity. The indications that we target do not use IV administration for viral vector delivery and do not use doses as high as those tested in these publications, and to date we have not observed the severe toxicities described in these publications with the naturally occurring AAV vectors that we use. However, we cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials. Any such results could impact our ability to develop a product candidate. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our gene therapy platform, or any similar or competitive gene therapy platforms, will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy technologies will not

be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our gene therapy platform or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in utilizing our manufacturing facility and achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

In addition, because our gene regulation technology is still in the research stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment that we cannot predict at this time. Also, animal models may not exist for some of the diseases we expect to pursue.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and may change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the EMA and FDA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health, or the NIH, had historically been subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Following an initial review, RAC members would make a recommendation as to whether the protocol raised important scientific, safety, medical, ethical or social issues that warranted in-depth discussion at the RAC's quarterly meetings. Although the FDA decides whether individual gene therapy protocols may proceed under an IND, the RAC's recommendations were shared with the FDA and the RAC public review process, if undertaken, could have impeded or delayed the initiation of a clinical trial, even if the FDA had reviewed the trial and approved its initiation or had notified the sponsor that the study may begin. Conversely, the FDA can put an IND on clinical hold even if the RAC provided a favorable review or has recommended against an in-depth, public review.

On August 17, 2018, the NIH issued a notice in the Federal Register and issued a public statement proposing changes to the oversight framework for gene therapy trials, including changes to the applicable NIH Guidelines to modify the roles and responsibilities of the RAC with respect to human clinical trials of gene therapy products, and requesting public comment on its proposed modifications. During the public comment period, which closed on October 16, 2018, the NIH has announced that it will no longer accept new human gene transfer protocols for review as part of the protocol registration process under the existing NIH Guidelines or convene the RAC to review individual clinical protocols. These trials will remain subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level will continue as otherwise set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored

by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Even though we may not be required to submit a protocol for our gene therapy product candidates through the NIH for RAC review, we will still be subject to significant regulatory oversight by the FDA, and in addition to the government regulators, the applicable IBC and institutional review board, or IRB, of each institution at which we or our collaborators conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review and approve the proposed clinical trial.

In Europe, the EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. The prospectively designed natural history studies with the same endpoints as our corresponding clinical trials may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

Clinical trials are expensive, time-consuming, difficult to design and implement, and involve an uncertain outcome. Further, we may encounter substantial delays in our clinical trials.

The clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and market

our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. In particular, because our product candidates are subject to regulation as biological drug products, we will need to demonstrate that they are safe, pure, and potent for use in their target indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and is subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Failure can occur at any time during the clinical trial process. Even if our future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted indications. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

To date, we have not completed any clinical trials required for the approval of our product candidates. Although we have already begun Phase 1/2 clinical trials, we may experience delays in conducting any clinical trials and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials;
- obtaining regulatory approval to commence a clinical trial;
- reaching an agreement on acceptable terms with clinical trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- obtaining institutional review board, or IRB, approval at each site;
- recruiting suitable patients to participate in a clinical trial;
- developing and validating the companion diagnostic to be used in a clinical trial, if applicable;
- having patients complete a clinical trial or return for post-treatment follow-up;
- clinical sites, contract research organizations, or other third parties deviating from trial protocol or dropping out of a trial;
- failure to perform in accordance with the FDA's good clinical practice, or GCP, requirements, or applicable regulatory guidelines in other countries;

- addressing patient safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate and we may not have funds to cover the costs;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and
- any future collaborators that conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;

- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. For example, our IND for AAV-RPE65 was filed in July 2017. On August 16, 2017, we received notification from the FDA supporting the use of the described batches of product candidate in the Phase 1/2 clinical trial. However, we received a recommendation from the FDA on a certain aspect of the manufacturing process for future clinical trials, thus putting our IND for AAV-RPE65 on partial clinical hold. We responded to the FDA on October 2, 2017 and, based on this response, the partial clinical hold was lifted on October 17, 2017. As another example, our IND for AAV-CNGB3 was filed on October 31, 2017. We received a question from the FDA around our injection device compatibility assay, thus putting our AAV-CNGB3 IND on clinical hold. In the device compatibility assay, the FDA noted a disparity between the target titer for the intended low dose dilution and the actual titer obtained on polymerase chain reaction, or PCR, analysis. The FDA requested clarification on whether this was an imprecise dilution scheme for the low dose or a PCR assay issue. We submitted our second response to the FDA on May 2, 2018 providing data that identified the issue as a PCR assay artifact and also showing data that we believe supports that this has now been addressed. On May 31, 2018, the FDA released the clinical hold on our IND for AAV-CNGB3.

Our most advanced product candidates, AAV-GAD, AAV-CNGB3, AAV-CNGA3, AAV-RPGR, AAV-RPE65 and AAV-AQP1, will require extensive clinical testing before we are prepared to submit a BLA or MAA for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications,

acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included, or supporting the information, in this Form 10-K should be viewed with caution. Further, the data and statistical information included, or supporting the information, in this Form 10-K, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact public perception of our current and future product candidates.

Our potential therapeutic products involve introducing genetic material into patients' cells. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical, or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize murine gamma-retroviral vectors, our product candidates use a viral delivery system. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

Even though we have been granted access to the PRIME scheme by the EMA for AAV-CNGB3 and the FDA granted Fast Track designation to AAV-RPGR and AAV-CNGB3, in the future we may seek and fail to obtain access to the PRIME scheme by the EMA or Fast Track designation by the FDA for other of our current or potential future product candidates. We may also seek and fail to obtain breakthrough therapy designation from the FDA for our current or any future product candidates. Such designations or access may also not lead to faster development or regulatory review or approval, and it does not increase the likelihood that our product candidates will receive marketing approval.

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of new drugs and biological products that meet certain criteria. For example, the FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs, or if the drug has been designated as a qualified infectious disease product. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. Under Fast Track, the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted if relevant criteria are satisfied, including an agreement with FDA on the proposed schedule for the submission of portions of the BLA, and the payment of applicable user fees before FDA may initiate a review. Even if Fast Track designation is granted, it may be rescinded if the product no longer meets the qualifying criteria. In April 2018, AAV-RPGR was designated a Fast Track program by the FDA for the treatment of X-linked retinitis pigmentosa owing to defects in RPGR. In August 2018, AAV-CNGB3 was designated a Fast Track program by the FDA for the treatment of achromatopsia caused by *CNGB3* mutations to improve visual function.

In 2012, the Food and Drug Administration Safety and Innovation Act, or FDASIA, established the breakthrough therapy designation. A sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the product candidate is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement over existing therapies on one or more clinically-significant endpoints, such as substantial treatment effects observed early in clinical development. Sponsors may request that FDA designate a product candidate as a breakthrough therapy at the time of or any time after the submission of an IND, but ideally before an end-of-phase 2 meeting with FDA. If the FDA grants breakthrough therapy designation, it may take actions appropriate to expedite the development and review of the product candidate, which may include but are not limited to holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the product candidate to ensure collection of appropriate data needed to support approval; more frequent written correspondence from the FDA about such things as the design of the proposed clinical trials and use of biomarkers; intensive guidance on an efficient drug development program, beginning as early as Phase 1; organizational commitment involving senior managers; and eligibility for rolling review and priority review of a BLA. Breakthrough therapy designation comes with all of the benefits of Fast Track designation, which means that the sponsor may file sections of the BLA for review on a rolling basis if certain conditions are satisfied, including an agreement with FDA on the proposed schedule for submission of portions of the application and the payment of applicable user fees before the FDA may initiate a review. Fast Track designation, priority review and breakthrough therapy designation do not change the standards for approval but may expedite the development or approval process.

Similarly, the EMA has established the PRIME scheme to expedite the development and review of product candidates that show a potential to address to a significant extent an unmet medical need, based on early clinical data. In February 2018, AAV-CNGB3 in the treatment of achromatopsia associated with defects in CNGB3 was admitted to the PRIME scheme of the EMA.

Fast Track designation and designation as a breakthrough therapy are within the discretion of the FDA. Accordingly, even if we believe one of our other product candidates meets the criteria for Fast Track designation

or designation as a breakthrough therapy and we seek such designation, the FDA may disagree and instead determine not to make such designation for such product candidate. We cannot be sure that our evaluation of our product candidates as qualifying for Fast Track designation or breakthrough therapy designation will meet the FDA's expectations. In any event, the receipt of a Fast Track designation or breakthrough therapy designation for a product candidate may not result in a faster development process, review, or approval compared to product candidates considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if additional product candidates are granted Fast Track designation or one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Similarly, access to the PRIME scheme is at the discretion of the EMA, and we cannot be sure that any additional current or future product candidates will be granted access to the scheme; that participation in the scheme will result in expedited regulatory review or approval of our product candidates; or that access to the scheme, once granted, will not be revoked.

We have received orphan drug designation from the FDA and EMA for AAV-CNGB3, AAV-CNGA3, AAV-RPE65, AAV-RPGR, AAV-AIPL1 and FDA for AAV-AQP1 and may seek orphan drug designation for additional product candidates in the future, but any orphan drug designations we have received or may receive in the future may not confer marketing exclusivity or other expected benefits.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as one occurring in a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating, or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax credits for qualified clinical testing, and user-fee waivers. In addition, if a product receives the first FDA approval of that drug for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the rare disease or condition. Under the FDA's regulations, the FDA will deny orphan drug exclusivity to a designated drug upon approval if the FDA has already approved another drug with the same active ingredient for the same indication, unless the drug is demonstrated to be clinically superior to the previously approved drug. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following approval for the approved therapeutic indication. This period may be reduced to six years if, at the end of the fifth year, the orphan drug designation criteria are no longer met, including where it is shown that the drug is sufficiently profitable not to justify maintenance of market exclusivity. In the European Union, a marketing authorization for an orphan designated product will not be granted if a similar drug has been approved in the European Union for the same therapeutic indication, unless the applicant can establish that its product is safer, more effective or otherwise clinically superior. A similar drug is a product containing a similar active substance or substances as those contained in an already authorized product. Similar active substance is defined as an identical active substance,

or an active substance with the same principal molecular structural features (but not necessarily all of the same molecular features) and which acts via the same mechanism.

We have obtained orphan drug designation from the FDA and European Commission for AAV-CNGB3 for the treatment of achromatopsia caused by mutations in the *CNGB3* gene, AAV-RPE65 for the treatment of Leber congenital amaurosis, AAV-RPGR for the treatment of retinitis pigmentosa and AAV-AIPL1 for the treatment of inherited retina dystrophy due to defects in *AIPL1* gene, and we obtained orphan drug designation from the FDA for AAV-CNGA3 for the treatment of achromatopsia due to autosomal-recessive *CNGA3* gene mutations and for AAV-AQP1 for the treatment of grade 2 and grade 3 late xerostomia from parotid gland hypofunction caused by radiotherapy, and we obtained orphan medicinal product designation from the EMA for AAV-CNGA3 for the treatment of achromatopsia. We plan to seek orphan drug designation for other current and future product candidates. Even with orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products, which could prevent us from marketing our product candidates if another company is able to obtain orphan drug exclusivity before we do. In addition, exclusive marketing rights in the United States may be unavailable if we seek approval for an indication broader than the orphan-designated indication or may be lost in the United States if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the drug to meet the needs of patients with the rare disease or condition following approval. Further, even if we obtain orphan drug exclusivity, that exclusivity may not effectively protect our product candidates from competition because different drugs with different active moieties can be approved for the same condition. In addition, the FDA and the EMA can subsequently approve products with the same active moiety for the same condition if the FDA or the EMA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. In addition, while we intend to seek orphan drug designation for other existing and future product candidates, we may never receive such designations. There have been legal challenges to aspects of the FDA's regulations and policies concerning the exclusivity provisions of the Orphan Drug Act, and future challenges could lead to changes that affect the protections afforded our product candidates in ways that are difficult to predict. In 2014, a U.S. district court invalidated the FDA's denial of orphan exclusivity to an orphan designated drug, which the FDA had based on its determination that the drug was not proven to be clinically superior to a previously approved "same drug." In response to the decision, the FDA released a policy statement stating that the court's decision is limited to the facts of that particular case and that the FDA will continue to deny orphan drug exclusivity to a designated drug upon approval if the drug is the "same" as a previously approved drug, unless the drug is demonstrated to be clinically superior to that previously approved drug. Since then, similar legal challenges have been initiated against the FDA for its denial of orphan drug exclusivity to other designated drugs, and in 2017, Congress amended the Orphan Drug Act to require a demonstration of clinical superiority upon approval as a condition of receiving orphan drug exclusivity when another "same drug" has already been approved for the same indication. In the future, there is the potential for additional legal challenges to the FDA's orphan drug regulations and policies, and it is uncertain how ongoing and future challenges might affect our business.

We and our contract manufacturer for plasmid are subject to significant regulation with respect to manufacturing our products. Our manufacturing facilities and the third-party manufacturing facility which we rely on may not continue to meet regulatory requirements and have limited capacity.

We currently have relationships with a limited number of suppliers for the manufacturing of plasmid, a component of our viral vectors and product candidates. We completed the fit-out of our cGMP manufacturing facility in early 2018. However, if we experience slowdowns or problems with our facility and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain

and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials in the European Union must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or MAA on a timely basis. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or MAA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Any contamination in our manufacturing process, shortages of raw materials or failure of our plasmid supplier to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in

the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. The natural history studies may fail to provide us with patients for our clinical trials because patients enrolled in the natural history studies may not be good candidates for our clinical trials, or may choose to not enroll in our clinical trials. We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. The enrollment of patients depends on many factors, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

In addition, our clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates or approved products for the same clinical indications, and this competition may reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors, or chose to be treated using Luxturna, a commercially available product by Spark Therapeutics, Inc. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which may reduce the number of patients who are available for our clinical trials in such clinical trial site.

Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates, or could render further development impossible.

Our product candidates may cause serious adverse events or undesirable side effects or have other properties which may delay or prevent their regulatory approval, limit the commercial profile of an approved label, or, result in significant negative consequences following marketing approval, if any.

Serious adverse events or undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA, EMA or other authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, toxicities or unexpected characteristics, including death. A risk in any gene therapy product based on viral vectors is the risk of insertional oncogenesis.

If unacceptable side effects or deaths arise in the development of our product candidates, we, the FDA, the IRBs at the institutions in which our studies are conducted, DSMB, EMA or CAT could suspend or terminate our clinical trials or the FDA, EMA or other regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Undesirable side effects or deaths in clinical trials with our product candidates may cause the FDA or comparable foreign regulatory authorities to place a clinical hold on the associated clinical trials, to require additional studies, or otherwise to delay or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

If any of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by any such product, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product;
- regulatory authorities may require additional warnings on the label, such as a “black box” warning or contraindication;
- we may be required to implement a Risk Evaluation and Mitigation Strategy, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- the product could become less competitive;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

Success in preclinical studies or clinical trials may not be indicative of results in future clinical trials.

Results from previous preclinical studies or clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. In addition, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience designing clinical trials and may be unable to design and execute a clinical trial to support regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Many companies in the pharmaceutical and

biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

The regulatory approval processes of the FDA, EMA and other regulatory authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA, EMA and other regulatory authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that none of our product candidates in clinical programs or any other product candidates we may seek to develop in the future will ever obtain regulatory approval. Neither we nor any future collaborator is permitted to market any of our product candidates in the United States or the European Union until we receive regulatory approval of a BLA from the FDA or a MAA from the EMA, respectively. It is possible that the FDA may refuse to accept for substantive review any biologic license applications, or BLAs, or the EMA any of our MAAs, that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates.

Prior to obtaining approval to commercialize a product candidate in the United States, the European Union or elsewhere, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA, EMA or foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA, EMA or other regulatory authorities. The FDA or EMA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or it may object to elements of our clinical development program. Depending on the extent of these or any other FDA or EMA required studies, approval of any regulatory approval applications that we submit may be delayed by several years, or may require us to expend significantly more resources than we have available.

Of the large number of potential products in development, only a small percentage successfully complete the FDA, EMA or other foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

Even if we and / or our Collaboration Agreement partner obtain FDA or EMA approval for AAV-GAD, AAV-CNGB3, AAV-CNGA3, AAV-RPGR, AAV-RPE65 or AAV-AQP1 in the United States or European Union, we may never obtain approval for or commercialize it in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one

jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising and promotional activities for such product, among other things, will be subject to extensive and ongoing requirements of and review by the FDA, EMA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and GCP requirements for any clinical trials that we conduct post-approval.

The FDA and EMA closely regulate the post-approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and EMA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the U.S. federal Food, Drug, and Cosmetic Act, or FDCA, relating to the promotion of prescription drugs may lead to FDA enforcement actions and investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or holds on clinical trials;
- withdrawal of the products from the market;

- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure or detention; or
- injunctions or the imposition of civil or criminal penalties.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and biologics and spur innovation and contains provisions applicable to the development of gene therapies, but its ultimate implementation is unclear. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Interim "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim "top-line" or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development

programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- a licensure framework for follow on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- establishment of a Center for Medicare & Medicaid Innovation at the Centers for Medicare & Medicaid Services, or CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, the Tax Cuts and Jobs Act of 2017 was enacted, which includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additionally, on December 14, 2018, a U.S. District Court Judge in Texas ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act. While such U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the law. The current Trump administration and Congress will likely continue to seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the ACA. It is uncertain the extent to which any such changes may impact our business or financial condition.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate

reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute will remain in effect through 2027 unless additional action is taken by Congress. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws or any other similar laws introduced in the future may result in additional reductions in Medicare and other health care funding, which could negatively affect our customers and accordingly, our financial operations.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. The Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. On January 31, 2019, the HHS Office of Inspector General proposed modifications to U.S. federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plan sponsors, Medicaid managed care organizations, and those entities' pharmacy benefit managers ("PBMs"), the purpose of which is to further reduce the cost of drug products to consumers. Although some of these, and other, proposals will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the

European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal civil and criminal false claims and civil monetary penalties laws, including the civil False Claims Act, which prohibit, among other things,, including through civil whistleblower or qui tam actions, individuals or entities from knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes which prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program,

or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and its implementing regulations, which imposes certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by covered entities subject to the rule, such as certain health plans, healthcare clearinghouses and healthcare providers as well as their business associates, independent contractors of a covered entity that perform certain services involving the use or disclosure of individually identifiable health information;
- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the U.S. Physician Payments Sunshine Act and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the government information related to certain payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; state and local laws that require the registration of pharmaceutical sales representatives; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and
- similar healthcare laws and regulations in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and requirements regarding the collection, distribution, use, security, and storage of personally identifiable information and other data relating to individuals (including the EU General Data Protection Regulation 2016/679, or GDPR.

As of May 25, 2018, the GDPR replaced the Data Protection Directive with respect to the processing of personal data in the European Union. The GDPR imposes many requirements for controllers and processors of personal data, including, for example, higher standards for obtaining consent from individuals to process their personal data, more robust disclosures to individuals and a strengthened individual data rights regime, shortened timelines for data breach notifications, limitations on retention and secondary use of information, increased

requirements pertaining to health data and pseudonymised (i.e., key-coded) data and additional obligations when we contract third-party processors in connection with the processing of the personal data. The GDPR allows EU member states to make additional laws and regulations further limiting the processing of genetic, biometric or health data. Failure to comply with the requirements of GDPR and the applicable national data protection laws of the EU member states may result in fines of up to €20,000,000 or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.

Our operations, including our development, testing and manufacturing activities, are subject to numerous environmental, health and safety laws and regulations. These laws and regulations govern, among other things, the controlled use, handling, release and disposal of and the maintenance of a registry for, hazardous materials and biological materials, such as chemical solvents, human cells, carcinogenic compounds, mutagenic compounds and compounds that have a toxic effect on reproduction, laboratory procedures and exposure to blood-borne pathogens. If we fail to comply with such laws and regulations, we could be subject to fines or other sanctions.

As with other companies engaged in activities similar to ours, we face a risk of environmental liability inherent in our current and historical activities, including liability relating to releases of or exposure to hazardous or biological materials. Environmental, health and safety laws and regulations are becoming more stringent. We may be required to incur substantial expenses in connection with future environmental compliance or remediation activities, in which case, the production efforts of our third-party manufacturers or our development efforts may be interrupted or delayed.

Due to our international operations, we are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act; the U.S. Foreign Corrupt Practices Act, or FCPA; and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA, and these other laws generally prohibit us, our officers and our employees and intermediaries from bribing, being bribed by, or providing prohibited payments or anything else of value to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high

risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA, or local anti-corruption laws. In addition, we cannot predict the nature, scope, or effect of future regulatory requirements to which any of our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We also are subject to other laws and regulations governing any international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the EU, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, or, collectively, the Trade Control laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA, or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA, and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement, and other sanctions and remedial measures and legal expenses. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws, or Trade Control laws by U.K., U.S., or other authorities, even if it is ultimately determined that we did not violate such laws, could be costly and time-consuming, require significant personnel resources, and harm our reputation.

We will seek to build and continuously improve our systems of internal controls and to remedy any weaknesses identified. There can be no assurance, however, that the policies and procedures will be followed at all times or effectively detect and prevent violations of the applicable laws by one or more of our employees, consultants, agents, or collaborators and, as a result, we could be subject to fines, penalties, or prosecution.

Risks Related to Commercialization

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

The development and commercialization of new gene therapy products is highly competitive. Moreover, the gene regulation and manufacturing fields are characterized by rapidly changing technologies, significant competition, and a strong emphasis on intellectual property. We will face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs, including inherited retinal diseases and neurodegenerative diseases. Some of these competitive products and therapies are based on scientific approaches that are similar to our approach, and others are based on entirely different approaches.

Our platform and products focus on the development of gene therapies and gene regulation technology. There are a number of companies developing ocular gene therapy products, including Applied Genetic Technologies Corporation, Nightstar Therapeutics plc and Spark Therapeutics, Inc. There are a number of companies developing gene therapy products for neurodegenerative diseases, including Voyager Therapeutics, Inc. and Axovant Sciences Ltd. In addition to competition from other gene therapies, any products we may develop may also face competition from other types of therapies, such as small molecule, antibody, protein or other therapies.

Many of our current or potential competitors, either alone or with their collaboration partners, have greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology, and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop or that would render any products that we may develop obsolete or non-competitive. Our competitors also may obtain FDA, EMA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomic or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability of coverage and adequacy of reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates, assuming FDA approval. Our ability to achieve acceptable levels of coverage and reimbursement for our products or procedures using our products by governmental authorities, private health insurers and other organizations will have an effect on our ability to successfully commercialize our product candidates. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Separate reimbursement for the product itself or the treatment or procedure in which our product is used may not be available. A decision by a third-party payor not to cover or separately reimburse for our products or procedures using our products, could reduce physician utilization of our products once approved. Assuming there is coverage for our product candidates or procedures using our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and reimbursement in the United States, the European Union or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may not be adequate or may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing third-party therapeutics may limit the amount we will be able to charge for our product candidates. These payors may deny or revoke the reimbursement status of a given product

or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates, and may not be able to obtain a satisfactory financial return on our product candidates.

There is significant uncertainty related to the insurance coverage and reimbursement of newly-approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice, and we believe that changes in these rules and regulations are likely.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially-reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed health care, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

Even if our product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

If our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant product revenues or become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments;

- effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our products for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our product together with other medications.

Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenues for a substantial period, the failure of this product to find market acceptance would harm our business and could require us to seek additional financing.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target indications of our programs, even if they are approved.

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. We expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States and European Union, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact the commercialization of our product candidates. Additionally, if the commercial launch of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain international markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the product and such collaborator's ability to successfully market and sell the product. We may pursue collaborative arrangements regarding the sale and marketing of AAV-GAD, , AAV-RPE65, AAV-AQP1 or other future gene therapy programs, if approved, for the United States and/or certain markets overseas; however, we cannot assure that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of AAV-GAD, AAV-RPE65 or AAV-AQP1, we may be forced to delay the potential

commercialization of AAV-GAD, AAV-RPE65 or AAV-AQP1 or reduce the scope of our sales or marketing activities for AAV-GAD, AAV-RPE65 or AAV-AQP1. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to AAV-GAD, AAV-RPE65 or AAV-AQP1 or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

Some indications targeted by our ophthalmology programs are rare, but we anticipate realizing synergies in commercializing of our IRD product candidates, should they be approved. Failure to realize synergies in our sales, marketing and distribution efforts may harm our commercialization efforts.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing AAV-GAD, AAV-RPE65 or AAV-AQP1 and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If we obtain approval to commercialize any products outside of the United States or the European Union, a variety of risks associated with international operations could adversely affect our business.

If AAV-GAD, AAV-RPE65 or AAV-AQP1 are approved for commercialization, we intend to enter into agreements with third parties to market them in certain jurisdictions outside the United States and the European Union. We expect that we will be subject to additional risks related to international pharmaceutical operations, including:

- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- reduced protection for intellectual property rights;
- foreign reimbursement, pricing and insurance regimes;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- greater difficulty with enforcing our contracts;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions; and
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own pre-clinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

Risks Related to Our Dependence on Third Parties

If our cGMP manufacturing facility is unable to supply our product candidates for all of our current preclinical, clinical and potential commercial needs, we will be forced to seek out third-party manufacturers. We currently contract with third parties for the manufacture of plasmid used in producing our product candidates. Relying on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We produce our product candidates in our facility. However, if our facility is damaged, suffers any form of delay or regulatory challenges, or we are unable to scale our internal manufacturing capabilities to meet demand for our product candidates, we will need to contract with third-party manufacturers to produce our product candidates.

We currently rely on third-party manufacturers for the manufacture of plasmid used in the production of our product candidates. We do not have a long-term supply agreement with any of the third-party manufacturers, and we purchase our required supply on a purchase order basis.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the possible breach of the manufacturing agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements that might be required by the FDA or EMA. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could adversely affect supplies of our candidates and harm our business, financial condition, results of operations, and prospects.

Any therapies that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop or any components required for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

We may collaborate with third parties for the development and commercialization of our product candidates. We may not succeed in establishing and maintaining collaborative relationships, which may significantly limit our ability to develop and commercialize our product candidates successfully, if at all.

We may seek collaborative relationships for the development and commercialization of our product candidates. Failure to obtain a collaborative relationship for our product candidates may significantly impair their commercial potential. We also may need to enter into collaborative relationships to provide funding to support our other research and development programs. The process of establishing and maintaining collaborative relationships is difficult, time-consuming and involves significant uncertainty, such as:

- a collaboration partner may shift its priorities and resources away from our product candidates due to a change in business strategies, or a merger, acquisition, sale or downsizing;
- a collaboration partner may seek to renegotiate or terminate their relationships with us due to unsatisfactory clinical results, manufacturing issues, a change in business strategy, a change of control or other reasons;
- a collaboration partner may cease development in therapeutic areas which are the subject of our strategic collaboration;
- a collaboration partner may not devote sufficient capital or resources towards our product candidates;
- a collaboration partner may change the success criteria for a product candidate thereby delaying or ceasing development of such candidate;
- a significant delay in initiation of certain development activities by a collaboration partner will also delay payment of milestones tied to such activities, thereby impacting our ability to fund our own activities;
- a collaboration partner could develop a product that competes, either directly or indirectly, with our product candidate;
- a collaboration partner with commercialization obligations may not commit sufficient financial or human resources to the marketing, distribution or sale of a product;
- a collaboration partner with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirements;

- a collaboration partner may terminate a strategic alliance;
- a dispute may arise between us and a partner concerning the research, development or commercialization of a product candidate resulting in a delay in milestones, royalty payments or termination of an alliance and possibly resulting in costly litigation or arbitration which may divert management attention and resources; and
- a partner may use our products or technology in such a way as to make us subject to litigation with a third party.

If any collaborator fails to fulfill its responsibilities in a timely manner, or at all, our research, clinical development, manufacturing or commercialization efforts related to that collaboration could be delayed or terminated, or it may be necessary for us to assume responsibility for expenses or activities that would otherwise have been the responsibility of our collaborator. If we are unable to establish and maintain collaborative relationships on acceptable terms or to successfully transition terminated collaborative agreements, we may have to delay or discontinue further development of one or more of our product candidates, undertake development and commercialization activities at our own expense or find alternative sources of capital. Moreover, any collaborative partners we enter into agreements with in the future may shift their priorities and resources away from our product candidates or seek to renegotiate or terminate their relationships with us.

Risks Related to Intellectual Property

We depend on proprietary technology licensed from others. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from third parties, we may not be able to continue developing our product candidates.

We currently in-license certain intellectual property from UCL Business, Plc, or UCLB, and Brandeis University, or Brandeis, and the National Institute of Dental and Craniofacial Research, or NIDCR, a division of the NIH. We are a party to agreements with UCLB for certain technology and AAV vector-related patents and with Brandeis for certain preclinical technology for the treatment of ALS. Further, we are party to an agreement with NIDCR for technology relating to the treatment of Sjogren's syndrome. We may enter into additional agreements, including license agreements, with other parties in the future that impose diligence, development and commercialization timelines, milestone payments, royalties, insurance and other obligations on us. For example, in exchange for the rights granted to us by UCLB, we are obligated to pay an annual management fee, milestone payments for certain commercial sales thresholds, and a certain percentages of proceeds on sublicensing revenues. If we fail to comply with our obligations to UCLB, Brandeis, NIDCR, or any of our other collaborators, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product candidate that is covered by these agreements, which could adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

We may rely on other third parties from whom we license proprietary technology to file and prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them. We may have limited control over these activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We may have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that may be licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than if we conduct them ourselves. The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have

greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire. If we are unable to obtain and maintain patent protection for our technology and product candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our proprietary technologies, product candidate development programs and product candidates. Our success depends in large part on our ability to secure and maintain patent protection in the United States and other countries with respect to our current product candidates and any future product candidates we may develop. We seek to protect our proprietary position by filing or collaborating with our licensors to file patent applications in the United States and abroad related to our proprietary technologies, development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Moreover, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing, and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our proprietary products and technology, including current product candidates, any future product candidates we may develop, and our gene regulation technology in the United States or in other foreign countries, in whole or in part. Alternately, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from issuing from a pending patent application or later invalidate or narrow the scope of an issued patent. For example, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Even if patents do successfully issue and even if such patents cover our current product candidates, any future product candidates we may develop and our gene regulation technology, third parties may challenge their validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful challenge to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any of our product candidates or gene regulation technology. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and our gene regulation technology under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their validity, breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future product candidates or technology, it could dissuade companies from collaborating with us to develop product candidates, encourage competitors to develop competing products or technologies and threaten our ability to commercialize future product candidates. Any such outcome could harm our business.

The patent position of biotechnology and pharmaceutical companies is highly uncertain, involves complex legal and factual questions, and is characterized by the existence of large numbers of patents and frequent litigation based on allegations of patent or other intellectual property infringement or violation. In addition, the laws of jurisdictions outside the United States may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. Since patent applications in the United States and other jurisdictions are confidential for a period of time after filing, we cannot be certain that we were the first to file for patents covering our inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in the issuance of patents, or may result in the issuance of patents which fail to protect our technology or products, in whole or in part, or which fail to effectively prevent others from commercializing competitive technologies and products.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Thus, even if our patent applications issue as patents, they may not issue in a form that will provide us with meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Third parties may assert claims against us alleging infringement of their patents and proprietary rights, or we may need to become involved in lawsuits to defend or enforce our patents, either of which could result in substantial costs or loss of productivity, delay or prevent the development and commercialization of our product candidates, prohibit our use of proprietary technology or sale of products or put our patents and other proprietary rights at risk.

Our commercial success depends, in part, upon our ability to develop, manufacture, market and sell our product candidates without alleged or actual infringement, misappropriation or other violation of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Litigation relating to infringement or misappropriation of patent and other intellectual property rights in the pharmaceutical and biotechnology industries is common, including patent infringement lawsuits, interferences, oppositions and *inter partes* reviews, and reexamination proceedings before the U.S. Patent and Trademark Office, or USPTO, and corresponding foreign patent offices. The various markets in which we plan to operate are subject to frequent and extensive litigation regarding patents and other intellectual property rights. In addition, many companies in intellectual property-dependent industries, including the biotechnology and pharmaceutical industries, have employed intellectual property litigation as a means to gain an advantage over their competitors. Numerous U.S., EU and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates, and as the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the intellectual property rights of third parties. Some claimants may have substantially greater resources than we do and may be able to sustain the costs of

complex intellectual property litigation to a greater degree and for longer periods of time than we could. In addition, patent holding companies that focus solely on extracting royalties and settlements by enforcing patent rights may target us.

We may be subject to third-party claims including infringement, interference or derivation proceedings, post-grant review and inter partes review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, and the holders of any such patents may be able to block our ability to commercialize the applicable product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. There may be third-party patents or patent applications with claims to compositions, formulations, or methods of treatment, prevention use, or manufacture of our product candidates or technologies. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our compositions, formulations, or methods of treatment, prevention or use, the holders of any such patents may be able to prohibit our use of those compositions, formulations, methods of treatment, prevention or use or other technologies, effectively blocking our ability to develop and commercialize the applicable product candidate until such patent expires or is finally determined to be invalid or unenforceable or unless we obtained a license.

In addition, defending such claims would cause us to incur substantial expenses and, if successful, could cause us to pay substantial damages if we are found to be infringing a third party's patent rights. These damages potentially include increased damages (possibly treble damages) and attorneys' fees if we are found to have infringed such rights willfully. Further, if a patent infringement suit is brought against us or our third-party service providers, our development, manufacturing or sales activities relating to the product or product candidate that is the subject of the suit may be delayed or terminated. As a result of patent infringement claims, or in order to avoid potential infringement claims, we may choose to seek, or be required to seek, a license from the third party, which may require payment of substantial royalties or fees, or require us to grant a cross-license under our intellectual property rights. These licenses may not be available on reasonable terms or at all. Even if a license can be obtained on reasonable terms, the rights may be nonexclusive, which would give our competitors access to the same intellectual property rights. If we are unable to enter into a license on acceptable terms, we could be prevented from commercializing one or more of our product candidates, or forced to modify such product candidates, or to cease some aspect of our business operations, which could harm our business significantly. We might also be forced to redesign or modify our product candidates so that we no longer infringe the third-party intellectual property rights, which may result in significant cost or delay to us, or which redesign or modification could be impossible or technically infeasible. Even if we were ultimately to prevail, any of these events could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business. In addition, if the breadth or strength of protection provided the patents and patent applications we own or in-license is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Competitors may infringe our patents or other intellectual property. If we or one of our licensors were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the United States and in Europe, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness lack of written description, or non-enablement. Third parties might allege unenforceability of our patents because during prosecution of the patent an individual connected with such prosecution withheld relevant information, or made a misleading statement. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. The outcome of proceedings involving assertions of invalidity and

unenforceability during patent litigation is unpredictable. With respect to the validity of patents, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution, but that an adverse third party may identify and submit in support of such assertions of invalidity. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Our patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without infringing our patents or other intellectual property rights.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors view these announcements in a negative light, the price of our ordinary shares could be adversely affected. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop, manufacture and market our product candidates.

We cannot guarantee that any of our or our licensors' patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, Europe and elsewhere that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. For example, in the United States, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States, the European Union and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our product candidates could be filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use of our product candidates. After issuance, the scope of patent claims remains subject to construction as determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our product candidates. We may incorrectly determine that our product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States, the European Union or elsewhere that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our product candidates.

If we fail to correctly identify or interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay monetary damages, we may be temporarily or permanently prohibited from commercializing our product candidates. We might, if possible, also be forced to

redesign our product candidates in a manner that no longer infringes third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological complexity and legal complexity. Therefore, obtaining and enforcing biotechnology and genetic medicine patents is costly, time-consuming and inherently uncertain. In addition, the Leahy-Smith America Invents Act, or the AIA, which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a "first-to-invent" to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents.

We may become involved in opposition, interference, derivation, inter partes review or other proceedings challenging our or our licensors' patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in-licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in

unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but, the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO, European and other patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance and annuity fees on any issued patent are due to be paid to the USPTO, European and other patent agencies over the lifetime of a patent. While an inadvertent failure to make payment of such fees or to comply with such provisions can in many cases be cured by additional payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance with such provisions will result in the abandonment or lapse of the patent or patent application, and the partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents within prescribed time limits. If we or our licensors fail to maintain the patents and patent applications covering our product candidates or if we or our licensors otherwise allow our patents or patent applications to be abandoned or lapse, it can create opportunities for competitors to enter the market, which would hurt our competitive position and could impair our ability to successfully commercialize our product candidates in any indication for which they are approved.

We enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In-licensing patents covering our product candidates in all countries throughout the world may similarly be prohibitively expensive, if such opportunities are available at all. And in-licensing or filing, prosecuting and defending patents even in only those jurisdictions in which we develop or commercialize our product candidates may be prohibitively expensive or impractical. Competitors may use our and our licensors' technologies in jurisdictions where we have not obtained patent protection or licensed patents to develop their own products and, further, may export otherwise infringing products to territories where we and our licensors have patent protection, but enforcement is not as strong as that in the United States or the European Union. These products may compete with our product candidates, and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications while they are still pending. The grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications may be rejected by the relevant patent office, while substantively similar applications are granted by others. For example, relative to other countries, China has a heightened requirement for patentability and specifically requires a detailed description of medical uses of a claimed drug. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for and launch generic versions of our products. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or regulations in the United States and the European Union, and many companies have encountered significant difficulties in protecting and defending proprietary rights in such jurisdictions. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets or other forms of intellectual property, which could make it difficult for us to prevent competitors in some jurisdictions from marketing competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, are likely to result in substantial costs and divert our efforts and attention from other aspects of our business, and additionally could put at risk our or our licensors' patents of being invalidated or interpreted narrowly, could increase the risk of our or our licensors' patent applications not issuing, or could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, while damages or other remedies may be awarded to the adverse party, which may be commercially significant. If we prevail, damages or other remedies awarded to us, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. Furthermore, while we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition in those jurisdictions.

In some jurisdictions, compulsory licensing laws compel patent owners to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties under patents relevant to our business, or if we or our licensors are prevented from enforcing patent rights against third parties, our competitive position may be substantially impaired in such jurisdictions.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. Even if we or our licensors obtain patents covering our product candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review and approval of new product candidates, patents protecting such candidates may expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of marketing exclusivity for our product candidates, our business may be harmed.

In the United States, a patent that covers an FDA-approved drug or biologic may be eligible for a term extension designed to restore the period of the patent term that is lost during the premarket regulatory review process conducted by the FDA. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension

under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch- Waxman Act, which permits a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. In the European Union, our product candidates may be eligible for term extensions based on similar legislation. In either jurisdiction, however, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Even if we are granted such extension, the duration of such extension may be less than our request. If we are unable to obtain a patent term extension, or if the term of any such extension is less than our request, the period during which we can enforce our patent rights for that product will be in effect shortened and our competitors may obtain approval to market competing products sooner. The resulting reduction of years of revenue from applicable products could be substantial.

Our proprietary rights may not adequately protect our technologies and product candidates, and do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- others, including inventors or developers of our owned or in-licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;
- we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;
- we or our licensors may fail to meet obligations to the U.S. government with respect to in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents;
- issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership, validity or enforceability of our or our licensors' patents or patent applications may be challenged by third parties; and
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

Our reliance on third parties may require us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets, confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets and confidential know-how to protect our technology, especially where patent protection is believed by us to be of limited value. However, trade secrets and confidential know-how are difficult to protect, and we have limited control over the protection of trade secrets and confidential know-how used by our licensors, collaborators and suppliers. Because we have relied in the past on third parties to manufacture our product candidates, because we may continue to do so in the future, and because we expect to collaborate with third parties on the development of our current product candidates and any future product candidates we develop, we may, at times, share trade secrets with them. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Under such circumstances, trade secrets and confidential know-how can be difficult to maintain as confidential.

To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with us prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. The need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our competitive position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations. Enforcing a claim that a third party obtained illegally and is using trade secrets and/or confidential know-how is expensive, time consuming and unpredictable, and the enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction. Courts outside the United States are sometimes less willing to protect proprietary information, technology and know-how.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected. Our trademark MeiraGTx has been registered in the EU and United States. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our unregistered trademarks or trade names. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks

and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

We may need to license additional intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

The growth of our business may depend in part on our ability to acquire or in-license additional proprietary rights. For example, our programs may involve product candidates that may require the use of additional proprietary rights held by third parties. Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compositions and pre-existing pharmaceutical compositions. These pharmaceutical products may be covered by intellectual property rights held by others. We may be required by the FDA, EMA or other foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors access to the same technologies licensed to us.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and reputational loss and be a distraction to our management and other employees.

Risks Related to Employee Matters and Managing Growth

We will need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

As of December 31, 2018, we had 68 full-time employees. We will need to significantly expand our organization, and we may have difficulty identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the

expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, our potential ability to generate revenue could be reduced and we may not be able to implement our business strategy. Many of the biotechnology companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop product candidates and operate our business will be limited.

Our future success depends on our ability to retain our key personnel and to attract, retain and motivate qualified personnel.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Alexandria Forbes, Ph.D., our President and Chief Executive Officer, Rich Giroux, our Chief Operating Officer and Stuart Naylor, Ph.D., our Chief Development Officer, as well as the other principal members of our management, scientific and clinical teams. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from terminating their employment with us at any time and, for certain of our executive officers, entitle them to receive severance payments in connection with their voluntary resignation of employment.

If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize product candidates successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be engaged by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to develop and commercialize product candidates will be limited.

Potential product liability lawsuits against us could cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, health care providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. On occasion, large judgments have been awarded in class action lawsuits based on products that had unanticipated adverse effects. If we cannot successfully defend against product liability claims,

we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation and significant negative media attention;
- withdrawal of participants from our clinical trials;
- significant costs to defend the related litigation and related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- inability to commercialize our product candidates;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- decreased demand for our product candidates, if approved for commercial sale; and
- loss of revenue.

Our insurance policies are expensive and protect us only from some business risks, which leaves us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, clinical trial liability, employment practices liability, property, auto, workers' compensation, umbrella, cyber and directors' and officers' insurance.

Any additional product liability insurance coverage we acquire in the future, may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and in the future we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If we obtain marketing approval for our product candidates, we intend to acquire insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. A successful product liability claim or series of claims brought against us could cause our share price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business, including preventing or limiting the commercialization of any product candidates we develop. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

We also expect that continuing to operate as a public company will make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers. We do not know, however, if we will be able to maintain existing insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our cash position and results of operations.

Our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could harm our business.

Misconduct by our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization, could include intentional,

reckless or negligent conduct or unauthorized activities that violate: (i) the laws and regulations of the FDA, EMA and other similar regulatory authorities, including those laws that require the reporting of true, complete and accurate information to such authorities; (ii) manufacturing standards; (iii) data privacy, security, fraud and abuse and other healthcare laws and regulations; or (iv) laws that require the reporting of true, complete and accurate financial information and data. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creation of fraudulent data in preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid, other U.S. federal healthcare programs or healthcare programs in other jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, individual imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our business and operations would suffer in the event of system failures.

Our computer systems, as well as those of our contractors and consultants, are vulnerable to damage from computer viruses, unauthorized access, natural disasters (including hurricanes), terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product candidate development programs. For example, the loss of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees, in our data centers and on our networks. The secure processing, maintenance and transmission of this information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or internal bad actors, or breached due to employee error, a technical vulnerability, malfeasance or other disruptions. Although, to our knowledge, we have not experienced any such material security breach to date, any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our operations, damage our reputation, and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and delay clinical development of our product candidates.

We may engage in acquisitions that could disrupt our business, cause dilution to our shareholders or reduce our financial resources.

In the future, we may enter into transactions to acquire other businesses, products or technologies. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by customers or investors. We may decide to incur debt in connection with an acquisition or issue our ordinary shares or other equity securities to the shareholders of the acquired company, which would reduce the percentage ownership of our existing shareholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. In addition, we may not be able to successfully integrate the acquired personnel, technologies and operations into our existing business in an effective, timely and nondisruptive manner. Acquisitions may also divert management attention from day-to-day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our operating results.

The United Kingdom's withdrawal from the European Union may have a negative effect on global economic conditions, financial markets and our business, which could reduce the price of our shares.

Following the vote of a majority of the eligible members of the electorate in the United Kingdom to withdraw from the European Union in a national referendum held on June 23, 2016, the U.K. government served notice under Article 50 of the Treaty of the European Union on March 29, 2017 to formally initiate a withdrawal process. The withdrawal agreement and political declaration that were endorsed at a special meeting of the European Council on November 25, 2018 did not receive the approval of the UK Parliament in January and March 2019. Further discussions are ongoing, although the European Commission has stated that the EU will not reopen the withdrawal agreement. The United Kingdom and the European Union have a two-year period under Article 50 to negotiate the terms for withdrawal. Any extension of the negotiation period for withdrawal will require the consent of all of the remaining 27 EU member states.

The referendum and anticipation of withdrawal have created significant uncertainty about the future relationship between the United Kingdom and the European Union. Lack of clarity about future U.K. laws and regulations as the United Kingdom determines which EU-derived laws and regulations to replace or replicate as part of a withdrawal, including healthcare and pharmaceutical regulations; financial laws and regulations; tax and free trade agreements; intellectual property rights; supply chain logistics; environmental, health, and safety laws and regulations; immigration laws; and employment laws, could decrease foreign direct investment in the United Kingdom, increase costs, depress economic activity, and restrict our access to capital. If the United Kingdom and the European Union are unable to negotiate mutually acceptable withdrawal terms or if other EU member states pursue withdrawal, barrier-free access between the U.K. and other EU member states or among the European economic area overall could be diminished or eliminated. These developments, or the perception that any of them could occur, have had and may continue to have a significant adverse effect on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and restrict the ability of key market participants to operate in certain financial markets. Asset valuations, currency exchange rates, and credit ratings may be especially subject to increased market volatility. In addition, changes to U.K. border and immigration policy could occur as a result of the United Kingdom's withdrawal from the European Union, affecting our ability to recruit and retain employees from outside the United Kingdom. Any of these factors could have an adverse effect on our business, financial condition, results of operations, and prospects.

Further, the vote for the United Kingdom's withdrawal from the European Union has resulted in a decision to move the EMA from the United Kingdom to the Netherlands, with operations currently scheduled to begin in the Netherlands by end of March 2019. This transition has caused, and may continue to cause, disruption in the administrative and medical scientific links between the EMA and the UK Medicines and Healthcare products Regulatory Agency, or the MHRA, including delays in granting clinical trial authorization or marketing

authorization, disruption of importation and export of active substance and other components of new drug formulations, and disruption of the supply chain for clinical trial product and final authorized formulations. The cumulative effects of the disruption to the regulatory framework may add considerably to the development lead time to marketing authorization and commercialization of products in the European Union and/or the United Kingdom.

Exchange rate fluctuations may adversely affect our results of operations and financial condition.

Owing to the international scope of our operations, fluctuations in exchange rates, particularly between the pound sterling and the U.S. dollar, may adversely affect us. Although some of our operations are based in the United Kingdom, we source research and development, manufacturing, consulting and other services from the United States and the European Union. Further, potential future revenue may be derived from abroad, particularly from the United States. As a result, our business and the market price of our securities may be affected by fluctuations in foreign exchange rates not only between the pound sterling and the U.S. dollar, but also the euro, which may have a significant impact on our results of operations and cash flows from period to period. Currently, we do not have any exchange rate hedging arrangements in place.

Risks Related to Our Ordinary Shares

The market price of our ordinary shares may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our ordinary shares.

Our share price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your ordinary shares at or above your purchase price. The market price for our ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- actual or expected changes in our growth rate relative to our competitors;
- results of clinical trials of our product candidates or those of our competitors;
- developments related to our existing or any future collaborations;
- regulatory or legal developments in the United States and other countries;
- development of new product candidates that may address our markets and make our product candidates less attractive;
- changes in physician, hospital or healthcare provider practices that may make our product candidates less useful;
- announcements by us, our partners or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;

- actual or expected changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- changes in accounting principles; and
- the other factors described in this “Item 1A. Risk Factors” section and elsewhere in this Form 10-K.

In addition, the stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, when the market price of a security has been volatile, holders of that security have sometimes instituted securities class action litigation against the issuer. If any of the holders of our ordinary shares were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our senior management would be diverted from the operation of our business. Any adverse determination in litigation could also subject us to significant liabilities. Broad market and industry factors may negatively affect the market price of our ordinary shares, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause the price of our ordinary shares to decline rapidly and unexpectedly. If the market price of our ordinary shares does not exceed the your purchase price, you may not realize any return on your investment in us and may lose some or all of your investment.

Our executive officers, directors and principal shareholders, if they choose to act together, have the ability to control or significantly influence all matters submitted to shareholders for approval.

As of December 31, 2018, our executive officers, directors and shareholders who owned more than 5% of our outstanding ordinary shares and their respective affiliates, in the aggregate, hold ordinary shares representing approximately60.2% of our outstanding ordinary shares.

As a result, if these shareholders choose to act together, they would be able to control or significantly influence all matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other shareholders may desire. Any of these actions could adversely affect the market price of our ordinary shares.

A significant portion of our total outstanding shares are eligible to be sold into the market, which could cause the market price of our ordinary shares to drop significantly, even if our business is doing well.

Sales of a substantial number of our ordinary shares in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our ordinary shares.

All lock-up agreements entered into in connection with our initial public offering expired on December 5, 2018. Subject to any applicable lockup agreement described below, our outstanding ordinary shares may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act, or to the extent that such shares have already been registered under the Securities Act and are held by non-affiliates of ours. Moreover, certain holders of ordinary shares have rights, subject to specified

conditions, to require us to file registration statements covering their shares or to include their ordinary shares in registration statements that we may file for ourselves or other shareholders, until such shares can otherwise be sold without restriction under Rule 144 or until the rights terminate pursuant to the terms of the shareholders agreement between us and such holders. We also have registered all ordinary shares that we may issue under our equity compensation plans or that are issuable upon exercise of outstanding options. Upon issuance, these ordinary shares can be freely sold in the public market, subject to volume limitations applicable to affiliates and any applicable lock-up agreements. Furthermore, we and our executive officers, directors and certain of our shareholders have agreed with the underwriters that, subject to certain exceptions, we and they will not directly or indirectly sell or otherwise transfer their ordinary shares for a period of 90 days after the completion of the offering.

Any sales of securities by these shareholders could have a negative impact on the trading price of our ordinary shares.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our ordinary shares less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012 ("JOBS Act"), and may remain an emerging growth company until the last day of the fiscal year following the fifth anniversary of our IPO. However, if certain events occur prior to the end of such five-year period, including if we become a "large accelerated filer," our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this Form 10-K;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of these accounting standards until they would otherwise apply to private companies. We have elected to take advantage of this extended transition period.

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common shares held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and our voting and non-voting common

shares held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure, are exempt from the auditor attestation requirements of Section 404, and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide selected financial data, supplemental financial information or risk factors.

We may choose to take advantage of some, but not all, of the available exemptions for emerging growth companies and smaller reporting companies. We cannot predict whether investors will find our ordinary shares less attractive if we rely on these exemptions. If some investors find our ordinary shares less attractive as a result, there may be a less active trading market for our ordinary shares and our shares price may be more volatile.

We will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company and smaller reporting company, we will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, The Nasdaq Global Select listing requirements and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing whether such controls are functioning as documented, and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If securities or industry analysts cease to publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our ordinary shares, our share price and trading volume could decline.

The trading market for our ordinary shares relies in part on the research and reports that industry or securities analysts publish about us or our business. We do not control these analysts. Furthermore, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our share performance, or if any of our preclinical studies or clinical trials and operating results fail to meet the expectations of analysts, our share price would likely decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our share price or trading volume to decline.

Anti-takeover provisions in our organizational documents and Cayman Islands law may discourage or prevent a change of control, even if an acquisition would be beneficial to our shareholders, which could depress the price of our ordinary shares and prevent attempts by our shareholders to replace or remove our current management.

Our memorandum and articles of association contain provisions that may discourage unsolicited takeover proposals that shareholders may consider to be in their best interests. Our board of directors is divided into three classes with staggered, three-year terms. Our board of directors has the ability to designate the terms of and issue preferred shares without shareholder approval. We are also subject to certain provisions under Cayman Islands law that could delay or prevent a change of control. Together these provisions may make more difficult the removal of management and may discourage transactions that otherwise could involve payment of a premium over prevailing market prices for our ordinary shares.

There may be difficulties in enforcing foreign judgments against our management or us.

Certain of our directors and management reside outside the United States. A significant portion of our assets and such persons' assets are located outside the United States. As a result, it may be difficult or impossible for investors to effect service of process upon us within the United States or other jurisdictions, including judgments predicated upon the civil liability provisions of the federal securities laws of the United States.

In particular, investors should be aware that there is uncertainty as to whether the courts of the Cayman Islands or any other applicable jurisdictions would recognize and enforce judgments of U.S. courts obtained against us or our directors or management as well as against the selling shareholders predicated upon the civil liability provisions of the securities laws of the United States or any state in the United States or entertain original actions brought in the Cayman Islands or any other applicable jurisdictions courts against us or our directors or officers as well as against the selling shareholders predicated upon the securities laws of the United States or any state in the United States.

The rights of our shareholders differ from the rights typically offered to shareholders of a U.S. corporation.

Our corporate affairs and the rights of holders of ordinary shares are governed by Cayman Islands law, including the provisions of the Cayman Islands Companies Law (2018 Revision), or the Companies Law, the common law of the Cayman Islands and by our memorandum and articles of association. We are also subject to the federal securities laws of the United States. The rights of shareholders to take action against the directors, actions by minority shareholders and the fiduciary responsibilities of our directors to us under Cayman Islands law are to a large extent governed by the common law of the Cayman Islands. The common law of the Cayman Islands is derived in part from comparatively limited judicial precedent in the Cayman Islands as well as from English common law, the decisions of whose courts are of persuasive authority, but are not binding on a court in the Cayman Islands. The rights of our shareholders and the fiduciary responsibilities of our directors under Cayman Islands law are different from what they would be under statutes or judicial precedent in some

jurisdictions in the United States. In particular, the Cayman Islands has a different body of securities laws as compared to the United States, and certain states, such as Delaware, may have more fully developed and judicially interpreted bodies of corporate law. In addition, Cayman Islands companies may not have standing to initiate a shareholders derivative action in a Federal court of the United States.

As a result of all of the above, public shareholders may have more difficulty in protecting their interests in the face of actions taken by management, members of the board of directors or controlling shareholders than they would as public shareholders of a United States company.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, shareholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our ordinary shares.

Section 404(a) of the Sarbanes-Oxley Act, or Section 404(a), requires that beginning with our second annual report following our IPO on June 7, 2018, management assess and report annually on the effectiveness of our internal control over financial reporting and identify any material weaknesses in our internal control over financial reporting. Although Section 404(b) of the Sarbanes-Oxley Act, or Section 404(b), requires our independent registered public accounting firm to issue an annual report that addresses the effectiveness of our internal control over financial reporting, we have opted to rely on the exemptions provided in the JOBS Act, and consequently will not be required to comply with SEC rules that implement Section 404(b) until such time as we are no longer an “emerging growth company.”

We expect our first Section 404(a) assessment will take place for our annual report for the fiscal year ending December 31, 2019. Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inadequate internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our ordinary shares.

Because we do not anticipate paying any cash dividends on our ordinary shares in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

Under Cayman Islands law, we may only make distributions by way of dividend out of profits, or out of our share premium account (provided that immediately following the date that the dividend is proposed to be paid we are able to pay our debts as they fall due in the ordinary course of business). We have never declared or paid any cash dividends on our ordinary shares. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. As a result, capital appreciation, if any, of our ordinary shares would be your sole source of gain on an investment in our ordinary shares for the foreseeable future. See the “Dividend Policy” section of this Form 10-K for additional information.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management’s attention and resources, which could harm our business.

We expect to be treated as resident in the United Kingdom for tax purposes, but may be treated as a dual resident company for United Kingdom tax purposes.

Our board of directors conducts our affairs so that the central management and control of the company is exercised in the United Kingdom. As a result, we expect to be treated as resident in the United Kingdom for UK tax purposes. Accordingly, we expect to be subject to UK taxation on our income and gains, except where an exemption applies.

However, we may be treated as a dual resident company for UK tax purposes. As a result, our right to claim certain reliefs from UK tax may be restricted, and changes in law or practice in the United Kingdom could result in the imposition of further restrictions on our right to claim UK tax reliefs.

We may be classified as a passive foreign investment company for U.S. federal income tax purposes, which could result in adverse U.S. federal income tax consequences to U.S. investors in our ordinary shares.

Based on the current and anticipated value of our assets, including goodwill, and the composition of our income, assets and operations, we do not believe we were a "passive foreign investment company," or PFIC, for the taxable year ending on December 31, 2018, and do not expect to be a PFIC for the current taxable year. However, the application of the PFIC rules is subject to uncertainty in several respects, and we cannot assure you that the U.S. Internal Revenue Service, or the IRS, will not take a contrary position. Furthermore, a separate determination must be made after the close of each taxable year as to whether we are a PFIC for that year. Accordingly, we cannot assure you that we were not a PFIC for our taxable year ending on December 31, 2018 and that we will not be a PFIC for our current taxable year or any future taxable year. A non-U.S. company will be considered a PFIC for any taxable year if (i) at least 75% of its gross income is passive income (including interest income), or (ii) at least 50% of the value of its assets (based on an average of the quarterly values of the assets during a taxable year) is attributable to assets that produce or are held for the production of passive income. If we were to be classified as a PFIC for any taxable year during which a U.S. Holder (as defined below under "Material U.S. Federal Income Tax Consequences") holds our ordinary shares, certain adverse U.S. federal income tax consequences could apply to such U.S. Holder, including (i) the treatment of all or a portion of any gain on disposition of our ordinary shares as ordinary income, (ii) the application of a deferred interest charge on such gain and the receipt of certain dividends and (iii) the obligation to comply with certain reporting requirements.

If a United States person is treated as owning at least 10% of our ordinary shares, such holder may be subject to adverse U.S. federal income tax consequences.

If a U.S. Holder is treated as owning (directly, indirectly or constructively) at least 10% of the value or voting power of our ordinary shares, such U.S. Holder may be treated as a "United States shareholder" with respect to each "controlled foreign corporation" in our group (if any). If our group includes one or more U.S. subsidiaries, certain of our non-U.S. subsidiaries could be treated as controlled foreign corporations (regardless of whether we are treated as a controlled foreign corporation). A United States shareholder of a controlled foreign corporation may be required to report annually and include in its U.S. taxable income its pro rata share of "Subpart F income," "global intangible low-taxed income" and investments in U.S. property by controlled foreign corporations, regardless of whether we make any distributions. An individual that is a United States shareholder with respect to a controlled foreign corporation generally would not be allowed certain tax deductions or foreign tax credits that would be allowed to a United States shareholder that is a U.S. corporation. Failure to comply with these reporting obligations may subject you to significant monetary penalties and may prevent the statute of limitations from starting with respect to your U.S. federal income tax return for the year for which reporting was due. We cannot provide any assurances that we will assist investors in determining whether any of our non-U.S. subsidiaries is treated as a controlled foreign corporation or whether such investor is treated as a United States shareholder with respect to any of such controlled foreign corporations. Further, we cannot provide any assurances that we will furnish to any United States shareholders information that may be necessary

to comply with the aforementioned reporting and tax payment obligations. U.S. Holders should consult their tax advisors regarding the potential application of these rules to their investment in our ordinary shares.

Comprehensive tax reform legislation could adversely affect our business and financial condition.

The U.S. government has enacted comprehensive tax legislation that includes significant changes to the taxation of business entities, referenced herein as the Tax Reform Act. These changes include, among others, a permanent reduction to the corporate income tax rate, limiting interest deductions and the use of net operating losses, adopting elements of a territorial tax system and introducing certain anti-base erosion provisions. We continue to examine the impact this tax reform legislation may have on our business. The effect of the Tax Reform Act on our business, whether adverse or favorable, is uncertain, and may not become evident for some period of time. U.S. Holders should consult their legal and tax advisors regarding any such legislation and the potential tax consequences of investing in our ordinary shares.

Changes in tax laws or challenges to our tax position could adversely affect our results of operations and financial condition.

We are subject to complex tax laws. Changes in tax laws, regulations and treaties, or the interpretation thereof, tax policy initiatives and reforms under consideration and the practices of tax authorities in jurisdictions in which we operate could adversely affect our tax position, including our effective tax rate or tax payments.

In October 2015, the Organization for Economic Co-Operation and Development released a final package of measures to be implemented by member nations in response to a 2013 action plan calling for a coordinated multi-jurisdictional approach to “base erosion and profit shifting” by multinational companies. Multiple member jurisdictions, including the countries in which we operate, have begun implementing recommended changes such as country-by-country reporting requirements and changes to double tax treaties. Additional multilateral changes are anticipated in upcoming years. We often rely on generally available interpretations of applicable tax laws, treaties and regulations. There cannot be certainty that the relevant tax authorities are in agreement with our interpretation of these laws, regulations or treaties, or with tax positions that we have taken. If our interpretation or tax position is challenged by the relevant tax authorities, we could be required to pay taxes that we currently do not collect or pay, may be subject to interest and penalties and there could be an increase to the costs of our services to track and collect such taxes, which could increase our costs of operations or our effective tax rate. Similarly, a tax authority could assert that we are subject to tax in a jurisdiction where we believe we have not established a taxable connection, often referred to as a “permanent establishment” under international tax treaties, and such an assertion, if successful, could increase our expected tax liability in one or more jurisdictions. The occurrence of any of the foregoing tax risks could have a material adverse effect on our business, financial condition and results of operations.

We are unable to predict what national or international tax reform may be proposed or enacted in the future or what effect such changes would have on our business, but such changes, to the extent they are brought into tax legislation, regulations, policies or practices, could impact the tax treatment of our earnings, adversely affect our profitability and increase the complexity, burden and cost of tax compliance.

We have significant net operating losses, or NOLs, and U.K. carryforward tax losses which we may not be able to realize or which may be restricted following the Reorganization Transactions or any future change of control. We also benefit from certain tax incentive regimes, such as research and development tax credits, in the jurisdictions in which we operate and any adverse change to these regimes, the application thereof or challenges to the tax position we have adopted under these regimes could adversely affect our results of operations and financial condition.

As of December 31, 2018, we had federal and state NOL carryforwards in the United States of $14.2 million and $14.2 million, respectively, and cumulative carryforward tax losses in the United Kingdom of

$94.1 million, which we expect to be available to reduce future taxable income subject to any relevant restrictions (including those in the UK that limit the percentage of profits that can be reduced by carried forward losses). The U.S. federal and state NOL carryforwards incurred prior to January 1, 2018 in the amount of approximately $6.8 million and $6.7 million will begin to expire in 2036. The U.K. carryforward tax losses will continue indefinitely, subject to relevant restrictions, under current UK legislation. Under the Tax Cuts and Jobs Act of 2017, U.S. federal NOL carryforwards generated after December 31, 2017 are not subject to expiration but such NOLs may only offset 80% of taxable income. As of December 31, 2018, we also had orphan drug and research and development credits in the U.S. in the amount of $1.1 million.

The NOL carryforwards and U.K. carryforward tax losses are subject to review and possible adjustment by the U.S., U.K. and state tax authorities. NOL carryforwards and U.K. carryforward tax losses may become subject to limitations in the event of certain cumulative changes in the ownership interest of significant shareholders, as defined under Sections 382 Internal Revenue Code, as well as the Corporation Tax Act 2010 Part 14 under the UK tax rules. This could limit the amount of NOLs or carryforward tax losses that we can utilize annually to offset future taxable income or tax liabilities. We have conducted a review of changes in the ownership interest of significant shareholders and determined that as of December 31, 2018, there were no limitations in the U.K. However, for U.S. purposes, we have determined that a change of ownership occurred in April 2016. We are still in the process of determining the annual limitation on losses that occurred prior to April 2016. Subsequent ownership changes and changes to the UK (or US) tax rules in respect of the utilization of losses carried forward may further affect the limitation in future years.

Additionally, we have not undertaken a study on the completeness of the U.S. research and development and orphan drug credits. As such, the U.S. research and development and orphan drug credits may change and may be subject to review and adjustment by the tax authorities.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not applicable.

ITEM 2. PROPERTIES

Our principal office is located at 430 East 29th Street, 10th Floor, New York, New York 10016, USA, where we lease 5,887 square feet of office space. We lease this office space under a lease that terminates on December 31, 2021.

We also own a long leasehold interest in the ground rights where our 29,000 square foot manufacturing facility is located, at 92 Britannia Walk, London N1 7NQ, United Kingdom. The long leasehold interest is for 125 years, expiring in 2126, and there is no rent payable thereunder.

ITEM 3. LEGAL PROCEEDINGS

We are not subject to any material legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF SECURITIES

Market Information

On June 8, 2018, our ordinary shares began trading on the Nasdaq Global Market under the symbol "MGTX." Prior to that time, there was no public market for our stock.

Holders of Record

As of March 15, 2019, there were 75 holders of record. The actual number of shareholders of our ordinary shares is greater than this number of record holders and includes shareholders who are beneficial owners but whose ordinary shares are held in street name by brokers and other nominees. This number of holders of record also does not include shareholders whose ordinary shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid any cash dividends on our ordinary shares. We intend to retain future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future. However, if we do pay a cash dividend on our ordinary shares in the future, we will only pay such dividend out of our profits or share premium (subject to solvency requirements) as permitted under Cayman Islands law.

Recent Sales of Unregistered Securities

On March 1, 2019, we consummated a private placement with various investors, including one of our existing shareholders, Perceptive Life Sciences Master Fund Ltd., pursuant to which we issued and sold an aggregate of 5,797,102 of our ordinary shares at a purchase price of $13.80 per share. Evercore Group L.L.C. and Chardan Capital Markets LLC served as co-lead placement agents for the private placement and received an aggregate underwriting commission of approximately $2.4 million. The financing was led by Johnson & Johnson Innovation – JJDC, Inc. ("JJDC"), the investment arm of Johnson & Johnson, which made a $40.0 million equity investment in the Company and received 2,898,550 ordinary shares. Each of the foregoing securities issuances were in reliance on the exemption contained in Section 4(a)(2) of the Securities Act, as transactions by issuers not involving a public offering.

In connection with our amendment and restatement of our license agreements with UCLB whereby we agreed to issue UCLB £1.5 million of the Company's ordinary shares, on March 21, 2019, we issued 158,832 of the Company's ordinary shares to UCLB. This securities issuance was in reliance on the exemption contained in Section 4(a)(2) of the Securities Act, as a transaction by issuers not involving a public offering.

Use of Proceeds

On June 12, 2018, we completed our IPO and issued and sold 5,000,000 ordinary shares at a price to the public of $15.00 per share receiving $65.2 million in proceeds after deducting underwriting discounts and commissions and offering expenses payable by us. There has been no material change in the expected use of the net proceeds from our IPO as described in our prospectus. As of December 31, 2018, we had used $34.1 million of such net proceeds and had total unrestricted cash and cash equivalents of $68.1 million.

ITEM 6. SELECTED FINANCIAL DATA

Not applicable.

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of financial condition and operating results our financial statements and the related notes appearing at the end of this Form 10-K. This discussion contains forward-looking statements that involve risks and uncertainties. As a result of many factors, such as those set forth in the section of this Form 10-K captioned "Item 1A. Risk Factors" and elsewhere in this Form 10-K, our actual results may differ materially from those anticipated in these forward-looking statements. For convenience of presentation some of the numbers have been rounded in the text below.

Overview

We are a vertically integrated, clinical stage gene therapy company with five programs in clinical development and a broad pipeline of preclinical and research programs. We have core capabilities in viral vector design and optimization, gene therapy manufacturing as well as a potentially transformative gene regulation technology. Led by an experienced management team, we have taken a portfolio approach by licensing, acquiring and developing technologies that give us depth across both product candidates and indications. Though initially focusing on the eye, salivary gland and central nervous system, we intend to expand our focus in the future to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

We are an exempted company incorporated under the laws of the Cayman Islands in 2018, and prior to that, we commenced operations as MeiraGTx Limited, a private limited company incorporated under the laws of England and Wales in 2015. Our discussion of our financial condition and results of operations is based upon our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. Since our formation, we have devoted substantially all of our resources to developing our technology platform, establishing our viral vector manufacturing facility and developing manufacturing processes, advancing the product candidates in our ophthalmology, salivary gland and neurodegenerative disease programs, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, and providing general and administrative support for these operations. In 2016, we completed the acquisition of assets held by BRI-Alzan, Inc., a Delaware corporation, including a worldwide license agreement to develop certain preclinical technology for the treatment of ALS. In October 2018, we acquired Vector Neurosciences, Inc., a Delaware corporation. In connection with that acquisition, we acquired its AAV-GAD gene therapy program which had completed a randomized, sham-controlled Phase 2 study for treatment of Parkinson's disease. To date, we have financed our operations primarily with cash on hand and proceeds from the sales of our Series A ordinary shares, Convertible Preferred C Shares and ordinary shares. Through December 31, 2018, we received gross proceeds of approximately $203.7 million from sales of our ordinary shares, Series A ordinary shares and Convertible Preferred C Shares. As of December 31, 2018, we had cash and cash equivalents of $68.1million.

We are a clinical stage company and have not generated any product revenues to date. We have five clinical programs and a pipeline of preclinical programs. Since inception, we have incurred significant operating losses. Our net losses for the years ended December 31, 2018 and 2017 were $82.9 million and $31.1 million, respectively. As of December 31, 2018, we had an accumulated deficit of $148.3 million. While we do not expect to generate revenue from sales of any products for several years, if at all, in January 2019, we entered into the Collaboration Agreement, which provides for Janssen to pay us an $100 million upfront payment and provide us with research funding, and we are eligible to receive potential milestone payments and royalties.

Our total operating expenses were $78.1 million and $31.7 million for the years ended December 31, 2018 and 2017, respectively. While we expect our operating expenses to increase substantially in connection with our ongoing development activities related to our product candidates, we believe that these increases may be partially offset by the research funding in connection with a collaboration, option and license agreement we entered into in January 2019. We anticipate that our expenses will increase due to costs associated with our

clinical development program targeting in achromatopsia due to mutations in the *CNGB3* or *CNGA3* gene, inherited retinal dystrophy caused by mutations in *RPE65*, or RPE65-deficiency, and X-Linked retinitis pigmentosa, or XLRP. In addition, we expect to continue incurring increasing costs associated with our clinical activities for *hAQP1* for the treatment of radiation-induced xerostomia and xerostomia associated with Sjogren's syndrome. We are currently evaluating potential next steps for clinical development of AAV-GAD, which remains pending future discussions with regulatory agencies. We also expect to incur expenses related to research activities in additional therapeutic areas to expand our pipeline, hiring additional personnel in manufacturing, research, clinical trials, quality and other functional areas, and associated cash and share-based compensation expense, as well as the further development of internal manufacturing capabilities and capacity and other associated costs including the management of our intellectual property portfolio.

As a result of these anticipated expenditures, we will require additional capital, which we may raise through equity offerings, debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable us to complete the development and potential commercialization of our product candidates. Furthermore, we expect to continue incurring costs associated with being a public company. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Based on our cash and cash equivalents at December 31, 2018 and after receiving an additional $77.4 million from a private placement of ordinary shares during the first quarter of 2019 and our receipt of the $100 million payment under the terms of the Collaboration Agreement, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into 2022. We have based these estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. See "—Liquidity and Capital Resources." Because of the numerous risks and uncertainties associated with the development of our product candidates, any future product candidates, our platform and technology and because the extent to which we may enter into collaborations with third parties for development of any of our product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

- the initiation, progress, timing, costs and results of our planned clinical trials for our product candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, EMA and other regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or any of our product candidates;
- the effect of competing technological and market developments;
- the costs and timing of further developing our manufacturing facilities in the United Kingdom;
- the costs of operating as a public company.
- the extent to which we in-license or acquire other products and technologies;

- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where we choose to commercialize our products; and
- the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

Adequate additional funds may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a shareholder. Any future debt financing or preferred equity or other financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute your ownership interests.

If we raise additional funds through collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce, or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenue from product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

Highlights and Recent Development

On October 5, 2018, we acquired Vector Neurosciences Inc. (“Vector”) and acquired Vector’s rights to the clinical stage gene therapy product candidate adeno-associated virus encoding glutamic acid decarboxylase (“AAV-GAD”), an investigational gene therapy medicine ready for continued Phase 2 clinical development for Parkinson’s disease. We issued an aggregate of 225,000 of our ordinary shares as initial merger consideration, consisting of 202,500 shares which were issued at the closing of the merger and an additional 22,500 shares to be issued 18 months following the closing.

On December 14, 2018, we acquired from Moorfields Eye Hospital NHS Foundation Trust of Moorfields Eye Hospital a long leasehold interest (the “Head Lease”) in the site of our manufacturing facility, 92 Britannia Walk, London N1 7LU, for a purchase price of approximately $6,615,000. As a result of this transaction, we are now the tenant under the Head Lease, which has a remaining term of 108 years, with no facility rent due.

In January and February, 2019, we amended and restated the following agreements: (i) the License Agreement, dated February 4, 2015, as amended, between Athena Vision Ltd. and UCLB; (ii) the License Agreement, dated July 28, 2017, as amended, between MeiraGTx UK II Limited and UCLB; and (iii) the License Agreement, dated March 15, 2018, among MeiraGTx Limited, MeiraGTx UK II Limited and UCLB to establish new stand-alone license agreements for our inherited retinal disease (“IRD”) programs. In connection with the stand-alone agreement related to CNGB3, we agreed to make an upfront payment to UCLB of £1,500,000 and issue £1,500,000 of our ordinary shares.

On January 30, 2019, we entered into a strategic collaboration with Janssen to develop and commercialize gene therapies for the treatment of inherited retinal diseases (IRDs). This Collaboration

Agreement provides for Janssen to pay us an $100 million upfront payment and provide us with research funding, and we are eligible to receive potential milestone payments and royalties. We will collaborate with Janssen to develop our current clinical programs in Retinitis Pigmentosa and two genetic forms of Achromatopsia and Janssen has the exclusive right to commercialize these products globally. We will manufacture these products for commercial supply. Janssen will pay 100% of the clinical and commercialization costs of the products and we are eligible to receive untiered 20 percent royalties on net sales of products and additional development and commercialization milestones of up to $340 million. In addition, we will enter a research collaboration with Janssen in the area of IRDs, with Janssen paying for the majority of the research costs. Janssen has the right to exclusively license any product coming out of the collaboration at the time of an IND. Janssen will then pay 100% of the clinical and commercialization costs for these products and we will receive an untiered royalty in the high teens on net sales as well as development milestones. In addition, we have entered into a manufacturing research collaboration with Janssen to further develop processes for manufacturing AAV viral vectors in which the costs of the research will be shared.

On March 1, 2019, we consummated a private placement with various investors, including one of our existing shareholders Perceptive Life Sciences Master Fund Ltd., pursuant to which we issued and sold an aggregate of 5,797,102 ordinary shares for gross proceeds of approximately $80.0 million. The financing was led by JJDC, the investment arm of Johnson & Johnson, which made a $40.0 million equity investment in the Company and received 2,898,550 ordinary shares.

Components of Our Results of Operations

Operating Expenses

Our operating expenses since inception have consisted primarily of general and administrative costs and research and development costs.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including share-based compensation, for personnel in our executive, finance, business development and administrative functions. General and administrative expenses also include legal fees relating to intellectual property and corporate matters; professional fees for accounting, auditing, tax and consulting services; insurance costs; travel expenses; and office facility-related expenses, which include direct depreciation costs.

We expect that our general and administrative expenses will increase in the future as we increase our personnel headcount to support increased research and development activities. We have also incurred and expect to continue to incur increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with Nasdaq and SEC requirements; director and officer insurance costs; and investor and public relations costs.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of our product candidates, and include:

- employee-related expenses, including salaries, benefits and travel of our research and development personnel;
- expenses incurred in connection with third-party vendors that conduct clinical and preclinical studies and manufacture the drug product for the clinical trials and preclinical activities;
- acquisition of in-process research and development;

- costs associated with clinical and preclinical activities including costs related to facilities, supplies, rent, insurance, certain legal fees, share-based compensation, and depreciation; and
- expenses incurred with the development and operation of our manufacturing facility.

We expense research and development costs as incurred.

We typically use our employee and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, license payments made under our licensing arrangements or other internal costs to specific development programs or product candidates. These costs are included in other research and development expenses in the table below.

The following table summarizes our research and development expenses:

	Year Ended December 31,		
	2018	2017	Change
Ophthalmology programs	$ 7,069,280	$ 4,133,015	$ 2,936,265
Salivary gland programs	1,136,355	913,706	222,649
Neurodegenerative diseases programs	5,164,761	2,220,843	2,943,918
Manufacturing	5,224,272	3,213,861	2,010,411
Other research and development costs	15,025,555	11,878,287	3,147,268
Total research and development expenses	$ 33,620,223	$ 22,359,712	$ 11,260,511

Research and development activities are central to our business model. We expect that our research and development expenses will continue to increase substantially for the foreseeable future as we initiate additional preclinical and clinical trials of our existing product candidates and continue to discover and develop additional product candidates. This increase in research and development costs may be partially offset by the research funding provided in connection with the Collaboration Agreement we entered into in January 2019

We cannot determine with certainty the duration and costs of future clinical trials of our product candidates or any other product candidate we may develop or if, when, or to what extent we will generate revenue from the commercialization and sale of any product candidate for which we obtain marketing approval. We may never succeed in obtaining marketing approval for any product candidate. The duration, costs and timing of clinical trials and development of our existing product candidates or any other product candidate we may develop will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of clinical trials of our existing product candidates, as well as of any future clinical trials of other product candidates and other research and development activities that we may conduct;
- uncertainties in clinical trial design and patient enrollment rates;
- the actual probability of success for our product candidates, including the safety and efficacy, early clinical data, competition, manufacturing capability and commercial viability;
- significant and changing government regulation and regulatory guidance;
- the timing and receipt of any marketing approvals; and
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that

product candidate. For example, if the FDA or another U.S. or foreign regulatory authority were to require us to conduct clinical trials beyond those that we anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in our clinical trials due to patient enrollment or other reasons, we would be required to expend significant additional financial resources and time on the completion of clinical development.

Other non-operating income (expense)

Other non-operating income (expense) includes the following:

Foreign currency (loss)gain

Our consolidated financial statements are presented in U.S. dollars, which is our reporting currency. The financial position and results of operations of our subsidiaries MeiraGTx UK II and MeiraGTx B.V. are measured using the foreign subsidiaries' local currency as the functional currency. MeiraGTx UK II cash accounts holding U.S. dollars are remeasured based upon the exchange rate at the date of remeasurement with the resulting gain or loss included in the consolidated statement of operations and comprehensive loss. Expenses of such subsidiaries have been translated into U.S. dollars at average exchange rates prevailing during the period. Assets and liabilities have been translated at the rates of exchange on the consolidated balance sheet date. The resulting translation gain and loss adjustments are recorded directly as a separate component of shareholders' equity and as other comprehensive loss on the consolidated statement of operations and comprehensive loss.

Convertible note inducement expense

Convertible note inducement expense consists of the issuance of a warrant to a convertible note holder as an inducement to convert the note payable into Series C preferred shares.

Change in fair value of warrant liability

We have determined that our warrants are classified as liabilities on our balance sheet because the Preferred Shares underlying the warrants have a redemption feature in the event of a change of control of the Company. The fair values of the warrants are estimated using the Black-Scholes valuation model with certain assumptions regarding risk free interest rate, expected volatility, expected divided yield and expected life. The Black-Scholes value of the warrants was recorded as a warrant liability and is remeasured quarterly. Any changes in the quarterly valuation of the warrants is charged to operations.

Income Taxes

The 2018 income tax provision consisted of current tax expense of $0 and a deferred tax benefit of $474,391. The 2018 deferred tax benefit includes a $474,391 benefit recorded due to the required intraperiod tax allocation resulting from the loss from continuing operations and other comprehensive income.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing in this Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Consolidation

The accompanying consolidated financial statements include the accounts of Meira Holdings and its wholly owned subsidiaries:

MeiraGTx Limited, a limited company under the laws of England and Wales ("Meira Limited);

MeiraGTx, LLC, a Delaware corporation ("Meira LLC");

BRI-Alzan, Inc., a Delaware corporation ("BRI-Alzan");

MeiraGTx B.V., a Netherlands corporation ("Meira BV");

MeiraGTx Neurosciences, Inc. a Delaware corporation ("Meira Neuro");

MeiraGTx UK II Limited, ("Meira UK II"), a limited company under the laws of England and Wales; and

MeiraGTx UK Limited ("Meira UK"), a limited company under the laws of England and Wales.

All intercompany balances and transactions between the consolidated companies have been eliminated in consolidation.

Foreign Currencies

Our consolidated financial statements are presented in U.S. dollars, our reporting currency. The financial position and results of operations of MeiraGTx UK II and MeiraGTx UK II cash accounts holding U.S. dollars are remeasuring based upon the exchange rate at the date of remeasurement with the resulting gain or loss included in the consolidated statement of operations and comprehensive loss. Expenses of such subsidiaries have been translated into U.S. dollars at average exchange rates prevailing during the period. Assets and liabilities have been translated at the rates of exchange on the consolidated balance sheet dates. The resulting translation gain and loss adjustments are recording directly as a separate component of shareholders' equity and as other comprehensive loss on the consolidated statements of operations and comprehensive loss.

Income Taxes

Since we have recurring losses and a valuation allowance against deferred tax assets, there was no tax expense (benefit) through December 31, 2017. For the year ended December 31, 2018, we recognized a tax benefit of $(474,391). As of December 31, 2018, we had federal and state net operating loss ("NOL") carryforwards in the United States of approximately $14.2 million and $14.2 million respectively, and in the U.K. of approximately $94.1 million, which are available to reduce future taxable income. The U.S. federal and state NOL carryforwards incurred prior to January 1, 2018 in the amount of approximately $6.8 million and $6.7 million, respectively, will begin to expire in 2036. The U.S. NOL incurred after December 31, 2017 and the U.K. NOL will be indefinitely carried forward. As of December 31, 2018, we also had orphan drug and research and development credits in the U.S. in the amount of $1.1 million, which will begin to expire 2036.

Research and Development

Research and development costs are charged to expense as incurred. These costs include, but are not limited to, employee-related expenses, including salaries, benefits and travel of our research and development personnel; expenses incurred under agreements with contract research organizations and investigative sites that

conduct clinical and preclinical studies and manufacture the drug product for the clinical studies and preclinical activities; acquisition of in-process research and development; facilities; supplies; rent, insurance, certain legal fees, stock-based compensation, depreciation and other costs associated with clinical and preclinical activities and regulatory operations. Refundable research and development tax credits received are recorded as an offset to these costs.

Costs for certain development activities, such as outside research programs funded by us, are recognized based on an evaluation of the progress to completion of specific tasks with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense, as the case may be.

Share-Based Compensation

Options

We grant share options to employees, non-employee members of our board of directors and non-employee consultants as compensation for services performed. Employee and non-employee members of the board of directors' awards of share-based compensation are accounted for in accordance with ASC 718, Compensation—Stock Compensation, or ASC 718. ASC 718 requires all share-based payments to employees and non-employee directors, including grants of share options, to be recognized in the statement of operations and comprehensive loss based on their grant date fair values. The grant date fair value of share options is estimated using the Black-Scholes option valuation model.

Using this model, fair value is calculated based on assumptions with respect to (i) the fair value our ordinary shares on the grant date; (ii) expected volatility of our ordinary share price, (iii) the periods of time over which employees and members of our board of directors are expected to hold their options prior to exercise (expected term), (iv) expected dividend yield on our ordinary shares, and (v) risk-free interest rates.

Our ordinary shares were not traded on a public exchange prior to our IPO in June 2018. Therefore, we believe that our future volatility will differ materially during the expected term from the volatility that would be calculated from our historical share prices to date. Consequently, expected volatility is based on an analysis of guideline companies in accordance with ASC 718. The expected dividend yield is zero as we have never paid dividends and do not currently anticipate paying any in the foreseeable future. Risk-free interest rates are based on quoted U.S. Treasury rates for securities with maturities approximating the option's expected term.

As of January 1, 2016, we early adopted ASU 2016-09, Improvements to Employee Share-Based Payment Accounting, and accounts for forfeitures as they occur from that date. Additionally, excess tax benefits and deficiencies will be recognized as income tax expense or benefit in the income statement. There was no cumulative effect adjustment as we did not issue any options prior to January 1, 2016.

We had accounted for options granted to non-employee consultants under ASC 505-50, Equity-Based Payments to Non-Employees. As such, we estimate the fair value of each such option using the Black-Scholes model, with the expected term of share options granted to non-employees initially equal to the options' maximum contractual life of ten years, at issuance. On each subsequent reporting date until performance is complete, we revalue all outstanding options granted to non-employee consultants during the vesting period of each tranche. Under ASC 505-50, upon re-measurement of each award, income or expense is recognized during its vesting term. Compensation cost relating to awards with service-based graded vesting schedules is recognized as general and administrative and research and development expenses in the consolidated statement of operations and comprehensive loss using the straight-line method. On July 1, 2018, we early adopted ASU 2018-07, Improvements to Nonemployee Share-Based Payment Accounting, which simplifies the accounting for share-based payments granted to nonemployees for goods and services. The adoption did not have a material effect on the consolidated financial statements.

Restricted Shares

In connection with certain service agreements and research agreements, we have granted restricted ordinary shares as compensation. The shares are recognized in the statement of operations and comprehensive loss based on their grant date fair values. Compensation cost relating to share grants with service-based graded vesting schedules is recognized based on the vesting schedule.

Results of Operations

The following table summarizes our results of operations for the years ended December 31, 2018 and 2017, respectively

	2018	2017	Change
Operating expenses:			
General and administrative	$ 44,483,938	$ 9,325,017	$ 35,158,921
Research and development	33,620,223	22,359,712	11,260,511
Total operating expenses	78,104,161	31,684,729	46,419,432
Loss from operations	(78,104,161)	(31,684,729)	(46,419,432)
Other non-operating income (expense)			
Other income	83,075	—	83,075
Foreign currency (loss) gain	(3,824,383)	1,676,117	(5,500,500)
Convertible note inducement expense	—	(553,500)	553,500
Change in fair value of warrant liability	(1,514,775)	(465,633)	(1,049,142)
Interest income	53,408	26,073	27,335
Interest expense	(33,429)	(42,863)	9,434
Loss before income taxes	(83,340,265)	(31,044,535)	(52,295,730)
Benefit for income taxes	474,391	—	474,391
Net loss	$ (82,865,874)	$ (31,044,535)	$ (51,821,339)

General and Administrative Expenses

General and administrative expenses were $44.5 million for the year ended December 31, 2018, compared to $9.3 million for the year ended December 31, 2017. The increase of $35.2 million was primarily due to increases of $19.7 million in payroll, $14.0 million in share-based compensation, $1.1 million in legal, $0.7 million in insurance $0.5 million in accounting, $0.4 million in travel, and $0.3 million in investor relations, which was partially offset by a decrease of $1.5 million in rent.

Research and Development Expenses

Research and development expenses for the year ended December 31, 2018 were $33.6 million, compared to $22.4 million for the year ended December 31, 2017. The increase of $11.2 million was primarily due to an increase in costs of $3.0 million for clinical trial costs related to our ophthalmology programs, $3.0 million for acquired neurology research and development, $2.3 million related costs of payroll and consultants, $1.6 million of depreciation, $1.3 million related to the preparation for production of our manufacturing facility, $1.0 million in share-based compensation, and $0.7 million in rent, which was partially offset an increase of $1.7 million in the research and development credit in the United Kingdom in 2018.

Foreign Currency (Loss) Gain

Foreign currency loss was $3.8 million for the year ended December 31, 2018 compared to a gain of $1.7 million for the year ended December 31, 2017. The change of $5.5 million was primarily due to a strengthening of the U.S. dollar against the pound sterling in 2018.

Convertible Note Inducement Expense

We recorded a $0.5 million convertible note inducement expense for the year ended December 31, 2017 primarily due to the issuance of a warrant to purchase 231,898 Convertible Preferred C Shares in 2017 to a convertible noteholder as an inducement to convert the note into Convertible Preferred C Shares.

Change in Fair Market Value of Warrant Liability

We recorded $1.5 million change in fair value of a warrant liability for the year ended December 31, 2018, compared to $0.5 million for the year ended December 31, 2017. The increase of $1.0 million was primarily due to the revaluation of certain warrants, which were issued to certain investors in September and November 2017, using the Black-Scholes valuation model at June 7, 2018, when the warrants were exercised and December 31, 2017.

Income Taxes

The 2018 income tax provision consisted of current tax expense of $0 and a deferred tax benefit of $474,391. The 2018 deferred tax benefit includes a $474,391 benefit recorded due to the required intraperiod tax allocation resulting from the loss from continuing operations and other comprehensive income.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We have not generated positive cash flows from operations, and there are no assurances that we will be successful in obtaining an adequate level of financing for the development and commercialization of our product candidates. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the preclinical and clinical development of our product candidates. We expect that our research and development and general and administrative costs will increase in connection with conducting preclinical studies and clinical trials for our product candidates, building out internal capacity to have product manufactured to support preclinical studies and clinical trials, expanding our intellectual property portfolio, and providing general and administrative support for our operations. As a result, we will need additional capital to fund our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements, or other sources.

We are required to maintain a stand-by letter of credit as a security deposit under a certain lease with ARE, an entity that is under common control with an entity that is a minority shareholder of the Company and whose executive chairman and founder is on our board of directors. Our bank requires us to maintain restricted cash balances to serve as collateral for the letter of credit issued to the landlord by the bank. As of December 31, 2018, the restricted cash balances for the ARE lease was invested in a commercial money market account. The restricted cash balance for the ARE lease remains at $123,376 through the end of the lease term in December 2021, plus three months. We had $123,376 of restricted cash included in long-term assets as of December 31, 2018. We do not currently have any approved products and have never generated any revenue from product sales. We have historically financed our operations primarily through the sale of our ordinary shares and Convertible Preferred C Shares. In January 2019, we entered into the Collaboration Agreement, which provides for Janssen to pay us an $100 million upfront payment and provide us with research funding, and we are eligible to receive potential milestone payments and royalties.

Cash Flows

We had $68.1 million and $8.5 million of cash and cash equivalents as of December 31, 2018 and 2017, respectively.

The following table summarizes our sources and uses of cash for the period presented:

	For the year ended December 31, 2018	2017
Net cash used in operating activities	$ (58,887,870)	$(18,055,386)
Net cash used in investing activities	(11,258,479)	(10,535,717)
Net cash provided by financing activities	130,040,415	19,340,215
Increase (decrease) in cash	$ 59,894,066	$ (9,250,888)

Operating Activities

During the year ended December 31, 2018, our cash used in operating activities of $58.9 million was primarily due to our net loss of $82.9 million as we incurred expenses associated with research activities on our clinical programs and research activities for our other product candidates and incurred general and administrative expenses. The loss included non-cash charges of $27.9 million, which consisted of $18.1 million of share-based compensation, issuance of shares for acquired research and development in the amount of $3.0 million, change in fair value of warrant liability in the amount of $1.5 million, depreciation of $2.1 million, and a foreign currency loss of $3.9 million, which was partially offset by an income tax benefit of $0.5 million. Additionally, current assets, consisting of prepaid expenses, other current assets and security deposits increased by $3.9 million. Current liabilities, consisting of accounts payable, accrued expenses, deferred rent, due to affiliate and other liabilities, decreased by $0.1 million

During the year ended December 31, 2017, our cash used in operating activities of $18.1 million was primarily due to our net loss of $31.0 million as we incurred expenses associated with research activities on our clinical programs and research activities for our other product candidates and incurred general and administrative expenses. The loss included non-cash charges of $3.0 million, which consisted of $3.0 million of share-based compensation, change in fair value of warrant liability in the amount of $0.5 million, convertible note inducement expense of $0.5 million and depreciation of $0.7 million, which was partially offset by a foreign currency gain of $1.7 million. Additionally, current liabilities, consisting of accounts payable, accrued expenses deferred rent, and due to affiliate, increased by $11.1 million. Current assets, consisting of prepaid expenses and other current assets, decreased by $1.2 million.

Investing Activities

Net cash used in investing activities for the years ended December 31, 2018 and December 31, 2017 of $11.3 million and $10.5 million, respectively, consisted of purchase of property plant and equipment, primarily for our manufacturing facility.

Financing Activities

Net cash provided by financing activities was $130.0 million for the year ended December 31, 2018, represented proceeds of $65.6 million from the issuance of Ordinary Shares in connection with our initial public offering, $56.1 million from the issuance of Convertible Preferred C Shares and $9.7 million from the exercise of warrants, which was partially offset by the repayment of a note payable in the amount of $1.4 million.

Net cash provided by financing activities was $19.3 million for the year ended December 31, 2017, represented proceeds of $16.8 million from the issuance of Convertible Preferred C Shares and $2.5 million from the issuance of a note payable

Funding Requirements

Our operating expenses increased substantially in 2018 and 2017 and are expected to increase substantially in the future in connection with our ongoing activities, particularly as we advance our clinical activities including scale-up of manufacturing processes and additional clinical trials. In addition, we expect to continue to incur additional costs associated with operating as a public company.

Specifically, our expenses will increase as we:

- pursue the preclinical and clinical development of our product candidates;
- scale up our manufacturing processes and capabilities to support our preclinical studies and clinical trials of our product candidates;
- in-license or acquire the rights to other products, product candidates or technologies;
- maintain, expand and protect our intellectual property portfolio;
- hire additional personnel in research, manufacturing and regulatory and clinical development as well as management personnel; and
- continue to expand our operational, financial and management systems and increase personnel, including personnel to support our operations as a public company.

Based on our planned use of the net proceeds of the IPO, our current cash and cash equivalents, the proceeds from a private placement of ordinary shares in March 2019 and the proceeds we are to receive pursuant to the terms of the Collaboration Agreement, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

Because of the numerous risks and uncertainties associated with research, development and commercialization of gene therapies, it is difficult to estimate with certainty the amount of our working capital requirements. Our future funding requirements will depend on many factors, including:

- the progress, costs and results of our preclinical development and initial clinical trials for our gene therapy programs;
- the progress, costs and results of our additional clinical, research and preclinical development programs in gene therapy;
- the costs and timing of process development and manufacturing scale-up activities associated with our clinical programs;
- our ability to establish and maintain strategic collaborations, licensing or other agreements and the financial terms of such agreements;
- the scope, progress, results and costs of any product candidates that we may derive from our platform technology or any other product candidates that we may develop;
- the extent to which we in-license or acquire rights to other products, product candidates or technologies; and
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against any intellectual property-related claims.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaboration agreements, other third-party funding, strategic alliances, licensing arrangements and marketing and distribution arrangements.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of ordinary shares. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through other third-party funding, collaboration agreements, strategic alliances, licensing arrangements or marketing and distribution arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following is a summary of our significant contractual obligations as of December 31, 2018.

		Payments by Period			
	Total	Less Than 1 Year	More Than 1 Year and Less Than 3	More Than 3 Years and Less Than 5	More Than 5 Years
Operating leases	4,615,676	889,465	1,828,838	700,569	1,196,805
Capitalized leases	35,894	35,894	—	—	—
	4,651,570	925,359	1,828,838	700,569	1,196,805

(1) Represents the leases for office, laboratory, and manufacturing space in London, United Kingdom and New York, New York under non-cancelable operating leases that expire between December 2021 and May 2027 and December 2021.
(2) Represents future payments under capitalized leases for office equipment.

The contractual obligations table does not include any potential future payments we may be required to make under (1) our license agreements with UCLB, Brandeis and other entities or (2) our sponsored research agreements with universities and commercial research organizations. These agreements may be terminated upon 30-90 days written notice and, therefore, the amounts to be paid by us are not fixed or determinable at this time.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements under applicable SEC rules and do not have any holdings in variable interest entities.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

We are exposed to market risks in the ordinary course of our business. These risks primarily include foreign currency exchange rate sensitivities. However, relative to foreign currency exposures as of December 31, 2018, a 10% unfavorable movement in foreign currency exchange rates would not expose us to a significant increase in net loss. We had cash and cash equivalents of $68.1 million as of December 31, 2018, which consist of non-interest-bearing bank deposits. Other than accounts payable and accrued expenses incurred in the ordinary course of business, we had no other debt outstanding as of December 31, 2018. We had cash and cash equivalents of $8.5 million as of December 31, 2017, which consist of bank deposits and money market funds. Such interest-earning instruments carry a degree of interest rate risk; however, historical fluctuations in interest income have not been significant for us. Other than accounts payable and accrued expenses incurred in the ordinary course of business, we had a note payable of $1.4 million as of December 31, 2017.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

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MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
FOR THE YEARS ENDED DECEMBER 31, 2018 AND 2017

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Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of MeiraGTX Holdings plc and Subsidiaries

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of MeiraGTX Holdings plc and Subsidiaries (the Company) as of December 31, 2018 and 2017, the related consolidated statements of operations and comprehensive loss**,** shareholders' deficit and cash flows for each of the two years in the period ended December 31, 2018, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2018, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2016.

Stamford, Connecticut
March 26, 2019

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS

	December 31, 2018	December 31, 2017
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 68,080,175	$ 8,548,638
Prepaid expenses	1,937,785	1,961,243
Other current assets	4,634,105	965,233
Total Current Assets	74,652,065	11,475,114
Property, plant and equipment, net	22,014,237	14,255,729
Security deposits	105,085	—
Restricted cash	123,376	123,376
TOTAL ASSETS	$ 96,894,763	$ 25,854,219
LIABILITIES, CONVERTIBLE PREFERRED C SHARES AND SHAREHOLDERS' EQUITY (DEFICIT)		
CURRENT LIABILITIES:		
Accounts payable	$ 3,042,861	$ 7,055,380
Accrued expenses	11,991,697	9,332,944
Note payable	—	1,442,009
Warrant liability	—	2,679,633
Capitalized lease obligation—current portion	27,199	30,850
Due to Kadmon	—	861,030
Other current liabilities	437,053	—
Total Current Liabilities	15,498,810	21,401,846
Capitalized lease obligation	7,097	34,298
Deferred rent	201,264	266,290
Asset retirement obligation	128,119	178,419
TOTAL LIABILITIES	15,835,290	21,880,853
COMMITMENTS		
CONVERTIBLE PREFERRED C SHARES		
Convertible Preferred C Shares 0 and 5,005,935 outstanding at December 31, 2018 and December 31, 2017, respectively (liquidation preference of $52,455,700 at December 31, 2017)	—	51,338,631
SHAREHOLDERS' EQUITY (DEFICIT):		
Ordinary Shares, $0.00003881 nominal value, 1,288,327,750 authorized 27,386,632 issued and outstanding at December 31, 2018 8,826,190 issued and 8,714,563 issued and outstanding at December 31, 2017	1,064	342
Capital in excess of nominal value	229,054,460	20,080,713
Accumulated other comprehensive income (loss)	293,666	(2,022,477)
Accumulated deficit	(148,289,717)	(65,423,843)
Total Shareholders' Equity (Deficit)	81,059,473	(47,365,265)
TOTAL LIABILITIES, CONVERTIBLE PREFERRED C SHARES AND SHAREHOLDERS' EQUITY (DEFICIT)	$ 96,894,763	$ 25,854,219

See Notes to Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	For the Year Ended December 31, 2018	2017
Operating expenses:		
General and administrative	$ 44,483,938	$ 9,325,017
Research and development	33,620,223	22,359,712
Total operating expenses	78,104,161	31,684,729
Loss from operations	(78,104,161)	(31,684,729)
Other non-operating income (expense):		
Other income	83,075	—
Foreign currency (loss) gain	(3,824,383)	1,676,117
Convertible note inducement expense	—	(553,500)
Change in fair value of warrant liability	(1,514,775)	(465,633)
Interest income	53,408	26,073
Interest expense	(33,429)	(42,863)
Loss before income taxes	(83,340,265)	(31,044,535)
Benefit for income taxes	474,391	—
Net loss	(82,865,874)	(31,044,535)
Other comprehensive income (loss):		
Foreign currency translation, net of tax of $474,391 and $0 in 2018 and 2017, respectively	2,316,143	(1,361,365)
Total comprehensive loss	$ (80,549,731)	$ (32,405,900)
Net loss	$ (82,865,874)	$ (31,044,535)
Accretion on convertible preferred C shares and warrants	(1,806,512)	(806,963)
Adjusted net loss	$ (84,672,386)	$ (31,851,498)
Basic and diluted net loss per ordinary share	$ (4.47)	$ (3.72)
Weighted-average number of ordinary shares outstanding	18,948,520	8,572,315

See Notes to Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED C SHARES AND SHAREHOLDERS' EQUITY (DEFICIT)
AS OF DECEMBER 31, 2018

	Convertible Preferred C Shares		Shareholders' Equity (Deficit)					
	Shares	Amount	Ordinary Shares	Amount	Capital in Excess of Nominal Value	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Shareholders' Equity (Deficit)
Balance at December 31, 2016	1,574,739	$ 32,833,660	8,818,461	$ 342	$ 17,900,995	$ (661,112)	$ (34,379,308)	$ (17,139,083)
Exercised stock options	—	—	1,288	—	9,950	—	—	9,950
Issuance of A ordinary shares in connection with a license agreement	—	—	6,441	—	17,000	—	—	17,000
Extinguishment of convertible preferred C shares, net of unaccreted issuance costs	(1,584,469)	(33,115,157)	—	—	33,115,157	—	—	33,115,157
Issuance of convertible preferred C shares in connection with extinguishment	3,168,929	33,206,360	—	—	(33,206,360)	—	—	(33,206,360)
Conversion of note payable into convertible preferred C shares	238,579	2,500,000	—	—	—	—	—	—
Issuance of convertible preferred C shares, net of warrants and issuance costs	1,608,157	15,198,008	—	—	—	—	—	—
Accretion of issuance costs on convertible preferred C shares	—	100,760	—	—	(100,760)	—	—	(100,760)
Accretion of warrants issued in connection with convertible preferred C shares	—	615,000	—	—	(615,000)	—	—	(615,000)
Share-based compensation	—	—	—	—	2,959,731	—	—	2,959,731
Foreign currency translation	—	—	—	—	—	(1,361,365)	—	(1,361,365)
Net loss for the year ended December 31, 2017	—	—	—	—	—	—	(31,044,535)	(31,044,535)
Balance at December 31, 2017	5,005,935	$ 51,338,631	8,826,190	$ 342	$ 20,080,713	$ (2,022,477)	$ (65,423,843)	$ (47,365,265)
Issuance of convertible preferred C shares in connection with payables	129,419	1,356,129	—	—	—	—	—	—
Issuance of convertible preferred C shares in connection with a license agreement	13,360	140,000	—	—	—	—	—	—
Sale of convertible preferred C shares, net of issuance costs	5,425,124	56,159,119	—	—	—	—	—	—
Accretion of issuance costs on convertible preferred C shares	—	761,012	—	—	(761,012)	—	—	(761,012)
Accretion of warrants issued in connection with convertible preferred C shares	—	1,045,500	—	—	(1,045,500)	—	—	(1,045,500)
Exercise of warrants	927,594	9,720,000	—	—	4,194,408	—	—	4,194,408
Conversion of convertible preferred C shares into A ordinary shares	(11,501,432)	(120,520,391)	11,501,432	446	120,519,945	—	—	120,520,391
Sale of ordinary shares in initial public offering, net of issuance costs of $9,807,622	—	—	5,000,000	194	65,192,184	—	—	65,192,378
Issuance of ordinary shares in connection with Vector Neurosciences acquisition			202,500	9	2,990,241	—	—	2,990,250
Share-based compensation	—	—	1,856,510	73	17,883,481	—	—	17,883,554
Foreign currency translation, net of income taxes	—	—	—	—	—	2,316,143	—	2,316,143
Net loss for the year ended December 31, 2018	—	—	—	—	—	—	(82,865,874)	(82,865,874)
Balance at December 31, 2018	—	$ —	27,386,632	$ 1,064	$229,054,460	$ 293,666	$(148,289,717)	$ 81,059,473

See Notes to Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF CASH FLOWS

	For the Year Ended December 31, 2018	2017
Cash flows from operating activities:		
Net loss	$ (82,865,874)	$ (31,044,535)
Adjustments to reconcile net loss to net cash used in operating activities:		
Preferred C shares issued in connection with a license agreement	140,000	—
Issuance of shares for services	—	17,000
Share-based compensation expense	17,883,554	2,959,731
Foreign currency loss (gain)	3,824,383	(1,676,117)
Depreciation	2,053,220	679,177
Amortization of interest on asset retirement obligation	(38,301)	19,313
Change in fair value of warrant liability	1,514,775	465,633
Convertible note inducement expense	—	553,500
Issuance of shares for acquired research and development expense	2,990,250	—
Issuance of note payable in connection with lease termination	—	1,442,009
Benefit for income taxes	(474,391)	—
(Increase) in operating assets:		
Prepaid expenses	(35,465)	(669,756)
Other current assets	(3,684,465)	(493,424)
Security deposits	(115,573)	—
Increase (decrease) in operating liabilities:		
Accounts payable	(2,119,493)	4,728,491
Accrued expenses	2,529,568	4,969,619
Due to Kadmon	(861,030)	317,992
Other liabilities	436,161	—
Deferred rent	(65,189)	(324,019)
Net cash used in operating activities	(58,887,870)	(18,055,386)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(11,258,479)	(10,535,717)
Net cash used in investing activities	(11,258,479)	(10,535,717)
Cash flows from financing activities:		
Payments on capitalized lease obligation	(30,852)	(24,388)
Exercise of warrants	9,720,000	—
Proceeds from the sale of ordinary shares	69,750,000	—
Issuance costs in connection with ordinary shares	(4,115,843)	—
Issuance costs in connection with convertible preferred C shares	(690,475)	—
Proceeds from the sale of convertible preferred C shares	56,849,594	16,854,653
(Payment) issuance of note payable	(1,442,009)	2,500,000
Proceeds from excercised stock options	—	9,950
Net cash provided by financing activities	130,040,415	19,340,215
Net increase (decrease) in cash, cash equivalents and restricted cash	59,894,066	(9,250,888)
Effect of exchange rate changes on cash	(362,529)	1,417
Cash, cash equivalents and restricted cash at beginning of year	8,672,014	17,921,485
Cash, cash equivalents and restricted cash at end of year	$ 68,203,551	$ 8,672,014
Supplemental disclosure of non-cash transactions:		
Fixed asset acquisition included in accounts payable and accrued expenses at end of year	$ 293,051	$ 415,650
Issuance of convertible preferred C shares in connection with payables	$ 1,356,129	$ —
Conversion of convertible preferred C shares into ordinary shares	$ 120,520,391	$ —
Reclassification of warrant liability upon exercise of warrants	$ 4,194,408	$ —
Capitalized lease obligation for equipment purchase	$ —	$ 78,063
Issuance costs in connection with sale of ordinary shares in accounts payable and accrued expenses at end of period	$ 441,779	$ —
Conversion of note payable into convertible preferred C shares	$ —	$ 2,500,000
Asset retirement obligation in connection with a lease	$ (29,804)	$ (75,011)
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 34,546	$ 20,894

See Notes to Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Principal Business Activity:

The Company

MeiraGTx Holdings plc (the "Company" or "Meira Holdings"), a limited company under the laws of the Cayman Islands, is a clinical-stage biotech company developing novel gene therapy treatments for a wide range of inherited and acquired disorders for which there are no effective treatments available. The Company is focused on developing therapies for ocular diseases, including rare inherited blindness as well as Xerostomia following radiation treatment for head and neck cancers and neurodegenerative diseases such as amyothrophic lateral sclerosis ("ALS") and Parkinson's disease ("PD").

Reorganization and Initial Public Offering

We commenced operations as MeiraGTx Limited, a private limited company incorporated under the laws of England and Wales in 2015. On May 28, 2018, the Board of Directors of MeiraGTx Limited approved the Reorganization Transactions, effective June 7, 2018, pursuant to which the Board of Directors approved the transfer of the shares held by each of the MeiraGTx Limited's shareholders for the equivalent class and number of shares issued by Meira Holdings. On June 7, 2018, the Company completed its initial public offering ("IPO"), selling 5,000,000 ordinary shares ("Ordinary Shares") at a public offering price of $15.00 per share, and receiving $65.2 million in net proceeds, after deducting underwriting discounts and commissions and offering expenses payable by us.

Reverse Share Split

On June 7, 2018 MeiraGTx Limited's Board of Directors and shareholders approved a 1:3.881 reverse share split. All share information presented in these financial statements and accompanying footnotes have been retroactively adjusted to reflect the decreased number of shares resulting from this action.

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Liquidity

The Company has not yet achieved profitable operations. There is no assurance that profitable operations, if ever achieved, could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, and commercialization of the Company's product candidates will require significant additional financing. The Company's accumulated deficit at December 31, 2018 totaled $(148,289,717), and management expects to incur substantial losses in future periods. The success of the Company is subject to certain risks and uncertainties, including among others, uncertainty of product development; competition in the Company's field of use; uncertainty of capital availability; uncertainty in the Company's ability to enter into agreements with collaborative partners; dependence on third parties; and dependence on key personnel. The Company has not generated positive cash flows from operations, and there are no assurances that the Company will be successful in obtaining an adequate level of financing for the development and commercialization of its product candidates.

As of December 31, 2018, the Company had cash and cash equivalents in the amount of $68,080,175, which consisted of depository accounts. On January 30, 2019, the Company entered into a collaboration, option and license agreement with Janssen Pharmaceuticals, Inc., one of the Janssen Pharmaceuticals Companies of Johnson & Johnson (the "Collaboration Agreement"). Under the terms of the Collaboration Agreement, the Company will receive an upfront payment of $100 million. The Company will also receive research funding for certain research, manufacturing, clinical development and commercialization costs, potential additional milestone payments upon the achievement of such milestones and royalties on future net sales of products. On February 27, 2019, the Company issued 5,797,102 ordinary shares in an $80 million private placement led by JJDC, Inc., the investment arm of Johnson & Johnson, (the "Private Placement") for net proceeds of $77.4 million. The Company estimates that its cash and cash equivalents on hand at December 31, 2018 as well as proceeds from the Collaboration Agreement and the Private Placement will be sufficient to cover its expenses for at least the next twelve months from the date of issuance of these financial statements.

Risks and Uncertainties

The Company operates in an industry that is subject to intense competition, government regulation and rapid technological change. The Company's operations are subject to significant risk and uncertainties including financial, operational, technological, regulatory and other risks, including the potential risk of business failure.

The Company's limited capital resources and operations to date have been funded primarily with the proceeds from private equity offerings and the IPO. In the future, the Company may seek to raise additional capital through equity offerings, debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable it to complete the development and potential commercialization of its product candidates.

2. Summary of Significant Accounting Policies:

Consolidation

The accompanying consolidated financial statements include the accounts of Meira Holdings and its wholly owned subsidiaries:

MeiraGTx Limited, a limited company under the laws of England and Wales ("Meira Limited);
MeiraGTx, LLC, a Delaware corporation ("Meira LLC");
BRI-Alzan, Inc., a Delaware corporation ("BRI-Alzan");
MeiraGTx B.V., a Netherlands corporation ("Meira BV");
MeiraGTx Neurosciences, Inc. a Delaware corporation ("Meira Neuro");
MeiraGTx UK II Limited, ("Meira UK II"), a limited company under the laws of England and Wales; and
MeiraGTx UK Limited ("Meira UK"), a limited company under the laws of England and Wales.

All intercompany balances and transactions between the consolidated companies have been eliminated in consolidation.

Use of Estimates

Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range

of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements if these results differ from historical experience, or other assumptions do not turn out to be substantially accurate, even if such assumptions are reasonable when made. In preparing these consolidated financial statements, management used significant estimates in the following areas, among others: valuation of A ordinary shares ("A Ordinary Shares") issued prior to the Company's initial public offering, the accounting for research and development costs, warrants, share-based compensation and accrued expenses.

Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The Company evaluated all events and transactions through March 26, 2019, the date these consolidated financial statements were issued. See Note 18 for additional information.

Cash and Cash Equivalents

The Company considers all highly liquid instruments with an original maturity of 90 days or less at the time of purchase to be cash equivalents. Cash and cash equivalents consist of deposits that are readily convertible into cash.

Warrant Liability

During 2017, the Company issued warrants to purchase Convertible Preferred C Shares (the "Preferred Shares") to certain investors. Due to the potential redemption feature of the underlying Preferred Shares, the warrants had been classified as a liability. Liability accounting requires that the fair value of warrants be remeasured each reporting period with changes recorded in the statements of operations and comprehensive loss. These Preferred Shares warrants remained outstanding until their exercise in June 2018, at which time the warrant liability was remeasured to fair value and reclassified to additional paid-in capital.

Financial Instruments

The carrying value of prepaid expenses, other current assets, accounts payable, accrued expenses, notes payable and amounts due to an affiliate reported in the consolidated balance sheets equal or approximate fair value due to their short maturities.

Fair Value Measurements

Fair value is defined as the price that would be received upon sale of an asset or paid upon transfer of a liability in an orderly transaction between market participants at the measurement date and in the principal or most advantageous market for that asset or liability. The fair value should be calculated based on assumptions that market participants would use in pricing the asset or liability, not on assumptions specific to the entity. In addition, the fair value of liabilities should include consideration of non-performance risk including our own credit risk.

The Company follows ASC Topic 820, *Fair Value Measurements and Disclosures*, or ASC 820, for application to financial assets and liabilities. In addition to defining fair value, the standard expands the disclosure requirements around fair value and establishes a fair value hierarchy for valuation inputs. The hierarchy prioritizes the inputs into three levels based on the extent to which inputs used in measuring fair

value are observable in the market. Each fair value measurement is reported in one of the three levels which are determined by the lowest level input that is significant to the fair value measurement in its entirety. These levels are:

- Level 1: Observable inputs such as quoted prices in active markets for identical assets the reporting entity has the ability to access as of the measurement date;
- Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and
- Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

The table below represents the values of the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis:

		Fair Value Measurement Using:		
Description	December 31, 2018	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable (Level 3)
Restricted cash	$ 123,376	$ 123,376	$ —	$ —

		Fair Value Measurement Using:		
Description	December 31, 2017	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable (Level 3)
Restricted cash	$ 123,376	$ 123,376	$ —	$ —
Warrants	$2,679,633	$ —	$ —	$2,679,633

The table below represents a rollforward of the assets and liabilities that are required to be measured at fair value on a recurring basis from December 31, 2016 to December 31, 2018:

	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Balance as of December 31, 2016	$ 444,844	$ —	$ —
Cash released from restriction	(321,468)	—	—
Fair value of warrants issued	—	—	2,214,000
Change in fair value of warrants	—	—	465,633
Balance as of December 31, 2017	123,376	—	2,679,633
Change in fair value of warrants			1,514,775
Exercise of warrants	—	—	(4,194,408)
Balance as of December 31, 2018	$ 123,376	$ —	$ —

The warrants were classified as liabilities because the underlying Preferred Shares had a redemption feature in the event of a change of control of the Company. On June 5, 2018, the warrants were exercised at which time the warrant liability was determined to be $4,194,408, which represented the difference in the market value of the Preferred Shares and the exercise price of the warrants. This resulted in an increase of the warrant liability in the amount of $1,514,775 for the year ended December 31, 2018. The related warrant liability of $4,194,408 was reclassified as Capital in Excess of Nominal Value at such time.

The fair values of the warrants were estimated using the Black-Scholes valuation model with the following assumptions:

	June 4, 2018	December 31, 2017	September 21, 2017
Risk-free interest rate	1.77%	1.72%	1.38%
Expected volatility	80%	80%	80%
Expected dividend yield	0	0	0
Expected life	1 day	9 months	18 months

For the unobservable inputs for the warrants, the expected volatility was determined at each measurement date by taking an average of the volatility of other publicly-traded peer biotechnology companies. The expected life was determined at each measurement date based upon the Company's estimate of the time until the Company has a conversion event, as described in Note 12.

The fair value of the Preferred Shares were based upon recent issuances of the Company's Preferred Shares on or about those dates.

The estimated fair values of the Company's warrants were not necessarily indicative of the amounts that would have been realized in a current market exchange. The determination of the fair value of the warrants were sensitive to changes in the assumptions used and a change in those inputs could result in a significantly higher or lower fair value measurement. If the volatility were to increase or the expected life were to increase, the fair value of the warrant would increase. Conversely, if the volatility were to decrease or the expected life were to decrease, the fair value of the warrant would decrease.

Convertible Preferred C Shares

The Preferred Shares were not redeemable, except in the event of a change of control which was outside the control of the Company and required shareholder approval. The redemption value of the Preferred Shares upon a change in control is equal to its liquidation value described below.

The Company is accounting for its Preferred Shares under the requirements of ASC 480, *Distinguishing Liabilities from Equity*, which establishes standards for how an issuer classifies and measures certain financial instruments with characteristics of both liabilities and equity. The carrying value of the Preferred Shares is presented as temporary equity and is increased by periodic accretions so that the carrying amount will equal the redemption amount at the estimated date that the Preferred Shares will be converted into Ordinary Shares. These increases are affected through charges against additional paid-in capital, to the extent it is available, or accumulated deficit. For all Preferred Shares issuance, the difference between the amount invested by the holders of the Preferred Shares, net of issuance costs and the initial fair value of warrants issued in connection with the Preferred Shares (if applicable), and the liquidation value of the Preferred Shares is recorded as accretion over the estimated life of the Preferred Shares. The accretion is added to net loss to arrive at the net loss available to Ordinary Shareholders in the calculation of loss per Ordinary Share.

Concentrations of Credit Risk

The Company maintains its cash and cash equivalents primarily in depository and money market accounts within two large financial institutions in the United States and one large financial institution in the United Kingdom. Cash balances deposited at these major financial banking institutions exceed the insured limit. The Company has not experienced any losses on its bank deposits and believes these deposits do not expose the Company to any significant credit risk.

Property, Plant and Equipment, Net

Property, plant and equipment are stated at cost, net of accumulated depreciation. Depreciation is calculated using the straight-line method over the estimated useful lives of the respective assets. Leasehold improvements are depreciated over the lesser of their useful lives or the life of the lease (see Note 5).

The estimated useful lives of the asset categories are set forth in the table below:

Asset Category	Useful Lives
Computer and office equipment	3 years
Laboratory equipment	5 years
Manufacturing equipment	7 years
Furniture and fixtures	5 years
Capitalized leasehold interest	25 years
Leasehold improvements	lesser of useful life or remaining term of lease

Expenditures for leasehold improvements are capitalized, and expenditures for maintenance and repairs are expensed to operations as incurred.

ASC Topic 360, *Property, Plant and Equipment*, addresses the financial accounting and reporting for impairment or disposal of long-lived assets. The Company reviews the recorded values of long-lived assets for impairment whenever events or changes in business circumstances indicate that the carrying amount of an asset or group of assets may not be fully recoverable. The Company recorded no impairment charges in 2018 or 2017.

Net Loss per Ordinary Share

Basic net loss per Ordinary Share is computed by dividing net loss by the weighted average number of shares of the Company's Ordinary Shares assumed to be outstanding during the period of computation. Diluted net loss per ordinary share is computed similar to basic net loss per share except that the denominator is increased to include the number of additional Ordinary Shares that would have been outstanding if the potential ordinary shares had been issued at the beginning of the year and if the additional Ordinary Shares were dilutive (treasury stock method) or the two-class method, whichever is more dilutive. For all periods presented, basic and diluted net loss per Ordinary Share are the same, as any additional Ordinary Share equivalents would be anti-dilutive (see Note 13).

Income Taxes

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes*, or ASC 740, which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Realization of net deferred tax assets is dependent on future taxable income. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some, or all, of the deferred tax assets will not be realized. Realization of net deferred tax assets is dependent on future taxable income (see Note 14).

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the

benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2018 and 2017, the Company does not have any significant uncertain tax positions.

The Company is required to estimate income taxes in each of the jurisdictions in which it operates.

The Company's reserves related to taxes are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized. As of December 31, 2018, the Company had no unrecognized tax benefits or related interest and penalties accrued.

In the United States, on December 22, 2017, the "Tax Cuts and Jobs Act" (the "Act") was signed into law. Substantially all of the provisions of the Act are effective for taxable years beginning after December 31, 2017. The Act includes significant changes to the Internal Revenue Code of 1986 (as amended, the "Code"), including amendments which significantly change the taxation of individuals, and business entities. The Act contains numerous provisions impacting the Company, the most significant of which reduces the Federal corporate statutory tax rate from 34% to 21%.

The staff of the U.S. Securities and Exchange Commission ("SEC") has recognized the complexity of reflecting the impacts of the Act, and on December 22, 2017 issued guidance in Staff Accounting Bulletin 118 ("SAB 118"), which clarifies accounting for income taxes under ASC 740 if information is not yet available or complete and provides for up to a one year period in which to complete the required analyses and accounting (the measurement period). SAB 118 describes three scenarios (or "buckets") associated with a company's status of accounting for income tax reform: (1) a company is complete with its accounting for certain effects of tax reform, (2) a company is able to determine a reasonable estimate for certain effects of tax reform and records that estimate as a provisional amount, or (3) a company is not able to determine a reasonable estimate and therefore continues to apply ASC 740, based on the provisions of the tax laws that were in effect immediately prior to the Act being enacted.

The various provisions under the Act deemed most relevant to the Company have been considered in preparation of its financial statements as of December 31, 2017. The Company had made a reasonable estimate for certain effects of tax reform and had recorded provisional amounts as part of its income tax provision. To the extent that clarifications or interpretations materialized in the future that would impact upon the effects of the Act incorporated into the December 31, 2017 financial statements, those effects would have been reflected in the future as or if they materialize. As of December 31, 2018, the Company has completed its accounting for all of the tax effects of the Act. Based on the additional analysis performed, no adjustments to the provisional amounts were made in the reporting period which had an impact to the tax provision or consolidated financial statements.

Research and Development

Research and development costs are charged to expense as incurred. These costs include, but are not limited to, employee-related expenses, including salaries, benefits and travel of the Company's research and development personnel; expenses incurred under agreements with contract research organizations and investigative sites that conduct clinical and preclinical studies and manufacture the drug product for the clinical studies and preclinical activities; facilities; supplies; rent, insurance, certain legal fees, share-based compensation, depreciation, other costs associated with clinical and preclinical activities and regulatory operations and acquisition of in-process research and development write-offs. Refundable research and development credits / tax credits received are recorded as an offset to these costs.

Costs for certain development activities, such as Company funded outside research programs, are recognized based on an evaluation of the progress to completion of specific tasks with respect to their actual

costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development expense, as the case may be.

Share-Based Compensation Expense

Options

The Company grants share options to employees, non-employee members of the Company's board of directors and non-employee consultants as compensation for services performed. Employee and non-employee members of the board of directors' awards of share-based compensation are accounted for in accordance with ASC 718, *Compensation - Stock Compensation*, or ASC 718. ASC 718 requires all share-based payments to employees and non-employee directors, including grants of share options, to be recognized in the Statement of Operations and Comprehensive Loss based on their grant date fair values. The grant date fair value of share options is estimated using the Black-Scholes option valuation model.

Using this model, fair value is calculated based on assumptions with respect to (i) the fair value of the Company's Ordinary Shares on the grant date; (ii) expected volatility of the Company's Ordinary Share price, (iii) the periods of time over which employees and members of the Company's board of directors are expected to hold their options prior to exercise (expected term), (iv) expected dividend yield on the Company's Ordinary Shares, and (v) risk-free interest rates.

As there had been no public market for the Company's Ordinary Shares until the Company's IPO on June 7, 2018, the estimated fair value of the Ordinary Shares until that time had been determined by the Company's board of directors as of the date of each option grant, with input from management, considering the most recently available third-party valuations of Ordinary Shares and the board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. The fair value of Ordinary Shares after the Company's IPO was determined based upon the closing share price on the date of grant.

The third party estimated the fair value of the equity using a special case of the market approach known as the backsolve method. The backsolve method was used to solve for the implied total equity value based on the Company's recent Series C financing round. Consideration was given to the rights and preferences of each of the Company's classes of equity and the expected time to a liquidity event. An option pricing allocation method, or OPM, was selected to allocate the total equity value. The OPM treats ordinary shares and preferred shares as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the Ordinary Shares have value only if the funds available for distribution to shareholders exceeded the value of the Preferred Shares' liquidation preference at the time of the liquidity event, such as a strategic sale or a merger. These third-party valuations resulted in a valuation of the Company's Ordinary Shares of $7.57, $2.64 and $5.63 per share as of December 31, 2016, September 15, 2017 and December 31, 2017, respectively.

The assumptions underlying these valuations represented management's best estimate, which involved inherent uncertainties and the application of management's judgment. As a result, if the Company had used different assumptions or estimates, the fair value of its Ordinary Shares and its share-based compensation expense could have been materially different.

Since the Company's Ordinary Shares had not been traded on a public exchange prior to the Company's IPO and have only been traded on a public exchange for a short period of time since the Company's IPO, the Company believes that it does not have sufficient company-specific information available to determine the expected term based on its historical data. As a result, the expected term of share options granted to

employees and members of the Company's board of directors is determined using the average of the vesting period and contractual life of the option, an accepted method for the Company's option grants under the SEC's Staff Accounting Bulletin No. 107 and No. 110, *Share-Based Payment.*

Similarly, the Company believes that its future volatility will differ materially during the expected term from the volatility that would be calculated from its historical share prices to date. Consequently, expected volatility is based on an analysis of guideline companies in accordance with ASC 718. The expected dividend yield is zero as the Company has never paid dividends and does not currently anticipate paying any in the foreseeable future. Risk-free interest rates are based on quoted U.S. Treasury rates for securities with maturities approximating the option's expected term.

The Company accounted for options granted to non-employee consultants under ASC 505-50, *Equity-Based Payments to Non-Employees*, or ASC 505-50. As such, the Company estimates the fair value of each such option using the Black-Scholes model, with the expected term of share options granted to non-employees initially equal to the options' maximum contractual life of ten years, at issuance. On each subsequent reporting date until performance is complete, the Company revalued all outstanding options granted to non-employee consultants during the vesting period of each tranche. Under ASC 505-50, upon re-measurement of each award, income or expense is recognized during its vesting term. Compensation cost relating to awards with service-based graded vesting schedules is recognized as general and administrative and research and development expenses in the consolidated statements of operations and comprehensive loss using the straight-line method.

On July 1, 2018, the Company early adopted ASU 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU No. 2018-07") which simplifies the accounting for share-based payments granted to nonemployees for goods and services. The ASU supersedes ASC 505-50 and expands the scope of ASC 718 to include *all* share-based payment arrangements related to the acquisition of goods and services from both nonemployees and employees. The adoption of ASU No. 2018-17 did not have a material effect on the consolidated financial statements.

Restricted Shares

In connection with certain service agreements and research agreements, the Company has granted restricted Ordinary Shares as compensation. The Ordinary Shares are recognized in the consolidated statements of operations and comprehensive loss based on their grant date fair values. Compensation cost relating to share grants with service-based graded vesting schedules is recognized based on the vesting schedule.

Leases

The Company recognizes rent expense for operating leases on a straight-line basis over the term of the lease, beginning on the date the Company takes possession of the property. Rent expense includes the base amounts stated in the lease agreement as well as the effect of reduced or free rent and rent escalations. At lease inception, the Company determines the lease term by assuming the exercise of those renewal options that are reasonably assured because of the significant economic penalty that exists for not exercising those options. The expected lease term is one of the factors used to determine whether a lease is classified as an operating or capital lease and is used to calculate the straight-line rent expense. The difference between the cash paid to the landlord and the amount recognized as rent expense on a straight-line basis is included in deferred rent and classified within long-term liabilities. Lease incentives made by landlords to or on behalf of the Company for leasehold improvements are recorded as deferred rent and classified as long-term liabilities.

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
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Asset Retirement Obligation

Accounting for Asset Retirement Obligations requires legal obligations associated with the retirement of long-lived assets to be recognized at fair value when incurred and capitalized as part of the related long-lived asset. In the absence of quoted market prices, we estimate the fair value of our asset retirement obligations using Level 3 present value techniques, in which estimates of future cash flows associated with retirement activities are discounted using a credit-adjusted risk-free rate. Asset retirement obligations currently reported on our Consolidated Balance Sheets were measured during a period of historically low interest rates. The impact on measurements of new asset retirement obligations using different rates in the future may be significant.

The Company uses estimates to determine the amount of the asset retirement obligations at the end of the lease term and discounts such asset retirement obligations using an estimated discount rate. Interest on the discounted asset retirement obligation is amortized over the term of the lease using the effective interest method and is recorded as interest expense in the consolidated statements of operations and comprehensive loss.

The change in asset retirement obligations is as folows:

	For the year ended December 31,	
	2018	2017
Balance at beginning of year	$178,419	$221,254
Inception of asset retirement obligation	69,286	—
Amortization of interest	(38,301)	19,313
Change in estimate	(99,090)	(75,011)
Effects of exchange rate	17,805	12,863
Balance at end of year	$128,119	$178,419

Other Comprehensive Income (Loss)

Other comprehensive income (loss) is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. The only component of other comprehensive income (loss) impacting the Company is foreign currency translation.

Foreign Currencies

The Company’s consolidated financial statements are presented in U.S. dollars, the reporting currency of the Company. The financial position and results of operations of Meira UK II and Meira B.V. are measured using the foreign subsidiaries’ local currency as the functional currency. Meira UK II’s cash accounts holding U.S. dollars are remeasured based upon the exchange rate at the date of remeasurement with the resulting gain or loss included in the consolidated statements of operations and comprehensive loss. Expenses of such subsidiaries have been translated into U.S. dollars at average exchange rates prevailing during the period. Assets and liabilities have been translated at the rates of exchange on the consolidated balance sheets dates. The resulting translation gain and loss adjustments are recorded directly as a separate component of shareholders’ equity and as other comprehensive loss on the consolidated statements of operations and comprehensive loss.

Segment Information

Management has concluded it has a single reporting segment for purposes of reporting financial condition and results of operations.

The following table summarizes non-current assets by geographical area:

	December 31, 2018	December 31, 2017
United States	$ 454,568	$ 436,463
United Kingdom	21,788,130	13,942,642
	$ 22,242,698	$ 14,379,105

Accounting Pronouncements Recently Adopted

In June 2018, the FASB issued ASU 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, which simplifies the accounting for share-based payments granted to nonemployees for goods and services. The ASU supersedes ASC 505-50 and expands the scope of ASC 718 to include *all* share-based payment arrangements related to the acquisition of goods and services from both nonemployees and employees. As a result, most of the guidance in ASC 718 associated with employee share-based payments, including most of its requirements related to classification and measurement, applies to nonemployee share-based payment arrangements. ASU 2018-07 generally requires an entity to use a modified retrospective transition approach, with a cumulative-effect adjustment to retained earnings as of the beginning of the period of adoption, for all (1) liability-classified nonemployee awards that have not been settled as of the adoption date and (2) equity-classified nonemployee awards for which a measurement date has not been established. The guidance is applicable to public business entities for fiscal years beginning after December 15, 2019 and interim periods within those years. Early adoption is permitted, and the Company adopted ASU No. 2018-07 on July 1, 2018, which did not have a material effect on the consolidated financial statements.

In January 2017, the FASB issued ASU No. 2017-01, *Business Combinations (Topic 805), Clarifying the Definition of a Business*, or ASU 2017-01, that clarifies the definition of a business to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. ASU 2017-01 requires an entity to evaluate if substantially all of the fair value of the gross assets acquired or disposed of is concentrated in a single identifiable asset or a group of similar identifiable assets; if so, the set of transferred assets and activities is not a business. ASU 2017-01 also requires a business to include at least an input and one substantive process that together significantly contribute to the ability to create output and removes the evaluation of whether a market participant could replace missing elements. ASU 2017-01 should be applied prospectively and is effective for annual periods beginning after December 15, 2017 and interim periods within those annual periods. The adoption of ASU 2017-01 on January 1, 2018 did not have a material effect on the Company's financial position, results of operations or cash flows.

In December 2016, the FASB issued ASU No. 2016-20, *Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers*, or ASU 2016-20, that allows entities not to disclose variable consideration allocated to performance obligations related to either: (1) sales-or usage-based royalties on licenses of intellectual property or (2) variable consideration allocated entirely to a wholly unsatisfied performance obligation or to a wholly unsatisfied promise to transfer a distinct good or service that forms part of a single performance obligation when certain criteria are met. ASU 2016-20 also requires entities that use any of the new or previously existing optional exemptions to expand their qualitative disclosures. It also makes 12 additional technical corrections and improvements to the new revenue standard, ASU 2014-09. The amendments have the same effective date and transition requirements as ASU 2014-09. The adoption of ASU 2016-20 did not have a material effect on its financial position, results of operations or cash flows.

In November 2016, the Financial Accounting Standards Board, or FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230), Restricted Cash* (a consensus of the Emerging Issues Task Force), or ASU 2016-18, which changes the presentation of the cash flow statement to include amounts generally described as restricted cash or restricted cash equivalents, together with cash and cash equivalents, when reconciling the beginning-of-period and end-of-period amounts shown on the statement of cash flows. ASU 2016-18 also requires additional disclosures concerning the nature of the restrictions on cash and cash equivalents and a reconciliation between amounts of cash, cash equivalents and restricted cash on the balance sheet and statement of cash flows for each period presented. ASU 2016-18 will be applied retrospectively to all periods presented and is effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted, including adoption in an interim period. Prior to adoption, the Company presented changes in restricted cash as an operating activity in the statement of cash flows. Upon adoption of ASU 2016-18 on January 1, 2018, such changes are now reflected in the beginning and ending balances of cash, cash equivalents and restricted cash for all periods presented.

In May 2016, the FASB issued ASU No. 2016-12, *Revenue from Contracts with Customers (Topic 606), Narrow-Scope Improvements and Practical Expedients*, or ASU 2016-12, which amends guidance in the new revenue standard, ASU No. 2014-09 *Revenue from Contracts with Customers (Topic 606)*, or ASU 2014-09, on collectability, noncash consideration, presentation of sales tax and transition. The amendments in ASU 2016-12 are effective for annual reporting periods beginning after December 15, 2017 (i.e., January 1, 2018), including interim periods within those reporting periods, which is the same as for ASU 2014-09, as amended by ASU No. 2015-14, *Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date*, or ASU 2015-14. In April 2016, the FASB issued ASU No. 2016-10, *Revenue from Contracts with Customers (Topic 606), Identifying Performance Obligations and Licensing*, or ASU 2016-10, which clarifies the principle for determining whether a good or service is "separately identifiable" from other promises in the contract and, therefore, should be accounted for as a separate performance obligation. In that regard, ASU 2016-10 requires that an entity determine whether its promise is to transfer individual goods or services to the customer, or a combined item (or items) to which the individual goods and services are inputs. In addition, ASU 2016-10 categorizes intellectual property, or IP, into two categories: "functional" and "symbolic." Functional IP has significant standalone functionality. All other IP is considered symbolic IP. Revenue from licenses of functional IP is generally recognized at a point in time, while revenue from licenses of symbolic IP is recognized over time. ASU 2016-10 has the same effective date and transition requirements as ASU 2014-09, as amended by ASU 2015-14. In March 2016, the FASB issued ASU No. 2016-08, *Revenue from Contracts with Customers (Topic 606), Principal versus Agent Considerations (Reporting Revenue Gross versus Net)*, or ASU 2016-08, which clarifies the implementation guidance on principal versus agent considerations contained in ASU 2014-09 by specifying that the determination as to whether an entity that is involved in providing a good or a service to a customer is a principal or an agent is based upon whether the entity controls the good or the service before it is transferred to the customer. ASU 2016-08 has the same effective date and transition requirements as ASU 2014-09, as amended by ASU 2015-14. The adoption of these ASU's on January 1, 2018 did not have a material effect on the Company's financial position, results of operations or cash flows.

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities.* The new guidance is intended to improve the recognition and measurement of financial instruments by requiring separate presentation of financial assets and financial liabilities by measurement category and form of financial asset (i.e., securities or loans and receivables) within the balance sheet or the accompanying notes to the financial statements, eliminating the requirement for public business entities to disclose the method(s) and significant assumptions used to estimate the fair value that is required to be disclosed for financial instruments measured at amortized cost within the balance sheet, requiring public business entities to use the exit price

notion when measuring the fair value of financial instruments for disclosure purposes, requiring equity investments (except those accounted for under the equity method of accounting, or those that result in consolidation of the investee) to be measured at fair value with changes in fair value recognized in net income, and requiring a reporting organization to present separately in other comprehensive income the portion of the total change in the fair value of a liability resulting from a change in the instrument-specific credit risk (also referred to as "own credit") when the organization has elected to measure the liability at fair value in accordance with the fair value option for financial instruments, among others. In February 2018, the FASB issued ASU No. 2018-03, *Technical Corrections and Improvements to Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities*, which is intended to clarify certain aspects of the guidance issued in ASU 2016-01. The Company adopted these standards effective January 1, 2018, which had no impact on the Company's consolidated financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In November 2018, the FASB issued Accounting Standards Update No. 2018-18, Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606 ("ASU 2018-18"). The standard amends Accounting Standards Codification 808, Collaborative Agreements and Accounting Standards Codification 606, Revenue from Contracts with Customers, to clarify the interaction between collaborative arrangement participants that should be accounted for as revenue under ASC 606. In transactions when the collaborative arrangement participant is a customer in the context of a unit of account, revenue should be accounted for using the guidance in Topic 606. The amendments in Update No. 2018-18 are effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years. The Company is currently evaluating the new guidance included in ASU 2018-18, but does not expect it to have a material impact on its consolidated financial statements.

In October 2016, the FASB issued ASU 2016-16, Income Taxes (Topic 740): *Intra-Entity Transfers of Assets Other than Inventory*, or ASU 2016-16 which requires that an entity recognize the income tax consequences of an intra-entity transfer of assets other than inventory when the transfer occurs. The guidance must be applied using the modified retrospective basis. The Company does not expect the provisions of ASU 2016-16 to have a material impact on its current financial statements. This update will be effective for the Company at the beginning of fiscal 2019.

In February 2016, the FASB issued ASU No. 2016-02, "Leases" (ASC 842). The amended guidance requires lessees to recognize lease liabilities and right-of-use assets on the balance sheet for all leases with terms longer than 12 months and provides enhanced disclosures on key information of leasing arrangements. In July 2018, further amendments were issued to clarify how to apply certain aspects of the amended lease guidance and to address certain implementation issues. The amended guidance is effective for us commencing in the first quarter of 2019. Early adoption is permitted. We plan to adopt the amended guidance on the effective date and expect to elect the package of practical expedients. We expect the adoption of the amended guidance will materially affect our consolidated balance sheet and that the primary impact will be recognition of minimum commitments at present value of our noncancelable operating leases as lease liabilities and corresponding right-of-use assets. In July 2018, the FASB issued ASU No. 2018-10, which provides narrow amendments to ASU No. 2016-02 to clarify how to apply the rate implicit in the lease, impairment of the net investment in the lease, lessee reassessment of lease classification, variable payments that depend on an index or rate and certain transition adjustments. In July 2018, the FASB also issued ASU No. 2018-11, which provides targeted improvements to ASU No. 2016-02 to provide entities the transition option to not apply the standard in the comparative periods presented in the year of adoption. The Company will adopt the new standard effective January 1, 2019 using the modified retrospective transition method using the practical expedients model and a discount rate in the range of 8% to 10%, and

elect to not apply the standard in the comparative periods presented in the year of adoption. We estimate that upon implementation, we will record a right-of-use asset between $3.3 million and $3.6 million and a corresponding liability between $3.6 million and $3.8 million. We are continuing to evaluate the impact that the amended lease guidance will have on our consolidated financial statements, systems, processes and internal controls.

3. Asset Acquisition:

On October 5, 2018, the Company entered into an agreement to acquire Vector Neurosciences Inc. ("Vector") pursuant to an Agreement and Plan of Merger (the "Merger Agreement") by and among the Company, Vector, VN Acquisition, Inc., a wholly-owned subsidiary of the Company ("Merger Sub 1"), VN Acquisition 2, Inc., a wholly-owned subsidiary of the Company ("Merger Sub 2"), the Vector stockholders named therein and the Vector stockholder representative, pursuant to which Merger Sub 1 was merged with and into Vector, with Vector being the surviving corporation ("Merger 1") and, immediately following Merger 1, Vector was merged with and into Merger Sub 2, with Merger Sub 2 being the surviving corporation (together with Merger 1, the "Merger"). As a result of the Merger, Vector is a wholly-owned subsidiary of the Company. The Company's board of directors, Vector's board of directors and Vector's stockholders have, in each case, unanimously approved the Merger, the Merger Agreement and the transactions contemplated by the Merger Agreement. The merger consideration to Vector's stockholders consists of 225,000 shares of the Company's Ordinary Shares as initial merger consideration, consisting of 202,500 shares which were issued at the closing of the Merger and an additional 22,500 shares to be issued 18 months following the closing, subject to any indemnification claims under the Merger Agreement (See Note 12).

In addition, pursuant to the terms of the Merger Agreement, the Company will issue to Vector's stockholders additional Ordinary Shares equal to a maximum value of $21,000,000 if specified regulatory milestones are met and will make royalty payments to Vector's stockholders in an amount equal to a percentage of the value of sales of certain products developed based on the Vector assets, which royalty payments are also payable in Ordinary Shares. The number of Ordinary Shares to be issued in connection with such milestones and royalties will be based on the three-day average closing price of the Company's Ordinary Shares immediately prior to the date of determination of the value of the payment.

The Company determined this transaction represented an asset acquisition as substantially all of the value was in the intellectual property as defined by ASC 805, *Business Combinations* ("ASC 805"). The asset acquisition of in process research and development was recorded at a fair value of $2,990,250 as of October 5, 2018. The acquired in process research and development was immediately charged to research and development expense in the consolidated statement of operations and comprehensive loss as of the acquisition date since the Company determined that there was no additional alternative use of these assets. Additionally, under ASC 805, the Company determined that as of the acquisition date and as of December 31, 2018, the contingent milestone payments in the aggregate amount of $21,000,000, and royalty payments have not been resolved and therefore have not been recorded as liability.

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4. Prepaid Expenses:

Prepaid expenses at December 31, 2018 and 2017 consist of the following:

	December 31, 2018	December 31, 2017
Insurance	$ 623,314	$ 163,284
Clinical Trial Costs	373,723	839,644
Research and Development	352,658	624,348
Other	330,233	160,595
Dues and License Fees	169,073	145,594
Rent	88,784	27,778
	$ 1,937,785	$ 1,961,243

5. Property, Plant and Equipment, net:

Property, plant and equipment, net at December 31, 2018 and 2017 consist of the following:

	December 31, 2018	December 31, 2017
Leasehold Improvements	$11,538,377	$10,873,895
Capitalized Leasehold Interest	7,150,611	—
Manufacturing Equipment	3,779,950	2,477,637
Laboratory Equipment	1,485,544	993,409
Computer and Office Equipment	334,525	276,100
Asset Retirement Obligation	113,678	153,133
Furniture & Fixtures	88,660	93,786
	24,491,345	14,867,960
Less: Accumulated depreciation	(2,477,108)	(612,231)
	$22,014,237	$14,255,729

In February 2016, the Company sublet a manufacturing facility for a term of 5 years, that included an additional 5-year option. This sub-lease was accounted for as an operating lease. On December 14, 2018, the Company executed a new sub-lease for this manufacturing facility whereby the sub landlords remaining term of 108 years was assigned to the Company. Under the new sub-lease, the Company paid a one-time fee of £5,250,000 (approximately $6,615,000 assuming a rate of $1.26 per GBP on the date of the acquisition) for the assignment and will no longer pay any base rent for the remaining 108 years. The one-time fee and related transaction costs, in the aggregate amount of £5,613,165 (approximately $7,150,611 using a rate of $1.2739 per GBP at December 31, 2018), have been accounted for as a capital lease and recorded as property, plant and equipment. The Company determined that the cost of the new sub-lease would be amortized on a straight-line basis over a 25-year estimated useful life.

In connection with the above-mentioned lease, the Company estimated that it had an asset retirement obligation at the end of the initial five-year lease term in the amount of $306,400. The Company discounted the asset retirement obligation using an 8% discount rate and recorded an asset retirement obligation in the amount of $205,659 as of December 31, 2016, which is included in leasehold improvements and was being depreciated over the five-year term of the lease (see Note 15). As of December 31, 2017, the Company determined that it was probable that it would exercise the additional five-year option provided for in the sub-lease. Therefore, the company remeasured the asset retirement obligation using the remaining eight-year new sub-lease term and recorded a reduction in the asset retirement obligation of $75,011 recorded in

leasehold improvements. On December 14, 2018, upon execution of the new sub-lease, the Company remeasured the asset retirement obligation using the remaining 25-year estimated useful life and recorded a reduction in the asset retirement obligation of $99,090 recorded in leasehold improvements.

In connection with two operating leases entered into in July 2018, the Company estimated that it had asset retirement obligations at the end of the eight-year terms in the amount of $140,129. The Company discount the asset retirement obligation using an 8% discount rate and recorded an asset retirement obligation in the aggregate amount of $69,286, which is included in leasehold improvements and is being depreciated over the eight-year term of the lease.

Capitalized leases in the amount of $95,880 are included in computer and office equipment at December 31, 2018 and 2017, and accumulated depreciation of $62,912 and 34,552 at December 31, 2018 and 2017, respectively.

A capitalized lease in the amount of $7,150,611 is included in capitalized leasehold interest at December 31, 2018 and accumulated depreciation of $6,928 at December 31, 2018.

Depreciation expense was $2,052,948 and $679,177 for the years ended December 31, 2018 and 2017 respectively.

6. Restricted Cash:

The Company is required to maintain a stand-by letter of credit as a security deposit under the ARE-East River Science Park LLC (“ARE”) lease (see Note 15) through the end of the lease term in December 2021, plus three months. The fair value of the letter of credit approximates its contract value. The Company’s bank requires the Company to maintain a restricted cash balance to serve as collateral for the letter of credit issued to the landlord by the bank. As of December 31, 2018 and 2017, the restricted cash balance for the ARE lease was invested in a commercial money market account.

The Company had $123,376 of restricted cash included in long-term assets as of December 31, 2018 and 2017 and is measured using level 1 inputs.

7. Accrued Expenses:

Accrued expenses at December 31, 2018 and 2017 were comprised of the following:

	December 31, 2018	December 31, 2017
Compensation and Benefits	$ 5,731,438	$ 2,386,903
Clinical Trial Costs	4,013,094	4,859,410
Professional Fees	914,540	231,923
Consulting	821,009	1,220,477
Research and Development	236,271	73,379
Rent	122,770	387,267
Interest	40,800	33,437
Other	111,775	140,148
	$ 11,991,697	$ 9,332,944

8. Capitalized Leases:

In 2015, the Company acquired certain office equipment in the amount of $17,817 under a 3-year lease arrangement. The Company determined that the lease should be capitalized since it contained a bargain

purchase option for the equipment at the end of the lease term. Total payments under the capital lease amounted to $20,502 and had an interest rate of 9.35%.

In 2017, the Company acquired additional office equipment in the amount of $78,063 under a 3-year lease arrangement. The Company determined that the lease should be capitalized since it contained a bargain purchase option for the equipment at the end of the lease term. Total payments under the capital lease will amount to $86,145 and has an interest rate of 6.90%.

In December 2018, the Company entered into a sub-lease for the remaining term of 108 years (see Note 5). The Company determined that the lease should be capitalized since the lease term exceeded 75% of the estimated 25-year useful life of the leasehold interest. The Company made a one-time upfront payment for the assignment of the lease and will not have any obligation to make future rent payments. The Company capitalized as property, plant and equipment, the initial one-time upfront payment and related transaction costs in the aggregate amount of $7,150,611.

The following is a schedule, by year, of future minimum lease payments under the capital leases together with the present value of the net minimum lease payments as of December 31, 2018:

2019	28,715
2020	7,179
Total minimum lease payments	35,894
Less: amount representing interest	(1,598)
Present value of net minimum lease payments	34,296
Less: current portion	(27,199)
Obligations under capital lease, less current portion	$ 7,097

9. Notes Payable

On October 26, 2017, in connection with an amendment and termination of a lease, the Company issued a promissory note in the amount of $1,442,009 to ARE, the landlord and also a related party (see Note 15). The note bears interest at the rate of 5% per annum and was due on December 31, 2018. However, if the Company had sufficient liquidity, as defined in the note, then the note, including accrued interest, would become due and payable at that time. In accordance with the sufficient liquidity provision, the Company repaid the note, plus accrued interest, in the amount of $1,472,433 during the three-month period ended March 31, 2018. The Company recorded interest expense in the consolidated statements of operations and comprehensive loss in connection with the note in the amount of $17,386 and $13,037 for the years ended December 31, 2018 and 2017, respectively.

10. Collaboration Agreement

On October 16, 2018, the Company entered into a research collaboration agreement with Janssen Pharmaceuticals, Inc., (“Janssen”), to develop regulatable gene therapy treatment using the Company’s proprietary riboswitch technology. As part of the agreement, the Company will use its proprietary riboswitch technology to engineer regulatable gene therapy constructs encoding proprietary gene sequences from Janssen.

Upon execution of the agreement, Janssen paid the stage 1 fee in the amount of $658,667 and such payment was recorded as deferred research funding. The stage 1 fee is being amortized over the estimated research term of eight months. During the year ended December 31, 2018, the Company amortized $224,576 of the deferred research funding, which was recorded as an offset to research and development expenses. Deferred research funding in the amount of $434,091 is included as other current liabilities on the consolidated balance sheet at December 31, 2018.

11. Share-Based Compensation

2018 and 2016 Equity Incentive Plans

The Company's 2018 Incentive Award Plan and 2016 Equity Incentive Plan (the "Plans"), were adopted by the Company's board of directors and shareholders. Under the Plans, the Company has granted share options to selected officers, employees and non-employee consultants. The Company's board of directors administer the Plans. Options granted under the Plans have a maximum contractual term of ten years. Options granted generally vest 25% on the first anniversary date of grant and the balance ratably over the next 36 months. Options granted to directors generally vest on the first anniversary date of grant. Upon the adoption of the 2018 Incentive Award Plan, the Company ceased issuing awards under the 2016 Equity Incentive Plan.

A summary of the Company's share option activity related to employees, non-employee members of the board of directors and non-employee consultants as of and for the years ended December 31, 2018 and 2017 is as follows:

STOCK OPTION TABLE

	Number of Options	Weighted-Average Exercise Price	Aggregate Instrinsic Value
Outstanding at December 31, 2016	333,660	$ 7.72	
Granted	611,933	3.73	
Exercised	(1,288)	(7.57)	
Expired	—	—	
Forfeited	(5,668)	(7.72)	
Outstanding at December 31, 2017	938,637	$ 5.12	
Granted	2,334,285	8.63	
Exercised	—	—	
Expired	—	—	
Forfeited	(10,557)	(5.51)	
Outstanding at December 31, 2018	3,262,365	$ 7.64	$6,903,313
Weighted average remaining contractual life of options outstanding as of December 31, 2017 (yrs)	9.09		
Weighted average remaining contractual life of options outstanding as of December 31, 2018 (yrs)	9.24		
Options exercisable at December 31, 2017	186,394	$ 7.72	
Options exercisable at December 31, 2018	535,241	$ 5.79	
Weighted average remaining contractual life of options exercisable as of December 31, 2017 (yrs)	8.21		
Weighted average remaining contractual life of options exercisable as of December 31, 2018 (yrs)	7.88		

The total fair value of options vested during the years ended December 31, 2018 and 2017 was $1,387,607 and $898,699, respectively.

During the years ended December 31, 2018 and 2017, the Company granted 2,334,285 and 611,933 share options, respectively. The grant date fair values of the stock options granted were estimated using the Black-Scholes option valuation model with the following ranges of assumptions (see Note 2):

	2018	2017
Risk-free interest rate	2.32% - 2.98%	2.28% - 2.51%
Expected volatility	90%	80%
Expected dividend yield	0%	0%
Expected life (in years)	5.5 - 10.0	5.5 - 10.0

As of December 31, 2018 and 2017, the total compensation expense relating to unvested options granted that had not yet been recognized was $17,415,098 and $987,413, respectively which is expected to be realized over a period of 4.0 and 3.42 years, respectively. The Company will issue shares upon exercise of options from Ordinary Shares reserved.

The weighted average grant date fair value of options granted during the years ended December 31, 2018 and 2017 was $6.53 and $3.10, respectively.

Restricted Ordinary Shares

In 2015, in connection with certain service and consulting agreements, certain employees and a consultant were awarded an aggregate of 867,935 restricted Ordinary Shares of the Company. Such shares were subject to forfeiture over a three-year service period. The shares granted to the consultant and employees were valued at $7.72 and $7.76 per share, respectively, and were included in loss from operations over the requisite service period. As of December 31, 2018, all such shares are no longer subject to forfeiture as the three-year service period has been completed.

On June 7, 2018, 1,306,348 restricted Ordinary Shares, which represented 5% of the fully-diluted outstanding shares of the Company as of such date, were issued to certain members of senior management in accordance with their employment agreements. One-third of such shares vested immediately, with the balance vesting quarterly over the next eight quarters beginning three months after the effectiveness of the Company's registration statement on Form S-1 filed with the SEC on June 7, 2018 (the "Registration Statement"). The shares were valued at $15.00 per share and the related share-based compensation expense, which is recognized over the requisite service period, is included in general and administrative expenses in the consolidated statements of operations and comprehensive loss. Additionally, under the terms of the employment agreements, the Company was required to pay the income taxes incurred by the grantees in connection with the grant of those restricted shares. Total compensation expense in connection with the issuance of those restricted Ordinary Shares, in the amount of $20,141,876, of which $10,156,868 was share-based, was recorded as general and administrative expense during the year ended December 31, 2018 (See Note 16).

A summary of the restricted Ordinary Shares is as follows:

	Ordinary Shares	$ Value
Non-vested at December 31, 2016	386,608	$ 3,020,191
Vesting during 2017	(280,695)	(2,154,330)
Non-vested at December 31, 2017	105,913	865,861
Issued during 2018	1,306,348	19,595,220
Vesting during 2018	(759,087)	(10,663,471)
Non-vested at December 31, 2018	653,174	$ 9,797,610

Ordinary Shares

On March 1, 2018, a funding milestone was met under the employment agreements for certain members of senior management. Accordingly, the employees were issued an aggregate of 550,162 fully vested Ordinary Shares, which represented 3% of the fully-diluted outstanding shares of the Company as of such date. The shares were recorded as share-based compensation in the amount of $3,096,104. Additionally, under the terms of the employment agreements, the Company was required to pay the income taxes incurred by the grantees in connection with the grant of those shares. Total compensation expense in connection with the issuance of those Ordinary Shares, in the amount of $6,456,215, of which $3,096,104 was share-based, was recorded as general and administrative expense during the year ended December 31, 2018.

During the years ended December 31, 2018 and 2017 the Company recognized total share-based compensation expense in the accompanying consolidated statements of operations and comprehensive loss as follows:

	2018	2017
Research and development	$ 3,372,054	$ 2,374,899
General and administrative	14,511,500	584,832
Total share based compensation	$ 17,883,554	$ 2,959,731

The Company does not expect to realize any tax benefits from its share option activity or the recognition of share-based compensation expense because the Company currently has net operating losses and has a full valuation allowance against its deferred tax assets. Accordingly, no amounts related to excess tax benefits have been reported in cash flows from operations or cash flows from financing activities for the years ended December 31, 2018 and 2017.

12. Ordinary Shares, Convertible Preferred C Shares and Shareholders' Deficit:

Ordinary Share Issuances

2018

As discussed in Note 11, on March 1, 2018, a funding milestone was met under the employment agreements for certain members of senior management. Accordingly, the employees were issued an aggregate of 550,162 fully vested Ordinary Shares.

In connection with the Company's initial public offering, on June 7, 2018, the Company issued 5,000,000 Ordinary Shares at an offering price of $15.00 per share for gross proceeds of $75,000,000, excluding offering costs of $9,807,622.

Also, as discussed in Note 11, on June 7, 2018, upon the effectiveness of the Company's Registration Statement, 1,306,348 restricted Ordinary Shares, which represented 5% of the fully-diluted outstanding shares of the Company as of such date, were issued to certain members of senior management in accordance with their employment agreements. One-third of such shares vested immediately, with the balance vesting quarterly over the next eight quarters.

On October 5, 2018, in connection with an acquisition, the Company issued 202,500 Ordinary Shares with an additional 22,500 shares to be issued 18 months following the closing, subject to any indemnification claims under the merger agreement.

2017

On July 31, 2017, the Company issued 1,288 Ordinary Shares in connection with the exercise of an option.

On August 16, 2017, the Company issued 6,441 Ordinary Shares in connection with a research agreement.

Convertible Preferred C Shares

Issuances

2018

During the year ended December 31, 2018, the Company issued 5,425,124 Preferred Shares at an offering price of approximately $10.48 per share for gross proceeds of $56,849,611, excluding offering costs of $690,473.

Also, during the year ended December 31, 2018, the Company issued 129,419 Preferred Shares in lieu of payment of accounts payable in the aggregate amount $1,355,097 to certain vendors.

On March 15, 2018, the Company issued 13,360 Preferred Shares in connection with a license agreement.

On June 7, 2018, upon effectiveness of the Company's Registration Statement on Form S-1, all of the 11,501,432 outstanding Preferred Shares were automatically converted into 11,501,432 Ordinary Shares. In connection with the conversion of the Preferred Shares, $664,718 of unaccredited financing costs were fully accreted.

2017

During the year ended December 31, 2017, the Company issued 9,739 Preferred Shares at an offering price of $20.96 per share and 1,598,418 Preferred Shares at an offering price of $10.48 per share for gross proceeds of $16,854,656, excluding offering costs of $98,804. The net proceeds of the offering were used for working capital, research and development and general corporate purposes.

On November 2, 2017, a note payable to a related party in the amount of $2,500,000 was converted at the rate of $10.48 per share, into 238,579 Preferred Shares (see Note 15).

Warrants

In connection with the issuance of 715,737 Preferred Shares on September 21, 2017, at an offering price of $10.48 per share, the Company issued warrants to purchase 695,696 Preferred Shares at an exercise price of $10.48 per share. The warrants expired on the first of the following to occur:

(i) an Asset Sale;

(ii) a Qualified IPO;

(iii) a Share Sale;

(iv) the winding up of the Company; or

(v) On the third anniversary of the date of issuance

The Black-Sholes value of the warrants in the amount of $1,660,500 was accounted for as a warrant liability and a discount to the Preferred Shares at the time of issuance and were being accreted over the expected term of the Preferred Shares (see Note 2).

The Black-Scholes value of the warrants in the amount of $553,500 was recorded as a warrant liability and charged to convertible note inducement expense within the statement operations and comprehensive loss at the time of issuance.

Both of the warrants were revalued under the Black-Scholes valuation model at December 31, 2017, which resulted in an increase of the warrant liability in the amount of $465,633, which was charged to change in fair value of warrant liability within the statement of operations and comprehensive loss.

The warrant liability at December 31, 2017 was $2,679,633.

On June 5, 2018, all of the outstanding warrants to purchase 927,594 Preferred Shares at an exercise price of approximately $10.48 per share were exercised for aggregate cash proceeds of $9,720,000.

13. Net Loss per Share

The Company computes net loss per share in accordance with ASC 260-10, *Earnings per Share* (see Note 2).

Basic and diluted net loss per share is computed as follows:

	2018	2017
Net loss—basic and diluted	$(82,865,874)	$(31,044,535)
Accretion of Preferred Shares financing costs	(1,806,512)	(191,963)
Accretion of warrant	—	(615,000)
Adjusted net loss—basic and diluted	$(84,672,386)	$(31,851,498)
Weighted-average ordinary shares outstanding:		
Basic and Diluted	18,948,520	8,572,315
Net loss per share:		
Basic and Diluted	$ (4.47)	$ (3.72)

The following securities are considered to be Ordinary Share equivalents, but were not included in the computation of diluted net loss per Ordinary Share because to do so would have been anti-dilutive:

	December 31, 2018	December 31, 2017
Preferred Shares	—	5,005,934
Restricted Ordinary Shares subject to forfeiture	653,174	105,913
Stock options	3,262,365	938,637
Warrants	—	927,594
	3,915,539	6,978,078

14. Income Taxes:

Since the Company has recurring losses and a valuation allowance against deferred tax assets, there is no tax expense (benefit) for the year ended 2017. For the year ended December 31, 2018, the Company recognized a tax benefit of $(474,391).

The subsidiaries each file separate tax returns in their respective tax jurisdictions.

As of December 31, 2018, the Company had federal and state net operating loss (“NOL”) carryforwards in the United States of approximately $14,210,000 and $14,155,000, respectively, and in the United Kingdom of approximately $94,100,000, which are available to reduce future taxable income. The U.S. federal and state NOL carry forwards incurred prior to January 1, 2018 in the amount of approximately $6.8 million and $6.7 million, respectively, will begin to expire in 2036. The U.S. NOL incurred after December 31, 2018 and the U.K. NOL will be indefinitely carried forward. Also, as of December 31, 2018, the Company had orphan drug and research and development credits in the U.S. in the amount of $1,134,000 which will begin to expire in 2036. The NOL carry forwards are subject to review and possible adjustment by the U.S., U.K. and state tax authorities. NOL carry forwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders, as defined under

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Sections 382 Internal Revenue Code, as well as CTA 2010 Part 14 under the UK tax rules. This could limit the amount of NOLs that the Company can utilize annually to offset future taxable income or tax liabilities. As of December 31, 2018, the Company has performed such an analysis and determined that there were no limitations in the U.K. However, for U.S. purposes the Company determined that a change of ownership occurred in April 2016. The Company is still in the process of determining the annual limitation on losses that occurred prior to April 2016. Subsequent ownership changes and proposed future changes to the UK (or US) tax rules in respect of the utilization of losses carried forward may further affect the limitation in future years, if any. Additionally, the Company has not undertaken a study on the completeness of the U.S. orphan drug and research and development credit.

The Company's pre tax earnings from the United Kingdom and United States locations are as follows:

	December 31, 2018	December 31, 2017
United Kingdom	$(73,359,977)	$(26,458,625)
United States	(9,980,287)	(4,585,910)
	$(83,340,264)	$(31,044,535)

The Company is subject to the corporate tax rate in the U.K. as a Limited U.K. corporation.

The following table summarizes a reconciliation of income tax benefit compared with the amounts at the U.K. statutory income tax rate:

	December 31, 2018		December 31, 2017	
Statutory rate	(15,834,650)	19.00%	(5,976,073)	19.25%
Permanent differences—other	1,438,934	-1.73%	654,648	-2.11%
RTP and other adjustments	387,509	-0.46%	(152,948)	0.49%
U.K. tax credit	1,707,489	-2.05%	539,136	-1.74%
U.S. tax credit	(436,250)	0.52%	(363,665)	1.17%
Foreign tax rate differential	(171,693)	0.21%	(673,619)	2.17%
State and local rate, net of federal tax	(1,159,522)	1.39%	(446,683)	1.44%
UK Rate Change (17% at expected DTA turn)	1,104,863	-1.33%	482,351	-1.55%
U.S. state rate change	(6,496)	0.01%	993,998	-3.20%
Change in valuation allowance	12,495,426	-14.99%	4,942,855	-15.92%
Actual income tax benefit effective tax rate	(474,391)	0.57%	—	0.00%

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The Expense/(Benefit) for income taxes from continuing operations consists of the following:

	December 31, 2018	December 31, 2017
Current Tax Expense/(Benefit)		
United Kingdom	—	—
United States	—	—
Total Current	—	—
Deferred Tax Expense/(Benefit)		
United Kingdom	$ (8,888,096)	$(3,759,109)
United States	(3,606,275)	(1,183,746)
Total Deferred	(12,494,371)	(4,942,855)
Change in Valuation Allowance	12,019,880	4,942,855
Total Income Tax Expense/(Benefit)	$ (474,391)	$ —

Income tax (benefit) expense for each year is allocated to continuing operations, discontinued operations, extraordinary items, other comprehensive income, the cumulative effects of accounting changes, and other charges or credits recorded directly to shareholders' equity. *ASC 740-20-45 Income Taxes, Intraperiod Tax Allocation, Other Presentation Matters* includes an exception to the general principle of intraperiod tax allocations. The codification source states that the tax effect of pretax income or loss from continuing operations generally should be determined by a computation that considers only the tax effects of items that are included in continuing operations. The exception to that incremental approach is that all items (i.e. other comprehensive income, discontinued operations, etc.) be considered in determining the amount of tax benefit that results from a loss from continuing operations and that benefit should be allocated to continuing operations. That is, when a company has a current period loss from continuing operations, management must consider income recorded in other categories in determining the tax benefit that is allocated to continuing operations. This includes situations in which a company has recorded a full valuation allowance at the beginning and end of the period, and the overall tax provision for the year is zero. The intraperiod tax allocation is performed once the overall tax provision has been computed and allocates that provision to various income statement (continuing operations, discontinued operations), other comprehensive income and balance sheet captions. While the intraperiod tax allocation does not change the overall tax provision, it results in a gross-up of the individual components. The level of application has been applied on the group level.

As the Company experienced a net loss from operations for the year ended December 31, 2018 and other comprehensive income from foreign currency translation adjustments, the Company has allocated income tax expense against the components of other comprehensive income in 2018 using a 17% effective tax rate. Income tax benefit for the year ended December 31, 2018 includes a benefit of $(474,391) due to the required intraperiod tax allocation. Conversely, other comprehensive income for the year ended December 31, 2018 includes income tax expense of $474,391.

Deferred Tax Assets/(Liabilities)

	December 31, 2018		
	Total	UK	US
Deferred Tax Assets:			
Net operating loss carryforwards	$ 20,646,185	$ 16,000,329	$ 4,645,856
Other	1,414,690	(46,604)	1,461,294
Tax Credit	1,133,656	—	1,133,656
Deferred tax assets	23,194,531	15,953,725	7,240,806
Less: valuation allowance	(23,194,531)	(15,953,725)	(7,240,806)
Net deferred tax asset	$ —	$ —	$ —

	December 31, 2017		
	Total	UK	US
Deferred Tax Assets:			
Net operating loss carryforwards	$ 9,462,690	$ 6,909,754	$ 2,552,937
Other	539,008	152,554	386,454
Tax Credit	697,406	—	697,406
Deferred tax assets	10,699,105	7,062,308	3,636,797
Less: valuation allowance	(10,699,105)	(7,062,308)	(3,636,797)
Net deferred tax asset	$ —	$ —	$ —

ASC 740 requires a valuation allowance to reduce the deferred tax assets reported if, based on the weight of available evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. After consideration of all the evidence, both positive and negative, the Company has recorded a full valuation allowance against its deferred tax assets at December 31, 2018 and 2017 because the Company's management has determined that is it more likely than not that these assets will not be fully realized.

Changes to the U.K. and U.S. corporation tax rates have been announced which will impact future accounting periods. In his budget of July 8, 2015, the Chancellor of the Exchequer announced a reduction in the U.K. corporation tax rate to 19% for the financial year beginning April 1, 2017 and a further reduction to 18% for the financial year beginning April 1, 2020. These changes received Royal Assent on November 18, 2015. The U.K. Finance Act 2016 provides for a further reduction in the corporation tax rate to 17% for the Financial Year beginning April 1, 2020. This change was enacted on September 15, 2016. As the Company does not expect to be able to utilize its NOL's in the U.K. prior to its financial year beginning on January 1, 2021, if at all, the deferred tax has been calculated using a tax rate of 17%.

In the United States, the corporation tax rate was reduced to 21% for the financial year beginning January 1, 2018. As these changes were enacted prior to the December 31, 2017 balance sheet date, deferred tax has been calculated accordingly in these consolidated financial statements, which represented a decrease in the prior years deferred tax assets of approximately $994,000.

The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2018 and 2017, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's statements of operations and comprehensive loss.

The Company files income tax returns in the United States, United Kingdom and the Netherlands, and various state jurisdictions. For the US, the statute of examination is open for tax years 2015, 2016, 2017 and 2018. For the UK and the Netherlands, the statute of examination is open for tax years 2017 and 2018.

MeiraGTx Holdings plc is a UK tax resident with no earnings in its foreign subsidiaries and the Company does not expect any temporary basis difference in its investment in these subsidiaries to reverse in the foreseeable future. Therefore, the Company has not recorded deferred taxes on the outside basis difference in its foreign subsidiaries. It is not probable to compute the amounts, if any.

15. Related Party Transactions:

Transition Services Agreement

Effective April 24, 2015, the Company entered into a transition services agreement (the "TSA") with Kadmon, which owned 12.9% of the Company at December 31, 2018, whereby Kadmon would provide office and laboratory facilities as well as certain other personnel support activities to the Company. Under the agreement, the Company is charged for (i) rent based upon the square footage of the office and laboratory facilities used by the Company (ii) other personnel support activities based upon the hours of the personnel providing the support activities, and (iii) and other direct costs incurred by Kadmon on behalf of the Company, plus a 7% administrative fee. The TSA terminated on April 24, 2018 and the Company is currently leasing office space on a month to month basis from Kadmon.

During the years ended December 31, 2018 and 2017, the Company incurred the following charges in connection with the TSA which are included in loss from operations:

	2018	2017
Rent	$557,698	$548,229
Personnel	6,493	39,721
Other	6,334	5,983
Total charges incurred	$570,525	$593,933

During the year ended December 31, 2018 and 2017, the Company made cash payments totaling $1,431,555 and $275,941, respectively.

The amount due to Kadmon at December 31, 2018 and 2017 is $0 and $861,030, respectively, and is disclosed as Due to Kadmon on the Company's consolidated balance sheets.

Research Agreement

Effective October 23, 2016, the Company entered into a four-year master services agreement with UCL Consultants Limited, an entity affiliated with University College of London ("UCL"), which is a shareholder of the Company. Pursuant to the agreement, UCL Consultants Limited provides pre-clinical research and development under the direction of the Company. In connection with the agreement, the Company issued several work orders during the years ended December 31, 2016 and 2017 in the aggregate amounts of £1,402,202, or approximately $1,885,000, based upon the average exchange rates during the years ended December 31, 2016 and 2017, respectively. Either party may terminate the agreement by giving 30 days written notice. Total research and development expenses under this agreement for the years ended December 31, 2018 and 2017 was approximately $636,000 and $538,000, respectively. Future obligations under the agreement equal £612,382, or approximately $779,685, through October 2020.

The amount due to UCL under the master services agreement at December 31, 2018 and 2017 is $389,101 and $775,315, respectively and is included in accounts payable and accrued expenses on the Company's consolidated balance sheets.

Effective September 1, 2016, the Company entered into a manufacturing and drug supply agreement with UCL. Pursuant to the agreement, UCL manufactured materials for the Company's clinical trials under the

direction of the Company. The agreement was terminated in January 2018. Total research and development expenses under this agreement for the years ended December 31, 2018 and 2017 was approximately $0 and $1,904,352, respectively.

The amount due to UCL under the manufacturing and drug supply agreement at December 31, 2018 and 2017 is $0 and $2,466,142, respectively and is included in accrued expenses on the balance sheet.

Leases

July 2016 Lease

Effective July 1, 2016, the Company entered into a non-cancellable operating lease for laboratory and related office facilities in New York with ARE, an entity that is under common control by an entity that is a minority shareholder of the Company and whose executive chairman and founder is a director of the Company. The lease provides for monthly base rent and property management fees, including rent escalations and rent holidays, plus operating expenses during the lease term, which expires on December 31, 2021. The Company records monthly rent expense on a straight-line basis from July 1, 2016 through December 31, 2021. As of December 31, 2018 and 2017, the balance of deferred rent, representing the difference between cash rent paid and straight-line rent expense, was $201,264 and $231,276, respectively.

Total rent expense under this operating lease was $487,555 and $487,559 for the years ended December 31, 2018 and 2017, respectively. As of December 31, 2018, the aggregate future minimum rental payments under this lease are $1,663,952.

In connection with the signing of this lease, the Company entered into a standby letter of credit agreement for $122,866, which serves as a security deposit for the premises. The standby letter of credit expired on July 7, 2017 and was automatically renewed annually through July 7, 2021. This standby letter of credit is secured with restricted cash in a money market account (see Note 6).

December 2016 Lease

Effective December 15, 2016, the Company entered into another non-cancellable operating lease with ARE, expiring on October 31, 2032, for laboratory and office facilities in New York. The lease provided for monthly base rent, including rent escalations, property management fees and rent holidays, plus operating expenses during the lease term. The Company recorded monthly rent expense on a straight-line basis from December 15, 2016 through October 31, 2032. On October 26, 2017, the lease was amended, whereby the lease would terminate on March 31, 2018 and only base rent and management fees in the aggregate amount of $563,507 would be due from November 1, 2017 through March 31, 2018. Under the amendment, the Company issued a note to ARE in the amount of $1,442,009 (see Note 9), removed the balance of the deferred rent and accrued the future rent payments, all of which were recorded as rent expense at the time of the amendment, in accordance with ASC 420, Exit and Disposal Activities, as the Company had a cease use date as of the date of the amendment.

Total rent expense under this operating lease was $0 and $1,660,806 for the years ended December 31, 2018 and 2017, respectively.

Convertible Note Payable

On May 1, 2017, the Company issued a convertible note in the amount of $2,500,000 to ARE. The note had an interest rate of 10% per annum and was convertible into Preferred Shares at any time at the option of the holder or would automatically convert into Preferred Shares in the event of an equity investment by a

mutually agreed upon institutional investor at a price per share equal to the lowest price paid per share by a purchaser of the Company's Preferred Shares. On November 2, 2017 the note was converted to 238,579 Preferred Shares at $2.70 per share. In accordance with the terms of the convertible note, the accrued interest in the amount of $145,833 was cancelled.

As an inducement to convert the convertible note, the Company issued a warrant to purchase 900,000 Preferred Shares, at an exercise price of $2.70 per share, to the holder of the convertible note, which was expenses in accordance with ASC 470 (see Note 12).

16. Commitments:

Operating Leases

In February 2016, the Company entered into a non-cancellable operating lease, expiring in February 2021, for manufacturing and office facilities in London, UK. The lease provides for an additional five-year term at the Company's option. The lease provides for monthly base rent, plus operating expenses and real estate taxes during the lease term.

Total rent expense under this operating lease was $273,430 and $279,303 for the years ended December 31, 2018 and 2017, respectively.

On October 1, 2017, the Company entered into a one-year non-cancellable operating lease, expiring in September 2018, for office and laboratory facilities in Leiden, Netherlands. The lease provides for monthly base rent plus operating expenses during the lease term. The lease provides for successive one-year extensions up to a maximum of four extensions. Total rent expense under this operating lease was $9,313 and $5,273 for the years ended December 31, 2018 and 2017, respectively.

In June 2017, the Company entered into two non-cancellable operating leases, expiring in July 2018, for office and laboratory facilities in London, UK. The lease provides for monthly base rent, rent holidays plus operating expenses and real estate taxes during the lease term. The Company records monthly rent expense on a straight-line basis from June 1, 2017 through July 23, 2018. As of December 31, 2018, the balance of deferred lease obligation, representing the difference between cash rent paid and straight-line rent expense, was $0. Total rent expense under theses operating leases was $73,846 and $85,222 for the years ended December 31, 2018 and 2017, respectively.

On July 27, 2018 the two leases for office and laboratory facilities in London, UK expired. Effective July 27, 2018, the Company entered into two new non-cancellable operating leases for the same office and laboratory facilities in London. The leases provide for annual base rent in the aggregate amount of approximately $363,000, plus operating expenses, through May 31, 2022, at which time the annual base rent will be revalued based on market rates at that time. The leases expire on May 24, 2027. Total rent expense under these operating leases was $148,561 and $0 for the years ended December 31, 2018 and 2017, respectively.

The aggregate future minimum rental payments under these operating leases are as follows:

2019	$ 353,782
2020	$ 350,284
2021	$ 350,284
2022	$ 350,284
2023	$ 350,284
Thereafter	$ 1,196,805
Total future rent payments	$ 2,951,723

The aggregate future minimum rental payments of all leases, including those discussed in Note 15 are as follows:

2019	$ 889,465
2020	$ 904,716
2021	$ 924,121
2022	$ 350,284
2023	$ 350,284
Thereafter	$ 1,196,805
Total future rent payments	$ 4,615,675

Service Agreements

On April 27, 2015, the Company entered into service agreements with a senior officer and a greater than 5% shareholder of the Company. Under the terms of the agreements, the employees will receive aggregate compensation of £300,000, which has been increased to a maximum aggregate amount of £410,000 per annum, or approximately $522,000 using exchange rates as of December 31, 2018. The agreements also provide for contributions to a defined contribution pension plan to be set up by the Company and a discretionary bonus. The agreements may be terminated at any time by either party by giving twelve-months' notice, or the Company may terminate the officer's employment effective immediately upon notice, and within 28 days making payment in lieu of notice consisting of a sum equivalent to the officer's annual salary for the relevant period. For the years ended December 31, 2018 and 2017, the Company recorded £1,001,000 and £724,000 or approximately $1,334,000 and $933,000, respectively, using the average exchange rates during the years ended December 31, 2018 and 2017, respectively, in research and development costs under these agreements. Future obligations to be paid under these agreements equal £150,333, or approximately $192,000, using exchange rates as of December 31, 2018.

In connection with the service agreements, on April 24, 2015, the employees were awarded, under a share award agreement (the "Share Award Agreement"), an aggregate of 2,704,800 restricted Ordinary Shares and 750 B ordinary shares, which B ordinary shares have been converted into Ordinary Shares, of the Company. Under the Share Award Agreement, such shares are subject to forfeiture ratably over a period of three years if the employee's do not remain an employee or consultant to the Company. The shares were valued at $7.76 per share and, in accordance with ASC 718, were charged to operations as share-based compensation ratably over the forfeiture period.

Employment Agreements

In February 2016, the Company entered into three-year employment agreements with certain senior officers of the Company. Under the terms of the agreements, which automatically renew for successive one-year terms, the employees will receive annual compensation in the aggregate amount of $710,000, which has been increased to a maximum aggregate amount of $1,075,000. The employment agreements also provide for an annual guaranteed cash bonus targeted at 100% of annual compensation. The agreements also provide for discretionary annual performance bonuses targeted to be not less than 50-60% of the employee's base salary and grants of restricted stock. In January 2018 the Company's compensation committee approved a discretionary bonus in the aggregate amount of $1,196,000. This discretionary bonus and the guaranteed bonus for 2017, in the amount of $850,000, were subject to compensation committee approval and meeting certain future funding conditions. On February 28, 2018, the funding conditions were met. The senior officers were granted their guaranteed and discretionary bonuses for the year ended December 31, 2018, in the aggregate amount of $3,415,000, which was paid in January 2019.

Additionally, the agreements provided for equity incentives of up to an aggregate maximum of 8.0% of the Company's fully diluted outstanding shares upon the attainment of certain milestones. On March 1, 2018, a funding milestone was met. Accordingly, the employees were issued an aggregate of 3% of the fully-diluted outstanding shares of the Company as of such date. On June 7, 2018, an additional milestone was met. Accordingly, the employees were issued an aggregate of 5% of the fully-diluted outstanding shares of the Company as of such date (see Note 11).

The employees are also entitled to participate in all incentive and deferred compensation and employee benefit programs available to employees and executive officers of the Company. Future obligations to be paid under these agreements equal $1,258,750, as of December 31, 2018.

Consulting and other Agreements

Effective September 28, 2015, the Company entered into a three-year consulting agreement with a consultant to provide ongoing strategic advice and to serve on the Company's board of directors. In connection with the agreement, the Company issued 662,910 restricted Ordinary Shares. Under the consulting agreement, such shares were subject to forfeiture ratably over a period of three years if the consultant does not remain a consultant to the Company. The shares were valued at $7.72 per share and were charged to general and administrative expenses upon the expiration of each forfeiture period. For the years ended December 31, 2018 and 2017, the Company recorded $263,970 and $351,960, respectively, in general and administrative expense under this agreement. There are no future obligations to be paid under the agreement.

Research Agreements

On April 24, 2015, the Company entered into a cooperative research and development agreement (CRADA) with the U.S. Department of Health & Human Services, as represented by the National Institute of Dental and Craniofacial Research (NIDCR) and Institute or Center of the National Institutes of Health (NIH). The CRADA provided for quarterly payments of $21,250 for three years through April 30, 2017 and a cost per patient for each patient enrolled in the Company's xerostomia clinical trial. The CRADA was amended on March 25, 2016 to extend the term through March 25, 2021 and to extend the annual payments throughout the revised term. Research and development expenses under the CRADA for the years ended December 31, 2018 and 2017 were $111,938 and $115,374, respectively. Future obligations to be paid under the CRADA, as amended, through March 25, 2021 equal $191,250.

On March 22, 2016, the Company entered into another CRADA with the NIDCR and NIH for the treatment of *Sjögren's syndrome* associated salivary hypofunction. The CRADA provides for quarterly payments of $104,500 for the first three years of the agreement plus a cost per patient for each patient enrolled in a clinical trial. The costs associated with years four and five of the *Sjögren's syndrome* CRADA will be determined at a later date. Total research and development expenses under this agreement for each of the years ended December 31, 2018 and 2017 were $418,000. There are no future obligations to be paid under the agreement.

Effective December 5, 2016, the Company entered into a three-year research collaboration agreement with Cornell University. Pursuant to the agreement, Cornell University provides research and development under the direction of the Company. In connection with the agreement, in July 2017, the Company issued 25,000 Ordinary Shares to Cornell University, which were recorded as research and development expenses in the amount of $17,000. The Company amended this agreement, effective June 12, 2017, to add a second three-year research collaboration project through September 2019. The Company further amended this agreement, effective October 18, 2018 to include additional costs related to the research. Total research and

development expenses under this agreement, as amended, for the years ended December 31, 2018 and 2017 were $1,625,152 and $1,029,904, respectively. Future obligations to be paid under the agreement through December 5, 2019 equal $2,002,228.

On February 14, 2017, the Company entered into a one-year research collaboration agreement with Cornell University in the amount of $679,473. On August 24, 2017, the agreement was amended to add an additional study in the amount of $182,520. Total research and development expenses under this agreement for the years ended December 31, 2018 and 2017 were $143,073 and $698,307, respectively.

License Agreements

Effective February 4, 2015, the Company entered into an exclusive worldwide license agreement with UCL Business, PLC ("UCL Business") to develop up to eight programs using certain ocular gene therapy technology. Under the terms of the agreement, as amended, the Company will pay UCL Business certain sales milestone payments, if achieved, in the aggregate amount of £39.8 million, or approximately $50.7 million using the exchange rate at December 31, 2018, and royalties on net sales, as defined upon commercialization. Additionally, the Company is responsible for all patent prosecution and maintenance costs incurred and will also pay UCL Business an annual maintenance fee of £50,000, or approximately $64,000, until the first commercial sale of a product. The agreement will terminate upon the later of (i) the last valid claim in a relevant product, (ii) the expiration of regulatory exclusivity to all licensed products, or (iii) the 10th anniversary of the first commercial sale of a product. Total research and development expenses under the agreement for the years ended December 31, 2018 and 2017 were $75,073 and $73,250, respectively.

Effective July 28, 2017, the Company entered into another exclusive worldwide license agreement with UCL Business for an additional program using certain ocular gene therapy technology. The Company will pay UCL Business certain milestone payments, royalties and annual maintenance fees under the same terms and conditions as the license dated February 4, 2015. Total research and development expenses under the agreement for the years ended December 31, 2018 and 2017 were $66,630 and $82,260, respectively.

Effective March 15, 2018, the Company entered into another exclusive worldwide license agreement with UCL Business for an additional program using certain ocular gene therapy technology. The Company will pay UCL Business certain milestone payments, royalties and annual maintenance fees under the same terms and conditions as the license dated February 4, 2015. Total research and development expenses under the agreement for the years ended December 31, 2018 and 2017 were $133,728 and $0, respectively.

On September 7, 2018, the Company entered into an exclusive licensing agreement with the National Institutes of Health for worldwide rights to expanded indications for use of AAV-AQP1 for treatment of xerostomia (dry mouth) and xerophthalmia (dry eye) associated with *Sjögren's syndrome.* This agreement expands the Company's original exclusive licensing agreement with the NIH for exclusive worldwide rights to AAV-AQP1 that was executed as of November 9, 2017. AAV-AQP1 is currently in Phase 1/2 development for treatment of grade 2 or 3 radiation-induced xerostomia. Total research and development expenses under the agreement for the years ended December 31, 2018 and 2017 were $50,000 and $0, respectively.

Effective January 1, 2016, the Company entered into an Agreement ("Agreement") and Plan of Merger to acquire all of the outstanding shares of BRI-Alzan from the shareholders of BRI-Alzan. In connection with the Agreement, the Company will pay certain development milestone payments if achieved, in the aggregate amount of $4.5 million, and annual royalty payments on annual net sales following the first commercial sale of any product containing the technology acquired. Total research and development expenses under the agreement for the years ended December 31, 2018 and 2017 were $15,000 and $30,000, respectively.

17. Employee Benefit Plans

United States

On January 1, 2017, Meira LLC adopted a defined contribution retirement plan that complies with Section 401(k) of the Internal Revenue Code. All Meira LLC employees over the age of 21 are eligible to participate in the plan after three consecutive months of service. Employees are able to defer a portion of their pay into the plan on the first day of the month or after the day all age and service requirements have been met. The plan provides for a Company matching contribution. All eligible employees receive an employer matching contribution equal to the lesser of the amount the employee contributes to the plan or 6% of their salary up to the annual IRS limit.

United Kingdom

On August 1, 2016, Meira UK II adopted a defined contribution group personal pension plan that complies with HM Revenue and Customs (HMRC) for tax relief. All Meira UK II employees are eligible to participate in the plan upon joining the company and providing the required services. All eligible employees, if they elect to join the pension scheme, receive an employer pension contribution equal to 7.5% to 10.0% of their pensionable earnings. Currently, employees are not required to contribute, but may make optional contributions up to the annual allowance HMRC limits.

Under the HMRC requirements, current required 6+minimum employer contributions are 5-6% but will rise to 8-9% after April 2019.

During the years ended December 31, 2018 and 2017, employer contributions to all plans were $440,368 and $252,700, respectively.

18. Subsequent Events:

Management has evaluated subsequent events through the date these financial statements were issued. Based on our evaluation, the following disclosures have been made:

License Agreement

On January 29, 2019, the Company amended and restated the following agreements: (i) the License Agreement, dated February 4, 2015, as amended, between the Company and UCL Business, Plc ("UCLB"); (ii) the License Agreement, dated July 28, 2017, as amended, between the Company and UCLB; and (iii) the License Agreement, dated March 15, 2018, between the Company and UCLB to establish new stand-alone license agreements for the following inherited retinal disease programs: (a) achromatopsia ("ACHM") caused by mutations in CNGB3; (b) ACHM caused by mutations in CNGA3; (c) X-linked retinitis pigmentosa ("XLRP"); and (d) RPE65-mediated IRD.

The Company's obligation to pay UCLB a share of certain sublicensing revenues, as was provided under the February 4, 2015 agreement, has been removed from each of the stand-alone agreements. Each of the stand-alone agreements now reflects terms substantially similar to those of the March 15, 2018 agreement.

Additionally, the new stand-alone agreement related to CNGB3 provides for the Company to pay UCLB an upfront payment of £1,500,000, or approximately $1,976,000, and issue £1,500,000 of the Company's ordinary shares.

Collaboration Agreement

On January 30, 2019, (the "Agreement Date"), the Company entered into a Collaboration Agreement with Janssen Pharmaceuticals, Inc. ("Janssen") for the research, development and commercialization of gene therapies for the treatment of inherited retinal diseases ("IRDs").

Under the terms of the agreement, the Company will receive a $100 million cash upfront payment. Janssen and the Company will collaborate to develop the Company's current clinical programs in Retinitis Pigmentosa and two genetic forms of Achromatopsia and Janssen has the exclusive right to commercialize these products globally. The Company will manufacture these products for commercial supply. Janssen will pay 100% of the clinical and commercialization costs of the products and the Company is eligible to receive untiered 20 percent royalties on net sales of products and additional development and commercialization milestones of up to $340 million. In addition, the Company and Janssen have entered into a research collaboration in the area of IRDs, with Janssen paying for the majority of the research costs. Janssen has the right to exclusively license any product coming out of the collaboration at the time of IND. Janssen will then pay 100% of the clinical and commercialization costs for these products and the Company will receive an untiered royalty on net sales in the high teens as well as development milestones. In addition, Janssen and the Company have entered into a manufacturing research collaboration to further develop processes for manufacturing AAV viral vectors in which the costs of the research will be shared.

Private Placement

On February 27, 2018, the Company issued 5,797,102 Ordinary Shares in a private placement for gross proceeds of $80 million, excluding offering costs of approximately $2.6 million. Johnson & Johnson Innovation – JJDC, Inc., the investment arm of Johnson and Johnson, led the offering and purchased 2,898,550 of the Ordinary shares issued on the same terms and conditions as the other investors in the offering.

In connection with the offering, the Company also entered into a registration rights agreement whereby, promptly following the date on which the Company becomes eligible to use a registration statement on Form S-3, but in no event later than July 31, 2019, the Company shall prepare and file a registration statement covering the resale of all of the Registrable Securities, as defined in the agreement. The Company shall use commercially reasonable efforts to have the registration statement declared effective as soon as practicable. If the registration statement is not declared effective prior to the 120th day after July 31, 2019 (or the 150th day if the Securities and Exchange Commission reviews such registration statement), then the Company will make pro rata payments in cash to each investor then holding Registrable Securities, as liquidated damages, in an amount equal to 1% of the aggregate amount invested by such investor for each thirty (30)-day period or pro rata for any portion thereof following the date by which such registration statement should have been effective.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

Not Applicable.

ITEM 9A. CONTROLS AND PROCEDURES

Limitations on Effectiveness of Controls and Procedures

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), evaluated, as of the end of the period covered by this Form 10-K, the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")). Based on that evaluation, our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer) concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2018.

Exemption from Management's Report on Internal Control Over Financial Reporting

This Form 10-K does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

In addition, for so long as we qualify as an "emerging growth company" as defined under the JOBS Act, our independent registered accounting firm is not required to issue an attestation report on our internal control over financial reporting.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended December 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item is incorporated by reference to our definitive proxy statement for our 2019 annual shareholder meeting to be filed with the SEC within 120 days of the fiscal year ended December 31, 2018.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item is incorporated by reference to our definitive proxy statement for our 2019 annual shareholder meeting to be filed with the SEC within 120 days of the fiscal year ended December 31, 2018.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Securities Authorized for Issuance Under Equity Compensation Plans (as of December 31, 2018)

The following table provides information as of December 31, 2018, regarding our ordinary shares that may be issued under the MeiraGTx Holdings plc 2016 Equity Incentive Plan, as amended (the "2016 Plan"), the MeiraGTx Holdings plc 2018 Incentive Award Plan (the "2018 Plan") and the MeiraGTx Holdings plc 2018 Employee Stock Purchase Plan (the "2018 ESPP").

Plan category:	Number of Securities to be Issued Upon Exercise of Outstanding Options, Warrants, and Rights (a)	Weighted-Average Exercise Price of Outstanding Options, Warrants, and Rights (b)	Number of Securities Available for Future Issuance Under Equity Compensation Plans (excludes securities reflected in column (a)) (c)
Equity compensation plans approved by shareholders			
2016 Plan(1)	1,603,765	$ 5.34	—
2018 Plan (2)	1,658,600	$ 9.87	1,396,396
2018 ESPP (3)	—	—	509,166
Equity compensation plans not approved by shareholders	—	—	—
Total	3,262,365	$ 7.64	1,905,562

(1) In connection with our IPO, we assumed the 2016 Plan. As the 2016 Plan was previously approved by our shareholders and, as we will not make future grants or awards under these plans, it is listed as "approved by shareholders." As such, the securities remaining available under the 2016 Plan have been excluded from the table above.

(2) Pursuant to the terms of the 2018 Plan, the number of ordinary shares available for issuance under the 2018 Plan automatically increases on each January 1, until and including January 1, 2028, by an amount equal to the lesser of: (a) 4% of the aggregate number of ordinary shares outstanding on the final day of the immediately preceding calendar year and (b) such smaller number of ordinary shares as is determined by our board of directors.

(3) Pursuant to the terms of the 2018 ESPP, the number of ordinary shares available for issuance under the 2018 ESPP automatically increases on each January 1, until and including January 1, 2028, by an amount equal to the lesser of: (a) 1% of the aggregate number of ordinary shares outstanding on the final day of the immediately preceding calendar year and (b) such smaller number of ordinary shares as is determined by our board of directors, subject to the limit set forth in the 2018 ESPP.

Other

The remaining information required by this Item is incorporated by reference to our definitive proxy statement for our 2019 annual shareholder meeting to be filed with the SEC within 120 days of the fiscal year ended December 31, 2018.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The information required by this Item is incorporated by reference to our definitive proxy statement for our 2019 annual shareholder meeting to be filed with the SEC within 120 days of the fiscal year ended December 31, 2018.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this Item is incorporated by reference to our definitive proxy statement for our 2019 annual shareholder meeting to be filed with the SEC within 120 days of the fiscal year ended December 31, 2018.

PART IV

ITEM 15. EXHIBITS

(a) List of documents filed as part of this Form 10-K:

(1) Financial Statements

The financial statements included in Part II, Item 8 of this document are filed as part of this Form 10-K.

(2) Financial Statement Schedules

All schedules have been omitted because they are not required or because the required information is given in the consolidated financial statements or notes thereto.

(3) Exhibits

The following documents are filed as exhibits to this Form 10-K.

Exhibit Number	Exhibit Description	Incorporated by Reference				Filed/ Furnished Herewith
		Form	File No.	Exhibit	Filing Date	
2.1†	Agreement and Plan of Merger, dated October 5, 2018, by and among MeiraGTx Holdings plc, Vector Neurosciences Inc., VN Acquisition, Inc., VN Acquisition 2, Inc., the Vector stockholders named therein and the Vector stockholder representative, Stephen Kaplitt.					*
3.1	Restated Articles of Association of the Registrant.	S-1	333-224914	3.1	5/29/18	
4.1	Specimen Share Certificate evidencing the ordinary shares of the Registrant.	S-1	333-224914	4.1	5/29/18	
4.2	Shareholder Agreement		333-224914	4.2	6/4/18	
10.1#	2016 Equity Incentive Plan, as amended, and form of option agreements thereunder.	S-1/A	333-224914	10.1	5/29/18	
10.2#	2018 Incentive Award Plan and forms of award agreements thereunder.	S-1/A	333-224914	10.2	5/29/18	
10.3#	Non-Employee Director Compensation Program.	S-1/A	333-224914	10.3	5/29/18	
10.4#	Form of Indemnification Agreement for Directors and Officers.	S-1/A	333-224914	10.4	5/29/18	
10.5	Lease Agreement, dated June 29, 2016, as amended, between MeiraGTx Limited and ARE-East River Science Park LLC.	S-1	333-224914	10.5	5/14/18	

Exhibit Number	Exhibit Description	Incorporated by Reference				
		Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
10.6	Lease Agreement, effective February 2, 2016, among MeiraGTx Limited, Moorfields Eye Hospital NHS, Foundation Trust and Kadmon Corporation LLC.	S-1	333-224914	10.6	5/14/18	
10.7#	Employment Agreement, dated February 15, 2016, between MeiraGTx Limited and Alexandria Forbes, Ph.D., as amended.	S-1/A	333-224914	10.7	5/29/18	
10.8#	Employment Agreement, dated February 15, 2016 between MeiraGTx Limited and Richard Giroux, as amended.	S-1/A	333-224914	10.8	5/29/18	
10.9#	Employment Agreement, dated April 27, 2015, between MeiraGTx Limited and Stuart Naylor, Ph.D., as amended	S-1/A	333-224914	10.9	5/29/18	
10.10†	Agreement and Plan of Merger, dated December 31, 2015, among MeiraGTx Acquisition Corporation, BRI-Alzan, Inc., F-Prime Inc., Gregory Petsko, Dagmar Ringe, Brandeis University and MeiraGTx Limited.	S-1/A	333-224914	10.14	5/29/18	
10.11#	2018 Employee Share Purchase Plan.	S-1/A	333-224914	10.15	5/29/18	
10.12#	UK Sub-Plan Under the 2018 Incentive Award Plan.					*
10.13#	Form of Option Grant Notice and Option Agreement Under the UK Sub-Plan to the 2018 Incentive Award Plan.					*
10.14#	Employment Offer Letter, dated October 2, 2018, between MeiraGTx Holdings plc and Katherine Breedis	8-K	001-38520	10.1	10/9/18	
10.15	Lease agreement by and between Moorfields Eye Hospital NHS Foundation Trust and MeiraGTx UK II Limited, dated July 30, 2018	10-Q	001-38520	10.4	8/08/18	
10.16	Lease agreement by and between Moorfields Eye Hospital NHS Foundation Trust and MeiraGTx UK II Limited, dated July 30, 2018.	10-Q	001-38520	10.5	8/08/18	
10.17	Transfer of Title, dated December 14, 2018, and Lease, dated October 12, 2001, relating to the Pharmacy Manufacturing Unit, Britannia Walk, London, England	8-K	001-38520	10.1	12/14/18	

Exhibit Number	Exhibit Description	Incorporated by Reference				
		Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
10.18	Overage Deed, dated December 14, 2018, between Moorfields Eye Hospital NHS Foundation Trust and MeiraGTx UK II Limited relating to the Pharmacy Manufacturing Unit, Britannia Walk, London, England	8-K	001-38520	10.2	12/14/18	
10.19†	Consulting Agreement, dated October 5, 2018, between MeiraGTx Holdings plc, Vector Consulting LLC, Michael G. Kaplitt, Matthew During, and Stephen B. Kaplitt.					*
10.20†	License Agreement (RPE65), dated January 29, 2019, as amended and restated by and among UCL Business PLC, MeiraGTx UK II Limited and MeiraGTx Limited.					*
10.21†	License Agreement (CNGB3), dated January 29, 2019, as amended and restated by and among UCL Business PLC, MeiraGTx Holdings plc, MeiraGTx UK II Limited and MeiraGTx Limited.					*
10.22†	License Agreement (CNGA3), dated January 29, 2019, as amended and restated by and among UCL Business PLC, MeiraGTx UK II Limited and MeiraGTx Limited.					*
10.23†	License Agreement (RPGR), dated February 5, 2019, as amended and restated by and among UCL Business PLC, MeiraGTx UK II Limited and MeiraGTx Limited.					*
10.24†	Amendment No. 4 to Exclusive License Agreement, dated January 29, 2019, between UCLB and MeiraGTx Limited.					*
10.25†	Collaboration, Option and License Agreement, dated January 30, 2019, by and among Janssen Pharmaceuticals, Inc., MeiraGTx UK II Limited and MeiraGTx Holdings plc.					*
10.26†	Registration Rights Agreement, dated February 26, 2019, by and among MeiraGTx Holdings plc and the investors named therein.	8-K	001-38520	10.2	2/26/19	
21	List of Subsidiaries					*
23.1	Consent of Ernst & Young LLP					*

Exhibit Number	Exhibit Description	Incorporated by Reference				
		Form	File No.	Exhibit	Filing Date	Filed/ Furnished Herewith
31.1	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) under the Securities Exchange Act of 1934, as amended.					*
31.2	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) under the Securities Exchange Act of 1934, as amended.					*
32.1	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					**
32.2	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					**
101.INS	XBRL Instance Document.					*
101.SCH	XBRL Taxonomy Extension Schema Document.					*
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					*
101.DEF	XBRL Taxonomy Definition Linkbase Document.					*
101.LAB	XBRL Taxonomy Label Linkbase Document.					*
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					*

* Filed herewith
** Furnished herewith
# Management contract or compensation plan or arrangement
† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment pursuant to Rule 406 under the Securities Act of 1933, as amended

Certain agreements filed as exhibits to this Form 10-K contain representations and warranties that the parties thereto made to each other. These representations and warranties have been made solely for the benefit of the other parties to such agreements and may have been qualified by certain information that has been disclosed to the other parties to such agreements and that may not be reflected in such agreements. In addition, these representations and warranties may be intended as a way of allocating risks among parties if the statements contained therein prove to be incorrect, rather than as actual statements of fact. Accordingly, there can be no reliance on any such representations and warranties as characterizations of the actual state of facts. Moreover, information concerning the subject matter of any such representations and warranties may have changed since the date of such agreements.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

MeiraGTx Holdings plc (Registrant)

Date: March 26, 2019

By: /s/ Alexandria Forbes
Alexandria Forbes
President and Chief Executive Officer and Director (Principal Executive Officer)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of Registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Alexandria Forbes, Ph.D Alexandria Forbes, Ph.D	President and Chief Executive Officer and Director (Principal Executive Officer)	March 26, 2019
/s/ Katherine Breedis Katherine Breedis	Chief Financial Officer (Principal Financial and Accounting Officer)	March 26, 2019
/s/ Keith R. Harris, Ph.D. Keith R. Harris, Ph.D.	Chairman of the Board and Director	March 26, 2019
/s/ Stuart Naylor, Ph.D. Stuart Naylor, Ph.D.	Director	March 26, 2019
/s/ Ellen Hukkelhoven Ellen Hukkelhoven	Director	March 26, 2019
/s/ Martin Indyk Martin Indyk	Director	March 26, 2019
/s/ Arnold J. Levine, Ph.D. Arnold J. Levine, Ph.D.	Director	March 26, 2019
/s/ Joel S. Marcus Joel S. Marcus	Director	March 26, 2019
/s/ Neil Mendoza Neil Mendoza	Director	March 26, 2019
/s/ Gregory S. Moss Gregory S. Moss	Director	March 26, 2019
/s/ Thomas E. Shenk, Ph.D. Thomas E. Shenk, Ph.D.	Director	March 26, 2019

Exhibit 2.1

Execution Version

AGREEMENT AND PLAN OF MERGER

This Agreement and Plan of Merger (this "**Agreement**") is made and entered into as of October 5, 2018, by and among **MeiraGTx Holdings plc**, a company incorporated in the Cayman Islands (the "**Buyer**"), **VN Acquisition, Inc.**, a Delaware corporation and a wholly-owned subsidiary of the Buyer ("**Merger Sub 1**"), **VN Acquisition 2, Inc.**, a Delaware corporation and a wholly-owned subsidiary of the Buyer ("**Merger Sub 2**"), **Vector Neurosciences Inc.**, a Delaware corporation, the **Company Stockholders** named on the signature pages hereto and Stephen B. Kaplitt, as the representative of the Company Stockholders (in such capacity, the "**Company Stockholder Representative**" and, collectively with the Buyer, Merger Sub 1, Merger Sub 2, the Company and the Company Stockholders, the "**Parties**"). "**Company Stockholders**" means each stockholder of the Company as of immediately prior to the Merger 1 Effective Time.

RECITALS

WHEREAS, the respective Boards of Directors of the Buyer, Merger Sub 1, Merger Sub 2 and the Company have each determined that it is advisable and in the best interests of the Buyer, Merger Sub 1, Merger Sub 2 and the Company, respectively, and their respective shareholders or stockholders that the Company be acquired indirectly by the Buyer;

WHEREAS, the acquisition of the Company by the Buyer shall be effected through the following transactions: (1) a merger of Merger Sub 1 with and into the Company, with the Company surviving as a wholly owned subsidiary of the Buyer ("**Merger 1**") and (2) a merger of the Company with and into Merger Sub 2, with Merger Sub 2 surviving as a wholly owned subsidiary of the Buyer ("**Merger 2**" and, together with Merger 1, the "**Mergers**"); and

WHEREAS, for United States federal income tax purposes, the Parties intend that the Mergers qualify as a reorganization within the meaning of Section 368(a) of the Internal Revenue Code, and the Parties intend, by executing this Agreement, to adopt a plan of reorganization within the meaning of Treasury Regulations Sections 1.368-2(g) and 1.368-3(a).

NOW THEREFORE, in consideration of the premises, and the mutual representations, warranties, covenants and agreements contained herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged by each of the Parties, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE I
THE MERGERS

1.1 The Mergers. Upon the terms and subject to the conditions set forth in this Agreement, Merger Sub 1 shall merge with and into the Company at the Merger 1 Effective Time. From and after the Merger 1 Effective Time, the separate corporate existence of Merger Sub 1 shall cease and the Company shall continue as the surviving entity of Merger 1 ("**Intermediate Surviving Corporation**"). Promptly following the consummation of Merger 1, and upon the terms and subject to the conditions set forth in this Agreement, the Company shall merge with and into Merger Sub 2 at the Merger 2 Effective Time. From and after the Merger 2 Effective Time, the separate corporate existence of the Company shall cease and Merger Sub 2 shall continue as the surviving entity of Merger 2 ("**Surviving Corporation**"). The Mergers shall have the effects set forth in Section 259 of the Delaware General Corporation Law.

1.2 The Closing. The Closing shall take place at the offices of Latham & Watkins LLP, 885 Third Avenue, New York, NY 10022, commencing at 10:00 a.m., local time, on the Closing Date, or such other place as the Parties may agree prior to the Closing.

1.3 Actions at the Closing. At the Closing:

(a) the Company shall deliver to the Buyer, Merger Sub 1 and Merger Sub 2 the various certificates, instruments and documents referred to in Section 6.1;

(b) the Buyer, Merger Sub 1 and Merger Sub 2 shall deliver to the Company the various certificates, instruments and documents referred to in Section 6.2;

(c) each Company Stockholder shall deliver to the Buyer for cancellation a certificate or certificates or an affidavit of book entry positions representing all of such Company Stockholder's Company Shares;

(d) the Buyer shall cause the Certificate of Merger 1 to be filed with the Secretary of State of the State of Delaware;

(e) the Buyer shall cause the Certificate of Merger 2 to be filed with the Secretary of State of the State of Delaware;

(f) the Buyer shall issue to each Company Stockholder the portion of the Closing Shares (as defined below) allocable to such Company Stockholder, as set forth on the Allocation Schedule and deliver a certificate or evidence of a book-entry position with respect to such Closing Shares to such Company Stockholders; and

(g) the Buyer shall deliver a copy of a resolution approved by the board of directors of the Buyer, authorizing the issuance to each Company Stockholder of the Closing Shares and Holdback Shares, the securities to be issued pursuant to the Consulting Agreement and the securities issuable pursuant to the Milestone Payments. Upon determination of the number of Holdback Shares which such Company Stockholder may ultimately be entitled to

receive pursuant to Section 1.5(a)(ii), the Buyer shall issue to each Company Stockholder the portion of the Holdback Shares allocable to such Company Stockholder. "**Holdback Shares**" shall mean 22,500 Buyer Capital Shares. The Holdback Shares shall be a source for effecting the satisfaction and discharge of any indemnification obligation owed by any Company Stockholder to the Buyer under this Agreement, and the Buyer shall have the right to recover any Losses for which it is entitled to indemnification (1) under Section 7.1(a) of this Agreement, via the forfeiture pursuant to Section 7.4(a) of the right to have issued the Holdback Shares, and (2) under Section 7.1(b) of this Agreement, via the forfeiture pursuant to Section 7.4(a) of the right to have issued Holdback Shares allocable to the applicable Company Stockholder as set forth on the Allocation Schedule.

1.4 Additional Actions. The Surviving Corporation may, at any time after the either the Merger 1 Effective Time or the Merger 2 Effective Time, take any action, including executing and delivering any document or instrument, in the name and on behalf of the Company, Merger Sub 1 or Merger Sub 2, in order to consummate the transactions contemplated by this Agreement.

1.5 Conversion of Company Shares; Conversion of Merger Sub 1 Shares.

(a) Conversion of Company Shares.

(i) At the Merger 1 Effective Time, by virtue of Merger 1 and without any action on the part of any Party or the holder of any Company Shares, all Company Shares issued and outstanding as of immediately prior to the Merger 1 Effective Time and held by any Company Stockholder shall be converted into and represent the right to receive (A) the number of Closing Shares allocable to such Company Stockholder, as set forth on the Allocation Schedule, (B) the number of Holdback Shares allocable to such Company Stockholder, less any Holdback Shares forfeited by such Company Stockholder in accordance with this Agreement, as set forth on the Allocation Schedule, (C) any Milestone Payment due pursuant to Section 1.11; and (D) any Royalty payments due pursuant to Section 1.12, in each case in accordance with and subject to the terms and conditions of this Agreement (including any indemnification obligations hereunder) and the Certificate of Incorporation. "**Closing Shares**" shall mean 202,500 Buyer Capital Shares.

(ii) On the date that is 18 months after the Closing Date the Buyer shall issue to each Company Stockholder from the Holdback Shares the number of the Releasable Shares (rounded down to the nearest whole share) allocable to such Company Stockholder, as set forth on the Allocation Schedule and deliver a certificate or evidence of a book-entry position with respect to such Releasable Shares to such Company Stockholder; provided that if on or prior to such date, the Buyer has delivered any Claim Notice or Expected Claim Notice to the Company

Stockholder Representative containing a claim which has not been resolved in accordance with Section 7.2, the Buyer shall not be required to issue a number of Holdback Shares (rounded down to the nearest whole share) equal to (A) the amount of the contested portion of such unresolved claim or claims divided by (B) the Buyer Share Value, and shall instead issue to each Company Stockholder the number of Releasable Shares (rounded down to the nearest whole share) that are allocable to such Company Stockholder; provided, further, that upon the resolution of all such unresolved claims, Buyer shall promptly issue to each Company Stockholder the Holdback Shares (rounded down to the nearest whole share) remaining, if any, allocable to such Company Stockholder, less the amount of such Holdback Shares forfeited by such Company Stockholder in accordance with this Agreement, if any. "**Releasable Shares**" shall mean a number of Buyer Shares (rounded down to the nearest whole share) equal to the number of Holdback Shares, *minus* the number of Buyer Shares equal to (1) the amount of all Losses, if any, for which Buyer has theretofore become entitled to indemnification pursuant to Section 7.1 and that has resulted in the forfeiture of the right to be issued the Holdback Shares pursuant to Section 7.4(a) and (2) the amount of any unresolved claim reflected in a Claim Notice or Expected Claim Notice, *divided* by (y) the Buyer Share Value.

(b) Cancellation of Treasury Stock. Any Company Shares held in the Company's treasury immediately prior to the Merger 1 Effective Time and any Company Shares owned by the Buyer, Merger Sub 1 or Merger Sub 2 shall be cancelled and retired without payment of any consideration therefor.

(c) Capital Stock of Merger Sub 1. Each share of common stock, $0.001 par value per share, of Merger Sub 1 issued and outstanding immediately prior to the Merger 1 Effective Time shall be converted into and thereafter evidence one share of common stock, $0.001 par value per share, of the Intermediate Surviving Corporation.

(d) Capital Stock of the Intermediate Surviving Corporation. Each share of common stock, $0.001 par value per share, of the Intermediate Surviving Corporation issued and outstanding immediately prior to the Merger 2 Effective Time shall be converted into and thereafter evidence one share of common stock, $0.001 par value per share, of the Surviving Corporation.

1.6 Certificate of Incorporation and Bylaws; Directors and Officers.

(a) The Certificate of Incorporation of the Company in effect as of immediately prior to the Merger 1 Effective Time will be amended and restated in its entirety in the form of the certificate of incorporation of Merger Sub 1 in effect as of immediately prior to the Merger 1 Effective Time, and as so amended shall be the certificate of incorporation of the Intermediate Surviving Corporation until amended or repealed in accordance with the provisions

thereof, and applicable Law. The Certificate of Incorporation of the Intermediate Surviving Corporation in effect as of immediately prior to the Merger 2 Effective Time will be amended and restated in its entirety in the form of the certificate of incorporation of Merger Sub 2 in effect as of immediately prior to the Merger 2 Effective Time, and as so amended shall be the certificate of incorporation of the Surviving Corporation until amended or repealed in accordance with the provisions thereof, and applicable Law.

(b) The bylaws of Merger Sub 1 in effect as of immediately prior to the Merger 1 Effective Time shall be the bylaws of the Intermediate Surviving Corporation until amended or repealed in accordance with the provisions thereof, the certificate of incorporation of the Intermediate Surviving Corporation, and applicable Law. The bylaws of the Intermediate Surviving Corporation in effect as of immediately prior to the Merger 2 Effective Time will be amended and restated in their entirety in the form of the bylaws of Merger Sub 2 in effect as of immediately prior to the Merger 2 Effective Time, and as so amended shall be the bylaws of the Surviving Corporation until amended or repealed in accordance with the provisions thereof, the certificate of incorporation of the Surviving Corporation, and applicable Law.

(c) The directors of Merger Sub 1 immediately prior to the Merger 1 Effective Time shall be the initial directors of the Intermediate Surviving Corporation, and the officers of Merger Sub 1 immediately prior to the Merger 1 Effective Time shall be the initial officers of the Intermediate Surviving Corporation, each to hold office in accordance with the Certificate of Incorporation and bylaws of the Intermediate Surviving Corporation. The directors of Merger Sub 2 immediately prior to the Merger 2 Effective Time shall be the initial directors of the Surviving Corporation, and the officers of Merger Sub 2 immediately prior to the Merger 2 Effective Time shall be the initial officers of the Surviving Corporation, each to hold office in accordance with the Certificate of Incorporation and bylaws of the Surviving Corporation.

1.7 No Further Rights. From and after the Merger 1 Effective Time, no Company Shares shall be deemed to be outstanding, and any holders of certificates formerly representing Company Shares shall cease to have any rights with respect thereto, except as provided herein or by Law.

1.8 Closing of Transfer Books. At the Merger 1 Effective Time, the stock transfer books of the Company shall be closed and no transfer of Company Shares shall thereafter be made. If, after the Merger 1 Effective Time, valid and authentic certificates formerly representing Company Shares are presented to the Buyer or the Surviving Corporation, they shall be cancelled and exchanged for the applicable consideration payable, if any, in respect of such Company Shares pursuant to Section 1.5, subject to the provisions of Article VII.

1.9 Company Stockholder Representative.

(a) To facilitate the administration of the transactions contemplated by this Agreement, including the resolution of any disputes relating to claims for indemnification pursuant to Article VII and any other actions required or permitted to be taken by the Company Stockholder Representative under this Agreement, the Company Stockholders, by their execution

and delivery of this Agreement and/or the Written Consent, hereby (i) designate the Company Stockholder Representative as their representative, attorney-in-fact and agent and (ii) authorize the Company Stockholder Representative to give and receive all notices required to be given under this Agreement, to amend this Agreement, and to take any and all additional action as is contemplated to be taken by or on their behalf or by the Company Stockholder Representative by the terms of this Agreement. All such actions shall be deemed to be facts ascertainable outside this Agreement and shall be binding on the Company Stockholders.

(b) In the event that the Company Stockholder Representative named in the Preamble above, or any successor thereto, becomes unable to perform his responsibilities hereunder or resigns from such position, a majority in interest of the Company Stockholders, voting together as a single class based on the relative size of their respective ownership interests in the Company as of immediately prior to the Merger 1 Effective Time (as reflected on the Allocation Schedule) (the "**Stockholder Majority**") shall select another representative to fill the vacancy of the Company Stockholder Representative named in this Agreement, and such substituted representative shall be deemed to be the Company Stockholder Representative for all purposes of this Agreement and the documents delivered pursuant hereto; provided, however, that such substitution shall not be effective prior to the time that the Company Stockholders provide written notice to the Buyer of such substitution. The Stockholder Majority may, from time to time, upon written notice to the Company Stockholder Representative and the Buyer, remove the Company Stockholder Representative and appoint a new Company Stockholder Representative in the place thereof.

(c) All decisions and actions by the Company Stockholder Representative in connection with the transactions contemplated by this Agreement, including the resolution and disposition of any claims for indemnification pursuant to Article VII and any other actions required or permitted to be taken by the Company Stockholder Representative under this Agreement, shall be binding upon each Company Stockholder, and no Company Stockholder shall have the right to object, dissent, protest or otherwise contest the same.

(d) Any decision, act, consent, waiver or instruction of the Company Stockholder Representative in connection with this Agreement shall constitute a decision of all the Company Stockholders and shall be final, binding and conclusive upon each Company Stockholder, and the Buyer and the Surviving Corporation shall be entitled to rely conclusively on the decisions, acts, consents, waivers and instructions of the Company Stockholder Representative as to any determination relating to the transactions contemplated by this Agreement as being the decision, act, consent, waiver or instruction of every Company Stockholder, including the resolution and disposition of any claims for indemnification pursuant to Article VII and any other actions required or permitted to be taken by the Company Stockholder Representative under this Agreement, all of which shall be binding upon each Company Stockholder; no Person shall have any cause of action against the Buyer, the Surviving Corporation, or any of their respective directors, officers, employees, agents or Affiliates for any action taken by the Buyer in reliance upon any decision, act, consent, waiver or instruction of the Company Stockholder Representative; and the Buyer and the Surviving Corporation are each hereby relieved from any liability to any Person for any acts done by it in accordance with such decision, act, consent, waiver or instruction of the Company Stockholder Representative.

(e) All actions, decisions and instructions of the Company Stockholder Representative shall be conclusive and binding upon each Company Stockholder and none of the Buyer, the Surviving Corporation or any Company Stockholder shall have any cause of action against the Company Stockholder Representative for any action taken, decision made or instruction given by the Company Stockholder Representative under this Agreement, except for fraud or willful breach of this Agreement by the Company Stockholder Representative.

(f) The provisions of this Section 1.9 are independent and severable, are irrevocable and coupled with an interest, and shall be enforceable notwithstanding any rights or remedies that the Buyer or any Company Stockholder may have in connection with the transactions contemplated by this Agreement.

(g) The Company Stockholders shall severally (based on the relative size of their respective ownership interests in the Company as of immediately prior to the Merger 1 Effective Time (as reflected on the Allocation Schedule)) and not jointly indemnify the Company Stockholder Representative for any reasonable expense incurred without gross negligence or bad faith on the part of the Company Stockholder Representative and arising out of or in connection with the acceptance or administration of its duties hereunder ("**Representative Reimbursable Expenses**"). Without limiting the foregoing, the Company Stockholder Representative shall have the right to engage legal counsel and other professional advisers to assist it in the administration of the Company Stockholder Representative's duties hereunder, and any and all reasonable and documented fees and expenses of such counsel and advisers shall be deemed Representative Reimbursable Expenses, and the Company Stockholder Representative shall not be liable to any Company Stockholder for any action taken or omitted to be taken under this Agreement in good faith in accordance with the advice of such counsel or other professional advisors.

1.10 Withholding Rights. Notwithstanding anything to the contrary, each of the Buyer, the Intermediate Surviving Corporation and the Surviving Corporation will be entitled to deduct and withhold from any consideration otherwise payable or deliverable pursuant to this Agreement to any Person, such amounts as may be required to be deducted and withheld with respect to the making of such payment under the Internal Revenue Code or any other Law. To the extent that amounts are so deducted or withheld by the Buyer, the Intermediate Surviving Corporation or the Surviving Corporation, such deducted or withheld amounts will be treated for all purposes of this Agreement as having been paid on behalf of such Person in respect of which such deduction or withholding was made. Buyer, the Intermediate Surviving Corporation or the Surviving Corporation will promptly furnish in writing notice to such Person of the amounts deducted or withheld, and the identity and address of the Governmental Entity to which such amounts have been or will be transmitted.

1.11 Milestone Payments. At any time after the Merger 2 Effective Time, upon the achievement of any of the milestones set forth in the chart below under the heading "Milestone"

(each, a "**Milestone**"), the payment set forth opposite such Milestone in the chart below (each, a "**Milestone Payment**") shall become due and payable by the Buyer to the Company Stockholders. Each Milestone Payment will be paid a maximum of one time (and one time only) for each Milestone achieved.

Milestone	**Milestone Payment**
Regulatory Approval by the FDA of any GAD Product (the "U.S. Regulatory Milestone").	A number of Buyer Capital Shares equal to $[***] divided by the Buyer Share Value on the date of achievement of the U.S. Regulatory Milestone
Regulatory Approval by the EMA of any GAD Product (the "EMA Regulatory Milestone").	A number of Buyer Capital Shares equal to $[***] divided by the Buyer Share Value on the date of achievement of the EMA Regulatory Milestone
Regulatory Approval by the FDA or EMA of any IP-related Covered Product in the United States or Europe (as applicable based on the regulatory authority granting Regulatory Approval) as of the date that such Regulatory Approval is obtained (the "IP Assets Related Milestone")	A number of Buyer Capital Shares equal to $[***] divided by the Buyer Share Value on the date of achievement of the IP Assets Related Milestone

(a) The Buyer shall pay or cause to be paid to the Company Stockholder Representative for distribution to the Company Stockholders (in accordance with the proportions set forth in the Allocation Schedule) within thirty (30) days following the achievement of the U.S. Regulatory Milestone, the EMA Regulatory Milestone or the IP Assets Related Milestone, the corresponding Milestone Payments. For clarity, the Milestone Payment related to the IP Assets Related Milestone shall only be paid once based on the first Regulatory Approval for a given IP-related Covered Product, and no additional Milestone Payments shall be due or payable in respect of any further Regulatory Approvals of any IP-related Covered Products.

(b) The right of the Company Stockholders to receive any Milestone Payment or Royalty Payment: (i) does not give the Company Stockholder Representative or the Company Stockholders dividend rights, voting rights, liquidation rights, preemptive rights or other rights of holders of capital stock of the Buyer; (ii) shall not be evidenced by a certificate or other instrument; (iii) shall not be assignable or otherwise transferable by the Company Stockholder Representative or Company Stockholders except to and among the Company Stockholders and subsequently shall not be assignable except by will, upon death or by operation of the laws of descent and distribution, or to a trust formed by a Company Stockholder for the sole benefit of immediate family members; (iv) shall not accrue or pay interest on any portion thereof; and

(v) does not represent any right other than the right to receive the consideration set forth in this Section 1.11. Any attempted transfer of the right to any Milestone Payment by any holder thereof (other than as specifically permitted by the immediately preceding sentence) shall be null and void.

(c) The Company Stockholders shall not be subject to any holding or standstill restrictions, or any other restrictions on resale, in respect of any Buyer Capital Shares received as Milestone Payments, except as provided in Sections 3.6 and 3.7 or for such restrictions as may be imposed by Law.

1.12 Net Sales Royalty Payments.

(a) Subject to Section 1.12(c), after the Merger 2 Effective Time and for the duration of the Royalty Term, the Buyer shall make Royalty payments to the Company Stockholders (in accordance with the proportions set forth in the Allocation Schedule) on a fiscal quarterly basis in an amount equal to [***] of aggregate, worldwide Net Sales of such GAD Products during the Royalty Term payable in Buyer Capital Shares based on the Buyer Share Value on the last day of each such fiscal quarter.

(b) Within thirty (30) days after the end of the first calendar quarter during which the First Commercial Sale of a GAD Product occurs, and within thirty (30) days after the end of each fiscal quarter thereafter during the Royalty Term, the Buyer shall deliver to the Company Stockholders, together with the applicable Royalty payment due (payable in Buyer Capital Shares in accordance with the proportions set forth in the Allocation Schedule), a written report of the aggregate, worldwide Net Sales of GAD Products for such fiscal quarter. In addition, and for clarification purposes only, the Buyer shall make Royalty payments on all Net Sales received during the Royalty Term, including those for which the Buyer receives payment after the Royalty Term has expired.

(c) If the Buyer has licensed rights that are necessary or reasonably useful to develop, manufacture or commercialize the GAD Products from one or more third party(ies) then the Buyer may deduct from the Royalties it would otherwise owe to the Company Stockholders pursuant to Section 1.12(a) for the GAD Products an amount equal to [***] of all amounts aggregate payable to such third party(ies) in consideration for such licensed rights, up to a maximum aggregate deduction of [***] of the Royalties otherwise due to the Company Stockholders on a fiscal quarterly basis pursuant to Section 1.12(a) for such GAD Products (the "**Capped Percentage**"). Any amounts in excess of the Capped Percentage in a given fiscal quarter will accrue and be deductible against Royalties otherwise due to the Company Stockholders pursuant to Section 1.12(a) for such GAD Products in subsequent quarters, until the accrued amounts are fully deducted (but in all events subject to the Capped Percentage). For clarity, the quarterly accruals of any amounts in excess of the Capped Percentage may be carried over from year to year, until the accrued amounts are fully deducted (but in all events subject to the Capped Percentage). The Buyer may, in its sole discretion, obtain any licenses or other rights from third parties as the Buyer deems reasonably necessary to make, use, sell, offer for sale, import and otherwise commercialize any GAD Products. Notwithstanding the foregoing, if

the Buyer has licensed rights from Medtronic that are necessary or reasonably useful to develop, manufacture or commercialize the GAD Products then the Buyer may deduct from the Royalties it would otherwise owe to the Company Stockholders pursuant to Section 1.12(a) for the GAD Products an amount equal to [***] of all amounts aggregate payable to Medtronic in consideration for such license rights, up to an additional [***] such that the Capped Percentage may be up to [***].

1.13 Tax Consequences. It is intended by the Parties hereto that, for United States federal income tax purposes the Mergers shall constitute a reorganization within the meaning of Section 368(a) of the Internal Revenue Code. The Parties hereto adopt this Agreement as a plan of reorganization within the meaning of Treasury Regulations Sections 1.368-2(g) and 1.368-3(a).

ARTICLE II
REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company represents and warrants to the Buyer, Merger Sub 1 and Merger Sub 2 that, except as set forth in the disclosure schedule delivered to the Buyer on the date of this Agreement (the "**Disclosure Schedule**"), the statements contained in this Article II are true and correct. The Disclosure Schedule shall be arranged in Sections and paragraphs corresponding to the numbered and lettered Sections and paragraphs contained in this Article II. The disclosures in any Section or paragraph of the Disclosure Schedule shall qualify only (a) the corresponding Section or paragraph in this Article II or Article III and (b) other Sections or paragraphs in this Article II or Article III to the extent that it is reasonably apparent from a reading of the disclosure that such disclosure also qualifies or applies to such other Section or paragraph.

2.1 Organization, Qualification and Corporate Power. The Company is a corporation duly organized, validly existing and in corporate and tax good standing under the Laws of the State of Delaware. The Company is duly qualified to conduct business and is in corporate and tax good standing under the Laws of each jurisdiction listed in Section 2.1 of the Disclosure Schedule, which jurisdictions constitute the only jurisdictions in which the nature of the Company's businesses or the ownership or leasing of its properties requires such qualification, except for such failures to be so qualified or in good standing that have not had, and would not reasonably be expected to have in the future, a Company Material Adverse Effect. The Company has all requisite corporate power and authority to carry on the businesses in which it is engaged and to own and use the properties owned and used by it. The Company has furnished to the Buyer complete and accurate copies of the Certificate of Incorporation. The Company is not in default under or in violation of any provision of the Certificate of Incorporation. The Company has not adopted bylaws.

2.2 Capitalization.

(a) The authorized capital stock of the Company consists of 5,000 Company Shares, par value $0.0000100000 per share, of which 100 shares are issued and outstanding.

(b) Section 2.2(b) of the Disclosure Schedule sets forth a complete and accurate list of the holders of capital stock of the Company, showing the number of shares of capital stock, and the class or series of such shares, held by each stockholder. Section 2.2(b) of the Disclosure Schedule also indicates all outstanding Company Shares, if any, that constitute restricted stock or that are otherwise subject to a repurchase or redemption right, indicating the name of the applicable stockholder, the vesting schedule (including any acceleration provisions with respect thereto), and the repurchase price payable by the Company. All of the issued and outstanding shares of capital stock of the Company have been duly authorized and validly issued and are fully paid and nonassessable. All of the issued and outstanding shares of capital stock of the Company have been offered, issued and sold by the Company in compliance with all applicable federal and state securities Laws.

(c) No subscription, warrant, option, convertible security or other right (contingent or otherwise) to purchase or acquire any shares of capital stock of the Company is authorized or outstanding. The Company has no obligation (contingent or otherwise) to issue any subscription, warrant, option, convertible security or other such right, or to issue or distribute to holders of any shares of its capital stock any evidences of Indebtedness or assets of the Company. The Company has no obligation (contingent or otherwise) to purchase, redeem or otherwise acquire any shares of its capital stock or any interest therein or to pay any dividend or to make any other distribution in respect thereof. There are no outstanding or authorized stock appreciation, phantom stock or similar rights with respect to the Company.

(d) Section 2.2(d)of the Disclosure Schedule lists each agreement, written or oral, between the Company and any holder of its securities, or among any holders of its securities, relating to the sale or transfer (including agreements relating to rights of first refusal, co-sale rights or "drag-along" rights), registration under the Securities Act, or voting, of the capital stock of the Company.

(e) There is no claim pending, and to the knowledge of the Company, no claim threatened, against the Company by any Person that seeks to assert: (i) ownership or rights to ownership of any shares of Company capital stock; (ii) any rights of a stockholder, including any option, preemptive rights or rights to notice or to vote; (iii) any rights under the Certificate of Incorporation of the Company, as amended or restated; or (iv) any claim that his, her or its shares have been wrongfully repurchased by the Company.

2.3 Authorization of Transaction. The Company has all requisite corporate power and authority to execute and deliver this Agreement and to perform its obligations hereunder. The execution and delivery by the Company of this Agreement and, subject to obtaining the unanimous approval of the stockholders of the Company (the "**Requisite Stockholder Approval**") in the form of the Written Consent, the consummation by the Company of the transactions contemplated hereby have each been duly and validly authorized by all necessary corporate action on the part of the Company. Without limiting the generality of the foregoing, the Board of Directors of the Company, by the unanimous vote of all directors (i) determined that the Mergers are advisable, fair and in the best interests of the Company and its stockholders, (ii) adopted this Agreement in accordance with the provisions of the Delaware General

Corporation Law, and (iii) directed that this Agreement and the Mergers be submitted to the stockholders of the Company for their adoption and approval and resolved to recommend that the stockholders of the Company vote in favor of the adoption of this Agreement and the approval of the Mergers. This Agreement has been duly and validly executed and delivered by the Company and, assuming due authorization, execution and delivery by the other Parties, constitutes a valid and binding obligation of the Company, enforceable against the Company in accordance with its terms, subject to bankruptcy, insolvency, fraudulent transfer, reorganization, moratorium and similar Laws of general applicability relating to or affecting creditors' rights and to general equity principles (the "**Bankruptcy and Equity Exception**").

2.4 Noncontravention. Subject to the filing of the Certificate of Merger 1 and the Certificate of Merger 2 as required by the Delaware General Corporation Law, none of the execution and delivery by the Company of this Agreement, the performance by the Company of any of its obligations hereunder or the consummation by the Company of the transactions contemplated hereby, does or will (a) conflict with or violate any provision of the Certificate of Incorporation of the Company, (b) require on the part of the Company any notice to or filing with, or any permit, authorization, consent or approval of, any Governmental Entity, (c) conflict with, result in a breach of, constitute (with or without due notice or lapse of time or both) a default under, result in the acceleration of obligations or loss of any right or benefit under, create in any party the right to terminate, modify or cancel, or require any notice, consent or waiver under, any material contract or other instrument to which the Company is a party or by which the Company is bound or to which any of the Company's assets is subject, (d) result in the imposition of any Security Interest upon any assets of the Company or (e) violate any order, writ, injunction, decree, statute, rule or regulation applicable to the Company or any of its properties or assets.

2.5 Subsidiaries. The Company does not have, nor has it ever had, any Subsidiaries. The Company does not control, nor has it ever controlled, directly or indirectly, nor does the Company have, nor has it ever had, any direct or indirect equity participation or similar interest in any Person.

2.6 Absence of Certain Changes. Except as set forth on Section 2.6 of the Disclosure Schedule, since December 31, 2015, the Company has conducted its business in the Ordinary Course of Business and there has occurred no event or development which, individually or in the aggregate, has had, or would reasonably be expected to have in the future, a Company Material Adverse Effect.

2.7 Liabilities. Section 2.7 of the Disclosure Schedule sets forth, as of December 31, 2017 and as of the date hereof, all liabilities and obligations of the Company required by GAAP to be set forth on a balance sheet of the Company (the "**Liability Statement**"). Except for the liabilities (a) set forth on the Liability Statement or (b) expressly disclosed in the Disclosure Schedule, (i) the Company has no liabilities or obligations of any kind greater than or equal to $10,000, individually or in the aggregate and (ii) there is no outstanding Company Debt.

2.8 Tax Matters.

(a) Except as set forth in Section 2.8(a) of the Disclosure Schedule, the Company has properly filed on a timely basis all income and other material Tax Returns that it was required to file, and all such Tax Returns are true, correct and complete in all material respects and were prepared in material compliance with all applicable Laws. Except as set forth in Section 2.8(a) of the Disclosure Schedule, the Company has paid on a timely basis all material Taxes, whether or not shown on any Tax Return, that were due and payable. As of the date of the latest Liability Statement, the unpaid Taxes of the Company did not exceed the reserve for Tax liability (excluding any reserve for deferred Taxes established to reflect timing differences between book and Tax income) set forth or included on the Liability Statement, and since the date of the latest Liability Statement, the Company has not incurred any liability for Taxes outside the ordinary course of business or inconsistent with past practice.

(b) All material Taxes that the Company is or was required by Law to withhold or collect have been duly withheld or collected and, to the extent required, have been properly paid to the appropriate Governmental Entity, and the Company has complied with all information reporting and backup withholding requirements, including the maintenance of required records with respect thereto, in connection with amounts paid to any employee, independent contractor, creditor, or other third party.

(c) The Company is not, and has never been, a member of an affiliated group with which it has filed (or been required to file) consolidated, combined, unitary or similar Tax Returns. The Company (i) does not have any liability under Treasury Regulation Section 1.1502-6 (or any comparable or similar provision of federal, state, local or foreign Law), as a transferee or successor, pursuant to any contractual obligation, or otherwise for any Taxes of any Person other than the Company, and (ii) is not a party to or bound by any Tax indemnity, Tax sharing, Tax allocation or similar agreement.

(d) The Company has delivered to the Buyer (i) complete and correct copies of all Tax Returns filed by the Company relating to Taxes for all taxable periods for which the applicable statute of limitations has not yet expired, (ii) complete and correct copies of all private letter rulings, revenue agent reports, information document requests, notices of proposed deficiencies, deficiency notices, protests, petitions, closing agreements, settlement agreements, pending ruling requests and any similar documents submitted by, received by, or agreed to by or on behalf of the Company relating to Taxes for all taxable periods for which the statute of limitations has not yet expired, if any, and (iii) complete and correct copies of all agreements, rulings, settlements or other Tax documents with or from any Governmental Entity relating to Tax incentives of the Company, if any.

(e) No examination or audit or other action of or relating to any Tax Return of the Company by any Governmental Entity is currently in progress or has been threatened in writing or, to the knowledge of the Company, in any other manner. No deficiencies for Taxes of the Company have been claimed, proposed or assessed, in each case in writing or, to the knowledge of the Company, in any other manner, by any Governmental Entity for any taxable period for which the period of assessment remains open. The Company has not been informed in writing or, to the knowledge of the Company, in any other manner, by any jurisdiction in which

the Company did not file a Tax Return that the jurisdiction believes that the Company was required to file any Tax Return that was not filed or is subject to Tax in such jurisdiction. The Company has not (i) waived any statute of limitations with respect to Taxes or agreed to extend the period for assessment or collection of any Taxes, which waiver or extension is still in effect, (ii) requested any extension of time within which to file any Tax Return, which Tax Return has not yet been filed, or (iii) executed or filed any power of attorney with any taxing authority, which is still in effect.

(f) The Company will not be required to include any material item of income in, or exclude any material item of deduction from, taxable income for any period (or any portion thereof) ending after the Closing Date as a result of (i) any adjustments under Section 481 of the Internal Revenue Code (or any similar adjustments under any provision of the Internal Revenue Code or the corresponding foreign, state or local Tax Law), (ii) installment sale or open transaction disposition made on or prior to the Closing Date, (iii) prepaid amount received on or prior to the Closing Date, or (iv) any election made pursuant to Section 108(i) of the Internal Revenue Code on or prior to the Closing Date.

(g) The Company has not been a United States real property holding corporation within the meaning of Section 897(c)(2) of the Internal Revenue Code during the applicable period specified in Section 897(c)(l)(A)(ii) of the Internal Revenue Code.

(h) The Company has not distributed to its shareholders or security holders stock or securities of a controlled corporation, nor has stock or securities of the Company been distributed, in a transaction to which Section 355 of the Internal Revenue Code applies.

(i) There are no liens or other encumbrances with respect to Taxes upon any of the assets or properties of the Company, other than with respect to Taxes not yet due and payable.

(j) The Company has not engaged in a “reportable transaction” as set forth in Treasury Regulation Section 1.6011-4(b) or a “listed transaction” as set forth in Treasury Regulation Section 301.6111-2(b)(2) or any analogous provision of state or local Law. The Company has disclosed on its federal income Tax Returns all positions taken therein that could give rise to a substantial understatement of federal income Tax within the meaning of Section 6662 of the Internal Revenue Code.

(k) The Company is and has been since its formation properly treated as a corporation for U.S. federal income tax purposes.

(l) The Company currently conducts a business. Such business is the Company’s “historic business” within the meaning of Treasury Regulations Section 1.368-1(d), and no assets of the Company have been sold, transferred, or otherwise disposed of that would prevent the Surviving Corporation from continuing the “historic business” of the Company or from using a “significant portion” of the Company’s “historic business assets” in a business following the Mergers, as such terms are used in Treasury Regulations Section 1.368-1(d).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(m) The Company is not an investment company within the meaning of Section 368(a)(2)(F)(iii) and (iv) of the Internal Revenue Code.

(n) The Company is not under the jurisdiction of a court in a Title 11 or similar case within the meaning of Section 368(a)(3)(A) of the Internal Revenue Code.

(o) The Company has not redeemed, purchased or otherwise acquired, or made any distributions with respect to, any of the Company's capital stock prior to and in contemplation of the Mergers, or otherwise as part of a plan of which the Mergers are a part.

(p) Neither the Company nor, to the knowledge of the Company, any Affiliate of the Company, has taken or agreed to take any action that would prevent the Mergers, from qualifying as a reorganization within the meaning of Section 368(a)(1)(A) of the Internal Revenue Code, and to the knowledge of the Company, there is no agreement, plan or other circumstance to which the Company is a party or of which the Company has knowledge that is not expressly contemplated by this Agreement and that would prevent the Mergers from qualifying as a reorganization within the meaning of Section 368(a)(1)(A) of the Internal Revenue Code.

2.9 Assets and Properties. Other than the Neurologix Assets (addressed below and in Section 2.12), the Company is the true and lawful owner, and has good title to, all of the tangible assets owned or purported to be owned by the Company, free and clear of all Security Interests. The Company is the true and lawful owner of, and has good title to, all of the Neurologix Assets. To the knowledge of the Company, no Person has asserted any Security Interest in the Neurologix Assets, other than Security Interests that may have been asserted prior or pursuant to the Neurologix Chapter 7 bankruptcy. Each such tangible asset is free from defects, has been maintained in accordance with normal industry practice, is in good operating condition and repair (subject to normal wear and tear) and is suitable for the purposes for which it presently is used. The Company does not own, and has never owned, any Real Property and does not lease, and has never leased, any Real Property. The Company has not sold, assigned, conveyed, transferred, pledged or otherwise encumbered any of its assets, including the Neurologix Assets.

2.10 Intellectual Property. Company Registrations. (a) Section 2.10(a) of the Disclosure Schedule lists all Company Registrations, in each case enumerating specifically the applicable filing or registration number, title, jurisdiction in which filing was made or from which registration issued, date of filing or issuance and names of all current applicant(s) and registered owners(s), as applicable, but excluding any Company Registrations in respect of any Neurologix Assets. All assignments of Company Registrations to the Company are valid and enforceable and have been properly executed and recorded. To the knowledge of the Company, all Company Registrations contain patentable subject matter and, except with respect to the Neurologix Assets, all issuance, renewal, maintenance and other payments that are or have become due with respect Company Registrations have been timely paid by or on behalf of the Company. Section 2.10(a) of the Disclosure Schedule lists all patents and patent applications owned or co-owned by Neurologix to the Company's knowledge, pursuant to (i) a basic online search of the U.S. Patent & Trademark Office website (www.uspto.gov), and (ii) the Neurologix

Bankruptcy Schedules. The Company has not made any payments with respect to the Neurologix Assets, and the Company has not received any notices or billing statements in respect of renewal, maintenance or other payments that are or may have become due with respect to the Neurologix Assets. Except as disclosed in Section 2.10(a) of the Disclosure Schedule, the Company has no knowledge of any renewal, maintenance or other payments that are or may have become due with respect to the Neurologix Assets.

(b) Prosecution Matters. Except with respect to the Neurologix Assets, there are no inventorship challenges, *inter partes* review, opposition or nullity proceedings or interferences declared, commenced or provoked, or to the knowledge of the Company threatened, with respect to any Company Registrations. The Company has complied with its duty of candor and disclosure to the United States Patent and Trademark Office and any relevant foreign patent office with respect to all Intellectual Property applications filed by or on behalf of the Company and has made no material misrepresentation in such applications. The Company has no knowledge of any information that would preclude the Company from having clear title to the Company Registrations. The Company has no knowledge of any inventorship challenges, *inter partes* review, opposition or nullity proceedings or interferences declared, commenced or provoked, or to the knowledge of the Company threatened, with respect to any Neurologix Assets.

(c) Ownership; Sufficiency. Except for the Neurologix Assets, each item of Company Intellectual Property will be owned or available for use by the Surviving Corporation immediately following the Closing without any impairment and on the same terms and conditions as it was available to the Company immediately prior to the Closing. To the knowledge of the Company, each item of Company Intellectual Property that is or was part of the Neurologix Assets will be owned or available for use by the Surviving Corporation immediately following the Closing without any impairment and on the same terms and conditions as it was available to the Company immediately prior to the Closing. Except for the Neurologix Assets, the Company is the sole and exclusive owner of all Company Owned Intellectual Property, free and clear of any Security Interests, except for any joint owners of the Company Owned Intellectual Property, all of whom are listed in Section 2.10(c) of the Disclosure Schedule. Except for the Neurologix Assets, the Company has a valid license to use the Company Licensed Intellectual Property to conduct the activities of the Company in the manner currently conducted, subject only to the terms of the agreements listed or required to be listed in Section 2.10(h) of the Disclosure Schedule. Except for the Neurologix Assets, the Company Intellectual Property constitutes all Intellectual Property necessary for the Company to conduct the activities of the Company in the manner currently conducted by the Company.

(d) Protection Measures. The Company has taken reasonable measures to protect the proprietary nature of each item of Company Owned Intellectual Property which is material to the Company, and to maintain in confidence all trade secrets and confidential information comprising a part of the Company Owned Intellectual Property. No confidential information or trade secrets have been disclosed by the Company to any Person except pursuant to valid and appropriate non-disclosure and/or license agreements that have not been breached, or disclosures otherwise protected by attorney-client privilege and a legal duty of confidentiality

owed by the recipient of such confidential information or trade secrets to the Company. The Company has complied in all material respects with all applicable contractual and legal requirements pertaining to information privacy and security. No complaint relating to an improper use or disclosure of, or a breach in the security of, any such information has been made in writing to the Company or, to the knowledge of the Company, threatened against the Company. To the knowledge of the Company, there has been no unauthorized disclosure of any third party proprietary or confidential information in the possession, custody or control of the Company.

(e) Infringement by Company. Except as listed on Section 2.10(e) of the Disclosure Schedule, no past or current activity of the Company (excluding activities of Neurologix) infringes or violates (or has infringed or violated), or constitutes (or constituted) a misappropriation of, any Intellectual Property rights of any third party. To the knowledge of the Company, no past or current activity of Neurologix infringes or violates (or has infringed or violated), or constitutes (or constituted) a misappropriation of, any Intellectual Property rights of any third party. For clarification purposes only, the foregoing representations and warranties in this subsection (e) expressly exclude any activities by the Surviving Corporation at or after the Merger 1 Effective Time. Section 2.10(e) of the Disclosure Schedule lists any written complaint, claim or notice, or threat of any of the foregoing (including any notification that a license under any patent is or may be required), received by the Company alleging any such infringement, violation or misappropriation by the Company and any request or demand for indemnification or defense received by the Company from any third party; and the Company has provided to the Buyer copies of all such complaints, claims, notices, requests, demands or threats in its possession relating to any alleged or potential infringement, violation or misappropriation.

(f) Infringement of Company Rights. To the knowledge of the Company, no Person is infringing, violating or misappropriating (or has infringed, violated or misappropriated) any of the Company Owned Intellectual Property or any Company Licensed Intellectual Property exclusively licensed to the Company by any Person. The Company has provided to the Buyer copies of all correspondence in its possession concerning the infringement, violation or misappropriation of any Company Intellectual Property, except for the Neurologix Assets. The Company has not received any correspondence concerning the infringement, violation or misappropriation of any Neurologix Assets, and the Company has no knowledge of any such correspondence.

(g) Outbound IP Agreements. Section 2.10(g) of the Disclosure Schedule identifies each license, covenant or other agreement (including any options to license) pursuant to which the Company has assigned, transferred, licensed, distributed or otherwise granted any right or access to any Person, or covenanted not to assert any right, with respect to any past, existing or future Company Intellectual Property. Except as disclosed in Section 2.10(g) of the Disclosure Schedule, the Company has no knowledge of the existence, status or enforceability of any license, covenant or other agreement that may have been entered into by Neurologix (including any options to license) pursuant to which Neurologix may have assigned, transferred, licensed, distributed or otherwise granted any right or access to any Person, or covenanted not to assert any right, with respect to any past, existing or future Neurologix Assets. The Company

has not agreed to indemnify any Person against any infringement, violation or misappropriation of any Intellectual Property rights, except as set forth in the Contracts listed in Section 2.11(a) of the Disclosure Schedule. The Company has no knowledge of the status or enforceability of any indemnification agreement that may have been entered into by Neurologix. The Company is not a member of or party to any patent pool, industry standards body, trade association or other organization pursuant to the rules of which it is obligated to license any existing or future Intellectual Property to any Person.

(h) Inbound IP Agreements. Section 2.10(h) of the Disclosure Schedule identifies (i) each item of Company Licensed Intellectual Property (but specifically excluding generally commercially available off the shelf software that has not been modified by or for the Company and is licensed to the Company for a one-time or annual fee of $10,000 or less) and the license or agreement pursuant to which the Company licenses it or has obtained an option to license it and (ii) each agreement, contract, assignment or other instrument pursuant to which the Company has obtained any joint or sole ownership interest in or to each item of Company Owned Intellectual Property; but in all cases except for the Neurologix Assets. Except as disclosed in Section 2.10(g) of the Disclosure Schedule, the Company has no knowledge of in respect of, the existence, status or enforceability of any license or agreement that may have been entered into by Neurologix.

(i) IP Assignments. Except as set forth in Section 2.10(i) of the Disclosure Schedule, each Person who has contributed to the development of any Company Owned Intellectual Property (but excluding the Neurologix Assets) has executed a valid and binding written agreement expressly assigning to the Company all right, title and interest in any inventions and works of authorship, whether or not patentable, invented, created, developed, conceived and/or reduced to practice during the term of such Person's engagement with the Company, and all Intellectual Property rights therein.

(j) Support and Funding. Except as set forth in Section 2.10(j) of the Disclosure Schedule, the Company has not received any support, funding, resources or assistance from any Governmental Entity, university, military, educational institution or research center and no such Person has any claim of right to, ownership of or other Security Interest on any Company Owned Intellectual Property.

(k) Effect on Buyer. Consummation of the transactions contemplated by this Agreement will not result in (i) the creation of any Security Interest on or license to any Intellectual Property that is owned by or licensed to the Buyer or any of its Affiliates prior to the Closing, (ii) Buyer or any of its Affiliates being bound by or subject to any non-compete or licensing obligation, covenant not to sue, or other restriction on the operation or scope of its business, which Buyer or its Affiliates were not bound by or subject to prior to the Closing, or (iii) Buyer or any of its Affiliates, or the Surviving Corporation, being obligated to pay any royalties, honoraria, fees or other similar payments to any Person (other than Taxes payable pursuant to Section 5.5) in excess of those payable by the Company prior to the Closing.

2.11 Contracts.

(a) Section 2.11(a) of the Disclosure Schedule lists all agreements (whether written or oral) currently in effect (either in whole or in part, including agreements with ongoing post-termination "tails" and ongoing post-termination obligations) to which the Company is a party, but excluding the Neurologix Assets and any agreements to which Neurologix is or was a party. The Company makes no representations or warranties as to whether or not any agreements related to the Neurologix Assets or to which Neurologix is a party are currently in force and effect. Except as described in Section 2.11(a) of the Disclosure Schedule, the Company is not a party to:

(i) any agreement (or group of related agreements) for the lease of personal property from or to third parties;

(ii) any agreement (or group of related agreements) in which the Company has granted manufacturing rights, "most favored nation" pricing provisions or marketing or distribution rights relating to any products or territory or has agreed to purchase a minimum quantity of goods or services or has agreed to purchase goods or services exclusively from a certain party;

(iii) any agreement concerning the establishment or operation of a partnership, joint venture or limited liability company;

(iv) any agreement (or group of related agreements) under which it has created, incurred, assumed or guaranteed (or may create, incur, assume or guarantee) Indebtedness;

(v) any agreement for the disposition of any significant portion of the assets of the Company or any agreement for the acquisition of the assets or business of any other entity;

(vi) any employment, independent contractor or consulting agreement;

(vii) any severance, retention, change in control or other similar agreement;

(viii) any agreement with a third party concerning the research, development or commercialization of Intellectual Property, confidentiality, noncompetition and/or nonsolicitation;

(ix) any agreement with any professional employer organization;

(x) any material agreement involving any current or former officer, director or stockholder of the Company or an Affiliate thereof;

(xi) any agreement which contains any provisions requiring the Company to indemnify any other party;

(xii) any agreement that purports to bind or otherwise could bind the Buyer or any Affiliate of the Buyer (other than the Company) in any way;

(xiii) any agreement under which the Company is restricted or prohibited from selling, licensing or otherwise distributing any of its technology or products, or providing services to, customers or potential customers, or otherwise engaging in any aspect of its business, in any geographic area, during any period of time or with any Person, or any segment of the market or line of business;

(xiv) any Government Contract; or

(xv) any other agreement (or group of related agreements) either involving more than $10,000 or not entered into in the Ordinary Course of Business.

(b) The Company has delivered to the Buyer a complete and accurate copy of each agreement (if any) listed or required to be listed in Section 2.10 of the Disclosure Schedule or Section 2.11(a) of the Disclosure Schedule. With respect to each agreement so listed or required to be listed: (i) the agreement is legal, valid, binding and enforceable against the Company, and to the knowledge of the Company, against each other party thereto, and is in full force and effect, subject to the Bankruptcy and Equity Exception; and (ii) neither the Company nor, to the knowledge of the Company, any other party, is in breach or violation of, or default under, any such agreement, and no event has occurred, is pending or, to the knowledge of the Company, is threatened, which, after the giving of notice, with lapse of time, or otherwise, would constitute a material breach or default by the Company or, to the knowledge of the Company, any other party under such agreement.

2.12 Neurologix, Inc.(a) The Transfer Statements and the Neurologix Bankruptcy Schedules attached at Annex 2.12 of the Disclosure Schedule list all Neurologix Assets acquired by the Company pursuant to the Foreclosure Action. To the knowledge of the Company: (i) the assets listed on Annex 2.12 of the Disclosure Schedule represent all assets that the Neurologix bankruptcy trustee offered for sale by auction in connection with the Neurologix Chapter 7 bankruptcy; and (ii) prior to the sale of assets in connection with its bankruptcy, Neurologix was the true and lawful owner, and had good title to, all the tangible assets owned and acquired or purported to be owned and acquired by the Company pursuant to the Foreclosure Action, free and clear of all Security Interests except such Security Interests existing at the time of filing of the Neurologix Chapter 7 bankruptcy petition.

(b) Attached at Annex 2.12 of the Disclosure Schedule are true and correct copies of: (i) the Transfer Statement, dated April 14, 2016, of the Company in respect of a portion of the Neurologix Assets; (ii) the Transfer Statement, dated July 13, 2016, of the Company in respect of a portion of the Neurologix Assets; and (iii) the Neurologix Bankruptcy Schedules.

2.13 Powers of Attorney. There are no outstanding powers of attorney executed on behalf of the Company.

2.14 Litigation. There is no Legal Proceeding which is pending or has been threatened in writing against the Company or any of its current or former officers, directors, employees or consultants (in their respective capacities as such). There are no judgments, orders or decrees outstanding against the Company or any of its current or former officers, directors, employees or consultants (in their respective capacities as such). There is no Legal Proceeding initiated by the Company pending, or which the Company has commenced preparations to initiate, against any other Person.

2.15 Employees.

(a) Other than the Company Stockholders, there are no current or former officers and directors of the Company. Other than the Company's attorneys and accountants, no person has been paid any compensation by the Company and no person is entitled to any compensation of any kind from the Company. The Company has no, and has never had any, employees or any obligations or liabilities to, or in respect of, any employees. The Company has never been obligated to pay any payroll Taxes. The Company is not liable for any payment to any trust or other fund or to any Governmental Entity, with respect to unemployment compensation benefits, social security or other benefits or obligations for employees (other than routine payments to be made in the normal course of business and consistently with past practice). The Company is, and at all times has been, in compliance with all applicable Laws and regulations respecting labor, employment, hiring and termination, fair employment practices, terms and conditions of employment, worker classification (including the proper classification of workers as independent contractors and consultants), workers' compensation, occupational health and safety and wage and hour Laws, and has complied with all employment agreements respecting current and past employees of the Company and has complied with all independent contractor and consulting agreements respecting current and former independent contractors and consultants of the Company. The Company is, and at all times has been, in compliance with the requirements of the Immigration Reform and Control Act of 1986, respecting current and past employees of the Company. The Company is not a party to any labor agreement with any labor organization, union, group or association and there are no Company employee unions (nor any other similar labor or employee organizations) under local statutes, custom or practice. There are no controversies pending or, to the knowledge of the Company, threatened, between the Company and any other person related to employment or consulting matters.

(b) The Company is not a party to, and has no commitments or obligations in respect of, any change in control, severance, stay bonuses or other bonuses or similar benefits with respect to the transactions contemplated.

(c) All persons who have performed services for the Company while classified as independent contractors have satisfied all Laws to be so classified, and the Company has fully and accurately reported their compensation on IRS Forms 1099 or other applicable Tax forms for independent contractors when required to do so. The Company has

provided to the Buyer a true, correct and complete list of all of its current consultants, advisory board members and independent contractors; copies of all consultant and independent contractor agreements have previously been delivered to the Buyer.

2.16 Employee Benefits.

(a) The Company does not sponsor, maintain, contribute to, or have any actual or contingent liability with respect to any Employee Benefit Plan and has not previously sponsored, maintained, contributed to or had any such actual or contingent liability. The Company neither has nor has had any ERISA Affiliates. No act or omission has occurred and no condition exists with respect to any Employee Benefit Plan that would subject the Company to (i) any fine, penalty, tax or liability of any kind imposed under ERISA or the Internal Revenue Code or (ii) any contractual indemnification or contribution obligation protecting any fiduciary, insurer or service provider with respect to any Employee Benefit Plan.

(b) Section 2.16(b) of the Disclosure Schedule discloses each: (i) agreement between the Company and any Person (A) the benefits of which are contingent, or the terms of which are altered, upon the occurrence of a transaction involving the Company of the nature of any of the transactions contemplated by this Agreement, (B) providing any term of employment or engagement or compensation guarantee or (C) providing severance benefits or other benefits after the termination of the applicable employment or engagement; (ii) agreement, plan or arrangement under which any person may receive payments from the Company that may be subject to the tax imposed by Section 4999 of the Internal Revenue Code or included in the determination of such person's "parachute payment" under Section 280G of the Internal Revenue Code; and (iii) agreement or plan binding the Company, including any stock option plan, stock appreciation right plan, restricted stock plan, stock purchase plan, severance benefit, retention or incentive plan or other Employee Benefit Plan, any of the benefits of which will be increased, funded, "grossed-up", or the vesting of the benefits of which will be accelerated, or under which any obligation will be forgiven by the occurrence of any of the transactions contemplated by this Agreement (or in combination with any other event such as employment termination) or the value of any of the benefits of which will be calculated on the basis of any of the transactions contemplated by this Agreement.

(c) There is no corporate-owned life insurance (COLI), split-dollar life insurance policy or any other life insurance policy on the life of any director, officer, employee or consultant of the Company.

(d) No Person is entitled to any accrued vacation, accrued sick time or earned time off from the Company.

2.17 Environmental Matters. The Company has complied with all applicable Environmental Laws. There is no pending or, to the knowledge of the Company, threatened civil or criminal litigation, written notice of violation, formal administrative proceeding, or, to the knowledge of the Company, investigation, inquiry or information request, relating to any Environmental Law involving the Company. The Company has no liabilities or obligations

arising from the release or threatened release of any Materials of Environmental Concern into the environment. The Company is not a party to or bound by any court order, administrative order, consent order or other agreement entered into in connection with any legal obligation or liability arising under any Environmental Law. The Company is not aware of any environmental liability of any solid or hazardous waste transporter or treatment, storage or disposal facility that has been used by the Company.

2.18 Legal Compliance. The Company is currently conducting, and has at all times conducted, its activities in compliance with each applicable Law in all material respects. The Company has not received any written notice or, to the knowledge of the Company, other notice alleging noncompliance with any applicable Law. The Company has no material liability for failure to comply with any Law and, to the knowledge of the Company, there is no act, omission, event or circumstance that would reasonably be expected to give rise to any such liability. The Company is not conducting and has not conducted any internal investigation with respect to any actual, potential or alleged violation of any Law.

2.19 Permits. Section 2.19 of the Disclosure Schedule sets forth a list of all Permits issued to or held by the Company, if any. Such listed Permits are the only Permits that are required for the Company to conduct its activities as currently conducted, except where the failure to hold any Permit would not be material to the Company. Each such Permit is in full force and effect; the Company is in material compliance with the terms of each such Permit; and, to the knowledge of the Company, no suspension or cancellation of any such Permit is threatened and there is no reasonable basis for believing that any such Permit will not be renewable upon expiration.

2.20 Unlawful Payments. The Company is and has been in compliance with the Foreign Corrupt Practices Act, 15 U.S.C. §§ 78dd-1, et seq., the Organization for Economic Cooperation and Development Convention Against Bribery of Foreign Public Officials in International Business Transactions and legislation implementing such convention, all other international anti-bribery conventions and all applicable anti-corruption or bribery Laws in any jurisdiction in which the Company has conducted its business (collectively, "**Anti-Bribery Laws**"). The Company has not received any written notice or, to the knowledge of the Company, any other notice that alleges that the Company, or any current or former representatives of the Company in their capacities as such, is in violation of, or has any liability under, any Anti-Bribery Laws, and no such potential violation of Anti-Bribery Laws has been discovered by the Company. The Company has not made and does not anticipate making any disclosures to any Governmental Entity for actual or potential violations of Anti-Bribery Laws. None of the Company's current or former directors, officers, employees or consultants is currently an officer, agent or employee of a Governmental Entity. The Company has not, nor have its current or former representatives in their capacities as such, directly or indirectly, offered, given, reimbursed, paid or promised to pay, or authorized the payment of, any money or other thing of value (including any fee, gift, sample, travel expense or entertainment) or any commission payment payable to (a) any official, officer, agent, employee or representative of any Governmental Entity or of any existing or prospective customer of the Company (whether or not owned by a Governmental Entity), (b) any political party or official thereof, (c) any

candidate for political or political party office or (d) any other Person affiliated with any such customer, political party or official or political office, in each case while knowing or having reason to believe that all or any portion of such money or thing of value would be offered, given, reimbursed, paid or promised, directly or indirectly, for unlawful purposes under the Anti-Bribery Laws.

2.21 No Customers; Suppliers. The Company has never sold or otherwise supplied any goods or services to any third party. Section 2.21 of the Disclosure Schedule sets forth a list of each supplier that is the sole supplier of any significant product or service to the Company. No such supplier has indicated within the past year that it will stop, or decrease the rate of, supplying products or services, as applicable, to the Company. The Company has no purchase order with an unpaid balance.

2.22 Certain Business Relationships With Affiliates. Except as set forth in Section 2.22 of the Disclosure Schedule, no Affiliate of the Company (a) owns any property or right, tangible or intangible, which is used in the current activities of the Company, or (b) has any claim or cause of action against the Company, or (c) owes any money to, or is owed any money by, the Company. There have not been any commercial transactions or relationships between the Company and any Affiliate thereof which occurred or have existed since the Company's inception.

2.23 Brokers' Fees. The Company has no liability or obligation to pay any fees or commissions to any broker, finder or agent with respect to the transactions contemplated by this Agreement.

2.24 Books and Records. The minute books and/or other similar records of the Company contain complete and accurate records of all actions taken at any meetings of the Company's stockholders, Board of Directors or any committee thereof and of all written consents executed in lieu of the holding of any such meeting. The Company has maintained books and/or other similar records with respect to, and which accurately reflect, in all material respects, the assets, business and operations of the Company. Section 2.24 of the Disclosure Schedule contains a list of all bank accounts and safe deposit boxes of the Company and the names of persons having signature authority with respect thereto or access thereto.

2.25 Disclosure. No representation or warranty by the Company contained in this Agreement, and no statement contained in the Disclosure Schedule or any certificate delivered or to be delivered by or on behalf of the Company pursuant to this Agreement, contains or will contain any untrue statement of a material fact or omits or will omit to state any material fact necessary, in light of the circumstances under which it was or will be made, in order to make the statements herein or therein not misleading.

ARTICLE III
REPRESENTATIONS AND WARRANTIES OF THE COMPANY STOCKHOLDERS

Each Company Stockholder represents and warrants to the Buyer, Merger Sub 1 and Merger Sub 2, individually (and not jointly), that the statements contained in this Article III are true and correct.

3.1 Organization and Power. If such Company Stockholder is not a natural Person, such Company Stockholder is a corporation or other entity duly organized, validly existing and in good standing under the laws of the jurisdiction of its formation and such Company Stockholder has all requisite power and authority to carry on the businesses in which it is engaged and to own and use the properties owned and used by it.

3.2 Authorization. Such Company Stockholder has all requisite power and authority to execute and deliver this Agreement and to perform its obligations hereunder. The execution and delivery by such Company Stockholder of this Agreement and the consummation by such Company Stockholder of the transactions contemplated hereby have each been duly and validly authorized by all necessary action on the part of such Company Stockholder. This Agreement has been duly and validly executed and delivered by such Company Stockholder and, assuming due authorization, execution and delivery by the other Parties, constitutes a valid and binding obligation of such Company Stockholder, enforceable against such Company Stockholder in accordance with its terms, subject to the Bankruptcy and Equity Exception.

3.3 Noncontravention. Subject to the filing of the Certificate of Merger 1 and the Certificate of Merger 2 as required by the Delaware General Corporation Law, none of the execution and delivery by such Company Stockholder of this Agreement, the performance by such Company Stockholder of any of its obligations hereunder or the consummation by such Company Stockholder of the transactions contemplated hereby, does or will (a) if such Company Stockholder is not a natural person, conflict with or violate any provision of the organizational documents of such Company Stockholder, (b) require on the part of such Company Stockholder any notice to or filing with, or any permit, authorization, consent or approval of, any Governmental Entity, (c) conflict with, result in a material breach of, constitute (with or without due notice or lapse of time or both) a material default under, result in the acceleration of obligations or loss of any right or benefit under, create in any party the right to terminate, modify or cancel, or require any notice, consent or waiver under, any material contract or other instrument to which such Company Stockholder is a party or by which such Company Stockholder is bound or to which any of such Company Stockholder's assets is subject, (d) result in the imposition of any Security Interest upon any of the Company Shares held by such Company Stockholder or (e) violate any order, writ, injunction, decree, statute, rule or regulation applicable to such Company Stockholder or any of such Company Stockholder's properties or assets.

3.4 Title to Company Shares. Such Company Stockholder holds beneficially and of record all of such Company Stockholder's Company Shares as set forth in Section 2.2(b) of the Disclosure Schedule, free and clear of any Security Interests (other than restrictions on transfer

arising under the Securities Act and state or foreign securities Laws). Such Company Stockholder is not a party to any voting trust, proxy, or other agreement or understanding with respect to the voting or transfer of any Company Shares.

3.5 Litigation. There is no Legal Proceeding which is pending or has been threatened in writing against such Company Stockholder that questions the validity of this Agreement or any action taken or to be taken by such Company Stockholder in connection herewith or that could reasonably be expected to adversely affect such Company Stockholder's ability to consummate the transactions contemplated by this Agreement.

3.6 Absence of Intent to Distribute. Such Company Stockholder is acquiring the Buyer Shares for its own account and not with a present view to, or for sale in connection with, any distribution thereof in violation of the Securities Act; and such Company Stockholder has no present or contemplated agreement, undertaking, arrangement, obligation, Indebtedness or commitment providing for the disposition thereof. Such Company Stockholder consents to the placement of the following legend on each certificate for the Buyer Shares until such time as the Buyer Shares are eligible for sale pursuant to Rule 144 under the Securities Act:

> "THE SECURITIES EVIDENCED BY THIS CERTIFICATE HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933 AND MAY NOT BE TRANSFERRED OR SOLD UNLESS (i) A REGISTRATION STATEMENT UNDER SUCH ACT IS THEN IN EFFECT WITH RESPECT THERETO, (ii) A WRITTEN OPINION FROM COUNSEL FOR THE ISSUER OR COUNSEL FOR THE HOLDER REASONABLY ACCEPTABLE TO THE ISSUER HAS BEEN OBTAINED TO THE EFFECT THAT NO SUCH REGISTRATION IS REQUIRED OR (iii) A 'NO ACTION' LETTER OR ITS THEN EQUIVALENT HAS BEEN ISSUED BY THE STAFF OF THE SECURITIES AND EXCHANGE COMMISSION WITH RESPECT TO SUCH TRANSFER OR SALE."

The Buyer may also place stop-transfer instructions in respect of the Buyer Shares with respect to such legend.

3.7 Restricted Securities.

(a) Such Company Stockholder is an "accredited investor" as such term is defined for purposes of Regulation D under the Securities Act. Such Company Stockholder understands that the Buyer Shares will not be registered under the Securities Act for the reason that the issuance of Buyer Shares provided for in this Agreement is exempt pursuant to Section 4 of the Securities Act and that the reliance of the Buyer on such exemption is predicated in part on such Company Stockholder's representations set forth herein. Such Company Stockholder represents that it is experienced in evaluating companies such as the Buyer, has such knowledge and experience in financial and business matters as to be capable of evaluating the merits and risks of its investment, and has the financial wherewithal to suffer the total loss of its investment in the Buyer Shares.

(b) Such Company Stockholder understands that the Buyer Shares may not be sold, transferred or otherwise disposed of without registration under the Securities Act or an exemption therefrom and that in the absence of an effective registration statement covering the Buyer Shares or an available exemption from registration under the Securities Act, the Buyer Shares must be held indefinitely.

3.8 Certain Information. Such Company Stockholder has carefully reviewed this Agreement, including the representations concerning the Buyer contained herein, and understands the information set forth herein. Such Company Stockholder has made detailed inquiry concerning the Buyer, its business and its personnel, and the officers and other representatives of the Buyer have made available to such Company Stockholder any and all written information which such Company Stockholder has requested and have answered to such Company Stockholder's satisfaction all inquiries made by such Company Stockholder. Such Company Stockholder understands that the Buyer Shares are being or will be issued without any particular offering or disclosure document. Such Company Stockholder has been represented by such financial, legal and tax counsel and others selected by such Company Stockholder as such Company Stockholder has found necessary or appropriate to consult concerning this transaction and to review and evaluate the financial, legal, tax, and other ramifications of an investment in the Buyer, including the tax consequences to such Company Stockholder of an investment in the Buyer Shares. Except as expressly set forth in this Agreement, no representation or warranty of any kind has been made by the Buyer, or any other Person, with respect to the risks or consequences of an investment in the Buyer. Without limiting the generality of the foregoing, except as expressly set forth in this Agreement, neither the Buyer nor any officer, director or other representative of the Buyer has made any representation or warranty with respect to any business plan, presentation, projection, estimate or budget delivered to or made available to such Company Stockholder with respect to future results of operations, future financial condition or the future business and operations of the Buyer.

3.9 Not a Bad Actor. Such Company Stockholder has not taken any of the actions set forth in, and is not otherwise subject to, the disqualification provisions of Rule 506(d)(1) under the Securities Act.

3.10 Brokers' Fees. Such Company Stockholder has no liability or obligation to pay any fees or commissions to any broker, finder or agent with respect to the transactions contemplated by this Agreement.

3.11 Section 368(a). To such Company Stockholder's knowledge after consultation with its tax counsel, such Company Stockholder has not taken or agreed to take any action that would prevent the Mergers from qualifying as a reorganization within the meaning of Section 368(a) of the Internal Revenue Code.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

ARTICLE IV
REPRESENTATIONS AND WARRANTIES OF THE BUYER, MERGER SUB 1 AND MERGER SUB 2

The Buyer, Merger Sub 1 and Merger Sub 2 represent and warrant to the Company Stockholders (jointly and severally) that the statements contained in this Article IV are true and correct.

4.1 Organization and Corporate Power. The Buyer is a company incorporated under the laws of the Cayman Islands. Merger Sub 1 and Merger Sub 2 are each a corporation duly organized, validly existing and in good standing under the Laws of the State of Delaware. The Buyer has all requisite corporate power and authority to carry on the businesses in which it is engaged and to own and use the properties owned and used by it. The Buyer has furnished to the Company complete and accurate copies of the Articles of Association and the Shareholder Agreement, as amended or restated, respectively. The Company is not in default under or in violation of any provision of the Articles of Association. The Company is not in default under or in violation of any provision of the Shareholder Agreement.

4.2 Capitalization.

(a) The authorized capital stock of the Buyer consists of $50,000 divided into 1,288,327,750 Buyer Capital Shares, of which 27,184,132 were issued and outstanding as of July 31, 2018.

(b) The table set forth under the heading “Principal Shareholders” set forth in the Company’s prospectus supplement, dated June 7, 2018 is accurate in all material respects as of such date. All of the issued and outstanding shares of capital stock of the Buyer have been duly authorized and validly issued and are fully paid and nonassessable. All of the issued and outstanding shares of capital stock of the Buyer have been offered, issued and sold by the Buyer in compliance with all applicable Laws.

(c) Other than as set forth in Section 4.2(c) of the Disclosure Schedule, no subscription, warrant, option, convertible security or other right (contingent or otherwise) to purchase or acquire any shares of capital stock of the Buyer is authorized or outstanding. The Buyer has no obligation (contingent or otherwise) to issue any subscription, warrant, option, convertible security or other such right, or to issue or distribute to holders of any shares of its capital stock any evidences of Indebtedness or assets of the Buyer. The Buyer has no obligation (contingent or otherwise) to purchase, redeem or otherwise acquire any shares of its capital stock or any interest therein or to pay any dividend or to make any other distribution in respect thereof. There are no outstanding or authorized stock appreciation, phantom stock or similar rights with respect to the Buyer.

(d) Section 4.2(d) of the Disclosure Schedule lists each agreement, written or oral, between the Buyer and any holder of its securities, or among any holders of its securities, relating to the sale or transfer (including agreements relating to rights of first refusal, co-sale rights or “drag-along” rights), registration under the Securities Act, or voting, of the capital stock of the Buyer.

(e) There is no claim pending, and to the knowledge of the Buyer, no claim threatened, against the Buyer by any Person that seeks to assert: (i) ownership or rights to ownership of any shares of Buyer capital stock; (ii) any rights of a stockholder, including any option, preemptive rights or rights to notice or to vote; (iii) any rights under the Articles of Association or the Shareholder Agreement; or (iv) any claim that his, her or its shares have been wrongfully repurchased by the Buyer.

4.3 Operations of Merger Sub 1 and Merger Sub 2. Merger Sub 1 and Merger Sub 2 are each a corporation newly formed by the Buyer for the sole purpose of effecting the Mergers, and has not engaged in any activity other than as contemplated in this Agreement.

4.4 Authorization of Transaction. Each of the Buyer, Merger Sub 1 and Merger Sub 2 has all requisite corporate power and authority to execute and deliver this Agreement and to perform its obligations hereunder. The execution and delivery by the Buyer, Merger Sub 1 and Merger Sub 2 of this Agreement and, subject to obtaining the approval of the Buyer as the sole stockholder of Merger Sub 1 and the approval of Merger Sub 1 as the sole stockholder of Merger Sub 2, the consummation by the Buyer, Merger Sub 1 and Merger Sub 2 of the transactions contemplated hereby have been duly and validly authorized by all necessary corporate action on the part of the Buyer, Merger Sub 1 and Merger Sub 2, respectively. This Agreement has been duly and validly executed and delivered by the Buyer, Merger Sub 1 and Merger Sub 2 and, assuming due authorization, execution and delivery by the other Parties, constitutes a valid and binding obligation of the Buyer, Merger Sub 1 and Merger Sub 2, enforceable against them in accordance with its terms, subject to the Bankruptcy and Equity Exception.

4.5 Noncontravention. Subject to the filing of the Certificate of Merger 1 and the Certificate of Merger 2 as required by the Delaware General Corporation Law, neither the execution and delivery by the Buyer, Merger Sub 1 or Merger Sub 2 of this Agreement, nor the consummation by the Buyer, Merger Sub 1 or Merger Sub 2 of the transactions contemplated hereby, does or will (a) conflict with or violate any provision of the charter or bylaws of the Buyer, Merger Sub 1 or Merger Sub 2, (b) require on the part of the Buyer, Merger Sub 1 or Merger Sub 2 any filing with, or permit, authorization, consent or approval of, any Governmental Entity, (c) conflict with, result in a material breach of, constitute (with or without due notice or lapse of time or both) a material default under, result in the acceleration of obligations under, create in any party any right to terminate, modify or cancel, or require any notice, consent or waiver under, any material contract or other instrument to which the Buyer, Merger Sub 1 or Merger Sub 2 is a party or by which either is bound or to which any of their assets are subject, except for (i) any conflict, breach, default, acceleration, termination, modification or cancellation which would not adversely affect the consummation of the transactions contemplated hereby or (ii) any notice, consent or waiver the absence of which would not adversely affect the consummation of the transactions contemplated hereby or (d) violate any order, writ, injunction, decree, statute, rule or regulation applicable to the Buyer, Merger Sub 1 or Merger Sub 2 or any of their properties or assets.

4.6 Issuance of Buyer Shares. The issuance and delivery of Buyer Shares in accordance with this Agreement has been duly authorized by all necessary corporate action on the part of the Buyer and, when issued as contemplated hereby, such Buyer Shares shall be duly authorized, duly and validly issued, fully paid and nonassessable.

ARTICLE V
COVENANTS

5.1 Closing Efforts. Each of the Parties shall use its Reasonable Best Efforts to take all actions and to do all things necessary, proper or advisable to consummate the transactions contemplated by this Agreement, including using its commercially reasonable efforts to ensure that (i) its representations and warranties remain true and correct in all material respects through, on and as of the Closing Date and (ii) the conditions to the obligations of the other Parties to consummate the Mergers are satisfied.

5.2 Governmental and Third-Party Notices and Consents; Bylaws.

(a) Each Party shall use its Reasonable Best Efforts to obtain, at its expense, all waivers, permits, consents, approvals or other authorizations from Governmental Entities, and to effect all registrations, filings and notices with or to Governmental Entities, as may be required for such Party to consummate the transactions contemplated by this Agreement and to otherwise comply with all applicable Laws and regulations in connection with the consummation of the transactions contemplated by this Agreement.

(b) The Company shall use its Reasonable Best Efforts to obtain, at its expense, all such waivers, consents or approvals from third parties, and to give all such notices to third parties, as are listed or required to be listed in Section 6.1 of the Disclosure Schedule.

(c) The Company shall not adopt bylaws.

5.3 Stockholder Approval. As expeditiously as possible following the execution of this Agreement, the Company shall secure and cause to be filed with the Company written consents in the form of Exhibit A ("**Written Consents**") duly executed by all Company Stockholders. The Company, acting through its Board of Directors, shall include in the Written Consent (a) the unanimous recommendation of its Board of Directors that the stockholders of the Company vote in favor of the adoption of this Agreement and the approval of the Mergers and (b) a statement that appraisal rights are available for the Company Shares pursuant to the Dissenters' Rights Statute and a copy thereof, or in the alternative, a written waiver of such appraisal rights duly executed by the applicable Company Stockholder.

5.4 Operation of Business. Except as contemplated by this Agreement, during the period from the date of this Agreement to the earlier of the termination of this Agreement in accordance with its terms or the Merger 1 Effective Time, the Company shall conduct its operations in the Ordinary Course of Business and in compliance with all applicable Laws and regulations, and for the avoidance of doubt, during such period shall not incur any obligations or liabilities in respect of any employees or Employee Benefit Plans.

5.5 Tax Matters. The Parties intend that the Mergers be treated as a "reorganization" within the meaning of Section 368(a) of the Internal Revenue Code. However, neither Buyer, Merger Sub 1 nor Merger Sub 2 makes any representation or warranty to the other or to the Company or any Company Stockholder regarding the Tax treatment of the Mergers or whether the Mergers will qualify as a "reorganization" under the Internal Revenue Code and any representation or warranty expressed to the contrary under any other provision of this Agreement shall be interpreted consistently with the provisions of this Section 5.5. Each of the Buyer, Merger Sub 1, Merger Sub 2, the Company and the Company Stockholders acknowledges that it is relying on its own advisors in connection with the Tax treatment of the Mergers and the other transactions contemplated by this Agreement. Each of the Buyer, Merger Sub 1, Merger Sub 2, the Company and the Company Stockholders each agree to use their respective commercially reasonable efforts to cause the Mergers to qualify, and will not take any actions not expressly contemplated by this Agreement which could reasonably be expected to prevent the Mergers from qualifying, as a "reorganization" under Section 368(a)(1)(A) of the Internal Revenue Code. The Parties agree to report the Mergers for U.S. federal income tax purposes as a "reorganization" within the meaning of Section 368(a) of the Internal Revenue Code (including by attaching the statement described in Treasury Regulations Section 1.368-3(a) on or with its return for the taxable year of the Mergers) unless (i) otherwise required by law or a taxing authority or (ii) the accounting firm preparing the applicable Tax Returns determines that there is no reasonable basis for taking such a position; and in the event of either (i) or (ii), the Parties shall promptly notify the other and shall consult promptly and in good faith in respect thereof.

5.6 Section 280G Matters. The Company is a "small business corporation" as defined in Section 1361(b) of the Internal Revenue Code. To the extent that (i) any "disqualified individual" (as such term is defined for purposes of Section 280G of the Internal Revenue Code) of the Company would be entitled to any payment or benefit as a result of the transactions contemplated by this Agreement and (ii) such payment or benefit would or could potentially constitute a "parachute payment" under Section 280G of the Internal Revenue Code or could reasonably be expected to result in the imposition of any excise Tax imposed under Section 4999 of the Internal Revenue Code, prior to the Closing:

(a) The Company shall obtain stockholder approval in accordance with the requirements of Section 280G(b)(5)(B) of the Internal Revenue Code and Regulations § 1.280G-1 thereunder (the "**280G Shareholder Approval Requirements**") in respect of the portion of such parachute payment that exceeds three times less one dollar such individual's "base amount" within the meaning of Section 280G(b)(3) of the Internal Revenue Code with respect to all such "disqualified individuals";

(b) The Company shall provide all required disclosure to all Persons entitled to vote under Section 280G(b)(5)(B)(ii) of the Internal Revenue Code prior to such vote and shall hold a vote of stockholders in the manner intended to satisfy the 280G Shareholder Approval Requirements;

(c) The Company shall obtain any required waivers or consents from the disqualified individual(s) prior to the vote, which vote shall establish whether the disqualified individual is entitled to receive or retain the waived payments or other waived compensation; and

(d) The Buyer and its counsel shall be given the right to review and comment on all documents required to be delivered to the Company Stockholders in connection with such vote and any required disqualified individual waivers or consents, and the Company shall reflect all reasonable comments of the Buyer thereon. The Buyer and its counsel shall be provided copies of all documents executed by the Company Stockholders and disqualified individuals in connection with the vote.

5.7 No Claim Against the Company. Effective as of the Closing, each Company Stockholder, by its execution and delivery of this Agreement and/or the Written Consent, hereby (a) waives any and all rights of indemnification, contribution and other similar rights against the Company (whether arising pursuant to any charter document of the Company, any contract, applicable Law or otherwise) arising out of the representations, warranties, covenants and agreements contained in this Agreement and/or out of the negotiation, execution or performance of this Agreement, and agrees that any claim of the Buyer, whether for indemnity or otherwise, may be asserted directly against the Company Stockholders or any Company Stockholder (solely to the extent, and subject to the limitations, provided in this Agreement), without any need for any claim against, or joinder of, the Company and (b) forever waives, releases and discharges (and hereby agrees to cause each of its representatives to forever waive, release and discharge) with prejudice the Company from any and all claims, rights (including rights of indemnification, contribution and other similar rights, from whatever source, whether under contract, law or otherwise), causes of action, protests, suits, disputes, orders, obligations, debts, demands, proceedings, contracts, agreements, promises, liabilities, controversies, costs, expenses, fees (including attorneys' fees), or damages of any kind, arising by any means (including subrogation, assignment, reimbursement, operation of law or otherwise), whether known or unknown, suspected or unsuspected, accrued or not accrued, foreseen or unforeseen, or mature or unmature related or with respect to, in connection with, or arising out of, directly or indirectly, any event, fact, condition, circumstance, occurrence, act or omission that was in existence (or that occurred or failed to occur) at or prior to the Closing; provided, however, this clause (b) shall not be construed as releasing any Party from its obligations otherwise expressly set forth in this Agreement or any agreement delivered pursuant to this Agreement (including, without limitation, the Buyer indemnification obligations set forth in Section 7.1(d)).

ARTICLE VI
CONDITIONS TO CONSUMMATION OF THE MERGER

6.1 Conditions to Obligations of the Buyer, Merger Sub 1 and Merger Sub 2. The obligation of each of the Buyer, Merger Sub 1 and Merger Sub 2 to consummate the Mergers is subject to the satisfaction (or waiver by the Buyer) of the following conditions:

(a) the Written Consent evidencing the Requisite Stockholder Approval shall have been duly executed and delivered to the Buyer;

(b) the Company shall have obtained at its own expense (and shall have provided copies thereof to the Buyer of) all of the waivers, permits, consents, approvals or other authorizations, and effected all of the registrations, filings, notices and contract terminations as are listed in Section 6.1 of the Disclosure Schedule or are otherwise required on the part of the Company to effect the Mergers, if any;

(c) the representations and warranties of the Company and the Company Stockholders set forth in this Agreement shall be true and correct, in each case as of the date of this Agreement and as of the Closing as though made as of the Closing, except to the extent such representations and warranties are specifically made as of a particular date (in which case such representations and warranties shall be true and correct as of such date);

(d) each of the Company and the Company Stockholders shall have performed or complied with, in all material respects, its agreements and covenants required to be performed or complied with under this Agreement as of or prior to the Closing;

(e) no Legal Proceeding shall be pending or threatened in writing wherein an unfavorable judgment, order, decree, stipulation or injunction could reasonably be expected to (i) prevent consummation of the transactions contemplated by this Agreement, (ii) cause the transactions contemplated by this Agreement to be rescinded following consummation or (iii) have, individually or in the aggregate, a Company Material Adverse Effect, and no such judgment, order, decree, stipulation or injunction shall be in effect;

(f) the Company shall have delivered to the Buyer, Merger Sub 1 and Merger Sub 2 the Company Closing Certificate;

(g) the Company shall have delivered to the Buyer a certificate executed by the Secretary of the Company certifying that attached thereto are (i) a true, complete and correct copy of the Certificate of Incorporation, as in effect on the Closing Date, and, in the case of the Certificate of Incorporation, certified by the Secretary of State of the State of Delaware, (ii) true, complete and correct copies of resolutions unanimously adopted by the Company's Board of Directors and stockholders, authorizing the execution and delivery of this Agreement, the transactions contemplated hereby and the performance by the Company of its obligations hereunder, which resolutions have not been modified, rescinded or revoked, and (iii) a specimen signature of the duly authorized officer of the Company signing this Agreement on behalf of the Company;

(h) the Company shall have delivered to the Buyer a certificate, issued by the Secretary of State of the State of Delaware and each other jurisdiction in which the Company is qualified to do business, if any, certifying as of a date no more than five (5) business days prior to the Closing Date that the Company is in good standing under the Laws of such jurisdiction;

(i) the Buyer shall have received copies of the resignations, effective as of the Closing, of each director and officer of the Company from such positions as a director or officer, as applicable;

(j) the Company shall have delivered to the Buyer evidence reasonably satisfactory to the Buyer that all contracts and other arrangements listed on Schedule 6.1(j) have been terminated on terms reasonably satisfactory to the Buyer;

(k) the Company shall have delivered to the Buyer a certification in form and substance reasonably satisfactory to the Buyer that the Company Shares are not United States real property interests as defined in Section 897(c) of the Internal Revenue Code, together with a notice to the Internal Revenue Service, in accordance with the Treasury Regulations under Sections 897 and 1445 of the Internal Revenue Code;

(l) the Company Stockholders and, if requested by the Company Stockholders, a limited liability company or other entity wholly owned by the Company Stockholders shall have entered into a consulting agreement, in substantially the form attached hereto as Exhibit B (the "**Consulting Agreement**"), with the Buyer (or a wholly-owned subsidiary of the Buyer).

6.2 Conditions to Obligations of the Company. The obligation of the Company and the Company Stockholders to consummate the Mergers is subject to the satisfaction of the following conditions:

(a) the representations and warranties of the Buyer, Merger Sub 1 and Merger Sub 2 set forth in this Agreement shall be true and correct, in each case as of the date of this Agreement and as of the Closing as though made as of the Closing, except to the extent such representations and warranties are specifically made as of a particular date (in which case such representations and warranties shall be true and correct as of such date);

(b) each of the Buyer, Merger Sub 1 and Merger Sub 2 shall have performed or complied with, in all material respects, its agreements and covenants required to be performed or complied with under this Agreement as of or prior to the Closing;

(c) no Legal Proceeding shall be pending or threatened in writing wherein an unfavorable judgment, order, decree, stipulation or injunction would (i) prevent consummation of the transactions contemplated by this Agreement, (ii) cause the transactions contemplated by this Agreement to be rescinded following consummation or (iii) have, individually or in the aggregate, a material adverse effect on the ability of the Buyer, Merger Sub 1 and Merger Sub 2 to perform their respective obligations under this Agreement or consummate the transactions contemplated by this Agreement, and no such judgment, order, decree, stipulation or injunction shall be in effect;

(d) the Buyer, Merger Sub 1 and Merger Sub 2 shall have delivered to the Company the Buyer Closing Certificate;

(e) the Buyer shall have delivered to the Company a certificate executed by the Secretary of each of the Buyer, Merger Sub 1 and Merger Sub 2 certifying that attached thereto are (i) a true, complete and correct copy of the Articles of Association of the Buyer, the

Certificate of Incorporation of Merger Sub 1 and the Certificate of Incorporation of Merger Sub 2, as in effect on the Closing Date, and, in the case of the Certificate of Incorporation, certified by the Secretary of State of the State of Delaware, (ii) true, complete and correct copies of resolutions duly and validly adopted by the Buyer's, Merger Sub 1's and Merger Sub 2's Boards of Directors, authorizing the execution and delivery of this Agreement, the transactions contemplated hereby and the performance by the Buyer, Merger Sub 1 and Merger Sub 2, respectively, of their obligations hereunder, which resolutions have not been modified, rescinded or revoked, and (iii) a specimen signature of the duly authorized officer of the Buyer, Merger Sub 1 and Merger Sub 2 signing this Agreement on behalf of the Buyer, Merger Sub 1 and Merger Sub 2, respectively;

(f) the Buyer shall have delivered to the Company a certificate, issued by the Secretary of State of the State of Delaware, certifying as of a date no more than five (5) business days prior to the Closing Date that Merger Sub 1 and Merger Sub 2 are in good standing under the Laws of such jurisdiction; and

(g) the Buyer (or a wholly-owned subsidiary of the Buyer) shall have entered into the Consulting Agreement with the Company Stockholders and, if requested by the Company Stockholders, a limited liability company or other entity wholly owned by the Company Stockholders.

ARTICLE VII
INDEMNIFICATION

7.1 Indemnification by the Company Stockholders and the Buyer.

(a) Expressly subject to the conditions and limitations set forth in this Article VII, the Company Stockholders shall severally and jointly defend and indemnify the Buyer in respect of, and hold it harmless against and will compensate and reimburse the Buyer for, any and all Losses incurred or suffered by the Surviving Corporation or the Buyer or any Affiliate thereof (regardless of whether such Losses relate to any Third-Party Action) resulting from, relating to or constituting:

(i) any inaccuracy in any representation or breach of any warranty of the Company contained in Article II or in the Company Closing Certificate, whether as of the date of this Agreement or as of the Closing Date;

(ii) any breach or nonperformance of (or noncompliance with) any covenant or agreement of the Company or any Company Stockholder contained in this Agreement to the extent required to be performed at or prior to the Closing;

(iii) any inaccuracy in the Allocation Schedule;

(iv) any claim by a stockholder or former stockholder of the Company, or any other Person, seeking to assert, or based upon: (A) ownership or rights to ownership of any shares of stock of the Company; (B) any rights of a stockholder (other than the right to receive the amounts payable to such holder pursuant to Article I), including any option, preemptive rights or rights to notice or to vote; (C) any rights under the Certificate of Incorporation of the Company; (D) any claim based on or arising out of any breach of fiduciary duty by the Company's Board of Directors or stockholders; or (E) any claim that his, her or its shares were wrongfully repurchased by the Company; in each case, which claim arises from acts or omissions of the Company or the Company Stockholders prior to the Closing Date;

(v) the following Taxes: (A) any Taxes for any taxable period (or portion thereof) ending on or before the Closing Date due and payable by the Company, which Taxes shall be apportioned to the pre-closing portion of such taxable period, in the case of a taxable period that includes but does not end on the Closing Date, (1) in the case of property or similar *ad valorem* Taxes, based on the number of days in the portion of such taxable period ending on the Closing Date as compared to the number of days in such taxable period, and (2) in the case of all other Taxes, on a closing of the books basis as of the Closing Date; (B) any Taxes for which the Company has any liability under Treasury Regulation Section 1.1502-6 or under any comparable or similar provision of state, local or foreign Laws as a result of being a member of an affiliated, consolidated, combined, unitary or similar group on or prior to the Closing Date; (C) any Taxes for which the Company has any liability as a transferee or successor, pursuant to any contractual obligation or otherwise, which Tax is attributable to the operations of the Company on or prior to the Closing Date or an event or transaction occurring before the Closing; and (D) any transfer, sales, use, stamp, conveyance, value added, recording, registration, documentary, filing and other non-income Taxes and administrative fees (including notary fees) imposed on or assessed against the Company prior to the Closing; provided, however, that the Company Stockholders shall have no liability, and shall not indemnify the Buyer, for any Taxes resulting from any actions or elections with respect to any tax period (or portion thereof) ending on or prior to the Closing Date made by Buyer, the Intermediate Surviving Corporation, Merger Sub 1, Merger Sub 2 or the Surviving Corporation as of or after the Merger 1 Effective Time;

(vi) to the extent not taken into account in determining the number of Closing Shares, any amounts that become payable (in whole or in part) by the Company to any Person as a result of the execution and delivery of this Agreement or the consummation of the transactions contemplated hereby, whether taken alone or in combination with any other event;

(vii) any fraud, intentional or knowing misrepresentation or willful breach on the part of the Company in connection with the transactions contemplated by this Agreement, but excluding any breach or alleged breach under the Consulting Agreement, which shall be resolved pursuant to the terms of the Consulting Agreement;

(viii) any claim arising from any agreement listed on Section 7.1(a)(viii) of the Disclosure Schedule; or

(ix) any Indebtedness or trade payables of the Company as of immediately prior to the Closing in excess of $10,000.

(b) Each Company Stockholder shall individually (and not jointly) defend and indemnify the Buyer in respect of, and hold it harmless against and will compensate and reimburse the Buyer for, any and all Losses incurred or suffered by the Surviving Corporation or the Buyer or any Affiliate thereof (regardless of whether such Losses relate to any Third-Party Action) resulting from, relating to or constituting: (i) any inaccuracy in any representation or breach of any warranty of such Company Stockholder contained in Article III or in the Company Closing Certificate, whether as of the date of this Agreement or as of the Closing Date; (ii) any breach or nonperformance of (or noncompliance with) any covenant or agreement of such Company Stockholder contained in this Agreement; or (iii) any fraud, intentional or knowing misrepresentation or willful breach on the part of such Company Stockholder in connection with the transactions contemplated by this Agreement, but excluding any breach or alleged breach under the Consulting Agreement, which shall be resolved pursuant to the terms of the Consulting Agreement.

(c) [intentionally omitted].

(d) The right to indemnification, payment, reimbursement, or other remedy based upon any representation, warranty, covenant or obligation pursuant to this Section 7.1 will not be affected by any investigation conducted or any knowledge acquired at any time, whether before or after the execution and delivery of this Agreement or the Closing Date, with respect to the accuracy or inaccuracy of, or compliance with, such representation, warranty, covenant, or obligation.

(e) Expressly subject to the conditions and limitations set forth in this Article VII, the Buyer shall defend and indemnify each of the Company Stockholders in respect of, and hold each of them harmless against and will compensate and reimburse each of the Company Stockholders for, any and all Losses incurred or suffered by any of them (regardless of whether such Losses relate to any Third-Party Action) resulting from, relating to or constituting:

(i) any inaccuracy in any representation or breach of any warranty of the Buyer contained in Article II or in the Buyer Closing Certificate, whether as of the date of this Agreement or as of the Closing Date;

(ii) any breach or nonperformance of (or noncompliance with) any covenant or agreement of the Buyer or the Surviving Corporation contained in this Agreement to the extent required to be performed at or prior to the Closing;

(iii) any fraud, intentional or knowing misrepresentation or willful breach on the part of the Buyer or the Surviving Corporation in connection with the transactions contemplated by this Agreement but excluding any breach or alleged breach under the Consulting Agreement, which shall be resolved pursuant to the terms of the Consulting Agreement; or

(iv) any Third-Party Action against the Surviving Corporation in respect of a claim for which the Company Stockholders have no indemnification obligations to the Buyer pursuant to Section 7.1(a) or (b) above; *provided*, *however*, that for purposes of this Section 7.1(e)(iv) only, the representations and warranties of the Company and the Company Stockholders shall be deemed to survive indefinitely.

7.2 Indemnification Claims.

(a) A Person entitled to indemnification under this Article VII (an "**Indemnified Party**") shall give reasonably prompt written notification to any other Person obligated to indemnify the Indemnified Party (an "**Indemnifying Party**") of the commencement, or threatened commencement, of any Third-Party Action that the Indemnified Party reasonably expects may result in a claim for indemnification pursuant to this Article VII. For purposes of notices and communications with respect to the Company Stockholders as potential indemnitees or indemnitors pursuant to this Article VII, "Indemnified Party" and "Indemnifying Party" shall mean the Company Stockholder Representative on behalf of the Company Stockholders. Such notification shall be given reasonably promptly after receipt by the Indemnified Party of notice of such Third-Party Action or threatened Action, and shall describe in reasonable detail (to the extent known by the Indemnified Party) the facts constituting the basis for such Third-Party Action and the amount of the claimed damages; provided, however, that no delay or failure on the part of the Indemnified Party in so notifying the Indemnifying Party shall relieve the Indemnifying Party of any liability or obligation hereunder except to the extent of any damage or liability caused by or arising out of such failure.

(b) The Indemnifying Party shall be entitled, at its own and sole expense, to participate in any defense of such Third-Party Action. The Indemnified Party shall have the right in its sole and absolute discretion to control the defense of any Third-Party Action against the Indemnified Party and the Indemnified Party shall thereafter from time to time promptly provide to the Indemnifying Party copies of all pleadings filed and all orders issued in such Third-Party Action, and shall consult in good faith with the Indemnifying Party at reasonable periodic intervals on matters regarding the defense and potential settlement of such Third-Party Action. The Indemnified Party shall have the right to settle, adjust or compromise any such Third-Party

Action, subject to Indemnifying Party's right to dispute its obligation to indemnify; provided, however, that except with the prior written consent of the Indemnifying Party (which consent shall not be unreasonably withheld, delayed or conditioned), no settlement of any such Third-Party Action with third-party claimants shall be determinative of any Losses relating to such matter.

(c) In order to seek indemnification under this Article VII, the Indemnified Party shall, as promptly as reasonably practicable, deliver a Claim Notice to the Indemnifying Party.

(d) Within twenty (20) business days after delivery of a Claim Notice, the Indemnifying Party shall deliver to the Indemnified Party a response (a "**Response**"), in which the Indemnifying Party shall either: (i) agree that the Indemnified Party is entitled to receive the Claimed Amount or the Agreed Amount or (ii) dispute that the Indemnified Party is entitled to receive any of the Claimed Amount.

(e) During the twenty (20)-business-day period following the delivery of a Response that reflects a Dispute, the Indemnifying Party and the Indemnified Party shall use good faith efforts to resolve the Dispute. If the Dispute is not resolved within such twenty (20)-business-day period, either Party may submit the Dispute to a court of competent jurisdiction in accordance with the terms of this Agreement. In the event of a violation of Section 7.1(a)(vii) or Section 7.1(b)(iii) by the Company or the Company Stockholders, which violation has been determined by a court or other competent judicial authority, in a final non-appealable order or decision, then the Indemnifying Party shall deliver to the Indemnified Party, promptly following the resolution of the Dispute (whether by mutual agreement, judicial decision or otherwise), payment of any amount required to be paid to the Indemnified Party consistent with the terms of the resolution of the Dispute, to the extent not satisfied via the forfeiture of the right to be issued Holdback Shares and, if applicable, the forfeiture and cancellation of Buyer Shares pursuant to Section 7.4.

(f) The Company Stockholder Representative shall have full power and authority on behalf of each Company Stockholder to take any and all actions on behalf of, execute any and all instruments on behalf of, and execute or waive any and all rights of, the Company Stockholders under this Article VII. The Company Stockholder Representative shall have no liability to any Company Stockholder for any action taken or omitted on behalf of the Company Stockholders pursuant to this Article VII.

7.3 Survival of Representations and Warranties. Each Party's representations and warranties in this Agreement shall survive the Closing and shall expire at 11:59 p.m., Eastern Time, on the date that is 18 months after the Closing Date; provided, however, that the representations and warranties contained in Sections, 2.5, 2.14, 2.15, 2.22, 3.6, 3.7, 3.8 and 3.9, shall survive until the date that is 24 months after the Closing Date; provided, further, that the representations and warranties contained in Sections 2.1, 2.2, 2.3, 2.4(a), 2.8, 2.10, 3.1, 3.2, 3.3, 3.10, 4.1, 4.2, 4.3, 4.4 and 4.5(a) shall survive until the date that is six (6) years after the Closing Date. If an Indemnified Party delivers to the Indemnifying Party, before expiration of a

representation or warranty, either a Claim Notice based upon a breach of such representation or warranty, or an Expected Claim Notice based upon a breach of such representation or warranty, then the applicable representation or warranty shall survive until, but only for purposes of, the resolution of any claims arising from or related to the matter covered by such notice. Except as provided above with respect to indemnification obligations under Section 7.1(a)(v), nothing in this Section 7.3 shall be construed to limit the survival of covenants, agreements and obligations that by their terms are to be performed or observed after the Merger 1 Effective Time or for which another time period is specified in this Agreement.

7.4 Limitations and Related Matters.(a) (a) The exclusive source for the recovery of any Losses for which the Buyer is entitled to indemnification under Section 7.1 (other than Section 7.1(a)(v), Section 7.1(a)(vii), Section 7.1(a)(viii), and Section 7.1(b)(iii)) shall be expressly limited to the forfeiture and cancellation of any Holdback Shares issuable to the Company Stockholders pursuant to this Agreement (and recovery of an amount equal to any proceeds received by any Company Stockholder in respect of the sale, transfer or other disposition thereof (assuming for all purposes that the proceeds per share are equal to the Buyer Share Value)).

(b) For purposes of determining the number of Buyer Shares that the Buyer may be entitled to recover pursuant to this Article VII, each Buyer Share shall be deemed to have a value equal to Buyer Share Value, and the number of shares recoverable shall be rounded down to the nearest whole share.

(c) [intentionally omitted].

(d) Other than for claims involving fraud, intentional or knowing misrepresentation or willful breach of this Agreement, but excluding any breach or alleged breach under the Consulting Agreement, which shall be resolved pursuant to the terms of the Consulting Agreement, and subject to the provisions of Section 10.13, from and after the Closing, the remedies set forth in this Article VII shall be the sole and exclusive remedies of the Parties with respect to any breach of the respective representations, warranties, covenants and agreements pursuant to this Agreement or otherwise arising in connection with the transactions contemplated by this Agreement, including the enforcement, interpretation or negotiation of this Agreement. For clarification purposes only, no Company Stockholder shall have any personal or individual liability to Buyer or any other Person under this Agreement, except for the Company Stockholder indemnification obligations to Buyer under this Article VII, as expressly limited by this Section 7.4.

(e) Nothing in this Section 7.4 shall be construed to limit the Parties' rights under Section 10.13. No Company Stockholder shall have any right of contribution against the Company or the Surviving Corporation with respect to any breach by the Company of any of its representations, warranties, covenants or agreements.

7.5 Tax Treatment of Indemnification Payments. All amounts paid under this Article VII shall be treated as adjustments to the purchase price for all Tax purposes unless otherwise required by Law.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

ARTICLE VIII
TERMINATION

8.1 Termination of Agreement. The Parties may terminate this Agreement prior to the Closing (whether before or after the Requisite Stockholder Approval), as provided below:

(a) the Parties may terminate this Agreement by mutual written consent;

(b) the Buyer may terminate this Agreement by giving written notice to the Company in the event the Company is in breach of any representation, warranty or covenant contained in this Agreement, and such breach (i) individually or in combination with any other such breach, would cause the conditions set forth in Section 6.1(c) or Section 6.1(d) not to be satisfied and (ii) is not cured within 10 days following delivery by the Buyer to the Company of written notice of such breach;

(c) the Company may terminate this Agreement by giving written notice to the Buyer in the event the Buyer, Merger Sub 1 or Merger Sub 2 is in breach of any representation, warranty or covenant contained in this Agreement, and such breach (i) individually or in combination with any other such breach, would cause the conditions set forth in Section 6.2(a) or Section 6.2(b) not to be satisfied and (ii) is not cured within 10 days following delivery by the Company to the Buyer of written notice of such breach;

(d) the Buyer may terminate this Agreement by giving written notice to the Company if the Closing shall not have occurred on or before the date that is forty-five (45) days after the date of this Agreement, by reason of the non-satisfaction of any condition precedent under Section 6.1 (unless the non-occurrence of the Closing results primarily from a breach or nonperformance by the Buyer, Merger Sub 1 or Merger Sub 2 of any representation, warranty, covenant or agreement contained in this Agreement);

(e) the Buyer may terminate this Agreement if the Company shall have failed to deliver evidence of the satisfaction of the condition set forth in Section 6.1(a) within four (4) hours after the execution and delivery of this Agreement; or

(f) the Company may terminate this Agreement by giving written notice to the Buyer if the Closing shall not have occurred on or before the date that is forty-five (45) days after the date of this Agreement, by reason of the non-satisfaction of any condition precedent under Section 6.2 (unless the non-occurrence of the Closing results primarily from a breach by the Company of any representation, warranty, covenant or agreement contained in this Agreement).

8.2 Effect of Termination. If any Party terminates this Agreement pursuant to Article IX, all obligations of the Parties hereunder shall terminate without any liability of any Party to any other Party (except for any liability of any Party for intentional breaches of this Agreement prior to such termination).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

ARTICLE IX
CERTAIN DEFINITIONS

Capitalized terms used and not otherwise defined in this Agreement shall have the respective meanings set forth below:

"**Affiliate**" shall mean any affiliate, as defined in Rule 12b-2 under the Exchange Act.

"**Agreed Amount**" shall mean part, but not all, of the Claimed Amount.

"**Agreement**" shall have the meaning set forth in the first paragraph of this Agreement.

"**Allocation Schedule**" shall mean the schedule identified as Schedule A in the Disclosure Schedules, which sets forth (i) the name and address of each Company Stockholder, (ii) the number of Company Shares held by such Company Stockholder, (iii) the number of Closing Shares allocable to such Company Stockholder, (iv) the number of Holdback Shares allocable to such Company Stockholder, (v) the proportion of each Milestone Payment allocable to such Company Stockholder, and (vi) the proportion of each Royalty Payment allocable to such Company Stockholder.

"**Arbitrator**" shall have the meaning set forth in Section 10.12.

"**Articles of Association**" shall mean the articles of association of the Buyer, as amended and restated.

"**Buyer Capital Shares**" shall mean the ordinary shares of the Buyer with a nominal value of $0.00003881 per ordinary share; provided that if the Buyer has experienced a Change of Control, then Buyer Capital Shares shall mean the shares of capital stock, membership interests or similar equity securities in the entity that is the successor-in-interest of the Buyer following such Change of Control.

"**Buyer Closing Certificate**" shall mean a certificate to the effect that each of the conditions specified in Section 6.2(a) through Section 6.2(c) is satisfied in all respects.

"**Buyer Covered Person**" means, with respect to the Buyer as an "issuer" for purposes of Rule 506 promulgated under the Securities Act, any Person listed in the first paragraph of Rule 506(d)(1).

"**Buyer Material Adverse Effect**" shall mean any material adverse change, event, circumstance, occurrence, state of facts or development that, individually or in the aggregate with all other changes, events, circumstances, occurrences, states of facts or developments occurring prior to the determination of a Buyer Material Adverse Effect, has a material adverse effect on, (i) the business, assets, prospects, Intellectual Property, liabilities, capitalization, condition (financial or other), or results of operations of the Buyer and its Subsidiaries, taken as a whole, or (ii) the ability of the Buyer, Merger Sub 1 or Merger Sub 2 to consummate the transactions contemplated hereby; provided, however, that any adverse effects attributable to any

of the following as they relate to the Buyer shall not be deemed to constitute, and the following shall not be taken into account in determining whether there has been or will be, a Buyer Material Adverse Effect: (a) changes after the date of this Agreement in conditions affecting the industries in which the Buyer or any of its Subsidiaries participates or the U.S. economy or financial markets as a whole (other than those that disproportionately affect the Buyer and its Subsidiaries, taken as a whole); (b) compliance by the Buyer with the terms of, or the taking of any action required by, this Agreement, or otherwise taken by the Buyer at the Company's or any Company Stockholder's express written direction; (c) any change in GAAP or applicable Laws (or interpretation thereof); and (d) any acts of God, calamities, acts of war or terrorism, or national or international political or social conditions.

"**Buyer Shares**" shall mean the Buyer Capital Shares issuable to the Company Stockholders pursuant to this Agreement.

"**Buyer Share Value**" shall mean: (i) if the Buyer Shares are not publicly traded on a national securities exchange, the fair market value as determined in good faith by the Company's Board of Directors and (ii) if the Buyer Shares are publicly traded on a national securities exchange, the average three (3) day closing price per Buyer Share for the three (3) days immediately prior to the date of determination of value as reported by such national securities exchange, in all cases, as adjusted for any stock split, stock dividend, combination, or other recapitalization or reclassification effected after the date hereof.

"**CERCLA**" shall mean the federal Comprehensive Environmental Response, Compensation and Liability Act of 1980, as amended.

"**Certificate of Incorporation**" shall mean the certificate of incorporation of the Company, Merger Sub 1 or Merger Sub 2, as the context requires, and as amended or restated.

"**Certificate of Merger 1**" shall mean the certificate of merger or other appropriate documents prepared and executed in accordance with Section 251(c) of the Delaware General Corporation Law to effect Merger 1.

"**Certificate of Merger 2**" shall mean the certificate of merger or other appropriate documents prepared and executed in accordance with Section 251(c) of the Delaware General Corporation Law to effect Merger 2.

"**Change of Control**" shall mean any transaction or series of related transactions involving: (i) the sale, lease, exclusive license, or other disposition of all or substantially all of the assets of the Buyer, (ii) any merger or consolidation of the Buyer into or with another person or entity (other than a merger or consolidation effected with an Affiliate of the Buyer or to change the Buyer's domicile), or any other corporate reorganization, in which the shareholders of the Buyer in their capacity as such immediately prior to such merger, consolidation or reorganization, own less than a majority of the Buyer's (or the surviving or successor entity's) outstanding voting power on a fully-diluted basis immediately after such merger, consolidation or reorganization; or (iii) any sale or other transfer by the shareholders in which the shareholders

of the Buyer in their capacity as such immediately prior to such sale or transfer own less than a majority of the Buyer's (or the surviving or successor entity's) outstanding voting power on a fully-diluted basis immediately after such sales or transfer.

"**Claim Notice**" shall mean a written notification which contains (i) a description of the Losses incurred or reasonably expected to be incurred by the Indemnified Party and the Claimed Amount of such Losses, to the extent then known, (ii) a statement that the Indemnified Party is entitled to indemnification under Article VII for such Losses and a reasonable explanation of the basis therefor, and (iii) a demand for payment in the amount of such Losses.

"**Claimed Amount**" shall mean the amount of any Losses incurred or reasonably expected to be incurred by the Indemnified Party.

"**Closing**" shall mean the closing of the transactions contemplated by this Agreement.

"**Closing Date**" shall mean the first date on which all of the conditions to the obligations of the Parties to consummate the transactions contemplated hereby (excluding the delivery at the Closing of any of the documents set forth in Article VI) are satisfied or waived, or such other date as may be mutually agreeable to the Parties.

"**Company**" shall mean Vector Neurosciences Inc., a/k/a and f/k/a "Vector Neurosciences LLC", a Delaware corporation, *provided that* no references to the "Company" shall be deemed or interpreted to be a reference to Neurologix, except as otherwise expressly stated.

"**Company Closing Certificate**" shall mean a certificate to the effect that each of the conditions specified in Section 6.1(a) through Section 6.1(e) is satisfied in all respects.

"**Company Debt**" shall mean the aggregate amount of (i) any Indebtedness of the Company, (ii) any guaranties or arrangements having the economic effect of a guaranty by the Company of any Indebtedness of any other Person, and (iii) any accrued interest or penalties on any of the foregoing.

"**Company Intellectual Property**" shall mean the Company Owned Intellectual Property and the Company Licensed Intellectual Property.

"**Company Licensed Intellectual Property**" shall mean all Intellectual Property that is licensed to the Company by any third party or that the Company is otherwise permitted by any third party to use.

"**Company Material Adverse Effect**" shall mean any material adverse change, event, circumstance, occurrence, state of facts or development that, individually or in the aggregate with all other changes, events, circumstances, occurrences, states of facts or developments occurring prior to the determination of a Company Material Adverse Effect, has a material adverse effect on, (i) the business, assets, prospects, Intellectual Property, liabilities, capitalization, condition (financial or other), or results of operations of the Company, taken as a

whole, or (ii) the ability of the Company to consummate the transactions contemplated hereby; provided, however, that any adverse effects attributable to any of the following as they relate to the Company shall not be deemed to constitute, and the following shall not be taken into account in determining whether there has been or will be, a Company Material Adverse Effect: (a) changes after the date of this Agreement in conditions affecting the industries in which the Company participates or the U.S. economy or financial markets as a whole (other than those that disproportionately affect the Company); (b) compliance by the Company with the terms of, or the taking of any action required by, this Agreement, or otherwise taken by the Company at the Buyer's express written direction; (c) any change in GAAP or applicable Laws (or interpretation thereof); and (d) any acts of God, calamities, acts of war or terrorism, or national or international political or social conditions.

"**Company Owned Intellectual Property**" shall mean all Intellectual Property owned or purported to be owned by the Company, in whole or in part.

"**Company Registrations**" shall mean, collectively, all Company Owned Intellectual Property that is Registered.

"**Company Shares**" shall mean the shares of common stock, $0000100000 par value per share, of the Company.

"**Company Stockholders**" shall mean the holders of record of the Company Shares outstanding immediately prior to the Merger 1 Effective Time.

"Consulting Agreement" shall have the meaning set forth in Section 6.1(l).

"**Contract Dispute**" shall have the meaning set forth in Section 10.11.

"**Contract Dispute Notice**" shall have the meaning set forth in Section 10.12.

"**Covered Products**" means the GAD Products and the IP-related Covered Products.

"**Dispute**" shall mean the dispute resulting if the Indemnifying Party in a Response disputes the Indemnifying Party's liability for all or part of the Claimed Amount.

"**Dissenters' Rights Statute**" shall mean Section 262 of the Delaware General Corporation Law.

"**Employee Benefit Plan**" shall mean (a) all employee benefit plans as defined in Section 3(3) of ERISA; and (b) all other pension, retirement, group insurance, severance pay, stay or retention bonus, deferred compensation, excess or supplemental benefit, vacation, stock, stock option, equity-based compensation, phantom stock, plant closing benefits, patent award programs, salary continuation or insurance for disability, consulting, or other compensation arrangements, workers' compensation, fringe benefit and incentive plans, programs, or arrangements, life insurance, tuition reimbursement or scholarship programs, employee discount programs, meals, travel, or vehicle allowances, any plans subject to Section 125 of the Internal

Revenue Code and any plans providing benefits or payments in the event of a change of control, change in ownership or effective control, or sale of a substantial portion (including all or substantially all) of the assets of any business or portion thereof which pertain to any current or former employee, director, officer or independent contractor of the Company and as to which the Company is a sponsor, a party or by which it is bound or with respect to which the Company has or may have any liability.

"**Environmental Law**" shall mean any Law relating to the environment, occupational health and safety, or exposure of Persons or property to Materials of Environmental Concern, including any statute, regulation, administrative decision or order pertaining to: (i) the presence of or the treatment, storage, disposal, generation, transportation, handling, distribution, manufacture, processing, use, import, export, labeling, recycling, registration, investigation or remediation of Materials of Environmental Concern or documentation related to the foregoing; (ii) air, water or noise pollution; (iii) surface water, groundwater or soil contamination; (iv) the release, threatened release, or accidental release of Materials of Environmental Concern, including emissions, discharges, injections, spills, escapes or dumping of Materials of Environmental Concern; (v) transfer of interests in or control of real property which may be contaminated; (vi) community or worker right-to-know disclosures with respect to Materials of Environmental Concern; (vii) the protection of wild life, marine life and wetlands, and endangered and threatened species; and (viii) storage tanks, vessels, containers, abandoned or discarded barrels and other closed receptacles. As used above, the term "release" shall have the meaning set forth in CERCLA.

"**ERISA**" shall mean the Employee Retirement Income Security Act of 1974, as amended.

"**ERISA Affiliate**" shall mean any entity that is, or at any applicable time was, a member of (i) a controlled group of corporations (as defined in Section 414(b) of the Internal Revenue Code), (ii) a group of trades or businesses under common control (as defined in Section 414(c) of the Internal Revenue Code), or (iii) an affiliated service group (as defined under Section 414(m) of the Internal Revenue Code or the regulations under Section 414(o) of the Internal Revenue Code), any of which includes or included the Company.

"**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended.

"**Expected Claim Notice**" shall mean a notice that, as a result of a Legal Proceeding instituted by or written claim made by a third party, the Indemnified Party reasonably expects to incur Losses for which it is entitled to indemnification under Article VII.

"**FDA**" means the United States Food and Drug Administration and any successor thereto.

"**First Commercial Sale**" means, with respect to any country, the first sale of a GAD Product to a third party purchaser after Regulatory Approval is granted with respect to such country for such GAD Product.

"**Foreclosure Action**" means, collectively, (i) the auction of certain assets formerly owned by Neurologix on April 14, 2016, (ii) the second auction of certain intellectual property assets formerly owned by Neurologix on July 13, 2016, and (iii) the acquisition of such assets by the Company through the exercise by the Company of its right, as a secured creditor, to credit bid at each foregoing auction.

"**EMA**" means the European Medicines Agency and any successor thereto.

"**GAD Products**" means any products produced by the Buyer or its Affiliates (directly or through any third party contract manufacturers) that uses the adeno-associated virus gene therapy encoding glutamic acid decarboxylase (formerly known as NLX-P101) for the treatment of Parkinson's disease that was previously under development by Neurologix.

"**GAAP**" shall mean United States generally accepted accounting principles, consistently applied.

"**Government Contract**" shall mean any prime contract or subcontract between the Company and any Governmental Entity, any prime contractor to a Governmental Entity or any subcontractor to any such Governmental Entity or prime contractor.

"**Governmental Entity**" shall mean any domestic or foreign court, arbitrational tribunal, administrative agency or commission or other governmental or regulatory authority or agency.

"**Hill Records**" means the documents and records, and any equipment and other tangible personal property, formerly owned by Neurologix and held in storage at Hill Archive in West Berlin, New Jersey.

"**Indebtedness**" shall mean, without duplication, with respect to any Person (i) all obligations for borrowed money or extensions of credit (including bank overdrafts and advances), (ii) all obligations evidenced by bonds, debentures, notes or other similar instruments, (iii) all obligations to pay the deferred purchase price of property or services, except trade accounts payable arising in the Ordinary Course of Business, (iv) all obligations as lessee capitalized in accordance with GAAP, (v) all obligations of others secured by a Security Interest on any asset, whether or not such obligations are assumed, (vi) all obligations, contingent or otherwise, directly or indirectly guaranteeing any obligations of any other Person, all obligations to reimburse the issuer in respect of letters of credit or under performance or surety bonds, or other similar obligations, (vii) all obligations in respect of bankers' acceptances and under reverse repurchase agreements, and (viii) all obligations in respect of futures contracts, swaps, other financial contracts and other similar obligations (determined on a net basis as if such contract or obligation was being terminated early on such date).

"**Intellectual Property**" shall mean all of the following throughout the world and all rights therein and thereto: (i) Patent Rights; (ii) Trademarks and all goodwill in the Trademarks; (iii) copyrights, designs, data and database rights and registrations and applications for registration thereof, including moral rights of authors; (iv) inventions, invention disclosures,

statutory invention registrations, trade secrets and confidential business information, know-how, manufacturing and product processes and techniques, research and development information, financial, marketing and business data, pricing and cost information, business and marketing plans and customer and supplier lists and information, whether patentable or nonpatentable, whether copyrightable or noncopyrightable and whether or not reduced to practice; (v) computer software and (vi) all other intellectual property and proprietary rights recognized in any country or jurisdictions in the world, including proprietary rights relating to any of the foregoing (including remedies against infringement thereof and rights of protection of interest therein under the Laws of all jurisdictions).

"**Internal Revenue Code**" shall mean the Internal Revenue Code of 1986, as amended.

"**Intermediate Surviving Corporation**" shall mean the Company, as the surviving corporation in Merger 1.

"**IP-related Covered Products**" means any products (other than the GAD Products) produced by the Buyer or its Affiliates (directly or through any third party contract manufacturers) that (a) uses or incorporates adeno-associated virus gene therapy encoding glutamic acid decarboxylase to treat a disease other than Parkinson's disease; or (b) (i) uses any of the Intellectual Property listed on Exhibit C, and (ii) is covered by a Valid Claim.

"**JAMS**" shall have the meaning set forth in Section 10.12.

"**knowledge**" with respect to the Company and the Company Stockholders, and in the context of phrases such as "to the knowledge of the Company,", "to the knowledge of the Company Stockholders", "the Company is not aware", "the Company has no knowledge" or any phrase of similar import, shall be deemed to refer to the actual knowledge of each of Michael G. Kaplitt, Matthew During and Stephen B. Kaplitt, after (a) performing a reasonable review of the books, records, files, email and other correspondence and other documents and information of the Company to which such Person has access or such other documents and information expressly specified in this Agreement and (b) consultation with the Company's outside advisors; *provided*, *however*, that with respect to clause (a), the Parties acknowledge that (i) no inspection of the Hill Records has been undertaken by Michael G. Kaplitt, Matthew During or Stephen B. Kaplitt, (ii) no such inspection by the Company Stockholders is required by the Buyer for purposes of this Agreement, and (iii) except for any actual knowledge held by Michael G. Kaplitt, Matthew During and Stephen B. Kaplitt, neither the Company nor the Company Stockholders shall be deemed to have "knowledge" of any matter solely because such information is contained in the Hill Records.

"**knowledge**" with respect to the Buyer, Merger Sub 1 and Merger Sub 2, and in the context of phrases such as "to the knowledge of Buyer,", "the Buyer is not aware", "the Buyer has no knowledge" or any phrase of similar import, shall be deemed to refer to the actual knowledge of Alexandria Forbes and Rich Giroux after (a) performing a reasonable review of the books, records, files, email and other correspondence and other documents and information of the Buyer, Merger Sub 1 and Merger Sub 2 to which such Person has access or such other documents and information expressly specified in this Agreement and (b) consultation with the Buyer's, Merger Sub 1's and Merger Sub 2's outside advisors.

"**Law**" shall mean any applicable domestic or foreign law, order, judgment, rule, code, statute, regulation, requirement, variance, decree, writ, injunction, award, ruling, Permit or ordinance of any Governmental Entity, including the common law and any applicable stock exchange rule or requirement.

"**Legal Proceeding**" shall mean any action, audit, suit, proceeding, claim, arbitration or investigation before any Governmental Entity or before any arbitrator.

"**Losses**" shall mean any and all debts, obligations and other liabilities (whether absolute, accrued, contingent, fixed or otherwise, known or unknown, or due or to become due, or otherwise), judgments, monetary damages, Taxes, fines, fees, penalties, interest obligations, deficiencies, expenses (including amounts paid in settlement, interest, court costs, reasonable costs of investigators, reasonable fees and expenses of attorneys, accountants, financial advisors and other experts, and other reasonable expenses of litigation, arbitration or other dispute resolution proceedings relating to a Third-Party Action or an indemnification claim under Article VII), but excluding any punitive, incidental, consequential, special or, indirect damages or damages relating to diminution in value or lost profits, unless paid or payable to a third party.

"**Materials of Environmental Concern**" shall mean any: pollutants or contaminants (as such terms are defined under the Clean Water Act, 33 U.S.C. Section 401 et seq.) or hazardous substances (as such terms are defined under CERCLA), pesticides (as such term is defined under the Federal Insecticide, Fungicide and Rodenticide Act), solid wastes and hazardous wastes (as such terms are defined under the Resource Conservation and Recovery Act), chemicals, other hazardous, radioactive or toxic materials, oil, petroleum and petroleum products (and fractions thereof), listed or subject to regulation under any Law, statute, rule, regulation, order, Permit, or directive due to its potential, directly or indirectly, to harm the environment or the health of humans or other living beings.

"**Merger 1 Effective Time**" shall mean the time at which Merger Sub 1 files the Certificate of Merger 1 with the Secretary of State of the State of Delaware.

"**Merger 2 Effective Time**" shall mean the time at which Merger Sub 2 files the Certificate of Merger 2 with the Secretary of State of the State of Delaware.

"**Net Sales**" means the gross amount received or receivable by Buyer (or any Affiliate, assignee or licensee of Buyer) for sales of a GAD Product to a third party purchaser, less the following to the extent included in such sale or otherwise actually allowed or incurred with respect to such sales: (a) discounts, including cash, trade and quantity discounts, price reduction programs, retroactive price adjustments with respect to sales of a GAD Product, charge-back payments and rebates granted to managed health care organizations or to federal, state and local governments (or their respective agencies, purchasers and reimbursers) or to trade customers, including but not limited to, wholesalers and chain and pharmacy buying groups, *provided that*

all of the foregoing shall be (i) negotiated and offered on an arm's length basis in accordance with the Buyer's customary business practices, (ii) offered on terms that are customary or usual for the relevant industry or product market, and (iii) otherwise permitted by applicable law; (b) credits or allowances for rejections or returns of GAD Products, including for recalls or damaged goods; (c) freight, postage, shipping and insurance charges for delivery of GAD Products, to the extent billed and paid; (d) customs duties, surcharges and other governmental charges incurred in connection with the exportation or importation of a GAD Product; (e) costs due to the factoring of receivables; (f) any write-downs or bad debts for uncollected receivables; and (g) taxes, duties or other governmental charges levied on, absorbed or otherwise imposed on sale of GAD Products, including value-added taxes, or other governmental charges otherwise measured by the billing amount, when included in billing, as adjusted for rebates and refunds, but specifically excluding taxes based on net income of the seller; in each case, only if the foregoing deductions are customary and reasonable, and are calculated in accordance with GAAP, consistently applied.

For sake of clarity and avoidance of doubt, sales by the Buyer of a GAD Product to a third party distributor of such GAD Product in a given country will be considered a sale to a third party purchaser. Any GAD Products that are: used (but not sold for consideration) for promotional or advertising purposes (including free samples); used for clinical or other research purposes; or supplied as part of a compassionate use program (or other program for providing a GAD Product before it has received Regulatory Approval in a country), will in each case not be considered in determining Net Sales hereunder.

If a GAD Product is sold as a combined product or service, Net Sales, for purposes of determining royalty payments on such GAD Product, will be calculated by multiplying the Net Sales of the combined product or service by the fraction A/A+B, in which A is the gross selling price (in the applicable country) of the GAD Product portion of the combined product or service when such GAD Product is sold separately during the applicable accounting period in which the sales of the combined product were made, and B is the gross selling price (in the applicable country) of the other products or services, as the case may be, of the combined product and/or service sold separately during the accounting period in question. All gross selling prices of the components of the combined product or service will be calculated as the average gross selling price of the components during the applicable accounting period for which the Net Sales are being calculated.

In any country, if no separate sale of either such above-designated GAD Product or such above designated elements of the end-user product and/or service are made during the accounting period in which the sale was made, or if gross retail selling price for an active functional element, component or service, as the case may be, cannot be determined for an accounting period, Net Sales allocable to the GAD Product in each such country will be determined by Buyer in good faith based on an equitable method of determining same that takes into account, on a country-by-country basis, variations in potency, the relative contribution of each active agent, component or service, as the case may be, in the combination, and relative value to the end user of each active agent, component or service, as the case may be.

"**Neurologix**" means Neurologix, Inc., a Delaware corporation.

"**Neurologix Assets**" means all assets (including all Intellectual Property and the Hill Records) formerly owned by Neurologix that were acquired by the Company pursuant to the Foreclosure Action.

"**Neurologix Bankruptcy Schedules**" means the schedules listing assets, liabilities and creditors in the Neurologix bankruptcy case filed in Delaware (Case No.12-10936 (CSS)) and attached at Annex 2.12 of the Disclosure Schedule.

"**Ordinary Course of Business**" shall mean the ordinary course of business consistent with past practice (including with respect to general timing, frequency and amount).

"**Patent Rights**" shall mean all patents, patent applications (including provisional patent applications), utility models, design registrations and certificates of invention and other governmental grants for the protection of inventions or industrial designs (including all related continuations, continuations-in-part, divisionals, reissues and reexaminations).

"**Permits**" shall mean all permits, licenses, registrations, certificates, orders, approvals, franchises, variances, waivers and similar rights issued by or obtained from any Governmental Entity (including those issued or required under Environmental Laws).

"**Person**" shall mean a natural person, partnership (general or limited), corporation, limited liability company, business trust, joint stock company, trust, unincorporated association, joint venture, Governmental Entity or other entity or organization.

"**Reasonable Best Efforts**" shall mean best efforts, to the extent commercially reasonable.

"**Registered**" shall mean issued by, registered with, renewed by or the subject of a pending application before any Governmental Entity or Internet domain name registrar.

"**Regulatory Approval**" means, with respect to a Covered Product, receipt or issuance of all approvals, licenses, registrations, clearances and authorizations by the FDA or EMA necessary to lawfully market and sell such GAD Product in the United States or Europe, as applicable.

"**Royalty**" and "**Royalties**," shall mean periodic payments to the Company Stockholders in the nature of royalties, calculated as a percentage of Net Sales of GAD Products, *provided that* such payments shall be payable to the Company Stockholders only to the extent the Buyer actually receives payments or proceeds on such Net Sales.

"**Royalty Term**" means, as to the GAD Products, the period beginning on the First Commercial Sale of such GAD Product in any country in the world and ending upon the 10th anniversary of the Buyer's receipt of Net Sales in respect of such First Commercial Sale of such GAD Product in such country.

"**Securities Act**" shall mean the Securities Act of 1933, as amended.

"**Security Interest**" shall mean any mortgage, pledge, security interest, encumbrance, charge or other lien (whether arising by contract or by operation of law), except that, for purposes of this Agreement (other than Article III), the following shall not constitute "Security Interests": (i) all statutory or other liens for current Taxes or assessments which are not yet due and payable; (ii) all landlords', workmen's, repairmen's, warehousemen's and carriers' liens and other similar liens imposed by Law, incurred in the Ordinary Course of Business; (iii) all Laws and judgments, orders, decrees, stipulations or injunctions; and (iv) all pledges or deposits in connection with workers compensation, unemployment insurance and other social security legislation.

"**Shareholder Agreement**" means the Shareholder Agreement, dated as of June 7, 2018, between Buyer, and the individual shareholders party thereto.

"**Subsidiary**" shall mean any corporation, partnership, trust, limited liability company or other non-corporate business enterprise in which the Company (or another Subsidiary) holds stock or other ownership interests representing (a) more than 50% of the voting power of all outstanding stock or ownership interests of such entity or (b) the right to receive more than 50% of the net assets of such entity available for distribution to the holders of outstanding stock or ownership interests upon a liquidation or dissolution of such entity.

"**Surviving Corporation**" shall mean Merger Sub 2, as the surviving corporation in Merger 2.

"**Taxes**" shall mean any and all taxes, charges, fees, duties, contributions, levies or other similar assessments or liabilities in the nature of a tax, including income, gross receipts, corporation, ad valorem, premium, value-added, net worth, capital stock, capital gains, documentary, recapture, alternative or add-on minimum, disability, registration, recording, excise, real property, personal property, sales, use, license, lease, service, service use, transfer, withholding, employment, unemployment, insurance, social security, national insurance, business license, business organization, environmental, workers compensation, payroll, profits, severance, stamp, occupation, escheat, windfall profits, customs duties, franchise, estimated and other taxes of any kind whatsoever imposed by the United States of America or any state, local or foreign government, or any agency or political subdivision thereof, and any interest, fines, penalties, assessments or additions to tax imposed with respect to such items or any contest or dispute thereof.

"**Tax Returns**" shall mean any and all reports, returns (including information returns), declarations, or statements relating to Taxes, including any schedule or attachment thereto and any related or supporting workpapers or information with respect to any of the foregoing, including any amendment thereof filed with or submitted to any Governmental Entity in connection with the determination, assessment, collection or payment of Taxes or in connection with the administration, implementation or enforcement of or compliance with any legal

requirement relating to any Tax, and including, for the avoidance of doubt, U.S. Department of the Treasury Form TD F 90-22.1.

"**Third-Party Action**" shall mean any lawsuit or Legal Proceeding by a Person other than a Party for which indemnification may be sought by a Party under Article VII.

"**Trademarks**" shall mean all trademarks and service marks, logos, Internet domain names, corporate names, doing business designations and other indicia of commercial source or origin (whether registered, common law, statutory or otherwise), together with all goodwill associated therewith, and all registrations and applications for registration of the foregoing.

"Valid Claim" means a claim in an issued patent listed on Exhibit C that has not: (a) expired or been canceled; (b) been declared invalid by an unreversed and unappealable or unappealed decision of a court or other appropriate body of competent jurisdiction; (c) been admitted to be invalid or unenforceable through reissue, disclaimer or otherwise; or (d) been abandoned.

ARTICLE X
MISCELLANEOUS

10.1 Press Releases and Announcements. Neither the Company (prior to the Closing) nor any Company Stockholder shall issue any press release or public announcement relating to the subject matter of this Agreement without the prior written approval of the Buyer. The Buyer shall give the Company Stockholder Representative a reasonable opportunity to review the press release initially announcing the Closing prior to the issuance thereof, and the Buyer shall give reasonable consideration to any comments of the Company Stockholder Representative relating to such press release if such comments are received by the Buyer within 24 hours of providing a draft of such press release to the Company Stockholder Representative.

10.2 Further Assurances; Post-Closing Cooperation. At any time or from time to time after the Closing, at the request of any Party, the other Parties shall execute and deliver to the requesting Party such other documents and instruments, provide such materials and information and take such other actions as the requesting Party may reasonably request to consummate the transactions contemplated by this Agreement and otherwise to cause the other Party or Parties to fulfill its or their respective obligations under this Agreement and the transactions contemplated hereby.

10.3 Third-Party Beneficiaries. The terms and provisions of this Agreement are intended solely for the benefit of the Parties and their respective successors or permitted assigns, and it is not the intention of the Parties to confer third-party beneficiary rights, and this Agreement does not confer any such rights, upon any other Person.

10.4 Entire Agreement. This Agreement (including the documents referred to herein) constitutes the entire agreement among the Parties and supersedes any prior understandings,

agreements or representations by or among the Parties, written or oral, with respect to the subject matter hereof; provided, however, that the Sections entitled "Exclusivity" and "Confidentiality" pursuant to that certain term sheet, dated June 28, 2016, between the Company and the Buyer shall survive until the Merger 1 Effective Time, at which time it shall terminate.

10.5 Succession and Assignment. This Agreement shall be binding upon and inure to the benefit of the Parties named herein and their respective successors and permitted assigns. No Party may assign any of its rights or delegate any of its obligations hereunder without the prior written approval of the other Parties; provided that Merger Sub 1 and Merger Sub 2 may assign its rights, interests and obligations hereunder to an Affiliate of the Buyer without the consent of any Party. For the avoidance of doubt, the Buyer or the Surviving Corporation may, without the consent of any Party, (a) assign all or any part of its rights or obligations hereunder to any Person (whether or not an Affiliate of the Buyer) in connection with a merger or consolidation of the Buyer or the sale, lease, exclusive license or other disposition of all or substantially all of the assets of the Buyer and (b) grant licenses of the Covered Product to Buyer's Affiliates or any Third Parties. Any purported assignment of rights or delegation of performance obligations in violation of this Section 10.5 is void.

10.6 Counterparts and Facsimile Signature. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument. This Agreement may be executed by facsimile signature.

10.7 Headings. The Section headings contained in this Agreement are inserted for convenience only and shall not affect in any way the meaning or interpretation of this Agreement.

10.8 Notices. All notices, requests, demands, claims, and other communications hereunder shall be in writing. Any notice, request, demand, claim or other communication hereunder shall be deemed duly delivered four (4) business days after it is sent by registered or certified mail, return receipt requested, postage prepaid, or one business day after it is sent for next business day delivery via a reputable nationwide overnight courier service, or on the same business day (if sent before 2 p.m. local time in the time zone of the recipient's physical address (as specified below) and otherwise on the next business day) if sent by fax with electronic or telephonic confirmation of receipt, in each case to the intended recipient as set forth below:

If to the Company (prior to the Closing)
or to the Company Stockholder
Representative or any Company
Stockholder:

Stephen B. Kaplitt
[contact information]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

If to the Buyer, Merger Sub 1 or Merger Sub 2 or (after the Closing) the Company:

MeiraGTx Holdings plc
430 East 29th Street, 10th Floor
New York, NY 10016
Attn: Richard Giroux
Tel: (646) 490-2971
Email: rich@meiragtx.com

With a copy (which shall not constitute notice) to:

Latham & Watkins LLP
John Hancock Tower
200 Clarendon Street
Boston, MA 02116
Attention: Peter N. Handrinos, Esq.
Tel: (617) 948-6060
Fax: (617) 948-6001
Email: Peter.Handrinos@lw.com

Any Party may give any notice, request, demand, claim or other communication hereunder using any other means (including personal delivery, expedited courier, messenger service, or ordinary mail), but no such notice, request, demand, claim or other communication that is given by such other means shall be deemed to have been duly given unless and until it actually is received by the party for whom it is intended. In addition, any notice, request, demand, claim or other communication hereunder delivered by email and acknowledged by reply email from the recipient shall be deemed to be effective delivery hereunder. Any Party may change the address to which notices, requests, demands, claims, and other communications hereunder are to be delivered by giving the other Parties notice in the manner herein set forth.

10.9 Amendments and Waivers. The Parties may mutually amend any provision of this Agreement at any time prior to the Closing; provided, however, that any amendment effected subsequent to the Requisite Stockholder Approval shall be subject to any restrictions contained in the Delaware General Corporation Law. No amendment of any provision of this Agreement shall be valid unless the same shall be in writing and signed by all of the Parties. No waiver of any right or remedy hereunder shall be valid unless the same shall be in writing and signed by the Party giving such waiver. No waiver by any Party with respect to any default, misrepresentation or breach of warranty or covenant hereunder shall be deemed to extend to any prior or subsequent default, misrepresentation or breach of warranty or covenant hereunder or affect in any way any rights arising by virtue of any prior or subsequent such occurrence.

10.10 Severability; Invalid Provisions. If any provision of this Agreement is finally judicially determined to be illegal, invalid or unenforceable under any present or future Law, and if the rights or obligations of any Party under this Agreement will not be materially and adversely affected thereby, (a) such provision shall be fully severable, (b) this Agreement shall be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement shall remain in full force and effect and shall not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there shall be added automatically as a part of this Agreement a legal, valid and enforceable provision as similar in terms to such illegal, invalid or unenforceable provision as may be possible.

10.11 Governing Law. This Agreement (and all claims, grievances, demands, controversies, causes of action or disputes of any nature whatsoever (including, but not limited to, tort and contract claims, and claims upon any law, statute, order, or regulation ("**Contract Disputes**")), shall be governed in all respects, including validity, interpretation, and effect, by and construed in accordance with the internal Laws of the State of Delaware without giving effect to any choice or conflict of Law provision or rule (whether of the State of Delaware or any other jurisdiction) that would cause the application of Laws of any jurisdictions other than those of the State of Delaware.

10.12 Arbitration. It is understood and agreed between the parties hereto that any and all Contract Disputes, arising out of, in connection with, or in relation to (a) this Agreement, or (b) questions of arbitrability under this Agreement, shall be resolved by final, binding, nonjudicial arbitration in accordance with the Federal Arbitration Act, 9 U.S.C. Section 1, et seq. pursuant to the following procedures:

(a) Any party may send another party or parties written notice identifying the matter in dispute and invoking the procedures of this Section (the "**Contract Dispute Notice**"). Within 14 days from delivery of the Dispute Notice, each party involved in the dispute shall meet at a mutually agreed location in New York, New York, for the purpose of determining whether they can resolve the dispute themselves by written agreement, and, if not, whether they can agree upon an impartial third-party arbitrator (the "**Arbitrator**") to whom to submit the matter in dispute for final and binding arbitration.

(b) If such parties fail to resolve the dispute by written agreement or agree on the Arbitrator within the later of fourteen (14) days from any such initial meeting or within thirty (30) days from the delivery of the Dispute Notice, any such party may make written application to the Judicial Arbitration and Mediation Services ("**JAMS**"), in New York, New York for the appointment of a single Arbitrator to resolve the dispute by arbitration. At the request of JAMS the parties involved in the dispute shall meet with JAMS at its offices within ten (10) days of such request to discuss the dispute and the qualifications and experience which each party respectively believes the Arbitrator should have; provided, however, that the selection of the Arbitrator shall be the exclusive decision of JAMS and shall be made within thirty (30) days of the written application to JAMS. The Arbitrator shall be a disinterested party.

(c) Within thirty (30) days of the selection of the Arbitrator, the parties involved in the dispute shall meet in New York, New York with such Arbitrator at a place and time designated by such Arbitrator after consultation with such parties and present their respective positions on the dispute. Each party shall have no longer than one day to present its position, the entire proceedings before the Arbitrator shall be no more than three consecutive days, and the decision of the Arbitrator shall be made in writing no more than thirty (30) days following the end of the proceeding. Such an award shall be a final and binding determination of the dispute and shall be fully enforceable as an arbitration decision in any court having jurisdiction and venue over such parties. The parties shall bear their own legal fees and expenses, and shall share equally fees and expenses of the Arbitrator and JAMS, regardless of the outcome. The non-prevailing party or parties (as determined by the Arbitrator) shall pay the

Arbitrator's fees. Except as may be required by applicable law (or as required to enter and enforce the judgment in court), no party (or its representative, witnesses or arbitrators) may disclose the existence, content or result of any arbitration under this Agreement without the prior written consent of the other party (or parties) involved in such arbitration.

10.13 Specific Performance. The Parties hereto agree that irreparable damage would occur to the Parties in the event that any of the provisions of this Agreement were not performed in accordance with its specific terms or were otherwise breached. It is agreed that the Parties shall be entitled to an injunction or injunctions to prevent breaches of this Agreement and to enforce specifically the terms and provisions hereof in any court of the United States or any state having jurisdiction without the requirement of posing a bond or other security, this being in addition to any other remedy to which the Parties are entitled at law or in equity.

10.14 Construction.

(a) The language used in this Agreement shall be deemed to be the language chosen by the Parties to express their mutual intent, and no rule of strict construction shall be applied against any Party.

(b) Any reference to any federal, state, local or foreign statute or Law shall be deemed also to refer to all rules and regulations promulgated thereunder, unless the context requires otherwise.

(c) Any reference to any Article, Section or paragraph shall be deemed to refer to an Article, Section or paragraph of this Agreement, unless the context clearly indicates otherwise.

(d) The Parties hereto agree that this Agreement is the product of negotiation between sophisticated parties and individuals, all of whom were represented by counsel, and each of whom had an opportunity to participate in and did participate in the drafting of each provision hereof. Accordingly, ambiguities in this Agreement, if any, shall not be construed strictly or in favor of or against any Party but rather shall be given a fair and reasonable construction without regard to the rule of *contra proferentem*.

(e) Unless the context of this Agreement otherwise requires, (i) words of either gender or the neuter include the other gender and the neuter, (ii) words using the singular number also include the plural number and words using the plural number also include the singular number, (iii) the terms "hereof," "herein," "hereby" and derivative or similar words refer to this entire Agreement as a whole and not to any particular Article, Section or other subdivision, (iv) the terms "Article" or "Section" or other subdivision refer to the specified Article, Section or other subdivision of the body of this Agreement, (v) the words "include," "includes," "including" and other similar words shall be deemed to be followed by the phrase "but not limited to," (vi) when a reference is made in this Agreement to Exhibits, such reference shall be to an Exhibit to this Agreement unless otherwise indicated, (vii) for any document or other item to have been "made available" heretofore or prior to the execution or date of this

Agreement such document or other item must be deposited at least twenty-four (24) hours prior to the date hereof (or if deposited more recently, provided notice of such deposit and a copy of thereof was given to the Buyer) into the data room heretofore established by the Company with written notice of such deposit and a copy of such deposit was made to the Buyer and (viii) all references to "dollars" or "$" shall mean United States dollars. All accounting terms used herein and not expressly defined herein shall have the meanings given to them under GAAP, unless otherwise expressly stated. When used herein, the terms "Party" or "Parties" refer to the Buyer, Merger Sub 1 and Merger Sub 2, on the one hand, and the Company, on the other hand, and the terms "third party," "third-party" or "third parties" refers to Persons other than the Buyer, Merger Sub 1 and Merger Sub 2, on the one hand, and the Company, on the other hand.

(f) The drafting and negotiation of the representations, warranties, covenants and conditions to the obligations of the Company, the Buyer, Merger Sub 1 and Merger Sub 2 herein reflect compromises, and certain provisions may overlap with other provisions or may address the same or similar subject matters in different ways or for different purposes. It is the intention of the Parties that, to the extent possible, unless provisions are by their terms mutually exclusive and effect cannot be given to both or all such provisions, (i) the representations, warranties, covenants and closing conditions in this Agreement shall be construed to be cumulative, (ii) each representation, warranty, covenant and closing condition in this Agreement shall be given full separate and independent effect, and (iii) no limitation in or exception to any representation, warranty, covenant or closing condition shall be construed to limit or apply to any other representation, warranty, covenant or closing condition unless such limitation or exception is expressly made applicable to such other representation, warranty, covenant or closing condition. Subject to Section 7.4, all remedies, either under this Agreement or by law or otherwise afforded, shall be cumulative and not alternative.

10.15 Expenses. The Company Stockholders, jointly and severally, shall bear all costs and expenses (including investment banking, legal and accounting fees and expenses) incurred by the Company or any Company Stockholder in connection with this Agreement and the transactions contemplated hereby. The Buyer shall bear all costs and expenses (including investment banking, legal and accounting fees and expenses) incurred by the Buyer in connection with this Agreement and the transactions contemplated hereby. For clarification purposes only, except as otherwise expressly provided herein (including, without limitation, the parties' respective indemnification obligations), the Company and the Company Stockholders shall be responsible for all costs and expenses of the Company incurred prior to the Closing, and the Buyer, Merger Sub 1, Merger Sub 2 and the Surviving Corporation shall be responsible for all costs and expenses of the Company incurred on or after the Closing.

[Remainder of Page Intentionally Left Blank]

IN WITNESS WHEREOF, the Parties have executed this Agreement under seal as of the date first above written.

MEIRAGTX HOLDINGS PLC

By: /s/ Richard Giroux
Name: Richard Giroux
Title: Chief Operating Officer

VN ACQUISITION, INC.

By: /s/ Richard Giroux
Name: Richard Giroux
Title: Secretary and Treasurer

VN ACQUISITION 2, INC.

By: /s/ Richard Giroux
Name: Richard Giroux
Title: Secretary and Treasurer

VECTOR NEUROSCIENCES INC.

By: /s/ Matthew During
Name: Matthew During
Title: President

COMPANY STOCKHOLDER REPRESENTATIVE

/s/ Stephen B. Kaplitt
Name: Stephen B. Kaplitt

SIGNATURE PAGE TO AGREEMENT AND PLAN OF MERGERS

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

COMPANY STOCKHOLDER

/s/ Michael G. Kaplitt
Name: Michael G. Kaplitt

COMPANY STOCKHOLDER

/s/ Matthew During
Name: Matthew During

COMPANY STOCKHOLDER

/s/ Stephen B. Kaplitt
Name: Stephen B. Kaplitt

SIGNATURE PAGE TO AGREEMENT AND PLAN OF MERGERS

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

MEIRAGTX HOLDINGS PLC
2018 INCENTIVE AWARD PLAN

SUB-PLAN FOR UK EMPLOYEES

1. Purpose

Pursuant to the powers granted by the Administrator in Section 10.5 of the MeiraGTx Holdings plc 2018 Incentive Award Plan (as it may be amended or restated from time to time, the "Plan"), the Administrator has adopted this Sub-Plan (the "Sub-Plan"). The purpose of the Sub-Plan is to promote the success and enhance the value of MeiraGTx Holdings plc (the "Company"), by linking the individual interests of Employees, to those of Company shareholders and by providing such individuals with an incentive for outstanding performance to generate superior returns to Company shareholders. The Sub-Plan is further intended to provide flexibility to the Company in its ability to motivate, attract, and retain the services of Employees upon whose judgment, interest, and special effort the successful conduct of the Company's operation is largely dependent. Only Employees may receive Awards under the Sub-Plan.

2. Definitions, Construction and Eligibility

(a) Capitalized terms used in the Sub-Plan which are not defined herein shall have the meaning given in the Plan, and where the context requires any references to the "Plan" in those definitions shall be a reference to the Sub-Plan. The singular pronoun shall include the plural where the context so indicates.

(b) In the event of a conflict between the terms of the Sub-Plan and the Plan with respect to Awards granted to Employees based in the United Kingdom under the Sub-Plan, the terms of the Sub-Plan will control.

2.1 Definitions

Wherever the following terms are: (i) used in the Sub-Plan; or (ii) used in the Plan but apply to Awards made under the Sub-Plan, they shall have the meanings specified below, unless the context clearly indicates otherwise:

(c) "Award" means, individually, or collectively, a grant under the Sub-Plan of an Option, a Share Appreciation Right, a Restricted Share award, a Restricted Share Unit award or an Other Share or Cash Based Award;

(d) "Service Provider" shall mean any person who is an Employee.

2.2 Eligibility

The Sub-Plan forms the rules of the employee share scheme applicable to Awards made under the Sub-Plan to Employees of the Company and any Subsidiaries based in the United Kingdom or in any other jurisdiction at the discretion of the Administrator. Other Service Providers who are not Employees (such as Consultants and non-employee Directors) are not eligible to receive Awards and become Participants under this Sub-Plan. References to the phrase "Service Provider" shall be interpreted as referring only to Employees when that phrase in the Plan is used in the context of the Sub-Plan and Awards granted to Employees under this Sub-Plan.

3. Administration and Delegation

The provisions of Article 3 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

4. Shares Available for Awards

(a) The provisions of Article 4 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

(b) The aggregate number of Shares which may be issued or transferred pursuant to Awards under the Sub-Plan, when taken together with the number of Shares which may be issued or transferred pursuant to Awards under the Plan or any other sub-plan, shall not exceed the limits specified by Article 4 of the Plan, as amended from time to time.

5. Options and Share Appreciation Rights

(a) Except as set out below, the provisions of Article 5 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

(b) Unless otherwise determined appropriate by the Administrator, any Option granted under this Sub-Plan shall be a Non-Qualified Stock Option.

6. Restricted Shares; Restricted Share Units

The provisions of Article 6 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan. On request by the Company, Participants tax resident in the United Kingdom will be required to make an election under Section 431 of Chapter 2 Income Tax (Earnings and Pensions) Act 2003 ("ITEPA") pursuant to which, for the relevant tax purposes, the market value of the Shares acquired will be calculated as if the Shares were not restricted. Participants tax resident in other jurisdictions may be required to make equivalent elections appropriate to their jurisdictions.

7. Other Share or Cash Based Awards

The provisions of Article 7 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

8. Adjustments For Changes In Ordinary Shares And Certain Other Events

The provisions of Article 8 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

9. General Provisions Applicable To Awards

(a) Except as set out below, the provisions of Article 9 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

(b) Section 9.5 of the Plan shall be amended so that the terms "taxes required by law to be withheld" and any similar phrases relating to tax obligations or tax liability when used in Section 9.5 shall include income tax, employee's National Insurance contributions and (at the discretion of the Company) employer's National Insurance contributions or other similar taxes arising in any jurisdiction (any a "Tax Liability"). The Participant will indemnify and keep indemnified the Company and his/her employing company, if different, from and against any liability for or obligation to pay any Tax Liability arising in consequence of any Award.

10. Miscellaneous

(a) Except as set out below, the provisions of Article 10 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

(b) The following language set out below is in addition to the terms of Article 10:

"Neither the Sub-Plan nor any Award made under the Sub-Plan shall give the Participant any rights to compensation or damages including for any loss or potential loss that the Participant may suffer by reason of being unable to exercise any Option or forfeiting any Award or Shares as a result of the termination of the Sub-Plan, the lapsing or termination of an Award or the Participant's Termination of Service including where any Termination of Service is subsequently held to be wrongful or unfair."

(c) The following language set out below shall replace Section 10.9:

"The Company and all its Subsidiaries may transfer, collect, use, process or disclose, in electronic or other form, such information to third parties, including where they are situated outside the European Economic Area in countries where the level of data protection may not be as high as in the Participant's country of residence, in the event that such disclosure is in their view required for the performance of their obligations under the Plan. The Company and all Group Companies shall ensure that such collection, use, processing and transfers are made in accordance with the EU General Data Protection Regulation and other applicable data protection laws in any other jurisdiction."

11. Definitions

(a) Except as set out below, the provisions of Article 11 of the Plan shall apply to this Sub-Plan as if references to the Plan are references to the Sub-Plan.

(b) Section 11.11 ("Consultant") shall not apply to this Sub-Plan.

(c) Section 11.38, ("Service Provider") shall mean an Employee.

Exhibit 10.13

MEIRAGTX HOLDINGS PLC
2018 INCENTIVE AWARD PLAN UK SUB-PLAN

OPTION GRANT NOTICE FOR UK PARTICIPANTS

Capitalized terms not specifically defined in this Option Grant Notice for UK Participants (the "***Grant Notice***") have the meanings given to them in the 2018 Incentive Award Plan UK Sub-Plan (the "***UK Sub-Plan***") of MeiraGTx Holdings plc (the "***Company***"), which incorporates terms from the Company's 2018 Incentive Award Plan (the "***Plan***").

The Company has granted to the participant listed below ("***Participant***") the option described in this Grant Notice (the "***Option***"), subject to the terms and conditions of the UK Sub-Plan and the Option Agreement attached as **Exhibit A** (the "***UK Option Agreement***"), both of which are incorporated into this Grant Notice by reference.

Participant:	
Grant Date:	
Exercise Price per Share:	
Shares Subject to the Option:	
Final Expiration Date:	
Vesting Schedule:	Subject to the terms of the Agreement, the Option will become vested and exercisable as to 25% of the Shares subject to the Option on the first anniversary of the grant date specified above and as to the remaining 75% of the Shares subject to the Option in 36 equal monthly installments occurring upon Participant's completion of each successive month of service as a Service Provider following the first anniversary of the grant date specified above.
Type of Option	Incentive Stock Option

By Participant's signature below, Participant agrees to be bound by the terms of this Grant Notice, the UK Sub-Plan and the UK Option Agreement. Participant has reviewed the UK Sub-Plan, this Grant Notice and the UK Option Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Grant Notice and fully understands all provisions of the UK Sub-Plan, this Grant Notice and the UK Option Agreement. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions arising under the UK Sub-Plan, this Grant Notice or the UK Option Agreement.

MEIRAGTX HOLDINGS PLC	**PARTICIPANT**
By: _____	_____
Name: _____	[Participant Name]
Title: _____	

Exhibit A

OPTION AGREEMENT FOR UK PARTICIPANTS

Capitalized terms not specifically defined in this UK Option Agreement have the meanings specified in the Grant Notice or, if not defined in the Grant Notice, in the UK Sub-Plan.

ARTICLE I.
GENERAL

1.1 Grant of Option. The Company has granted to Participant the Option effective as of the grant date set forth in the Grant Notice (the "***Grant Date***").

1.2 Incorporation of Terms of UK Sub-Plan. The Option is subject to the terms and conditions set forth in this UK Option Agreement and the UK Sub-Plan, which is incorporated herein by reference. In the event of any inconsistency between the UK Sub-Plan and this UK Option Agreement, the terms of the UK Option Agreement will control.

ARTICLE II.
PERIOD OF EXERCISABILITY

2.1 Commencement of Exercisability. The Option will vest and become exercisable according to the vesting schedule in the Grant Notice (the "***Vesting Schedule***") except that any fraction of a Share as to which the Option would be vested or exercisable will be accumulated and will vest and become exercisable only when a whole Share has accumulated. Notwithstanding anything in the Grant Notice, the UK Sub-Plan or this UK Option Agreement to the contrary, unless the Administrator otherwise determines, the Option will immediately expire and be forfeited as to any portion that is not vested and exercisable as of the Participant's Termination of Service for any reason.

2.2 Duration of Exercisability. The Vesting Schedule is cumulative. Any portion of the Option which vests and becomes exercisable will remain vested and exercisable until the Option expires. The Option will be forfeited immediately upon its expiration.

2.3 Expiration of Option. The Option may not be exercised to any extent by anyone after, and will expire on, the first of the following to occur:

(a) The final expiration date in the Grant Notice;

(b) Except as the Administrator may otherwise approve, the expiration of three (3) months from the date of Participant's Termination of Service, unless Participant's Termination of Service is for Cause or by reason of Participant's death or Disability;

(c) Except as the Administrator may otherwise approve, the expiration of one (1) year from the date of Participant's Termination of Service by reason of Participant's death or Disability; and

(d) Except as the Administrator may otherwise approve, Participant's Termination of Service for Cause.

ARTICLE III.
EXERCISE OF OPTION

3.1 Person Eligible to Exercise. During Participant's lifetime, only Participant may exercise the Option. After Participant's death, any exercisable portion of the Option may, prior to the time the Option expires, be exercised by Participant's Designated Beneficiary as provided in the UK Sub-Plan.

3.2 Partial Exercise. Any exercisable portion of the Option or the entire Option, if then wholly exercisable, may be exercised, in whole or in part, according to the procedures in the UK Sub-Plan at any time prior to the time the Option or portion thereof expires, except that the Option may only be exercised for whole Shares.

3.3 UK Tax Obligations.

(a) Tax Indemnity. Participant agrees to indemnify and keep indemnified the Company and his/her employing company ("***Employer***"), if different, from and against any liability for or obligation to pay any Tax Liability (a "***Tax Liability***" being any liability for income tax, employee's National Insurance contributions and (at the discretion of the Company) employer's National Insurance Contributions (or other similar obligations to pay tax and social security wherever in the world arising)) that is attributable to: (1) the grant or exercise of, or any benefit derived by Participant from, the Option or the Shares which are the subject of the Option; (2) the transfer or issue of Shares to Participant on satisfaction of the Option or any other benefit on exercise of the Option; (3) any restrictions applicable to the Shares held by the Participant ceasing to apply to those shares; or (4) the disposal of any Shares.

(b) Tax Liability. The Option cannot be exercised until Participant has made such arrangements as the Company may require for the satisfaction of any Tax Liability that may arise in connection with the exercise of the Option and/or the acquisition of the Shares by the Participant. The Company shall not be required to issue, allot or transfer Shares until Participant has satisfied this obligation.

(c) Election. Participant undertakes that, upon request by the Company, he/she will (on or within 14 days of acquiring the Shares) join with his/her Employer in electing, pursuant to Section 431(1) of the Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") that, for relevant tax purposes, the market value of the Shares acquired on exercise of the Option on any occasion will be calculated as if the Shares were not restricted and Sections 425 to 430 (inclusive) of ITEPA are not to apply to such Shares.

(d) The Company has the right and option, but not the obligation, to treat Participant's failure to provide timely payment in accordance with the UK Sub-Plan of any withholding tax arising in connection with the Option as Participant's election to satisfy all or any portion of the withholding tax by requesting the Company retain Shares otherwise issuable under the Option.

(e) Participant acknowledges that Participant is ultimately liable and responsible for all taxes owed in connection with the Option, regardless of any action the Company or any Subsidiary takes with respect to any tax withholding obligations that arise in connection with the Option. Neither the Company nor any Subsidiary makes any representation or undertaking regarding the treatment of any tax withholding in connection with the awarding, vesting or exercise of the Option or the subsequent sale of Shares. The Company and the Subsidiaries do not commit and are under no obligation to structure the Option to reduce or eliminate Participant's Tax Liability.

ARTICLE IV.
OTHER PROVISIONS

4.1 Adjustments. Participant acknowledges that the Option is subject to adjustment, modification and termination in certain events as provided in this UK Option Agreement and the UK Sub-Plan.

4.2 Notices. Any notice to be given under the terms of this UK Option Agreement to the Company must be in writing and addressed to the Company in care of the Company's Secretary at the Company's principal office or the Secretary's then-current email address or facsimile number. Any notice to be given under the terms of this UK Option Agreement to Participant must be in writing and addressed to Participant (or, if Participant is then deceased, to the person entitled to exercise the Option) at Participant's last known mailing address, email address or facsimile number in the Company's personnel files. By a notice given pursuant to this Section, either party may designate a different address for notices to be given to that party. Any notice will be deemed duly given when actually received, when sent by email, when sent by certified mail (return receipt requested) and deposited with postage prepaid in a post office or branch post office regularly maintained by the United States Postal Service, when delivered by a nationally recognized express shipping company or upon receipt of a facsimile transmission confirmation.

4.3 Titles. Titles are provided herein for convenience only and are not to serve as a basis for interpretation or construction of this UK Option Agreement.

4.4 Conformity to Securities Laws. Participant acknowledges that the UK Sub-Plan, the Grant Notice and this UK Option Agreement are intended to conform to the extent necessary with all Applicable Laws and, to the extent Applicable Laws permit, will be deemed amended as necessary to conform to Applicable Laws.

4.5 Successors and Assigns. The Company may assign any of its rights under this UK Option Agreement to single or multiple assignees, and this UK Option Agreement will inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer set forth in the UK Sub-Plan, this UK Option Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

4.6 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the UK Sub-Plan or this UK Option Agreement, if Participant is subject to Section 16 of the Exchange Act, the UK Sub-Plan, the Grant Notice, this UK Option Agreement and the Option will be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the Exchange Act (including any amendment to Rule 16b-3) that are requirements for the application of such exemptive rule. To the extent Applicable Laws permit, this UK Option Agreement will be deemed amended as necessary to conform to such applicable exemptive rule.

4.7 Entire Agreement. The UK Sub-Plan, the Grant Notice and this UK Option Agreement (including any exhibit hereto) constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

4.8 Agreement Severable. In the event that any provision of the Grant Notice or this UK Option Agreement is held illegal or invalid, the provision will be severable from, and the illegality or invalidity of the provision will not be construed to have any effect on, the remaining provisions of the Grant Notice or this UK Option Agreement.

4.9 Limitation on Participant's Rights. Participation in the UK Sub-Plan confers no rights or interests other than as herein provided. This UK Option Agreement creates only a contractual obligation on the part of the Company as to amounts payable and may not be construed as creating a trust. Neither the UK Sub-Plan nor any underlying program, in and of itself, has any assets. Participant will have only the rights of a general unsecured creditor of the Company with respect to amounts credited and benefits payable, if any, with respect to the Option, and rights no greater than the right to receive the Shares as a general unsecured creditor with respect to the Option, as and when exercised pursuant to the terms hereof.

4.10 Not a Contract of Employment. Nothing in the UK Sub-Plan, the Grant Notice or this UK Option Agreement confers upon Participant any right to continue in the employ of the Company or any Subsidiary or interferes with or restricts in any way the rights of the Company and its Subsidiaries, which rights are hereby expressly reserved, to discharge or terminate the services of Participant at any time for any reason whatsoever, with or without Cause, except to the extent expressly provided otherwise in a written agreement between the Company or a Subsidiary and Participant.

4.11 Counterparts. The Grant Notice may be executed in one or more counterparts, including by way of any electronic signature, subject to Applicable Law, each of which will be deemed an original and all of which together will constitute one instrument.

4.12 Incentive Stock Options. If the Option is designated as an Incentive Stock Option:

(a) Participant acknowledges that to the extent the aggregate fair market value of shares (determined as of the time the option with respect to the shares is granted) with respect to which options intended to qualify as "incentive stock options" under Section 422 of the Code, including the Option, are exercisable for the first time by Participant during any calendar year exceeds $100,000 or if for any other reason such options do not qualify or cease to qualify for treatment as "incentive stock options" under Section 422 of the Code, such options (including the Option) will be treated as non-qualified options. Participant further acknowledges that the rule set forth in the preceding sentence will be applied by taking the Option and other options into account in the order in which they were granted, as determined under Section 422(d) of the Code. Participant acknowledges that amendments or modifications made to the Option pursuant to the UK Sub-Plan that would cause the Option to become a Non-Qualified Stock Option will not materially or adversely affect Participant's rights under the Option, and that any such amendment or modification shall not require Participant's consent. Participant also acknowledges that if the Option is exercised more than three (3) months after Participant's Termination of Service as an Employee, other than by reason of death or disability, the Option will be taxed as a Non-Qualified Stock Option.

(b) Participant will give prompt written notice to the Company of any disposition or other transfer of any Shares acquired under this UK Option Agreement if such disposition or other transfer is made (a) within two (2) years from the Grant Date or (b) within one (1) year after the transfer of such Shares to Participant. Such notice will specify the date of such disposition or other transfer and the amount realized, in cash, other property, assumption of indebtedness or other consideration, by Participant in such disposition or other transfer.

4.13 Data Protection. The Company and all its Subsidiaries may transfer, collect, use, process or disclose, in electronic or other form, such information to third parties, including where they are situated outside the European Economic Area in countries where the level of data protection may not be as high as in the Participant's country of residence, in the event that such disclosure is in their view required for the performance of their obligations under the Plan. The Company and all Group Companies shall ensure that such collection, use, processing and transfers are made in accordance with the EU General Data Protection Regulation and other applicable data protection laws in any other jurisdiction.

4.14 Acknowledgement. Participant acknowledges that neither this UK Option Agreement nor the UK Sub-Plan has been issued, nor has it been approved by, an authorised person within the meaning of the Financial Services and Markets Act 2000 of the United Kingdom and is being directed at the Participant because the offer to which this UK Option Agreement and the UK Sub-Plan relate has been determined as having regard to the Participant's circumstances as an employee of the Company. This UK Option Agreement is strictly confidential and is not for distribution to, and may not be acted upon by, any other person other than the person to whom it has been specifically addressed.

* * * * *

Exhibit 10.19

Execution Version

CONSULTING AGREEMENT

THIS CONSULTING AGREEMENT (this "**Agreement**") is made and entered into as of October 5, 2018, by and between **MeiraGTx Holdings plc**, a company incorporated in the Cayman Islands (the "**Company**") with offices at 450 East 29th Street, 15th Floor, New York, NY 10016, **Vector Consulting LLC**, a limited liability company organized in New York with a mailing address at 62 N. Livingston Avenue, Livingston, NJ 07039 (the "**Consulting Entity**"), Michael G. Kaplitt, an individual residing at 1113 York Avenue, Apt. 26E, New York, NY 10065 , Matthew During, an individual residing at 8 Nearwater Road, Rowayton, CT 06853, and Stephen B. Kaplitt, an individual residing at 62 N. Livingston Avenue, Livingston, NJ 07039 (each such individual, a "**Consultant**" and collectively, the "**Consultants**"). The Company, the Consulting Entity and the Consultants are collectively referred to as the "**Parties**" and each a "**Party**".

INTRODUCTION

On October 5, 2018, the Company acquired Vector Neurosciences Inc., a Delaware corporation ("**Vector**"), pursuant to that certain Agreement and Plan of Merger, dated October 5, 2018 (the "**Merger Agreement**"), pursuant to which VN Acquisition, Inc., a wholly-owned subsidiary of the Company merged with and into Vector with Vector surviving as a wholly owned subsidiary of the Company ("**Merger 1**") followed by Vector merging with and into VN Acquisition 2, Inc. with VN Acquisition 2, Inc. surviving as the surviving corporation (together with Merger 1, the "**Mergers**"). Capitalized terms used herein but not otherwise defined herein shall have the meaning ascribed to such terms in the Merger Agreement.

The Consultants were the only stockholders of Vector prior to Merger 1.

The Consultants are the only equity holders and members of the Consulting Entity.

The Company, the Consulting Entity and the Consultants desire to establish the terms and conditions under which the Consulting Entity will provide services to the Company. In consideration of the mutual covenants and promises contained herein and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged by the parties hereto, the parties agree as follows:

1. Services; Competitive Activities.

1.1 The Consulting Entity agrees to make available the services of each Consultant to perform the Services, to and for the Company in connection with the Project as may be reasonably requested from time to time by the Company as set forth on Schedule A to this Agreement. The Company acknowledges and agrees that: (a) the Services are not to be provided on a full-time basis; (b) each Consultant has work and professional commitments and

activities unrelated to the Services; and (c) the Services reasonably requested by the Company hereunder shall not unduly or unreasonably interfere with any unrelated commitments and activities of the Consultants, *provided that*, subject to the foregoing reasonableness requirement, Consultants shall perform the Services with commercially reasonably diligence and commercially reasonable best efforts.

1.2 Subject to Section 3.2, the Company shall pay for all costs and expenses necessary for, or incurred in relation to, the Services and the Project, and the Consulting Entity and the Consultants shall not be responsible for any such costs or expenses.

1.3 During the Consultation Period (as defined below) and for a period of twelve (12) months thereafter, the Consulting Entity and each Consultant shall not engage in any Competitive Activity.

2. Term. This Agreement shall commence on the date hereof and shall continue until terminated in accordance with the provisions of Section 4 (the term of effectiveness of this Agreement being referred to herein as the "**Consultation Period**").

3. Compensation.

3.1 Equity Grants. The Company shall issue to the Consultants the Options set forth on Schedule A to this Agreement.

3.2 Reimbursement of Expenses. The Company shall reimburse the Consulting Entity or the Consultants individually (as applicable) for all reasonable and necessary documented out of pocket expenses incurred or paid by the Consulting Entity or a Consultant in connection with, or related to, the performance of services under this Agreement, *provided that* no single expenses greater than $1,000 shall be incurred without the prior written approval of the Company (which may be delivered by email). To the extent the Consulting Entity or a Consultant has expenses for reimbursement, the Consulting Entity or such Consultant shall submit to the Company itemized statements on a monthly basis for each month in which such expenses are incurred, in a form reasonably satisfactory to the Company, of such expenses incurred in the previous monthly period. Unless disputed in good faith, the Company shall pay to the Consulting Entity or the Consultant amounts shown on each such statement within 30 days after receipt thereof. The Parties shall promptly and diligently cooperate in good faith to address any good faith disputes related to expenses.

4. Termination.

4.1 Termination for Convenience. The Company may, subject in all events to the terms and provisions of this Agreement and without prejudice to any right or remedy it may have due to any failure of the Consulting Entity or any Consultant to perform the Consulting Entity's or the Consultants' obligations under this Agreement, terminate the Consultation Period for no reason or any reason other than those provided in Section 4.2(a) or 4.3, effective upon ten (10) Business Days' prior written notice to the Consulting Entity.

4.2 Termination for Breach.

(a) The Company may terminate this Agreement upon a material breach of this Agreement by the Consulting Entity or any Consultant by providing thirty (30) days' prior written notice to the Consulting Entity and the Consultants describing with reasonable specificity the alleged breach or breaches. The termination shall become effective at the end of the notice period unless the breaching Consulting Entity or Consultant cures such breach or breaches during such notice period. Notwithstanding the foregoing, if the breach, by its nature, is incurable, the Company may terminate this Agreement immediately upon written notice to the Consulting Entity and the Consultants.

(b) The Consulting Entity and the Consultants may terminate this Agreement for Cause or upon a material breach of this Agreement by the Company by providing thirty (30) days' prior written notice to the Company describing with reasonable specificity the event(s) constituting "Cause" or the alleged breach or breaches. The termination shall become effective at the end of the notice period unless the Company cures such event(s) constituting "Cause" or breach or breaches during such notice period. Notwithstanding the foregoing, if the breach, by its nature, is incurable, the Consulting Entity and the Consultants may terminate this Agreement immediately upon written notice to the Company.

4.3 Termination for Changes in Consulting Entity. The Company may terminate this Agreement effective upon five (5) Business Days' prior written notice to the Consulting Entity in the event that: (a) the Consulting Entity amends its operating agreement or similar organizational documents, which amendments have or could reasonably be expected to have a material adverse effect on the ability of the Consulting Entity or the Consultants to provide the Services; (b) Michael G. Kaplitt and Matthew During both cease to provide Consulting Services on behalf of the Consulting Entity for any reason other than Cause; or (c) the equity ownership of the Consulting Entity changes from the equity ownership set forth on Schedule B to this Agreement without the prior written approval of the Company.

4.4 Effects of Termination.

(a) In the event of termination by the Company pursuant to Section 4.1, or by the Consulting Entity pursuant to Section 4.2(b), the Consultation Period shall terminate immediately, and the following provisions shall survive: Sections 6 through 18 (inclusive) and Sections 4.1 and 4.3 of Schedule A.

(b) In the event of termination by the Company pursuant to Section 4.2(a) or for any reason pursuant to Section 4.3, the Consultation Period shall terminate immediately, and the following provisions shall survive: Sections 6 through 18 (inclusive).

(c) Upon termination of the Consultation Period for any reason, the Consulting Entity and the Consultants (as applicable) shall be entitled to payment for expense reimbursements due under Section 3.2 for expenses incurred prior to the effective date of termination, and, except as expressly provided in this Section 4.4, the Company shall have no further obligations to the Consulting Entity or any Consultant hereunder.

5. Cooperation. The Consulting Entity and each Consultant shall use its, his or her commercially reasonable efforts in the performance of the Consulting Entity's and each Consultant's obligations under this Agreement. The Company shall provide such access to its information and property as may be reasonably required in order to permit the Consulting Entity and each Consultant to perform such person's obligations hereunder. The Consulting Entity and each Consultant shall reasonably cooperate with the Company's personnel, shall not interfere with the conduct of the Company's business and shall observe all rules, regulations and security requirements of the Company concerning the safety of persons and property, *provided that* such rules, regulations and requirements shall have been (i) transmitted to the Consultants in writing (including by email) or (ii) otherwise made available to the Consultants in a manner reasonably expected to make the Consultants aware of such rules, regulations and requirements.

6. Inventions and Proprietary Information.

6.1 Inventions.

(a) All inventions, discoveries, computer programs, data, technology, designs, innovations and improvements (whether or not patentable and whether or not copyrightable) which are made, conceived, reduced to practice, created, written, designed or developed by the Consulting Entity or any Consultant, solely or jointly with others and whether during normal business hours or otherwise, (i) during and prior to the Consultation Period if resulting from or directly related to the Project or the Services, (ii) during the Consultation Period if resulting from or directly related to the business of the Company or (iii) after the Consultation Period if resulting or directly derived from Proprietary Information (as defined below) (collectively under clauses (i), (ii) and (iii), "**Inventions**"), shall be the sole property of the Company. The Consulting Entity and each Consultant hereby assigns to the Company all Inventions and any and all related patents, copyrights, trademarks, trade names, and other industrial and intellectual property rights and applications therefor, in the United States and elsewhere and appoints any officer of the Company as the Consulting Entity and each Consultant's duly authorized attorney to execute, file, prosecute and protect the same before any government agency, court or authority. Upon the request of the Company and at the Company's expense, the Consulting Entity and each Consultant shall execute such further assignments, documents and other instruments as may be necessary or desirable to fully and completely assign all Inventions to the Company and to assist the Company in applying for, obtaining and enforcing patents or copyrights or other rights in the United States and in any foreign country with respect to any Invention. The Consulting Entity and each Consultant also hereby waives all claims to moral rights in any Inventions.

(b) The Consulting Entity and each Consultant shall promptly disclose to the Company all Inventions and will maintain adequate and current written records (in the form of notes, sketches, drawings and as may be specified by the Company) to document the conception and/or first actual reduction to practice of any Invention. Such written records shall be available to and remain the sole property of the Company at all times.

6.2 Proprietary Information.

(a) The Consulting Entity and each Consultant acknowledges that its, his or her relationship with the Company is one of high trust and confidence and that in the course of the Consulting Entity's and such Consultant's service to the Company it, he or she will have access to and contact with Proprietary Information. Subject to subsection (c) below, the Consulting Entity and each Consultant agrees that it, he or she will not, during the Consultation Period or at any time thereafter, disclose to others, or use for its, his or her benefit or the benefit of others, any Proprietary Information or Invention.

(b) For purposes of this Agreement, Proprietary Information shall mean, by way of illustration and not limitation, all information (whether or not patentable and whether or not copyrightable) owned, possessed or used by the Company, including, without limitation, any Invention, formula, vendor information, customer information, apparatus, equipment, trade secret, process, research, report, technical data, know-how, computer program, software, software documentation, hardware design, technology, marketing or business plan, forecast, unpublished financial statement, budget, license, price, cost and employee list that is communicated to, learned of, developed or otherwise acquired by the Consulting Entity or any Consultant in the course of such person's service as a consultant to the Company.

(c) The Consulting Entity's and each Consultant's obligations under this Section 6.2 shall not apply to any information that (i) is or becomes known to the general public under circumstances involving no breach by the Consulting Entity or any Consultant or agents of the Consulting Entity of the terms of this Section 6.2, (ii) is generally disclosed to third parties by the Company without restriction on such third parties, (iii) is approved for release by written authorization of an officer of the Company or (iv) is required to be disclosed pursuant to order or decision of any judicial, administrative or regulatory authority, pursuant to subpoena or similar legal compulsion, *provided*, *that* the Consulting Entity or Consultants shall give the Company prior written notice thereof as far in advance of such disclosure as reasonably practicable and shall reasonably cooperate with the Company in the event the Company seeks (at its own cost and expense) to prevent, limit or delay such disclosure.

(d) Upon termination of this Agreement or at any other time upon request by the Company, the Consulting Entity and each Consultant shall promptly deliver to the Company all records, files, memoranda, notes, designs, data, reports, price lists, customer lists, drawings, plans, computer programs, software, software documentation, sketches, laboratory and research notebooks and other documents (and all copies or reproductions of such materials) relating to the business of the Company.

(e) The Consulting Entity and each Consultant represents that its, his or her retention as a consultant with the Company and such person's performance under this Agreement does not, and shall not, breach any agreement that obligates it, him or her to keep in

confidence any trade secrets or confidential or proprietary information of the Consulting Entity or any Consultant or of any other party or to refrain from competing, directly or indirectly, with the business of any other party or otherwise conflict with any of his or her agreements or obligations to any other party. The Consulting Entity and each Consultant shall not disclose to the Company any trade secrets or confidential or proprietary information of any other party.

(f) The Consulting Entity and each Consultant acknowledges that the Company from time to time may have agreements with other persons or with the United States Government, or agencies thereof, that impose obligations or restrictions on the Company regarding inventions made during the course of work under such agreements or regarding the confidential nature of such work. The Consulting Entity and each Consultant agrees to be bound by all such obligations and restrictions that are known to it, him or her and to take all action necessary to discharge the obligations of the Company under such agreements (at the Company's sole cost and expense, if any).

(g) *Defend Trade Secrets Act Notice of Immunity Rights*. The Consulting Entity and each Consultant acknowledges that the Company has provided the Consulting Entity and each Consultant with the following notice of immunity rights in compliance with the requirements of the Defend Trade Secrets Act: (i) such Consulting Entity or Consultant will not be held criminally or civilly liable under any Federal or State trade secret law for the disclosure of Proprietary Information that is made in confidence to a Federal, State, or local government official or to an attorney solely for the purpose of reporting or investigating a suspected violation of law, (ii) such Consulting Entity or Consultant will not be held criminally or civilly liable under any Federal or State trade secret law for the disclosure of Proprietary Information that is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal, and (iii) if such Consulting Entity or Consultant files a lawsuit for retaliation by the Company for reporting a suspected violation of law, such Consulting Entity or Consultant may disclose the Proprietary Information to its attorney and use the Proprietary Information in the court proceeding, if Consultant files any document containing the Proprietary Information under seal, and does not disclose the Proprietary Information, except pursuant to court order.

6.3 Remedies. The Consulting Entity and each Consultant acknowledges that any breach of the provisions of this Section 6 shall result in serious and irreparable injury to the Company for which the Company cannot be adequately compensated by monetary damages alone. The Consulting Entity and each Consultant agrees, therefore, that, in addition to any other remedy it may have, the Company shall be entitled to enforce the specific performance of this Agreement by the Consulting Entity and any Consultant and to seek both temporary and permanent injunctive relief (to the extent permitted by law) without the necessity of proving actual damages.

6.4 Portfolio Reference. Upon termination of this Agreement, the Consulting Entity and each Consultant may disclose to prospective third party contractors or employers the fact that the Consulting Entity and each Consultant performed certain services for the Company, subject to the receipt of prior written permission from the Company with respect to such disclosure and the scope thereof. Notwithstanding the foregoing, the Consulting Entity and each Consultant may freely disclose at any time only the fact that [***].

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

7. Non-Solicitation. During the Consultation Period and for a period of twelve (12) months thereafter, the Consulting Entity and each Consultant shall not, either alone or in association with others, (i) solicit, or permit any organization directly or indirectly controlled by the Consulting Entity and any Consultant to solicit, any employee of the Company to leave the employ of the Company, or (ii) solicit for employment, hire or engage as an independent contractor, or permit any organization directly or indirectly controlled by the Consulting Entity and any Consultant to solicit for employment, hire or engage as an independent contractor, any person who was employed by the Company at any time during the term of the Consulting Entity's and the Consultant's engagement with the Company; provided, that this clause (ii) shall not apply to (x) any individual whose employment with the Company has been terminated for a period of six months or longer or (y) general advertisements for employment or recruitment efforts conducted by any recruitment agency, provided that such advertisements or recruitment efforts are not directed at any such individual.

8. Other Agreements. The Consulting Entity and each Consultant hereby represents that, except as the Consulting Entity or any applicable Consultant has disclosed in writing to the Company, none of the Consulting Entity nor any Consultant is bound by the terms of any agreement with any current or prior employer or other party to refrain from using or disclosing any trade secret or confidential or proprietary information in the course of the Consulting Entity's and each Consultant's relationship with the Company, to refrain from competing, directly or indirectly, with the business of such employer or any other party or to refrain from soliciting employees, customers or suppliers of such employer or other party; in all cases, except where such terms would not prohibit or materially interfere with the ability of Consultants and the Consulting Entity to provide the Services and perform its and their obligations hereunder. The Consulting Entity and each Consultant agrees to furnish the Company with a copy of any such agreement within or under its or their possession or control upon request, except to the extent such disclosure is prohibited by law or contractual obligation, in which case the Consulting Entity and each Consultant agrees to furnish the Company with a copy of such agreement redacted in order to comply with such law or contractual obligation, and with respect to limitations based on contractual obligations, to request the other party to the contract to waive such obligations.

9. Independent Contractor Status; Benefits.

9.1 The Consulting Entity and each Consultant shall perform all services under this Agreement as an "independent contractor" and not as an employee or agent of the Company and, as an independent contractor, the Consulting Entity and each Consultant will be solely responsible for complying with all applicable laws, rules and regulations concerning income, employment (or self-employment) and other taxes, social security contributions, pension fund contributions, unemployment contributions and similar matters and the Company shall not be required to withhold income, employment or other taxes from payments to the Consulting Entity or the Consultants. None of the Consulting Entity nor any Consultant is authorized to assume or create any obligation or responsibility, express or implied, on behalf of, or in the name of, the Company or to bind the Company in any manner.

9.2 None of the Consulting Entity nor the Consultants shall be entitled to any benefits, coverages or privileges, including, without limitation, social security, unemployment, medical or pension payments, made available to employees of the Company. If any Consultant is reclassified by a state or federal agency or court as an employee of the Company, the Consultant will become a reclassified employee and will receive no benefits except those mandated by state or federal law, even if by the terms of the Company's benefit plans in effect at the time of such reclassification, the Consultant would otherwise be eligible for such benefits.

10. Notices. All notices, requests, demands, claims, and other communications hereunder shall be in writing. Any notice, request, demand, claim or other communication hereunder shall be deemed duly delivered four (4) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, or one business day after it is sent for next business day delivery via a reputable nationwide overnight courier service, or on the same business day (if sent before 2 p.m. local time in the time zone of the recipient's physical address (as specified below) and otherwise on the next business day) if sent by fax with electronic or telephonic confirmation of receipt, in each case to the intended recipient as set forth below:

If to the Consulting Entity or Stephen B. Kaplitt:

Stephen B. Kaplitt
[contact information]

If to Michael G. Kaplitt or Matthew During:

Michael G. Kaplitt
[contact information]

Matthew During
[contact information]

If to the Company:

MeiraGTx Holdings plc
430 East 29th Street, 10th Floor
New York, NY 10016
Attn: Richard Giroux
Tel: (646) 490-2971
Email: rich@meiragtx.com

With a copy (which shall not constitute notice) to:

Latham & Watkins LLP
John Hancock Tower
200 Clarendon Street
Boston, MA 02116
Attention: Peter N. Handrinos, Esq.
Tel: (617) 948-6060
Fax: (617) 948-6001
Email: Peter.Handrinos@lw.com

Any Party may give any notice, request, demand, claim or other communication hereunder using any other means (including personal delivery, expedited courier, messenger service, or ordinary mail), but no such notice, request, demand, claim or other communication that is given by such other means shall be deemed to have been duly given unless and until it actually is received by the party for whom it is intended. In addition, any notice, request, demand, claim or other communication hereunder delivered by email and acknowledged by reply

email from the recipient shall be deemed to be effective delivery hereunder. Any Party may change the address to which notices, requests, demands, claims, and other communications hereunder are to be delivered by giving the other Parties notice in the manner herein set forth.

11. Pronouns. Whenever the context may require, any pronouns used in this Agreement shall include the corresponding masculine, feminine or neuter forms, and the singular forms of nouns and pronouns shall include the plural, and vice versa.

12. Entire Agreement. The Merger Agreement and this Agreement constitute the entire agreement between the parties and supersedes all prior agreements and understandings, whether written or oral, relating to the subject matter of this Agreement.

13. Amendment. This Agreement may be amended or modified only by a written instrument executed by all Parties.

14. Governing Law. This Agreement shall be construed, interpreted and enforced in accordance with the laws of the State of New York without regard to conflict of law principles that would result in the application of any law other than the State of New York.

15. Successors and Assigns. This Agreement shall be binding upon, and inure to the benefit of, both parties and their respective successors and assigns, including any affiliate of the Company or any corporation with which, or into which, the Company may be merged or which may succeed to its assets or business, provided, however, that the obligations of the Consulting Entity and each Consultant are personal and shall not be assigned by it, him or her.

16. Interpretation. If any restriction set forth in Section 1 or Section 7 is found by any court of competent jurisdiction to be unenforceable because it extends for too long a period of time or over too great a range of activities or in too broad a geographic area, it shall be interpreted to extend only over the maximum period of time, range of activities or geographic area as to which it may be enforceable.

17. [Reserved].

18. Miscellaneous.

18.1 No delay or omission by any Party in exercising any right under this Agreement shall operate as a waiver of that or any other right. A waiver or consent given by a Party on any one occasion shall be effective only in that instance and shall not be construed as a bar or waiver of any right on any other occasion.

18.2 The captions of the sections of this Agreement are for convenience of reference only and in no way define, limit or affect the scope or substance of any section of this Agreement.

18.3 In the event that any provision of this Agreement shall be invalid, illegal or otherwise unenforceable, the validity, legality and enforceability of the remaining provisions shall in no way be affected or impaired thereby.

18.4 This Agreement may be executed in multiple counterparts by facsimile or other reliable electronic reproduction (including, without limitation, transmission by pdf), each of which shall be taken together as one and the same instrument.

18.5 Arbitration. Except for any dispute, controversy or claim described in Section 18.6 (which shall be handled exclusively in accordance with Section 18.6), it is understood and agreed between the Parties hereto that any and all claims, grievances, demands, controversies, causes of action or disputes of any nature whatsoever (including, but not limited to, tort and contract claims, and claims upon any law, statute, order, or regulation) (hereinafter "**Disputes**"), arising out of, in connection with, or in relation to (a) this Agreement, or (b) questions of arbitrability under this Agreement, shall be resolved by final, binding, nonjudicial arbitration in accordance with the Federal Arbitration Act, 9 U.S.C. Section 1, et seq. pursuant to the following procedures:

(a) Any Party may send another Party or Parties written notice identifying the matter in dispute and invoking the procedures of this Section (the "**Dispute Notice**"). Within 14 days from delivery of the Dispute Notice, each Party involved in the dispute shall meet at a mutually agreed location in New York, New York, for the purpose of determining whether they can resolve the dispute themselves by written agreement, and, if not, whether they can agree upon an impartial third-party arbitrator (the "**Arbitrator**") to whom to submit the matter in dispute for final and binding arbitration.

(b) If such Parties fail to resolve the dispute by written agreement or agree on the Arbitrator within the later of fourteen (14) days from any such initial meeting or within thirty (30) days from the delivery of the Dispute Notice, any such Party may make written application to the Judicial Arbitration and Mediation Services ("**JAMS**"), in New York, New York for the appointment of a single Arbitrator to resolve the dispute by arbitration. At the request of JAMS the Parties involved in the dispute shall meet with JAMS at its offices within ten (10) days of such request to discuss the dispute and the qualifications and experience which each Party respectively believes the Arbitrator should have; *provided*, *however*, that the selection of the Arbitrator shall be the exclusive decision of JAMS and shall be made within thirty (30) days of the written application to JAMS. The Arbitrator shall be a disinterested party.

(c) Within thirty (30) days of the selection of the Arbitrator, the Parties involved in the dispute shall meet in New York, New York with such Arbitrator at a place and time designated by such Arbitrator after consultation with such Parties and present their respective positions on the dispute. Each Party shall have no longer than one day to present its position, the entire proceedings before the Arbitrator shall be no more than three consecutive days, and the decision of the Arbitrator shall be made in writing no more than thirty (30) days following the end of the proceeding. Such an award shall be a final and binding determination of the dispute and shall be fully enforceable as an arbitration decision in any court having

jurisdiction and venue over such Parties. The Parties shall bear their own legal fees and expenses, and shall share equally fees and expenses of the Arbitrator and JAMS, regardless of the outcome. Except as may be required by applicable law (or as required to enter and enforce the judgment in court), no Party (or its representative, witnesses or arbitrators) may disclose the content or result of any arbitration under this Agreement without the prior written consent of the other Party (or Parties) involved in such arbitration.

18.6 Patents and Trademarks; Equitable Relief.

(a) Any dispute, controversy or claim arising out of, relating to or in connection with the scope, validity, enforceability or infringement of any patent rights shall in each case be submitted to a court of competent jurisdiction in the territory in which such patent rights were granted or arose.

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(b) Any dispute, controversy or claim arising out of, relating to or in connection with the need to seek preliminary or injunctive measures or other equitable relief (e.g., in the event of a potential or actual breach of the confidentiality and non-use provisions in Section 6.2) need not be resolved through the procedure described in Section 18.5 but may be immediately brought in a court of competent jurisdiction.

[*Remainder of Page Intentionally Left Blank*]

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IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the day and year set forth above.

THE COMPANY: MEIRAGTX HOLDINGS PLC

By: /s/ Richard Giroux
Name: Richard Giroux
Title: Chief Operating Officer

CONSULTING ENTITY: VECTOR CONSULTING LLC

By: /s/ Matthew During
Name: Matthew During
Title: President

CONSULTANT:

/s/ Michael G. Kaplitt
Michael G. Kaplitt

CONSULTANT:

/s/ Matthew During
Matthew During

CONSULTANT:

/s/ Stephen B. Kaplitt
Stephen B. Kaplitt

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SCHEDULE A

Description of Services and Payments

1. Certain Definitions. As used in this Agreement, the following terms shall have the following respective meanings:

"**Cause**" means any of: (a) a breach by the Company of any of its material obligations under this Agreement; (b) a decision by the Company to cease funding or other support for (i) the development activities related to the Services, or (ii) the development, marketing and sale of GAD Products; (c) a decision by the Company to reduce, delay or suspend the funding or other support for the development activities related to the Services in a manner that would reasonably be expected to prevent or unreasonably delay Regulatory Approval for GAD Products; (d) death or Disability of both Michael G. Kaplitt and Matthew During; or (e) insolvency or liquidation of the Company.

"**Competitive Activity**" means employment with, owning any interest in, or acting as a paid consultant, advisor, officer, director or manager of or to any business engaged in: (a) the research, development, sale or marketing of any application of glutamic acid decarboxylase (GAD) gene therapy or neuropeptide (NPY) gene therapy; (b) any business activity conducted by the Company on the date of this Agreement; or (c) any business activity relating to the Services conducted or contemplated to be conducted by the Company on the date of this Agreement or during the Consultation Period; *provided*, *however*, that notwithstanding the foregoing, "Competitive Activity" shall expressly exclude the following: (u) activities by Michael G. Kaplitt for or on behalf of Circuit Therapeutics, Inc. or its affiliates that do not directly compete with the Services or the Covered Products; (v) any passive investment by a Consultant (whether debt, equity or otherwise) in a publicly-traded security, where such investment does not result in the Consultant owning 5.0% or more of the equity interests of such publicly-traded company; (w) any investments by a Consultant (whether debt, equity or otherwise) in any non-publicly-traded company which investments do not, in the aggregate, exceed 15.0% of the total investment capital received by such company; (x) any activities conducted by or for the primary benefit of a charitable, not-for-profit or academic entity (including, without limitation, accredited hospitals and universities); (y) speaking, lecturing and writing for publication or at academic or trade meetings and conferences; and (z) any activities by Stephen B. Kaplitt acting in his capacity as an attorney.

"**Covered Products**" means the GAD Products and the IP-related Covered Products.

"**FDA**" means the United States Food and Drug Administration and any successor thereto.

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"**Disability**" means the inability to perform, with or without reasonable accommodation, the essential functions of the Services for a total of three months during any six month period as a result of incapacity due to mental or physical illness as determined by a physician selected in good faith by the Company. A Consultant's refusal to submit to examination by such physician for the purpose of determining whether a Disability exists shall be conclusive evidence that that no such Disability exists.

"**EMA**" means the European Medicines Agency and any successor thereto.

"**GAD Products**" means any products produced by the Company or its Affiliates (directly or through any third party contract manufacturers) that uses the adeno-associated virus gene therapy encoding glutamic acid decarboxylase (formerly known as NLX-P101) for the treatment of Parkinson's disease that was previously under development by Neurologix.

"**IP-related Covered Products**" means any products (other than the GAD Products) produced by the Company or its Affiliates (directly or through any third party contract manufacturers) that (a) uses or incorporates adeno-associated virus gene therapy encoding glutamic acid decarboxylase to treat a disease other than Parkinson's disease; or (b)(i) uses any of the intellectual property listed on Appendix 1, and (ii) is covered by a Valid Claim.

"**Project**" means the Company's project to develop, obtain Regulatory Approval for, and market and sell Covered Products.

"**Regulatory Approval**" means, with respect to a Covered Product, receipt or issuance of all approvals, licenses, registrations, clearances and authorizations by the FDA or EMA necessary to lawfully market and sell such GAD Product in the United States or Europe, as applicable.

"**Services**" shall have the meaning set forth in Section 2 of this Schedule A.

"Valid Claim" means a claim in an issued patent listed on Appendix 1 that has not: (a) expired or been canceled; (b) been declared invalid by an unreversed and unappealable or unappealed decision of a court or other appropriate body of competent jurisdiction; (c) been admitted to be invalid or unenforceable through reissue, disclaimer or otherwise; or (d) been abandoned.

2. Scope of Work. The following consulting, advisory and related services in support and furtherance of the Project ("**Services**") shall be performed by the Consulting Entity and the Consultants, jointly:

2.1 advise and assist with clinical development strategies for Covered Products including Regulatory Approval in the U.S. and in Europe (with the assistance of regulatory consultants);

2.2 prepare development plans to be presented to the Board of Directors;

2.3 advise and assist with the timely preparation of documents to be submitted to the FDA and other health authorities for review, including for filing of one or more New Drug Applications (NDA), as dictated by Board-approved development plans (with the assistance of regulatory consultants);

2.4 advise and assist with the strategic definition and tactical development of clinical trials programs, including protocol writing, interpretation of clinical data, and literature reviews;

2.5 advise and assist with ensuring that all clinical trials are in keeping with approved timelines and budgets, with potential obstacles identified and solutions implemented to avoid delays in clinical trial implementation;

2.6 advise and assist with ensuring the work with colleagues and collaborators are coordinated and that all people, systems, processes and materials required for clinical trials are available and appropriately prepared;

2.7 advise and assist with ensuring that clinical trials are conducted in accordance with applicable regulatory requirements and guidelines;

2.8 advise and assist with ensuring the identification, recruitment and selection of appropriate clinical investigators and contract research organizations, resulting in appropriate negotiation of contracts;

2.9 advise and assist with ensuring the timely medical review and reporting of adverse events;

2.10 advise and assist with ensuring the timely preparation of presentations reporting results of clinical trials to internal and external audiences;

2.11 participate in meetings with the Company's executive team and other employees from time to time as reasonably requested by the Company; or

2.12 provide such consulting, advisory and related services related to or in furtherance of the foregoing.

3. Schedule. The Consulting Entity and each Consultant will coordinate the furnishing of Services pursuant to this Agreement with the Company in order that such services can be provided in such a way as to generally conform to the business schedules of the Company, but the method of performance, time of performance, place of performance, hours utilized in such performance, and other details of the manner of performance of Services hereunder will be within the reasonable discretion of the Consultants and the Consulting Entity.

4. Payments

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.1 Initial Equity Grants. Upon execution of this Agreement, the Consultants shall be granted the following options ("**Options**") to purchase ordinary shares of the Company ("**Company Shares**") under the Company's 2018 Incentive Award Plan (the "**Plan**"), subject to the terms and provisions of Section 4.3 of this Schedule A and a stock option agreement in substantially the form set forth in Annex 4.1. The exercise price per share of each Option will be the Fair Market Value (as defined in the Plan) on the Option's date of grant:

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Consultant	Number of Options	Final Expiration Date
Michael G. Kaplitt	[***]	10 years from grant date
Matthew During	[***]	10 years from grant date
Stephen B. Kaplitt	[***]	10 years from grant date

4.2 Annual Equity Grants. During the Consultation Period, and subject to the approval of the Company's Board of Directors, the Consultants shall be eligible to be granted, in the discretion of the Company's Board of Directors, additional Options to purchase Company Shares under the Plan at a price per share equal to the Fair Market Value on the date of grant. The Options shall be granted annually, if at all, as determined by the Board of Directors. The Options shall be subject to all terms, vesting schedules and other provisions set forth in the Plan and in a separate option agreement, which shall be in substantially the same form as used for contemporaneous Option grants to the Company's employees generally.

4.3 Death or Permanent Disability; Termination. If, during the term of this Agreement, Michael G. Kaplitt, Stephen B. Kaplitt or Matthew During is no longer able to perform the Services due to death or Disability, the vesting of the Options granted pursuant to Section 4.1 and Section 4.2 to such Consultant that remain unvested at the time of death or Disability shall be accelerated and finally determined pursuant to the table below with no further vesting:

Timing of Death or Initial Occurrence of Disability	Final Vested Percentage of Unvested Options
Prior to the first patient being enrolled in a Phase 3 clinical trial of any GAD Product or IP-related Covered Product	25%
Following the first patient being enrolled in a Phase 3 clinical trial of any GAD Product or IP-related Covered Product, but prior to the submission by the Company (and acceptance by the FDA) of a new drug application to the FDA	50%
Following Regulatory Approval by the FDA or EMA of any GAD Product or IP-related Covered Product	100%

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Appendix 1 to Schedule A

[***]

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Annex 4.1 to Schedule A

Form of Stock Option Agreement

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MEIRAGTX HOLDINGS PLC
2018 INCENTIVE AWARD PLAN

OPTION GRANT NOTICE

Capitalized terms not specifically defined in this Option Grant Notice (the "***Grant Notice***") have the meanings given to them in the 2018 Incentive Award Plan (as amended from time to time, the "***Plan***") of MeiraGTx Holdings plc (the "***Company***").

The Company has granted to the participant listed below ("***Participant***") the option described in this Grant Notice (the "***Option***"), subject to the terms and conditions of the Plan and the Option Agreement attached as **Exhibit A** (the "***Agreement***"), both of which are incorporated into this Grant Notice by reference.

Participant:

Grant Date:

Exercise Price per Share:

Shares Subject to the Option:

Final Expiration Date:

Vesting Commencement Date:

Vesting Schedule: [To be specified in individual award agreements]

Type of Option [Incentive Stock Option/Non-Qualified Stock Option]

By Participant's signature below, Participant agrees to be bound by the terms of this Grant Notice, the Plan and the Agreement. Participant has reviewed the Plan, this Grant Notice and the Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Grant Notice and fully understands all provisions of the Plan, this Grant Notice and the Agreement. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions arising under the Plan, this Grant Notice or the Agreement.

MEIRAGTX HOLDINGS PLC

By: _____

Name: _____

Title: _____

PARTICIPANT

_____
[Participant Name]

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Exhibit A

OPTION AGREEMENT

Capitalized terms not specifically defined in this Agreement have the meanings specified in the Grant Notice or, if not defined in the Grant Notice, in the Plan.

ARTICLE I.
GENERAL

1.1 Grant of Option. The Company has granted to Participant the Option effective as of the grant date set forth in the Grant Notice (the "***Grant Date***").

1.2 Incorporation of Terms of Plan. The Option is subject to the terms and conditions set forth in this Agreement and the Plan, which is incorporated herein by reference. In the event of any inconsistency between the Plan and this Agreement, the terms of the Plan will control.

ARTICLE II.
PERIOD OF EXERCISABILITY

2.1 Commencement of Exercisability. The Option will vest and become exercisable according to the vesting schedule in the Grant Notice (the "***Vesting Schedule***") except that any fraction of a Share as to which the Option would be vested or exercisable will be accumulated and will vest and become exercisable only when a whole Share has accumulated. Notwithstanding anything in the Grant Notice, the Plan or this Agreement to the contrary, unless the Administrator otherwise determines, the Option will immediately expire and be forfeited as to any portion that is not vested and exercisable as of Participant's Termination of Service for any reason.

2.2 Duration of Exercisability. The Vesting Schedule is cumulative. Any portion of the Option which vests and becomes exercisable will remain vested and exercisable until the Option expires. The Option will be forfeited immediately upon its expiration.

2.3 Expiration of Option. The Option may not be exercised to any extent by anyone after, and will expire on, the first of the following to occur:

(a) The final expiration date in the Grant Notice;

(b) Except as the Administrator may otherwise approve, the expiration of three (3) months from the date of Participant's Termination of Service, unless Participant's Termination of Service is for Cause or by reason of Participant's death or Disability;

(c) Except as the Administrator may otherwise approve, the expiration of one (1) year from the date of Participant's Termination of Service by reason of Participant's death or Disability; and

(d) Except as the Administrator may otherwise approve, Participant's Termination of Service for Cause.

ARTICLE III.
EXERCISE OF OPTION

3.1 Person Eligible to Exercise. During Participant's lifetime, only Participant may exercise the Option. After Participant's death, any exercisable portion of the Option may, prior to the time the Option expires, be exercised by Participant's Designated Beneficiary as provided in the Plan.

3.2 Partial Exercise. Any exercisable portion of the Option or the entire Option, if then wholly exercisable, may be exercised, in whole or in part, according to the procedures in the Plan at any time prior to the time the Option or portion thereof expires, except that the Option may only be exercised for whole Shares.

3.3 Tax Withholding.

(a) The Company has the right and option, but not the obligation, to treat Participant's failure to provide timely payment in accordance with the Plan of any withholding tax arising in connection with the Option as Participant's election to satisfy all or any portion of the withholding tax by requesting the Company retain Shares otherwise issuable under the Option.

(b) Participant acknowledges that Participant is ultimately liable and responsible for all taxes owed in connection with the Option, regardless of any action the Company or any Subsidiary takes with respect to any tax withholding obligations that arise in connection with the Option. Neither the Company nor any Subsidiary makes any representation or undertaking regarding the treatment of any tax withholding in connection with the awarding, vesting or exercise of the Option or the subsequent sale of Shares. The Company and the Subsidiaries do not commit and are under no obligation to structure the Option to reduce or eliminate Participant's tax liability.

ARTICLE IV.
OTHER PROVISIONS

4.1 Adjustments. Participant acknowledges that the Option is subject to adjustment, modification and termination in certain events as provided in this Agreement and the Plan.

4.2 Notices. Any notice to be given under the terms of this Agreement to the Company must be in writing and addressed to the Company in care of the Company's Secretary at the Company's principal office or the Secretary's then-current email address or facsimile number. Any notice to be given under the terms of this Agreement to Participant must be in writing and addressed to Participant (or, if Participant is then deceased, to the person entitled to exercise the Option) at Participant's last known mailing address, email address or facsimile number in the Company's personnel files. By a notice given pursuant to this Section, either party may designate a different address for notices to be given to that party. Any notice will be deemed duly given when actually received, when sent by email, when sent by certified mail (return receipt requested) and deposited with postage prepaid in a post office or branch post office regularly maintained by the United States Postal Service, when delivered by a nationally recognized express shipping company or upon receipt of a facsimile transmission confirmation.

4.3 Titles. Titles are provided herein for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

4.4 Conformity to Securities Laws. Participant acknowledges that the Plan, the Grant Notice and this Agreement are intended to conform to the extent necessary with all Applicable Laws and, to the extent Applicable Laws permit, will be deemed amended as necessary to conform to Applicable Laws.

4.5 Successors and Assigns. The Company may assign any of its rights under this Agreement to single or multiple assignees, and this Agreement will inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer set forth in the Plan, this Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

4.6 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan or this Agreement, if Participant is subject to Section 16 of the Exchange Act, the Plan, the Grant Notice, this Agreement and the Option will be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the Exchange Act (including any amendment to Rule 16b-3) that are requirements for the application of such exemptive rule. To the extent Applicable Laws permit, this Agreement will be deemed amended as necessary to conform to such applicable exemptive rule.

4.7 Entire Agreement. The Plan, the Grant Notice and this Agreement (including any exhibit hereto) constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

4.8 Agreement Severable. In the event that any provision of the Grant Notice or this Agreement is held illegal or invalid, the provision will be severable from, and the illegality or invalidity of the provision will not be construed to have any effect on, the remaining provisions of the Grant Notice or this Agreement.

4.9 Limitation on Participant's Rights. Participation in the Plan confers no rights or interests other than as herein provided. This Agreement creates only a contractual obligation on the part of the Company as to amounts payable and may not be construed as creating a trust. Neither the Plan nor any underlying program, in and of itself, has any assets. Participant will have only the rights of a general unsecured creditor of the Company with respect to amounts credited and benefits payable, if any, with respect to the Option, and rights no greater than the right to receive the Shares as a general unsecured creditor with respect to the Option, as and when exercised pursuant to the terms hereof.

4.10 Not a Contract of Employment. Nothing in the Plan, the Grant Notice or this Agreement confers upon Participant any right to continue in the employ or service of the Company or any Subsidiary or interferes with or restricts in any way the rights of the Company and its Subsidiaries, which rights are hereby expressly reserved, to discharge or terminate the services of Participant at any time for any reason whatsoever, with or without Cause, except to the extent expressly provided otherwise in a written agreement between the Company or a Subsidiary and Participant.

4.11 Counterparts. The Grant Notice may be executed in one or more counterparts, including by way of any electronic signature, subject to Applicable Law, each of which will be deemed an original and all of which together will constitute one instrument.

4.12 Incentive Stock Options. If the Option is designated as an Incentive Stock Option:

(a) Participant acknowledges that to the extent the aggregate fair market value of shares (determined as of the time the option with respect to the shares is granted) with respect to which options intended to qualify as "incentive stock options" under Section 422 of the Code, including the Option, are exercisable for the first time by Participant during any calendar year exceeds $100,000 or if for any other reason such options do not qualify or cease to qualify for treatment as "incentive stock options" under Section 422 of the Code, such options (including the Option) will be treated as non-qualified stock options. Participant further acknowledges that the rule set forth in the preceding sentence will be applied by taking the Option and other options into account in the order in which they were granted, as determined

under Section 422(d) of the Code. Participant acknowledges that amendments or modifications made to the Option pursuant to the Plan that would cause the Option to become a Non-Qualified Stock Option will not materially or adversely affect Participant's rights under the Option, and that any such amendment or modification shall not require Participant's consent. Participant also acknowledges that if the Option is exercised more than three (3) months after Participant's Termination of Service as an Employee, other than by reason of death or disability, the Option will be taxed as a Non-Qualified Stock Option.

(b) Participant will give prompt written notice to the Company of any disposition or other transfer of any Shares acquired under this Agreement if such disposition or other transfer is made (a) within two (2) years from the Grant Date or (b) within one (1) year after the transfer of such Shares to Participant. Such notice will specify the date of such disposition or other transfer and the amount realized, in cash, other property, assumption of indebtedness or other consideration, by Participant in such disposition or other transfer.

*** * * * ***

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Schedule B

Equity Ownership of Consulting Entity

Michael G. Kaplitt	45%
Matthew During	45%
Stephen B. Kaplitt	10%

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 10.20

Confidential Treatment Requested by MeiraGTx Holdings plc

LICENCE AGREEMENT

between

UCL Business Plc

and

MeiraGTx UK II Limited

and

MeiraGTx Limited

Dated: 29 January 2019

Ref: [***] (RPE65)

Version: 9

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

THIS AGREEMENT is made 29 January, 2019

BETWEEN:

(1) **UCL BUSINESS PLC**, a company incorporated in England and Wales under company registration number 02776963 whose registered office is The Network Building, 97 Tottenham Court Road, London W1T 4TP ("**UCLB**");

and

(2) **MEIRAGTX UK II LIMITED**, a company incorporated in England and Wales with registered number 09348737 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ (the "**Licensee**"); and

(3) **MEIRAGTX LIMITED,** a company incorporated in England and Wales with registered number 09501998 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ ("**Meira**").

WHEREAS:

A. University College London ("**UCL**") has developed certain ocular gene technologies and owns certain intellectual property rights relating to those gene therapies that the Licensee wishes to acquire rights to for the development and commercialisation of Licensed Products.

B. UCL has assigned to UCLB all of its right, title and interest in and to such property.

C. The Parties previously entered into a certain License Agreement dated as of February 4, 2015 (the "**Original Commencement Date**"), amended on March 27, 2015, July 28, 2017, and December 14, 2017, as well as a certain Licence Addendum Number 5 dated December 15, 2017 (collectively, the "**Original Agreement**").

D. The Parties now desire to amend the Original Agreement to exclude the Specified Technology [***] and to terminate the Licence Addendum Number 5, and enter into a new agreement for the Specified Technology [***] on the terms set out in this Agreement.

E. It is the policy of UCLB that its activities in licensing intellectual property take into consideration ethical and socially responsible licensing principles, including ensuring that Licensed Products are made available to fulfil unmet needs in developing countries, and the Licensee acknowledges and agrees to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil such needs, all in accordance with the provisions of this Agreement.

NOW IT IS AGREED as follows:

1. DEFINITIONS

1.1 In this Agreement:

Agreement means this agreement (including the Schedules);

Affiliate in relation to a Party, means any entity or person that Controls, is Controlled by, or is under common Control with that Party;

Claims means all demands, claims and liability (whether criminal or civil, in contract, tort or otherwise) for losses, damages, costs and expenses of any nature whatsoever and all costs and expenses (including legal costs) incurred in connection therewith;

Commencement Date means the date of this Agreement;

Competing Product means any [***];

Confidential Information means the Know-how, the Materials and all other technical or commercial information that:

a) in respect of information provided in documentary form or by way of a model or in other tangible form, at the time of provision is marked or otherwise designated to show expressly that it is imparted in confidence or which a reasonable person would expect to be confidential; and

b) in respect of information that is imparted orally, any information that the Disclosing Party or its representatives informed the Receiving Party at the time of disclosure or which a reasonable person would expect to be confidential;

Control means direct or indirect beneficial ownership of 50% (or, outside a Party's home territory, such lesser percentage as is the maximum permitted level of foreign investment) or more of the share capital, stock or other participating interest carrying the right to vote or to distribution of profits of that Party, as the case may be;

Diligent Efforts means, with respect to efforts to be expended by the Licensee with respect to any objective under this Agreement, diligent, reasonable, good faith efforts to accomplish such objective [***], it being understood and agreed that with respect to the research, development, or commercialization of a product, such efforts will be substantially equivalent to those that would normally be exerted or employed by [***], when utilizing sounds and reasonable scientific, medical and business practice and judgment;

Disclosing Party has the meaning given in Clause 3.2;

Field means ocular gene therapy;

First Commercial Sale means the first sale to a third party of a Licensed Product in a given regulatory jurisdiction after all regulatory and marketing approvals have been obtained for such Licensed Product in such jurisdiction. A sale shall not be deemed to have occurred if a Licensed Product is provided pursuant to an early access or compassionate use;

Indemnitees has the meaning given in Clause 9.7;

Intellectual Property means any and all patents, utility models, registered designs, unregistered design rights, copyright, database rights, rights in respect of confidential information, rights under data exclusivity laws, rights under orphan drug laws, rights under unfair competition laws, property rights in biological or chemical materials, extension of the terms of any such rights (including supplementary protection certificates), applications for and the right to apply any of the foregoing registered property and rights, and similar or analogous rights in any part of the Territory;

Know-how means:

a) the inventions claimed in the Patents; and

b) the technical information relating to the inventions claimed in the Patents and data described in the Part B of Schedule 1;

Licensed Products means any and all products that are developed, manufactured, used, or sold by or on behalf of the Licensee or its Affiliates or Sub-licensees and which (a) are within (or are manufactured using a process described in) any claim of the Patents; and/or (b) incorporate, or their development or manufacture makes use of, any of the Know-how and/or the Materials;

Licensed Technology means the Patents, the Know-how and the Materials set out in Schedule 1;

Materials means any and all of the materials referred to in Part C of Schedule 1;

Net Sales Value means in respect of the [***]

Original Commencement Date has the meaning given in the Recitals;

Original Agreement has the meaning given in the Recitals;

Parties means UCLB, the Licensee and Meira, and "Party" shall mean either of them;

Patent Costs means [***].

Patents means any and all of the patents and patent applications referred to in Part A of Schedule 1;

Principal Investigators means [***].

Receiving Party has the meaning given in Clause 3.2;

Regulatory Exclusivity means, with respect to a Licensed Product, any exclusive rights or protection which are recognised, afforded or granted by any regulatory authority in any country or region with respect to the Licensed Product other than through patent rights;

Sub-licensee means any third party (other than an Affiliate) to whom the Licensee grants a sub-licence of its rights under this Agreement in accordance with Clause 2.3;

Territory means worldwide;

Valid Claim means a claim of a patent or patent application that has not been abandoned or allowed to lapse or expired or been held invalid or unenforceable by a court of competent jurisdiction in a final and non-appealable judgment.

2. GRANT OF RIGHTS

2.1 **Licence**

UCLB hereby grants to the Licensee and its Affiliates, and the Licensee hereby accepts on its own behalf and on behalf of its Affiliates, subject to the provisions of this Agreement:

2.1.1 an exclusive (even as to UCL) licence under the Patents, the Know-how and the Materials, with the right to sub-license, subject to Clause 2.3, to develop, commercialise, manufacture, have manufactured, use, sell and have sold Licensed Products only in the Field and in the Territory.

2.2 UCLB shall at the Licensee's request and cost execute such formal licences as may be necessary to enable the Licensee to register the licences granted to it under this Agreement with the Patent Offices in the relevant Territory. Such formal licence will reflect the terms of this Agreement where possible and for the avoidance of doubt if there is a conflict in the terms of such formal licence and this Agreement, the terms of this Agreement shall prevail. [***]

2.3 **Sub-Licensing**

The Licensee shall have the right to grant sub-licenses under the license in Clause 2.1 to its Affiliates or other third parties through one or more levels of Sub-licensees except that the Licensee may not grant such a sub-license to any person or the Affiliates of any person involved in: the tobacco industry (as defined by the Cancer Research UK Code of Practice on Tobacco Industry Funding to Universities detailed in Schedule 3); arms dealing; gambling operations; the promotion of violence; child labour or any other illegal activity. A grant of any sub-licence shall be conditioned on the following:

(a) The Licensee shall enter into a written agreement with each Sub-licensee and shall ensure that the provisions of each sub-licence are consistent with the provisions of this Agreement, and the Licensee shall ensure that:

(i) the sub-licence sets out all the proposed terms agreed between the Licensee and the Sub-licensee, including, in particular, all terms as to remuneration;

(ii) the Sub-licensee will maintain complete and accurate records in sufficient detail to permit UCLB to confirm the accuracy of the calculation of royalty payments under this Agreement; and

(iii) the sub-licence imposes obligations of confidentiality on the Sub-licensee which are no less onerous than those set out in Clause 3.2.

(b) The Licensee shall procure that each Sub-licensee complies fully at all times with the provisions of its sub-licence.

(c) The Licensee shall be liable for all acts and omissions of its Sub-licensees that, if committed by the Licensee, would constitute a breach of any of the provisions of this Agreement.

(d) The Licensee shall provide UCLB with a copy of any sub-licence [***] ([***]) days after execution of such sub-licence, provided that the Licensee may redact confidential or proprietary terms from such copy, including financial terms.

(e) Each sub-licence shall terminate automatically upon termination of this Agreement for any reason (but not expiry of this Agreement under Clause 10.1), except where the Sub-licensee was not implicated in or at fault in any circumstances which led to the termination of this Agreement, UCLB shall on receiving a written request from the relevant Sub-licensee within [***] ([***]) days following the date of termination of this Agreement enter into a licence agreement with the Sub-licensee for the Licensed Technology on terms substantially the same as the terms set out in this Agreement (except that the Sub-licensee shall not be obliged to pay to UCLB any sums equivalent to those sums set out in Clauses 4.1 or 4.3 which have already been paid to UCLB by the Licensee prior to the date of termination).

2.4 **Reservation of Rights**

2.4.1 UCLB reserves for itself and UCL the non-exclusive, irrevocable, worldwide, royalty-free right to:

(a) Use the Licensed Technology in the Field solely for academic (non-commercially funded) research, publication and teaching; and

(b) Grant licenses to academic third parties to use the Licensed Technology in academic research collaborations with UCL and such licenses shall not be sublicensable; and

(c) Grant license of the Licensed Technology to post graduate student of UCL for the purpose of conducting a programme of post graduate academic research and such licenses shall not by sublicensable.

In exercising the rights described in Clause 2.4.1(b) and (c), UCL and UCLB shall comply with the provisions of Clause 3 as regards confidentiality of the Know-how.

2.4.2 UCL and UCLB will refer a request from a third party for a licence to use the Patents in clinical trials or for diagnostic purposes involving human subjects to the Licensee, and the Licensee shall liaise directly with such third party.

2.4.3 Except for the licences expressly granted by this Clause 2, UCLB grants no rights to the Licensee under this Agreement to or under any intellectual property other than the Patents, the Know-how and the Materials and hereby reserves all rights under the Patents, the Know-how and the Materials outside the Field.

2.4.4 Nothing in this Agreement shall limit or otherwise affect UCL's ability to apply for noncommercial grant funding or comply with such grant terms and conditions. In the event that any terms of this Agreement conflicts with the terms of any non-commercial grant funding, the Parties shall negotiate in good faith to amend the terms of this Agreement to allow UCL to access such funding provided that nothing herein shall require the Licensee to agree to alter or modify the scope of the licence granted to it in this Clause 2.

2.5 **Affiliates**

The Licensee shall:

2.5.1 ensure that its Affiliates comply fully with the terms of this Agreement;

2.5.2 be responsible for any breach of or non-compliance with this Agreement by its Affiliates as if the breach or non-compliance had been a breach or non-compliance by the Licensee;

2.5.3 indemnify in accordance with Clause 9.7 each of the Indemnitees against any Claims which are awarded against or suffered by any of the Indemnitees as a result of any breach of or non-compliance with this Agreement by its Affiliates; and

2.5.4 ensure that if any Affiliate ceases to be an Affiliate as a result of a change of Control or otherwise, that unless a sub-licence agreement in accordance with Clause 2.3 is entered into with such an Affiliate, such former Affiliate immediately upon such cessation:

(a) ceases developing, manufacturing, having manufactured, using, selling and/ or having sold Licensed Products and ceases all use or exploitation of the Licensed Technology, for as long as any of the relevant Patents remains in force and/or the Know-how remains confidential;

(b) returns to the Licensee or destroys any documents or other materials in the former Affiliate's possession or under its control and that contain Confidential Information provided under this Agreement relating to the Licensed Technology and/ or Licensed Products;

(c) to the extent possible, takes all action necessary to have any product licences, marketing authorisations, pricing and/ or reimbursement approvals (and any applications for any of the foregoing) which relate to Licensed Products transferred into the name of the Licensee.

2.6 **Use of Licensed Technology in Combination**

UCLB acknowledges and agrees that the Licensee shall be entitled to use the Licensed Technology in combination with other technology, patents, know-how and materials licensed by UCLB to the Licensee under separate licence agreements and with any improvements to the Licensed Technology developed or generated by the Licensee.

3. KNOW-HOW AND CONFIDENTIAL INFORMATION

3.1 **Confidentiality of Know-how and Materials**

The Licensee undertakes that for so long as the Know-how and/or the Materials remains confidential, it shall (and shall ensure that its Affiliates and Sub-licensees) take all reasonable precautions to prevent unauthorised access to the Know-how and the Materials and protect the Know-how and the Materials in the same manner as it (or they) protect(s) its (or their) own proprietary information, and shall not (and shall ensure that its Affiliates and Sub-licensees do not) use the Know-how or the Materials for any purpose, except as expressly licensed hereby and in accordance with the provisions of this Agreement. For the avoidance of doubt, to the extent that any Materials, Know-how or information relating to the Patents falls within the public domain (without any breach of this Agreement or any other obligation of confidentiality), then UCL, the Principal Investigators and UCLB shall be free to use such information without restriction in the same way that any third party would have the freedom to use it.

3.2 **Confidentiality Obligations**

Each Party ("**Receiving Party**") undertakes:

3.2.1 to maintain as secret and confidential all Confidential Information obtained from, in the case of UCLB, the Licensee or Meira as applicable, and in the case of the Licensee and Meira, UCLB ("**Disclosing Party**") in the course of or in anticipation of this Agreement and to respect the Disclosing Party's rights therein;

3.2.2 to use such Confidential Information only for the purposes of or as permitted by this Agreement; and

3.2.3 subject to Clause 3.3, to disclose such Confidential Information only to those of its employees, contractors, Affiliates, and Sub-licensees (if any) to whom and to the extent that such disclosure is reasonably necessary for the purposes of this Agreement.

3.3 **Permitted Disclosure**

3.3.1 The Licensee shall have the right to disclose Confidential Information received from UCLB to:

(a) potential or actual customers of Licensed Products to the extent reasonably necessary to promote the sale or use of Licensed Products and provided that the customer has agreed to confidentiality provisions at least as restrictive as set forth herein;

(b) to existing or potential Sub-licensees, collaborators, investors or lenders provided that such third parties have agreed to confidentiality provisions at least as restrictive as set forth herein; and

(c) to its Board of Directors (or similar governing body) and its counsel, accountants and other professional advisers.

3.4 **Exceptions to Obligations**

The provisions of Clause 3.2 shall not apply to Confidential Information which the Receiving Party can demonstrate by reasonable written evidence:

3.4.1 was, prior to the Original Commencement Date, in the possession of the Receiving Party and at its free disposal and was not obtained or otherwise acquired directly or indirectly from the Disclosing Party or its Affiliates or their respective employees, students or representatives; or

3.4.2 is subsequently disclosed to the Receiving Party without any obligations of confidence by a third party; or

3.4.3 is or becomes generally available to the public through no act or default of the Receiving Party or its agents, employees, Affiliates or Sub-licensees; or

3.4.4 the Receiving Party is required to disclose by or to the courts of any competent jurisdiction, or to any government regulatory agency or financial authority, provided that the Receiving Party shall:

(a) inform the Disclosing Party as soon as is reasonably practicable;

(b) at the Disclosing Party's request and cost seek to persuade the court, agency or authority to have the information treated in a confidential manner, where this is possible under the court, agency or authority's procedures; and

(c) where the disclosure is unavoidable, limit the disclosure of Confidential information to the minimum extent required by law; or

3.4.5 which a Party is advised by its information officer that it is required to disclose under the Freedom of Information Act 2000 or the Environmental Information Regulations 2004.

3.5 **Disclosure to Employees**

The Receiving Party shall procure that all of its employees, contractors, Affiliates and Sub-licensees who have access to any of the Disclosing Party's Confidential Information to which Clause 3.2 applies, shall be made aware of the obligations of confidence and are bound by obligations of confidentiality at least as restrictive as those set forth herein (which it undertakes to enforce and for which it is legally responsible) and the Receiving Party shall only disclose the Disclosing Party's Confidential Information to those of its subsidiaries, employees, and officers as need to have access thereto wholly necessarily and exclusively for the purposes of this Agreement.

4. CONSIDERATION

4.1 Milestone Payments

Within [***] ([***]) days following achievement of each of the following milestone events by Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant milestone event has been achieved, provide documentary evidence of such achievement as appropriate and pay to UCLB, within a period of [***] ([***]) days, the amount(s) set out next to such milestone event below:

Milestone Event	**Amount to be paid**
[***]	£[***]
[***]	£[***]

4.2 **Annual Management Fees**

On each date referred to in the following table, the Licensee shall pay to UCLB the annual management fee set out next to such date in the table.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Date	Amount to be paid
Upon each anniversary of the Original Commencement Date until [***]	£50,000

4.3 Sales Linked Milestone Payments

Upon the first achievement of each of the sales linked milestones set out in the following table by the Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant sales linked milestone has been achieved, provide the relevant documentary evidence and pay to UCLB the amount(s) set out next to such event in the table:

Sales Linked Milestones	Amount to be paid
When Net Sales Value reaches £[***]	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (When sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]

4.4 Royalties on Net Sales

For each Licensed Product in each country, the Licensee shall pay to UCLB a royalty of [***]% ([***] per cent) being a percentage of the Net Sales Value of such Licensed Product sold by Licensee, its Affiliates or Sub-licensees. The Licensee's obligations to pay such royalty for a given Licensed Product in a given country shall begin after the First Commercial Sale of such Licensed Product in such country and shall end on the later to occur of the following: (a)

expiration of the last Valid Claim of a Patent claiming such Licensed Product in such country; or (b) the tenth (10th) anniversary of the date of such First Commercial Sale in such country; or (c) the expiration of any Regulatory Exclusivity with respect to such Licensed Products in the relevant country.

4.5 Combination Products

If any Licensed Products are incorporated in any other product ("**Combination Product**") sold by the Licensee or its Affiliates and the Licensed Product is not priced separately from the Combination Product, the Net Sales Value of such Licensed Product shall be deemed to be the fair market value of the Licensed Product in the country of sale when sold separately or if not sold separately in the country of sale, in comparable countries and territories or if neither of the foregoing apply, a reasonable amount which fairly reflects the value of the Licensed Product within the Combination Product assuming the Licensed Product is not being sold as a loss leader.

4.6 Payment Frequency

Royalties due under this Agreement, except for the payments due under Clauses, 4.1, 4.2 and 4.3, which are payable upon the date/time specified in Clauses 4.1, 4.2 and 4.3 as appropriate, shall be paid within [***] ([***]) days following the end of each calendar quarter ending on 31 March, 30 June, 30 September and 31 December in each year, in respect of sales of Licensed Products made during such quarter, and within [***] ([***]) days following the termination of this Agreement.

4.7 Payment terms

All sums due under this Agreement:

4.7.1 are exclusive of Value Added Tax which where applicable will be paid by the Licensee to UCLB in addition;

4.7.2 shall be paid in pounds sterling in cash by transferring an amount in aggregate to the following Account name: UCL Business Plc, Sort Code: 20 10 53, Account number: 30782270, Address: Barclays Bank Plc, PO Box 11345, London, W12 8GG, and in the case of income or amounts received by the Licensee or its Affiliates in a currency other than pounds sterling, the royalty shall be calculated in the other currency and then converted into equivalent pounds sterling at the relevant daily spot rate for that currency as quoted in the Financial Times newspaper on the last business day of the quarter in relation to which the royalties are payable;

4.7.3 will be made without any set-off, deduction or withholding except as may be required by law. If the Licensee is required by law to make any deduction or to withhold any part of any amount due to UCLB under this Agreement, the Licensee will give to UCLB proper evidence of the amount deducted or withheld and payment of that amount to the relevant taxation authority, and will do all things in its power to enable or assist UCLB to claim exemption from or, if that is not possible, to obtain a credit for the amount deducted or withheld under any applicable double taxation or similar agreement from time to time in force; and

4.7.4 shall be made by the due date, failing which UCLB may charge interest on any outstanding amount on a daily basis at a rate equivalent to [***]% above the Bank of England pound sterling base rate then in force in London.

4.8 Royalty Statements

The Licensee shall send to UCLB, at the same time as each royalty payment is made in accordance with Clause 4.4, a statement setting out for the relevant calendar quarter:

4.8.1 in respect of each territory or region in which Licensed Products are sold;

4.8.2 the types of Licensed Product sold;

4.8.3 the quantity of each type sold;

4.8.4 the total invoiced price for each type of Licensed Product sold;

4.8.5 where relevant, details of any Licensed Products that have been sold other than on arm's length terms for a cash consideration, including the relevant open market price or (if not available) the reasonable price attributed thereto;

4.8.6 the amounts deducted from the Net Sales Value as referred to in paragraph (i) to (iv) of that definition (broken down on a product by product and category by category basis); and

4.8.7 the aggregate royalties on Net Sales Value due to UCLB;

in each case expressed both in local currency and pounds sterling and showing the conversion rates used, during the period to which the royalty payment relates.

4.9 Records

The Licensee shall keep at its normal place of business detailed and up to date records and accounts showing the quantity, description and invoiced price or non-cash consideration for all Licensed Products sold by it or its Affiliates or on its or its Affiliates' behalf, broken down in each case on a country by country basis, and being sufficient to ascertain the payments due to UCLB under this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

The Licensee shall make such records and accounts available, on reasonable notice, for inspection during business hours by an independent chartered accountant nominated by UCLB for the purpose of verifying the accuracy of any statement or report given by the Licensee to UCLB under this Clause 4.9. The Licensee shall co-operate reasonably with any such accountant, and shall promptly provide all information and assistance reasonably requested by such accountant. The accountant shall be required to keep confidential all information learnt during any such inspection, and to disclose to UCLB only such details as may be necessary to report on the accuracy of the Licensee's statement or report. UCLB shall be responsible for the accountant's charges unless the accountant certifies that there is an inaccuracy of more than [***]% ([***] percent) in any royalty statement, in which case the Licensee shall pay his charges in respect of that inspection.

The Licensee shall ensure that UCLB has the same rights as those set out in this Clause 4.9 in respect of the Licensee's Affiliates and Sub-licensees.

The Licensee shall co-operate with UCLB in good faith to resolve any discrepancies identified during any such inspection and [***], together with interest on late payment as specified in Clause 4.7.4, within [***] following receipt of a copy of the independent chartered accountant's report.

4.10 **Accounting Standards**

Where this Agreement requires a financial calculation to be made or an action to be taken by a party, such calculation will be made or taken in accordance with the generally accepted accounting principles followed by such party.

5. COMMERCIALISATION

5.1 **General Diligence**

The Licensee shall use Diligent Efforts to develop and commercially exploit Licensed Products throughout the Territory (including obtaining all and any regulatory approvals which may be required to market and sell the Licensed Products) for the benefit of both Parties.

5.2 **Competing Activities**

The Licensee shall notify UCLB in confidence if it or any of its Affiliates or its Sub-licensees commences any marketing, sale or commercialisation of any Competing Product or enters into an agreement with any other person with respect to any such activities.

5.3 **Development Plan**

The Licensee's initial plan for developing and commercialising Licensed Products is set out in Schedule 4 (the "**Initial Development Plan**"). The Licensee shall provide to UCLB on each anniversary of the Original Commencement Date a written update to the Initial Development Plan that shall:

5.3.1 report on all activities conducted under this Agreement by the Licensee and its Affiliates and Sub-licensees since the Original Commencement Date or the date of the previous update (as appropriate);

5.3.2 (where applicable) set out the milestone events achieved since the Original Commencement Date or the date of the previous update (as appropriate) and the Licensee's reasonable estimate of the dates for achieving any future milestone events;

5.3.3 set out the current and projected activities being taken or planned to be taken by the Licensee and its Affiliates and Sub-licensees to bring Licensed Products to market in the Territory; and

5.3.4 set out the projected sales of Licensed Products (based on the Licensee's or Sub-licensee's current forecasts) for each of the next [***] ([***]) years following the date of the report.

UCLB's receipt or approval of any update to the Updated Development Plan shall not be taken to waive or qualify the Licensee's obligations under Clause 5.1.

5.4 **Annual Meeting**

In respect of the Licensed Technology, the Licensee will on UCLB's request meet with UCLB at least once per calendar year, following the submission of the update to the relevant Development Plan pursuant to Clause 5.3, to discuss progress with development and commercialisation of the Licensed Technology.

5.5 **Development Milestones**

In addition to the Licensee's obligations under Clause 5.1, the Licensee shall for each Licensed Technology use Diligent Efforts to achieve the development milestone events specified in Schedule 4 by the dates set out therein. In any instance in which it becomes apparent that a particular development milestone will not be met due to regulatory, technical, safety or efficacy-related reasons or Force Majeure event (pursuant to Clause 11.1), the Parties in good faith will agree upon an appropriate adjust of such milestone and any subsequent milestones.

5.6 **Reporting of First Commercial Sale**

The Licensee will promptly notify UCLB in writing of the First Commercial Sale of each Licensed Product on a commercial basis in each country within the Territory.

5.7 **Reporting for Impact Purposes**

5.7.1 The Licensee acknowledges that part of UCLB's purpose in licensing the Patents, Know-how and the Materials to the Licensee pursuant this Agreement is to ensure that the Patents, Know-how and the Materials are made available for use and commercial

exploitation with the intention of benefitting society and the economy. In order to enable UCLB and UCL to monitor the benefit that they are providing, and to enable UCL to demonstrate the impact of its research activities, to society and the economy, the Licensee will upon request provide to UCLB [***], a written report describing in reasonable detail how it has used the Patents, Know-how and the Materials and the societal and economic benefits generated therefrom.

5.7.2 UCLB shall notify and seek permission from the Licensee in advance, in writing if it wishes to use any written reports received from the Licensee (and the information contained therein) pursuant to Clause 5.7.1 in applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities, supplying a written copy of the application for research or other grant related funding or submission (or the relevant sections thereof). The Licensee will respond to UCLB in writing within [***] ([***]) days of receipt of such written information and subject to the removal of any confidential information as notified in such written request by the Licensee, UCLB and UCL shall be entitled to submit the approved applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities.

5.8 **Quality**

The Licensee shall ensure that all of the Licensed Products marketed by it and its Affiliates and Sub-licensees are of satisfactory quality and comply with all applicable laws and regulations in each part of the Territory.

5.9 **Marking of Licensed Products**

To the extent permitted under the laws of any country, the Licensee shall mark and cause its Affiliates and Sub-Licensees to mark each Licensed Product with the number of each issued Patent which applies to the Licensed Product and a statement that such Licensed Products are sold under licence from UCL Business plc.

5.10 **Disposals of Licensed Products for Free**

Notwithstanding the terms of Clause 5.1, the Licensee shall be entitled to supply a reasonable number of Licensed Products to third parties free of charge as promotional items for the purpose of establishing a market for the Licensed Products in the relevant country or territory or for research, evaluation and testing purposes, or for clinical development, provided that the quantity of Licensed Products supplied for free (or for the cost of manufacture) in each country or territory is not excessive and is in line with normal industry practice in such country or territory. Any Licensed Products disposed of to third parties in accordance with this Clause 5.10 shall not be taken into account for the purposes of calculating Net Sales Value.

5.11 **Referral to Expert**

If UCLB considers at any time during the period of this Agreement that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, then the matter shall be referred to an independent expert to answer the following questions:

5.11.1 whether the Licensee has complied with its obligations under Clause 5.1 or 5.3; and if not

5.11.2 what specific action the Licensee should have taken and/or now needs to take ("**Specific Action**") in order to fulfil such obligations and within what period the Specific Action should be taken ("**Action Period**").

The independent expert shall be appointed in accordance with the provisions of Schedule 2 and his decision shall be final and binding on the Parties.

5.12 **Consequences of Expert's Decision**

If the expert determines that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, and if the Licensee fails to take the Specific Action within the Action Period, UCLB shall be entitled, by giving, at any time within [***] ([***]) months after the end of that Action Period, not less than [***] ([***]) months' notice, to (a) convert the licence granted under Clause 2.1 into a non-exclusive licence or (b) terminate this Agreement.

6. ACCESS TO MEDICINES AND ETHICAL LICENSING

Licensee shall use Diligent Efforts to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil unmet needs in developing countries.

7. COMPLIANCE WITH LAWS

7.1 **General Compliance with Laws**

The Licensee will at all times (and will ensure its Affiliates and Sub-licensees) comply with all legislation, rules, regulations and statutory requirements applying to and obtain any consents necessary for its use of the Patents, the Know-how and the Materials, the development, manufacture, and sale of Licensed Products in any country or territory.

7.2 **Bribery Act**

The Licensee shall (and shall procure that any persons associated with it engaged in the performance of this Agreement including its Affiliates and Sub-licensees shall):

7.2.1 comply with all applicable laws and codes of practice relating to anti-bribery and anti-corruption including the Bribery Act 2010 and without prejudice to the foregoing generality, shall not engage in any activity, practice or conduct which would constitute an offence under sections 1, 2 or 6 of the Bribery Act 2010 or do or omit to do any act that will cause or lead UCLB to be in breach of the Bribery Act 2010;

7.2.2 comply with UCLB's ethics, anti-bribery and anti-corruption policies as notified to the Licensee from time to time and have, maintain in place and enforce throughout the term of this Agreement adequate procedures to ensure compliance with Clause 7.2.1; and

7.2.3 promptly report to UCLB any request or demand for any undue financial or other advantage of any kind received in connection with the performance of this Agreement.

For the purpose of this Clause 7.2, the meaning of adequate procedures and whether a person is associated with another person shall be determined in accordance with the Bribery Act 2010 (and any guidance issued under section 9 of that Act). Breach of this Clause 7.2 shall be deemed a material breach of this Agreement entitling UCLB to terminate under Clause 10.3.1.

7.3 **Export Control Regulations**

The Licensee shall ensure that, in using the Patents, Know-how or Materials and in selling Licensed Products, it and its Affiliates, employees, sub-contractors and Sub-licensees comply fully with any United Nations trade sanctions or EU or UK legislation or regulation, from time to time in force, which impose arms embargoes or control the export of goods, technology or software, including weapons of mass destruction and arms, military, paramilitary and security equipment and dual-use items (items designed for civil use but which can be used for military purposes) and certain drugs and chemicals.

8. INTELLECTUAL PROPERTY

8.1 **Obtain and Maintain the Patents**

8.1.1 The Licensee shall be responsible for the drafting, filing, prosecution and maintenance of all of the Patents at the Licensee's cost and expense. Subject to resource availability, UCLB shall use commercially reasonable efforts to provide such assistance as the Licensee may request to prosecute and maintain the Patents[***].

8.1.2 The Patents will be filed, prosecuted and maintained in the countries and territories where Licensee normally files its patent applications and patents for other gene therapy products. The Licensee shall notify UCLB of any decisions as to which (if any) additional countries to file and maintain Patents in.

8.1.3 The Licensee shall consult with UCLB in relation to all material changes to the patent claims or specifications that would have the effect of reducing or limiting the scope of the Patents, and not make any such changes without the prior written consent of UCLB. Such consent shall not be unreasonably withheld or delayed provided that UCLB has been given as much notice as is practicable, and in any event no less than [***] days' notice (or such shorter period for response dictated by the relevant patent office) of such proposed changes, and has been given an opportunity to file divisionals, continuations and/or such other types of protection to cover any claims or subject matter that the Licensee intends to remove from the scope of the Patents. If UCLB fails to respond before the end of the [***] day period (or such shorter period for response dictated by the relevant patent office), the Licensee may proceed with the proposed changes to the patent claims or specifications. The Licensee will ensure that UCLB receives copies of all correspondence to and from Patent Offices in respect of the Patents, including copies of all documents generated in or with such correspondence, and shall be given reasonable notice (or such shorter period for response dictated by the relevant patent office) of and the opportunity to participate in any conference calls or meetings with the Licensee's patent attorneys in relation to the drafting, filing, prosecution and maintenance of the Patents, so that UCLB may be continuously informed of progress with the drafting, filing, prosecution and maintenance of the Patents. Such involvement of UCLB under this Clause 8.1.3 shall be at UCLB's cost and expense.

8.1.4 If the Licensee wishes to abandon any application contained with the Patents or not to maintain any such Patent, it shall give [***] ([***]) months' prior written notice to UCLB and on the expiry of such notice period the licences of the relevant Patents granted to the Licensee under this Agreement shall cease.

8.1.5 In the event that any of rights granted hereunder become non-exclusive, responsibility for the drafting, filing, prosecution and maintenance of all of the Patents shall revert to UCLB.

8.2 **Infringement of the Patents, the Know-how and/or the Materials**

8.2.1 The Licensee and UCLB shall promptly give to each other written notice if it becomes aware of any infringement or potential infringement of any of the Patents or any unauthorised use of the Know-how or the Materials or any challenge to the validity or ownership of the Patents, the Know-how or the Materials and the Licensee and UCLB shall consult with each other to decide the best way to respond to such infringement, unauthorised use or challenge.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

8.2.2 The Licensee shall have the primary obligation and right to take action against any third party alleged to be infringing the Patents or making unauthorised use of the Know-how or the Materials and to defend the Patents against challenges to validity or ownership at its sole expense, provided that:

(a) the Licensee and UCLB shall use their commercially reasonable efforts to eliminate the infringement without litigation. If the efforts of the Licensee, and UCLB are not successful in eliminating the infringement within [***]([***]) days after the infringer has been formally notified of the infringement by the Licensee, the Licensee shall have the right after consulting with UCLB, to commence suit on its own account;

(b) UCLB shall on the Licensee's request cooperate with the Licensee in such action [***];

(c) the Licensee shall be solely responsible for the conduct of the action or for settlement thereof and shall be entitled to all damages received from such action, subject to Clause 8.2.4; and

(d) if the Licensee is unsuccessful in persuading the alleged infringer to desist within [***] ([***]) months of the Licensee first becoming aware of any potential infringement of the Patents for any Licensed Technology or fails without a commercially reasonable basis (relative to the Licensed Technology or other technology licenced to Licensee by UCLB) to initiate an infringement action within [***] ([***]) months of becoming aware of such infringement, UCLB shall have the right, at its sole discretion, to prosecute such infringement under its sole control [***].

8.2.3 Before starting or defending or settling any legal action under Clause 8.2.2, the Licensee shall consult with UCLB as to the advisability of the action or defence or settlement, its effect on the good name of UCLB, the public interest, and how the action or defence should be conducted.

8.2.4 The Licensee shall [***] in such action or defence.

8.2.5 UCLB shall if reasonably requested by the Licensee agree to be joined in any suit to enforce such rights or will take such action in its own name [***] and shall have the right to be separately represented by its own counsel [***]. Notwithstanding the foregoing, [***].

8.3 **Infringement of Third Party Rights**

8.3.1 If any warning letter or other notice of infringement is received by the Licensee or UCLB, or legal suit or other action is brought against the Licensee or UCLB, alleging infringement of third party rights in the manufacture, use or sale of any Licensed Product or use of any Patents, Know-how or Materials, that Party shall (in the case of UCLB) promptly provide full details to the Licensee and (in the case of the Licensee) promptly provide full details to UCLB, and the Licensee and UCLB shall discuss the best way to respond.

8.3.2 The Licensee shall have the right but not the obligation to defend such suit to the extent it relates to Licensee's or its Affiliates' or Sub-licensee's activities and shall have the right to settle with such third party, provided that [***]. In the event that the Licensee, Affiliates or Sub-licensees do not take forward an action, UCLB shall have the right, at its sole discretion, to defend such suit under its sole control and [***].

9. WARRANTIES AND LIABILITY

9.1 **Warranties by UCLB**

UCLB warrants as of the Original Commencement Date and undertakes as follows to its reasonable knowledge and without having undertaken any due and careful enquires whether specific or general in nature:

9.1.1 It is the owner of the Patents;

9.1.2 it has the authority to grant the licences under this Agreement; and.

9.1.3 so far as it is aware (having made no enquiry of any third parties or conducted any freedom to operate searches), use and exploitation of the Patents will not infringe the intellectual property rights of any third party.

UCLB warrants and undertakes:

9.1.4 it has full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on UCLB; and

9.2 **Warranties by the Licensee**

The Licensee warrants as of the Original Commencement Date and undertakes that in respect of the Licensed Technology that:

9.2.1 full power and authority to enter into and perform this Agreement, which, when executed, will constitute valid and legally binding obligations on the Licensee;

9.2.2 entry into this Agreement will not result in any breach of, or violation of the terms or provisions of, the constitutional documents of the Licensee or any other agreement or instrument by which it is bound;

9.2.3 so far as it is aware (having made no enquiry of any third parties), use and exploitation of the Patents will not infringe the intellectual property rights of any third party;

9.2.4 neither it nor any of its Affiliates is currently researching, developing, marketing, selling or otherwise commercialising any Competing Product ("**Competing Activities**"), nor has any of them entered into an agreement with any other person with respect to any Competing Activities; and

9.2.5 it shall notify UCLB if it or any of its Affiliates or its Sub-licensees commences any Competing Activities or enters into an agreement with any other person with respect to any Competing Activities.

9.3 **Warranties by Meira**

Licensee warrants as of the Original Commencement Date and undertakes that full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on Licensee.

9.4 **Acknowledgements**

The Licensee acknowledges that:

9.4.1 the inventions claimed in the Patents, and the Know-how and the Materials are at an early stage of development. Accordingly, specific results cannot be guaranteed and any results, materials, information or other items (together "**Delivered Items**") provided under this Agreement are provided "as is" and without any express or implied warranties, representations or undertakings. As examples, but without limiting the foregoing, UCLB does not give any warranty that Delivered Items are of merchantable or satisfactory quality, are fit for any particular purpose, comply with any sample or description, or are viable, uncontaminated, safe or non-toxic.

9.4.2 UCLB has not performed any searches or investigations into the existence of any third party rights that may affect any of the Patents, Know-how or Materials or the use and exploitation of any of the Patents, Know-how or Materials.

9.5 **No Other Warranties**

9.5.1 Each of the Parties acknowledges that, in entering into this Agreement, it does not do so in reliance on any representation, warranty or other provision except as expressly provided in this Agreement, and any conditions, warranties or other terms implied by statute or common law are excluded from this Agreement to the fullest extent permitted by law.

9.5.2 Without limiting the scope of Clause 9.5.1, UCLB does not make any representation nor give any warranty or undertaking:

(a) express or implied, including, without limitation, any implied warranties of merchantability or of fitness for a particular purpose with respect to any Patent, trademark, software, non-public or other information, or tangible research property, licensed or otherwise provided to the Licensee hereunder and hereby disclaims the same;

(b) as to the efficacy or usefulness of the Patents, Know-how or Materials; or

(c) whatsoever with regard to the scope of any of the Patents or that any of the Patents is or will be valid or (in the case of an application) will proceed to grantor that such Patents may be exploited by the Licensee, Affiliate or Sub-licensee without infringing other patents; or

(d) that the Materials or the method used in making or using the Materials are free from liability for patent infringement; or

(e) that the use of any of the Patents, Know-how or Materials, Licensed Technology, the manufacture, sale or use of the Licensed Products, or the exercise of any of the rights granted under this Agreement will not infringe any intellectual property or other rights of any other person; or

(f) that the Know-how or any other information communicated by UCLB to the Licensee under or in connection with this Agreement will produce Licensed Products of satisfactory quality or fit for the purpose for which the Licensee intended or that any product will not have any defect, latent or otherwise, and whether or not discoverable by inspection; or

(g) as imposing any obligation on UCLB to bring or prosecute actions or proceedings against third parties for infringement or to defend any action or proceedings for revocation of any of the Patents; or

(h) as imposing any liability on UCLB in the event that any third party supplies Licensed Products to customers located in the Territory; or

(i) that there will be no similar or competitive products or services manufactured, used, sold or supplied by any third party in the Territory.

9.6 **Responsibility for Development of Licensed Products**

The Licensee shall be exclusively responsible for its and its Affiliates' and Sub-licensees' use of the Patents, Know-how and Materials, the technical and commercial development and manufacture of Licensed Products and for incorporating any modifications or developments thereto that may be necessary or desirable, for all Licensed Products sold or supplied, notwithstanding any consultancy services or other contributions that UCLB and/or UCL may provide in connection with such activities.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

9.7 **Indemnity**

The Licensee shall indemnify each of UCLB and UCL, and each of their respective officers, directors, Council members, employees and representatives (together, the "**Indemnitees**") against all third party Claims that may be asserted against or suffered by any of the Indemnitees and which relate to:

9.7.1 the use by the Licensee or any of its Affiliates or Sub-licensees of any of the Patents, Know-how or Materials; or

9.7.2 use of the Licensed Technology by or on behalf of the Licensee or any of its Affiliates or Sub-licensees; or

9.7.3 the development, manufacture, use, marketing or sale of, or any other dealing in, any of the Licensed Products, by or on behalf of the Licensee or any of its Affiliates or Sub-licensees, or subsequently by any customer or any other person, including claims based on product liability laws.

The indemnity given by the Licensee to each Indemnitee under this Clause 9.7 will not apply to any third party Claim to the extent that it is attributable to the negligence, gross negligence, reckless misconduct or intentional misconduct of any Indemnitee.

9.8 **Limitations of Liability**

9.8.1 To the extent that UCLB or any of its Affiliates has any liability in contract, tort, or otherwise under or in connection with this Agreement, including any liability for breach of warranty, their liability shall be limited in accordance with the following provisions of this Clause 9.8.

9.8.2 The aggregate liability of UCLB and any of its Affiliates shall be limited to the total income that UCLB has received from the Licensee pursuant to this Agreement (but excluding any other costs or expenses associated with drafting, filing, prosecuting, maintaining or defending any Patents or providing any assistance to the Licensee) during the period of [***] ([***]) years preceding the date on which the liability arises, or fifty thousand pounds (£50,000) sterling, whichever is the higher.

9.8.3 The liability of the Licensee to UCLB shall be limited to the limit of its insurance as set out in Clause 9.9.1, except that in the case of product liability, the liability of the Licensee under this Agreement shall be unlimited.

9.8.4 In no circumstances shall any Party or any Indemnitee be liable for any loss, damage, costs or expenses of any nature that is (a) of an indirect, special or consequential nature or (b) any loss of profits (whether direct or indirect), revenue, business opportunity or goodwill, which arises directly or indirectly from that Party's breach or

nonperformance of this Agreement, or negligence in the performance of this Agreement or from any liability arising in any other way out of the subject matter of this Agreement even if the Party bringing the claim has advised any other Party or the relevant Indemnitee of the possibility of those losses arising, or if such losses were within the contemplation of the Parties or the Indemnitee.

9.8.5 Nothing in this Agreement excludes any Party's liability to the extent that it may not be so excluded under applicable law, including any such liability for death or personal injury caused by that Party's negligence, or liability for fraud or fraudulent misrepresentation.

9.9 Insurance

9.9.1 The Licensee shall take out with a reputable insurance company and maintain at all times during the term of this Agreement public and product liability and professional indemnity insurance including against all loss of and damage to property (whether real, personal or intellectual) and injury to persons including death arising out of or in connection with this Agreement and the Licensee's and its Affiliates' and Sub-licensees' use of the Patents, Know-how or Materials and use, sale of or any other dealing in any of the Licensed Products. Such insurances may be limited in respect of one claim provided that such limit must be at least [***] pounds (£[***]) sterling, unless the Licensee commences any business in manufacturing, distribution, supply or otherwise make available to the public any products, in which case such limit must be at least [***] pounds (£[***]) sterling. Such insurance shall continue to be maintained for a further [***] years from the end of this Agreement.

9.9.2 The Licensee will produce to UCLB at all times upon demand proof that the insurance cover required pursuant to Clause 9.9.1 is in force and evidence that all premiums have been paid up to date. If UCLB becomes aware that the Licensee has failed to maintain the insurance required pursuant to Clause 9.9.1, UCLB may effect such insurance and the Licensee will reimburse UCLB for the reasonable cost of effecting and maintaining such insurance on demand.

10. DURATION AND TERMINATION

10.1 Commencement and Expiry

This Agreement shall commence as of the Commencement Date and, unless terminated earlier in accordance with this Clause 10, the licences granted hereunder shall continue in force on a country by country basis until the later of the last payment obligation of Licensee expires under this Agreement. Upon such expiry, Licensee's licenses under this Agreement shall become full-paid, perpetual and irrevocable.

10.2 Termination of the Original Agreement

Upon the Commencement Date, pursuant to Amendment No. 4 to the Original Agreement, the Licensed Technology shall be excluded from the Original Agreement, and Licence Addendum Number 5 shall be deemed to have terminated. All other provisions of the Original Agreement (including the options set out in Clause 2.6 of the Original Agreement which have not been exercised by the Licensee prior to the Commencement Date, i.e., the options in relation to Specified Technologies [***]) together with any accrued rights of the Parties under the Original Agreement prior to the Commencement Date shall continue in full force and effect.

10.3 Early Termination

Each Party (the "**Terminating Party**") may terminate this Agreement at any time by notice in writing to the other Parties ("**Other Parties**"), such notice to take effect as specified in the notice:

10.3.1 If, in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, is in material breach of this Agreement and, in the case of a breach capable of remedy within thirty (30) days, the breach is not remedied within thirty (30) days of the Other Parties receiving notice specifying the breach and requiring its remedy or where the breach relates to non-payment of an undisputed sum due under this Agreement, the sum is not paid in full within fourteen (14) days following the Other Party receiving notice specifying the nonpayment and requiring payment in full; provided however, that in respect on breaches not relating to non-payment, if such breach is capable of being cured but cannot be cured within such thirty (30) day period and the Other Party initiates actions to cure such breach within such period and thereafter diligently pursues such actions, the Other Party shall have such additional period as is reasonable under the circumstances to cure such breach; it being understood that no such extension shall apply with respect to any undisputed payment obligations or extend beyond six (6) months from the end of such thirty (30) day period. In the event there is a genuine dispute between the Parties with respect to any alleged breach hereunder, no purported termination of this Agreement pursuant to this Clause 10.3.1 shall take effect while the Parties are actively working to resolve such dispute; or

10.3.2 if:

(a) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, becomes insolvent or unable to pay its debts as and when they become due;

(b) an order is made or a resolution is passed for the winding up of in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB (other than voluntarily for the purpose of solvent amalgamation or reconstruction);

(c) a liquidator, administrator, administrative receiver, receiver or trustee is appointed in respect of the whole or any part of, in the case of UCLB, either of the Other Parties', or in the case of the Licensee or Meira, UCLB's, assets or business;

(d) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, makes any composition with its creditors;

(e) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, ceases to continue its business; or

(f) any event analogous to the events referred to in paragraphs (a) to (e) above occurs in any other jurisdiction.

10.4 UCLB may terminate this Agreement by giving written notice to the Licensee and Meira, such termination to take effect forthwith or as otherwise stated in the notice:

10.4.1 if there is any change of Control of the Licensee involving the categories of persons or Affiliates of persons prohibited by Clause 2.3;

10.4.2 the Licensee is in persistent breach of the Agreement at least [***] ([***]) times in a calendar year, wherein the nature of each such breach is the same in each instance, and where the Parties have failed to agree on a mechanism to remedy the persistent nature of such breaches within a reasonable period following UCLB notifying Licensee of the persistent breaches and requesting that the Licensee enters into discussions with UCLB as to mechanisms for remedying the persistent breaches or if the Parties have agreed to a mechanism to remedy the persistence of such breach by that mechanism if not fully complied with by the Licensee; or

10.4.3 if the Licensee shall enter into any sub-licence with any of the categories of persons or Affiliates of persons prohibited by Clause 2.3 which may, adversely affect UCL's and/or UCLB's reputation;

10.4.4 subject to Clause 5.5, if the Licensee fails to achieve any of the milestone events described in Schedule 4 provided that if achievement of any of the milestone events should be compromised due to technical, legal or regulatory issues, the Parties shall first meet and UCLB will work with the Licensee to manage the delivery schedule and provided that the Licensee is using Diligent Efforts to correct the issues, the applicable deadline in Schedule 4 shall be extended by six (6) months or such other time period as shall be agreed between the Parties in writing after which if the Licensee has not achieved the milestone UCLB shall be entitled, subject to Clauses 5.11 and 5.12, to terminate this Agreement by giving written notice to the Licensee.

10.5 A Party's right of termination under this Agreement, and the exercise of any such right, shall be without prejudice to any other right or remedy (including any right to claim damages) that such Party may have in the event of a breach of contract or other default by any other Party.

10.6 Consequences of Termination

10.6.1 Upon expiry of the period of this Agreement, and subject to all royalties and any other sums due to UCLB under this Agreement having been duly paid, the Licensee shall have a fully paid up licence to the Patents, the Know-how and the Materials of the same scope as set forth in Clause 2.1 without any further obligation to pay any further sums to UCLB under Clause 4. Notwithstanding the foregoing the Licensee acknowledges that once each Patent expires or is abandoned or withdrawn or allowed to lapse in any country or territory, third parties in that country or territory will be entitled to use the inventions claimed in the Patent and that accordingly the licence granted to the Licensee under Clause 2.1 will no longer be exclusive in that country or territory.

10.6.2 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4:

(a) the Licensee and its Affiliates and Sub-licensees shall be entitled to sell, use or otherwise dispose of (subject to payment of royalties under Clause 4) any unsold or unused stocks of the Licensed Products for a period of [***] ([***]) months following the date of termination;

(b) subject to paragraph (a) above, any license that has not become fully paid-up in accordance with Clause 10.1 shall terminate and the Licensee and its Affiliates (and subject to Clause 2.3, its Sub-licensees) shall no longer be licensed to use or otherwise exploit the Patents and/or the Know-how and/or the Materials, in so far and for as long as any of the Patents remains in force and the Know-how remains confidential;

(c) the Licensee shall consent to the cancellation of any formal licence granted to it, or of any registration of it in any register, in relation to any of the Patents;

(d) the Licensee will, promptly on UCLB's request, provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which are in the possession of Licensee or its patent agents relating to the Patents and which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents;

(e) except as set out in Clause 2.3, all sub-licences of the Patents and/or the Know-how and/or the Materials granted by the Licensee pursuant to this Agreement will automatically terminate;

(f) UCLB shall, upon the written request of either of the other Parties, and each of the Licensee and Meira shall, upon the written request of UCLB, return or destroy any documents or other materials that are in its or its Affiliates possession or under its or their control and that contain the requesting Party's Confidential Information.

10.6.3 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, in the event that UCLB would be unable, absent a licence from the Licensee, to use or permit others to use or to exploit or permit others to exploit the relevant Licensed Technology without infringing intellectual property rights in any invention developed by the Licensee, whether solely or jointly with others ("Blocking Invention"), the Licensee shall be deemed to have granted UCLB the irrevocable non-exclusive right to use, exploit and permit others to use and exploit the Blocking Invention only in conjunction with the relevant Licensed Technology. The Licensee shall at the request of UCLB provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents licensed under this Agreement;

10.7 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, the Licensee shall, to the extent it is able to do so without being in breach of any obligation owed to a third party, disclose to UCLB full details of any and all Intellectual Property generated at any time by or on behalf of the Licensee as a result of the exercise of the Licensee's rights under this Agreement ("Licensee IP") and, upon UCLB's written request within [***] ([***]) days following such disclosure, negotiate in good faith to agree the terms of an exclusive or non-exclusive licence to UCLB (as UCLB may request) under the Licensee IP. If the Parties fail to agree the terms of such a licence within [***] days following commencement of such negotiation, despite negotiating in good faith, UCLB's rights under this Clause shall lapse. If Licensee may terminate this Agreement under Clause 10.3.1 (for UCLB or its Affiliates uncured material breach), then Licensee may elect, in lieu of terminating the entire Agreement, to have all licenses granted to the Licensee under this Agreement continue in force, subject to Licensee's fulfilment of [***] percent ([***]%) of its payment obligations under Clause 4 after what would have been the effective date of such termination.

10.8 Upon termination of this Agreement for any reason, the provisions of Clauses 1, 2.3, 2.5, 3.1 to 3.5, 4 (in respect of amounts paid and payable to UCLB in respect of the period up to and including the date of termination), 5.7, 7, 9, 10.7, 10.8, 10.8 and 11 of this Agreement shall remain in force**.**

11. GENERAL

11.1 Force Majeure

11.1.1 Any delays in or failure of performance by a Party under this Agreement will not be considered a breach of this Agreement and if and to the extent that such delay or failure is caused by occurrences beyond the reasonable control of that Party including acts of God; acts, regulations and laws of any government; strikes or other concerted acts of workers; fire; floods; explosions; riots; wars; rebellion; and sabotage; and any time for performance hereunder will be extended by the actual time of delay caused by any such occurrence.

11.1.2 If (a) UCLB or (b) the Licensee or Meira is prevented from carrying out its obligations:

(a) under this Agreement for a continuous period of [***] ([***]) months, the Licensee (in the case of (a)) or UCLB (in the case of (b)), may terminate this Agreement on giving [***] ([***]) days prior written notice provided always that at the date upon which termination becomes effective the Party which was prevented from carrying out its obligations under this Agreement remains so prevented.

11.2 Amendment

This Agreement may only be amended in writing signed by duly authorised representatives of the Parties.

11.3 Assignment and Third Party Rights

11.3.1 Subject to Clause 11.3.3, the Licensee shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement, nor any of the Patents, Know-how or Materials, without the prior written consent of UCLB.

11.3.2 UCLB may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.3.3 The Licensee, subject to obtaining the consent of UCLB which shall not be unreasonably withheld or delayed (except in relation to those categories of persons or Affiliates of persons prohibited by Clause 2.3), may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party to which it transfers all or substantially all of its assets or business, provided that the assignee undertakes to UCLB to be bound by and perform the obligations of the assignor under this Agreement. However, the Licensee shall not have such a right to assign this Agreement if it is insolvent.

11.3.4 Meira shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement without the prior written consent of UCLB.

11.4 Waiver

Any waiver given under or in relation to this Agreement shall be in writing and signed by or on behalf of the relevant Party. No failure or delay on the part of a Party to exercise any right or remedy under this Agreement shall be construed or operate as a waiver thereof, nor shall any single or partial exercise of any right or remedy preclude the further exercise of such right or remedy.

11.5 Invalid Clauses

If any provision or part of this Agreement is held to be invalid, amendments to this Agreement may be made by the addition or deletion of wording as appropriate to remove the invalid part or provision but otherwise retain the provision and the other provisions of this Agreement to the maximum extent permissible under applicable law.

11.6 No Agency

No Party shall act or describe itself as the agent of the other, nor shall it make or represent that it has authority to make any commitments on the other's behalf.

11.7 Interpretation

In this Agreement:

11.7.1 the headings are used for convenience only and shall not affect its interpretation; references to persons shall include incorporated and unincorporated persons; references to the singular include the plural and vice versa; and references to the masculine include the feminine;

11.7.2 references to Clauses and Schedules mean clauses of, and schedules to, this Agreement;

11.7.3 references in this Agreement to termination shall include termination by expiry;

11.7.4 where the word "including" is used it shall be understood as meaning "including without limitation";

11.7.5 any reference to any English law term for any action, remedy, method or judicial proceeding, legal document, legal status, court, official or any legal concept or thing shall in respect of any jurisdiction other than England be deemed to include what most nearly approximates in that jurisdiction to the English law term;

11.7. 6 where there is any conflict or inconsistency between the main body of this Agreement and any of the schedules, then the main body of the Agreement shall prevail;

11.7.7 time shall be of the essence in relation to the performance of Meira's and the Licensee's obligations under this Agreement; and

11.7.8 any reference to the sale of a Licensed Product by the Licensee or its Affiliates or Sub-licensees will be taken to include any supply or other disposal of Licensed Products, and the term sold shall be construed accordingly.

11.8 Notices. Addresses for Service

11.8.1 Any notice to be given under this Agreement shall be in English, in writing and shall be delivered by first class recorded delivery mail (if sent to an inland address) or by international courier (if sent to an address outside of the United Kingdom), to the address of the relevant Party set out at the head of this Agreement, or such other address as that Party may from time to time notify to the other Parties in accordance with this Clause 11.8.

11.8.2 Notices sent as above shall be deemed to have been received [***] ([***]) working day after the day of posting in the case of delivery inland first class recorded delivery mail, or [***] ([***]) working days after the date of collection by the international courier.

11.9 Law and Jurisdiction

The validity, construction and performance of this Agreement, and any contractual and non-contractual claims arising hereunder, shall be governed by English law and shall be subject to the exclusive jurisdiction of the English courts to which the Parties hereby submit, except that a Party may seek an interim injunction (or an equivalent remedy) in any court of competent jurisdiction.

11.10 Entire Agreement

This Agreement, including its Schedules, sets out the entire agreement between the Parties relating to its subject matter and supersedes all prior oral or written agreements, arrangements or understandings between them relating to such subject matter including, without limitation, the parts of the Original Agreement that pertains to the Specified Technology [***] and the Licence Addendum Number 5. Subject to Clause 9.8.5, the Parties acknowledge that they are not relying on any representation, agreement, term or condition which is not set out in this

Agreement. For clarity, this Agreement is not intended to supersede or affect the effectiveness of the parts of the Original Agreement that does not pertain to the Specified Technology [***] and the Licence Addendum Number 5. Such parts of the Original Agreement that does not pertain to the Specified Technology [***] and the Licence Addendum Number 5 includes, without limitation, the parts that pertains to the Specified Technology [***] through [***].

11.11 Third Parties

Except for the rights of UCL as provided in Clause 2.4, the rights of the Indemnitees as provided in Clause 9.7, the limitations of liability afforded to the Indemnitees pursuant to Clause 9.8, who may in their own right enforce and rely on the provisions of those Clauses, this Agreement does not create any right enforceable by any person who is not a party to it ("**Third Party**") under the Contracts (Rights of Third Parties) Act 1999, but this Clause 11.11 does not affect any right or remedy of a Third Party which exists or is available apart from that Act.

11.12 Non-use of Names; Announcements

11.12.1 The Licensee shall not use, and shall ensure that its Affiliates and Sub-licensees do not use, the name, any adaptation of the name, any logo, trademark or other device of UCLB, nor of the inventors named on the Patents nor the Principal Investigators in any advertising, promotional or sales materials without prior written consent obtained from UCLB in each case, except that the Licensee may state that it is licensed by UCLB under the Patents.

11.12.2 Except as permitted under Clauses 3.3.1 and 5.7, no Party shall make any press or other public announcement concerning any aspect of this Agreement, or make any use of the name or trademarks of any other Party in connection with or in consequence of this Agreement, without the prior written consent of the relevant other Party.

11.13 Escalation

If the Licensee or Meira on the one hand, and UCLB on the other, are unable to reach agreement on any issue concerning this Agreement or the Project within [***] days after one either has notified the other of that issue, they will refer the matter to the [***] in the case of UCLB, and to the [***] in the case of the Licensee and Meira in an attempt to resolve the issue within the time specified elsewhere in this Agreement in the case of other disputes. Any Party may bring proceedings in a court of competent jurisdiction if the matter has not been resolved within that prescribed period, and any Party may apply to the court for an injunction, whether or not any issue has been escalated under this Clause 11.13.

[Signature Page Follows]

EXECUTED on the date set out at the head of this Agreement.

For and on behalf of

UCL Business PLC

/s/ [***]
Signed

[***]
Print name

[***]
Title

29 January 2019
Date

For and on behalf of

MEIRAGTX UK II LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

For and on behalf of

MEIRAGTX LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 1

LICENSED TECHNOLOGY

Part A: The Patents

[***]

Part B: The Know-how

[***]

Part C: The Materials

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 2

APPOINTMENT OF EXPERT

For the purposes of this Schedule 2 only, the "Parties" shall mean the Licensee and UCLB. If either Party wishes to appoint an independent expert (the "**Expert**") to determine any matter pursuant to any Clause of this Agreement, the following procedures will apply:

1. The Party wishing to appoint the Expert (the "**Appointing Party**") will serve a written notice on the other Party (the "**Responding Party**"). The written notice will specify the Clause pursuant to which the appointment is to be made and will contain reasonable details of the matter(s) which the Appointing Party wishes to refer to the Expert for determination.

2. The Parties shall within [***] ([***]) days following the date of the Appointing Party's written notice use all reasonable efforts to agree who is to be appointed as the Expert to determine the relevant matter(s). If the Parties are unable to agree upon the identity of the Expert within that timescale, the Expert shall be appointed by the President (for the time being) of the Licensing Executives Society Britain and Ireland upon written request of either Party.

3. Each Party will within [***] ([***]) days following appointment of the Expert, prepare and submit to the Expert and the other Party a detailed written statement setting out its position on the matter(s) in question and including any proposals which it may wish to make for settlement or resolution of the relevant matter.

4. Each Party will have [***] ([***]) days following receipt of the other Party's written statement to respond in writing thereto. Any such response will be submitted to the other Party and the Expert.

5. The Expert will if he/ she deems appropriate be entitled to seek clarification from the Parties as to any of the statements or proposals made by either Party in their written statement or responses. Each Party will on request make available all information in its possession and shall give such assistance to the Expert as may be reasonably necessary to permit the Expert to make his/ her determination.

6. The Expert will issue his/ her decision on the matter(s) referred to him/ her in writing as soon as reasonably possible, but at latest within [***] ([***]) months following the date of his/ her appointment. The Expert's decision shall (except in the case of manifest error) be final and binding on the Parties.

7. The Expert will at all times act as an independent and impartial expert and not as an arbitrator.

8. The Expert's charges will be borne as he/ she determines in his written decision.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 3

DEFINITION OF TOBACCO INDUSTRY FUNDING (REVISED 2009)

FROM THE CANCER RESEARCH UK CODE OF PRACTICE ON TOBACCO INDUSTRY FUNDING TO UNIVERSITIES.

http://www.cancerresearchuk.org/science/funding/terms-conditions/funding-policies/policy-tobacco/

A tobacco company is defined for the purposes of this policy as one that:

- Derives over 5% of revenues from manufacturing tobacco products;
- Derives 15%+ of revenues from the manufacture of products necessary for the production of tobacco products;
- Derives 15% of revenues from the sale of tobacco products (and has 30 or more staff);
- Owns a tobacco company (the company owns 50% or more of a tobacco company);
- Is more than 50% owned by a company with tobacco involvement.

The following do not constitute tobacco industry funding for the purposes of this Code:

- legacies from tobacco industry investments (provided these are sold on immediately)
- funding from a trust or foundation no longer having any connection with the tobacco industry even though it may bear a name that (for historical reasons) has tobacco industry associations.

SCHEDULE 4

DEVELOPMENT PLAN

The Development Plan for the Licensed Technology is shown below

Activity	**Timeline**
Phase I/II Start	[***]
Phase I/II Finish	[***]
Phase III /pivotal confirmatory study Start	[***]
Phase III /pivotal confirmatory study Finish	[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 10.21

Confidential Treatment Requested by MeiraGTx Holdings plc

LICENCE AGREEMENT

between

UCL Business Plc

and

MeiraGTx UK II Limited

and

MeiraGTx Limited

and

MeiraGTx Holdings plc

Dated: 29 January 2019

Ref: [***] **(CNGB3)**

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

THIS AGREEMENT is made 29 January, 2019

BETWEEN:

(1) **UCL BUSINESS PLC**, a company incorporated in England and Wales under company registration number 02776963 whose registered office is The Network Building, 97 Tottenham Court Road, London W1T 4TP ("**UCLB**");

and

(2) **MEIRAGTX UK II LIMITED**, a company incorporated in England and Wales with registered number 09348737 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ (the "**Licensee**"); and

(3) **MEIRAGTX LIMITED,** a company incorporated in England and Wales with registered number 09501998 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ ("**Meira**"); and

(4) for the purposes of Section 4.1 only, **MEIRAGTX HOLDINGS PLC,** a company incorporated in England and Wales with registered number BR020669 and having its UK establishment address at 92 Britannia Walk, London, United Kingdom, N1 7NQ.

WHEREAS:

A. University College London ("**UCL**") has developed certain ocular gene technologies and owns certain intellectual property rights relating to those gene therapies that the Licensee wishes to acquire rights to for the development and commercialisation of Licensed Products.

B. UCL has assigned to UCLB all of its right, title and interest in and to such property.

C. The Parties previously entered into a certain License Agreement dated as of February 4, 2015 (the "**Original Commencement Date**"), amended on March 27, 2015, July 28, 2017, and December 14, 2017, as well as a certain Licence Addendum Number 1 dated 4 February 2015 (collectively, the "**Original Agreement**").

D. The Parties now desire to amend the Original Agreement to exclude the Specified Technology [***] and to terminate the Licence Addendum Number 1, and enter into a new agreement for the Specified Technology [***] on the terms set out in this Agreement.

E. It is the policy of UCLB that its activities in licensing intellectual property take into consideration ethical and socially responsible licensing principles, including ensuring that Licensed Products are made available to fulfil unmet needs in developing countries, and the Licensee acknowledges and agrees to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil such needs, all in accordance with the provisions of this Agreement.

NOW IT IS AGREED as follows:

1. DEFINITIONS

1.1 In this Agreement:

Agreement means this agreement (including the Schedules);

Affiliate in relation to a Party, means any entity or person that Controls, is Controlled by, or is under common Control with that Party;

Claims means all demands, claims and liability (whether criminal or civil, in contract, tort or otherwise) for losses, damages, costs and expenses of any nature whatsoever and all costs and expenses (including legal costs) incurred in connection therewith;

Commencement Date means the date of this Agreement;

Competing Product means [***];

Confidential Information means the Know-how, the Materials and all other technical or commercial information that:

a) in respect of information provided in documentary form or by way of a model or in other tangible form, at the time of provision is marked or otherwise designated to show expressly that it is imparted in confidence or which a reasonable person would expect to be confidential; and

b) in respect of information that is imparted orally, any information that the Disclosing Party or its representatives informed the Receiving Party at the time of disclosure or which a reasonable person would expect to be confidential;

Control means direct or indirect beneficial ownership of 50% (or, outside a Party's home territory, such lesser percentage as is the maximum permitted level of foreign investment) or more of the share capital, stock or other participating interest carrying the right to vote or to distribution of profits of that Party, as the case may be;

Diligent Efforts means, with respect to efforts to be expended by the Licensee with respect to any objective under this Agreement, diligent, reasonable, good faith efforts to accomplish such objective [***], it being understood and agreed that with respect to the research, development, or commercialization of a product, such efforts will be substantially equivalent to those that would normally be exerted or employed by [***], when utilizing sounds and reasonable scientific, medical and business practice and judgment;

Disclosing Party has the meaning given in Clause 3.2;

Field means ocular gene therapy;

First Commercial Sale means the first sale to a third party of a Licensed Product in a given regulatory jurisdiction after all regulatory and marketing approvals have been obtained for such Licensed Product in such jurisdiction. A sale shall not be deemed to have occurred if a Licensed Product is provided pursuant to an early access or compassionate use;

Indemnitees has the meaning given in Clause 9.7;

Intellectual Property means any and all patents, utility models, registered designs, unregistered design rights, copyright, database rights, rights in respect of confidential information, rights under data exclusivity laws, rights under orphan drug laws, rights under unfair competition laws, property rights in biological or chemical materials, extension of the terms of any such rights (including supplementary protection certificates), applications for and the right to apply any of the foregoing registered property and rights, and similar or analogous rights in any part of the Territory;

Know-how means:

a) the inventions claimed in the Patents; and

b) the technical information relating to the inventions claimed in the Patents and data described in the Part B of Schedule 1;

Licensed Products means any and all products that are developed, manufactured, used, or sold by or on behalf of the Licensee or its Affiliates or Sub-licensees and which (a) are within (or are manufactured using a process described in) any claim of the Patents; and/or (b) incorporate, or their development or manufacture makes use of, any of the Know-how and/or the Materials;

Licensed Technology means the Patents, the Know-how and the Materials set out in Schedule 1;

Materials means any and all of the materials referred to in Part C of Schedule 1;

Net Sales Value means in respect of [***]

Original Commencement Date has the meaning given in the Recitals;

Original Agreement has the meaning given in the Recitals;

Parties means UCLB, the Licensee and Meira, and "Party" shall mean either of them;

Patent Costs means [***].

Patents means any and all of the patents and patent applications referred to in Part A of Schedule 1;

Principal Investigators means [***].

Receiving Party has the meaning given in Clause 3.2;

Regulatory Exclusivity means, with respect to a Licensed Product, any exclusive rights or protection which are recognised, afforded or granted by any regulatory authority in any country or region with respect to the Licensed Product other than through patent rights;

Sub-licensee means any third party (other than an Affiliate) to whom the Licensee grants a sub-licence of its rights under this Agreement in accordance with Clause 2.3;

Territory means worldwide;

Valid Claim means a claim of a patent or patent application that has not been abandoned or allowed to lapse or expired or been held invalid or unenforceable by a court of competent jurisdiction in a final and non-appealable judgment.

2. GRANT OF RIGHTS

2.1 **Licence**

UCLB hereby grants to the Licensee and its Affiliates, and the Licensee hereby accepts on its own behalf and on behalf of its Affiliates, subject to the provisions of this Agreement:

2.1.1 an exclusive (even as to UCL) licence under the Patents, the Know-how and the Materials, with the right to sub-license, subject to Clause 2.3, to develop, commercialise, manufacture, have manufactured, use, sell and have sold Licensed Products only in the Field and in the Territory.

2.2 **UCLB shall at the Licensee's request and cost execute such formal licences as may be necessary to enable the Licensee to register the licences granted to it under this Agreement with the Patent Offices in the relevant Territory. Such formal licence will reflect the terms of this Agreement where possible and for the avoidance of doubt if there is a conflict in the terms of such formal licence and this Agreement, the terms of this Agreement shall prevail.** [***]**.**

2.3 **Sub-Licensing**

The Licensee shall have the right to grant sub-licenses under the license in Clause 2.1 to its Affiliates or other third parties through one or more levels of Sub-licensees except that the Licensee may not grant such a sub-license to any person or the Affiliates of any person involved in: the tobacco industry (as defined by the Cancer Research UK Code of Practice on Tobacco Industry Funding to Universities detailed in Schedule 3); arms dealing; gambling operations; the promotion of violence; child labour or any other illegal activity. A grant of any sub-licence shall be conditioned on the following:

(a) The Licensee shall enter into a written agreement with each Sub-licensee and shall ensure that the provisions of each sub-licence are consistent with the provisions of this Agreement, and the Licensee shall ensure that:

(i) the sub-licence sets out all the proposed terms agreed between the Licensee and the Sub-licensee, including, in particular, all terms as to remuneration;

(ii) the Sub-licensee will maintain complete and accurate records in sufficient detail to permit UCLB to confirm the accuracy of the calculation of royalty payments under this Agreement; and

(iii) the sub-licence imposes obligations of confidentiality on the Sub-licensee which are no less onerous than those set out in Clause 3.2.

(b) The Licensee shall procure that each Sub-licensee complies fully at all times with the provisions of its sub-licence.

(c) The Licensee shall be liable for all acts and omissions of its Sub-licensees that, if committed by the Licensee, would constitute a breach of any of the provisions of this Agreement.

(d) The Licensee shall provide UCLB with a copy of any sub-licence [***] ([***]) days after execution of such sub-licence, provided that the Licensee may redact confidential or proprietary terms from such copy, including financial terms.

(e) Each sub-licence shall terminate automatically upon termination of this Agreement for any reason (but not expiry of this Agreement under Clause 10.1), except where the Sub-licensee was not implicated in or at fault in any circumstances which led to the termination of this Agreement, UCLB shall on receiving a written request from the relevant Sub-licensee within [***] ([***]) days following the date of termination of this Agreement enter into a licence agreement with the Sub-licensee for the Licensed Technology on terms substantially the same as the terms set out in this Agreement (except that the Sub-licensee shall not be obliged to pay to UCLB any sums equivalent to those sums set out in Clauses 4.1 or 4.3 which have already been paid to UCLB by the Licensee prior to the date of termination).

2.4 **Reservation of Rights**

2.4.1 UCLB reserves for itself and UCL the non-exclusive, irrevocable, worldwide, royalty-free right to:

(a) Use the Licensed Technology in the Field solely for academic (non-commercially funded) research, publication and teaching; and

(b) Grant licenses to academic third parties to use the Licensed Technology in academic research collaborations with UCL and such licenses shall not be sublicensable; and

(c) Grant license of the Licensed Technology to post graduate student of UCL for the purpose of conducting a programme of post graduate academic research and such licenses shall not by sublicensable.

In exercising the rights described in Clause 2.4.1(b) and (c), UCL and UCLB shall comply with the provisions of Clause 3 as regards confidentiality of the Know-how.

2.4.2 UCL and UCLB will refer a request from a third party for a licence to use the Patents in clinical trials or for diagnostic purposes involving human subjects to the Licensee, and the Licensee shall liaise directly with such third party.

2.4.3 Except for the licences expressly granted by this Clause 2, UCLB grants no rights to the Licensee under this Agreement to or under any intellectual property other than the Patents, the Know-how and the Materials and hereby reserves all rights under the Patents, the Know-how and the Materials outside the Field.

2.4.4 Nothing in this Agreement shall limit or otherwise affect UCL's ability to apply for noncommercial grant funding or comply with such grant terms and conditions. In the event that any terms of this Agreement conflicts with the terms of any non-commercial grant funding, the Parties shall negotiate in good faith to amend the terms of this Agreement to allow UCL to access such funding provided that nothing herein shall require the Licensee to agree to alter or modify the scope of the licence granted to it in this Clause 2.

2.5 **Affiliates**

The Licensee shall:

2.5.1 ensure that its Affiliates comply fully with the terms of this Agreement;

2.5.2 be responsible for any breach of or non-compliance with this Agreement by its Affiliates as if the breach or non-compliance had been a breach or non-compliance by the Licensee;

2.5.3 indemnify in accordance with Clause 9.7 each of the Indemnitees against any Claims which are awarded against or suffered by any of the Indemnitees as a result of any breach of or non-compliance with this Agreement by its Affiliates; and

2.5.4 ensure that if any Affiliate ceases to be an Affiliate as a result of a change of Control or otherwise, that unless a sub-licence agreement in accordance with Clause 2.3 is entered into with such an Affiliate, such former Affiliate immediately upon such cessation:

(a) ceases developing, manufacturing, having manufactured, using, selling and/ or having sold Licensed Products and ceases all use or exploitation of the Licensed Technology, for as long as any of the relevant Patents remains in force and/or the Know-how remains confidential;

(b) returns to the Licensee or destroys any documents or other materials in the former Affiliate's possession or under its control and that contain Confidential Information provided under this Agreement relating to the Licensed Technology and/ or Licensed Products;

(c) to the extent possible, takes all action necessary to have any product licences, marketing authorisations, pricing and/ or reimbursement approvals (and any applications for any of the foregoing) which relate to Licensed Products transferred into the name of the Licensee.

2.6 **Use of Licensed Technology in Combination**

UCLB acknowledges and agrees that the Licensee shall be entitled to use the Licensed Technology in combination with other technology, patents, know-how and materials licensed by UCLB to the Licensee under separate licence agreements and with any improvements to the Licensed Technology developed or generated by the Licensee.

3. KNOW-HOW AND CONFIDENTIAL INFORMATION

3.1 **Confidentiality of Know-how and Materials**

The Licensee undertakes that for so long as the Know-how and/or the Materials remains confidential, it shall (and shall ensure that its Affiliates and Sub-licensees) take all reasonable precautions to prevent unauthorised access to the Know-how and the Materials and protect the Know-how and the Materials in the same manner as it (or they) protect(s) its (or their) own

proprietary information, and shall not (and shall ensure that its Affiliates and Sub-licensees do not) use the Know-how or the Materials for any purpose, except as expressly licensed hereby and in accordance with the provisions of this Agreement. For the avoidance of doubt, to the extent that any Materials, Know-how or information relating to the Patents falls within the public domain (without any breach of this Agreement or any other obligation of confidentiality), then UCL, the Principal Investigators and UCLB shall be free to use such information without restriction in the same way that any third party would have the freedom to use it.

3.2 **Confidentiality Obligations**

Each Party ("**Receiving Party**") undertakes:

3.2.1 to maintain as secret and confidential all Confidential Information obtained from, in the case of UCLB, the Licensee or Meira as applicable, and in the case of the Licensee and Meira, UCLB ("**Disclosing Party**") in the course of or in anticipation of this Agreement and to respect the Disclosing Party's rights therein;

3.2.2 to use such Confidential Information only for the purposes of or as permitted by this Agreement; and

3.2.3 subject to Clause 3.3, to disclose such Confidential Information only to those of its employees, contractors, Affiliates, and Sub-licensees (if any) to whom and to the extent that such disclosure is reasonably necessary for the purposes of this Agreement.

3.3 **Permitted Disclosure**

3.3.1 The Licensee shall have the right to disclose Confidential Information received from UCLB to:

(a) potential or actual customers of Licensed Products to the extent reasonably necessary to promote the sale or use of Licensed Products and provided that the customer has agreed to confidentiality provisions at least as restrictive as set forth herein;

(b) to existing or potential Sub-licensees, collaborators, investors or lenders provided that such third parties have agreed to confidentiality provisions at least as restrictive as set forth herein; and

(c) to its Board of Directors (or similar governing body) and its counsel, accountants and other professional advisers.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

3.4 **Exceptions to Obligations**

The provisions of Clause 3.2 shall not apply to Confidential Information which the Receiving Party can demonstrate by reasonable written evidence:

3.4.1 was, prior to the Original Commencement Date, in the possession of the Receiving Party and at its free disposal and was not obtained or otherwise acquired directly or indirectly from the Disclosing Party or its Affiliates or their respective employees, students or representatives; or

3.4.2 is subsequently disclosed to the Receiving Party without any obligations of confidence by a third party; or

3.4.3 is or becomes generally available to the public through no act or default of the Receiving Party or its agents, employees, Affiliates or Sub-licensees; or

3.4.4 the Receiving Party is required to disclose by or to the courts of any competent jurisdiction, or to any government regulatory agency or financial authority, provided that the Receiving Party shall:

(a) inform the Disclosing Party as soon as is reasonably practicable;

(b) at the Disclosing Party's request and cost seek to persuade the court, agency or authority to have the information treated in a confidential manner, where this is possible under the court, agency or authority's procedures; and

(c) where the disclosure is unavoidable, limit the disclosure of Confidential information to the minimum extent required by law; or

3.4.5 which a Party is advised by its information officer that it is required to disclose under the Freedom of Information Act 2000 or the Environmental Information Regulations 2004.

3.5 **Disclosure to Employees**

The Receiving Party shall procure that all of its employees, contractors, Affiliates and Sub-licensees who have access to any of the Disclosing Party's Confidential Information to which Clause 3.2 applies, shall be made aware of the obligations of confidence and are bound by obligations of confidentiality at least as restrictive as those set forth herein (which it undertakes to enforce and for which it is legally responsible) and the Receiving Party shall only disclose the Disclosing Party's Confidential Information to those of its subsidiaries, employees, and officers as need to have access thereto wholly necessarily and exclusively for the purposes of this Agreement.

4. CONSIDERATION

4.1 Upfront Payments

Furthermore, UCLB shall receive the following one-time payments in connection with the grant of the licence in Clause 2.1:

(a) Licensee shall pay to UCLB £1,500,000 in cash within [***] ([***]) days following the Commencement Date.

(b) MeiraGTx Holdings plc shall issue £1,500,000 in securities of MeiraGTx Holdings plc [***]. The number of such securities of will be equal to the quotient resulting from dividing: (x) £1,500,000; by (y) [***].

Milestone Payments

Within [***] ([***]) days following achievement of each of the following milestone events by Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant milestone event has been achieved, provide documentary evidence of such achievement as appropriate and pay to UCLB, within a period of [***] ([***]) days, the amount(s) set out next to such milestone event below:

Milestone Event	Amount to be paid
[***]	£ [***]
[***]	£ [***]

4.2 **Annual Management Fees**

On each date referred to in the following table, the Licensee shall pay to UCLB the annual management fee set out next to such date in the table.

Date	Amount to be paid
Upon each anniversary of the Original Commencement Date until [***]	£50,000

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.3 **Sales Linked Milestone Payments**

Upon the first achievement of each of the sales linked milestones set out in the following table by the Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant sales linked milestone has been achieved, provide the relevant documentary evidence and pay to UCLB the amount(s) set out next to such event in the table:

Sales Linked Milestones	**Amount to be paid**
When Net Sales Value reaches £[***]	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (When sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]

4.4 **Royalties on Net Sales**

For each Licensed Product in each country, the Licensee shall pay to UCLB a royalty of [***]% ([***] per cent) being a percentage of the Net Sales Value of such Licensed Product sold by Licensee, its Affiliates or Sub-licensees. The Licensee's obligations to pay such royalty for a given Licensed Product in a given country shall begin after the First Commercial Sale of such Licensed Product in such country and shall end on the later to occur of the following: (a) expiration of the last Valid Claim of a Patent claiming such Licensed Product in such country; or (b) the tenth (10^{th}) anniversary of the date of such First Commercial Sale in such country; or (c) the expiration of any Regulatory Exclusivity with respect to such Licensed Products in the relevant country.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.5 **Combination Products**

If any Licensed Products are incorporated in any other product ("**Combination Product**") sold by the Licensee or its Affiliates and the Licensed Product is not priced separately from the Combination Product, the Net Sales Value of such Licensed Product shall be deemed to be the fair market value of the Licensed Product in the country of sale when sold separately or if not sold separately in the country of sale, in comparable countries and territories or if neither of the foregoing apply, a reasonable amount which fairly reflects the value of the Licensed Product within the Combination Product assuming the Licensed Product is not being sold as a loss leader.

4.6 Payment Frequency

Royalties due under this Agreement, except for the payments due under Clauses, 4.1, 4.2 and 4.3, which are payable upon the date/time specified in Clauses 4.1, 4.2 and 4.3 as appropriate, shall be paid within [***] ([***]) days following the end of each calendar quarter ending on 31 March, 30 June, 30 September and 31 December in each year, in respect of sales of Licensed Products made during such quarter, and within [***] ([***]) days following the termination of this Agreement.

4.7 **Payment terms**

All sums due under this Agreement:

4.7.1 are exclusive of Value Added Tax which where applicable will be paid by the Licensee to UCLB in addition;

4.7.2 shall be paid in pounds sterling in cash by transferring an amount in aggregate to the following Account name: UCL Business Plc, Sort Code: 20 10 53, Account number: 30782270, Address: Barclays Bank Plc, PO Box 11345, London, W12 8GG, and in the case of income or amounts received by the Licensee or its Affiliates in a currency other than pounds sterling, the royalty shall be calculated in the other currency and then converted into equivalent pounds sterling at the relevant daily spot rate for that currency as quoted in the Financial Times newspaper on the last business day of the quarter in relation to which the royalties are payable;

4.7.3 will be made without any set-off, deduction or withholding except as may be required by law. If the Licensee is required by law to make any deduction or to withhold any part of any amount due to UCLB under this Agreement, the Licensee will give to UCLB proper evidence of the amount deducted or withheld and payment of that amount to the relevant taxation authority, and will do all things in its power to enable or assist UCLB to claim exemption from or, if that is not possible, to obtain a credit for the amount deducted or withheld under any applicable double taxation or similar agreement from time to time in force; and

4.7.4 shall be made by the due date, failing which UCLB may charge interest on any outstanding amount on a daily basis at a rate equivalent to [***]% above the Bank of England pound sterling base rate then in force in London.

4.8 Royalty Statements

The Licensee shall send to UCLB, at the same time as each royalty payment is made in accordance with Clause 4.4, a statement setting out for the relevant calendar quarter:

4.8.1 in respect of each territory or region in which Licensed Products are sold;

4.8.2 the types of Licensed Product sold;

4.8.3 the quantity of each type sold;

4.8.4 the total invoiced price for each type of Licensed Product sold;

4.8.5 where relevant, details of any Licensed Products that have been sold other than on arm's length terms for a cash consideration, including the relevant open market price or (if not available) the reasonable price attributed thereto;

4.8.6 the amounts deducted from the Net Sales Value as referred to in paragraph (i) to (iv) of that definition (broken down on a product by product and category by category basis); and

4.8.7 the aggregate royalties on Net Sales Value due to UCLB;

in each case expressed both in local currency and pounds sterling and showing the conversion rates used, during the period to which the royalty payment relates.

4.9 Records

The Licensee shall keep at its normal place of business detailed and up to date records and accounts showing the quantity, description and invoiced price or non-cash consideration for all Licensed Products sold by it or its Affiliates or on its or its Affiliates' behalf, broken down in each case on a country by country basis, and being sufficient to ascertain the payments due to UCLB under this Agreement.

The Licensee shall make such records and accounts available, on reasonable notice, for inspection during business hours by an independent chartered accountant nominated by UCLB for the purpose of verifying the accuracy of any statement or report given by the Licensee to UCLB under this Clause 4.9. The Licensee shall co-operate reasonably with any such accountant, and shall promptly provide all information and assistance reasonably requested by

such accountant. The accountant shall be required to keep confidential all information learnt during any such inspection, and to disclose to UCLB only such details as may be necessary to report on the accuracy of the Licensee's statement or report. UCLB shall be responsible for the accountant's charges unless the accountant certifies that there is an inaccuracy of more than [***]% ([***] percent) in any royalty statement, in which case the Licensee shall pay his charges in respect of that inspection.

The Licensee shall ensure that UCLB has the same rights as those set out in this Clause 4.9 in respect of the Licensee's Affiliates and Sub-licensees.

The Licensee shall co-operate with UCLB in good faith to resolve any discrepancies identified during any such inspection and [***], together with interest on late payment as specified in Clause 4.7.4, within [***] following receipt of a copy of the independent chartered accountant's report.

4.10 **Accounting Standards**

Where this Agreement requires a financial calculation to be made or an action to be taken by a party, such calculation will be made or taken in accordance with the generally accepted accounting principles followed by such party.

5. COMMERCIALISATION

5.1 **General Diligence**

The Licensee shall use Diligent Efforts to develop and commercially exploit Licensed Products throughout the Territory (including obtaining all and any regulatory approvals which may be required to market and sell the Licensed Products) for the benefit of both Parties.

5.2 **Competing Activities**

The Licensee shall notify UCLB in confidence if it or any of its Affiliates or its Sub-licensees commences any marketing, sale or commercialisation of any Competing Product or enters into an agreement with any other person with respect to any such activities.

5.3 **Development Plan**

The Licensee's initial plan for developing and commercialising Licensed Products is set out in Schedule 4 (the "**Initial Development Plan**"). The Licensee shall provide to UCLB on each anniversary of the Original Commencement Date a written update to the Initial Development Plan that shall:

5.3.1 report on all activities conducted under this Agreement by the Licensee and its Affiliates and Sub-licensees since the Original Commencement Date or the date of the previous update (as appropriate);

5.3.2 (where applicable) set out the milestone events achieved since the Original Commencement Date or the date of the previous update (as appropriate) and the Licensee's reasonable estimate of the dates for achieving any future milestone events;

5.3.3 set out the current and projected activities being taken or planned to be taken by the Licensee and its Affiliates and Sub-licensees to bring Licensed Products to market in the Territory; and

5.3.4 set out the projected sales of Licensed Products (based on the Licensee's or Sub-licensee's current forecasts) for each of the next [***] ([***]) years following the date of the report.

UCLB's receipt or approval of any update to the Updated Development Plan shall not be taken to waive or qualify the Licensee's obligations under Clause 5.1.

5.4 **Annual Meeting**

In respect of the Licensed Technology, the Licensee will on UCLB's request meet with UCLB at least once per calendar year, following the submission of the update to the relevant Development Plan pursuant to Clause 5.3, to discuss progress with development and commercialisation of the Licensed Technology.

5.5 **Development Milestones**

In addition to the Licensee's obligations under Clause 5.1, the Licensee shall for each Licensed Technology use Diligent Efforts to achieve the development milestone events specified in Schedule 4 by the dates set out therein. In any instance in which it becomes apparent that a particular development milestone will not be met due to regulatory, technical, safety or efficacy-related reasons or Force Majeure event (pursuant to Clause 11.1), the Parties in good faith will agree upon an appropriate adjust of such milestone and any subsequent milestones.

5.6 **Reporting of First Commercial Sale**

The Licensee will promptly notify UCLB in writing of the First Commercial Sale of each Licensed Product on a commercial basis in each country within the Territory.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

5.7 **Reporting for Impact Purposes**

5.7.1 The Licensee acknowledges that part of UCLB's purpose in licensing the Patents, Know-how and the Materials to the Licensee pursuant this Agreement is to ensure that the Patents, Know-how and the Materials are made available for use and commercial exploitation with the intention of benefitting society and the economy. In order to enable UCLB and UCL to monitor the benefit that they are providing, and to enable UCL to demonstrate the impact of its research activities, to society and the economy, the Licensee will upon request provide to UCLB [***], a written report describing in reasonable detail how it has used the Patents, Know-how and the Materials and the societal and economic benefits generated therefrom.

5.7.2 UCLB shall notify and seek permission from the Licensee in advance, in writing if it wishes to use any written reports received from the Licensee (and the information contained therein) pursuant to Clause 5.7.1 in applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities, supplying a written copy of the application for research or other grant related funding or submission (or the relevant sections thereof). The Licensee will respond to UCLB in writing within [***] ([***]) days of receipt of such written information and subject to the removal of any confidential information as notified in such written request by the Licensee, UCLB and UCL shall be entitled to submit the approved applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities.

5.8 **Quality**

The Licensee shall ensure that all of the Licensed Products marketed by it and its Affiliates and Sub-licensees are of satisfactory quality and comply with all applicable laws and regulations in each part of the Territory.

5.9 **Marking of Licensed Products**

To the extent permitted under the laws of any country, the Licensee shall mark and cause its Affiliates and Sub-Licensees to mark each Licensed Product with the number of each issued Patent which applies to the Licensed Product and a statement that such Licensed Products are sold under licence from UCL Business plc.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

5.10 **Disposals of Licensed Products for Free**

Notwithstanding the terms of Clause 5.1, the Licensee shall be entitled to supply a reasonable number of Licensed Products to third parties free of charge as promotional items for the purpose of establishing a market for the Licensed Products in the relevant country or territory or for research, evaluation and testing purposes, or for clinical development, provided that the quantity of Licensed Products supplied for free (or for the cost of manufacture) in each country or territory is not excessive and is in line with normal industry practice in such country or territory. Any Licensed Products disposed of to third parties in accordance with this Clause 5.10 shall not be taken into account for the purposes of calculating Net Sales Value.

5.11 **Referral to Expert**

If UCLB considers at any time during the period of this Agreement that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, then the matter shall be referred to an independent expert to answer the following questions:

5.11.1 whether the Licensee has complied with its obligations under Clause 5.1 or 5.3; and if not

5.11.2 what specific action the Licensee should have taken and/or now needs to take ("**Specific Action**") in order to fulfil such obligations and within what period the Specific Action should be taken ("**Action Period**").

The independent expert shall be appointed in accordance with the provisions of Schedule 2 and his decision shall be final and binding on the Parties.

5.12 **Consequences of Expert's Decision**

If the expert determines that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, and if the Licensee fails to take the Specific Action within the Action Period, UCLB shall be entitled, by giving, at any time within [***] ([***]) months after the end of that Action Period, not less than [***] ([***]) months' notice, to (a) convert the licence granted under Clause 2.1 into a non-exclusive licence or (b) terminate this Agreement.

6. ACCESS TO MEDICINES AND ETHICAL LICENSING

Licensee shall use Diligent Efforts to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil unmet needs in developing countries.

7. COMPLIANCE WITH LAWS

7.1 General Compliance with Laws

The Licensee will at all times (and will ensure its Affiliates and Sub-licensees) comply with all legislation, rules, regulations and statutory requirements applying to and obtain any consents necessary for its use of the Patents, the Know-how and the Materials, the development, manufacture, and sale of Licensed Products in any country or territory.

7.2 Bribery Act

The Licensee shall (and shall procure that any persons associated with it engaged in the performance of this Agreement including its Affiliates and Sub-licensees shall):

7.2.1 comply with all applicable laws and codes of practice relating to anti-bribery and anti-corruption including the Bribery Act 2010 and without prejudice to the foregoing generality, shall not engage in any activity, practice or conduct which would constitute an offence under sections 1, 2 or 6 of the Bribery Act 2010 or do or omit to do any act that will cause or lead UCLB to be in breach of the Bribery Act 2010;

7.2.2 comply with UCLB's ethics, anti-bribery and anti-corruption policies as notified to the Licensee from time to time and have, maintain in place and enforce throughout the term of this Agreement adequate procedures to ensure compliance with Clause 7.2.1; and

7.2.3 promptly report to UCLB any request or demand for any undue financial or other advantage of any kind received in connection with the performance of this Agreement.

For the purpose of this Clause 7.2, the meaning of adequate procedures and whether a person is associated with another person shall be determined in accordance with the Bribery Act 2010 (and any guidance issued under section 9 of that Act). Breach of this Clause 7.2 shall be deemed a material breach of this Agreement entitling UCLB to terminate under Clause 10.3.1.

7.3 Export Control Regulations

The Licensee shall ensure that, in using the Patents, Know-how or Materials and in selling Licensed Products, it and its Affiliates, employees, sub-contractors and Sub-licensees comply fully with any United Nations trade sanctions or EU or UK legislation or regulation, from time to time in force, which impose arms embargoes or control the export of goods, technology or software, including weapons of mass destruction and arms, military, paramilitary and security equipment and dual-use items (items designed for civil use but which can be used for military purposes) and certain drugs and chemicals.

8. INTELLECTUAL PROPERTY

8.1 **Obtain and Maintain the Patents**

8.1.1 The Licensee shall be responsible for the drafting, filing, prosecution and maintenance of all of the Patents at the Licensee's cost and expense. Subject to resource availability, UCLB shall use commercially reasonable efforts to provide such assistance as the Licensee may request to prosecute and maintain the Patents[***].

8.1.2 The Patents will be filed, prosecuted and maintained in the countries and territories where Licensee normally files its patent applications and patents for other gene therapy products. The Licensee shall notify UCLB of any decisions as to which (if any) additional countries to file and maintain Patents in.

8.1.3 The Licensee shall consult with UCLB in relation to all material changes to the patent claims or specifications that would have the effect of reducing or limiting the scope of the Patents, and not make any such changes without the prior written consent of UCLB. Such consent shall not be unreasonably withheld or delayed provided that UCLB has been given as much notice as is practicable, and in any event no less than [***] days' notice (or such shorter period for response dictated by the relevant patent office) of such proposed changes, and has been given an opportunity to file divisionals, continuations and/or such other types of protection to cover any claims or subject matter that the Licensee intends to remove from the scope of the Patents. If UCLB fails to respond before the end of the [***] day period (or such shorter period for response dictated by the relevant patent office), the Licensee may proceed with the proposed changes to the patent claims or specifications. The Licensee will ensure that UCLB receives copies of all correspondence to and from Patent Offices in respect of the Patents, including copies of all documents generated in or with such correspondence, and shall be given reasonable notice (or such shorter period for response dictated by the relevant patent office) of and the opportunity to participate in any conference calls or meetings with the Licensee's patent attorneys in relation to the drafting, filing, prosecution and maintenance of the Patents, so that UCLB may be continuously informed of progress with the drafting, filing, prosecution and maintenance of the Patents. Such involvement of UCLB under this Clause 8.1.3 shall be at UCLB's cost and expense.

8.1.4 If the Licensee wishes to abandon any application contained with the Patents or not to maintain any such Patent, it shall give [***] ([***]) months' prior written notice to UCLB and on the expiry of such notice period the licences of the relevant Patents granted to the Licensee under this Agreement shall cease.

8.1.5 In the event that any of rights granted hereunder become non-exclusive, responsibility for the drafting, filing, prosecution and maintenance of all of the Patents shall revert to UCLB.

8.2 **Infringement of the Patents, the Know-how and/or the Materials**

8.2.1 The Licensee and UCLB shall promptly give to each other written notice if it becomes aware of any infringement or potential infringement of any of the Patents or any unauthorised use of the Know-how or the Materials or any challenge to the validity or ownership of the Patents, the Know-how or the Materials and the Licensee and UCLB shall consult with each other to decide the best way to respond to such infringement, unauthorised use or challenge.

8.2.2 The Licensee shall have the primary obligation and right to take action against any third party alleged to be infringing the Patents or making unauthorised use of the Know-how or the Materials and to defend the Patents against challenges to validity or ownership at its sole expense, provided that:

(a) the Licensee and UCLB shall use their commercially reasonable efforts to eliminate the infringement without litigation. If the efforts of the Licensee, and UCLB are not successful in eliminating the infringement within [***] ([***]) days after the infringer has been formally notified of the infringement by the Licensee, the Licensee shall have the right after consulting with UCLB, to commence suit on its own account;

(b) UCLB shall on the Licensee's request cooperate with the Licensee in such action [***];

(c) the Licensee shall be solely responsible for the conduct of the action or for settlement thereof and shall be entitled to all damages received from such action, subject to Clause 8.2.4; and

(d) if the Licensee is unsuccessful in persuading the alleged infringer to desist within [***] ([***]) months of the Licensee first becoming aware of any potential infringement of the Patents for any Licensed Technology or fails without a commercially reasonable basis (relative the Licensed Technology or to other technology licenced to Licensee by UCLB) to initiate an infringement action within [***] ([***]) months of becoming aware of such infringement, UCLB shall have the right, at its sole discretion, to prosecute such infringement under its sole control and [***].

8.2.3 Before starting or defending or settling any legal action under Clause 8.2.2, the Licensee shall consult with UCLB as to the advisability of the action or defence or settlement, its effect on the good name of UCLB, the public interest, and how the action or defence should be conducted.

8.2.4 The Licensee shall [***] in such action or defence.

8.2.5 UCLB shall if reasonably requested by the Licensee agree to be joined in any suit to enforce such rights or will take such action in its own name [***] and shall have the right to be separately represented by its own counsel [***]. Notwithstanding the foregoing, [***].

8.3 **Infringement of Third Party Rights**

8.3.1 If any warning letter or other notice of infringement is received by the Licensee or UCLB, or legal suit or other action is brought against the Licensee or UCLB, alleging infringement of third party rights in the manufacture, use or sale of any Licensed Product or use of any Patents, Know-how or Materials, that Party shall (in the case of UCLB) promptly provide full details to the Licensee and (in the case of the Licensee) promptly provide full details to UCLB, and the Licensee and UCLB shall discuss the best way to respond.

8.3.2 The Licensee shall have the right but not the obligation to defend such suit to the extent it relates to Licensee's or its Affiliates' or Sub-licensee's activities and shall have the right to settle with such third party, provided that [***]. In the event that the Licensee, Affiliates or Sub-licensees do not take forward an action, UCLB shall have the right, at its sole discretion, to defend such suit under its sole control and [***].

9. WARRANTIES AND LIABILITY

9.1 **Warranties by UCLB**

UCLB warrants as of the Original Commencement Date and undertakes as follows to its reasonable knowledge and without having undertaken any due and careful enquires whether specific or general in nature:

9.1.1 It is the owner of the Patents;

9.1.2 it has the authority to grant the licences under this Agreement; and.

9.1.3 so far as it is aware (having made no enquiry of any third parties or conducted any freedom to operate searches), use and exploitation of the Patents will not infringe the intellectual property rights of any third party.

UCLB warrants and undertakes:

9.1.4 it has full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on UCLB; and

9.2 **Warranties by the Licensee**

The Licensee warrants as of the Original Commencement Date and undertakes that in respect of the Licensed Technology that:

9.2.1 full power and authority to enter into and perform this Agreement, which, when executed, will constitute valid and legally binding obligations on the Licensee;

9.2.2 entry into this Agreement will not result in any breach of, or violation of the terms or provisions of, the constitutional documents of the Licensee or any other agreement or instrument by which it is bound;

9.2.3 so far as it is aware (having made no enquiry of any third parties), use and exploitation of the Patents will not infringe the intellectual property rights of any third party;

9.2.4 neither it nor any of its Affiliates is currently researching, developing, marketing, selling or otherwise commercialising any Competing Product ("**Competing Activities**"), nor has any of them entered into an agreement with any other person with respect to any Competing Activities; and

9.2.5 it shall notify UCLB if it or any of its Affiliates or its Sub-licensees commences any Competing Activities or enters into an agreement with any other person with respect to any Competing Activities.

9.3 **Warranties by Meira**

Licensee warrants as of the Original Commencement Date and undertakes that full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on Licensee.

9.4 **Acknowledgements**

The Licensee acknowledges that:

9.4.1 the inventions claimed in the Patents, and the Know-how and the Materials are at an early stage of development. Accordingly, specific results cannot be guaranteed and any results, materials, information or other items (together "**Delivered Items**") provided under this Agreement are provided "as is" and without any express or implied warranties, representations or undertakings. As examples, but without limiting the foregoing, UCLB does not give any warranty that Delivered Items are of merchantable or satisfactory quality, are fit for any particular purpose, comply with any sample or description, or are viable, uncontaminated, safe or non-toxic.

9.4.2 UCLB has not performed any searches or investigations into the existence of any third party rights that may affect any of the Patents, Know-how or Materials or the use and exploitation of any of the Patents, Know-how or Materials.

9.5 **No Other Warranties**

9.5.1 Each of the Parties acknowledges that, in entering into this Agreement, it does not do so in reliance on any representation, warranty or other provision except as expressly provided in this Agreement, and any conditions, warranties or other terms implied by statute or common law are excluded from this Agreement to the fullest extent permitted by law.

9.5.2 Without limiting the scope of Clause 9.5.1, UCLB does not make any representation nor give any warranty or undertaking:

(a) express or implied, including, without limitation, any implied warranties of merchantability or of fitness for a particular purpose with respect to any Patent, trademark, software, non-public or other information, or tangible research property, licensed or otherwise provided to the Licensee hereunder and hereby disclaims the same;

(b) as to the efficacy or usefulness of the Patents, Know-how or Materials; or

(c) whatsoever with regard to the scope of any of the Patents or that any of the Patents is or will be valid or (in the case of an application) will proceed to grantor that such Patents may be exploited by the Licensee, Affiliate or Sub-licensee without infringing other patents; or

(d) that the Materials or the method used in making or using the Materials are free from liability for patent infringement; or

(e) that the use of any of the Patents, Know-how or Materials, Licensed Technology, the manufacture, sale or use of the Licensed Products, or the exercise of any of the rights granted under this Agreement will not infringe any intellectual property or other rights of any other person; or

(f) that the Know-how or any other information communicated by UCLB to the Licensee under or in connection with this Agreement will produce Licensed Products of satisfactory quality or fit for the purpose for which the Licensee intended or that any product will not have any defect, latent or otherwise, and whether or not discoverable by inspection; or

(g) as imposing any obligation on UCLB to bring or prosecute actions or proceedings against third parties for infringement or to defend any action or proceedings for revocation of any of the Patents; or

(h) as imposing any liability on UCLB in the event that any third party supplies Licensed Products to customers located in the Territory; or

(i) that there will be no similar or competitive products or services manufactured, used, sold or supplied by any third party in the Territory.

9.6 **Responsibility for Development of Licensed Products**

The Licensee shall be exclusively responsible for its and its Affiliates' and Sub-licensees' use of the Patents, Know-how and Materials, the technical and commercial development and manufacture of Licensed Products and for incorporating any modifications or developments thereto that may be necessary or desirable, for all Licensed Products sold or supplied, notwithstanding any consultancy services or other contributions that UCLB and/or UCL may provide in connection with such activities.

9.7 **Indemnity**

The Licensee shall indemnify each of UCLB and UCL, and each of their respective officers, directors, Council members, employees and representatives (together, the "**Indemnitees**") against all third party Claims that may be asserted against or suffered by any of the Indemnitees and which relate to:

9.7.1 the use by the Licensee or any of its Affiliates or Sub-licensees of any of the Patents, Know-how or Materials; or

9.7.2 use of the Licensed Technology by or on behalf of the Licensee or any of its Affiliates or Sub-licensees; or

9.7.3 the development, manufacture, use, marketing or sale of, or any other dealing in, any of the Licensed Products, by or on behalf of the Licensee or any of its Affiliates or Sub-licensees, or subsequently by any customer or any other person, including claims based on product liability laws.

The indemnity given by the Licensee to each Indemnitee under this Clause 9.7 will not apply to any third party Claim to the extent that it is attributable to the negligence, gross negligence, reckless misconduct or intentional misconduct of any Indemnitee.

9.8 **Limitations of Liability**

9.8.1 To the extent that UCLB or any of its Affiliates has any liability in contract, tort, or otherwise under or in connection with this Agreement, including any liability for breach of warranty, their liability shall be limited in accordance with the following provisions of this Clause 9.8.

9.8.2 The aggregate liability of UCLB and any of its Affiliates shall be limited to the total income that UCLB has received from the Licensee pursuant to this Agreement (but excluding any other costs or expenses associated with drafting, filing, prosecuting, maintaining or defending any Patents or providing any assistance to the Licensee) during the period of [***] ([***]) years preceding the date on which the liability arises, or fifty thousand pounds (£50,000) sterling, whichever is the higher.

9.8.3 The liability of the Licensee to UCLB shall be limited to the limit of its insurance as set out in Clause 9.9.1, except that in the case of product liability, the liability of the Licensee under this Agreement shall be unlimited.

9.8.4 In no circumstances shall any Party or any Indemnitee be liable for any loss, damage, costs or expenses of any nature that is (a) of an indirect, special or consequential nature or (b) any loss of profits (whether direct or indirect), revenue, business opportunity or goodwill, which arises directly or indirectly from that Party's breach or nonperformance of this Agreement, or negligence in the performance of this Agreement or from any liability arising in any other way out of the subject matter of this Agreement even if the Party bringing the claim has advised any other Party or the relevant Indemnitee of the possibility of those losses arising, or if such losses were within the contemplation of the Parties or the Indemnitee.

9.8.5 Nothing in this Agreement excludes any Party's liability to the extent that it may not be so excluded under applicable law, including any such liability for death or personal injury caused by that Party's negligence, or liability for fraud or fraudulent misrepresentation.

9.9 **Insurance**

9.9.1 The Licensee shall take out with a reputable insurance company and maintain at all times during the term of this Agreement public and product liability and professional indemnity insurance including against all loss of and damage to property (whether real, personal or intellectual) and injury to persons including death arising out of or in connection with this Agreement and the Licensee's and its Affiliates' and Sub-licensees' use of the Patents, Know-how or Materials and use, sale of or any other

dealing in any of the Licensed Products. Such insurances may be limited in respect of one claim provided that such limit must be at least [***] pounds (£[***]) sterling, unless the Licensee commences any business in manufacturing, distribution, supply or otherwise make available to the public any products, in which case such limit must be at least [***] pounds (£[***]) sterling. Such insurance shall continue to be maintained for a further [***] years from the end of this Agreement.

9.9.2 The Licensee will produce to UCLB at all times upon demand proof that the insurance cover required pursuant to Clause 9.9.1 is in force and evidence that all premiums have been paid up to date. If UCLB becomes aware that the Licensee has failed to maintain the insurance required pursuant to Clause 9.9.1, UCLB may effect such insurance and the Licensee will reimburse UCLB for the reasonable cost of effecting and maintaining such insurance on demand.

10. DURATION AND TERMINATION

10.1 Commencement and Expiry

This Agreement shall commence as of the Commencement Date and, unless terminated earlier in accordance with this Clause 10, the licences granted hereunder shall continue in force on a country by country basis until the later of the last payment obligation of Licensee expires under this Agreement. Upon such expiry, Licensee's licenses under this Agreement shall become full-paid, perpetual and irrevocable.

10.2 Termination of the Original Agreement

Upon the Commencement Date, pursuant to Amendment No. 4 to the Original Agreement, the Licensed Technology shall be excluded from the Original Agreement, and Licence Addendum Number 1 shall be deemed to have terminated. All other provisions of the Original Agreement (including the options set out in Clause 2.6 of the Original Agreement which have not been exercised by the Licensee prior to the Commencement Date, i.e., the options in relation to Specified Technologies [***]) together with any accrued rights of the Parties under the Original Agreement prior to the Commencement Date shall continue in full force and effect.

10.3 Early Termination

Each Party (the "**Terminating Party**") may terminate this Agreement at any time by notice in writing to the other Parties ("**Other Parties**"), such notice to take effect as specified in the notice:

10.3.1 If, in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, is in material breach of this Agreement and, in the case of a breach capable of remedy within thirty (30) days, the breach is not remedied within thirty (30) days of the Other Parties receiving notice specifying the breach and requiring its remedy or where the breach relates to non-payment of an undisputed sum due under this Agreement, the sum is not paid in full within fourteen (14) days following the Other Party receiving notice specifying the nonpayment and requiring payment in full; provided however, that in respect on breaches not relating to non-payment, if such breach is capable of being cured but cannot be cured within such thirty (30) day period and the Other Party initiates actions to cure such breach within such period and thereafter diligently pursues such actions, the Other Party shall have such additional period as is reasonable under the circumstances to cure such breach; it being understood that no such extension shall apply with respect to any undisputed payment obligations or extend beyond six (6) months from the end of such thirty (30) day period. In the event there is a genuine dispute between the Parties with respect to any alleged breach hereunder, no purported termination of this Agreement pursuant to this Clause 10.3.1 shall take effect while the Parties are actively working to resolve such dispute; or

10.3.2 if:

(a) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, becomes insolvent or unable to pay its debts as and when they become due;

(b) an order is made or a resolution is passed for the winding up of in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB (other than voluntarily for the purpose of solvent amalgamation or reconstruction);

(c) a liquidator, administrator, administrative receiver, receiver or trustee is appointed in respect of the whole or any part of, in the case of UCLB, either of the Other Parties', or in the case of the Licensee or Meira, UCLB's, assets or business;

(d) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, makes any composition with its creditors;

(e) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, ceases to continue its business; or

(f) any event analogous to the events referred to in paragraphs (a) to (e) above occurs in any other jurisdiction.

10.4 UCLB may terminate this Agreement by giving written notice to the Licensee and Meira, such termination to take effect forthwith or as otherwise stated in the notice:

10.4.1 if there is any change of Control of the Licensee involving the categories of persons or Affiliates of persons prohibited by Clause 2.3;

10.4.2 the Licensee is in persistent breach of the Agreement at least [***] ([***]) times in a calendar year, wherein the nature of each such breach is the same in each instance, and where the Parties have failed to agree on a mechanism to remedy the persistent nature of such breaches within a reasonable period following UCLB notifying Licensee of the persistent breaches and requesting that the Licensee enters into discussions with UCLB as to mechanisms for remedying the persistent breaches or if the Parties have agreed to a mechanism to remedy the persistence of such breach by that mechanism if not fully complied with by the Licensee; or

10.4.3 if the Licensee shall enter into any sub-licence with any of the categories of persons or Affiliates of persons prohibited by Clause 2.3 which may, adversely affect UCL's and/or UCLB's reputation;

10.4.4 subject to Clause 5.5, if the Licensee fails to achieve any of the milestone events described in Schedule 4 provided that if achievement of any of the milestone events should be compromised due to technical, legal or regulatory issues, the Parties shall first meet and UCLB will work with the Licensee to manage the delivery schedule and provided that the Licensee is using Diligent Efforts to correct the issues, the applicable deadline in Schedule 4 shall be extended by six (6) months or such other time period as shall be agreed between the Parties in writing after which if the Licensee has not achieved the milestone UCLB shall be entitled, subject to Clauses 5.11 and 5.12, to terminate this Agreement by giving written notice to the Licensee.

10.5 A Party's right of termination under this Agreement, and the exercise of any such right, shall be without prejudice to any other right or remedy (including any right to claim damages) that such Party may have in the event of a breach of contract or other default by any other Party.

10.6 Consequences of Termination

10.6.1 Upon expiry of the period of this Agreement, and subject to all royalties and any other sums due to UCLB under this Agreement having been duly paid, the Licensee shall have a fully paid up licence to the Patents, the Know-how and the Materials of the same scope as set forth in Clause 2.1 without any further obligation to pay any further sums to UCLB under Clause 4. Notwithstanding the foregoing the Licensee acknowledges that once each Patent expires or is abandoned or withdrawn or allowed to lapse in any country or territory, third parties in that country or territory will be entitled to use the inventions claimed in the Patent and that accordingly the licence granted to the Licensee under Clause 2.1 will no longer be exclusive in that country or territory.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.6.2 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4:

(a) the Licensee and its Affiliates and Sub-licensees shall be entitled to sell, use or otherwise dispose of (subject to payment of royalties under Clause 4) any unsold or unused stocks of the Licensed Products for a period of [***] ([***]) months following the date of termination;

(b) subject to paragraph (a) above, any license that has not become fully paid-up in accordance with Clause 10.1 shall terminate and the Licensee and its Affiliates (and subject to Clause 2.3, its Sub-licensees) shall no longer be licensed to use or otherwise exploit the Patents and/or the Know-how and/or the Materials, in so far and for as long as any of the Patents remains in force and the Know-how remains confidential;

(c) the Licensee shall consent to the cancellation of any formal licence granted to it, or of any registration of it in any register, in relation to any of the Patents;

(d) the Licensee will, promptly on UCLB's request, provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which are in the possession of Licensee or its patent agents relating to the Patents and which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents;

(e) except as set out in Clause 2.3, all sub-licences of the Patents and/or the Know-how and/or the Materials granted by the Licensee pursuant to this Agreement will automatically terminate;

(f) UCLB shall, upon the written request of either of the other Parties, and each of the Licensee and Meira shall, upon the written request of UCLB, return or destroy any documents or other materials that are in its or its Affiliates possession or under its or their control and that contain the requesting Party's Confidential Information.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.6.3 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, in the event that UCLB would be unable, absent a licence from the Licensee, to use or permit others to use or to exploit or permit others to exploit the relevant Licensed Technology without infringing intellectual property rights in any invention developed by the Licensee, whether solely or jointly with others ("Blocking Invention"), the Licensee shall be deemed to have granted UCLB the irrevocable non-exclusive right to use, exploit and permit others to use and exploit the Blocking Invention only in conjunction with the relevant Licensed Technology. The Licensee shall at the request of UCLB provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents licensed under this Agreement;

10.7 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, the Licensee shall, to the extent it is able to do so without being in breach of any obligation owed to a third party, disclose to UCLB full details of any and all Intellectual Property generated at any time by or on behalf of the Licensee as a result of the exercise of the Licensee's rights under this Agreement ("Licensee IP") and, upon UCLB's written request within [***] ([***]) days following such disclosure, negotiate in good faith to agree the terms of an exclusive or non-exclusive licence to UCLB (as UCLB may request) under the Licensee IP. If the Parties fail to agree the terms of such a licence within [***] days following commencement of such negotiation, despite negotiating in good faith, UCLB's rights under this Clause shall lapse. If Licensee may terminate this Agreement under Clause 10.3.1 (for UCLB or its Affiliates uncured material breach), then Licensee may elect, in lieu of terminating the entire Agreement, to have all licenses granted to the Licensee under this Agreement continue in force, subject to Licensee's fulfilment of [***] percent ([***]%) of its payment obligations under Clause 4 after what would have been the effective date of such termination.

10.8 Upon termination of this Agreement for any reason, the provisions of Clauses 1, 2.3, 2.5, 3.1 to 3.5, 4 (in respect of amounts paid and payable to UCLB in respect of the period up to and including the date of termination), 5.7, 7, 9, 10.7, 10.8, 10.8 and 11 of this Agreement shall remain in force**.**

11. GENERAL

11.1 Force Majeure

11.1.1 Any delays in or failure of performance by a Party under this Agreement will not be considered a breach of this Agreement and if and to the extent that such delay or failure is caused by occurrences beyond the reasonable control of that Party including acts of God; acts, regulations and laws of any government; strikes or other concerted acts of workers; fire; floods; explosions; riots; wars; rebellion; and sabotage; and any time for performance hereunder will be extended by the actual time of delay caused by any such occurrence.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.1.2 If (a) UCLB or (b) the Licensee or Meira is prevented from carrying out its obligations:

(a) under this Agreement for a continuous period of [***] ([***]) months, the Licensee (in the case of (a)) or UCLB (in the case of (b)), may terminate this Agreement on giving [***] ([***]) days prior written notice provided always that at the date upon which termination becomes effective the Party which was prevented from carrying out its obligations under this Agreement remains so prevented.

11.2 Amendment

This Agreement may only be amended in writing signed by duly authorised representatives of the Parties.

11.3 **Assignment and Third Party Rights**

11.3.1 Subject to Clause 11.3.3, the Licensee shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement, nor any of the Patents, Know-how or Materials, without the prior written consent of UCLB.

11.3.2 UCLB may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party.

11.3.3 The Licensee, subject to obtaining the consent of UCLB which shall not be unreasonably withheld or delayed (except in relation to those categories of persons or Affiliates of persons prohibited by Clause 2.3), may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party to which it transfers all or substantially all of its assets or business, provided that the assignee undertakes to UCLB to be bound by and perform the obligations of the assignor under this Agreement. However, the Licensee shall not have such a right to assign this Agreement if it is insolvent.

11.3.4 Meira shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement without the prior written consent of UCLB.

11.4 Waiver

Any waiver given under or in relation to this Agreement shall be in writing and signed by or on behalf of the relevant Party. No failure or delay on the part of a Party to exercise any right or remedy under this Agreement shall be construed or operate as a waiver thereof, nor shall any single or partial exercise of any right or remedy preclude the further exercise of such right or remedy.

11.5 Invalid Clauses

If any provision or part of this Agreement is held to be invalid, amendments to this Agreement may be made by the addition or deletion of wording as appropriate to remove the invalid part or provision but otherwise retain the provision and the other provisions of this Agreement to the maximum extent permissible under applicable law.

11.6 No Agency

No Party shall act or describe itself as the agent of the other, nor shall it make or represent that it has authority to make any commitments on the other's behalf.

11.7 Interpretation

In this Agreement:

11.7.1 the headings are used for convenience only and shall not affect its interpretation; references to persons shall include incorporated and unincorporated persons; references to the singular include the plural and vice versa; and references to the masculine include the feminine;

11.7.2 references to Clauses and Schedules mean clauses of, and schedules to, this Agreement;

11.7.3 references in this Agreement to termination shall include termination by expiry;

11.7.4 where the word "including" is used it shall be understood as meaning "including without limitation";

11.7.5 any reference to any English law term for any action, remedy, method or judicial proceeding, legal document, legal status, court, official or any legal concept or thing shall in respect of any jurisdiction other than England be deemed to include what most nearly approximates in that jurisdiction to the English law term;

11.7.6 where there is any conflict or inconsistency between the main body of this Agreement and any of the schedules, then the main body of the Agreement shall prevail;

11.7.7 time shall be of the essence in relation to the performance of Meira's and the Licensee's obligations under this Agreement; and

11.7.8 any reference to the sale of a Licensed Product by the Licensee or its Affiliates or Sub-licensees will be taken to include any supply or other disposal of Licensed Products, and the term sold shall be construed accordingly.

11.8 **Notices. Addresses for Service**

11.8.1 Any notice to be given under this Agreement shall be in English, in writing and shall be delivered by first class recorded delivery mail (if sent to an inland address) or by international courier (if sent to an address outside of the United Kingdom), to the address of the relevant Party set out at the head of this Agreement, or such other address as that Party may from time to time notify to the other Parties in accordance with this Clause 11.8.

11.8.2 Notices sent as above shall be deemed to have been received [***] ([***]) working day after the day of posting in the case of delivery inland first class recorded delivery mail, or [***] ([***]) working days after the date of collection by the international courier.

11.9 Law and Jurisdiction

The validity, construction and performance of this Agreement, and any contractual and non-contractual claims arising hereunder, shall be governed by English law and shall be subject to the exclusive jurisdiction of the English courts to which the Parties hereby submit, except that a Party may seek an interim injunction (or an equivalent remedy) in any court of competent jurisdiction.

11.10 Entire Agreement

This Agreement, including its Schedules, sets out the entire agreement between the Parties relating to its subject matter and supersedes all prior oral or written agreements, arrangements or understandings between them relating to such subject matter including, without limitation, the parts of the Original Agreement that pertains to the Specified Technology [***] and the Licence Addendum Number 1. Subject to Clause 9.8.5, the Parties acknowledge that they are not relying on any representation, agreement, term or condition which is not set out in this Agreement. For clarity, this Agreement is not intended to supersede or affect the effectiveness of the parts of the Original Agreement that does not pertain to the Specified Technology [***] and the Licence Addendum Number 1. Such parts of the Original Agreement that does not pertain to the Specified Technology [***] and the Licence Addendum Number 1 includes, without limitation, the parts that pertains to the Specified Technology [***] through [***].

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.11 Third Parties

Except for the rights of UCL as provided in Clause 2.4, the rights of the Indemnitees as provided in Clause 9.7, the limitations of liability afforded to the Indemnitees pursuant to Clause 9.8, who may in their own right enforce and rely on the provisions of those Clauses, this Agreement does not create any right enforceable by any person who is not a party to it ("**Third Party**") under the Contracts (Rights of Third Parties) Act 1999, but this Clause 11.11 does not affect any right or remedy of a Third Party which exists or is available apart from that Act.

11.12 **Non-use of Names; Announcements**

11.12.1 The Licensee shall not use, and shall ensure that its Affiliates and Sub-licensees do not use, the name, any adaptation of the name, any logo, trademark or other device of UCLB, nor of the inventors named on the Patents nor the Principal Investigators in any advertising, promotional or sales materials without prior written consent obtained from UCLB in each case, except that the Licensee may state that it is licensed by UCLB under the Patents.

11.12.2 Except as permitted under Clauses 3.3.1 and 5.7, no Party shall make any press or other public announcement concerning any aspect of this Agreement, or make any use of the name or trademarks of any other Party in connection with or in consequence of this Agreement, without the prior written consent of the relevant other Party.

11.13 **Escalation**

If the Licensee or Meira on the one hand, and UCLB on the other, are unable to reach agreement on any issue concerning this Agreement or the Project within [***] days after one either has notified the other of that issue, they will refer the matter to the [***] in the case of UCLB, and to the [***] in the case of the Licensee and Meira in an attempt to resolve the issue within the time specified elsewhere in this Agreement in the case of other disputes. Any Party may bring proceedings in a court of competent jurisdiction if the matter has not been resolved within that prescribed period, and any Party may apply to the court for an injunction, whether or not any issue has been escalated under this Clause 11.13.

[Signature Page Follows]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

EXECUTED on the date set out at the head of this Agreement.

For and on behalf of

UCL Business PLC

/s/ [***]
Signed

[***]
Print name

[***]
Title

29 January 2019
Date

For and on behalf of

MEIRAGTX UK II LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

For and on behalf of

MEIRAGTX LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 1

LICENSED TECHNOLOGY

Part A: The Patents

[***]

Part B: The Know-how

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 2

APPOINTMENT OF EXPERT

For the purposes of this Schedule 2 only, the "Parties" shall mean the Licensee and UCLB. If either Party wishes to appoint an independent expert (the "**Expert**") to determine any matter pursuant to any Clause of this Agreement, the following procedures will apply:

1. The Party wishing to appoint the Expert (the "**Appointing Party**") will serve a written notice on the other Party (the "**Responding Party**"). The written notice will specify the Clause pursuant to which the appointment is to be made and will contain reasonable details of the matter(s) which the Appointing Party wishes to refer to the Expert for determination.

2. The Parties shall within [***] ([***]) days following the date of the Appointing Party's written notice use all reasonable efforts to agree who is to be appointed as the Expert to determine the relevant matter(s). If the Parties are unable to agree upon the identity of the Expert within that timescale, the Expert shall be appointed by the President (for the time being) of the Licensing Executives Society Britain and Ireland upon written request of either Party.

3. Each Party will within [***] ([***]) days following appointment of the Expert, prepare and submit to the Expert and the other Party a detailed written statement setting out its position on the matter(s) in question and including any proposals which it may wish to make for settlement or resolution of the relevant matter.

4. Each Party will have [***] ([***]) days following receipt of the other Party's written statement to respond in writing thereto. Any such response will be submitted to the other Party and the Expert.

5. The Expert will if he/ she deems appropriate be entitled to seek clarification from the Parties as to any of the statements or proposals made by either Party in their written statement or responses. Each Party will on request make available all information in its possession and shall give such assistance to the Expert as may be reasonably necessary to permit the Expert to make his/ her determination.

6. The Expert will issue his/ her decision on the matter(s) referred to him/ her in writing as soon as reasonably possible, but at latest within [***] ([***]) months following the date of his/ her appointment. The Expert's decision shall (except in the case of manifest error) be final and binding on the Parties.

7. The Expert will at all times act as an independent and impartial expert and not as an arbitrator.

8. The Expert's charges will be borne as he/ she determines in his written decision.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 3

DEFINITION OF TOBACCO INDUSTRY FUNDING (REVISED 2009)

FROM THE CANCER RESEARCH UK CODE OF PRACTICE ON TOBACCO INDUSTRY FUNDING TO UNIVERSITIES.

http://www.cancerresearchuk.org/science/funding/terms-conditions/funding-policies/policy-tobacco/

A tobacco company is defined for the purposes of this policy as one that:

- Derives over 5% of revenues from manufacturing tobacco products;
- Derives 15%+ of revenues from the manufacture of products necessary for the production of tobacco products;
- Derives 15% of revenues from the sale of tobacco products (and has 30 or more staff);
- Owns a tobacco company (the company owns 50% or more of a tobacco company);
- Is more than 50% owned by a company with tobacco involvement.

The following do not constitute tobacco industry funding for the purposes of this Code:

- legacies from tobacco industry investments (provided these are sold on immediately)
- funding from a trust or foundation no longer having any connection with the tobacco industry even though it may bear a name that (for historical reasons) has tobacco industry associations.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 4

DEVELOPMENT PLAN

The Development Plan for the Licensed Technology is shown below

Activity	Timeline
Preclin/manufacture	[***]
Initiate Phase I/II	[***]
Initiate Phase II/III	[***]
Complete Phase II/III	[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

LICENCE AGREEMENT

between

UCL Business Plc

and

MeiraGTx UK II Limited

and

MeiraGTx Limited

Dated: 29 January 2019

Ref: [***] **(CNGA3)**

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

THIS AGREEMENT is made 29 January, 2019

BETWEEN:

(1) **UCL BUSINESS PLC**, a company incorporated in England and Wales under company registration number 02776963 whose registered office is The Network Building, 97 Tottenham Court Road, London W1T 4TP ("**UCLB**");

and

(2) **MEIRAGTX UK II LIMITED**, a company incorporated in England and Wales with registered number 09348737 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ (the "**Licensee**"); and

(3) **MEIRAGTX LIMITED,** a company incorporated in England and Wales with registered number 09501998 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ ("**Meira**").

WHEREAS:

A. University College London ("**UCL**") has developed certain ocular gene technologies and owns certain intellectual property rights relating to those gene therapies that the Licensee wishes to acquire rights to for the development and commercialisation of Licensed Products.

B. UCL has assigned to UCLB all of its right, title and interest in and to such property.

C. The Parties previously entered into a certain License Agreement dated as of March 15, 2018 (the "**Original Commencement Date**") (the "**Original Agreement**").

D. The Parties now desire to amend the Original Agreement and enter into a new agreement for the Specified Technology [***] on the terms set out in this Agreement.

E. It is the policy of UCLB that its activities in licensing intellectual property take into consideration ethical and socially responsible licensing principles, including ensuring that Licensed Products are made available to fulfil unmet needs in developing countries, and the Licensee acknowledges and agrees to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil such needs, all in accordance with the provisions of this Agreement.

NOW IT IS AGREED as follows:

1. DEFINITIONS

1.1 In this Agreement:

Agreement means this agreement (including the Schedules);

Affiliate in relation to a Party, means any entity or person that Controls, is Controlled by, or is under common Control with that Party;

Claims means all demands, claims and liability (whether criminal or civil, in contract, tort or otherwise) for losses, damages, costs and expenses of any nature whatsoever and all costs and expenses (including legal costs) incurred in connection therewith;

Commencement Date means the date of this Agreement;

Competing Product means any [***];

Confidential Information means the Know-how, the Materials and all other technical or commercial information that:

a) in respect of information provided in documentary form or by way of a model or in other tangible form, at the time of provision is marked or otherwise designated to show expressly that it is imparted in confidence or which a reasonable person would expect to be confidential; and

b) in respect of information that is imparted orally, any information that the Disclosing Party or its representatives informed the Receiving Party at the time of disclosure or which a reasonable person would expect to be confidential;

Control means direct or indirect beneficial ownership of 50% (or, outside a Party's home territory, such lesser percentage as is the maximum permitted level of foreign investment) or more of the share capital, stock or other participating interest carrying the right to vote or to distribution of profits of that Party, as the case may be;

Diligent Efforts means, with respect to efforts to be expended by the Licensee with respect to any objective under this Agreement, diligent, reasonable, good faith efforts to accomplish such objective [***] it being understood and agreed that with respect to the research, development, or commercialization of a product, [***], when utilizing sounds and reasonable scientific, medical and business practice and judgment;

Disclosing Party has the meaning given in Clause 3.2;

Field means ocular gene therapy;

First Commercial Sale means the first sale to a third party of a Licensed Product in a given regulatory jurisdiction after all regulatory and marketing approvals have been obtained for such Licensed Product in such jurisdiction. A sale shall not be deemed to have occurred if a Licensed Product is provided pursuant to an early access or compassionate use;

Indemnitees has the meaning given in Clause 9.7;

Intellectual Property means any and all patents, utility models, registered designs, unregistered design rights, copyright, database rights, rights in respect of confidential information, rights under data exclusivity laws, rights under orphan drug laws, rights under unfair competition laws, property rights in biological or chemical materials, extension of the terms of any such rights (including supplementary protection certificates), applications for and the right to apply any of the foregoing registered property and rights, and similar or analogous rights in any part of the Territory;

Know-how means:

a) the inventions claimed in the Patents; and

b) the technical information relating to the inventions claimed in the Patents and data described in the Part B of Schedule 1;

Licensed Products means any and all products that are developed, manufactured, used, or sold by or on behalf of the Licensee or its Affiliates or Sub-licensees and which (a) are within (or are manufactured using a process described in) any claim of the Patents; and/or (b) incorporate, or their development or manufacture makes use of, any of the Know-how and/or the Materials;

Licensed Technology means the Patents, the Know-how and the Materials set out in Schedule 1;

Materials means any and all of the materials referred to in Part C of Schedule 1;

Net Sales Value means in respect of [***]

Original Commencement Date has the meaning given in the Recitals;

Original Agreement has the meaning given in the Recitals;

Parties means UCLB, the Licensee and Meira, and "Party" shall mean either of them;

Patent Costs means [***].

Patents means any and all of the patents and patent applications referred to in Part A of Schedule 1;

Principal Investigators means [***].

Receiving Party has the meaning given in Clause 3.2;

Regulatory Exclusivity means, with respect to a Licensed Product, any exclusive rights or protection which are recognised, afforded or granted by any regulatory authority in any country or region with respect to the Licensed Product other than through patent rights;

Sub-licensee means any third party (other than an Affiliate) to whom the Licensee grants a sub-licence of its rights under this Agreement in accordance with Clause 2.3;

Territory means worldwide;

Valid Claim means a claim of a patent or patent application that has not been abandoned or allowed to lapse or expired or been held invalid or unenforceable by a court of competent jurisdiction in a final and non-appealable judgment.

2. GRANT OF RIGHTS

2.1 **Licence**

UCLB hereby grants to the Licensee and its Affiliates, and the Licensee hereby accepts on its own behalf and on behalf of its Affiliates, subject to the provisions of this Agreement:

2.1.1 an exclusive (even as to UCL) licence under the Patents, the Know-how and the Materials, with the right to sub-license, subject to Clause 2.3, to develop, commercialise, manufacture, have manufactured, use, sell and have sold Licensed Products only in the Field and in the Territory.

2.2 UCLB shall at the Licensee's request and cost execute such formal licences as may be necessary to enable the Licensee to register the licences granted to it under this Agreement with the Patent Offices in the relevant Territory. Such formal licence will reflect the terms of this Agreement where possible and for the avoidance of doubt if there is a conflict in the terms of such formal licence and this Agreement, the terms of this Agreement shall prevail. [***]

2.3 **Sub-Licensing**

The Licensee shall have the right to grant sub-licenses under the license in Clause 2.1 to its Affiliates or other third parties through one or more levels of Sub-licensees except that the Licensee may not grant such a sub-license to any person or the Affiliates of any person involved in: the tobacco industry (as defined by the Cancer Research UK Code of Practice on Tobacco Industry Funding to Universities detailed in Schedule 3); arms dealing; gambling operations; the promotion of violence; child labour or any other illegal activity. A grant of any sub-licence shall be conditioned on the following:

(a) The Licensee shall enter into a written agreement with each Sub-licensee and shall ensure that the provisions of each sub-licence are consistent with the provisions of this Agreement, and the Licensee shall ensure that:

(i) the sub-licence sets out all the proposed terms agreed between the Licensee and the Sub-licensee, including, in particular, all terms as to remuneration;

(ii) the Sub-licensee will maintain complete and accurate records in sufficient detail to permit UCLB to confirm the accuracy of the calculation of royalty payments under this Agreement; and

(iii) the sub-licence imposes obligations of confidentiality on the Sub-licensee which are no less onerous than those set out in Clause 3.2.

(b) The Licensee shall procure that each Sub-licensee complies fully at all times with the provisions of its sub-licence.

(c) The Licensee shall be liable for all acts and omissions of its Sub-licensees that, if committed by the Licensee, would constitute a breach of any of the provisions of this Agreement.

(d) The Licensee shall provide UCLB with a copy of any sub-licence [***] ([***]) days after execution of such sub-licence, provided that the Licensee may redact confidential or proprietary terms from such copy, including financial terms.

(e) Each sub-licence shall terminate automatically upon termination of this Agreement for any reason (but not expiry of this Agreement under Clause 10.1), except where the Sub-licensee was not implicated in or at fault in any circumstances which led to the termination of this Agreement, UCLB shall on receiving a written request from the relevant Sub-licensee within [***] ([***]) days following the date of termination of this Agreement enter into a licence agreement with the Sub-licensee for the Licensed Technology on terms substantially the same as the terms set out in this Agreement (except that the Sub-licensee shall not be obliged to pay to UCLB any sums equivalent to those sums set out in Clauses 4.1 or 4.3 which have already been paid to UCLB by the Licensee prior to the date of termination).

2.4 **Reservation of Rights**

2.4.1 UCLB reserves for itself and UCL the non-exclusive, irrevocable, worldwide, royalty-free right to:

(a) Use the Licensed Technology in the Field solely for academic (non-commercially funded) research, publication and teaching; and

(b) Grant licenses to academic third parties to use the Licensed Technology in academic research collaborations with UCL and such licenses shall not be sublicensable; and

(c) Grant license of the Licensed Technology to post graduate student of UCL for the purpose of conducting a programme of post graduate academic research and such licenses shall not by sublicensable.

In exercising the rights described in Clause 2.4.1(b) and (c), UCL and UCLB shall comply with the provisions of Clause 3 as regards confidentiality of the Know-how.

2.4.2 UCL and UCLB will refer a request from a third party for a licence to use the Patents in clinical trials or for diagnostic purposes involving human subjects to the Licensee, and the Licensee shall liaise directly with such third party.

2.4.3 Except for the licences expressly granted by this Clause 2, UCLB grants no rights to the Licensee under this Agreement to or under any intellectual property other than the Patents, the Know-how and the Materials and hereby reserves all rights under the Patents, the Know-how and the Materials outside the Field.

2.4.4 Nothing in this Agreement shall limit or otherwise affect UCL's ability to apply for noncommercial grant funding or comply with such grant terms and conditions. In the event that any terms of this Agreement conflicts with the terms of any non-commercial grant funding, the Parties shall negotiate in good faith to amend the terms of this Agreement to allow UCL to access such funding provided that nothing herein shall require the Licensee to agree to alter or modify the scope of the licence granted to it in this Clause 2.

2.5 **Affiliates**

The Licensee shall:

2.5.1 ensure that its Affiliates comply fully with the terms of this Agreement;

2.5.2 be responsible for any breach of or non-compliance with this Agreement by its Affiliates as if the breach or non-compliance had been a breach or non-compliance by the Licensee;

2.5.3 indemnify in accordance with Clause 9.7 each of the Indemnitees against any Claims which are awarded against or suffered by any of the Indemnitees as a result of any breach of or non-compliance with this Agreement by its Affiliates; and

2.5.4 ensure that if any Affiliate ceases to be an Affiliate as a result of a change of Control or otherwise, that unless a sub-licence agreement in accordance with Clause 2.3 is entered into with such an Affiliate, such former Affiliate immediately upon such cessation:

(a) ceases developing, manufacturing, having manufactured, using, selling and/ or having sold Licensed Products and ceases all use or exploitation of the Licensed Technology, for as long as any of the relevant Patents remains in force and/or the Know-how remains confidential;

(b) returns to the Licensee or destroys any documents or other materials in the former Affiliate's possession or under its control and that contain Confidential Information provided under this Agreement relating to the Licensed Technology and/ or Licensed Products;

(c) to the extent possible, takes all action necessary to have any product licences, marketing authorisations, pricing and/ or reimbursement approvals (and any applications for any of the foregoing) which relate to Licensed Products transferred into the name of the Licensee.

2.6 Use of Licensed Technology in Combination

UCLB acknowledges and agrees that the Licensee shall be entitled to use the Licensed Technology in combination with other technology, patents, know-how and materials licensed by UCLB to the Licensee under separate licence agreements and with any improvements to the Licensed Technology developed or generated by the Licensee.

3. KNOW-HOW AND CONFIDENTIAL INFORMATION

3.1 Confidentiality of Know-how and Materials

The Licensee undertakes that for so long as the Know-how and/or the Materials remains confidential, it shall (and shall ensure that its Affiliates and Sub-licensees) take all reasonable precautions to prevent unauthorised access to the Know-how and the Materials and protect the Know-how and the Materials in the same manner as it (or they) protect(s) its (or their) own proprietary information, and shall not (and shall ensure that its Affiliates and Sub-licensees do not) use the Know-how or the Materials for any purpose, except as expressly licensed hereby and in accordance with the provisions of this Agreement. For the avoidance of doubt, to the extent that any Materials, Know-how or information relating to the Patents falls within the public domain (without any breach of this Agreement or any other obligation of confidentiality), then UCL, the Principal Investigators and UCLB shall be free to use such information without restriction in the same way that any third party would have the freedom to use it.

3.2 Confidentiality Obligations

Each Party ("**Receiving Party**") undertakes:

3.2.1 to maintain as secret and confidential all Confidential Information obtained from, in the case of UCLB, the Licensee or Meira as applicable, and in the case of the Licensee and Meira, UCLB ("**Disclosing Party**") in the course of or in anticipation of this Agreement and to respect the Disclosing Party's rights therein;

3.2.2 to use such Confidential Information only for the purposes of or as permitted by this Agreement; and

3.2.3 subject to Clause 3.3, to disclose such Confidential Information only to those of its employees, contractors, Affiliates, and Sub-licensees (if any) to whom and to the extent that such disclosure is reasonably necessary for the purposes of this Agreement.

3.3 Permitted Disclosure

3.3.1 The Licensee shall have the right to disclose Confidential Information received from UCLB to:

(a) potential or actual customers of Licensed Products to the extent reasonably necessary to promote the sale or use of Licensed Products and provided that the customer has agreed to confidentiality provisions at least as restrictive as set forth herein;

(b) to existing or potential Sub-licensees, collaborators, investors or lenders provided that such third parties have agreed to confidentiality provisions at least as restrictive as set forth herein; and

(c) to its Board of Directors (or similar governing body) and its counsel, accountants and other professional advisers.

3.4 **Exceptions to Obligations**

The provisions of Clause 3.2 shall not apply to Confidential Information which the Receiving Party can demonstrate by reasonable written evidence:

3.4.1 was, prior to the Original Commencement Date, in the possession of the Receiving Party and at its free disposal and was not obtained or otherwise acquired directly or indirectly from the Disclosing Party or its Affiliates or their respective employees, students or representatives; or

3.4.2 is subsequently disclosed to the Receiving Party without any obligations of confidence by a third party; or

3.4.3 is or becomes generally available to the public through no act or default of the Receiving Party or its agents, employees, Affiliates or Sub-licensees; or

3.4.4 the Receiving Party is required to disclose by or to the courts of any competent jurisdiction, or to any government regulatory agency or financial authority, provided that the Receiving Party shall:

(a) inform the Disclosing Party as soon as is reasonably practicable;

(b) at the Disclosing Party's request and cost seek to persuade the court, agency or authority to have the information treated in a confidential manner, where this is possible under the court, agency or authority's procedures; and

(c) where the disclosure is unavoidable, limit the disclosure of Confidential information to the minimum extent required by law; or

3.4.5 which a Party is advised by its information officer that it is required to disclose under the Freedom of Information Act 2000 or the Environmental Information Regulations 2004.

3.5 Disclosure to Employees

The Receiving Party shall procure that all of its employees, contractors, Affiliates and Sub-licensees who have access to any of the Disclosing Party's Confidential Information to which Clause 3.2 applies, shall be made aware of the obligations of confidence and are bound by obligations of confidentiality at least as restrictive as those set forth herein (which it undertakes to enforce and for which it is legally responsible) and the Receiving Party shall only disclose the Disclosing Party's Confidential Information to those of its subsidiaries, employees, and officers as need to have access thereto wholly necessarily and exclusively for the purposes of this Agreement.

4. CONSIDERATION

4.1 Milestone Payments

Within [***] ([***]) days following achievement of each of the following milestone events by Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant milestone event has been achieved, provide documentary evidence of such achievement as appropriate and pay to UCLB, within a period of [***] ([***]) days, the amount(s) set out next to such milestone event below:

Milestone Event	Amount to be paid
[***]	£[***]
[***]	£[***]

4.2 Annual Management Fees

On each date referred to in the following table, the Licensee shall pay to UCLB the annual management fee set out next to such date in the table.

Date	Amount to be paid
Upon each anniversary of the Original Commencement Date until [***]	£50,000

4.3 Sales Linked Milestone Payments

Upon the first achievement of each of the sales linked milestones set out in the following table by the Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant sales linked milestone has been achieved, provide the relevant documentary evidence and pay to UCLB the amount(s) set out next to such event in the table:

Sales Linked Milestones	Amount to be paid
When Net Sales Value reaches £[***]	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (When sales cumulatively reach £[***])	£[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£[***]

4.4 Royalties on Net Sales

For each Licensed Product in each country, the Licensee shall pay to UCLB a royalty of [***]% ([***] per cent) being a percentage of the Net Sales Value of such Licensed Product sold by Licensee, its Affiliates or Sub-licensees. The Licensee's obligations to pay such royalty for a given Licensed Product in a given country shall begin after the First Commercial Sale of such Licensed Product in such country and shall end on the later to occur of the following: (a) expiration of the last Valid Claim of a Patent claiming such Licensed Product in such country; or (b) the tenth (10th) anniversary of the date of such First Commercial Sale in such country; or (c) the expiration of any Regulatory Exclusivity with respect to such Licensed Products in the relevant country.

4.5 Combination Products

If any Licensed Products are incorporated in any other product ("**Combination Product**") sold by the Licensee or its Affiliates and the Licensed Product is not priced separately from the Combination Product, the Net Sales Value of such Licensed Product shall be deemed to be the fair market value of the Licensed Product in the country of sale when sold separately or if not sold separately in the country of sale, in comparable countries and territories or if neither of the foregoing apply, a reasonable amount which fairly reflects the value of the Licensed Product within the Combination Product assuming the Licensed Product is not being sold as a loss leader.

4.6 Payment Frequency

Royalties due under this Agreement, except for the payments due under Clauses, 4.1, 4.2 and 4.3, which are payable upon the date/time specified in Clauses 4.1, 4.2 and 4.3 as appropriate, shall be paid within [***] ([***]) days following the end of each calendar quarter ending on 31 March, 30 June, 30 September and 31 December in each year, in respect of sales of Licensed Products made during such quarter, and within [***] ([***]) days following the termination of this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.7 Payment terms

All sums due under this Agreement:

4.7.1 are exclusive of Value Added Tax which where applicable will be paid by the Licensee to UCLB in addition;

4.7.2 shall be paid in pounds sterling in cash by transferring an amount in aggregate to the following Account name: UCL Business Plc, Sort Code: 20 10 53, Account number: 30782270, Address: Barclays Bank Plc, PO Box 11345, London, W12 8GG, and in the case of income or amounts received by the Licensee or its Affiliates in a currency other than pounds sterling, the royalty shall be calculated in the other currency and then converted into equivalent pounds sterling at the relevant daily spot rate for that currency as quoted in the Financial Times newspaper on the last business day of the quarter in relation to which the royalties are payable;

4.7.3 will be made without any set-off, deduction or withholding except as may be required by law. If the Licensee is required by law to make any deduction or to withhold any part of any amount due to UCLB under this Agreement, the Licensee will give to UCLB proper evidence of the amount deducted or withheld and payment of that amount to the relevant taxation authority, and will do all things in its power to enable or assist UCLB to claim exemption from or, if that is not possible, to obtain a credit for the amount deducted or withheld under any applicable double taxation or similar agreement from time to time in force; and

4.7.4 shall be made by the due date, failing which UCLB may charge interest on any outstanding amount on a daily basis at a rate equivalent to [***]% above the Bank of England pound sterling base rate then in force in London.

4.8 Royalty Statements

The Licensee shall send to UCLB, at the same time as each royalty payment is made in accordance with Clause 4.4, a statement setting out for the relevant calendar quarter:

4.8.1 in respect of each territory or region in which Licensed Products are sold;

4.8.2 the types of Licensed Product sold;

4.8.3 the quantity of each type sold;

4.8.4 the total invoiced price for each type of Licensed Product sold;

4.8.5 where relevant, details of any Licensed Products that have been sold other than on arm's length terms for a cash consideration, including the relevant open market price or (if not available) the reasonable price attributed thereto;

4.8.6 the amounts deducted from the Net Sales Value as referred to in paragraph (i) to (iv) of that definition (broken down on a product by product and category by category basis); and

4.8.7 the aggregate royalties on Net Sales Value due to UCLB;

in each case expressed both in local currency and pounds sterling and showing the conversion rates used, during the period to which the royalty payment relates.

4.9 Records

The Licensee shall keep at its normal place of business detailed and up to date records and accounts showing the quantity, description and invoiced price or non-cash consideration for all Licensed Products sold by it or its Affiliates or on its or its Affiliates' behalf, broken down in each case on a country by country basis, and being sufficient to ascertain the payments due to UCLB under this Agreement.

The Licensee shall make such records and accounts available, on reasonable notice, for inspection during business hours by an independent chartered accountant nominated by UCLB for the purpose of verifying the accuracy of any statement or report given by the Licensee to UCLB under this Clause 4.9. The Licensee shall co-operate reasonably with any such accountant, and shall promptly provide all information and assistance reasonably requested by such accountant. The accountant shall be required to keep confidential all information learnt during any such inspection, and to disclose to UCLB only such details as may be necessary to report on the accuracy of the Licensee's statement or report. UCLB shall be responsible for the accountant's charges unless the accountant certifies that there is an inaccuracy of more than [***]% ([***] percent) in any royalty statement, in which case the Licensee shall pay his charges in respect of that inspection.

The Licensee shall ensure that UCLB has the same rights as those set out in this Clause 4.9 in respect of the Licensee's Affiliates and Sub-licensees.

The Licensee shall co-operate with UCLB in good faith to resolve any discrepancies identified during any such inspection and [***], together with interest on late payment as specified in Clause 4.7.4, within [***] following receipt of a copy of the independent chartered accountant's report.

4.10 Accounting Standards

Where this Agreement requires a financial calculation to be made or an action to be taken by a party, such calculation will be made or taken in accordance with the generally accepted accounting principles followed by such party.

5. COMMERCIALISATION

5.1 General Diligence

The Licensee shall use Diligent Efforts to develop and commercially exploit Licensed Products throughout the Territory (including obtaining all and any regulatory approvals which may be required to market and sell the Licensed Products) for the benefit of both Parties.

5.2 Competing Activities

The Licensee shall notify UCLB in confidence if it or any of its Affiliates or its Sub-licensees commences any marketing, sale or commercialisation of any Competing Product or enters into an agreement with any other person with respect to any such activities.

5.3 Development Plan

The Licensee's initial plan for developing and commercialising Licensed Products is set out in Schedule 4 (the "**Initial Development Plan**"). The Licensee shall provide to UCLB on each anniversary of the Original Commencement Date a written update to the Initial Development Plan that shall:

5.3.1 report on all activities conducted under this Agreement by the Licensee and its Affiliates and Sub-licensees since the Original Commencement Date or the date of the previous update (as appropriate);

5.3.2 (where applicable) set out the milestone events achieved since the Original Commencement Date or the date of the previous update (as appropriate) and the Licensee's reasonable estimate of the dates for achieving any future milestone events;

5.3.3 set out the current and projected activities being taken or planned to be taken by the Licensee and its Affiliates and Sub-licensees to bring Licensed Products to market in the Territory; and

5.3.4 set out the projected sales of Licensed Products (based on the Licensee's or Sub-licensee's current forecasts) for each of the next [***] ([***]) years following the date of the report.

UCLB's receipt or approval of any update to the Updated Development Plan shall not be taken to waive or qualify the Licensee's obligations under Clause 5.1.

5.4 Annual Meeting

In respect of the Licensed Technology, the Licensee will on UCLB's request meet with UCLB at least once per calendar year, following the submission of the update to the relevant Development Plan pursuant to Clause 5.3, to discuss progress with development and commercialisation of the Licensed Technology.

5.5 Development Milestones

In addition to the Licensee's obligations under Clause 5.1, the Licensee shall for each Licensed Technology use Diligent Efforts to achieve the development milestone events specified in Schedule 4 by the dates set out therein. In any instance in which it becomes apparent that a particular development milestone will not be met due to regulatory, technical, safety or efficacy-related reasons or Force Majeure event (pursuant to Clause 11.1), the Parties in good faith will agree upon an appropriate adjust of such milestone and any subsequent milestones.

5.6 Reporting of First Commercial Sale

The Licensee will promptly notify UCLB in writing of the First Commercial Sale of each Licensed Product on a commercial basis in each country within the Territory.

5.7 Reporting for Impact Purposes

5.7.1 The Licensee acknowledges that part of UCLB's purpose in licensing the Patents, Know-how and the Materials to the Licensee pursuant this Agreement is to ensure that the Patents, Know-how and the Materials are made available for use and commercial exploitation with the intention of benefitting society and the economy. In order to enable UCLB and UCL to monitor the benefit that they are providing, and to enable UCL to demonstrate the impact of its research activities, to society and the economy, the Licensee will upon request provide to UCLB [***], a written report describing in reasonable detail how it has used the Patents, Know-how and the Materials and the societal and economic benefits generated therefrom.

5.7.2 UCLB shall notify and seek permission from the Licensee in advance, in writing if it wishes to use any written reports received from the Licensee (and the information contained therein) pursuant to Clause 5.7.1 in applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities, supplying a written copy of the application for research or other grant related funding

or submission (or the relevant sections thereof). The Licensee will respond to UCLB in writing within [***] ([***]) days of receipt of such written information and subject to the removal of any confidential information as notified in such written request by the Licensee, UCLB and UCL shall be entitled to submit the approved applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities.

5.8 Quality

The Licensee shall ensure that all of the Licensed Products marketed by it and its Affiliates and Sub-licensees are of satisfactory quality and comply with all applicable laws and regulations in each part of the Territory.

5.9 Marking of Licensed Products

To the extent permitted under the laws of any country, the Licensee shall mark and cause its Affiliates and Sub-Licensees to mark each Licensed Product with the number of each issued Patent which applies to the Licensed Product and a statement that such Licensed Products are sold under licence from UCL Business plc.

5.10 Disposals of Licensed Products for Free

Notwithstanding the terms of Clause 5.1, the Licensee shall be entitled to supply a reasonable number of Licensed Products to third parties free of charge as promotional items for the purpose of establishing a market for the Licensed Products in the relevant country or territory or for research, evaluation and testing purposes, or for clinical development, provided that the quantity of Licensed Products supplied for free (or for the cost of manufacture) in each country or territory is not excessive and is in line with normal industry practice in such country or territory. Any Licensed Products disposed of to third parties in accordance with this Clause 5.10 shall not be taken into account for the purposes of calculating Net Sales Value.

5.11 Referral to Expert

If UCLB considers at any time during the period of this Agreement that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, then the matter shall be referred to an independent expert to answer the following questions:

5.11.1 whether the Licensee has complied with its obligations under Clause 5.1 or 5.3; and if not

5.11.2 what specific action the Licensee should have taken and/or now needs to take ("**Specific Action**") in order to fulfil such obligations and within what period the Specific Action should be taken ("**Action Period**").

The independent expert shall be appointed in accordance with the provisions of Schedule 2 and his decision shall be final and binding on the Parties.

5.12 Consequences of Expert's Decision

If the expert determines that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, and if the Licensee fails to take the Specific Action within the Action Period, UCLB shall be entitled, by giving, at any time within [***] ([***]) months after the end of that Action Period, not less than [***] ([***]) months' notice, to (a) convert the licence granted under Clause 2.1 into a non-exclusive licence or (b) terminate this Agreement.

6. ACCESS TO MEDICINES AND ETHICAL LICENSING

Licensee shall use Diligent Efforts to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil unmet needs in developing countries.

7. COMPLIANCE WITH LAWS

7.1 **General Compliance with Laws**

The Licensee will at all times (and will ensure its Affiliates and Sub-licensees) comply with all legislation, rules, regulations and statutory requirements applying to and obtain any consents necessary for its use of the Patents, the Know-how and the Materials, the development, manufacture, and sale of Licensed Products in any country or territory.

7.2 Bribery Act

The Licensee shall (and shall procure that any persons associated with it engaged in the performance of this Agreement including its Affiliates and Sub-licensees shall):

7.2.1 comply with all applicable laws and codes of practice relating to anti-bribery and anti-corruption including the Bribery Act 2010 and without prejudice to the foregoing generality, shall not engage in any activity, practice or conduct which would constitute an offence under sections 1, 2 or 6 of the Bribery Act 2010 or do or omit to do any act that will cause or lead UCLB to be in breach of the Bribery Act 2010;

7.2.2 comply with UCLB's ethics, anti-bribery and anti-corruption policies as notified to the Licensee from time to time and have, maintain in place and enforce throughout the term of this Agreement adequate procedures to ensure compliance with Clause 7.2.1; and

7.2.3 promptly report to UCLB any request or demand for any undue financial or other advantage of any kind received in connection with the performance of this Agreement.

For the purpose of this Clause 7.2, the meaning of adequate procedures and whether a person is associated with another person shall be determined in accordance with the Bribery Act 2010 (and any guidance issued under section 9 of that Act). Breach of this Clause 7.2 shall be deemed a material breach of this Agreement entitling UCLB to terminate under Clause 10.3.1.

7.3 Export Control Regulations

The Licensee shall ensure that, in using the Patents, Know-how or Materials and in selling Licensed Products, it and its Affiliates, employees, sub-contractors and Sub-licensees comply fully with any United Nations trade sanctions or EU or UK legislation or regulation, from time to time in force, which impose arms embargoes or control the export of goods, technology or software, including weapons of mass destruction and arms, military, paramilitary and security equipment and dual-use items (items designed for civil use but which can be used for military purposes) and certain drugs and chemicals.

8. INTELLECTUAL PROPERTY

8.1 **Obtain and Maintain the Patents**

8.1.1 The Licensee shall be responsible for the drafting, filing, prosecution and maintenance of all of the Patents at the Licensee's cost and expense. Subject to resource availability, UCLB shall use commercially reasonable efforts to provide such assistance as the Licensee may request to prosecute and maintain the Patents[***].

8.1.2 The Patents will be filed, prosecuted and maintained in the countries and territories where Licensee normally files its patent applications and patents for other gene therapy products. The Licensee shall notify UCLB of any decisions as to which (if any) additional countries to file and maintain Patents in.

8.1.3 The Licensee shall consult with UCLB in relation to all material changes to the patent claims or specifications that would have the effect of reducing or limiting the scope of the Patents, and not make any such changes without the prior written consent of UCLB. Such consent shall not be unreasonably withheld or delayed provided that UCLB has been given as much notice as is practicable, and in any event no less than [***] days' notice (or such shorter period for response dictated by the relevant patent office) of such proposed changes, and has been given an opportunity to file

divisionals, continuations and/or such other types of protection to cover any claims or subject matter that the Licensee intends to remove from the scope of the Patents. If UCLB fails to respond before the end of the [***] day period (or such shorter period for response dictated by the relevant patent office), the Licensee may proceed with the proposed changes to the patent claims or specifications. The Licensee will ensure that UCLB receives copies of all correspondence to and from Patent Offices in respect of the Patents, including copies of all documents generated in or with such correspondence, and shall be given reasonable notice (or such shorter period for response dictated by the relevant patent office) of and the opportunity to participate in any conference calls or meetings with the Licensee's patent attorneys in relation to the drafting, filing, prosecution and maintenance of the Patents, so that UCLB may be continuously informed of progress with the drafting, filing, prosecution and maintenance of the Patents. Such involvement of UCLB under this Clause 8.1.3 shall be at UCLB's cost and expense.

8.1.4 If the Licensee wishes to abandon any application contained with the Patents or not to maintain any such Patent, it shall give [***] ([***]) months' prior written notice to UCLB and on the expiry of such notice period the licences of the relevant Patents granted to the Licensee under this Agreement shall cease.

8.1.5 In the event that any of rights granted hereunder become non-exclusive, responsibility for the drafting, filing, prosecution and maintenance of all of the Patents shall revert to UCLB.

8.2 Infringement of the Patents, the Know-how and/or the Materials

8.2.1 The Licensee and UCLB shall promptly give to each other written notice if it becomes aware of any infringement or potential infringement of any of the Patents or any unauthorised use of the Know-how or the Materials or any challenge to the validity or ownership of the Patents, the Know-how or the Materials and the Licensee and UCLB shall consult with each other to decide the best way to respond to such infringement, unauthorised use or challenge.

8.2.2 The Licensee shall have the primary obligation and right to take action against any third party alleged to be infringing the Patents or making unauthorised use of the Know-how or the Materials and to defend the Patents against challenges to validity or ownership at its sole expense, provided that:

(a) the Licensee and UCLB shall use their commercially reasonable efforts to eliminate the infringement without litigation. If the efforts of the Licensee, and UCLB are not successful in eliminating the infringement within [***] ([***]) days after the infringer has been formally notified of the infringement by the Licensee, the Licensee shall have the right after consulting with UCLB, to commence suit on its own account;

(b) UCLB shall on the Licensee's request cooperate with the Licensee in such action [***];

(c) the Licensee shall be solely responsible for the conduct of the action or for settlement thereof and shall be entitled to all damages received from such action, subject to Clause 8.2.4; and

(d) if the Licensee is unsuccessful in persuading the alleged infringer to desist within [***] ([***]) months of the Licensee first becoming aware of any potential infringement of the Patents for any Licensed Technology or fails without a commercially reasonable basis (relative to the Licensed Technology or other technology licenced to Licensee by UCLB) to initiate an infringement action within [***] ([***]) months of becoming aware of such infringement, UCLB shall have the right, at its sole discretion, to prosecute such infringement under its sole control [***].

8.2.3 Before starting or defending or settling any legal action under Clause 8.2.2, the Licensee shall consult with UCLB as to the advisability of the action or defence or settlement, its effect on the good name of UCLB, the public interest, and how the action or defence should be conducted.

8.2.4 The Licensee shall [***] in such action or defence.

8.2.5 UCLB shall if reasonably requested by the Licensee agree to be joined in any suit to enforce such rights or will take such action in its own name [***] and shall have the right to be separately represented by its own counsel [***] Notwithstanding the foregoing, [***].

8.3 Infringement of Third Party Rights

8.3.1 If any warning letter or other notice of infringement is received by the Licensee or UCLB, or legal suit or other action is brought against the Licensee or UCLB, alleging infringement of third party rights in the manufacture, use or sale of any Licensed Product or use of any Patents, Know-how or Materials, that Party shall (in the case of UCLB) promptly provide full details to the Licensee and (in the case of the Licensee) promptly provide full details to UCLB, and the Licensee and UCLB shall discuss the best way to respond.

8.3.2 The Licensee shall have the right but not the obligation to defend such suit to the extent it relates to Licensee's or its Affiliates' or Sub-licensee's activities and shall have the right to settle with such third party, provided that [***]. In the event that the Licensee, Affiliates or Sub-licensees do not take forward an action, UCLB shall have the right, at its sole discretion, to defend such suit under its sole control and [***].

9. WARRANTIES AND LIABILITY

9.1 Warranties by UCLB

UCLB warrants as of the Original Commencement Date and undertakes as follows to its reasonable knowledge and without having undertaken any due and careful enquires whether specific or general in nature:

9.1.1 It is the owner of the Patents;

9.1.2 it has the authority to grant the licences under this Agreement; and.

9.1.3 so far as it is aware (having made no enquiry of any third parties or conducted any freedom to operate searches), use and exploitation of the Patents will not infringe the intellectual property rights of any third party.

UCLB warrants and undertakes:

9.1.4 it has full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on UCLB; and

9.2 Warranties by the Licensee

The Licensee warrants as of the Original Commencement Date and undertakes that in respect of the Licensed Technology that:

9.2.1 full power and authority to enter into and perform this Agreement, which, when executed, will constitute valid and legally binding obligations on the Licensee;

9.2.2 entry into this Agreement will not result in any breach of, or violation of the terms or provisions of, the constitutional documents of the Licensee or any other agreement or instrument by which it is bound;

9.2.3 so far as it is aware (having made no enquiry of any third parties), use and exploitation of the Patents will not infringe the intellectual property rights of any third party;

9.2.4 neither it nor any of its Affiliates is currently researching, developing, marketing, selling or otherwise commercialising any Competing Product ("**Competing Activities**"), nor has any of them entered into an agreement with any other person with respect to any Competing Activities; and

9.2.5 it shall notify UCLB if it or any of its Affiliates or its Sub-licensees commences any Competing Activities or enters into an agreement with any other person with respect to any Competing Activities.

9.3 Warranties by Meira

Licensee warrants as of the Original Commencement Date and undertakes that full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on Licensee.

9.4 Acknowledgements

The Licensee acknowledges that:

9.4.1 the inventions claimed in the Patents, and the Know-how and the Materials are at an early stage of development. Accordingly, specific results cannot be guaranteed and any results, materials, information or other items (together "**Delivered Items**") provided under this Agreement are provided "as is" and without any express or implied warranties, representations or undertakings. As examples, but without limiting the foregoing, UCLB does not give any warranty that Delivered Items are of merchantable or satisfactory quality, are fit for any particular purpose, comply with any sample or description, or are viable, uncontaminated, safe or non-toxic.

9.4.2 UCLB has not performed any searches or investigations into the existence of any third party rights that may affect any of the Patents, Know-how or Materials or the use and exploitation of any of the Patents, Know-how or Materials.

9.5 No Other Warranties

9.5.1 Each of the Parties acknowledges that, in entering into this Agreement, it does not do so in reliance on any representation, warranty or other provision except as expressly provided in this Agreement, and any conditions, warranties or other terms implied by statute or common law are excluded from this Agreement to the fullest extent permitted by law.

9.5.2 Without limiting the scope of Clause 9.5.1, UCLB does not make any representation nor give any warranty or undertaking:

(a) express or implied, including, without limitation, any implied warranties of merchantability or of fitness for a particular purpose with respect to any Patent, trademark, software, non-public or other information, or tangible research property, licensed or otherwise provided to the Licensee hereunder and hereby disclaims the same;

(b) as to the efficacy or usefulness of the Patents, Know-how or Materials; or

(c) whatsoever with regard to the scope of any of the Patents or that any of the Patents is or will be valid or (in the case of an application) will proceed to grantor that such Patents may be exploited by the Licensee, Affiliate or Sub-licensee without infringing other patents; or

(d) that the Materials or the method used in making or using the Materials are free from liability for patent infringement; or

(e) that the use of any of the Patents, Know-how or Materials, Licensed Technology, the manufacture, sale or use of the Licensed Products, or the exercise of any of the rights granted under this Agreement will not infringe any intellectual property or other rights of any other person; or

(f) that the Know-how or any other information communicated by UCLB to the Licensee under or in connection with this Agreement will produce Licensed Products of satisfactory quality or fit for the purpose for which the Licensee intended or that any product will not have any defect, latent or otherwise, and whether or not discoverable by inspection; or

(g) as imposing any obligation on UCLB to bring or prosecute actions or proceedings against third parties for infringement or to defend any action or proceedings for revocation of any of the Patents; or

(h) as imposing any liability on UCLB in the event that any third party supplies Licensed Products to customers located in the Territory; or

(i) that there will be no similar or competitive products or services manufactured, used, sold or supplied by any third party in the Territory.

9.6 Responsibility for Development of Licensed Products

The Licensee shall be exclusively responsible for its and its Affiliates' and Sub-licensees' use of the Patents, Know-how and Materials, the technical and commercial development and manufacture of Licensed Products and for incorporating any modifications or developments thereto that may be necessary or desirable, for all Licensed Products sold or supplied, notwithstanding any consultancy services or other contributions that UCLB and/or UCL may provide in connection with such activities.

9.7 Indemnity

The Licensee shall indemnify each of UCLB and UCL, and each of their respective officers, directors, Council members, employees and representatives (together, the "**Indemnitees**") against all third party Claims that may be asserted against or suffered by any of the Indemnitees and which relate to:

9.7.1 the use by the Licensee or any of its Affiliates or Sub-licensees of any of the Patents, Know-how or Materials; or

9.7.2 use of the Licensed Technology by or on behalf of the Licensee or any of its Affiliates or Sub-licensees; or

9.7.3 the development, manufacture, use, marketing or sale of, or any other dealing in, any of the Licensed Products, by or on behalf of the Licensee or any of its Affiliates or Sub-licensees, or subsequently by any customer or any other person, including claims based on product liability laws.

The indemnity given by the Licensee to each Indemnitee under this Clause 9.7 will not apply to any third party Claim to the extent that it is attributable to the negligence, gross negligence, reckless misconduct or intentional misconduct of any Indemnitee.

9.8 Limitations of Liability

9.8.1 To the extent that UCLB or any of its Affiliates has any liability in contract, tort, or otherwise under or in connection with this Agreement, including any liability for breach of warranty, their liability shall be limited in accordance with the following provisions of this Clause 9.8.

9.8.2 The aggregate liability of UCLB and any of its Affiliates shall be limited to the total income that UCLB has received from the Licensee pursuant to this Agreement (but excluding any other costs or expenses associated with drafting, filing, prosecuting, maintaining or defending any Patents or providing any assistance to the Licensee) during the period of [***] ([***]) years preceding the date on which the liability arises, or fifty thousand pounds (£50,000) sterling, whichever is the higher.

9.8.3 The liability of the Licensee to UCLB shall be limited to the limit of its insurance as set out in Clause 9.9.1, except that in the case of product liability, the liability of the Licensee under this Agreement shall be unlimited.

9.8.4 In no circumstances shall any Party or any Indemnitee be liable for any loss, damage, costs or expenses of any nature that is (a) of an indirect, special or consequential nature or (b) any loss of profits (whether direct or indirect), revenue, business opportunity or goodwill, which arises directly or indirectly from that Party's breach or nonperformance of this Agreement, or negligence in the performance of this Agreement or from any liability arising in any other way out of the subject matter of this Agreement even if the Party bringing the claim has advised any other Party or the relevant Indemnitee of the possibility of those losses arising, or if such losses were within the contemplation of the Parties or the Indemnitee.

9.8.5 Nothing in this Agreement excludes any Party's liability to the extent that it may not be so excluded under applicable law, including any such liability for death or personal injury caused by that Party's negligence, or liability for fraud or fraudulent misrepresentation.

9.9 Insurance

9.9.1 The Licensee shall take out with a reputable insurance company and maintain at all times during the term of this Agreement public and product liability and professional indemnity insurance including against all loss of and damage to property (whether real, personal or intellectual) and injury to persons including death arising out of or in connection with this Agreement and the Licensee's and its Affiliates' and Sub-licensees' use of the Patents, Know-how or Materials and use, sale of or any other dealing in any of the Licensed Products. Such insurances may be limited in respect of one claim provided that such limit must be at least [***] pounds (£[***]) sterling, unless the Licensee commences any business in manufacturing, distribution, supply or otherwise make available to the public any products, in which case such limit must be at least [***] pounds (£[***]) sterling. Such insurance shall continue to be maintained for a further [***] years from the end of this Agreement.

9.9.2 The Licensee will produce to UCLB at all times upon demand proof that the insurance cover required pursuant to Clause 9.9.1 is in force and evidence that all premiums have been paid up to date. If UCLB becomes aware that the Licensee has failed to maintain the insurance required pursuant to Clause 9.9.1, UCLB may effect such insurance and the Licensee will reimburse UCLB for the reasonable cost of effecting and maintaining such insurance on demand.

10. DURATION AND TERMINATION

10.1 Commencement and Expiry

This Agreement shall commence as of the Commencement Date and, unless terminated earlier in accordance with this Clause 10, the licences granted hereunder shall continue in force on a country by country basis until the later of the last payment obligation of Licensee expires under this Agreement. Upon such expiry, Licensee's licenses under this Agreement shall become full-paid, perpetual and irrevocable.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.2 Termination of the Original Agreement

Upon the Commencement Date, pursuant to Amendment No. 4 to the Original Agreement, the Original Agreement shall be deemed to have terminated.

10.3 Early Termination

Each Party (the "**Terminating Party**") may terminate this Agreement at any time by notice in writing to the other Parties ("**Other Parties**"), such notice to take effect as specified in the notice:

10.3.1 If, in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, is in material breach of this Agreement and, in the case of a breach capable of remedy within thirty (30) days, the breach is not remedied within thirty (30) days of the Other Parties receiving notice specifying the breach and requiring its remedy or where the breach relates to non-payment of an undisputed sum due under this Agreement, the sum is not paid in full within fourteen (14) days following the Other Party receiving notice specifying the nonpayment and requiring payment in full; provided however, that in respect on breaches not relating to non-payment, if such breach is capable of being cured but cannot be cured within such thirty (30) day period and the Other Party initiates actions to cure such breach within such period and thereafter diligently pursues such actions, the Other Party shall have such additional period as is reasonable under the circumstances to cure such breach; it being understood that no such extension shall apply with respect to any undisputed payment obligations or extend beyond six (6) months from the end of such thirty (30) day period. In the event there is a genuine dispute between the Parties with respect to any alleged breach hereunder, no purported termination of this Agreement pursuant to this Clause 10.3.1 shall take effect while the Parties are actively working to resolve such dispute; or

10.3.2 if:

(a) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, becomes insolvent or unable to pay its debts as and when they become due;

(b) an order is made or a resolution is passed for the winding up of in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB (other than voluntarily for the purpose of solvent amalgamation or reconstruction);

(c) a liquidator, administrator, administrative receiver, receiver or trustee is appointed in respect of the whole or any part of, in the case of UCLB, either of the Other Parties', or in the case of the Licensee or Meira, UCLB's, assets or business;

(d) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, makes any composition with its creditors;

(e) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, ceases to continue its business; or

(f) any event analogous to the events referred to in paragraphs (a) to (e) above occurs in any other jurisdiction.

10.4 UCLB may terminate this Agreement by giving written notice to the Licensee and Meira, such termination to take effect forthwith or as otherwise stated in the notice:

10.4.1 if there is any change of Control of the Licensee involving the categories of persons or Affiliates of persons prohibited by Clause 2.3;

10.4.2 the Licensee is in persistent breach of the Agreement at least [***] ([***]) times in a calendar year, wherein the nature of each such breach is the same in each instance, and where the Parties have failed to agree on a mechanism to remedy the persistent nature of such breaches within a reasonable period following UCLB notifying Licensee of the persistent breaches and requesting that the Licensee enters into discussions with UCLB as to mechanisms for remedying the persistent breaches or if the Parties have agreed to a mechanism to remedy the persistence of such breach by that mechanism if not fully complied with by the Licensee; or

10.4.3 if the Licensee shall enter into any sub-licence with any of the categories of persons or Affiliates of persons prohibited by Clause 2.3 which may, adversely affect UCL's and/or UCLB's reputation;

10.4.4 subject to Clause 5.5, if the Licensee fails to achieve any of the milestone events described in Schedule 4 provided that if achievement of any of the milestone events should be compromised due to technical, legal or regulatory issues, the Parties shall first meet and UCLB will work with the Licensee to manage the delivery schedule and provided that the Licensee is using Diligent Efforts to correct the issues, the applicable deadline in Schedule 4 shall be extended by six (6) months or such other time period as shall be agreed between the Parties in writing after which if the Licensee has not achieved the milestone UCLB shall be entitled, subject to Clauses 5.11 and 5.12, to terminate this Agreement by giving written notice to the Licensee.

10.5 A Party's right of termination under this Agreement, and the exercise of any such right, shall be without prejudice to any other right or remedy (including any right to claim damages) that such Party may have in the event of a breach of contract or other default by any other Party.

10.6 Consequences of Termination

10.6.1 Upon expiry of the period of this Agreement, and subject to all royalties and any other sums due to UCLB under this Agreement having been duly paid, the Licensee shall have a fully paid up licence to the Patents, the Know-how and the Materials of the same scope as set forth in Clause 2.1 without any further obligation to pay any further sums to UCLB under Clause 4. Notwithstanding the foregoing the Licensee acknowledges that once each Patent expires or is abandoned or withdrawn or allowed to lapse in any country or territory, third parties in that country or territory will be entitled to use the inventions claimed in the Patent and that accordingly the licence granted to the Licensee under Clause 2.1 will no longer be exclusive in that country or territory.

10.6.2 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4:

(a) the Licensee and its Affiliates and Sub-licensees shall be entitled to sell, use or otherwise dispose of (subject to payment of royalties under Clause 4) any unsold or unused stocks of the Licensed Products for a period of [***] ([***]) months following the date of termination;

(b) subject to paragraph (a) above, any license that has not become fully paid-up in accordance with Clause 10.1 shall terminate and the Licensee and its Affiliates (and subject to Clause 2.3, its Sub-licensees) shall no longer be licensed to use or otherwise exploit the Patents and/or the Know-how and/or the Materials, in so far and for as long as any of the Patents remains in force and the Know-how remains confidential;

(c) the Licensee shall consent to the cancellation of any formal licence granted to it, or of any registration of it in any register, in relation to any of the Patents;

(d) the Licensee will, promptly on UCLB's request, provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which are in the possession of Licensee or its patent agents relating to the Patents and which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents;

(e) except as set out in Clause 2.3, all sub-licences of the Patents and/or the Know-how and/or the Materials granted by the Licensee pursuant to this Agreement will automatically terminate;

(f) UCLB shall, upon the written request of either of the other Parties, and each of the Licensee and Meira shall, upon the written request of UCLB, return or destroy any documents or other materials that are in its or its Affiliates possession or under its or their control and that contain the requesting Party's Confidential Information.

10.6.3 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, in the event that UCLB would be unable, absent a licence from the Licensee, to use or permit others to use or to exploit or permit others to exploit the relevant Licensed Technology without infringing intellectual property rights in any invention developed by the Licensee, whether solely or jointly with others ("Blocking Invention"), the Licensee shall be deemed to have granted UCLB the irrevocable non-exclusive right to use, exploit and permit others to use and exploit the Blocking Invention only in conjunction with the relevant Licensed Technology. The Licensee shall at the request of UCLB provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents licensed under this Agreement;

10.7 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, the Licensee shall, to the extent it is able to do so without being in breach of any obligation owed to a third party, disclose to UCLB full details of any and all Intellectual Property generated at any time by or on behalf of the Licensee as a result of the exercise of the Licensee's rights under this Agreement ("Licensee IP") and, upon UCLB's written request within [***] ([***]) days following such disclosure, negotiate in good faith to agree the terms of an exclusive or non-exclusive licence to UCLB (as UCLB may request) under the Licensee IP. If the Parties fail to agree the terms of such a licence within [***] days following commencement of such negotiation, despite negotiating in good faith, UCLB's rights under this Clause shall lapse. If Licensee may terminate this Agreement under Clause 10.3.1 (for UCLB or its Affiliates uncured material breach), then Licensee may elect, in lieu of terminating the entire Agreement, to have all licenses granted to the Licensee under this Agreement continue in force, subject to Licensee's fulfilment of [***] percent ([***]%) of its payment obligations under Clause 4 after what would have been the effective date of such termination.

10.8 Upon termination of this Agreement for any reason, the provisions of Clauses 1, 2.3, 2.5, 3.1 to 3.5, 4 (in respect of amounts paid and payable to UCLB in respect of the period up to and including the date of termination), 5.7, 7, 9, 10.6, 10.7, 10.8 and 11 of this Agreement shall remain in force**.**

11. GENERAL

11.1 Force Majeure

11.1.1 Any delays in or failure of performance by a Party under this Agreement will not be considered a breach of this Agreement and if and to the extent that such delay or failure is caused by occurrences beyond the reasonable control of that Party including acts of God; acts, regulations and laws of any government; strikes or other concerted acts of workers; fire; floods; explosions; riots; wars; rebellion; and sabotage; and any time for performance hereunder will be extended by the actual time of delay caused by any such occurrence.

11.1.2 If (a) UCLB or (b) the Licensee or Meira is prevented from carrying out its obligations:

(a) under this Agreement for a continuous period of [***] ([***]) months, the Licensee (in the case of (a)) or UCLB (in the case of (b)), may terminate this Agreement on giving [***] ([***]) days prior written notice provided always that at the date upon which termination becomes effective the Party which was prevented from carrying out its obligations under this Agreement remains so prevented.

11.2 Amendment

This Agreement may only be amended in writing signed by duly authorised representatives of the Parties.

11.3 Assignment and Third Party Rights

11.3.1 Subject to Clause 11.3.3, the Licensee shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement, nor any of the Patents, Know-how or Materials, without the prior written consent of UCLB.

11.3.2 UCLB may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party.

11.3.3 The Licensee, subject to obtaining the consent of UCLB which shall not be unreasonably withheld or delayed (except in relation to those categories of persons or Affiliates of persons prohibited by Clause 2.3), may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party to which it transfers all or substantially all of its assets or business, provided that the assignee undertakes to UCLB to be bound by and perform the obligations of the assignor under this Agreement. However, the Licensee shall not have such a right to assign this Agreement if it is insolvent.

11.3.4 Meira shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement without the prior written consent of UCLB.

11.4 Waiver

Any waiver given under or in relation to this Agreement shall be in writing and signed by or on behalf of the relevant Party. No failure or delay on the part of a Party to exercise any right or remedy under this Agreement shall be construed or operate as a waiver thereof, nor shall any single or partial exercise of any right or remedy preclude the further exercise of such right or remedy.

11.5 Invalid Clauses

If any provision or part of this Agreement is held to be invalid, amendments to this Agreement may be made by the addition or deletion of wording as appropriate to remove the invalid part or provision but otherwise retain the provision and the other provisions of this Agreement to the maximum extent permissible under applicable law.

11.6 No Agency

No Party shall act or describe itself as the agent of the other, nor shall it make or represent that it has authority to make any commitments on the other's behalf.

11.7 Interpretation

In this Agreement:

11.7.1 the headings are used for convenience only and shall not affect its interpretation; references to persons shall include incorporated and unincorporated persons; references to the singular include the plural and vice versa; and references to the masculine include the feminine;

11.7.2 references to Clauses and Schedules mean clauses of, and schedules to, this Agreement;

11.7.3 references in this Agreement to termination shall include termination by expiry;

11.7.4 where the word "including" is used it shall be understood as meaning "including without limitation";

11.7.5 any reference to any English law term for any action, remedy, method or judicial proceeding, legal document, legal status, court, official or any legal concept or thing shall in respect of any jurisdiction other than England be deemed to include what most nearly approximates in that jurisdiction to the English law term;

11.7.6 where there is any conflict or inconsistency between the main body of this Agreement and any of the schedules, then the main body of the Agreement shall prevail;

11.7.7 time shall be of the essence in relation to the performance of Meira's and the Licensee's obligations under this Agreement; and

11.7.8 any reference to the sale of a Licensed Product by the Licensee or its Affiliates or Sub-licensees will be taken to include any supply or other disposal of Licensed Products, and the term sold shall be construed accordingly.

11.8 Notices. Addresses for Service

11.8.1 Any notice to be given under this Agreement shall be in English, in writing and shall be delivered by first class recorded delivery mail (if sent to an inland address) or by international courier (if sent to an address outside of the United Kingdom), to the address of the relevant Party set out at the head of this Agreement, or such other address as that Party may from time to time notify to the other Parties in accordance with this Clause 11.8.

11.8.2 Notices sent as above shall be deemed to have been received [***] ([***]) working day after the day of posting in the case of delivery inland first class recorded delivery mail, or [***] ([***]) working days after the date of collection by the international courier.

11.9 Law and Jurisdiction

The validity, construction and performance of this Agreement, and any contractual and non-contractual claims arising hereunder, shall be governed by English law and shall be subject to the exclusive jurisdiction of the English courts to which the Parties hereby submit, except that a Party may seek an interim injunction (or an equivalent remedy) in any court of competent jurisdiction.

11.10 Entire Agreement

This Agreement, including its Schedules, sets out the entire agreement between the Parties relating to its subject matter and supersedes all prior oral or written agreements, arrangements or understandings between them relating to such subject matter including, without limitation, the parts of the Original Agreement that pertains to the Specified Technology [***]. Subject to Clause 9.8.5, the Parties acknowledge that they are not relying on any representation, agreement, term or condition which is not set out in this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.11 Third Parties

Except for the rights of UCL as provided in Clause 2.4, the rights of the Indemnitees as provided in Clause 9.7, the limitations of liability afforded to the Indemnitees pursuant to Clause 9.8, who may in their own right enforce and rely on the provisions of those Clauses, this Agreement does not create any right enforceable by any person who is not a party to it ("**Third Party**") under the Contracts (Rights of Third Parties) Act 1999, but this Clause 11.11 does not affect any right or remedy of a Third Party which exists or is available apart from that Act.

11.12 **Non-use of Names; Announcements**

11.12.1 The Licensee shall not use, and shall ensure that its Affiliates and Sub-licensees do not use, the name, any adaptation of the name, any logo, trademark or other device of UCLB, nor of the inventors named on the Patents nor the Principal Investigators in any advertising, promotional or sales materials without prior written consent obtained from UCLB in each case, except that the Licensee may state that it is licensed by UCLB under the Patents.

11.12.2 Except as permitted under Clauses 3.3.1 and 5.7, no Party shall make any press or other public announcement concerning any aspect of this Agreement, or make any use of the name or trademarks of any other Party in connection with or in consequence of this Agreement, without the prior written consent of the relevant other Party.

11.13 **Escalation**

If the Licensee or Meira on the one hand, and UCLB on the other, are unable to reach agreement on any issue concerning this Agreement or the Project within [***] days after one either has notified the other of that issue, they will refer the matter to the [***] in the case of UCLB, and to the [***] in the case of the Licensee and Meira in an attempt to resolve the issue within the time specified elsewhere in this Agreement in the case of other disputes. Any Party may bring proceedings in a court of competent jurisdiction if the matter has not been resolved within that prescribed period, and any Party may apply to the court for an injunction, whether or not any issue has been escalated under this Clause 11.13.

[Signature Page Follows]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

EXECUTED on the date set out at the head of this Agreement.

For and on behalf of

UCL Business PLC

/s/ [***]
Signed

[***]
Print name

[***]
Title

29 January 2019
Date

For and on behalf of

MEIRAGTX UK II LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

For and on behalf of

MEIRAGTX LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

President and CEO
Title

Jan 29, 2019
Date

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 1

LICENSED TECHNOLOGY

Part A: The Patents

[***]

Part B: The Know-how

[***]

Part C: The Materials

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 2

APPOINTMENT OF EXPERT

For the purposes of this Schedule 2 only, the "Parties" shall mean the Licensee and UCLB. If either Party wishes to appoint an independent expert (the "**Expert**") to determine any matter pursuant to any Clause of this Agreement, the following procedures will apply:

1. The Party wishing to appoint the Expert (the "**Appointing Party**") will serve a written notice on the other Party (the "**Responding Party**"). The written notice will specify the Clause pursuant to which the appointment is to be made and will contain reasonable details of the matter(s) which the Appointing Party wishes to refer to the Expert for determination.

2. The Parties shall within [***] ([***]) days following the date of the Appointing Party's written notice use all reasonable efforts to agree who is to be appointed as the Expert to determine the relevant matter(s). If the Parties are unable to agree upon the identity of the Expert within that timescale, the Expert shall be appointed by the President (for the time being) of the Licensing Executives Society Britain and Ireland upon written request of either Party.

3. Each Party will within [***] ([***]) days following appointment of the Expert, prepare and submit to the Expert and the other Party a detailed written statement setting out its position on the matter(s) in question and including any proposals which it may wish to make for settlement or resolution of the relevant matter.

4. Each Party will have [***] ([***]) days following receipt of the other Party's written statement to respond in writing thereto. Any such response will be submitted to the other Party and the Expert.

5. The Expert will if he/ she deems appropriate be entitled to seek clarification from the Parties as to any of the statements or proposals made by either Party in their written statement or responses. Each Party will on request make available all information in its possession and shall give such assistance to the Expert as may be reasonably necessary to permit the Expert to make his/ her determination.

6. The Expert will issue his/ her decision on the matter(s) referred to him/ her in writing as soon as reasonably possible, but at latest within [***] ([***]) months following the date of his/ her appointment. The Expert's decision shall (except in the case of manifest error) be final and binding on the Parties.

7. The Expert will at all times act as an independent and impartial expert and not as an arbitrator.

8. The Expert's charges will be borne as he/ she determines in his written decision.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 3

DEFINITION OF TOBACCO INDUSTRY FUNDING (REVISED 2009)

FROM THE CANCER RESEARCH UK CODE OF PRACTICE ON TOBACCO INDUSTRY FUNDING TO UNIVERSITIES.

http://www.cancerresearchuk.org/science/funding/terms-conditions/funding-policies/policy-tobacco/

A tobacco company is defined for the purposes of this policy as one that:

- Derives over 5% of revenues from manufacturing tobacco products;
- Derives 15%+ of revenues from the manufacture of products necessary for the production of tobacco products;
- Derives 15% of revenues from the sale of tobacco products (and has 30 or more staff);
- Owns a tobacco company (the company owns 50% or more of a tobacco company);
- Is more than 50% owned by a company with tobacco involvement.

The following do not constitute tobacco industry funding for the purposes of this Code:

- legacies from tobacco industry investments (provided these are sold on immediately)
- funding from a trust or foundation no longer having any connection with the tobacco industry even though it may bear a name that (for historical reasons) has tobacco industry associations.

SCHEDULE 4

DEVELOPMENT PLAN

The Development Plan for the Licensed Technology is shown below

Activity	**Timeline**
Phase I/II Start	[***]
Phase I/II Finish	[***]
Phase III /pivotal confirmatory study Start	[***]
Phase III /pivotal confirmatory study Finish	[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 10.23

Confidential Treatment Requested by MeiraGTx Holdings plc

LICENCE AGREEMENT

between

UCL Business Plc

and

MeiraGTx UK II Limited

and

MeiraGTx Limited

Dated: 5 February 2019

Ref: [***] **(RPGR)**

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

THIS AGREEMENT is made 5 February, 2019

BETWEEN:

(1) **UCL BUSINESS PLC**, a company incorporated in England and Wales under company registration number 02776963 whose registered office is The Network Building, 97 Tottenham Court Road, London W1T 4TP ("**UCLB**");

and

(2) **MEIRAGTX UK II LIMITED**, a company incorporated in England and Wales with registered number 09348737 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ (the "**Licensee**"); and

(3) **MEIRAGTX LIMITED,** a company incorporated in England and Wales with registered number 09501998 and having its registered office at 92 Britannia Walk, London, United Kingdom, N1 7NQ ("**Meira**").

WHEREAS:

A. University College London ("**UCL**"), the Medical Research Council ("**MRC**"), Massachusetts Eye and Ear Infirmary ("**MEEI**"), and National Institutes of Health which is a part of the U.S. Department of Health and Human Services ("**NIH**") developed certain technology and owned certain intellectual property rights relating to a gene therapy for treating retinitis pigmentosa using an abbreviated form of a human Retinitis Pigmentosa GTPase Regulator (RPGR) gene that the Licensee wishes to acquire rights to for the development and commercialisation of Licensed Products.

B. UCL has assigned to UCLB all of its right, title and interest in and to such property. The NIH has licensed, maintaining certain reserved rights, all of its right, title and interest in and to the Patents, the Know-how and the Materials to MEEI. MEEI has granted to UCLB, maintaining certain reserved rights, an exclusive licence under the Patents and to use the Know-how and Materials.

C. The Licensee and UCLB previously entered into a certain License Agreement dated as of July 28, 2017 (the "**Original Commencement Date**") (the "**Original Agreement**").

D. The Parties now desire to amend the Original Agreement and enter into a new agreement for the Specified Technology [***] on the terms set out in this Agreement.

E. It is the policy of UCLB that its activities in licensing intellectual property take into consideration ethical and socially responsible licensing principles, including ensuring that Licensed Products are made available to fulfil unmet needs in developing countries, and the Licensee acknowledges and agrees to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil such needs, all in accordance with the provisions of this Agreement.

NOW IT IS AGREED as follows:

1. DEFINITIONS

1.1 In this Agreement:

Agreement means this agreement (including the Schedules);

Affiliate in relation to a Party, means any entity or person that Controls, is Controlled by, or is under common Control with that Party;

Claims means all demands, claims and liability (whether criminal or civil, in contract, tort or otherwise) for losses, damages, costs and expenses of any nature whatsoever and all costs and expenses (including legal costs) incurred in connection therewith;

Commencement Date means the date of this Agreement;

Competing Product means any [***];

Confidential Information means the Know-how, the Materials and all other technical or commercial information that:

a) in respect of information provided in documentary form or by way of a model or in other tangible form, at the time of provision is marked or otherwise designated to show expressly that it is imparted in confidence or which a reasonable person would expect to be confidential; and

b) in respect of information that is imparted orally, any information that the Disclosing Party or its representatives informed the Receiving Party at the time of disclosure or which a reasonable person would expect to be confidential;

Control means direct or indirect beneficial ownership of 50% (or, outside a Party's home territory, such lesser percentage as is the maximum permitted level of foreign investment) or more of the share capital, stock or other participating interest carrying the right to vote or to distribution of profits of that Party, as the case may be;

Diligent Efforts means, with respect to efforts to be expended by the Licensee with respect to any objective under this Agreement, diligent, reasonable, good faith efforts to accomplish such objective [***], it being understood and agreed that with respect to the research, development, or commercialization of a product, such efforts will be substantially equivalent to those that would normally be exerted or employed by [***], when utilizing sounds and reasonable scientific, medical and business practice and judgment;

Disclosing Party has the meaning given in Clause 3.2;

Field means ocular gene therapy;

First Commercial Sale means the first sale to a third party of a Licensed Product in a given regulatory jurisdiction after all regulatory and marketing approvals have been obtained for such Licensed Product in such jurisdiction. A sale shall not be deemed to have occurred if a Licensed Product is provided pursuant to an early access or compassionate use;

Indemnitees has the meaning given in Clause 9.7;

Intellectual Property means any and all patents, utility models, registered designs, unregistered design rights, copyright, database rights, rights in respect of confidential information, rights under data exclusivity laws, rights under orphan drug laws, rights under unfair competition laws, property rights in biological or chemical materials, extension of the terms of any such rights (including supplementary protection certificates), applications for and the right to apply any of the foregoing registered property and rights, and similar or analogous rights in any part of the Territory;

Know-how means:

a) the inventions claimed in the Patents; and

b) the technical information relating to the inventions claimed in the Patents and data described in the Part B of Schedule 1;

Licensed Products means any and all products that are developed, manufactured, used, or sold by or on behalf of the Licensee or its Affiliates or Sub-licensees and which (a) are within (or are manufactured using a process described in) any claim of the Patents; and/or (b) incorporate, or their development or manufacture makes use of, any of the Know-how and/or the Materials;

Licensed Technology means the Patents, the Know-how and the Materials set out in Schedule 1;

Materials means any and all of the materials referred to in Part C of Schedule 1;

MEEI lndemnitees has the meaning given in Clause 10.7.1;

Net Sales Value means in respect of [***]

Original Commencement Date has the meaning given in the Recitals;

Original Agreement has the meaning given in the Recitals;

Parties means UCLB, the Licensee and Meira, and “Party” shall mean either of them;

Patent Costs means [***].

Patents means any and all of the patents and patent applications referred to in Part A of Schedule 1;

Principal Investigators means [***].

Receiving Party has the meaning given in Clause 3.2;

Regulatory Exclusivity means, with respect to a Licensed Product, any exclusive rights or protection which are recognised, afforded or granted by any regulatory authority in any country or region with respect to the Licensed Product other than through patent rights;

Sub-licensee means any third party (other than an Affiliate) to whom the Licensee grants a sub-licence of its rights under this Agreement in accordance with Clause 2.3;

Territory means worldwide;

Valid Claim means a claim of a patent or patent application that has not been abandoned or allowed to lapse or expired or been held invalid or unenforceable by a court of competent jurisdiction in a final and non-appealable judgment.

2. GRANT OF RIGHTS

2.1 **Licence**

UCLB hereby grants to the Licensee and its Affiliates, and the Licensee hereby accepts on its own behalf and on behalf of its Affiliates, subject to the provisions of this Agreement:

2.1.1 an exclusive (even as to UCL) licence under the Patents, the Know-how and the Materials, with the right to sub-license, subject to Clause 2.3, to develop, commercialise, manufacture, have manufactured, use, sell and have sold Licensed Products only in the Field and in the Territory.

2.2 **UCLB shall at the Licensee's request and cost execute such formal licences as may be necessary to enable the Licensee to register the licences granted to it under this Agreement with the Patent Offices in the relevant Territory. Such formal licence will reflect the terms of this Agreement where possible and for the avoidance of doubt if there is a conflict in the terms of such formal licence and this Agreement, the terms of this Agreement shall prevail.** [***]**.**

2.3 **Sub-Licensing**

The Licensee shall have the right to grant sub-licenses under the license in Clause 2.1 to its Affiliates or other third parties through one or more levels of Sub-licensees except that the Licensee may not grant such a sub-license to any person or the Affiliates of any person involved in: the tobacco industry (as defined by the Cancer Research UK Code of Practice on Tobacco Industry Funding to Universities detailed in Schedule 3); arms dealing; gambling operations; the promotion of violence; child labour or any other illegal activity. A grant of any sub-licence shall be conditioned on the following:

(a) The Licensee shall enter into a written agreement with each Sub-licensee and shall ensure that the provisions of each sub-licence are consistent with the provisions of this Agreement, and the Licensee shall ensure that:

(i) the sub-licence sets out all the proposed terms agreed between the Licensee and the Sub-licensee, including, in particular, all terms as to remuneration;

(ii) the Sub-licensee will maintain complete and accurate records in sufficient detail to permit UCLB to confirm the accuracy of the calculation of royalty payments under this Agreement; and

(iii) the sub-licence imposes obligations of confidentiality on the Sub-licensee which are no less onerous than those set out in Clause 3.2.

(b) The Licensee shall procure that each Sub-licensee complies fully at all times with the provisions of its sub-licence.

(c) The Licensee shall be liable for all acts and omissions of its Sub-licensees that, if committed by the Licensee, would constitute a breach of any of the provisions of this Agreement.

(d) The Licensee shall provide UCLB with a copy of any sub-licence [***] ([***]) days after execution of such sub-licence, provided that the Licensee may redact confidential or proprietary terms from such copy, including financial terms.

(e) Each sub-licence shall terminate automatically upon termination of this Agreement for any reason (but not expiry of this Agreement under Clause 10.1), except where the Sub-licensee was not implicated in or at fault in any circumstances which led to the termination of this Agreement, UCLB shall on receiving a written request from the relevant Sub-licensee within [***] ([***]) days following the date of termination of this Agreement enter into a licence agreement with the Sub-licensee for the Licensed Technology on terms substantially the same as the terms set out in this Agreement (except that the Sub-licensee shall not be obliged to pay to UCLB any sums equivalent to those sums set out in Clauses 4.1 or 4.3 which have already been paid to UCLB by the Licensee prior to the date of termination).

2.4 Reservation of Rights

2.4.1 UCLB reserves for itself and UCL the non-exclusive, irrevocable, worldwide, royalty-free right to:

(a) Use the Licensed Technology in the Field solely for academic (non-commercially funded) research, publication and teaching; and

(b) Grant licenses to academic third parties to use the Licensed Technology in academic research collaborations with UCL and such licenses shall not be sublicensable; and

(c) Grant license of the Licensed Technology to post graduate student of UCL for the purpose of conducting a programme of post graduate academic research and such licenses shall not by sublicensable.

In exercising the rights described in Clause 2.4.1(b) and (c), UCL and UCLB shall comply with the provisions of Clause 3 as regards confidentiality of the Know-how.

2.4.2 UCLB reserves for MEEI the non-exclusive, irrevocable, worldwide, royalty-free right to:

(a) use the Patents, the Know-how and the Materials solely for MEEl's own internal non-commercially funded research, publication and teaching, excluding use in human subjects, clinical trials or for diagnostic purposes involving human subjects;

(b) to license other academic institutions to use the Patents, the Know-how and the Materials solely in non-commercially funded academic research collaborations with MEEI, excluding research for use in human subjects, clinical trials or research for diagnostic purposes involving human subjects; and

(c) to grant licences of the Patents, the Know-how and the Materials to other academic, governmental or not-for-profit organisations to use the Patents, the Know-how and the Materials solely for non-commercial research purposes and not for use in human subjects, clinical trials or for diagnostic purposes involving human subjects.

2.4.3 MEEI and UCLB will refer a request from a third party for a licence to use the Patents in clinical trials or for diagnostic purposes involving human subjects to the Licensee, and the Licensee shall liaise directly with such third party.

2.4.4 UCLB reserves for the U.S. Government only the irrevocable, royalty-free, paid-up right to practice and have practiced the rights under the Patents throughout the world by or on behalf of the U.S. Government and on behalf of any foreign government or international organisation pursuant to any existing or future treaty or agreement to which the U.S. Government is a signatory.

2.4.5 UCLB reserves the right, if required by the NIH, to grant sub-licenses of the rights under the Patents to responsible applicants, on terms that are reasonable under the circumstances when necessary to fulfill health or safety needs or when necessary to meet requirements for public use specified by U.S. Federal regulations.

2.4.6 UCLB reserves for the NIH only the right to require the Licensee, to grant sub-licenses of the rights under the Patents to responsible applicants, on terms that are reasonable under the circumstances when necessary to fulfill health or safety needs or when necessary to meet requirements for public use specified by U.S. Federal regulations.

2.4.7 In addition to the reserved rights of Clause 2.4.5, UCLB reserves the right, should it be required by the NIH, to grant a non-transferable, non-exclusive license to make and to use any tangible embodiment of the Patents and to practice any process(es) included within the Patents for purposes of internal research and not for purposes of commercial manufacture or distribution or in lieu of purchase, on reasonable terms and conditions.

2.4.8 In addition to the reserved rights of Clause 2.4.6, UCLB reserves for the NIH only the right to require the Licensee to grant a non-transferable, non-exclusive license to make and to use any tangible embodiment of the Patents and to practice any process(es) included within the Patents for purposes of internal research and not for purposes of commercial manufacture or distribution or in lieu of purchase, on reasonable terms and conditions.

2.4.9 UCLB reserves for the MRC the non-exclusive right to:

(a) use the Patents for its own internal, not-for-profit and non-commercially funded research, teaching and publicity, excluding use in human subjects, clinical trials or for diagnostic purposes involving human subjects; and

(b) licence academic institutions who employ [***] to use the Patents in not-for-profit and non-commercially funded research, excluding research for use in human subjects, clinical trials or research for diagnostic purposes involving human subjects.

2.4.10 Except for the licences expressly granted by this Clause 2, UCLB grants no rights to the Licensee under this Agreement to or under any intellectual property other than the Patents, the Know-how and the Materials and hereby reserves all rights under the Patents, the Know-how and the Materials outside the Field.

2.4.11 Nothing in this Agreement shall limit or otherwise affect UCL's ability to apply for non-commercial grant funding or comply with such grant terms and conditions. In the event that any terms of this Agreement conflicts with the terms of any non-commercial grant funding, the Parties shall negotiate in good faith to amend the terms of this Agreement to allow UCL to access such funding provided that nothing herein shall require the Licensee to agree to alter or modify the scope of the licence granted to it in this Clause 2.

2.5 Affiliates

The Licensee shall:

2.5.1 ensure that its Affiliates comply fully with the terms of this Agreement;

2.5.2 be responsible for any breach of or non-compliance with this Agreement by its Affiliates as if the breach or non-compliance had been a breach or non-compliance by the Licensee;

2.5.3 indemnify in accordance with Clause 9.7 each of the Indemnitees against any Claims which are awarded against or suffered by any of the Indemnitees as a result of any breach of or non-compliance with this Agreement by its Affiliates; and

2.5.4 ensure that if any Affiliate ceases to be an Affiliate as a result of a change of Control or otherwise, that unless a sub-licence agreement in accordance with Clause 2.3 is entered into with such an Affiliate, such former Affiliate immediately upon such cessation:

(a) ceases developing, manufacturing, having manufactured, using, selling and/ or having sold Licensed Products and ceases all use or exploitation of the Licensed Technology, for as long as any of the relevant Patents remains in force and/or the Know-how remains confidential;

(b) returns to the Licensee or destroys any documents or other materials in the former Affiliate's possession or under its control and that contain Confidential Information provided under this Agreement relating to the Licensed Technology and/ or Licensed Products;

(c) to the extent possible, takes all action necessary to have any product licences, marketing authorisations, pricing and/ or reimbursement approvals (and any applications for any of the foregoing) which relate to Licensed Products transferred into the name of the Licensee.

2.6 **Use of Licensed Technology in Combination**

UCLB acknowledges and agrees that the Licensee shall be entitled to use the Licensed Technology in combination with other technology, patents, know-how and materials licensed by UCLB to the Licensee under separate licence agreements and with any improvements to the Licensed Technology developed or generated by the Licensee.

3. KNOW-HOW AND CONFIDENTIAL INFORMATION

3.1 **Confidentiality of Know-how and Materials**

The Licensee undertakes that for so long as the Know-how and/or the Materials remains confidential, it shall (and shall ensure that its Affiliates and Sub-licensees) take all reasonable precautions to prevent unauthorised access to the Know-how and the Materials and protect the Know-how and the Materials in the same manner as it (or they) protect(s) its (or their) own proprietary information, and shall not (and shall ensure that its Affiliates and Sub-licensees do not) use the Know-how or the Materials for any purpose, except as expressly licensed hereby and in accordance with the provisions of this Agreement. For the avoidance of doubt, to the extent that any Materials, Know-how or information relating to the Patents falls within the public domain (without any breach of this Agreement or any other obligation of confidentiality), then UCL, the Principal Investigators and UCLB shall be free to use such information without restriction in the same way that any third party would have the freedom to use it.

3.2 **Confidentiality Obligations**

Each Party ("**Receiving Party**") undertakes:

3.2.1 to maintain as secret and confidential all Confidential Information obtained from, in the case of UCLB, the Licensee or Meira as applicable, and in the case of the Licensee and Meira, UCLB ("**Disclosing Party**") in the course of or in anticipation of this Agreement and to respect the Disclosing Party's rights therein;

3.2.2 to use such Confidential Information only for the purposes of or as permitted by this Agreement; and

3.2.3 subject to Clause 3.3, to disclose such Confidential Information only to those of its employees, contractors, Affiliates, and Sub-licensees (if any) to whom and to the extent that such disclosure is reasonably necessary for the purposes of this Agreement.

3.3 **Permitted Disclosure**

3.3.1 UCLB shall have the right to provide the MRC and MEEI with brief annual updates on the status of the commercial exploitation of the Patents, the Materials and the Know-how.

3.3.2 UCLB shall have the right to disclose to the MRC under binding obligations of confidentiality:

(a) that it has entered into this Agreement with the Licensee;

(b) details of all milestone payments and royalty payments provided for in this Agreement;

(c) a copy of the Initial Development Plan;

(d) details of any development milestones provided for in this Agreement.

3.3.3 The Licensee shall have the right to disclose Confidential Information received from UCLB to:

(a) potential or actual customers of Licensed Products to the extent reasonably necessary to promote the sale or use of Licensed Products and provided that the customer has agreed to confidentiality provisions at least as restrictive as set forth herein;

(b) to existing or potential Sub-licensees, collaborators, investors or lenders provided that such third parties have agreed to confidentiality provisions at least as restrictive as set forth herein; and

(c) to its Board of Directors (or similar governing body) and its counsel, accountants and other professional advisers.

3.4 **Exceptions to Obligations**

The provisions of Clause 3.2 shall not apply to Confidential Information which the Receiving Party can demonstrate by reasonable written evidence:

3.4.1 was, prior to the Original Commencement Date, in the possession of the Receiving Party and at its free disposal and was not obtained or otherwise acquired directly or indirectly from the Disclosing Party or its Affiliates or their respective employees, students or representatives; or

3.4.2 is subsequently disclosed to the Receiving Party without any obligations of confidence by a third party; or

3.4.3 is or becomes generally available to the public through no act or default of the Receiving Party or its agents, employees, Affiliates or Sub-licensees; or

3.4.4 the Receiving Party is required to disclose by or to the courts of any competent jurisdiction, or to any government regulatory agency or financial authority, provided that the Receiving Party shall:

(a) inform the Disclosing Party as soon as is reasonably practicable;

(b) at the Disclosing Party's request and cost seek to persuade the court, agency or authority to have the information treated in a confidential manner, where this is possible under the court, agency or authority's procedures; and

(c) where the disclosure is unavoidable, limit the disclosure of Confidential information to the minimum extent required by law; or

3.4.5 which a Party is advised by its information officer that it is required to disclose under the Freedom of Information Act 2000 or the Environmental Information Regulations 2004.

3.5 **Disclosure to Employees**

The Receiving Party shall procure that all of its employees, contractors, Affiliates and Sub-licensees who have access to any of the Disclosing Party's Confidential Information to which Clause 3.2 applies, shall be made aware of the obligations of confidence and are bound by obligations of confidentiality at least as restrictive as those set forth herein (which it undertakes to enforce and for which it is legally responsible) and the Receiving Party shall only disclose the Disclosing Party's Confidential Information to those of its subsidiaries, employees, and officers as need to have access thereto wholly necessarily and exclusively for the purposes of this Agreement.

3.6 **Response to NIH**

If the Licensee is notified of a determination of a conflict of interest regarding the Patents by the NIH it shall provide a response to such determination to the NIH within the period given by NIH to respond to such determination.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4. CONSIDERATION

4.1 Milestone Payments

Within [***] ([***]) days following achievement of each of the following milestone events by Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant milestone event has been achieved, provide documentary evidence of such achievement as appropriate and pay to UCLB, within a period of [***] ([***]) days, the amount(s) set out next to such milestone event below:

Milestone Event	Amount to be paid
[***]	£ [***]
[***]	£ [***]

4.2 Annual Management Fees

On each date referred to in the following table, the Licensee shall pay to UCLB the annual management fee set out next to such date in the table.

Date	Amount to be paid
Upon each anniversary of the Original Commencement Date until [***]	£50,000

4.3 Sales Linked Milestone Payments

Upon the first achievement of each of the sales linked milestones set out in the following table by the Licensee, its Affiliates or Sub-licensees, the Licensee shall notify UCLB in writing that the relevant sales linked milestone has been achieved, provide the relevant documentary evidence and pay to UCLB the amount(s) set out next to such event in the table:

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Sales Linked Milestones	**Amount to be paid**
When Net Sales Value reaches £[***]	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (When sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]
On the next £[***] of Net Sales Value (when sales cumulatively reach £[***])	£ [***]

4.4 Royalties on Net Sales

For each Licensed Product in each country, the Licensee shall pay to UCLB a royalty of [***]% ([***] per cent) being a percentage of the Net Sales Value of such Licensed Product sold by Licensee, its Affiliates or Sub-licensees. The Licensee's obligations to pay such royalty for a given Licensed Product in a given country shall begin after the First Commercial Sale of such Licensed Product in such country and shall end on the later to occur of the following: (a) expiration of the last Valid Claim of a Patent claiming such Licensed Product in such country; or (b) the tenth (10th) anniversary of the date of such First Commercial Sale in such country; or (c) the expiration of any Regulatory Exclusivity with respect to such Licensed Products in the relevant country.

4.5 Combination Products

If any Licensed Products are incorporated in any other product ("**Combination Product**") sold by the Licensee or its Affiliates and the Licensed Product is not priced separately from the Combination Product, the Net Sales Value of such Licensed Product shall be deemed to be the fair market value of the Licensed Product in the country of sale when sold separately or if not sold separately in the country of sale, in comparable countries and territories or if neither of the foregoing apply, a reasonable amount which fairly reflects the value of the Licensed Product within the Combination Product assuming the Licensed Product is not being sold as a loss leader.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.6 Payment Frequency

Royalties due under this Agreement, except for the payments due under Clauses, 4.1, 4.2 and 4.3, which are payable upon the date/time specified in Clauses 4.1, 4.2 and 4.3 as appropriate, shall be paid within [***] ([***]) days following the end of each calendar quarter ending on 31 March, 30 June, 30 September and 31 December in each year, in respect of sales of Licensed Products made during such quarter, and within [***] ([***]) days following the termination of this Agreement.

4.7 Payment terms

All sums due under this Agreement:

4.7.1 are exclusive of Value Added Tax which where applicable will be paid by the Licensee to UCLB in addition;

4.7.2 shall be paid in pounds sterling in cash by transferring an amount in aggregate to the following Account name: UCL Business Plc, Sort Code: 20 10 53, Account number: 30782270, Address: Barclays Bank Plc, PO Box 11345, London, W12 8GG, and in the case of income or amounts received by the Licensee or its Affiliates in a currency other than pounds sterling, the royalty shall be calculated in the other currency and then converted into equivalent pounds sterling at the relevant daily spot rate for that currency as quoted in the Financial Times newspaper on the last business day of the quarter in relation to which the royalties are payable;

4.7.3 will be made without any set-off, deduction or withholding except as may be required by law. If the Licensee is required by law to make any deduction or to withhold any part of any amount due to UCLB under this Agreement, the Licensee will give to UCLB proper evidence of the amount deducted or withheld and payment of that amount to the relevant taxation authority, and will do all things in its power to enable or assist UCLB to claim exemption from or, if that is not possible, to obtain a credit for the amount deducted or withheld under any applicable double taxation or similar agreement from time to time in force; and

4.7.4 shall be made by the due date, failing which UCLB may charge interest on any outstanding amount on a daily basis at a rate equivalent to [***] above the Bank of England pound sterling base rate then in force in London.

4.8 Royalty Statements

The Licensee shall send to UCLB, at the same time as each royalty payment is made in accordance with Clause 4.4, a statement setting out for the relevant calendar quarter:

4.8.1 in respect of each territory or region in which Licensed Products are sold;

4.8.2 the types of Licensed Product sold;

4.8.3 the quantity of each type sold;

4.8.4 the total invoiced price for each type of Licensed Product sold;

4.8.5 where relevant, details of any Licensed Products that have been sold other than on arm's length terms for a cash consideration, including the relevant open market price or (if not available) the reasonable price attributed thereto;

4.8.6 the amounts deducted from the Net Sales Value as referred to in paragraph (i) to (iv) of that definition (broken down on a product by product and category by category basis); and

4.8.7 the aggregate royalties on Net Sales Value due to UCLB;

in each case expressed both in local currency and pounds sterling and showing the conversion rates used, during the period to which the royalty payment relates.

4.9 Records

The Licensee shall keep at its normal place of business detailed and up to date records and accounts showing the quantity, description and invoiced price or non-cash consideration for all Licensed Products sold by it or its Affiliates or on its or its Affiliates' behalf, broken down in each case on a country by country basis, and being sufficient to ascertain the payments due to UCLB under this Agreement.

The Licensee shall make such records and accounts available, on reasonable notice, for inspection during business hours by an independent chartered accountant nominated by UCLB for the purpose of verifying the accuracy of any statement or report given by the Licensee to UCLB under this Clause 4.9. The Licensee shall co-operate reasonably with any such accountant, and shall promptly provide all information and assistance reasonably requested by such accountant. The accountant shall be required to keep confidential all information learnt during any such inspection, and to disclose to UCLB only such details as may be necessary to report on the accuracy of the Licensee's statement or report. UCLB shall be responsible for the accountant's charges unless the accountant certifies that there is an inaccuracy of more than [***]% ([***] percent) in any royalty statement, in which case the Licensee shall pay his charges in respect of that inspection.

The Licensee shall ensure that UCLB has the same rights as those set out in this Clause 4.9 in respect of the Licensee's Affiliates and Sub-licensees.

The Licensee shall co-operate with UCLB in good faith to resolve any discrepancies identified during any such inspection and [***]**,** together with interest on late payment as specified in Clause 4.7.4, within [***] following receipt of a copy of the independent chartered accountant's report.

4.10 **Accounting Standards**

Where this Agreement requires a financial calculation to be made or an action to be taken by a party, such calculation will be made or taken in accordance with the generally accepted accounting principles followed by such party.

5. COMMERCIALISATION

5.1 **General Diligence**

The Licensee shall use Diligent Efforts to develop and commercially exploit Licensed Products throughout the Territory (including obtaining all and any regulatory approvals which may be required to market and sell the Licensed Products) for the benefit of both Parties.

5.2 **Competing Activities**

The Licensee shall notify UCLB in confidence if it or any of its Affiliates or its Sub-licensees commences any marketing, sale or commercialisation of any Competing Product or enters into an agreement with any other person with respect to any such activities.

5.3 **Development Plan**

The Licensee's initial plan for developing and commercialising Licensed Products is set out in Schedule 4 (the "**Initial Development Plan**"). The Licensee shall provide to UCLB on each anniversary of the Original Commencement Date a written update to the Initial Development Plan that shall:

5.3.1 report on all activities conducted under this Agreement by the Licensee and its Affiliates and Sub-licensees since the Original Commencement Date or the date of the previous update (as appropriate);

5.3.2 (where applicable) set out the milestone events achieved since the Original Commencement Date or the date of the previous update (as appropriate) and the Licensee's reasonable estimate of the dates for achieving any future milestone events;

5.3.3 set out the current and projected activities being taken or planned to be taken by the Licensee and its Affiliates and Sub-licensees to bring Licensed Products to market in the Territory; and

5.3.4 set out the projected sales of Licensed Products (based on the Licensee's or Sub-licensee's current forecasts) for each of the next [***] ([***]) years following the date of the report.

UCLB's receipt or approval of any update to the Updated Development Plan shall not be taken to waive or qualify the Licensee's obligations under Clause 5.1.

5.4 **Annual Meeting**

In respect of the Licensed Technology, the Licensee will on UCLB's request meet with UCLB at least once per calendar year, following the submission of the update to the relevant Development Plan pursuant to Clause 5.3, to discuss progress with development and commercialisation of the Licensed Technology.

5.5 **Development Milestones**

In addition to the Licensee's obligations under Clause 5.1, the Licensee shall for each Licensed Technology use Diligent Efforts to achieve the development milestone events specified in Schedule 4 by the dates set out therein. In any instance in which it becomes apparent that a particular development milestone will not be met due to regulatory, technical, safety or efficacy-related reasons or Force Majeure event (pursuant to Clause 11.1), the Parties in good faith will agree upon an appropriate adjust of such milestone and any subsequent milestones.

5.6 **Reporting of First Commercial Sale**

The Licensee will promptly notify UCLB in writing of the First Commercial Sale of each Licensed Product on a commercial basis in each country within the Territory.

5.7 **Reporting for Impact Purposes**

5.7.1 The Licensee acknowledges that part of UCLB's purpose in licensing the Patents, Know-how and the Materials to the Licensee pursuant this Agreement is to ensure that the Patents, Know-how and the Materials are made available for use and commercial exploitation with the intention of benefitting society and the economy. In order to enable UCLB and UCL to monitor the benefit that they are providing, and to enable UCL to demonstrate the impact of its research activities, to society and the economy, the Licensee will upon request provide to UCLB [***], a written report describing in reasonable detail how it has used the Patents, Know-how and the Materials and the societal and economic benefits generated therefrom.

5.7.2 UCLB shall notify and seek permission from the Licensee in advance, in writing if it wishes to use any written reports received from the Licensee (and the information contained therein) pursuant to Clause 5.7.1 in applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities, supplying a written copy of the application for research or other grant related funding or submission (or the relevant sections thereof). The Licensee will respond to UCLB in writing within [***] ([***]) days of receipt of such written information and subject to the removal of any confidential information as notified in such written request by the Licensee, UCLB and UCL shall be entitled to submit the approved applications for research or other grant related funding and in submissions to Higher Education funding bodies such as HEFCE and/ or HEIF (or any replacements for either of those entities) and like entities.

5.8 **Quality**

The Licensee shall ensure that all of the Licensed Products marketed by it and its Affiliates and Sub-licensees are of satisfactory quality and comply with all applicable laws and regulations in each part of the Territory.

5.9 **Marking of Licensed Products**

To the extent permitted under the laws of any country, the Licensee shall mark and cause its Affiliates and Sub-Licensees to mark each Licensed Product with the number of each issued Patent which applies to the Licensed Product and a statement that such Licensed Products are sold under licence from UCL Business plc.

5.10 **Disposals of Licensed Products for Free**

Notwithstanding the terms of Clause 5.1, the Licensee shall be entitled to supply a reasonable number of Licensed Products to third parties free of charge as promotional items for the purpose of establishing a market for the Licensed Products in the relevant country or territory or for research, evaluation and testing purposes, or for clinical development, provided that the quantity of Licensed Products supplied for free (or for the cost of manufacture) in each country or territory is not excessive and is in line with normal industry practice in such country or territory. Any Licensed Products disposed of to third parties in accordance with this Clause 5.10 shall not be taken into account for the purposes of calculating Net Sales Value.

5.11 **Referral to Expert**

If UCLB considers at any time during the period of this Agreement that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, then the matter shall be referred to an independent expert to answer the following questions:

5.11.1 whether the Licensee has complied with its obligations under Clause 5.1 or 5.3; and if not

5.11.2 what specific action the Licensee should have taken and/or now needs to take ("**Specific Action**") in order to fulfil such obligations and within what period the Specific Action should be taken ("**Action Period**").

The independent expert shall be appointed in accordance with the provisions of Schedule 2 and his decision shall be final and binding on the Parties.

5.12 **Consequences of Expert's Decision**

If the expert determines that the Licensee has failed to comply with its obligations under Clause 5.1 or 5.3, and if the Licensee fails to take the Specific Action within the Action Period, UCLB shall be entitled, by giving, at any time within [***] ([***]) months after the end of that Action Period, not less than [***] ([***]) months' notice, to (a) convert the licence granted under Clause 2.1 into a non-exclusive licence or (b) terminate this Agreement.

5.13 **Use and sale in United States**

The Licensee agrees that for use and sale of the rights under the Patents in the United States, any products embodying the Patents, or produced through use of the Patents, shall be manufactured substantially in the United States, unless a waiver is granted by the NIH. UCLB shall use reasonable efforts, and shall ensure that MEEI use reasonable efforts to assist in the preparation and obtaining of such a waiver, if requested in writing by Licensee and at Licensee's reasonable expense.

6. ACCESS TO MEDICINES AND ETHICAL LICENSING

Licensee shall use Diligent Efforts to carry out its activities under this Agreement in a manner which complies with ethical and socially responsible licensing principles and which is designed to fulfil unmet needs in developing countries.

7. COMPLIANCE WITH LAWS

7.1 **General Compliance with Laws**

The Licensee will at all times (and will ensure its Affiliates and Sub-licensees) comply with all legislation, rules, regulations and statutory requirements applying to and obtain any consents necessary for its use of the Patents, the Know-how and the Materials, the development, manufacture, and sale of Licensed Products in any country or territory.

7.2 **Bribery Act**

The Licensee shall (and shall procure that any persons associated with it engaged in the performance of this Agreement including its Affiliates and Sub-licensees shall):

7.2.1 comply with all applicable laws and codes of practice relating to anti-bribery and anti-corruption including the Bribery Act 2010 and without prejudice to the foregoing generality, shall not engage in any activity, practice or conduct which would constitute an offence under sections 1, 2 or 6 of the Bribery Act 2010 or do or omit to do any act that will cause or lead UCLB to be in breach of the Bribery Act 2010;

7.2.2 comply with UCLB's ethics, anti-bribery and anti-corruption policies as notified to the Licensee from time to time and have, maintain in place and enforce throughout the term of this Agreement adequate procedures to ensure compliance with Clause 7.2.1; and

7.2.3 promptly report to UCLB any request or demand for any undue financial or other advantage of any kind received in connection with the performance of this Agreement.

For the purpose of this Clause 7.2, the meaning of adequate procedures and whether a person is associated with another person shall be determined in accordance with the Bribery Act 2010 (and any guidance issued under section 9 of that Act). Breach of this Clause 7.2 shall be deemed a material breach of this Agreement entitling UCLB to terminate under Clause 10.3.1.

7.3 **Export Control Regulations**

The Licensee shall ensure that, in using the Patents, Know-how or Materials and in selling Licensed Products, it and its Affiliates, employees, sub-contractors and Sub-licensees comply fully with any United Nations trade sanctions or EU or UK legislation or regulation, from time to time in force, which impose arms embargoes or control the export of goods, technology or software, including weapons of mass destruction and arms, military, paramilitary and security equipment and dual-use items (items designed for civil use but which can be used for military purposes) and certain drugs and chemicals.

8. INTELLECTUAL PROPERTY

8.1 **Obtain and Maintain the Patents**

8.1.1 The Licensee shall be responsible for the drafting, filing, prosecution and maintenance of all of the Patents at the Licensee's cost and expense. Subject to resource availability, UCLB shall use commercially reasonable efforts to provide such assistance as the Licensee may request to prosecute and maintain the Patents[***].

8.1.2 The Patents will be filed, prosecuted and maintained in the countries and territories where Licensee normally files its patent applications and patents for other gene therapy products. The Licensee shall notify UCLB of any decisions as to which (if any) additional countries to file and maintain Patents in.

8.1.3 The Licensee shall consult with UCLB in relation to all material changes to the patent claims or specifications that would have the effect of reducing or limiting the scope of the Patents, and not make any such changes without the prior written consent of UCLB. Such consent shall not be unreasonably withheld or delayed provided that UCLB has been given as much notice as is practicable, and in any event no less than [***] days' notice (or such shorter period for response dictated by the relevant patent office) of such proposed changes, and has been given an opportunity to file divisionals, continuations and/or such other types of protection to cover any claims or subject matter that the Licensee intends to remove from the scope of the Patents. If UCLB fails to respond before the end of the [***] day period (or such shorter period for response dictated by the relevant patent office), the Licensee may proceed with the proposed changes to the patent claims or specifications. The Licensee will ensure that UCLB receives copies of all correspondence to and from Patent Offices in respect of the Patents, including copies of all documents generated in or with such correspondence, and shall be given reasonable notice (or such shorter period for response dictated by the relevant patent office) of and the opportunity to participate in any conference calls or meetings with the Licensee's patent attorneys in relation to the drafting, filing, prosecution and maintenance of the Patents, so that UCLB may be continuously informed of progress with the drafting, filing, prosecution and maintenance of the Patents. Such involvement of UCLB under this Clause 8.1.3 shall be at UCLB's cost and expense.

8.1.4 If the Licensee wishes to abandon any application contained with the Patents or not to maintain any such Patent, it shall give [***] ([***]) months' prior written notice to UCLB and on the expiry of such notice period the licences of the relevant Patents granted to the Licensee under this Agreement shall cease.

8.1.5 In the event that any of rights granted hereunder become non-exclusive, responsibility for the drafting, filing, prosecution and maintenance of all of the Patents shall revert to UCLB.

8.2 Infringement of the Patents, the Know-how and/or the Materials

8.2.1 The Licensee and UCLB shall promptly give to each other (and MEEI) written notice if it becomes aware of any infringement or potential infringement of any of the Patents or any unauthorised use of the Know-how or the Materials or any challenge to the validity or ownership of the Patents, the Know-how or the Materials and the Licensee and UCLB shall consult with each other (and MEEI) to decide the best way to respond to such infringement, unauthorised use or challenge.

8.2.2 The Licensee shall have the primary obligation and right to take action against any third party alleged to be infringing the Patents or making unauthorised use of the Know-how or the Materials and to defend the Patents against challenges to validity or ownership at its sole expense, provided that:

(a) the Licensee, UCLB and MEEI, in cooperation with the NIH, shall use their commercially reasonable efforts to eliminate the infringement without litigation. If the efforts of the Licensee, UCLB and MEEI are not successful in eliminating the infringement within [***] ([***]) days after the infringer has been formally notified of the infringement by the Licensee, the Licensee shall have the right after consulting with MEEI, NIH and UCLB, to commence suit on its own account;

(b) UCLB shall procure that MEEI shall and that MEEI shall procure that the NIH shall on the Licensee's or UCLB's request cooperate with the Licensee in such action [***];

(c) UCLB shall on the Licensee's request cooperate with the Licensee in such action and [***];

(d) the Licensee shall be solely responsible for the conduct of the action or for settlement thereof and shall be entitled to all damages received from such action, subject to Clause 8.2.4; and

(e) if the Licensee is unsuccessful in persuading the alleged infringer to desist within [***] ([***]) months of the Licensee first becoming aware of any potential infringement of the Patents for any Licensed Technology or fails without a commercially reasonable basis (relative to the Licensed Technology or other technology licenced to Licensee by UCLB) to initiate an infringement action within [***] ([***]) months of becoming aware of such infringement, UCLB shall have the right, at its sole discretion, to prosecute such infringement under its sole control and [***]**.**

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

8.2.3 Before starting or defending or settling any legal action under Clause 8.2.2, the Licensee shall consult with UCLB as to the advisability of the action or defence or settlement, its effect on the good name of UCLB, the public interest, and how the action or defence should be conducted.

8.2.4 The Licensee shall [***] in such action or defence.

8.2.5 UCLB shall if reasonably requested by the Licensee agree to be joined in any suit to enforce such rights or will take such action in its own name [***] and shall have the right to be separately represented by its own counsel [***]. Notwithstanding the foregoing, [***].

8.2.6 UCLB shall procure that MEEI shall and that MEEI shall procure that the NIH shall if reasonably requested by UCLB or the Licensee agree to be joined in any suit to enforce such rights or will take such action in its or their own name(s) [***]. Notwithstanding the foregoing, [***].

8.2.7 If within [***] ([***]) months of the Licensee or UCLB giving to the other and MEEI written notice or receiving written notice from the other or MEEI of any potential infringement of the Patents, the Licensee and UCLB are both unsuccessful in persuading the alleged infringer to desist or fail to initiate an infringement action, MEEI shall have the right, at its sole discretion, to prosecute such infringement under its sole control and [***].

8.3 Infringement of Third Party Rights

8.3.1 If any warning letter or other notice of infringement is received by the Licensee or UCLB, or legal suit or other action is brought against the Licensee or UCLB, alleging infringement of third party rights in the manufacture, use or sale of any Licensed Product or use of any Patents, Know-how or Materials, that Party shall (in the case of UCLB) promptly provide full details to the Licensee and (in the case of the Licensee) promptly provide full details to UCLB, and the Licensee and UCLB shall discuss the best way to respond with MEEI.

8.3.2 The Licensee shall have the right but not the obligation to defend such suit to the extent it relates to Licensee's or its Affiliates' or Sub-licensee's activities and shall have the right to settle with such third party, provided that [***]. In the event that the Licensee, Affiliates or Sub-licensees do not take forward an action, UCLB shall have the right, at its sole discretion, to defend such suit under its sole control and [***].

9. WARRANTIES AND LIABILITY

9.1 **Warranties by UCLB**

UCLB warrants as of the Original Commencement Date and undertakes as follows to its reasonable knowledge and without having undertaken any due and careful enquires whether specific or general in nature:

9.1.1 It, MEEI and NIH are the owners of the Patents;

9.1.2 it has the authority to grant the licences under this Agreement; and.

9.1.3 so far as it is aware (having made no enquiry of any third parties or conducted any freedom to operate searches), use and exploitation of the Patents will not infringe the intellectual property rights of any third party.

UCLB warrants and undertakes:

9.1.4 it has full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on UCLB; and

9.2 **Warranties by the Licensee**

The Licensee warrants as of the Original Commencement Date and undertakes that in respect of the Licensed Technology that:

9.2.1 full power and authority to enter into and perform this Agreement, which, when executed, will constitute valid and legally binding obligations on the Licensee;

9.2.2 entry into this Agreement will not result in any breach of, or violation of the terms or provisions of, the constitutional documents of the Licensee or any other agreement or instrument by which it is bound;

9.2.3 so far as it is aware (having made no enquiry of any third parties), use and exploitation of the Patents will not infringe the intellectual property rights of any third party;

9.2.4 neither it nor any of its Affiliates is currently researching, developing, marketing, selling or otherwise commercialising any Competing Product ("**Competing Activities**"), nor has any of them entered into an agreement with any other person with respect to any Competing Activities; and

9.2.5 it shall notify UCLB if it or any of its Affiliates or its Sub-licensees commences any Competing Activities or enters into an agreement with any other person with respect to any Competing Activities.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

9.3 **Warranties by Meira**

Licensee warrants as of the Original Commencement Date and undertakes that full power and authority to enter into and perform this Agreement which, when executed, will constitute valid and legally binding obligations on Licensee.

9.4 **Acknowledgements**

The Licensee acknowledges that:

9.4.1 the inventions claimed in the Patents, and the Know-how and the Materials are at an early stage of development. Accordingly, specific results cannot be guaranteed and any results, materials, information or other items (together "**Delivered Items**") provided under this Agreement are provided "as is" and without any express or implied warranties, representations or undertakings. As examples, but without limiting the foregoing, UCLB does not give any warranty that Delivered Items are of merchantable or satisfactory quality, are fit for any particular purpose, comply with any sample or description, or are viable, uncontaminated, safe or non-toxic.

9.4.2 UCLB has not performed any searches or investigations into the existence of any third party rights that may affect any of the Patents, Know-how or Materials or the use and exploitation of any of the Patents, Know-how or Materials.

9.5 **No Other Warranties**

9.5.1 Each of the Parties acknowledges that, in entering into this Agreement, it does not do so in reliance on any representation, warranty or other provision except as expressly provided in this Agreement, and any conditions, warranties or other terms implied by statute or common law are excluded from this Agreement to the fullest extent permitted by law.

9.5.2 Without limiting the scope of Clause 9.5.1, UCLB does not make any representation nor give any warranty or undertaking:

(a) express or implied, including, without limitation, any implied warranties of merchantability or of fitness for a particular purpose with respect to any Patent, trademark, software, non-public or other information, or tangible research property, licensed or otherwise provided to the Licensee hereunder and hereby disclaims the same;

(b) as to the efficacy or usefulness of the Patents, Know-how or Materials; or

(c) whatsoever with regard to the scope of any of the Patents or that any of the Patents is or will be valid or (in the case of an application) will proceed to grantor that such Patents may be exploited by the Licensee, Affiliate or Sub-licensee without infringing other patents; or

(d) that the Materials or the method used in making or using the Materials are free from liability for patent infringement; or

(e) that the use of any of the Patents, Know-how or Materials, Licensed Technology, the manufacture, sale or use of the Licensed Products, or the exercise of any of the rights granted under this Agreement will not infringe any intellectual property or other rights of any other person; or

(f) that the Know-how or any other information communicated by UCLB to the Licensee under or in connection with this Agreement will produce Licensed Products of satisfactory quality or fit for the purpose for which the Licensee intended or that any product will not have any defect, latent or otherwise, and whether or not discoverable by inspection; or

(g) as imposing any obligation on UCLB to bring or prosecute actions or proceedings against third parties for infringement or to defend any action or proceedings for revocation of any of the Patents; or

(h) as imposing any liability on UCLB in the event that any third party supplies Licensed Products to customers located in the Territory; or

(i) that there will be no similar or competitive products or services manufactured, used, sold or supplied by any third party in the Territory.

9.6 Responsibility for Development of Licensed Products

The Licensee shall be exclusively responsible for its and its Affiliates' and Sub-licensees' use of the Patents, Know-how and Materials, the technical and commercial development and manufacture of Licensed Products and for incorporating any modifications or developments thereto that may be necessary or desirable, for all Licensed Products sold or supplied, notwithstanding any consultancy services or other contributions that UCLB and/or UCL may provide in connection with such activities.

9.7 **Indemnity**

The Licensee shall indemnify each of UCLB and UCL, and each of their respective officers, directors, Council members, employees and representatives (together, the "**Indemnitees**") against all third party Claims that may be asserted against or suffered by any of the Indemnitees and which relate to:

9.7.1 the use by the Licensee or any of its Affiliates or Sub-licensees of any of the Patents, Know-how or Materials; or

9.7.2 use of the Licensed Technology by or on behalf of the Licensee or any of its Affiliates or Sub-licensees; or

9.7.3 the development, manufacture, use, marketing or sale of, or any other dealing in, any of the Licensed Products, by or on behalf of the Licensee or any of its Affiliates or Sub-licensees, or subsequently by any customer or any other person, including claims based on product liability laws.

The indemnity given by the Licensee to each Indemnitee under this Clause 9.7 will not apply to any third party Claim to the extent that it is attributable to the negligence, gross negligence, reckless misconduct or intentional misconduct of any Indemnitee.

Licensee shall indemnify, defend and hold harmless MEEI and its trustees, officers, medical and professional staff, employees and agents and their respective successors, heirs and assigns (the "**MEEI lndemnitees**"), against any liability, damage, loss or expense (including reasonable attorney's fees and expenses of litigation) incurred by or imposed upon the MEEI lndemnitees or any one of them in connection with any third party claims, suits, actions, demands or judgments: arising out of any theory of product liability (including, but not limited to, actions in the form of contract, tort, warranty, or strict liability) concerning any product, process or service made, used or sold or any right or license granted under this Agreement.

Licensee's indemnification of the MEEI Indemnitees under this Clause 9.7 shall not apply to liability, damage, loss or expense to the extent that it is directly attributable to the negligent activities, reckless misconduct or intentional misconduct of the MEEI lndemnitees.

Licensee agrees, at its own expense, to provide attorneys reasonably acceptable to MEEI to defend against any actions brought or filed against any party indemnified hereunder with respect to the subject of indemnity contained herein, whether or not such actions are rightfully brought.

Licensee's indemnification of the MEEI Indemnitees under this Clause 9.7 shall survive expiration or termination of this Agreement.

9.8 **Limitations of Liability**

9.8.1 To the extent that UCLB or any of its Affiliates has any liability in contract, tort, or otherwise under or in connection with this Agreement, including any liability for breach of warranty, their liability shall be limited in accordance with the following provisions of this Clause 9.8.

9.8.2 The aggregate liability of UCLB and any of its Affiliates shall be limited to the total income that UCLB has received from the Licensee pursuant to this Agreement (but excluding any other costs or expenses associated with drafting, filing, prosecuting, maintaining or defending any Patents or providing any assistance to the Licensee) during the period of [***] ([***]) years preceding the date on which the liability arises, or fifty thousand pounds (£50,000) sterling, whichever is the higher.

9.8.3 The liability of the Licensee to UCLB shall be limited to the limit of its insurance as set out in Clause 9.9.1, except that in the case of product liability, the liability of the Licensee under this Agreement shall be unlimited.

9.8.4 In no circumstances shall any Party or any Indemnitee be liable for any loss, damage, costs or expenses of any nature that is (a) of an indirect, special or consequential nature or (b) any loss of profits (whether direct or indirect), revenue, business opportunity or goodwill, which arises directly or indirectly from that Party's breach or nonperformance of this Agreement, or negligence in the performance of this Agreement or from any liability arising in any other way out of the subject matter of this Agreement even if the Party bringing the claim has advised any other Party or the relevant Indemnitee of the possibility of those losses arising, or if such losses were within the contemplation of the Parties or the Indemnitee.

9.8.5 Nothing in this Agreement excludes any Party's liability to the extent that it may not be so excluded under applicable law, including any such liability for death or personal injury caused by that Party's negligence, or liability for fraud or fraudulent misrepresentation.

9.9 **Insurance**

9.9.1 The Licensee shall take out with a reputable insurance company and maintain at all times during the term of this Agreement public and product liability and professional indemnity insurance including against all loss of and damage to property (whether real, personal or intellectual) and injury to persons including death arising out of or in connection with this Agreement and the Licensee's and its Affiliates' and Sub-licensees' use of the Patents, Know-how or Materials and use, sale of or any other

dealing in any of the Licensed Products. Such insurances may be limited in respect of one claim provided that such limit must be at least [***] pounds (£[***]) sterling, unless the Licensee commences any business in manufacturing, distribution, supply or otherwise make available to the public any products, in which case such limit must be at least [***] pounds (£[***]) sterling. Such insurance shall continue to be maintained for a further [***] years from the end of this Agreement.

9.9.2 The Licensee will produce to UCLB at all times upon demand proof that the insurance cover required pursuant to Clause 9.9.1 is in force and evidence that all premiums have been paid up to date. If UCLB becomes aware that the Licensee has failed to maintain the insurance required pursuant to Clause 9.9.1, UCLB may effect such insurance and the Licensee will reimburse UCLB for the reasonable cost of effecting and maintaining such insurance on demand.

9.9.3 Beginning no later than the time any Licensed Products are being commercially distributed or sold (other than for the purpose of obtaining regulatory approvals) by Licensee or by a Sub-licensee, affiliate or agent of Licensee, Licensee shall, at its own cost and expense procure and maintain Commercial General Liability (CGL) insurance or other coverage acceptable to MEEI in amounts not less than [***] and naming the MEEI lndemnitees as additional insureds. Such CGL or other insurance shall provide:

(a) Product liability coverage, and

(b) Contractual liability coverage for Licensee's indemnification under Clause 9.7 of this Agreement.

9.9.4 If Licensee elects to self-insure all or parts of the limits described above (including deductibles or retentions which are in excess of $[***] annual aggregate) such self-insurance program must be acceptable to MEEI and CRICO. The minimum amount of insurance coverage required under this Clause 9.9.4 shall not be construed to create a limit of Licensee's liability with respect to its indemnification under Clause 10.7 of this Agreement. Licensee shall provide MEEI with written evidence of such insurance upon request of MEEI. Licensee shall provide MEEI with written notice at least [***] ([***]) days prior to the cancellation, non-renewal or material change in such insurance, if

(a) Licensee does not obtain replacement insurance providing comparable coverage within such [***] ([***]) day period, MEEI shall have the right to terminate this Agreement effective at the end of such [***] ([***]) days without notice of any additional waiting period.

9.9.5 Licensee shall maintain such CGL or other insurance during:

(a) the period that any Licensed Products are being commercially distributed or sold (other than for the purpose of obtaining regulatory approvals) by Licensee or by a Sub-licensee, affiliate or agent of Licensee;

(b) reasonable period after the period referred to in Clause 9.9.5(a) above, which in no event shall be less than [***] ([***]) years.

9.9.6 This Clause 9.9 shall survive expiration or termination of this Agreement.

10. DURATION AND TERMINATION

10.1 Commencement and Expiry

This Agreement shall commence as of the Commencement Date and, unless terminated earlier in accordance with this Clause 10, the licences granted hereunder shall continue in force on a country by country basis until the later of the last payment obligation of Licensee expires under this Agreement. Upon such expiry, Licensee's licenses under this Agreement shall become full-paid, perpetual and irrevocable.

10.2 Termination of the Original Agreement

Upon the Commencement Date, pursuant to Amendment No. 4 to the Original Agreement, the Original Agreement shall be deemed to have terminated.

10.3 Early Termination

Each Party (the "**Terminating Party**") may terminate this Agreement at any time by notice in writing to the other Parties ("**Other Parties**"), such notice to take effect as specified in the notice:

10.3.1 If, in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, is in material breach of this Agreement and, in the case of a breach capable of remedy within thirty (30) days, the breach is not remedied within thirty (30) days of the Other Parties receiving notice specifying the breach and requiring its remedy or where the breach relates to non-payment of an undisputed sum due under this Agreement, the sum is not paid in full within fourteen (14) days following the Other Party receiving notice specifying the nonpayment and requiring payment in full; provided however, that in respect on breaches not relating to non-payment, if such breach is capable of being cured but cannot be cured within such thirty (30) day period and the Other Party initiates actions to cure such breach within such period and

thereafter diligently pursues such actions, the Other Party shall have such additional period as is reasonable under the circumstances to cure such breach; it being understood that no such extension shall apply with respect to any undisputed payment obligations or extend beyond six (6) months from the end of such thirty (30) day period. In the event there is a genuine dispute between the Parties with respect to any alleged breach hereunder, no purported termination of this Agreement pursuant to this Clause 10.3.1 shall take effect while the Parties are actively working to resolve such dispute; or

10.3.2 if:

(a) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, becomes insolvent or unable to pay its debts as and when they become due;

(b) an order is made or a resolution is passed for the winding up of in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB (other than voluntarily for the purpose of solvent amalgamation or reconstruction);

(c) a liquidator, administrator, administrative receiver, receiver or trustee is appointed in respect of the whole or any part of, in the case of UCLB, either of the Other Parties', or in the case of the Licensee or Meira, UCLB's, assets or business;

(d) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, makes any composition with its creditors;

(e) in the case of UCLB, either of the Other Parties, or in the case of the Licensee or Meira, UCLB, ceases to continue its business; or

(f) any event analogous to the events referred to in paragraphs (a) to (e) above occurs in any other jurisdiction.

10.4 UCLB may terminate this Agreement by giving written notice to the Licensee and Meira, such termination to take effect forthwith or as otherwise stated in the notice:

10.4.1 if there is any change of Control of the Licensee involving the categories of persons or Affiliates of persons prohibited by Clause 2.3;

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.4.2 the Licensee is in persistent breach of the Agreement at least [***] ([***]) times in a calendar year, wherein the nature of each such breach is the same in each instance, and where the Parties have failed to agree on a mechanism to remedy the persistent nature of such breaches within a reasonable period following UCLB notifying Licensee of the persistent breaches and requesting that the Licensee enters into discussions with UCLB as to mechanisms for remedying the persistent breaches or if the Parties have agreed to a mechanism to remedy the persistence of such breach by that mechanism if not fully complied with by the Licensee; or

10.4.3 if the Licensee shall enter into any sub-licence with any of the categories of persons or Affiliates of persons prohibited by Clause 2.3 which may, adversely affect UCL's and/or UCLB's reputation;

10.4.4 subject to Clause 5.5, if the Licensee fails to achieve any of the milestone events described in Schedule 4 provided that if achievement of any of the milestone events should be compromised due to technical, legal or regulatory issues, the Parties shall first meet and UCLB will work with the Licensee to manage the delivery schedule and provided that the Licensee is using Diligent Efforts to correct the issues, the applicable deadline in Schedule 4 shall be extended by six (6) months or such other time period as shall be agreed between the Parties in writing after which if the Licensee has not achieved the milestone UCLB shall be entitled, subject to Clauses 5.11 and 5.12, to terminate this Agreement by giving written notice to the Licensee.

10.5 A Party's right of termination under this Agreement, and the exercise of any such right, shall be without prejudice to any other right or remedy (including any right to claim damages) that such Party may have in the event of a breach of contract or other default by any other Party.

10.6 Consequences of Termination

10.6.1 Upon expiry of the period of this Agreement, and subject to all royalties and any other sums due to UCLB under this Agreement having been duly paid, the Licensee shall have a fully paid up licence to the Patents, the Know-how and the Materials of the same scope as set forth in Clause 2.1 without any further obligation to pay any further sums to UCLB under Clause 4. Notwithstanding the foregoing the Licensee acknowledges that once each Patent expires or is abandoned or withdrawn or allowed to lapse in any country or territory, third parties in that country or territory will be entitled to use the inventions claimed in the Patent and that accordingly the licence granted to the Licensee under Clause 2.1 will no longer be exclusive in that country or territory.

10.6.2 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4:

(a) the Licensee and its Affiliates and Sub-licensees shall be entitled to sell, use or otherwise dispose of (subject to payment of royalties under Clause 4) any unsold or unused stocks of the Licensed Products for a period of [***] ([***]) months following the date of termination;

(b) subject to paragraph (a) above, any license that has not become fully paid-up in accordance with Clause 10.1 shall terminate and the Licensee and its Affiliates (and subject to Clause 2.3, its Sub-licensees) shall no longer be licensed to use or otherwise exploit the Patents and/or the Know-how and/or the Materials, in so far and for as long as any of the Patents remains in force and the Know-how remains confidential;

(c) the Licensee shall consent to the cancellation of any formal licence granted to it, or of any registration of it in any register, in relation to any of the Patents;

(d) the Licensee will, promptly on UCLB's request, provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which are in the possession of Licensee or its patent agents relating to the Patents and which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents;

(e) except as set out in Clause 2.3, all sub-licences of the Patents and/or the Know-how and/or the Materials granted by the Licensee pursuant to this Agreement will automatically terminate;

(f) UCLB shall, upon the written request of either of the other Parties, and each of the Licensee and Meira shall, upon the written request of UCLB, return or destroy any documents or other materials that are in its or its Affiliates possession or under its or their control and that contain the requesting Party's Confidential Information.

10.6.3 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, in the event that UCLB would be unable, absent a licence from the Licensee, to use or permit others to use or to exploit or permit others to exploit the relevant Licensed Technology without infringing intellectual property rights in any invention developed by the Licensee, whether solely or jointly with others ("Blocking Invention"), the Licensee shall be deemed to have granted UCLB the irrevocable non-exclusive right to use, exploit and permit others to use and exploit the Blocking Invention only in conjunction with the relevant Licensed

Technology. The Licensee shall at the request of UCLB provide (and will ensure that its patent agents provide) to UCLB all information, documentation and assistance (including executing documents) which UCLB may reasonably require to enable it to continue with the drafting, filing, prosecution and maintenance of the Patents licensed under this Agreement;

10.7 Upon termination of this Agreement by UCLB under Clause 10.3.1 (for Licensee's uncured material breach) or under Clause 10.4, the Licensee shall, to the extent it is able to do so without being in breach of any obligation owed to a third party, disclose to UCLB full details of any and all Intellectual Property generated at any time by or on behalf of the Licensee as a result of the exercise of the Licensee's rights under this Agreement ("Licensee IP") and, upon UCLB's written request within [***] ([***]) days following such disclosure, negotiate in good faith to agree the terms of an exclusive or non-exclusive licence to UCLB (as UCLB may request) under the Licensee IP. If the Parties fail to agree the terms of such a licence within [***] days following commencement of such negotiation, despite negotiating in good faith, UCLB's rights under this Clause shall lapse. If Licensee may terminate this Agreement under Clause 10.3.1 (for UCLB or its Affiliates uncured material breach), then Licensee may elect, in lieu of terminating the entire Agreement, to have all licenses granted to the Licensee under this Agreement continue in force, subject to Licensee's fulfilment of [***] percent ([***]%) of its payment obligations under Clause 4 after what would have been the effective date of such termination.

10.8 Upon termination of this Agreement for any reason, the provisions of Clauses 1, 2.3, 2.5, 3.1 to 3.5, 4 (in respect of amounts paid and payable to UCLB in respect of the period up to and including the date of termination), 5.7, 7, 9, 10.7, 10.8, 10.8 and 11 of this Agreement shall remain in force**.**

11. GENERAL

11.1 Force Majeure

11.1.1 Any delays in or failure of performance by a Party under this Agreement will not be considered a breach of this Agreement and if and to the extent that such delay or failure is caused by occurrences beyond the reasonable control of that Party including acts of God; acts, regulations and laws of any government; strikes or other concerted acts of workers; fire; floods; explosions; riots; wars; rebellion; and sabotage; and any time for performance hereunder will be extended by the actual time of delay caused by any such occurrence.

11.1.2 If (a) UCLB or (b) the Licensee or Meira is prevented from carrying out its obligations:

(a) under this Agreement for a continuous period of [***] ([***]) months, the Licensee (in the case of (a)) or UCLB (in the case of (b)), may terminate this Agreement on giving [***] ([***]) days prior written notice provided always that at the date upon which termination becomes effective the Party which was prevented from carrying out its obligations under this Agreement remains so prevented.

11.2 Amendment

This Agreement may only be amended in writing signed by duly authorised representatives of the Parties.

11.3 **Assignment and Third Party Rights**

11.3.1 Subject to Clause 11.3.3, the Licensee shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement, nor any of the Patents, Know-how or Materials, without the prior written consent of UCLB.

11.3.2 UCLB may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party.

11.3.3 The Licensee, subject to obtaining the consent of UCLB which shall not be unreasonably withheld or delayed (except in relation to those categories of persons or Affiliates of persons prohibited by Clause 2.3), may assign all its rights and obligations under this Agreement together with its rights in the Patents, Know-how and Materials to any third party to which it transfers all or substantially all of its assets or business, provided that the assignee undertakes to UCLB to be bound by and perform the obligations of the assignor under this Agreement. However, the Licensee shall not have such a right to assign this Agreement if it is insolvent.

11.3.4 Meira shall not assign, mortgage, charge or otherwise transfer any rights or obligations under this Agreement without the prior written consent of UCLB.

11.4 Waiver

Any waiver given under or in relation to this Agreement shall be in writing and signed by or on behalf of the relevant Party. No failure or delay on the part of a Party to exercise any right or remedy under this Agreement shall be construed or operate as a waiver thereof, nor shall any single or partial exercise of any right or remedy preclude the further exercise of such right or remedy.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.5 Invalid Clauses

If any provision or part of this Agreement is held to be invalid, amendments to this Agreement may be made by the addition or deletion of wording as appropriate to remove the invalid part or provision but otherwise retain the provision and the other provisions of this Agreement to the maximum extent permissible under applicable law.

11.6 No Agency

No Party shall act or describe itself as the agent of the other, nor shall it make or represent that it has authority to make any commitments on the other's behalf.

11.7 Interpretation

In this Agreement:

11.7.1 the headings are used for convenience only and shall not affect its interpretation; references to persons shall include incorporated and unincorporated persons; references to the singular include the plural and vice versa; and references to the masculine include the feminine;

11.7.2 references to Clauses and Schedules mean clauses of, and schedules to, this Agreement;

11.7.3 references in this Agreement to termination shall include termination by expiry;

11.7.4 where the word "including" is used it shall be understood as meaning "including without limitation";

11.7.5 any reference to any English law term for any action, remedy, method or judicial proceeding, legal document, legal status, court, official or any legal concept or thing shall in respect of any jurisdiction other than England be deemed to include what most nearly approximates in that jurisdiction to the English law term;

11.7.6 where there is any conflict or inconsistency between the main body of this Agreement and any of the schedules, then the main body of the Agreement shall prevail;

11.7.7 time shall be of the essence in relation to the performance of Meira's and the Licensee's obligations under this Agreement; and

11.7.8 any reference to the sale of a Licensed Product by the Licensee or its Affiliates or Sub-licensees will be taken to include any supply or other disposal of Licensed Products, and the term sold shall be construed accordingly.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

11.8 **Notices. Addresses for Service**

11.8.1 Any notice to be given under this Agreement shall be in English, in writing and shall be delivered by first class recorded delivery mail (if sent to an inland address) or by international courier (if sent to an address outside of the United Kingdom), to the address of the relevant Party set out at the head of this Agreement, or such other address as that Party may from time to time notify to the other Parties in accordance with this Clause 11.8.

11.8.2 Notices sent as above shall be deemed to have been received [***] ([***]) working day after the day of posting in the case of delivery inland first class recorded delivery mail, or [***] ([***]) working days after the date of collection by the international courier.

11.9 Law and Jurisdiction

The validity, construction and performance of this Agreement, and any contractual and non-contractual claims arising hereunder, shall be governed by English law and shall be subject to the exclusive jurisdiction of the English courts to which the Parties hereby submit, except that a Party may seek an interim injunction (or an equivalent remedy) in any court of competent jurisdiction.

11.10 Entire Agreement

This Agreement, including its Schedules, sets out the entire agreement between the Parties relating to its subject matter and supersedes all prior oral or written agreements, arrangements or understandings between them relating to such subject matter including, without limitation, the parts of the Original Agreement that pertains to the Specified Technology [***]. Subject to Clause 9.8.5, the Parties acknowledge that they are not relying on any representation, agreement, term or condition which is not set out in this Agreement.

11.11 Third Parties

Except for the rights of UCL, MEEI and NIH as provided in Clause 2.4, the rights of the Indemnitees and MEEI Indemnitees as provided in Clause 9.7, the limitations of liability afforded to the Indemnitees pursuant to Clause 9.8, the rights of MEEI under Clause 9.9 and the rights of MEEI and NIH under Clause 8, who may in their own right enforce and rely on the provisions of those Clauses, this Agreement does not create any right enforceable by any person who is not a party to it ("**Third Party**") under the Contracts (Rights of Third Parties) Act 1999, but this Clause 11.11 does not affect any right or remedy of a Third Party which exists or is available apart from that Act. The Parties may amend, renew, terminate or otherwise vary all or any of the provisions of this Agreement, including Clauses 2.4, 8, 9.7, and 9.8, without the consent of MEEI, NIH and/or the MEEI lndemnitees

11.12 **Non-use of Names; Announcements**

11.12.1 The Licensee shall not use, and shall ensure that its Affiliates and Sub-licensees do not use, the name, any adaptation of the name, any logo, trademark or other device of UCLB, nor of the inventors named on the Patents nor the Principal Investigators in any advertising, promotional or sales materials without prior written consent obtained from UCLB in each case, except that the Licensee may state that it is licensed by UCLB under the Patents.

11.12.2 Except as permitted under Clauses 3.3.1 and 5.7, no Party shall make any press or other public announcement concerning any aspect of this Agreement, or make any use of the name or trademarks of any other Party in connection with or in consequence of this Agreement, without the prior written consent of the relevant other Party.

11.13 Escalation

If the Licensee or Meira on the one hand, and UCLB on the other, are unable to reach agreement on any issue concerning this Agreement or the Project within [***] days after one either has notified the other of that issue, they will refer the matter to the [***] in the case of UCLB, and to the [***] in the case of the Licensee and Meira in an attempt to resolve the issue within the time specified elsewhere in this Agreement in the case of other disputes. Any Party may bring proceedings in a court of competent jurisdiction if the matter has not been resolved within that prescribed period, and any Party may apply to the court for an injunction, whether or not any issue has been escalated under this Clause 11.13.

[Signature Page Follows]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

EXECUTED on the date set out at the head of this Agreement.

For and on behalf of

UCL Business PLC

/s/ [***]
Signed

[***]
Print name

[***]
Title

5 February 2019
Date

For and on behalf of

MEIRAGTX UK II LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

CEO
Title

2.5.19
Date

For and on behalf of

MEIRAGTX LIMITED

/s/ Zandy Forbes
Signed

Zandy Forbes
Print name

CEO
Title

2.5.19
Date

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 1

LICENSED TECHNOLOGY

Part A: The Patents

[***]

Part B: The Know-how

[***]

Part C: The Materials

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 2

APPOINTMENT OF EXPERT

For the purposes of this Schedule 2 only, the "Parties" shall mean the Licensee and UCLB. If either Party wishes to appoint an independent expert (the "**Expert**") to determine any matter pursuant to any Clause of this Agreement, the following procedures will apply:

1. The Party wishing to appoint the Expert (the "**Appointing Party**") will serve a written notice on the other Party (the "**Responding Party**"). The written notice will specify the Clause pursuant to which the appointment is to be made and will contain reasonable details of the matter(s) which the Appointing Party wishes to refer to the Expert for determination.

2. The Parties shall within [***] ([***]) days following the date of the Appointing Party's written notice use all reasonable efforts to agree who is to be appointed as the Expert to determine the relevant matter(s). If the Parties are unable to agree upon the identity of the Expert within that timescale, the Expert shall be appointed by the President (for the time being) of the Licensing Executives Society Britain and Ireland upon written request of either Party.

3. Each Party will within [***] ([***]) days following appointment of the Expert, prepare and submit to the Expert and the other Party a detailed written statement setting out its position on the matter(s) in question and including any proposals which it may wish to make for settlement or resolution of the relevant matter.

4. Each Party will have [***] ([***]) days following receipt of the other Party's written statement to respond in writing thereto. Any such response will be submitted to the other Party and the Expert.

5. The Expert will if he/ she deems appropriate be entitled to seek clarification from the Parties as to any of the statements or proposals made by either Party in their written statement or responses. Each Party will on request make available all information in its possession and shall give such assistance to the Expert as may be reasonably necessary to permit the Expert to make his/ her determination.

6. The Expert will issue his/ her decision on the matter(s) referred to him/ her in writing as soon as reasonably possible, but at latest within [***] ([***]) months following the date of his/ her appointment. The Expert's decision shall (except in the case of manifest error) be final and binding on the Parties.

7. The Expert will at all times act as an independent and impartial expert and not as an arbitrator.

8. The Expert's charges will be borne as he/ she determines in his written decision.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 3

DEFINITION OF TOBACCO INDUSTRY FUNDING (REVISED 2009)

FROM THE CANCER RESEARCH UK CODE OF PRACTICE ON TOBACCO INDUSTRY FUNDING TO UNIVERSITIES.

http://www.cancerresearchuk.org/science/funding/terms-conditions/funding-policies/policy-tobacco/

A tobacco company is defined for the purposes of this policy as one that:

- Derives over 5% of revenues from manufacturing tobacco products;
- Derives 15%+ of revenues from the manufacture of products necessary for the production of tobacco products;
- Derives 15% of revenues from the sale of tobacco products (and has 30 or more staff);
- Owns a tobacco company (the company owns 50% or more of a tobacco company);
- Is more than 50% owned by a company with tobacco involvement.

The following do not constitute tobacco industry funding for the purposes of this Code:

- legacies from tobacco industry investments (provided these are sold on immediately)
- funding from a trust or foundation no longer having any connection with the tobacco industry even though it may bear a name that (for historical reasons) has tobacco industry associations.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

SCHEDULE 4

DEVELOPMENT PLAN

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

AMENDMENT NO. 4 TO EXCLUSIVE LICENSE AGREEMENT

This AMENDMENT NO. 4 TO EXCLUSIVE LICENSE AGREEMENT (this "Amendment"), effective as of January 29, 2019 ("Amendment Effective Date"), is entered into by and between MeiraGTx Limited (registered number 9501998), having a place of business located at 92 Britannia Walk, London, United Kingdom, N1 7NQ United Kingdom ("MeiraGTx") and UCL Business PLC (registered number 02776963), whose registered office is The Network Building, 97 Tottenham Court Road, London W1T 4TP United Kingdom ("UCLB"). MeiraGTx and UCLB are each sometimes referred to herein as a "Party" and collectively referred to herein as the "Parties".

WITNESSETH:

WHEREAS, UCLB and Athena Vison Ltd., a company incorporated under the laws of England and Wales under company registration number 09348737 ("Athena") entered into a License Agreement dated 4 February 2015, as amended by Amendment No. 1 to Exclusive License Agreement, effective as of 27 March 2015, Amendment No. 2, effective as of July 28, 2017, and Amendment No.3, effective as of December 14, 2017 (as amended, the "Licence Agreement"), pursuant to which UCLB licensed to Athena certain technology and intellectual property relating to ocular gene therapy, which UCLB acquired by assignment from University College London, for development and commercialization by Athena;

WHEREAS, UCLB, Athena, and MeiraGTx entered into a Deed of Novation and Amendment in 2016 ("Deed of Novation and Amendment") pursuant to which the parties thereto agreed to, among other things, novate Athena's rights, obligations, and liabilities under the Licence Agreement to MeiraGTx on the terms and conditions of the Deed of Novation and Amendment;

WHEREAS, UCLB and MeiraGTx UK II Limited (an affiliate of MeiraGTx) entered into a separate licence agreement, dated July 28, 2017 to consolidate UCLB's rights to an ocular gene therapy for **RPGR** ("**RPGR** Licence Agreement");

WHEREAS, UCLB, MeiraGTx and MeiraGTx UK II Limited (an affiliate of MeiraGTx) entered into a separate licence agreement, dated March 15, 2018 to consolidate UCLB's rights to an ocular gene therapy for **CNGA3** ("**CNGA3** Licence Agreement"); and

WHEREAS, UCLB and MeiraGTx further wish to amend the Licence Agreement in certain respects.

NOW, THEREFORE, in consideration of the foregoing statements and the mutual agreements and covenants herein contained, and for other good and valuable consideration, the sufficiency of which are hereby acknowledged, the Parties hereby agree as follows.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Section 1. **Definitions**. Except as set for the herein, capitalized terms not otherwise defined or amended in this Amendment shall have the meaning ascribed to them in the Licence Agreement, as amended by this Amendment. References to Articles, Clauses or Schedules are to the same with all their subparts as they appear in the Licence Agreement. References to Sections are to the numbered paragraphs with all their subparts as they appear in this Amendment, and references to Exhibits are to the exhibits attached to this Amendment, and incorporated herein by reference.

Section 2. **Amendment to the Licence Agreement**.

(a) **CNGB3**. Licence Addendum Number 1, dated 4 February 2015 for technology **[***]** (**CNGB3**), is hereby deleted in its entirety as of the Amendment Effective Date and the Parties have entered into the stand-alone licence agreement attached as Exhibit 1. For clarity, the Parties acknowledge and agree that such licence agreement supersedes the rights and obligations of the Parties set out in Licence Addendum Number 1 as of the Amendment Effective Date, and MeiraGTx or its affiliates will not owe UCLB any payments for any licensed products under Licence Addendum Number 1 or the related Licence Agreement.

(b) **RPE65**. Licence Addendum Number 5, dated December 2017 for technology **[***]** (**RPE65**), is hereby deleted in its entirety as of the Amendment Effective Date, and Parties have entered into the stand-alone licence agreement attached as Exhibit 2. For clarity, the Parties acknowledge and agree that such licence agreement supersedes the rights and obligations of the Parties set out in Licence Addendum Number 5 as of the Amendment Effective Date, and MeiraGTx or its affiliates will not owe UCLB any payments for any licensed products under Licence Addendum Number 5 or the related Licence Agreement.

(c) **RPGR**. The **RPGR** Licence Agreement shall terminate in its entirety as of the date on which UCLB notifies MeiraGTx in writing that it has obtained approval from MEEI and MRC for the new stand alone licence agreement attached as Exhibit 3, and Parties shall enter into the stand-alone licence agreement attached as Exhibit 3. For clarity, the Parties acknowledge and agree that such licence agreement (once it becomes effective) shall supersede the rights and obligations of the Parties set out in Licence Addendum Number 3, for technology **[***]** (**RPGR**) as of July 28, 2017, and that Licence Addendum Number 3 shall be deleted from the Licence Agreement from such date, and MeiraGTx or its affiliates will not owe UCLB any payments for any licensed products under Licence Addendum Number 3, the related Licence Agreement or the **RPGR** Licence Agreement.

(d) **CNGA3**. The **CNGA3** Licence Agreement is hereby terminated in its entirety as of the Amendment Effective Date, and Parties have entered into the stand-alone licence agreement attached as Exhibit 4. For clarity, the Parties acknowledge and agree that such licence agreement supersedes the rights and obligations of the Parties set out in Licence Addendum Number 2, dated 4 February 2015 for technology **[***]** (**CNGA3**) as of March 15, 2018, and that Licence Addendum Number 2 has been deleted from the Licence Agreement, and MeiraGTx or its affiliates will not owe UCLB any payments for any licensed products under Licence Addendum Number 2, the related Licence Agreement or the **RPGR** Licence Agreement.

(e) **Option Rights**. All other provisions of the Licence Agreement (including without limitation the options set out in Clause 2.6 of the Licence Agreement which have not been exercised by MeiraGTx prior to the date of termination, i.e., the options in relation to Specified Technologies **[***]**) together with any accrued rights of the Parties under the Licence Agreement prior to the Commencement Date shall continue in full force and effect.

Section 3. **Other**.

(a) **Effect of Amendment**. From and after the Amendment Effective Date, all references to the Licence Agreement shall mean the Licence Agreement as amended by this Amendment.

(b) **Counterparts**. This Amendment may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Signature to his Amendment transmitted by fax, by email in "portable document format" (".pdf") or by any other electronic means intended to preserve the original graphic and pictorial appearance of this Amendment shall have the same effect as physical delivery of the paper document bearing original signature.

(c) **Entire Amendment**. This Amendment contains the entire understanding of the Parties with respect to the subject matter of this Amendment. Except as specifically modified and amended hereby, all of the terms, provisions, requirements and specifications contained in the Licence Agreement remain in full force and effect. Except as otherwise expressly provided herein, the Parties do not intend to, and the execution of this Amendment shall not, in any manner, impair the Agreement, the purpose of this Amendment being simply to amend certain specific provision of the Agreement only and to confirm and carry forward the Licence Agreement, as hereby amended, in full force and effect. This Amendment may be amended, or any term hereof modified, only by a written instrument executed by both Parties.

(d) **Notices**. Any notices, requests and other communications hereunder shall be in writing and shall be personally delivered or sent by fax transmission (and promptly confirmed by personal delivery, registered or certified mail or overnight courier) or by registered or certified mail, return receipt required, postage prepaid, or sent by internally-recognized overnight courier, in each case to the respective address specified below, or such other address as may be specified in writing to the other Party hereto:

If to MeiraGTx to:
MeiraGTx Limited
92 Britannia Walk, London

United Kingdom, N1 7NQ United Kingdom
Fax No.: **[***]**
Attention: Chief Operating Officer

If to UCLB to:
UCL Business PLC
The Network Building
97 Tottenham Court Road
London W1T 4TP United Kingdom
Fax No.: **[***]**
Attention: Director of Legal Affairs

(e) **Law and Jurisdiction**. The validity, construction and performance of this Amendment, and any contractual and non-contractual claims arising hereunder, shall be governed by English law and shall be subject to the exclusive jurisdiction of the English courts to which the Parties hereby submit, except that a Party may seek an interim injunction (or an equivalent remedy) in any court of competent jurisdiction.

[*Signature Page Follows*]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

IN WITNESS WHEREOF, the Parties have caused this Amendment to be effective as of the Amendment Effective Date.

MEIRAGTX LIMITED

By [***]
Name: [***]
Title: [***]

UCL BUSINESS PLC

By /s/ Zandy Forbes
Name: Zandy Forbes
Title: President and CEO

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 1

Coverpage for New standalone Licence Agreement for CNGB3

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 2

Coverpage for New standalone Licence Agreement for RPE65

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 3

Coverpage for New standalone Licence Agreement for CNGA3

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 4

Coverpage for New standalone Licence Agreement for RPGR

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 10.25

Confidential Treatment Requested by MeiraGTx Holdings plc

EXECUTION VERSION

COLLABORATION, OPTION AND LICENSE AGREEMENT

BY AND BETWEEN

JANSSEN PHARMACEUTICALS, INC.,

MEIRAGTX UK II LIMITED

AND

MEIRAGTX HOLDINGS PLC

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

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Exhibits

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Exhibit 4.4: MeiraGTx Subcontractors

Exhibit 8.2: Key Terms of Clinical Supply Agreement and Clinical Quality Assurance Agreement

Exhibit 8.3: Key Terms of Commercial Supply Agreement and Commercial Quality Assurance Agreement

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Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

COLLABORATION, OPTION AND LICENSE AGREEMENT

This Collaboration, Option and License Agreement (this "**Agreement**") is made as of January 30, 2019 (the "**Execution Date**"), by and between Janssen Pharmaceuticals, Inc., a Pennsylvania corporation located at 1125 Trenton-Harbourton Road, Titusville, NJ 08560, United States of America ("**Janssen**"), on the one hand, and MeiraGTx UK II Limited, a company organized and existing under the laws of England, located at 25 Provost Street, London N1 7NH, United Kingdom and MeiraGTx Holdings plc, a Cayman Islands corporation located at 430 East 29th Street, 10th Floor, New York, NY 10016, United States of America (MeiraGTx UK II Limited and MeiraGTx Holdings plc, individually or collectively, "**MeiraGTx**"), on the other hand. Janssen and MeiraGTx are each referred to individually as a "**Party**" and together as the "**Parties**."

RECITALS

WHEREAS, MeiraGTx has technology and expertise in gene therapy, including viral vector design and optimization and gene therapy manufacturing, and is applying such technology to develop therapeutic products, including the Clinical IRD Products (as defined below);

WHEREAS, Janssen, together with its Affiliates, is engaged in the Research, Development, and Commercialization of biopharmaceutical products globally;

WHEREAS, MeiraGTx and Janssen desire to enter into a collaboration to jointly Develop the Clinical IRD Products to permit Janssen to Commercialize such Clinical IRD Products under an exclusive license from MeiraGTx, all under the terms and conditions set forth herein;

WHEREAS, MeiraGTx and Janssen also desire to enter into a collaboration where Janssen would co-fund MeiraGTx's research on Research IRD Products (as defined below), and in return, MeiraGTx would grant Janssen an exclusive option to obtain an exclusive license to further Develop and Commercialize certain Research IRD Products, all under the terms and conditions set forth herein; and

WHEREAS, MeiraGTx and Janssen desire to collaborate on the Development of certain manufacturing and technical improvements and provide certain additional Manufacturing services, under the terms and conditions set forth herein.

NOW, THEREFORE, in consideration of the mutual covenants and agreements herein contained, the Parties agree as follows.

1. DEFINITIONS

Unless the context otherwise requires, the capitalized terms in this Agreement have the meanings set forth below or the meaning as designated in the indicated places throughout this Agreement.

1.1. "**Accounting Standards**" means GAAP, as generally and consistently applied throughout each Party's organization.

1.2. "**Acquired Party**" has the meaning set forth in Section 4.6(d).

1.3. "**Act**" means the United States Federal Food, Drug and Cosmetic Act, 21 U.S.C. §§ 301 et seq. and the United States Public Health Service (PHS) Act, 42 U.S.C. §§ 201 et seq.

1.4. "**Advancement Criteria**" means the specific, objective criteria and standards that need to be fulfilled for a successful IND Submission in accordance with 21 C.F.R. §312 and any foreign counterpart to such regulation and any other requirements of any Regulatory Authority.

1.5. "**Adverse Event**" means (a) any untoward medical occurrence in a Clinical Study subject or in a patient who is administered a Product, whether or not considered related to such Product, including any undesirable sign (including abnormal laboratory findings of clinical concern), symptom, or disease associated with the use of a Product or (b) finding from any animal or in vitro testing or toxicology study, whether or not conducted by the sponsor, that suggests a significant risk in humans exposed to the drug.

1.6. "**Affiliate**" means, with respect to a Party, any Person that, directly or indirectly, controls, is controlled by, or is under common control with that Party, for so long as such control exists. For the purpose of this definition, "control" means any of the following: (a) direct or indirect ownership of fifty percent (50%) or more of the shares of stock entitled to vote for the election of directors, in the case of a corporation, or fifty percent (50%) or more of the equity interest in the case of any other type of legal entity, (b) status as a general partner in any partnership, or (c) any other arrangement whereby the entity or person controls or has the right to control the board of directors or equivalent governing body of a corporation or other entity, or the ability to cause the direction of the management or policies of a corporation or other entity, whether through ownership of voting securities, by contract or otherwise. In the case of entities organized under the laws of certain countries, the maximum percentage ownership permitted by law for a foreign investor may be less than fifty percent (50%), and in such case, such lower percentage shall be substituted in the preceding sentence, *provided* that such foreign investor has the power to direct the management and policies of such entity.

1.7. "**Affordable Basis**" means selling a Product for [***]. In determining Affordable Basis, the Parties recognize that, to the extent that a Party engages a Third Party in the Commercialization of a Product on an Affordable Basis, [***].

1.8. "**Agreement**" has the meaning set forth in the first paragraph of this Agreement.

1.9. "**Alliance Manager**" has the meaning set forth in Section 5.1.

1.10. "**Annual Net Sales**" means Net Sales of Product(s) in a Calendar Year.

1.11. "**Anti-Corruption Laws**" means all Applicable Laws and international financial institution rules regarding corruption, bribery, ethical business conduct, money laundering, political contributions, gifts and gratuities, or lawful expenses to public officials, healthcare professionals, and private persons, agency relationships, commissions, lobbying, books and records, and financial controls, including the FCPA.

1.12. "**Applicable Law**" means any applicable law, statute, code, ordinance, rule or regulation, enforceable guideline or other requirement, order, injunction, judgment, writ, stipulation, award, arbitration award, decree, other pronouncement having the effect of law, constitution or treaty enacted, promulgated, issued, enforced or entered by any Governmental Authority applicable to any Party or such Party's businesses, properties or assets, as may be amended from time to time.

1.13. "**Audited Party**" has the meaning set forth in Section 10.14(b).

1.14. "**Auditing Party**" has the meaning set forth in Section 10.14(b).

1.15. "**Auditor**" has the meaning set forth in Section 10.14(b).

1.16. "**Biosimilar Product**" means, in a particular country with respect to a particular Product, any biopharmaceutical product that: (a) has received all necessary approvals by the applicable Regulatory Authorities in such country to market and sell such product as a biopharmaceutical product; (b) is marketed or sold by a Third Party who is not a licensee or Sublicensee of Janssen or its Affiliates regarding such biopharmaceutical product; and (c) is approved as (i) a "biosimilar" (in the United States) of such Product, (ii) a "similar biological medicinal product" (in the EU) with respect to which such Product is the "reference medicinal product," or (iii) if not in the US or EU, the foreign equivalent of a "biosimilar" or "similar biological medicinal product" of such Product, in each case, for use in such country pursuant to a regulatory approval process governing approval of generic biologics based on the then-current standards for regulatory approval in such country (*e.g.*, the Biologics Price Competition and Innovation Act of 2009 or an equivalent under foreign law).

1.17. "**BLA**" means: (a) a Biologics License Application as defined in the Act and the regulations promulgated thereunder; (b) an MAA in the EU; or (c) any equivalent or comparable application, registration or certification in any other country or region.

1.18. "**Business Day**" means any day that is not a Saturday, Sunday, or other day on which commercial banks are authorized or required to be closed in New York, New York.

1.19. "**Calendar Quarter**" means a financial quarter based on the J&J Universal Calendar for that year that is used by Janssen and Affiliates for internal and external reporting purposes; *provided*, *however*, that the first Calendar Quarter of the Term extends from the Effective Date to the end of the then-current Calendar Quarter, and the last Calendar Quarter extends from the first day of such Calendar Quarter until the effective date of the termination or expiration of this Agreement.

1.20. "**Calendar Year**" means a year based on the J&J Universal Calendar for that year; *provided*, *however*, that the first Calendar Year of the Term extends from the Effective Date to the end of the then-current Calendar Year, and the last Calendar Year extends from the first day of such Calendar Year for the year during which termination or expiration of this Agreement will occur until the effective date of the termination or expiration of this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.21. "**CAPA**" has the meaning set forth in Section 3.6.

1.22. "**cGCP**" means the then-current ethical, scientific and quality standards, practices and procedures required by FDA for designing, conducting, recording and reporting trials that involve the participation of human subjects, as set forth in FDA regulations in 21 C.F.R. Parts 11, 50, 54, 56, and 312 and related FDA guidance documents, including the guidelines entitled "Guidance for Industry E6 Good Clinical Practice: Consolidated Guidance" and by the International Conference on Harmonization E6: Good Clinical Practices Consolidated Guideline, or the equivalent Applicable Law of an applicable Regulatory Authority.

1.23. "**cGLP**" means the then-current good laboratory practice standards as required by the FDA under 21 C.F.R. Part 58 and all applicable FDA rules, regulations, orders and guidance, and the requirements with respect to current good laboratory practices prescribed by the European Community, the OECD (Organization for Economic Cooperation and Development Council) and the ICH Guidelines, or the equivalent Applicable Law of an applicable Regulatory Authority.

1.24. "**cGMP**" means the then-current good manufacturing practices as required by the FDA under provisions of 21 C.F.R. Parts 210 and 211 and all applicable FDA rules, regulations, orders and guidance, and the requirements with respect to current good manufacturing practices prescribed by the European Community under provisions of "The Rules Governing Medicinal Products in the European Community, Volume 4, Good Manufacturing Practices, Annex 13, Manufacture of Investigational Medicinal Products, July 2003," or the equivalent Applicable Law of an applicable Regulatory Authority.

1.25. "**Challenge**" or "**Challenging**" means, with respect to any MeiraGTx Patents, MeiraGTx Research Patents or Joint Patents, to contest the validity or enforceability of any such Patents, in whole or in part, in any court, arbitration proceeding or other tribunal, including the United States Patent and Trademark Office, the European Patent Office, and the United States International Trade Commission. As used in this term "Challenge", the term "**contest**" includes [***].

1.26. "**Challenged Patent**" has the meaning set forth in Section 15.2(c).

1.27. "**Change of Control**" means, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent at least fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation; (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of fifty percent (50%) or more of the combined voting power of the outstanding securities of such Party; or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's assets or business to which the subject matter of this Agreement relates.

1.28. "**Claims**" means all Third Party demands, claims, actions, suits, causes of action and proceedings.

1.29. "**Clinical Development Budget**" has the meaning set forth in Section 6.1(d). Each Clinical Development Budget, upon approval in accordance with the terms and conditions herewith, will be automatically incorporated into this Agreement by reference and form a part of this Agreement.

1.30. "**Clinical Development Costs**" means, with respect to each Clinical IRD Product [***] all costs and expenses incurred on or after the Effective Date in connection with the performance of any Clinical Development Plan Activities for such Clinical IRD Product in accordance with the applicable Clinical Development Plan, including FTE Costs and fees charged by Third Party service providers, but excluding (a) overhead costs and capital expenditures and (b) all Clinical Development Manufacturing Costs.

1.31. "**Clinical Development Manufacturing Activities**" has the meaning set forth in Section 8.4(a).

1.32. "**Clinical Development Manufacturing Budget**" has the meaning set forth in Section 8.4(b). Each Clinical Development Manufacturing Budget, upon approval in accordance with the terms and conditions herewith, will be automatically incorporated into this Agreement by reference and form a part of this Agreement.

1.33. "**Clinical Development Manufacturing Costs**" means the fully-burdened cost incurred on or after the Effective Date (and, solely with respect to the Clinical Supply of the CNGA3 Product, such costs incurred prior to the Effective Date and as specified on Exhibit 1.34-1) by either Party or any of their respective Affiliates in connection with the performance of any Clinical Development Manufacturing Activities in accordance with this Agreement and consistent with (a) the applicable CMC Development Plan, (b) the Clinical Supply Agreement, and (c) all components defined in Commercial Manufacturing Costs, including in each case ((a)-(c)), all FTE Costs, Out-of-Pocket Costs, and depreciation of capital expenditures.

1.34. "**Clinical Development Plan**" means the strategic plan for Developing a Clinical IRD Product, as such plan may be agreed to, approved, amended or updated from time to time in accordance with Section 6.1(c). Each Clinical Development Plan will be automatically incorporated into this Agreement by reference and form a part of Exhibit 1.34 in accordance with Section 6.1(c). The anticipated costs and timelines for activities under the Clinical Development Plan for the CNGA3 Product are attached hereto as Exhibit 1.34-1. The anticipated costs and timelines for activities under the Clinical Development Plan for the CNGB3 Product are attached hereto as Exhibit 1.34-2. The anticipated costs and timelines for activities under the Clinical Development Plan for the RPGR Product are attached hereto as Exhibit 1.34-3.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.35. "**Clinical Development Plan Activities**" has the meaning set forth in Section 6.1(c).

1.36. "**Clinical Development Plan Term**" means the term of a Clinical Development Plan, as set forth in such Clinical Development Plan, *provided* that in no event will the Clinical Development Plan Term exceed the Term.

1.37. "**Clinical Development Records**" has the meaning set forth in Section 6.1(g).

1.38. "**Clinical IRD Patents**" has the meaning set forth in Section 11.4(b).

1.39. "**Clinical IRD Products**" means: (a) the CNGB3 Product; (b) the CNGA3 Product; (c) the RPGR Product; and (d) [***].

1.40. "**Clinical IRD Target**" means: (a) the CNGB3 Target; (b) the CNGA3 Target; (c) the RPGR Target; and (d) [***].

1.41. "**Clinical IRD Target Indication**" means: (a) the CNGB3 Target Indication; (b) the CNGA3 Target Indication; (c) the RPGR Target Indication; and (d) [***].

1.42. "**Clinical Quality Assurance Agreement**" has the meaning set forth in Section 8.2.

1.43. "**Clinical Study**" means a (a) Phase 1 Study, (b) Phase 1/2 Study, (c) Phase 2 Study, (d) Phase 3 Study, (e) Pivotal Study or (f) other prospective study (including a non-interventional study or Natural History Study) or post-Regulatory Approval study, in each case of this subsection (f) in humans to obtain information regarding a disease state or product, including information relating to the safety, tolerability, pharmacological activity, pharmacokinetics, dose ranging or efficacy of the product.

1.44. "**Clinical Supply**" means, with respect to a Clinical IRD Product or Janssen Research IRD Product, such Product Manufactured for use in a Clinical Study of such Product under this Agreement.

1.45. "**Clinical Supply Agreement**" has the meaning set forth in Section 8.2.

1.46. "**CMC**" means chemistry, manufacturing and controls.

1.47. "**CMC Development Collaboration**" has the meaning set forth in Section 8.4(a).

1.48. "**CMC Development Inventions**" means all Inventions, including CMC data, arising out of the CMC Development Collaboration, whether made solely by or on behalf of a Party or jointly with the other Party under this Agreement.

1.49. "**CMC Development Know-How**" means all Know-How, including CMC data, arising out of the CMC Development Collaboration, whether made solely by or on behalf of a Party or jointly with the other Party under this Agreement.

1.50. "**CMC Development Patents**" mean all Patents Covering patentable CMC Development Inventions or CMC Development Know-How.

1.51. "**CMC Development Plan**" means the strategic plan for the CMC Development Collaboration, as such plan may be agreed to, approved, amended or updated from time to time in accordance with Section 8.4(a). Each CMC Development Plan will be automatically incorporated into this Agreement by reference and form a part of Exhibit 1.51.

1.52. "**CMC Development Plan Activities**" has the meaning set forth in Section 8.4(a).

1.53. "**CMC Development Technology**" means the CMC Development Patents, CMC Development Inventions and CMC Development Know-How.

1.54. "**CMC Development Term**" has the meaning set forth in Section 2.3.

1.55. "**CMO**" means a Third Party contract Manufacturing organization.

1.56. "**CNGA3 Product**" means: (a) MeiraGTx's Gene Therapy Product [***] for the treatment of the CNGA3 Target Indication by expressing the CNGA3 Target; [***].

1.57. "**CNGA3 Target**" means the [***].

1.58. "**CNGA3 Target Indication**" means the inherited retinal disease resulting from the loss of function of the CNGA3 Target.

1.59. "**CNGB3 Product**" means: (a) MeiraGTx's Gene Therapy Product [***] for the treatment of the CNGB3 Target Indication by expressing the CNGB3 Target; [***].

1.60. "**CNGB3 Target**" means the [***].

1.61. "**CNGB3 Target Indication**" means the inherited retinal disease resulting from the loss of function of the CNGB3 Target.

1.62. "**Code**" means the United States Bankruptcy Code, 11 U.S.C. §§ 101 et seq.

1.63. "Commercial Manufacturing Costs" means [***].

1.64. "**Commercial Milestone Event**" has the meaning set forth in Section 10.7(a).

1.65. "**Commercial Milestone Payment**" has the meaning set forth in Section 10.7(a).

1.66. "**Commercial Quality Assurance Agreement**" has the meaning set forth in Section 8.3.

1.67. "**Commercial Supply**" means a Product Manufactured for Commercialization of such Product, including for Product launch.

1.68. "**Commercial Supply Agreement**" has the meaning set forth in Section 8.3.

1.69. "**Commercialize**" or "**Commercialization**" means to market, promote, detail, conduct Medical Affairs, distribute, import, export, offer to sell, use or sell biopharmaceutical products or conduct other commercialization activities, including activities directed to obtaining Pricing Approvals, conducting pre- and post-Regulatory Approval activities and launching and promoting such biopharmaceutical products in each country, as applicable.

1.70. "**Commercially Reasonable Efforts**" means, with respect to the efforts to be expended by a Party with respect to any objective under this Agreement, those reasonable, good faith efforts normally used by such Party under similar circumstances for similar products or product candidates owned or controlled by such Party, or to which such Party has similar rights, which product or product candidate is of similar market potential in such country and is at a similar stage in its development or product life, taking into account all relevant scientific, technical, operational, commercial, economic and other factors that may affect the Development, Regulatory Approval, Manufacturing or Commercialization of a product, including (as applicable): actual and potential issues of safety, efficacy or stability; expected and actual product profile (including product modality, category and mechanism of action); stage of development or life cycle status; actual and projected Development, Regulatory Approval, Manufacturing, and Commercialization costs, timelines and budgets; any issues regarding the ability to Manufacture or have Manufactured the product; the likelihood of obtaining Regulatory Approvals; the timing of such approvals; labeling or anticipated labeling; the then-current competitive environment and the likely competitive environment at the time of projected entry into the market, including the expected and actual competitiveness of alternative products; past performance of the product or similar products; present and future market potential; existing or projected pricing, sales, reimbursement and profitability; and expected and actual proprietary position, strength and duration of patent protection and anticipated regulatory or other exclusivity as such company would normally use to accomplish a similar objective under similar circumstances. With respect to a Party's obligations, Commercially Reasonable Efforts requires that the Party, to the extent doing so would be required by this Agreement and the definition of "Commercially Reasonable Efforts" to do so: [***] To the extent that [***]. To the extent that [***].

1.71. "**Committee**" means the Joint Steering Committee, the Joint Research Committee, the Joint Development Committee, the Joint Manufacturing Committee or any other subcommittee established under Section 5.2(b), as applicable.

1.72. "**Competing Product**" means, with respect to any Product, any Gene Therapy Product that [***].

1.73. "**Completion Notice**" has the meaning set forth in Section 3.7(c).

1.74. "**Confidential Information**" means all confidential Know-How and other confidential information and data of a Party that is disclosed by or on behalf of a Party or any of its Affiliates or otherwise made available to the other Party or its Affiliates, whether made available orally, in writing or in electronic form, including information comprising or relating to concepts, discoveries, inventions, data, designs or formulae in relation to this Agreement or of a financial, commercial, business, operational or technical nature. For clarity, the terms and conditions of this Agreement shall constitute the Confidential Information of both Parties and each Party will be deemed a Receiving Party with respect thereto, and all research results shall constitute the Confidential Information of both Parties during the Term (subject to Section 15.4(b)).

1.75. "**Control**" or "**Controlled**" means, with respect to any Intellectual Property Rights, the legal authority or right (whether by ownership, license or otherwise, other than pursuant to this Agreement) of a Party to grant a license or a sublicense of or under such Intellectual Property Rights to another Person, or to otherwise disclose such Intellectual Property Rights to another Person, without violating any Applicable Law, breaching the terms of any agreement with a Third Party or misappropriating the proprietary or trade secret information of a Third Party, and, subject to Section 10.10, without incurring payment obligations by reason of licensing, sublicensing, or providing access to the other Party with respect thereto (unless such other Party agrees in writing to bear all such costs arising from the license, sublicense, or access to such item by such other Party). Notwithstanding anything to the contrary in this Agreement, in the event of a Change of Control of a Party, (a) any Intellectual Property Rights Controlled by any acquiring entity (and not Controlled by such Party or its Affiliates) immediately prior to the effective date of such Change of Control and (b) any Intellectual Property Rights independently developed or acquired by or on behalf of any acquiring entity without access to or use of any Intellectual Property Rights used or made available under this Agreement or pre-acquisition employees of such Party or its pre-acquisition Affiliates, in each case ((a) and (b)) shall not be deemed to be Controlled by such Party or its Affiliates after the effective date of such Change of Control for purposes of this Agreement.

1.76. "**Controlling Party**" has the meaning set forth in Section 11.5(d).

1.77. "**Cover**" or "**Covered**" means that, but for a license granted to a Person under a Valid Claim of a Patent, the act of Developing, Manufacturing, or Commercializing by such Person would infringe, or contribute to or induce the infringement of, such Valid Claim.

1.78. "**CPR Mediation Procedure**" has the meaning set forth in Section 18.1(b).

1.79. "**CPR Rules**" has the meaning set forth in Section 18.1(c)(i).

1.80. "**Damages**" means all losses, liabilities, damages, taxes, costs and expenses of every kind and nature (including reasonable attorneys' fees).

1.81. "**Data Exclusivity Right**" means any data exclusivity rights or exclusive marketing rights conferred by any Regulatory Authority with respect to a Product (other than Patents), including orphan drug exclusivity, market exclusivity, data exclusivity, or pediatric exclusivity.

1.82. "**Debarred Person**" means a Person that is: (a) debarred from or disqualified under the Act or any other governmental program; (b) on any of the FDA clinical investigator enforcement lists (including, the (i) Disqualified/Totally Restricted List, (ii) Restricted List and (iii) Adequate Assurances List); (c) excluded from participation in any governmental healthcare program or other federal or state program, convicted of an offense under 42 U.S.C. § 1320a-7, or otherwise deemed ineligible for participation in health care or federal or state programs; or (d) is the subject of a conviction described in 21 U.S.C. 335a or is subject to any similar sanction.

1.83. "**Deemed Sublicensee Distributor**" means any Third Party appointed by Janssen or any of its Affiliates or its or their sublicensees to distribute, market, and sell Product, with or without packaging rights, in one or more countries in the Territory, in circumstances where: (a) the Third Party purchases Product from Janssen or its Affiliates or its or their sublicensees and otherwise makes an upfront, royalty or other payment (separate from a payment for supply of Product) to Janssen or its Affiliates or its or their sublicensees with respect to Product; or (b) the Third Party engages in material promotional activity under a co-promotion agreement with Janssen with respect to Product. For clarity, a Deemed Sublicensee Distributor shall be deemed a Sublicensee for the purpose of Net Sales and corresponding Royalty calculations.

1.84. "**Develop**" or "**Development**" means any and all drug development activities, other than Research activities, conducted before or after obtaining Regulatory Approval that are reasonably related to or leading to the development, preparation and submission of data and information to a Regulatory Authority for the purpose of obtaining, supporting or expanding Regulatory Approval or to the appropriate body for obtaining, supporting or expanding Pricing Approval, including all activities related to pharmacokinetic profiling, design and conduct of Clinical Studies, regulatory affairs, regulatory strategy, safety matters, statistical analysis, report writing, and Regulatory Filing creation and submission (including the services of outside advisors and consultants in connection therewith).

1.85. "**Development Milestone Event**" has the meaning set forth in Section 10.6.

1.86. "**Development Milestone Payment**" has the meaning set forth in Section 10.6.

1.87. "**Disclosing Party**" has the meaning set forth in Section 12.1.

1.88. "**Dispute**" has the meaning set forth in Section 18.1(a).

1.89. "**Distributor**" means any Third Party appointed by Janssen or any of its Affiliates or its or their sublicensees to distribute, market, and sell Product, with or without packaging rights, in one or more countries in the Territory, in circumstances where: (a) the Third Party purchases Product from Janssen or its Affiliates or its or their sublicensees, but does not otherwise make any upfront, royalty or other payment (separate from a payment for supply of Product) to Janssen or its Affiliates or its or their sublicensees with respect to Product; and (b) the Third Party does not engage in any material promotional activity under a co-promotion agreement with Janssen with respect to Product.

1.90. "**Dollar**" or "**Dollars**" or "**$**" means the legal tender of the United States of America.

1.91. "**Drug Master File**" means, for each Product, the drug master file that includes detailed information about such Product, processes, facilities, articles or Materials used in the Manufacture of such Product, which is or is intended to be submitted to a Regulatory Authority, including "Drug Master Files" as defined in 21 C.F.R. § 314.420, any non-United States equivalents and any equivalent information applicable for a Gene Therapy Product.

1.92. "**Effective Date**" has the meaning set forth in Section 14.1

1.93. "**EMA**" means the European Medicines Agency or any successor entity thereto.

1.94. "**End-of-Phase 2 Meeting**" means any "End-of-Phase 2 Meeting" as described in 21 CFR 312.47, or, with respect to the EU, a similar meeting with the EMA.

1.95. "**EU**" means the European Union, as its membership may be constituted from time to time, and any successor thereto; *except* that, for purposes of this Agreement, the EU will be deemed to include [***].

1.96. "**European Commission**" means the executive of the EU that promotes its general interest.

1.97. "**Execution Date**" has the meaning set forth in the first paragraph of this Agreement.

1.98. "**Executive Officers**" means the Chief Executive Officer of MeiraGTx and the Global Head of Janssen Research and Development of Janssen.

1.99. "**Existing Third Party Obligations**" has the meaning set forth in Section 10.10(a).

1.100. "**FCPA**" means the U.S. Foreign Corrupt Practices Act (15 U.S.C. § 78dd-1, et seq.).

1.101. "**FDA**" means the United States Food and Drug Administration or any successor entity thereto.

1.102. "**Field**" means the diagnosis, prevention or treatment of diseases and other conditions in all Indications in humans.

1.103. "**Final Report**" means, with respect to each Research Plan, a final report containing, at a minimum, the contents of Exhibit 1.103, including [***]. A Final Report must contain the results of Research Plan Activities that [***].

1.104. "**Final Report Delivery Date**" has the meaning set forth in Section 3.7(c).

1.105. "**First Commercial Sale**" means, with respect to any Product, and on a country-by-country basis, the first commercial sale in an arm's length transaction of such Product to a Third Party by Janssen, its Affiliates or Sublicensees in such country following receipt of applicable Regulatory Approval and Pricing Approval of such Product in such country. For

clarity, the First Commercial Sale of a Product shall not include: (a) any distribution or other sale solely where the Product is supplied without charge or at the actual Manufacturing cost thereof (without allocation of indirect costs or any markup); (b) any sale by Janssen to its Affiliates or Sublicensees for further re-sale by such Affiliate or Sublicensee; or (c) sales for clinical trial purposes, early access or compassionate use programs.

1.106. [***]

1.107. "**Force Majeure**" has the meaning set forth in Section 19.4.

1.108. "**FTE**" means a full-time person, or more than one person working the equivalent of a full-time person, working directly on performing Research Plan Activities, Clinical Development Plan Activities or CMC Development Plan Activities, where "full-time" is considered [***] per Calendar Year. For clarity, indirect personnel (including support functions such as managerial, financial, legal or business development) shall not constitute FTEs.

1.109. "**FTE Costs**" means, for any period, the FTE Rate multiplied by the number of FTEs in such period.

1.110. "**FTE Rate**" means [***] The FTE Rate shall be adjusted annually in proportion to the Consumer Price Index for all Urban Consumers for [***] to [***], as published by the U.S. Department of Labor, Bureau of Statistics (national CPI-U; Base Period: 1982-84=100; available at http://www.bls.gov/cpi/home.htm). Rate changes shall be effective as of the first (1st) day of the first (1st) Calendar Quarter of the Calendar Year. Notwithstanding the foregoing, for any time period during the Term that is less than a full Calendar Year, the above referenced rate will be proportionately reduced to reflect such portion of FTEs for such full Calendar Year.

1.111. "**Future Third Party Obligations**" means any Intellectual Property Rights of a Third Party that Cover the composition of matter, use or Manufacture of, or are necessary or useful to use or make, a Product, excluding any Intellectual Property Rights under any Existing Third Party Obligation.

1.112. "**GAAP**" means accounting principles generally accepted in the United States of America, as in effect from time to time, consistently applied.

1.113. "**Gene Therapy Product**" means any [***] product that delivers [***] for purposes of [***].

1.114. "**Governing Law**" has the meaning set forth in Section 18.2.

1.115. "**Governmental Authority**" means any applicable government authority, court, tribunal, arbitrator, agency, department, legislative body, commission or other instrumentality of: (a) any government of any country or territory; (b) any nation, state, province, county, city or other political subdivision thereof; (c) any supranational body; or (d) any arbitrator with binding authority.

1.116. "**HSR Act**" means the Hart-Scott-Rodino Act of 1976.

1.117. "**HSR Clearance Date**" means the earliest date on which the Parties have actual knowledge that all applicable waiting periods under the HSR Act with respect to the transactions contemplated under this Agreement have expired or have been terminated.

1.118. "**HSR Filing**" means filings by Janssen and MeiraGTx with the United States Federal Trade Commission and the United States Department of Justice of a Notification and Report Form for Certain Mergers and Acquisitions (as that term is defined in the HSR Act) with respect to the matters set forth in this Agreement, together with all required documentary attachments thereto, or any foreign form substantially equivalent thereto.

1.119. "**Improved Clinical IRD Product**" means, for any Clinical IRD Product, any Gene Therapy Product that: (a) contains [***]; and (b) [***].

1.120. "**Improved Janssen Research IRD Product**" means, for any Janssen Research IRD Product, any Gene Therapy Product that: (a) contains [***]; and (b) [***].

1.121. "**IND**" means an Investigational New Drug Application (including any amendments thereto) filed with the FDA pursuant to 21 CFR Part 312 before the commencement of a clinical trial of a Product, or a similar application filed with an applicable Regulatory Authority outside of the United States such as a clinical trial application or a clinical trial exemption, or any other equivalent or related regulatory submission, license or authorization.

1.122. "**IND Submission**" means the submission of an IND to a Regulatory Authority in a Major Market Country.

1.123. "**Indemnification Claim Notice**" has the meaning set forth in Section 17.3(b).

1.124. "**Indemnified Party**" has the meaning set forth in Section 17.3(b).

1.125. "**Indemnifying Party**" has the meaning set forth in Section 17.3(b).

1.126. "**Indemnitee**" means a MeiraGTx Indemnitee or a Janssen Indemnitee, as the context requires.

1.127. "**Indication**" means (a) any disease, condition or syndrome, or (b) any sign or symptom of or associated with a disease, condition or syndrome.

1.128. "**Initial Research Plan Term**" has the meaning set forth in Section 2.2(b).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.129. "**Insolvency Event**" means, in relation to either Party, any one of the following: (a) that Party becomes insolvent according to Applicable Law; (b) that Party is the subject of voluntary or involuntary bankruptcy proceedings instituted on behalf of or against such Party (except for involuntary bankruptcy proceedings, which are dismissed within [***]; (c) an administrative receiver, receiver and manager, interim receiver, custodian, sequestrator, or similar officer is appointed in respect of that Party; (d) a notice shall have been issued to convene a meeting for the purpose of passing a resolution to wind up that Party, or such a resolution shall have been passed other than a resolution for the solvent reconstruction or reorganization of that Party; (e) a resolution shall have been passed by that Party or that Party's directors to make an application for an administration order or to appoint an administrator; or (f) that Party proposes or makes any general assignment, composition, or arrangement with or for the benefit of all or some of that Party's creditors or makes or suspends or threatens to suspend making payments to all or some of that Party's creditors.

1.130. "**Intellectual Property Rights**" means any Know-How, Inventions, Patents, and copyrights.

1.131. "**Interest Rate**" has the meaning set forth in Section 10.13(f).

1.132. "**Interim Report**" has the meaning set forth in Section 3.7(b).

1.133. "**Internal Research**" means non-commercial research activities conducted by Janssen.

1.134. "**Invention**" means any process, method, composition of matter, article of manufacture, discovery, improvement, or finding, including Know-How, patentable or otherwise, that is first developed, generated, conceived or reduced to practice as a result of a Party (acting solely or jointly with the other Party) exercising its rights or carrying out its obligations in accordance with this Agreement, whether directly or via its Affiliates, employees, agents or independent contractors, including all rights, title and interests in and to the intellectual property rights in and to any of the foregoing.

1.135. "**IRD Gene**" means a gene whose loss of function is responsible for an inherited retinal disease.

1.136. "**J&J Universal Calendar**" means the Johnson & Johnson universal calendar for a given year. The J&J Universal Calendar for 2019 and 2020 is set forth on Exhibit 1.136 attached hereto, and the J&J Universal Calendar for subsequent years will be provided to MeiraGTx by Janssen upon MeiraGTx's request in writing from time to time.

1.137. "**Janssen**" has the meaning set forth in the first paragraph of this Agreement.

1.138. "**Janssen Indemnitees**" has the meaning set forth in Section 17.1.

1.139. "**Janssen Quality Requirements**" means, with respect to (a) MeiraGTx's Manufacturing or supply obligations for any Products under this Agreement and (b) the Clinical Studies to be conducted pursuant to this Agreement, in each case ((a) and (b)) the standards and requirements that meet Janssen's or any Regulatory Authorities' quality requirements.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.140. "**Janssen Research IRD Patents**" has the meaning set forth in Section 11.4(d)(ii).

1.141. "**Janssen Research IRD Product**" means (a) any Research IRD Product for which Janssen has exercised the applicable Option in accordance with Section 3.9(b), and (b) any Improved Janssen Research IRD Product.

1.142. "**Janssen Technology**" means all Patents, Inventions and Know-How Controlled by Janssen or its Affiliates during the Term that are necessary to conduct the Research Plan Activities for a Research IRD Product in accordance with the respective Research Plan, conduct the Clinical Development Plan Activities for a Clinical IRD Product in accordance with the respective Clinical Development Plan or conduct the CMC Development Plan Activities in accordance with the respective CMC Development Plan, in each case of the foregoing to the extent the results of such activities are or will be incorporated into, with Janssen's prior written permission, any Clinical IRD Product, Research IRD Product or Manufacturing process therefor.

1.143. "**Joint Development Committee**" or "**JDC**" means the committee established as set forth in Section 5.4(a).

1.144. "**Joint Inventions**" has the meaning set forth in Section 11.1(c).

1.145. "**Joint Manufacturing Committee**" or "**JMC**" means the committee established as set forth in Section 5.5(a).

1.146. "**Joint Patents**" has the meaning set forth in Section 11.1(c).

1.147. "**Joint Product Patents**" mean all Joint Patents that Cover (a) one or more components of a Product; or (b) any approved use of a Product; [***].

1.148. "**Joint Research Committee**" or "**JRC**" means the committee established as set forth in Section 5.3(a).

1.149. "**Joint Steering Committee**" or "**JSC**" means the committee established as set forth in Section 5.2(a).

1.150. "**Joint Technology**" means Joint Patents and Joint Inventions.

1.151. "**JRD**" has the meaning set forth in Section 10.13(g).

1.152. "**Know-How**" means any non-public or proprietary information and all other proprietary rights (including technical and scientific information) that may exist or be created under the laws of any jurisdiction in the world, including technical information, know-how, data (including pharmacological, toxicological, non-clinical and clinical data, analytical and quality control data, Manufacturing data and descriptions, market data, financial data or descriptions), Materials, research results, inventions (whether patentable or not), discoveries, trade secrets, specifications, instructions, processes, formulae, expertise, other

technology applicable to compounds, formulations, compositions or products, to their Manufacture, Development, registration, use or Commercialization, methods of assaying or testing them or processes for their Manufacture, formulations containment, compositions incorporating or comprising them, including all biological, chemical, pharmacological, biochemical, toxicological, pharmaceutical, physical and analytical, safety, quality control, Manufacturing, preclinical and clinical data, Regulatory Filings or Regulatory Materials and copies thereof, relevant to the Development, Manufacture, use or Commercialization of or which may be useful in studying, testing, development, production or formulation of products, or intermediates for the synthesis thereof, but excluding Patents.

1.153. "**Licensed Patent Action**" has the meaning set forth in Section 11.5(a).

1.154. "**Loss of Market Exclusivity Due to a Biosimilar Product**" means, with respect to any Product in any country, that both of the following events have occurred: (a) [***] and (b) one or more Biosimilar Product(s) of such Product are marketed or sold by one or more Third Parties who are not licensees or Sublicensees of Janssen or its Affiliates in such country.

1.155. "**Loss of Market Exclusivity Due to a Competing Product**" means, with respect to any [***] in any country, that all of the following events have occurred: (a) one or more Competing Product(s) of such [***] (other than a Biosimilar Product of such [***]) are marketed or sold by one or more Third Parties who are not licensees or Sublicensees of Janssen or its Affiliates in such country; [***]

1.156. "**MAA**" means an application for the authorization or approval to market Product(s) in any country or group of countries outside the United States, as defined by Applicable Law and filed with the Regulatory Authority of a given country or group of countries.

1.157. "Major European Countries" means [***].

1.158. "**Major Market Countries**" means the [***].

1.159. "**Manufacture**" or "**Manufacturing**" means activities directed to producing, manufacturing, processing, sourcing of materials, filling, finishing, packaging, labeling, quality assurance testing and release, shipping and storage of a product.

1.160. "**Manufacturing Costs**" means Clinical Development Manufacturing Costs or Commercial Manufacturing Costs, as applicable to the Product. For clarity, in no event shall [***].

1.161. "**Manufacturing Services Agreement**" has the meaning set forth in Section 8.5.

1.162. "**Materials**" means any tangible compositions of matter, articles of manufacture, assays, chemical, biological or physical materials, and other similar materials, including media composition.

1.163. "**Medical Affairs**" means activities conducted by a Party's or its Affiliate's medical affairs department, including communications with key opinion leaders, medical education, symposia, advisory boards (to the extent related to medical affairs or clinical guidance), activities performed in connection with patient registries, and other medical programs and communications, including educational grants, research grants (including conducting investigator-initiated studies), and charitable donations to the extent related to medical affairs and not to other activities that do not involve the promotion, marketing, sale, or other Commercialization of Products and are not conducted by a Party's medical affairs department.

1.164. "**MeiraGTx**" has the meaning set forth in the first paragraph of this Agreement.

1.165. "**MeiraGTx Facility**" means the Manufacturing facility operated by MeiraGTx or its Affiliate located at 92 Britannia Walk, London, N1 7NQ, United Kingdom, or such other Manufacturing facility operated by MeiraGTx or one of its Affiliates as mutually agreed by the Parties in writing.

1.166. "**MeiraGTx Indemnitees**" has the meaning set forth in Section 17.2.

1.167. "**MeiraGTx Know-How**" means any Know-How or Inventions Controlled by MeiraGTx or any of its Affiliates as of the Execution Date or thereafter until this Agreement expires or terminates, which are: (a) used in or otherwise relating to any Clinical IRD Product; or (b) otherwise used by MeiraGTx in the Development, Manufacture, or Commercialization of any Clinical IRD Product in the Field. The MeiraGTx Know-How excludes (y) any Know-How or Inventions embodied solely in the MeiraGTx [***] Technology and (z) any Joint Inventions.

1.168. "**MeiraGTx Patents**" means any Patents Controlled by MeiraGTx or any of its Affiliates as of the Execution Date or thereafter until this Agreement expires or terminates, which Cover any Clinical IRD Products, Clinical IRD Targets, MeiraGTx Know-How in the Field or Clinical Development Plan Activities, including such Patents set forth on Exhibit 1.168. The MeiraGTx Patents exclude (a) any Patents that solely Cover the MeiraGTx [***] Technology and (b) any Joint Patents.

1.169. "**MeiraGTx Research Know-How**" means any Know-How or Inventions Controlled by MeiraGTx or any of its Affiliates as of the Execution Date or thereafter until this Agreement expires or terminates that are used in or otherwise relating to any Research IRD Product (including any Janssen Research IRD Product). The MeiraGTx Research Know-How excludes (a) any Know-How or Inventions embodied solely in the MeiraGTx [***] Technology and (b) any Joint Inventions.

1.170. "**MeiraGTx Research Patents**" means any Patents Controlled by MeiraGTx or any of its Affiliates as of the Execution Date or thereafter until this Agreement expires or terminates that claim or otherwise Cover any Research IRD Products (including any Janssen Research IRD Products), Research IRD Targets or MeiraGTx Research Know-How in the Field or otherwise Cover any Research Plan Activities. The MeiraGTx Research Patents exclude (a) any Patents that solely Cover the MeiraGTx [***] Technology and (b) any Joint Patents.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.171. "**MeiraGTx Research Technology**" means the MeiraGTx Research Know-How and the MeiraGTx Research Patents.

1.172. "**MeiraGTx [***] Technology**" means MeiraGTx's [***] technology, which directly relates to [***].

1.173. "**MeiraGTx Technology**" means the MeiraGTx Know-How and the MeiraGTx Patents.

1.174. "**Missing Information Notice**" has the meaning set forth in Section 3.7(c).

1.175. "**Natural History Study**" means a human clinical study that follows a group of people over time who have, or are at risk of developing, a specific medical condition or disease.

1.176. "**Net Sales**" means the gross amounts invoiced on sales of a Product by Janssen, or any of its Affiliates or Sublicensees, to a Third Party purchaser in an arms-length transaction, less the following customary and commercially reasonable deductions, determined in accordance with Janssen's Accounting Standards and internal policies and actually taken, paid, accrued, allocated or allowed based on good faith estimates:

(a) trade, cash or quantity discounts, allowances and credits, excluding commissions for commercialization;

(b) excise taxes, use taxes, tariffs, sales taxes and customs duties, or other government charges imposed on the sale of Product (including value added tax, but only to the extent that such value added taxes are not reimbursable or refundable), and specifically excluding, for clarity, any income taxes assessed against the income arising from such sale;

(c) compulsory or negotiated payments and cash rebates or other expenditures to governmental authorities (or designated beneficiaries thereof) in the context of any national or local health insurance programs or similar programs; including pay-for-performance agreements, risk sharing agreements and government levied fees as a result of the Affordable Care Act;

(d) rebates, chargebacks, administrative fees and discounts (or equivalent thereof) to managed health care organizations, group purchasing organizations, insurers, pharmacy benefit managers (or equivalent thereof), specialty pharmacy providers, governmental authorities or their agencies or purchasers, reimbursers or trade customers, as well as amounts owed to patients through co-pay assistance cards or similar forms of rebate to the extent the latter are directly related to the prescribing of Product;

(e) outbound freight, shipment and insurance costs to the extent included in the price and separately itemized on the invoice price;

(f) retroactive price reductions, credits or allowances actually granted upon claims, rejections or returns of Product, including for recalls or damaged or expired goods, billing errors and reserves for returns;

(g) any invoiced amounts which are not collected by the selling Party or its Affiliates or Sublicensees, including bad debts; and

(h) any deductions in the context of payments that are due or collected significantly after invoice issuance.

All aforementioned deductions shall only be allowable to the extent they are commercially reasonable by Janssen and shall be determined, on a country-by-country basis, as incurred in the ordinary course of business in type and amount verifiable based on Janssen's and its Affiliates' reporting system. All such discounts, allowances, credits, rebates, and other deductions shall be fairly and equitably allocated to Product and other products of Janssen and its Affiliates and Sublicensees such that Product does not bear a disproportionate portion of such deductions.

The following shall be excluded from Net Sales calculations for all purposes: (a) sales of Product for the use in conducting Clinical Studies or other scientific testing of Product in a country, (b) compassionate and named patient sales or sales on an Affordable Basis and (c) any disposition of Product as free samples, donations, patient assistance, test marketing programs or other similar programs or studies.

1.177. "**Non-Withholding Party**" has the meaning set forth in Section 10.13(d).

1.178. "**Operational Team**" has the meaning set forth in Section 5.6.

1.179. "**Option**" has the meaning set forth in Section 3.9(b).

1.180. "**Option Election Date**" has the meaning set forth in Section 3.9(b).

1.181. "**Option Exercise Notice**" has the meaning set forth in Section 3.9(b).

1.182. "**Option Fee**" has the meaning set forth in Section 3.9(b).

1.183. "**Option Opt-Out**" has the meaning set forth in Section 3.9(b).

1.184. "**Option Opt-Out Date**" has the meaning set forth in Section 3.9(b).

1.185. "**Option Period**" means, for a given Research IRD Product, the period (a) beginning on the date that the Parties finalize a Research Plan for such Research IRD Target associated with such Research IRD Product and (b) expiring on (i) such date that is [***] after the Final Report Delivery Date with respect to such Research IRD Target associated with such Research IRD Product or (ii) such date that the Parties otherwise mutually agree to in writing.

1.186. "**Out-of-Pocket Costs**" means, with respect to certain activities hereunder, direct expenses paid or payable by either Party or its Affiliates to Third Parties for services or materials specifically identifiable and incurred to conduct such activities for such Product in the Territory, including payments to contract personnel (including contractors, consultants and subcontractors) in each case, pursuant to the applicable Clinical Development Plan, Research Plan or CMC Development Plan, and provided that such expenses shall have been recorded as income statement items in accordance with such Party's Accounting Standards and shall not include any pre-paid amounts, capital expenditures, or items intended to be covered by the FTE Rate.

1.187. "**Party**" or "**Parties**" has the meaning set forth in the first paragraph of this Agreement.

1.188. "**Patents**" means any and all (a) patents, (b) pending patent applications, including all provisionals applications, divisionals, continuations, substitutions, continuations-in-part, divisions and renewals, and all patents granted thereon, (c) all patents-of-addition, reissues, reexaminations and extensions or restorations by existing or future extension or restoration mechanisms, including supplementary protection certificates or the equivalent thereof, (d) inventor's certificates, (e) any other form of government-issued right substantially similar to any of the foregoing, and (f) all United States and foreign counterparts of any of the foregoing.

1.189. "**Person**" means any individual, limited liability or general partnership, limited liability company, joint venture, firm, corporation, association, trust, unincorporated organization or other entity or body, including a Governmental Authority.

1.190. "**Phase 1 Study**" means a clinical study of an investigational product in patients with the primary objective of characterizing its safety, tolerability, and pharmacokinetics and identifying a recommended dose and regimen for future studies. The investigational product can be administered to patients as a single agent or in combination with other investigational or marketed agents and shall be deemed commenced when the first patient in such study has received his or her initial dose of a product. A "Phase 1 Study" shall include any clinical trial that would satisfy the requirements of 21 C.F.R. § 312.21(a), or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States.

1.191. "**Phase 1/2 Study**" means a combined Phase 1 Study and Phase 2 Study.

1.192. "**Phase 2 Study**" means a clinical study of an investigational product in patients with the primary objective of characterizing its activity in a specific disease state as well as generating more detailed safety, tolerability, and pharmacokinetics information. The investigational product can be administered to patients as a single agent or in combination with other investigational or marketed agents and shall be deemed commenced when the first patient in such study has received his or her initial dose of a product. A "Phase 2 Study" shall include any clinical trial that would satisfy the requirements of 21 C.F.R. § 312.21(b), or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States.

1.193. "**Phase 3 Study**" means a clinical study of an investigational product in patients that incorporates accepted endpoints for confirmation of statistical significance of efficacy and safety with the aim to obtain Regulatory Approval in any country as described in 21 C.F.R. § 312.21(c), or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States. The investigational product can be administered to patients as a single agent or in combination with other investigational or marketed agents and shall be deemed commenced when the first patient in such study has received his or her initial dose of a product. For clarity, Phase 3 Studies include clinical studies of approved products for unapproved Indications.

1.194. "**Pivotal Study**" means a human clinical study in any country that is prospectively designed to generate data intended to satisfy the requirements of 21 C.F.R. § 312.21(c) in the U.S. or a similar clinical study prescribed by a Regulatory Authority from another country, from time to time, pursuant to Applicable Law. For clarity, a Pivotal Study may be a Phase 2 Study, a Phase 1/2 Study, or a Phase 3 Study.

1.195. "**PO**" has the meaning set forth in Section 10.13(c).

1.196. [***]

1.197. "**Pricing Approval**" means, with respect to a product and any country or regulatory jurisdiction, any pricing and reimbursement approvals that are commercially necessary to conduct a launch of such product in such country or regulatory jurisdiction (even if such approvals are not legally required to launch such product in such country or regulatory jurisdiction). For purposes of illustration, with respect to the Major European Countries, the following pricing and reimbursement approvals are examples of those that are currently necessary to conduct a launch of a drug or biological product: in France, publication of the reimbursed price level in the official journal and registration on a reimbursement list by or on behalf of Comité Economique des Produits de Santé or Haute Autorité de Santé (or a successor agency); in Italy, publication of reimbursement in the Government's Official Gazette (by Agenzia Italiana del Farmaco or a successor agency); in Germany, execution of contract with the head association of sick funds (GKV-Spitzenverband, Gesetzlichen Krankenversicherung, or a successor agency); in Spain, authorization by La Comisión Interministerial de Precios de los Medicamentos or La Comisión Nacional para el Uso Racional de los Medicamentos (or a successor agency) for national patient access to reimbursement by or on behalf of a Governmental Authority; and in the United Kingdom, a recommendation by the National Institute for Health and Care Excellence (or a successor agency) to obtain mandatory funding to enable broad market access.

1.198. "**Prior CDA**" means that certain Confidential Disclosure Agreement, dated [***].

1.199. "**Priority Review Voucher**" means a priority review voucher issued by the United States Department of Health and Human Services that entitles the holder of such voucher to Priority Review of a single human drug application submitted under Section 505(b)(1) of the Act or Section 351(a) of the United States Public Health Service Act, as further defined in Section 529(a)(2) of the Act (21 U.S.C. § 360ff(a)(2)).

1.200. "**Privacy and Data Security Laws**" means all applicable privacy, security and data protection laws, rules, regulations, and guidelines with respect to privacy, security and data protection including the collection, processing, storage, protection and disclosure of Sensitive Information.

1.201. "**Process Development Activities**" has the meaning set forth in Section 8.4(a).

1.202. "**Process Development Budget**" has the meaning set forth in Section 8.4(c). Each Process Development Budget, upon approval in accordance with the terms and conditions herewith, will be automatically incorporated into this Agreement by reference and form a part of this Agreement.

1.203. "**Process Development Costs**" means the fully-burdened cost incurred on or after the Effective Date by either Party or any of their respective Affiliates in connection with the performance of any Process Development Activities in accordance with this Agreement and consistent with the applicable CMC Development Plan, including all FTE Costs, Out-of-Pocket Costs, and depreciation of capital expenditures.

1.204. "**Product**" means, as applicable, any Clinical IRD Product or Janssen Research IRD Product.

1.205. "**Product Infringement**" has the meaning set forth in Section 11.6.

1.206. "**Product Marks**" has the meaning set forth in Section 11.8.

1.207. "**Qualified Change of Control**" means, with respect to MeiraGTx, a Change of Control in which the acquiring Person, as of the time of such Change of Control, (a) [***] or (b) [***].

1.208. "**Quality Agreement**" means, as applicable, the Clinical Quality Assurance Agreement or the Commercial Quality Assurance Agreement.

1.209. "**Receiving Party**" has the meaning set forth in Section 12.1.

1.210. "**Regulatory Approval**" means, with respect to each product in any country or jurisdiction, the approval of the applicable Regulatory Authority necessary for the marketing and sale of such product in such country or jurisdiction by the relevant Regulatory Authority, excluding separate pricing or reimbursement approvals that may be required, as it may be amended or updated from time to time.

1.211. "**Regulatory Authority**" means any Governmental Authority responsible for granting Regulatory Approvals for Products, including the FDA, EMA, European Commission and any corresponding national or regional regulatory authorities.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.212. "**Regulatory Filings**" means, with respect to any product, any application or submission to a Regulatory Authority of any appropriate regulatory application, and shall include any submission to a regulatory advisory board, MAA, and any supplement or amendment thereto. For the avoidance of doubt, Regulatory Filings shall include any BLA or the corresponding application in any other country or group of countries.

1.213. "**Regulatory Lead Party**" means, on a Clinical IRD Product-by-Clinical IRD Product basis, the Party allocated primary responsibility for all regulatory matters relating to such Clinical IRD Product including all Regulatory Filings and related Regulatory Materials in accordance with Section 7.1(a).

1.214. "**Regulatory Materials**" means any notifications, communication, correspondence, registrations, approvals, or other filings made to, received from or otherwise conducted with a Regulatory Authority related to Developing, Manufacturing, or otherwise Commercializing a biopharmaceutical product in a particular country or jurisdiction, other than Regulatory Filings.

1.215. "**Research**" or "**Researching**" means activities, other than Development, Manufacturing and Commercialization, related to the advance, design, delivery, discovery, generation, identification, optimization, profiling, characterization, production, process development, cell line development, pre-clinical development or non-clinical or pre-clinical studies of drug candidates and products, including such non-clinical studies and other material Development activities to be undertaken to generate data sufficient to enable the filing of an IND.

1.216. "**Research Budget**" has the meaning set forth in Section 3.4. Each Research Budget, upon approval in accordance with the terms and conditions herewith, will be automatically incorporated into this Agreement by reference and form a part of this Agreement.

1.217. "**Research Budget Cap**" has the meaning set forth in Section 3.4.

1.218. "**Research Costs**" has the meaning set forth in Section 3.8.

1.219. "**Research IRD Patents**" has the meaning set forth in Section 11.4(d)(i).

1.220. "**Research IRD Product**" has the meaning set forth in Section 3.2.

1.221. "**Research IRD Target**" means any IRD Gene nominated and approved by the JRC in accordance with Section 3.1.

1.222. "**Research IRD Target Indication**" means, with respect to any Research IRD Target, the inherited retinal disease resulting from the loss of function of such Research IRD Target, as specified in a Research Plan.

1.223. "**Research Plan**" means the strategic plan for Researching a Research IRD Product, as such plan may be agreed to, approved, amended or updated from time to time in accordance with Section 3.2. Each Research Plan, upon approval in accordance with the terms and conditions herewith, will be automatically incorporated into this Agreement by reference and form a part of Exhibit 1.223. In addition, each initial Research Plan will be attached hereto as a sequentially numbered part of Exhibit 1.223 upon approval in accordance with this Agreement (*e.g.*, Exhibit 1.223-1, Exhibit 1.223-2).

1.224. "**Research Plan Activities**" has the meaning set forth in Section 3.2.

1.225. "**Research Plan Term**" means, subject to Section 2.2(b), the Initial Research Plan Term, together with if applicable, the Research Tail Period.

1.226. "**Research Records**" has the meaning set forth in Section 3.5.

1.227. "**Research Results**" mean all tangible Materials, and copies of all material data, results, and research records relating to a Research IRD Product generated in connection with a Research Plan.

1.228. "**Research Tail Notice**" has the meaning set forth in Section 2.2(b).

1.229. "**Research Tail Period**" has the meaning set forth in Section 2.2(b).

1.230. "**Residuals**" means [***] hereunder.

1.231. "**Review Period**" has the meaning set forth in Section 13.4(a).

1.232. "**Right of Reference**" means the "right of reference or use" as that term is defined in 21 C.F.R. § 314.3(b) and any foreign counterpart to such regulation.

1.233. "**Royalty**" or "**Royalties**" has the meaning set forth in Section 10.8.

1.234. "**Royalty Term**" has the meaning set forth in Section 10.9(a).

1.235. "[***] **Product**" means: (a) MeiraGTx's Gene Therapy Product [***] for the treatment of the [***] Target Indication by expressing the [***] Target; [***].

1.236. "[***] **Product Opt-In Date**" has the meaning set forth in Section 2.1(b)(iii).

1.237. "[***] **Target**" means the [***].

1.238. "[***] **Target Indication"** means the inherited retinal disease resulting from the loss of function of the [***] Target.

1.239. "**RPGR Product**" means: (a) MeiraGTx's Gene Therapy Product [***] for the treatment of the RPGR Target Indication by expressing the RPGR Target; [***].

1.240. "**RPGR Target**" means the [***].

1.241. "**RPGR Target Indication**" means the inherited retinal disease resulting from the loss of function of the RPGR Target.

1.242. "**Sales & Royalty Report**" means a written report or reports showing each of: (a) the Net Sales of each Product in the Territory, on a US and ex-US basis, during the reporting period by Janssen and its Affiliates and Sublicensees; and (b) the Royalties payable, in United States Dollars, which shall have accrued hereunder with respect to such Net Sales.

1.243. [***]

1.244. "**Senior Officers**" means, for Janssen, the Therapeutic Area Head for Cardio Vascular and Metabolism or another senior executive designee with responsibilities and seniority comparable thereto, and for MeiraGTx, the Chief Executive Officer of MeiraGTx.

1.245. "**Sensitive Information**" means personally identifiable information, which information may include names, address, other contact information, social security number, date of birth, passwords, protected health information, biometrics, personal identification numbers and codes or other information or data that is protected by Applicable Laws or can be used for identity theft.

1.246. "**Sole Inventions**" has the meaning set forth in Section 11.1(b).

1.247. "**Sponsorship Transfer Date**" means, on a Clinical IRD Product-by-Clinical IRD Product basis and country-by-country basis, the date, [***], on which Janssen shall become the sponsor of the IND for such Clinical IRD Product.

1.248. "**Sublicensee**" means any Person, other than an Affiliate or a Distributor (but not a Deemed Sublicensee Distributor), to which a Party grants a sublicense of any right granted to such Party hereunder in accordance with the terms of this Agreement.

1.249. "**Supply Agreement**" means, as applicable, the Clinical Supply Agreement or the Commercial Supply Agreement.

1.250. "**Target**" means, as applicable, any Clinical IRD Target or Research IRD Target.

1.251. "**Target Indication**" means, with respect to any IRD Gene, the inherited retinal disease resulting from the loss of function of such IRD Gene.

1.252. "**Technology**" means Patents and Know-How.

1.253. "**Term**" has the meaning set forth in Section 15.1.

1.254. "**Terminated Product**" means any Product pursuant to which this Agreement is expired or terminated in accordance with the terms and conditions herein.

1.255. "**Territory**" means all countries and territories of the world.

1.256. "**Third Party**" means any Person other than a Party or an Affiliate of a Party.

1.257. "**Third Party Infringement**" has the meaning set forth in Section 11.5(a).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

1.258. "**Third Party License**" means a written agreement between a Party or its Affiliates and a Third Party to license or acquire Third Party Intellectual Property Rights, including any such agreement entered into as a result of settlement of any claims for infringement of Third Party Intellectual Property Rights.

1.259. "[***] **Clinical Development Budget Period**" has the meaning set forth in Section 6.1(d).

1.260. "[***] **Clinical Development Manufacturing Budget Period**" has the meaning set forth in Section 8.4(b).

1.261. "[***] **Process Development Budget Period**" has the meaning set forth in Section 8.4(c).

1.262. "[***] **Research Budget Period**" has the meaning set forth in Section 3.4.

1.263. "**Trade Control Laws**" mean all applicable statutory and regulatory requirements related to export controls, economic sanctions, trade embargoes, imports of goods and payment of custom duties.

1.264. "**Trademarks**" mean all registered and unregistered trademarks, service marks, trade dress, trade names, logos, insignias, symbols, designs, and all other indicia of ownership, and combinations thereof.

1.265. "[***]" means [***].

1.266. "**United States**" or "**U.S.**" means the United States of America, its territories and possessions.

1.267. "**U.S. Export Control Laws**" means all applicable U.S. laws and regulations relating to the export or re-export of commodities, technologies or services, including the Export Administration Act of 1979, 24 U.S.C. §§ 2401-2420, the International Emergency Economic Powers Act, 50 U.S.C. §§ 1701-1706, the Trading with the Enemy Act, 50 U.S.C. §§ 1 et seq., the Arms Export Control Act, 22 U.S.C. §§ 2778-2779, the International Boycott Provisions of Section 999 of the U.S. Internal Revenue Code of 1986, the U.S. Department of Commerce's Export Administration Regulations, the U.S. Department of State's International Traffic in Arms Regulations, and the economic sanctions programs administered by the U.S. Department of Treasury's Office of Foreign Asset Controls.

1.268. "**Valid Claim**" means: (a) a claim of any issued and unexpired Patent that (i) has not been dedicated to the public, disclaimed, revoked or held unenforceable or invalid by a decision of a Governmental Authority of competent jurisdiction from which no appeal can be taken, or a decision of a Governmental Authority of competent jurisdiction that can be appealed, but with respect to which an appeal has not been taken within the time allowed for appeal, and (ii) has not been disclaimed or admitted to be invalid or unenforceable through reissue, disclaimer or otherwise; or (b) a claim of any pending patent application that (i) has not been cancelled, withdrawn or abandoned, without being re-filed in another application in

the applicable jurisdiction, (ii) has not been finally rejected by an administrative agency or other governmental action from which no appeal can be taken and (iii) has not been pending or filed more than [***] from the earliest possible priority date for such patent application; *provided* that if such claim is later issued, it shall from the issuance date forward be deemed to be a Valid Claim.

1.269. "**Withholding Party**" has the meaning set forth in Section 10.13(d).

2. OVERVIEW OF COLLABORATION

2.1. Overview of Clinical Development Plan Activities.

(a) General Scope. The Parties will jointly Develop Clinical IRD Products through seeking Regulatory Approval for each of the Clinical IRD Products in accordance with the terms and conditions of this Agreement. MeiraGTx will have the primary responsibility to Develop each Clinical IRD Product in accordance with the Clinical Development Plan for each such Clinical IRD Product, including where applicable, conducting any necessary Development in order to submit the applicable Regulatory Filings to Regulatory Authorities. With respect to any Clinical IRD Product, following Regulatory Approval of such Clinical IRD Product in a country, Janssen will be responsible for conducting, at its cost and expense, Commercialization and post-approval Development of such Clinical IRD Product in such country. This Section 2.1(a) is qualified in its entirety by the more detailed provisions of this Agreement set forth below (including Section 2.1(b) and Section 6.1).

(b) [***] Product.

(i) *[***] Meetings*. As of the Execution Date, (A) the [***] Product is deemed not to be a Clinical IRD Product or Research IRD Product under this Agreement, (B) the [***] Target is deemed not to be a Clinical IRD Target or a Research IRD Target under this Agreement and (C) the [***] Target Indication is deemed not to be a Clinical IRD Target Indication under this Agreement. To the extent not prohibited by Applicable Law, Janssen shall have the right to participate in any [***] in each case relating to the [***] Product, and MeiraGTx shall provide the [***] to Janssen. Prior to any [***], MeiraGTx shall also provide Janssen for review and comment at least [***] in advance of any deadline for comments [***] copies [***] by or on behalf of MeiraGTx prior to the relevant submission with respect to the [***] Product to Janssen, and incorporate reasonable comments thereto provided by Janssen.

(ii) *Meeting Between Parties*. Within [***] following the date that MeiraGTx provides to Janssen [***] with respect to the [***] Product, the Parties shall meet to discuss MeiraGTx's plans for Developing the [***] Product. At or prior to such meeting between the Parties, MeiraGTx shall provide to Janssen all information in MeiraGTx's possession or control reasonably necessary for Janssen to evaluate whether to make the [***] Product a Clinical IRD Product hereunder, including MeiraGTx's [***] with respect to the [***] Product, information regarding [***] of the [***] Product, profile and full statistical analyses of the efficacy and safety of the [***] Product [***], MeiraGTx's good faith forecasts of all future Clinical Development Costs of the [***] Product (including a proposed Clinical Development Plan and proposed Clinical Development Budget for the [***] Product as contemplated in Section 6.1(c) and Section 6.1(d)) and [***].

(iii) *Opt-In Process*. Within [***] following the date that the Parties meet to discuss MeiraGTx's plans for Developing the [***] Product pursuant to Section 2.1(b)(ii), Janssen will notify MeiraGTx in writing whether Janssen will make the [***] Product a Clinical IRD Product hereunder. If Janssen notifies MeiraGTx that Janssen elects to make the [***] Product a Clinical IRD Product hereunder (such date, the "[***] **Product Opt-In Date**"), then (A) the [***] Product will automatically become a Clinical IRD Product, (B) the [***] Target will automatically become a Clinical IRD Target, (C) the [***] Target Indication will automatically become a Clinical IRD Target Indication, (D) the Development, Manufacturing and Commercialization terms applicable to Clinical IRD Products shall immediately go into effect in accordance herewith with respect to the [***] Product and (E) Janssen shall [***].

(iv) *Declining to Opt-In*. If Janssen notifies MeiraGTx in writing that Janssen will not elect to make the[***] Product a Clinical IRD Product hereunder, or if Janssen fails to notify MeiraGTx during such [***] period set forth in Section 2.1(b)(iii) that Janssen will elect to make the [***] Product a Clinical IRD Product hereunder, then, as of such date of notice or as of the end of such [***] period, as applicable: (A) the [***] Product will not become a Clinical IRD Product or Research IRD Product under this Agreement, the [***] Target will not become a Clinical IRD Target or Research IRD Target under this Agreement, and the [***] Target Indication will not become a Clinical IRD Target Indication or a Research IRD Target Indication under this Agreement; (B) MeiraGTx will retain its rights with regard to the Research, Development, Manufacture or Commercialization of the [***] Product and [***] Target; (C) MeiraGTx may continue the Research, Development, Manufacture and Commercialization of the [***] Product and [***] Target outside of this Agreement, [***]; (D) Janssen shall have [***]; (E) Janssen shall have no obligation to Develop, Manufacture or Commercialize the [***] Product; (F) Janssen shall have [***]; and (G) each of MeiraGTx and Janssen shall not be subject to the exclusivity obligations set forth in Section 4.6 with respect to the [***] Target.

2.2. Overview of Research Plan Activities.

(a) General Scope. During the applicable Research Plan Term, and in accordance with the terms and conditions of this Agreement, MeiraGTx will conduct Research on each Research IRD Target and design, synthesize, test and optimize Research IRD Products designed to treat the Research IRD Target Indication associated with such Research IRD Target in accordance with the applicable Research Plan and Janssen's feedback, with the aim of achieving IND Submission for each of the Research IRD Products. As part of any Research Plan, upon the Parties' mutual agreement, MeiraGTx may initiate Natural History Studies prior to any IND Submission with respect to the applicable Research IRD Target Indication associated with such Research IRD Target. Each Research IRD Target will be Researched according to a separate Research Plan, and Janssen will have an exclusive Option to select Research IRD Products arising out of any Research Plan Activities to advance such Research IRD Products, at Janssen's sole cost and expense, into Development and Commercialization in accordance with Section 3.9(b). This Section 2.2(a) is qualified in its entirety by the more detailed provisions of this Agreement set forth below (including Article 3).

(b) Research Plan Term. With respect to each Research Plan, MeiraGTx will begin the respective Research Plan Activities on or [***] by the JSC pursuant to Section 3.2. MeiraGTx will continue such Research Plan Activities for [***] thereafter (the "**Initial Research Plan Term**"). Subject to all the other terms and conditions of this Agreement, Janssen may elect, at the end of the Initial Research Plan Term, to continue funding any ongoing and previously approved Research Plan Activities in accordance with the previously approved Research Budget and Section 3.8, until the respective Final Report Delivery Date or such other reasonable date mutually agreed by the Parties, by so notifying MeiraGTx in writing at least [***] before the end of the Initial Research Plan Term (such additional period, the "**Research Tail Period**," and such notice, the "**Research Tail Notice**"). Any changes during the Research Tail Period to the respective ongoing and previously approved Research Plan and Research Plan Activities from the end of the Initial Research Plan Term shall require the Parties' mutual written agreement. If MeiraGTx does not receive the Research Tail Notice from Janssen at least [***] before the end of the Initial Research Plan Term, then the Research Plan Activities shall cease [***] after the date that such Research Plan is first approved by the JSC. Notwithstanding the foregoing, nothing shall obligate MeiraGTx to conduct any further Research Plan Activities as of (a) the Final Report Delivery Date with respect to such Research Plan or (b) such date that this Agreement terminates with respect to such Research Plan.

2.3. Overview of CMC Development Plan Activities and Other Manufacturing Activities.

During the Term, and in accordance with the terms and conditions of this Agreement and the applicable Supply Agreements: (a) MeiraGTx will Manufacture certain Products for Janssen; and (b) [***] Beginning on such date that the Parties finalize a CMC Development Plan and for [***] thereafter ("**CMC Development Term**"), the Parties will collaborate together to Develop the Manufacturing process used for each clinical program undertaken hereunder in connection with each Clinical IRD Product and Research IRD Product. This Section 2.3 is qualified in its entirety by the more detailed provisions of this Agreement set forth below (including Article 8).

3. NON-CLINICAL RESEARCH

3.1. Research IRD Targets.

During the [***] period beginning on the Effective Date (or, if Janssen provides written notice to MeiraGTx at least [***] prior to the expiration of such [***] period of Janssen's election to extend such period by another [***], during the [***] period beginning on the Effective Date), the JRC will nominate and approve IRD Genes (including, at the JRC's discretion, any IRD Gene set forth in Exhibit 1.221) as Research IRD Targets. Subject to Section 3.2, any IRD Gene approved by the JRC as a Research IRD Target will become the subject of a Research Plan. During the applicable Research Plan Term or as otherwise mutually agreed by the Parties, MeiraGTx will conduct Research on each such Research IRD Target, subject to the terms and conditions herein.

3.2. Research Plans.

[***], the Parties will, through the JRC, jointly create a Research Plan for each Research IRD Target. Each Research Plan will set forth for such Research IRD Target: [***] The JRC shall develop and submit each initial Research Plan to the JSC for its review and approval, [***] in accordance herewith, and each initial Research Plan will be automatically attached hereto and form a part of Exhibit 1.223. From time to time, and at least on an annual basis, the JRC will develop, review, and approve amendments or updates to each Research Plan, subject to the terms and conditions of this Agreement. Each such amended or updated Research Plan will be deemed to be automatically replacing the respective Research Plan previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. Each Research Plan shall also be consistent with the terms of this Agreement.

3.3. Conduct of Research Activities.

Following approval of each Research Plan as set forth in Section 3.2, with respect to each Research Plan, MeiraGTx will use Commercially Reasonable Efforts to perform (itself or through its Affiliates or subject to Section 4.4, permitted subcontractors) the Research Plan Activities associated with such Research Plan in accordance with the applicable Research Plan (including the timelines set forth therein) for the duration of the Research Plan Term. In performing its respective Research Plan Activities, MeiraGTx: (a) will conduct such activities in a good scientific manner and in compliance with all Applicable Laws in all material respects, including, where applicable, cGMP, cGLP, cGCP, good pharmacovigilance practices and current international regulatory standards; (b) will not employ or use any Debarred Person; and (c) will not use any MeiraGTx [***] Technology during the course of performing Research Plan Activities or incorporate any MeiraGTx [***] Technology into any Research IRD Product [***].

3.4. Research Budget.

Each Research Plan will be subject to a rolling budget covering Research Costs associated with the anticipated Research Plan Activities under such Research Plan to be performed during the [***] (such [***] period, the "[***] **Research Budget Period**"), in each case broken down by Calendar Quarter and broken out on a line item basis to show Out-of-Pocket Costs (including for Manufacturing related activities) and FTE Costs of FTEs directly engaged to perform each such Research Plan Activity (each, as agreed to, approved, amended or updated from time to time in accordance with this Section 3.4, a "**Research Budget**"). No Research Budget for any Calendar Year shall exceed in any event the applicable Calendar Year cap provided in Exhibit 3.4 attached hereto without the prior written consent of both Parties (such cap, as may be amended by the Parties in writing, the "**Research Budget Cap**"). [***], the JRC shall develop and submit to the JSC for its review and approval, a Research Budget for each Research Plan, and upon the JSC's approval, such Research Budget will be automatically incorporated into this Agreement by reference and form a part of this Agreement. Each Research Budget will be reviewed by the JRC and approved by the JSC (a) [***] based on: (i) the Parties' good faith estimation of the anticipated Research Plan Activities to be conducted during the relevant [***] Research Budget Period; and (ii) information prepared by the Parties in good faith for their own internal planning processes relating to anticipated Research Plan Activities for such Research IRD Product; or (b) whenever the total Research Costs for any given Calendar Quarter are reasonably expected to be at least [***] percent ([***]%) higher than the Research Budget for such Calendar Quarter, whether as a result of any amendments to the Research Plan or increases in costs for the Research Plan Activities

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already planned for such Calendar Quarter. Each such amended or updated Research Budget will automatically replace the respective Research Budget previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. With respect to any Calendar Quarter, in no event will a Party be responsible for any Research Costs that exceed the respective portion of the then-current Research Budget recommended and proposed by the JRC and approved by the JSC, except as otherwise provided in Section 10.2(b) or mutually agreed by the Parties in writing.

3.5. Research Records.

MeiraGTx will prepare and maintain, and cause its Affiliates and their respective employees and permitted subcontractors to prepare and maintain, records, accounts, notes, reports, data and laboratory notebooks with respect to the Research Plan Activities hereunder ("**Research Records**"), in sufficient detail and in a good scientific manner appropriate for scientific, regulatory and intellectual property protection purposes and in compliance with Applicable Law, which Research Records shall: (a) be segregated from other research activities not performed under this Agreement; and (b) be complete and accurate, and fully and accurately reflect all work done, data and developments made, and results achieved in the performance of the Research Plan Activities. MeiraGTx shall retain, and cause its Affiliates and their respective employees and permitted subcontractors to retain, Research Records for at least [***] or such longer period as may be required by Applicable Law. MeiraGTx shall comply with Janssen's data policies set forth on Exhibit 3.5 attached hereto with regard to Research Records.

3.6. Audits.

With respect to any facility or site at which MeiraGTx conducts any Research Plan Activities, Janssen shall have the right, [***] upon reasonable written notice by Janssen, and during normal business hours, to inspect such site and facility of MeiraGTx or to accompany MeiraGTx to inspect any subcontractor site once per year and also for cause, to verify MeiraGTx's compliance with Applicable Law in carrying out its obligations under this Agreement, including those relating to cGMP, cGLP, cGCP and good pharmacovigilance practices. In the event that any such facility or site is found to be non-compliant with cGMP, cGLP, cGCP or good pharmacovigilance practices during such an audit, and such non-compliance relates to or impacts any Research Plan Activities hereunder, MeiraGTx shall submit to Janssen proposed Corrective and Preventative Actions ("**CAPA**") within [***] after Janssen provides notice of such non-compliance. Janssen shall have the right to review and comment on such CAPA, which comments MeiraGTx shall consider in good faith. MeiraGTx shall use Commercially Reasonable Efforts to implement such CAPA [***] after review and comment by Janssen. Except as may otherwise be provided in a Supply Agreement, if any Regulatory Authority or any other Governmental Authority conducts or gives notice of its intent to conduct any audit or inspection at any offices or facilities (including Research facilities) of MeiraGTx or any applicable permitted subcontractor where such audit or inspection relates to any Research IRD Product, then MeiraGTx will [***] notify Janssen and, to the extent such audit or inspection relates to a Research IRD Product and to the extent practicable and not prohibited by Applicable Law, secure for Janssen the right to participate in any such audit or inspection.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

3.7. Research Reports and Materials.

(a) General. MeiraGTx will keep Janssen reasonably informed regarding the status, progress, and results of its Research Plan Activities under each Research Plan, including a review of results (including Manufacturing related campaign reports) and progress against timelines in such Research Plan through regularly scheduled JRC (and, if applicable, Operational Team) meetings. Through the course of such discussions, and notwithstanding anything herein to the contrary, with respect to each Research Plan, the Parties will mutually agree on the lead Research IRD Product to treat the Research IRD Target Indication with respect to any Research IRD Target (and on the plan for pre-IND Manufacturing of such lead Research IRD Product for the respective IND).

(b) Interim Reports. On [***], with respect to each Research Plan, MeiraGTx shall create and submit to the JRC (and, if applicable, the Operational Team) for its review and discussion, a written report (such report, an "**Interim Report**") that includes: (i) a summary of the Research Plan Activities completed during the most recently completed [***] under such Research Plan; (ii) a copy of all results and data generated during such period related to such Research Plan; (iii) progress against the timeline set forth in such Research Plan, with appropriate documentation to substantiate all such activities and results; and (iv) Research Costs incurred in the most recently completed [***] under such Research Plan. MeiraGTx shall also make its employees and consultants available for an in-person or telephonic meeting with Janssen at least [***] every [***] to discuss MeiraGTx's progress with respect to the conduct of Research Plan Activities.

(c) Final Report. MeiraGTx shall provide Janssen with a written Final Report within [***] after the completion or earlier termination of each Research Plan. Janssen shall have [***] following receipt of the Final Report to notify MeiraGTx in writing if Janssen in good faith believes that any information is missing from the Final Report (a "**Missing Information Notice**"), which Missing Information Notice shall identify with reasonable detail such required information that Janssen believes is missing. If Janssen delivers a Missing Information Notice to MeiraGTx, MeiraGTx shall deliver the missing required information identified in such Missing Information Notice as soon as practicable to Janssen, and Janssen will [***] notify MeiraGTx in writing upon Janssen's confirmation that MeiraGTx has provided to Janssen all such missing required information that MeiraGTx must provide (a "**Completion Notice**"). The Final Report will be deemed complete on the date on which MeiraGTx receives the Completion Notice. If Janssen does not deliver a timely Missing Information Notice to MeiraGTx within the [***] period described above in this Section 3.7(c), the initial Final Report will be deemed complete on the date such [***] period lapses. For purposes of this Agreement, "**Final Report Delivery Date**" means the date upon which Janssen has received a complete Final Report in accordance with this Section 3.7(c). During the [***] period following the Final Report Delivery Date, MeiraGTx shall: (i) promptly update the Final Report if any new data relating to any Research IRD Product or Research IRD Target becomes available; and (ii) upon Janssen's reasonable request, provide to Janssen and its representatives reasonable access during normal business hours to MeiraGTx's personnel to discuss such updated Final Report.

3.8. Research Costs.

Subject to Section 10.2, the Parties will be responsible for the documented FTE Costs and Out-of-Pocket Costs, in each case, incurred by or on behalf of MeiraGTx during the Research Plan Term in accordance with the then-current Research Plan, including the cost of any Natural History Studies that are part of such Research Plan (collectively, the "**Research Costs**") as follows: Janssen will be responsible for [***] percent ([***]%) of all Research Costs under each Research Plan, and MeiraGTx will be responsible for [***] percent ([***]%) of all Research Costs under each Research Plan, in each case to the extent that such Research Costs are incurred [***]. All Research Costs will be reconciled and paid in accordance with the procedure described in Section 10.2. Notwithstanding anything herein to the contrary, Janssen will [***].

3.9. Option.

(a) Provision of Information. During the applicable Research Plan Term, Janssen and the JRC and JSC will assess the results provided in each Interim Report provided under a Research Plan, and the JSC will review and determine whether the Advancement Criteria for the respective Research IRD Product have been met. Janssen will determine (based on the Interim Reports or the Final Report for a given Research IRD Product) whether it wishes to further Develop any such Research IRD Product by exercising the Option as set forth in Section 3.9(b).

(b) Exercise of Option. Subject to the terms and conditions of this Agreement, with respect to each Research IRD Target, MeiraGTx hereby grants to Janssen an exclusive (even as to MeiraGTx) option (each, an "**Option**"), exercisable during the applicable Option Period, to obtain, on a Research IRD Target-by-Research IRD Target basis, the exclusive license (as set forth in Section 4.1(b)(ii)) to each Research IRD Product that treats the Research IRD Target Indication resulting from the loss of function of such Research IRD Target. With respect to each Research IRD Target, during the applicable Option Period, Janssen may exercise the applicable Option for such Research IRD Target by delivering a notice of Janssen's exercise of such Option to MeiraGTx (each, an "**Option Exercise Notice**") and paying MeiraGTx [***], which fee shall be non-creditable and non-refundable (each, an "**Option Fee**," and such date of receipt of notice, the "**Option Election Date**"). After receipt of such Option Exercise Notice, MeiraGTx shall submit an invoice to Janssen for such Option Fee, and Janssen shall make such Option Fee payment to MeiraGTx within [***] after Janssen's receipt of such invoice. For clarity, the [***]. As of such Option Election Date, each such Research IRD Product for which Janssen has exercised an Option will become a Janssen Research IRD Product, the exclusive license granted to Janssen in Section 4.1(b)(ii) for such Janssen Research IRD Product will become effective, and Janssen shall assume all responsibility, at Janssen's sole cost and expense, for the further Development, Manufacture, and Commercialization of such Research IRD Product in accordance with this Agreement. In addition, with respect to any Option, and on a Research IRD Target-by-Research IRD Target basis, Janssen may deliver a written notice to MeiraGTx of its intention not to exercise such Option. If with respect to any given Option Janssen fails to provide an Option Exercise Notice before the expiration of the applicable Option Period, or if Janssen provides written notice to MeiraGTx that Janssen does not wish to exercise the applicable Option, then such Option will expire on a Research IRD Target-by-Research IRD Target basis (each, an "**Option Opt-Out**," and each of such dates, an "**Option Opt-Out Date**"),

and: (i) the exclusivity obligations applicable to MeiraGTx under Section 4.6(c) shall expire, and the exclusivity obligations applicable to Janssen under 4.6(b) shall not take effect, in each case with respect to each Research IRD Product that treats the Research IRD Target Indication resulting from the loss of function of such Research IRD Target; (ii) MeiraGTx may freely Develop, Manufacture and Commercialize, or grant to any Third Party rights to Develop, Manufacture and Commercialize, at MeiraGTx's sole cost and expense, each such Research IRD Product without further obligation to Janssen; (iii) the non-exclusive license granted from MeiraGTx to Janssen in Section 4.1(b)(i) will terminate with respect to the Research Plan Activities applicable to each such Research IRD Product; (iv) Janssen shall have [***] except as otherwise expressly provided herein; (v) Janssen shall have no obligation to Develop or Commercialize any such Research IRD Product; and (vi) Janssen shall have [***].

4. LICENSES

4.1. Licenses to Janssen.

(a) Clinical IRD Products. Subject to the terms and conditions of this Agreement and each Supply Agreement, MeiraGTx hereby grants, on behalf of itself and its Affiliates, to Janssen an exclusive (even as to MeiraGTx), royalty-bearing, sublicensable (through multiple tiers, solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license under the MeiraGTx Technology and MeiraGTx's interest in the Joint Technology during the Term, to Develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, and otherwise exploit, Manufacture and Commercialize Clinical IRD Products in the Field in the Territory. For clarity, Janssen may not exercise its rights to make, have made (except by MeiraGTx or its Affiliates or permitted CMOs) or Manufacture Clinical IRD Products in the Field in the Territory except as expressly permitted in a Supply Agreement.

(b) Research IRD Products.

(i) *Non-Exclusive License to Perform Obligations*. Subject to the terms and conditions of this Agreement, MeiraGTx hereby grants, on behalf of itself and its Affiliates, to Janssen a non-exclusive, sublicensable (through multiple tiers, solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license under the MeiraGTx Research Technology and MeiraGTx's interest in the Joint Technology during the Term, for purposes of Janssen conducting its obligations under this Agreement, including any Research Plan Activities assigned to Janssen in the respective Research Plan.

(ii) *Exclusive License Following Option Exercise*. Subject to the terms and conditions of this Agreement and each Supply Agreement, with respect to each Janssen Research IRD Product, MeiraGTx hereby grants, on behalf of itself and its Affiliates, to Janssen an exclusive (even as to MeiraGTx), royalty-bearing, sublicensable (through multiple tiers, solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license, under the MeiraGTx Research Technology and MeiraGTx's interest in the Joint Technology during the Term, to Research, Develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, and otherwise exploit, Manufacture and Commercialize such Janssen Research IRD Product in the Field in the Territory. For clarity, Janssen may not exercise its rights to make, have made (except by MeiraGTx or its Affiliates or permitted CMOs) or Manufacture Janssen Research IRD Products in the Field in the Territory except as expressly permitted in a Supply Agreement.

(c) Internal Research License. Subject to the terms and conditions of this Agreement, MeiraGTx hereby grants, on behalf of itself and its Affiliates, to Janssen a non-exclusive, royalty-free, sublicensable (but only to its Affiliates) license, under the MeiraGTx Technology, MeiraGTx Research Technology and MeiraGTx's interest in the Joint Technology to conduct Internal Research in the Field in the Territory. The license granted under this Section 4.1(c) shall be perpetual and survive any expiration of this Agreement and any termination of this Agreement by Janssen under Section 15.2(a)(i) for MeiraGTx's uncured material breach or under Section 15.2(b) for a MeiraGTx Insolvency Event. In addition, upon an Option Opt-Out for a Research IRD Product pursuant to Section 3.9(b), the license granted under this Section 4.1(c) shall be perpetual and survive with respect to such Research IRD Product, except as otherwise expressly provided in this Section 4.1(c). [***]

4.2. Licenses to MeiraGTx.

(a) Clinical IRD Products.

(i) *License Back for Performance of Obligations and Manufacture.* Subject to the terms and conditions of this Agreement, with respect to each Clinical IRD Product, Janssen hereby grants, on behalf of itself and its Affiliates, to MeiraGTx a non-exclusive, royalty-free, sublicensable (solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license, under the MeiraGTx Technology and both Parties' interest in the Joint Technology to: (A) perform MeiraGTx's obligations under this Agreement with respect to such Clinical IRD Product; and (B) make, have made and otherwise Manufacture such Clinical IRD Product in accordance with the terms herewith. (ii) *Non-exclusive License to Janssen Technology.* Subject to the terms and conditions of this Agreement, with respect to each Clinical IRD Product, Janssen hereby grants, on behalf of itself and its Affiliates, to MeiraGTx, a non-exclusive, royalty-free, sublicensable (solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license under the Janssen Technology during the Term, for purposes of MeiraGTx conducting its obligations under this Agreement, including any Clinical Development Plan Activities assigned to MeiraGTx in the respective Clinical Development Plan or the Manufacture of any Clinical IRD Products in accordance with the terms herewith.

(b) Research IRD Products.

(i) *Non-Exclusive License Back for Manufacture.* Subject to the terms and conditions of this Agreement, with respect to each Janssen Research IRD Product, Janssen hereby grants, on behalf of itself and its Affiliates, to MeiraGTx a non-exclusive, royalty-free, sublicensable (solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license, under the MeiraGTx Research Technology and Janssen's interest in the Joint Technology to make, have made and otherwise Manufacture such Janssen Research IRD Product in accordance with the terms herewith.

(ii) *Non-Exclusive License to Janssen Technology.* Subject to the terms and conditions of this Agreement, with respect to each Research IRD Product (including any Janssen Research IRD Product), Janssen hereby grants, on behalf of itself and its Affiliates, to MeiraGTx, a non-exclusive, royalty-free, sublicensable (solely as provided in Section 4.3), transferable (in accordance with Section 19.1) license under the Janssen Technology during the Term, for purposes of MeiraGTx conducting its obligations under this Agreement, including any Research Plan Activities assigned to MeiraGTx in the respective Research Plan or the Manufacture of any Research IRD Products in accordance with the terms herewith.

4.3. Sublicense Rights.

Janssen may sublicense through multiple tiers the rights granted to it by MeiraGTx under Section 4.1(a) and Section 4.1(b) at any time [***]; *provided* that: (a) each such sublicense shall be consistent with the terms and conditions of this Agreement (including Section 4.1 and Article 12) and shall be subject to any Third Party License requirements with respect to any Technology in-licensed by MeiraGTx and (b) each sublicense to a Third Party collaborator shall be in writing and shall contain terms prohibiting the Third Party collaborator from using any MeiraGTx Confidential Information, MeiraGTx Know-How or MeiraGTx Research Know-How for any purpose inconsistent with this Agreement. In the case of any sublicense by Janssen, Janssen shall remain primarily responsible for, and directly liable to MeiraGTx for, the payment of all royalties payable based on Net Sales of Product by any Janssen Sublicensee and the payment of all Development Milestone Payments and Commercial Milestone Payments even if a Sublicensee is responsible for the achievement of any such milestone with respect to any Product. Either Party may exercise its rights and perform its rights and obligations under this Agreement itself or through any of its Affiliates. Except for MeiraGTx sublicensing to an Affiliate or as permitted under Section 4.4, MeiraGTx may not sublicense the rights granted to it by Janssen under this Agreement without first obtaining, in each case, Janssen's prior written consent (which shall not be unreasonably withheld, delayed, or conditioned), and any such sublicense must be consistent with the terms of this Agreement (including Section 4.2 and Article 12).

4.4. Subcontractors.

Each Party may engage subcontractors to perform any obligations assigned to it under this Agreement, except that: (a) [***]; (b) prior to [***]; (c) prior to [***]; (d) the subcontracting Party shall remain fully responsible for the work allocated to, and payment to, such subcontractors to the same extent it would if it had done such work itself; (e) each contract between a Party and a subcontractor shall be consistent with the provisions of this Agreement, including (i) obligations of confidentiality and non-use applicable to Confidential Information that are at least as stringent as those set forth in Article 12 and (ii) obligations of assignment of all Inventions and other Intellectual Property Rights developed in the course of performing any such work under this Agreement to the subcontracting Party and obligations of cooperation to execute any documents to confirm or perfect such assignment; and (f) the subcontracting Party shall remain at all times fully liable for all acts or omissions of such subcontractor. Any reference to a Party's performance hereunder shall include references to any performance by its subcontractors permitted in accordance with this Section 4.4.

4.5. Third Party Licenses.

All rights licensed to a Party from a Third Party and sublicensed to the other Party under this Agreement will be subject to and subordinate to the terms of the applicable Third Party License to the extent such terms apply to a sublicensee of such Third Party Intellectual Property Rights, except that no Party shall be obligated to comply with any such Third Party License until such Third Party License has been disclosed to such Party.

4.6. Exclusivity.

(a) Janssen Exclusivity for Clinical IRD Targets. On a Clinical IRD Target-by-Clinical IRD Target basis, beginning on (i) the Effective Date with respect to the CNGA3 Target, CNGB3 Target and RPGR Target and (ii) the [***] Product Opt-In Date with respect to the [***] Target, and ending on the earlier of (A) [***] of the Effective Date or (B) the expiration or termination of this Agreement with respect to the Clinical IRD Product associated with such Clinical IRD Target, [***] other than Researching, Developing, Manufacturing, and Commercializing Clinical IRD Products in accordance with the terms and conditions of this Agreement. In addition, on a Clinical IRD Product-by-Clinical IRD Product basis, beginning on [***] and for [***] thereafter, [***]

(b) Janssen Exclusivity for Research IRD Targets. On a Research IRD Target-by-Research IRD Target basis, beginning on the Option Election Date associated with the Janssen Research IRD Product intended to treat the Research IRD Target Indication resulting from the loss of function of such Research IRD Target and ending on the earlier of (i) the [***] of the Option Election Date associated with the Janssen Research IRD Product intended to treat such Research IRD Target Indication resulting from the loss of function of such Research IRD Target or (ii) the expiration or termination of this Agreement with respect to the Janssen Research IRD Product intended to treat such Research IRD Target Indication resulting from the loss of function of such Research IRD Target, [***] other than Researching, Developing, Manufacturing, and Commercializing Janssen Research IRD Products in accordance with the terms and conditions of this Agreement.

(c) MeiraGTx Exclusivity Obligations. Beginning on the Effective Date, [***] other than Researching, Developing, Manufacturing and Commercializing Gene Therapy Products in accordance with the terms and conditions of this Agreement. The prohibition contained in the immediately preceding sentence shall end:

[***]

provided, *however*, that if both subsections (ii) and (iii) above apply to any one particular case, the longer period described in subsection (ii) or (iii) shall apply thereto.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(d) Mergers and Acquisitions Involving Competing Products. Subject to Section 4.6(e), in the event that a Third Party merges with a Party or becomes an Affiliate of a Party or assets of a Third Party are combined with a Party or an Affiliate of a Party ("**Acquired Party**") during the Term through merger, acquisition, consolidation or other similar transaction, and as of the closing date of such merger, acquisition, consolidation or other similar transaction, such Third Party is engaged in the Research, Development, Manufacture or Commercialization of a Competing Product, then, unless the Parties mutually agree otherwise, the Acquired Party or its new Affiliate (as applicable) shall have [***] from the closing date of such transaction to wind down or complete the Divestiture of such Competing Product, and its new Affiliate's conduct of the Research, Development, Manufacture and Commercialization of such Competing Product during such [***] period shall not be deemed a breach of the Acquired Party's exclusivity obligations set forth in this Section 4.6; *provided* that such new Affiliate conducts the Research, Development, Manufacture and Commercialization of such Competing Product during such [***] period independently of the activities of this Agreement and does not use any of the other Party's Intellectual Property Rights or Confidential Information (except as may be separately licensed by such other Party to such new Affiliate) in such conduct. "**Divestiture**", as used in this Section 4.6(d), means the sale or transfer of rights to the Research, Development, Manufacture and Commercialization of a Competing Product to a Third Party without receiving a continuing share of profit, royalty payment or other economic interest in the success of such Competing Product.

(e) Qualified Change of Control. In the event of a Qualified Change of Control involving MeiraGTx, Janssen shall have the right to terminate [***], by providing MeiraGTx with written notice at least [***] after the announcement of such Qualified Change of Control, with such termination to be effective within [***] of such notice; *provided* that all other obligations under this Agreement, including under Section 4.1 and Article 10, shall remain in full force and effect. For clarity, upon Janssen's termination notice to MeiraGTx in accordance with the preceding sentence, (i) all Committees shall be disbanded and Janssen shall have sole and exclusive control with respect to any activities previously conducted pursuant to this Agreement and (ii) Janssen shall not have any further obligations to share any information, updates or reports with MeiraGTx with respect to any activities previously conducted pursuant to this Agreement, *except* with respect to Section 10.6, Section 10.7, Section 10.8, Section 10.9, Section 10.11 (including Sales & Royalty Reports) and any other applicable payment obligations. In the event that Janssen elects to not exercise its rights under this Section 4.6(e), the terms of Section 4.6(d) shall apply.

4.7. No Other Rights.

Each Party expressly reserves and retains all Patents, Know-How, and other intellectual property rights not expressly granted herein, and no right or license under any Intellectual Property Rights of either Party is granted or shall be granted by implication, estoppel or otherwise. Each licensee Party covenants to the licensor Party that the licensee Party shall not (and shall require that any of its Affiliates or Sublicensees shall not) use any of the licensor Party's Intellectual Property Rights licensed under this Agreement for any purpose other than as expressly provided under this Agreement for such licensee Party.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

4.8. MeiraGTx Know-How Transfer.

(a) Clinical IRD Products. Within [***] following the Effective Date, MeiraGTx will deliver to Janssen all copies of: (i) MeiraGTx Know-How reasonably necessary or useful for the Development or Commercialization of the Clinical IRD Products; and (ii) documents and files related to the MeiraGTx Patents Covering the Clinical IRD Products. Thereafter, on a [***] basis during the Term or as otherwise mutually agreed by the Parties, MeiraGTx shall [***] disclose to Janssen (through the JSC, JDC, or JRC, as applicable) any additional MeiraGTx Know-How reasonably necessary or useful for the Development or Commercialization of any Clinical IRD Product that came into existence after the prior disclosure, will provide reasonable assistance to Janssen in connection with understanding and using all such MeiraGTx Know-How for purposes consistent with the licenses and rights granted to Janssen under Section 4.1 and will provide any new documents or files related to the MeiraGTx Patents Covering the Clinical IRD Products that came into MeiraGTx's possession or control after the prior disclosure. All costs of the transfer set forth in this Section 4.8(a) shall be deemed Clinical Development Costs hereunder and paid in accordance with the terms and conditions set forth in Section 6.1(f).

(b) Research IRD Products. With respect to any Janssen Research IRD Product, within [***] after Janssen's exercise of the respective Option, MeiraGTx will deliver to Janssen all copies of: (i) MeiraGTx Research Know-How reasonably necessary or useful for the Development or Commercialization of the applicable Janssen Research IRD Product; and (ii) documents and files related to the MeiraGTx Research Patents Covering the Janssen Research IRD Product. Thereafter, on a [***] basis during the Term or as otherwise mutually agreed by the Parties, MeiraGTx shall [***] disclose to Janssen (through the JSC, JDC, or JRC, as applicable) any additional MeiraGTx Research Know-How reasonably necessary or useful for the Development or Commercialization of any Janssen Research IRD Product that came into existence after the prior disclosure, will provide reasonable assistance to Janssen in connection with understanding and using all such MeiraGTx Research Know-How for purposes consistent with the licenses and rights granted to Janssen under Section 4.1 and will [***]. All costs of the transfer set forth in this Section 4.8(b) shall be [***].

5. GOVERNANCE

5.1. Alliance Managers. Within [***] following the Effective Date, each Party shall designate an individual employee to facilitate communication and coordination of the Parties' activities under this Agreement and to provide support and guidance to the JSC (each, an "**Alliance Manager**"). Each Alliance Manager may not serve as a representative of its respective Party on any Committee.

5.2. Joint Steering Committee.

(a) Purpose; Formation. Within [***] following the Effective Date, the Parties shall establish a joint steering committee (the "**JSC**" or "**Joint Steering Committee**"). The JSC shall monitor, make certain decisions, and provide strategic oversight of the activities under this Agreement and facilitate communications between the Parties with respect to the Research, Development, Manufacturing, and Commercialization of the Products.

(b) Specific Responsibilities. In addition to providing general oversight of all activities hereunder, the JSC shall in particular have the following responsibilities, in each case to the extent the applicable decisions are not made at the level of another Committee: (i) reviewing and approving each initial Clinical Development Plan; (ii) reviewing and approving the overall regulatory strategy for the Clinical IRD Products; (iii) approving all material Regulatory Filings and Regulatory Materials as necessary, subject to Section 7.1(c); (iv) reviewing and approving each initial Research Plan for a Research IRD Target; (v) reviewing and approving each initial CMC Development Plan; (vi) approving, upon recommendation and proposal by the applicable Committee, Research Budgets, Clinical Development Budgets, Clinical Development Manufacturing Budgets and Process Development Budgets as provided herein (*provided* that no Research Budget for a Calendar Year shall exceed the respective Research Budget Cap for such Calendar Year); (vii) facilitating the flow of information with respect to the Development and Commercialization of the Products; (viii) receiving and discussing reports from the other Committees; (ix) providing guidance to the other Committees on all significant strategic issues that fall within the scope of such Committees; (x) establishing such additional joint subcommittees as it deems necessary to achieve the objectives and intent of this Agreement; (xi)facilitating the prosecution and maintenance with respect to Patents pursuant to Section 11.4 and resolving disputes with respect to such Patents; (xii) resolving disputes for which it is responsible as provided in this Agreement; and (xiii) performing such other functions as expressly provided in this Agreement.

5.3. Joint Research Committee.

(a) Purpose; Formation. Within [***] following the Effective Date, the Parties shall establish a committee to oversee the Research Plan Activities and otherwise facilitate the flow of information between the Parties with respect to, and provide a forum to discuss, Research Plan Activities (the "**JRC**" or "**Joint Research Committee**").

(b) Specific Responsibilities. The JRC shall be responsible for: (i) nominating and approving IRD Genes to become Research IRD Targets; (ii) discussing, preparing, and recommending for submission to the JSC for approval, each initial Research Plan and discussing and approving as applicable amendments thereto; (iii) discussing, preparing, and recommending for submission to the JSC for approval, each Research Budget; (iv) overseeing and directing the Research Plan Activities, including Natural History Studies; (v) reviewing and discussing all reports describing the Research Plan Activities and the Research Results (including Interim Reports and Final Reports); (vi) performing such other functions as may be delegated to it by the JSC; and (vii) performing such other functions expressly delegated to it in this Agreement.

5.4. Joint Development Committee.

(a) Purpose; Formation. Within [***] following the Effective Date, the Parties shall establish a committee to oversee the Clinical Development Plan Activities of the Parties with respect to each Clinical IRD Product and otherwise facilitate the flow of information between the Parties with respect to, and provide a forum to discuss, the Development of Products (the "**JDC**" or "**Joint Development Committee**").

(b) Specific Responsibilities. The JDC shall in particular have the following responsibilities: (i) reviewing, amending, and recommending for approval by the JSC, each initial Clinical Development Plan and discussing and approving as applicable amendments thereto; (ii) discussing, preparing, and recommending for submission to the JSC for approval, each Clinical Development Budget; (iii) reviewing and monitoring the Parties' Clinical Development Plan Activities and progress against each Clinical Development Plan, including facilitating discussions between the Parties regarding the Development of each Clinical IRD Product; (iv) reviewing and recommending to the JSC for approval all material Regulatory Filings and all Regulatory Materials as necessary or as requested by the JSC, subject to Section 7.1(c); (v) discussing and recommending to the JSC for approval the overall regulatory strategy for each Clinical IRD Product; (vi) discussing the Development reports; (vii) determining the timing of the transition between the Parties with respect to regulatory matters pursuant to Section 7.1(a)(iii); (viii) reviewing and monitoring the Parties' Development activities with respect to safety matters; (ix) developing and reviewing the overall publication strategy for the Parties in connection with this Agreement, subject to Section 13.4; (x) performing such other functions as may be delegated to it by the JSC; and (xi) performing such other functions expressly delegated to it in this Agreement.

5.5. Joint Manufacturing Committee.

(a) Purpose; Formation. Within [***] following the Effective Date, the Parties shall establish a committee to oversee and coordinate the Manufacturing activities of the Parties with respect to each Clinical IRD Product or Research IRD Product and otherwise facilitate the flow of information between the Parties with respect to, and provide a forum to discuss, the Manufacturing of Products and the CMC Development Collaboration (the "**JMC**" or "**Joint Manufacturing Committee**").

(b) Specific Responsibilities. The JMC shall in particular have the following responsibilities: (i) reviewing, amending, and recommending for approval by the JSC initial Manufacturing plans, initial CMC Development Plans and discussing and approving as applicable amendments thereto, including with respect to capacity; (ii) discussing, preparing, and recommending for submission to the JSC for approval each Clinical Development Manufacturing Budget and Process Development Budget; (iii) overseeing the Manufacturing of Products used in Development activities, including discussing any potential supply issues, interruptions, the outcome of any Regulatory Authority inspection of Manufacturing facilities used by or on behalf of MeiraGTx, and any remedial actions required, if applicable, as a result of such inspection; (iv) overseeing the CMC Development Plan Activities for all Clinical IRD Products, Research IRD Products, and Janssen Research IRD Products; (v) collaborating on the creation of the Manufacturing-related Regulatory Filings and all Regulatory Materials for any Clinical IRD Product and any Research IRD Product being Manufactured by or on behalf of MeiraGTx; (vi) overseeing the Know-How transfer of Manufacturing technology to Janssen in accordance with any Supply Agreement; (vii) reviewing and approving specifications for Clinical IRD Products and Research IRD Products; (viii) performing such other functions as may be delegated to it by the JSC; and (ix) performing such other functions expressly delegated to it in this Agreement.

5.6. Operational Teams.

From time-to-time, each of the JSC, JRC, JDC or JMC may establish and delegate specific matters or duties within its responsibilities to directed teams (each, an "**Operational Team**"), the composition, operation, and responsibilities of which will be determined by the applicable establishing Committee. Operational Teams may be established on an *ad hoc* basis for purposes of a specific activity or on such other basis as the applicable establishing Committee may determine. Each Operational Team will report to, and its activities will be subject to the oversight of, the applicable establishing Committee and the JSC, and no Operational Team's authority may exceed that specified for the applicable establishing Committee. Any disagreement between the representatives of the Parties on any Operational Teams will be referred to the applicable establishing Committee for resolution in accordance with Section 5.8.

5.7. Committee Representatives and Meetings.

(a) Committee Representatives. Each Party shall initially appoint [***], and no more than [***] representatives to each Committee, with the exception of the JSC, which shall have [***] representatives from each Party. Each Committee representative shall have appropriate knowledge and expertise within the applicable Party to make decisions or recommendations arising within the scope of the applicable Committee's responsibilities. Each Party may replace its representatives on any Committee upon written notice to the other Party. Each Party shall appoint [***] of its representatives on each Committee to act as a co-chairperson of such Committee. The responsibility for running each meeting of each Committee shall alternate between the co-chairpersons of such Committee from meeting-to-meeting, with Janssen's co-chairperson running the first meeting of each Committee. The co-chairpersons of each Committee shall jointly prepare and circulate agendas to such Committee's representatives before each such Committee meeting and shall direct the preparation of reasonably detailed documentation for each such Committee meeting, which shall be approved by the Committee's co-chairpersons and circulated to Committee representatives within [***] of such meeting. Unless mutually agreed upon by the Parties, any member of one Committee shall not be a member of another Committee under this Agreement, and Alliance Managers and Senior Officers may not serve on any Committee.

(b) Non-Committee Representatives. Each Party may [***] invite [***], in addition to its representatives, to attend Committee meetings in a non-voting capacity; *provided* that if either Party intends to have any Third Party attend such a meeting, such Party shall obtain the other Party's prior written consent for such Third Party to attend such meeting, which consent shall not be unreasonably withheld, conditioned, or delayed; and *provided further* that each Party's co-chairperson of the JMC may attend any JDC meeting in a non-voting capacity and each Party's co-chairperson of the JDC may attend any JMC meeting in a non-voting capacity, in each case without the consent of the other Party. Each Party shall be responsible for ensuring that each Committee member that it appoints and each Third Party that it invites to a Committee meeting complies with the confidentiality and non-use obligations set forth in this Agreement.

(c) Meetings. Each Committee shall hold meetings at such times as it elects to do so, but at least [***] in the case of the JRC, the JDC and the JMC, and at least [***] in the case of the JSC; *provided* that the JSC shall hold its first meeting no later than [***] from the Effective Date. Meetings of any Committee may be held in person or by audio or video teleconference; *provided* that unless otherwise agreed by the Parties, at least [***] meeting per [***] for each Committee shall be held in person. Notwithstanding Section 5.7(a), upon the Parties' mutual agreement, any meeting of a Committee may be combined with any meeting of another Committee. Each Party shall be responsible for all of its own costs and expenses of participating in any Committee meetings. No action taken at any meeting of a Committee shall be effective unless at least [***] Committee representative of each Party is participating in such meeting.

(d) Dissolution. Each Committee will continue to exist until the earlier of completion of such Committee's obligations under this Agreement or mutual agreement of the Parties to disband such Committee; *provided* that following the dissolution of the JSC, the JSC may, upon the Parties' agreement, continue to meet on a [***] basis (or more or less frequently, if mutually agreed by the Parties) solely to serve as a forum for sharing and discussing information.

5.8. Resolution of Committee Disputes.

(a) Votes. All decisions of each Committee shall be made by [***] vote, with each Party's representatives on the respective Committee collectively having [***] vote.

(b) Disputes. If, after reasonable discussion and good-faith consideration of each Party's view on a particular matter before any Committee other than the JSC and within the scope of its authority, the representatives of the Parties on such Committee cannot reach an agreement as to such matter within [***] after such matter was brought to such Committee for resolution, such disagreement shall be referred to the JSC for resolution. If, after reasonable discussion and good-faith consideration of each Party's view on a particular matter before the JSC and within the scope of its authority, the representatives of the Parties on the JSC cannot reach an agreement as to such matter within [***] after such matter was brought to the JSC for resolution or after such matter has been referred to the JSC from another Committee, such disagreement shall be referred to the Senior Officers for resolution.

(c) Final Decision-Making Authority. If the Senior Officers cannot in good faith resolve a particular matter within [***] after such matter has been referred to them, then: [***]

(d) No Other Powers. Notwithstanding anything herein to the contrary, each Committee shall have only the powers assigned expressly to it in this Article 5 and elsewhere in this Agreement, and no Committee shall have any power to amend, modify or waive compliance with this Agreement, or to impose additional obligations on a Party beyond those provided in this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

6. DEVELOPMENT

6.1. Clinical IRD Products.

(a) Clinical IRD Products. Subject to the terms and conditions of this Agreement (including Section 2.1(b)), the Parties will collaborate with one another to Develop the Clinical IRD Products.

(b) Responsibility. Subject to the oversight of the JSC and JDC, the Parties will jointly collaborate with one another on the Development of each Clinical IRD Product in accordance with this Agreement and the applicable Clinical Development Plan for such Clinical IRD Product, including conducting any necessary Research to support BLA approval for such Clinical IRD Product.

(c) Clinical Development Plans. The anticipated costs and timelines for activities under the Clinical Development Plans for the CNGA3 Product, the CNGB3 Product and the RPGR Product are attached hereto as parts of Exhibit 1.34. [***] with respect to the CNGA3 Product, CNGB3 Product and RPGR Product, the JDC shall develop and submit to the JSC for its review and approval, a Clinical Development Plan for each such Clinical IRD Product, and upon the JSC's approval, each such initial Clinical Development Plan will be attached hereto and form a part of Exhibit 1.34. If Janssen elects to make the [***] Product a Clinical IRD Product in accordance with Section 2.1(b), no later than [***] following the [***] Product Opt-In Date, the JDC shall develop and submit to the JSC for its review and approval, a Clinical Development Plan for the [***] Product, and upon the JSC's approval, such initial Clinical Development Plan shall be attached hereto and form a part of Exhibit 1.34. In the event that any Clinical Development Plan for a Clinical IRD Product conflicts with the anticipated costs and timelines set forth in Exhibit 1.34 for such Clinical IRD Product, the Clinical Development Plan shall control. Each Clinical Development Plan will set forth in reasonable detail at all times (i) all activities that are necessary or useful to be undertaken to achieve and maintain Regulatory Approval in the relevant Major Market Country for such Clinical IRD Product (the "**Clinical Development Plan Activities**"), (ii) an estimated timeline for completing such activities and the respective Clinical Development Plan Term, (iii) the respective deliverables for such activities and (iv) the allocation of responsibilities between the Parties for performance of each such activity. The terms of, and Clinical Development Plan Activities set forth in, each Clinical Development Plan will at all times be designed to expedite Regulatory Approval, reimbursement and Commercialization of the Clinical IRD Product that is the subject of such Clinical Development Plan, and to be in compliance with all Applicable Laws and in accordance with professional and ethical standards customary in the biopharmaceutical industry. From time to time, and at least on an annual basis, the JDC will develop, review and approve amendments or updates to each Clinical Development Plan for a Clinical IRD Product, subject to the remaining terms and conditions of this Agreement. Each such amended or updated Clinical Development Plan will automatically replace the respective Clinical Development Plan previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. Each Clinical Development Plan shall also be consistent with the terms of this Agreement.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(d) Clinical Development Budgets. Each Clinical Development Plan, when approved by the Parties in accordance with Section 6.1(c), will be subject to a rolling budget covering Clinical Development Costs associated with the anticipated Clinical Development Plan Activities for such Clinical IRD Product to be performed during the [***] (such [***] period, the "[***] **Clinical Development Budget Period**"), in each case broken down by Calendar Quarter and broken out on a line item basis to show Out-of-Pocket Costs and FTE Costs of FTEs directly engaged to perform each such Development activity including for each Development activity with respect to each Clinical Study conducted thereunder (each, as agreed to, approved, amended or updated from time to time in accordance with this Section 6.1(d), a "**Clinical Development Budget**"). [***] with respect to the CNGA3 Product, CNGB3 Product and RPGR Product, and [***] in the event that Janssen elects to make the [***] Product a Clinical IRD Product in accordance with Section 2.1(b), the JDC shall develop and submit to the JSC for its review and approval, a Clinical Development Budget for the respective Clinical IRD Product, and upon the JSC's approval, each such Clinical Development Budget will be automatically incorporated into this Agreement by reference and form a part of this Agreement. Each Clinical Development Budget will be reviewed by the JDC and approved by the JSC (i) at least [***] (and [***] of the applicable [***] Clinical Development Budget Period) based on: (A) the Parties' good faith estimation of the anticipated Clinical Development Plan Activities to be conducted during the relevant [***] Clinical Development Budget Period; and (B) information prepared by the Parties in good faith for their own internal planning processes relating to anticipated Clinical Development Plan Activities for such Clinical IRD Product; or (ii) whenever the total Clinical Development Costs for any given Calendar Quarter are reasonably expected to be at least [***] percent ([***]%) higher than the Clinical Development Budget for such Calendar Quarter, whether as a result of any amendments to the Clinical Development Plan or increases in costs for the Clinical Development Plan Activities already planned for such Calendar Quarter (*provided*, *however*, that following the beginning of a Calendar Year, the JSC may not increase the Clinical Development Budget for such Calendar Year by more than [***] percent ([***]%) without the Parties' prior written approval). Each such amended or updated Clinical Development Budget will automatically replace the respective Clinical Development Budget previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. With respect to any Calendar Quarter, in no event will a Party be responsible for any Clinical Development Costs that exceed the respective portion of the then-current Clinical Development Budget recommended and proposed by the JDC and approved by the JSC, except as otherwise provided in Section 10.3(b).

(e) Conduct of Clinical Development Activities. On a Clinical IRD Product-by-Clinical IRD Product basis, Janssen and MeiraGTx will each use Commercially Reasonable Efforts to perform their respective Clinical Development Plan Activities in accordance with each Clinical Development Plan (including the timelines set forth therein). In performing its respective Clinical Development Plan Activities, each Party: (i) will conduct such activities in good scientific manner and in compliance with all Applicable Laws in all material respects, including, where applicable, those relating to cGMP, cGLP, cGCP, good pharmacovigilance practices and requirements for protection of human subjects; (ii) will not employ or use any Debarred Person; and (iii) in the case of MeiraGTx, will not use any MeiraGTx [***] Technology during the course of performing any Clinical Development Plan Activities or incorporate any MeiraGTx [***] Technology into any Clinical IRD Product unless otherwise mutually agreed to by the Parties.

(f) Clinical Development Costs. Janssen will be responsible for one hundred percent (100%) of all Clinical Development Costs for each Clinical IRD Product; *provided, however*, that in no event will any Clinical Development Costs for any Calendar Quarter exceed the applicable Clinical Development Budget for such Calendar Quarter, except as otherwise provided in Section 10.3(b). Notwithstanding anything herein to the contrary, in no event [***].

(g) Clinical Development Records. Each Party will prepare and maintain, and cause its Affiliates and their respective employees and permitted subcontractors to prepare and maintain, records, accounts, notes, reports and data with respect to the Clinical Development Plan Activities hereunder ("**Clinical Development Records**"), in sufficient detail and in a good scientific manner appropriate for scientific, regulatory, and intellectual property protection purposes and in compliance with Applicable Law and such Party's standard practices (including cGLP with respect to activities that require cGLP compliance to be submitted in Regulatory Filings including INDs and BLAs), which Clinical Development Records will: (i) be segregated from other development activities not performed under this Agreement; and (ii) be complete and accurate, and fully and accurately reflect all work done, data and developments made, and results achieved in the performance of the Clinical Development Plan Activities. Each Party shall retain, and cause its Affiliates and their respective and permitted subcontractors to retain, Clinical Development Records for at least [***] or such longer period as may be required by Applicable Law. Each Party shall comply with Janssen's data policies set forth on Exhibit 3.5 attached hereto with regard to Clinical Development Records.

(h) Audits. With respect to any facility or site at which a Party conducts any Clinical Development Plan Activities, and subject to the terms of any agreement between such Party and any applicable permitted subcontractor with respect to any facility or site of such subcontractor, the other Party shall have the right, at its own expense, upon reasonable written notice by such other Party, and during normal business hours, to inspect such site and facility of such Party or to accompany such Party to inspect any subcontractor site once per year and also for cause, to verify such Party's compliance with Applicable Law in carrying out its obligations under this Agreement, including those relating to cGMP, cGLP, cGCP, good pharmacovigilance practices and requirements for protection of human subjects. In the event that any such facility or site is found to be non-compliant with cGMP, cGLP, cGCP, good pharmacovigilance practices and requirements for protection of human subjects during such an audit, and such non-compliance relates to or impacts any Clinical Development Plan Activities hereunder, the audited Party shall submit to the auditing Party proposed CAPA within [***] after the auditing Party provides notice of such non-compliance. The auditing Party shall have the right to review and comment on such CAPA, which comments the audited Party shall consider in good faith. The audited Party shall use Commercially Reasonable Efforts to implement such CAPA [***] after review and comment by the auditing Party. Except as may otherwise be provided in a Supply Agreement, if any Regulatory Authority or any other Governmental Authority conducts or gives notice of its intent to conduct any audit or inspection at any offices or facilities (including Development facilities) of MeiraGTx or any applicable permitted subcontractor where such audit or inspection relates to any Clinical IRD Product, then MeiraGTx will [***] notify Janssen and, to the extent such audit or inspection relates to a Clinical IRD Product and to the extent practicable and not prohibited by Applicable Law, secure for Janssen the right to participate in any such audit or inspection.

(i) Reports. Each Party will: (i) provide to the JDC, on a [***], or [***] by the JDC, an update regarding any Clinical Development Plan Activities conducted by or on behalf of such Party hereunder; and (ii) promptly (and where safety is concerned, immediately within [***]) share with the other Party all material developments and information that it comes to possess relating to the Development of each Clinical IRD Product, including: (A) safety concerns (including Adverse Events and any planned or actual inspection of any MeiraGTx facility by a Regulatory Authority or audit of any subcontractor providing material services to MeiraGTx by MeiraGTx or any Regulatory Authority); (B) study reports and data generated from Clinical Studies and (C) any significant Clinical Development Costs incurred by MeiraGTx. Each Party shall also make its employees and consultants available for an in-person or telephonic meeting with the other Party at least once every Calendar Quarter to discuss such Party's progress with respect to the conduct of the Clinical Development Plan Activities hereunder.

(j) Improved Clinical IRD Products. The Parties will only Develop an Improved Clinical IRD Product in accordance with the terms herewith with the written consent of both Parties; *provided*, *however*, that [***], then [***].

6.2. Janssen Research IRD Products.

(a) Responsibility and Costs. Janssen will be solely responsible for conducting, at its sole cost and expense, Development of each Janssen Research IRD Product, except that Janssen will use Commercially Reasonable Efforts to Develop [***].

(b) Reports. Janssen will provide to the JDC, on a [***] basis for its review and discussion, a high-level report summarizing: (i) any material Development and regulatory activities for each Janssen Research IRD Product under Development by or on behalf of Janssen over the prior Calendar Quarter; and (ii) any planned future Development and regulatory activities for each Janssen Research IRD Product (if any), including those activities it anticipates to initiate or has initiated for the following Calendar Year.

(c) Additional Support for Janssen Research IRD Products. On a Janssen Research IRD Product-by-Janssen Research IRD Product basis, Janssen may request that MeiraGTx assist in subsequent Development of such Janssen Research IRD Product. At MeiraGTx's sole discretion, it may elect to provide such assistance, in which case it will so notify Janssen in writing and thereafter MeiraGTx's costs of Developing such Janssen Research IRD Product shall be [***].

(d) Improved Janssen Research IRD Products. The Parties will only Research or Develop an Improved Janssen Research IRD Product in accordance with the terms herewith with the written consent of both Parties, *provided*, *however*, that [***], then [***].

7. REGULATORY

7.1. Clinical IRD Products.

(a) Responsibility for Regulatory Matters.

(i) *Regulatory Lead Party*. Subject to the review and approval of the JDC and JSC, on a Clinical IRD Product-by-Clinical IRD Product and country-by-country basis, (A) MeiraGTx will be the Regulatory Lead Party prior to [***]; and (B) Janssen will be the Regulatory Lead Party on and after [***]. On and after [***] for a Clinical IRD Product, Janssen will have responsibility for all regulatory matters relating to such Clinical IRD Product, including with respect to Regulatory Filings and meetings with Regulatory Authorities; *provided* that MeiraGTx will reasonably cooperate with Janssen to provide any reasonable additional assistance or materials reasonably requested by Janssen.

(ii) *General*. Subject to the review and approval of the JDC and this Section 7.1, on a Clinical IRD Product-by-Clinical IRD Product and country-by-country basis, until [***], MeiraGTx as the Regulatory Lead Party for such Clinical IRD Product shall be responsible for (A) overseeing, monitoring and coordinating all regulatory actions, communications, and filings with, and submissions to, each Regulatory Authority with respect thereto, (B) interfacing, corresponding and meeting with each Regulatory Authority with respect thereto and (C) seeking and maintaining all Regulatory Filings with respect to such Clinical IRD Product.

(iii) *Transition*. With respect to each Clinical IRD Product, within a reasonable period following the Effective Date as determined by the JDC, MeiraGTx shall take all actions required by the FDA (and, where relevant, other Regulatory Authorities) to designate Janssen as the express and authorized regulatory agent of record for MeiraGTx in the United States (and, as applicable, other countries) for the purposes of Development and regulatory activities for such Clinical IRD Product in the United States (and, as applicable, such other countries). On a Clinical IRD Product-by-Clinical IRD Product basis and country-by-country basis, upon a date prior to the applicable Sponsorship Transfer Date as reasonably determined by the JDC, MeiraGTx will [***] assign and transfer to Janssen or its designee all existing Regulatory Filings and other Regulatory Materials with respect to such Clinical IRD Product, including all Drug Master Files and all written correspondence, minutes of meetings and memoranda of oral communications with any Regulatory Authority, on electronic media (including rendered source documents when available), to the extent not already provided to Janssen previously; *provided*, *however*, that Regulatory Filings and other Regulatory Materials with respect to such Clinical IRD Product generated on or after the Effective Date shall be provided in accordance with Janssen's instructions (including Janssen's formatting and Electronic Common Technical Document (eCTD) requirements). Each Party will submit to the applicable Regulatory Authority all filings, letters and other documentation necessary to effect such assignment and transfer as soon as practicable, in an efficient and seamless manner for such Clinical IRD Product. Each Party shall provide to the other Party a copy of any and all notices received by such Party from such Regulatory Authority confirming such assignment and transfer.

(iv) *Right of Reference*. For each Clinical IRD Product, each Party hereby grants and will cause its Affiliates, licensees, and Sublicensees to grant to the other Party, a Right of Reference to, and a right to access, copy and use, all information and data (including all CMC information) included in or used in support of any Drug Master File maintained by or on behalf of such Party that relates to such Clinical IRD Product to the extent necessary for the other Party to Develop or Manufacture such Clinical IRD Product in accordance with the applicable Clinical Development Plan or CMC Development Plan. From and after the Sponsorship Transfer Date, MeiraGTx hereby grants and will cause its Affiliates, licensees, and Sublicensees to grant to Janssen, a Right of Reference to, and a right to copy, access, and otherwise use, all information and data (including all CMC information) included in or used in support of any Drug Master File maintained by or on behalf of MeiraGTx that relates to such Clinical IRD Product to the extent not transferred to Janssen pursuant to Section 7.1(a)(iii). Notwithstanding anything to the contrary in this Agreement, MeiraGTx will not, and will cause its Affiliates, licensees, and Sublicensees not to, withdraw or inactivate any Regulatory Filing that Janssen, its Affiliates or Sublicensees reference or otherwise use pursuant to this Section 7.1(a)(iv). In addition, Janssen hereby grants and will cause its Affiliates, licensees, and Sublicensees to grant to MeiraGTx a Right of Reference to, and a right to access, copy and use, all information and data (including CMC information) included in or used in support of any Drug Master File maintained by or on behalf of Janssen that relate to the RPGR Product to the extent necessary to Research, Develop, Manufacture or Commercialize the [***] Product, solely in the event that Janssen elects to not make the [***] Product a Clinical IRD Product in accordance with Section 2.1(b). [***]

(b) Regulatory Meetings. Until the Sponsorship Transfer Date for any Clinical IRD Product, MeiraGTx will: (i) provide Janssen with reasonable advance notice of all substantive meetings, conferences, and discussions (whether in person or by telephonic or video conference) with any Regulatory Authorities pertaining to such Clinical IRD Product; (ii) provide Janssen with draft briefing materials and meeting presentations for review reasonably in advance and consider in the preparation of such meetings, conferences or discussions any reasonable input timely provided by Janssen; and (iii) to the extent not prohibited by Applicable Law, grant Janssen the right to participate in any such meetings, conferences or discussions and facilitate such participation (such number of representatives to attend to be determined by Janssen). If Janssen elects not to participate in such meetings, conferences or discussions, MeiraGTx shall provide Janssen, upon Janssen's request, with written summaries of such meetings, conferences or discussions in English after the conclusion thereof.

(c) Regulatory Filings. Until the Sponsorship Transfer Date of any Clinical IRD Product, MeiraGTx will: (i) provide to Janssen for review and comment in accordance with such timeframes specified by the JDC (such timeframe to be no less than [***] in advance of any regulatory deadline, or if any Regulatory Authority deadline is sooner, as reasonably in advance as possible), copies in English of all Regulatory Filings and Regulatory Materials to be submitted (other than routine correspondence and administrative documents and documents related to Pricing Approval) by or on behalf of MeiraGTx prior to the relevant submission with respect to such Clinical IRD Product; (ii) incorporate reasonable comments thereto provided by Janssen; and (iii) [***] notify and provide Janssen any Regulatory Materials (other than routine correspondence and administrative documents and documents related to Pricing Approval) received from any Regulatory Authority with respect to such Clinical IRD Product.

(d) Regulatory Vouchers. If a Priority Review Voucher is issued to MeiraGTx for any Clinical IRD Product, then MeiraGTx will transfer such Priority Review Voucher to Janssen. If Janssen elects to transfer to a Third Party or otherwise monetize such Priority Review Voucher, the proceeds thereof will be allocated [***], with [***] percent ([***]%) to Janssen and [***] percent ([***]%) to MeiraGTx. If Janssen uses such Priority Review Voucher for one of its programs or for the programs of one of its Affiliates, then: (i) the value of such Priority Review Voucher will be determined by: (A) taking the values of the [***] most recent publicly disclosed sales or transfers of a Priority Review Voucher as of the time that the Party was granted such Priority Review Voucher; (B) removing the highest and lowest of such [***] publicly disclosed values; and (C) averaging the values of the remaining [***] publicly disclosed values; and (ii) the determined value of such Priority Review Voucher will be allocated [***], with [***] percent ([***]%) to Janssen and [***] percent ([***]%) to MeiraGTx.

(e) [***] Product. In the event that Janssen notifies MeiraGTx that Janssen does not elect to make the [***] Product a Clinical IRD Product or that Janssen fails to notify MeiraGTx that Janssen will make the [***] Product a Clinical IRD Product, in each case as set forth in Section 2.1(b), then MeiraGTx shall [***] notify Janssen of [***] that directly relates to or impacts any Clinical IRD Product or Janssen Research IRD Product. In addition, if Janssen becomes aware of [***], MeiraGTx shall [***] provide any [***].

7.2. Research IRD Products.

(a) Responsibility and Costs for Regulatory Matters. For each Janssen Research IRD Product, Janssen will be solely responsible, [***] for determining the regulatory plans and strategies and other regulatory matters relating to such Janssen Research IRD Product, including: (i) overseeing, monitoring and coordinating all regulatory actions, communications and filings with, and submissions to, each Regulatory Authority with respect to such Janssen Research IRD Product; and (ii) interfacing, corresponding, and meeting with each Regulatory Authority. Janssen shall invite MeiraGTx to attend, in an observational role or, to the extent permitted by Applicable Law, in a participatory role, material meetings and telephone conferences with regulators regarding such Janssen Research IRD Product. MeiraGTx will cooperate with and provide reasonable assistance to Janssen and its designees upon Janssen's request in connection with filings to any Regulatory Authority relating to such Janssen Research IRD Product, including by executing any required documents, providing access to personnel and providing Janssen with copies of all reasonably required documentation (such documents to be generated and provided to Janssen in accordance with Janssen's formatting requirements and Electronic Common Technical Document (eCTD) requirements, to the extent that such documents are generated on or after the Effective Date).

(b) Ownership of Regulatory Filings. For each Janssen Research IRD Product, Janssen or its designee will own all Regulatory Filings and related Regulatory Materials with respect to each such Janssen Research IRD Product, including any Drug Master Files maintained by or on behalf of MeiraGTx primarily related to and reasonably necessary for the Development of such

Janssen Research IRD Product. At Janssen's request, MeiraGTx will [***] assign and transfer to Janssen, all Regulatory Filings and related Regulatory Materials with respect to such Janssen Research IRD Product that is in the possession or control of MeiraGTx and exclusively related to such Janssen Research IRD Product (such Regulatory Materials to be generated and provided to Janssen in rendered source document format when available and in accordance with Janssen's formatting requirements and Electronic Common Technical Document (eCTD) requirements, to the extent that such documents are generated on or after the Effective Date), and each Party will submit all filings, letters and other documentation necessary to effect such assignment and transfer to the applicable Regulatory Authority as soon as reasonably practicable, but no later than [***] after such request for such Janssen Research IRD Product. Each Party shall provide to the other Party a copy of any and all notices received by such Party from such Regulatory Authority confirming such assignment and transfer.

(c) Right of Reference. MeiraGTx hereby grants, and will cause its Affiliates, licensees, and Sublicensees to grant, to Janssen, at the request of Janssen or its Affiliates or Sublicensees, a Right of Reference to, and a right to copy, access, and otherwise use, all information and data (including all CMC information) included in or used in support of any Drug Master File maintained by or on behalf of MeiraGTx that relates to a Janssen Research IRD Product to the extent necessary to Research, Develop, Manufacture or Commercialize such Janssen Research IRD Product, in each case, not transferred to Janssen pursuant to Section 7.2(b). Notwithstanding anything to the contrary in this Agreement, MeiraGTx will not, and will cause its Affiliates, licensees, and Sublicensees not to, withdraw or inactivate any Regulatory Filing that Janssen, its Affiliates or Sublicensees reference or otherwise use pursuant to this Section 7.2(c). [***]

(d) Regulatory Vouchers. Janssen shall have the sole right to apply for any Priority Review Voucher for any Janssen Research IRD Product. [***].

7.3. Pharmacovigilance.

On a Product-by-Product basis, the Parties shall negotiate in good faith and mutually agree upon a written pharmacovigilance agreement for each Product no later than the earliest of (a) close of the Phase 3 Study for such Product, (b) the Sponsorship Transfer Date for such Product, or (c) engagement in Commercialization for such Product. Each such written pharmacovigilance agreement shall contain provisions to ensure that the safety data exchange and other pharmacovigilance responsibilities of both Parties with respect to regulatory reporting requirements are fulfilled in accordance with Applicable Laws with respect to such Product. Janssen will take the lead for safety and hold the global safety database on behalf of both Parties for all Clinical IRD Products no later than [***], Janssen will take the lead for safety and hold the global safety database on behalf of both Parties for all Janssen Research IRD Products as of such date determined by the JDC, and the respective pharmacovigilance agreement shall contain safety lead and global safety database provisions consistent with the foregoing.

7.4. Clinical Study Quality Agreement.

The Parties shall use good faith efforts to negotiate and execute [***] following the Effective Date an agreement to govern the conduct and quality of Clinical Studies, consistent with good pharmacovigilance practices and Janssen Quality Requirements.

8. MANUFACTURING

8.1. Product Manufacturing outside Supply Agreements.

(a) For Research. Subject to the terms and conditions of this Agreement and the oversight of the JSC and JMC, MeiraGTx will Manufacture (i) Clinical IRD Products required for Research under Section 6.1(b) in accordance with the applicable CMC Development Plans and (ii) Research IRD Products for Research in accordance with the applicable CMC Development Plans, in each case ((i) and (ii)) in accordance with Janssen Quality Requirements.

(b) For Development. Subject to the terms and conditions of this Agreement and the oversight of the JSC and JMC, MeiraGTx will Manufacture Clinical IRD Products for use in respective Clinical Studies in accordance with Janssen Quality Requirements. (c) MeiraGTx Facility. All Products to be Manufactured by MeiraGTx as set forth in this Section 8.1 will be Manufactured at the MeiraGTx Facility. If MeiraGTx wishes to use a CMO to Manufacture any Product, MeiraGTx shall first offer such Manufacturing opportunity to Janssen, and Janssen shall have [***] to respond regarding whether it wishes to be engaged as a CMO of such Product for MeiraGTx. If Janssen responds to MeiraGTx within such [***] period that Janssen wishes to be engaged as a CMO, then the Parties will [***] negotiate in good faith and agree upon the terms of such Manufacturing. If Janssen responds to MeiraGTx within such [***] period that Janssen does not wish to be engaged as a CMO, or if Janssen fails to respond within such [***] period, then MeiraGTx may use a Third Party as its CMO, *provided* that MeiraGTx obtains Janssen's prior written consent thereto (not to be unreasonably withheld, conditioned or delayed) prior to engaging such CMO for such services.

(d) Price for Supply of Products. The costs for Manufacturing Products in accordance with Section 8.1(a) shall be set forth in the Clinical Development Manufacturing Budget. The Manufacturing of Clinical IRD Products in accordance with Section 8.1(b) shall be [***]. Janssen shall [***].

8.2. Clinical Supply Agreement.

At such time as directed by the JSC and subject to the oversight of the JMC, the Parties will [***] negotiate in good faith a definitive clinical supply agreement for MeiraGTx to Manufacture and supply Janssen Research IRD Products for use in respective Clinical Studies ("**Clinical Supply Agreement**"), along with the associated quality agreement for such Janssen Research IRD Products ("**Clinical Quality Assurance Agreement**"). The Clinical Supply Agreement and the Clinical Quality Assurance Agreement will contain such terms provided in Exhibit 8.2 attached hereto. The Clinical Supply Agreement and Clinical Quality Assurance Agreement will each also

be consistent with the terms and conditions of this Agreement and contain other terms and conditions customary for agreements of such nature. The Clinical Supply Agreement and the Clinical Quality Assurance Agreement will each be executed prior to the initiation of any Clinical Study applicable to the Products supplied thereunder.

8.3. Commercial Supply Agreement.

At such time as directed by the JSC and subject to the oversight of the JMC, the Parties will [***] negotiate in good faith a definitive commercial supply agreement for MeiraGTx to Manufacture and supply Clinical IRD Products for Commercial Supply ("**Commercial Supply Agreement**"), along with the associated quality agreement for such Clinical IRD Products ("**Commercial Quality Assurance Agreement**"). The Commercial Supply Agreement and Commercial Quality Assurance Agreement will contain such terms provided in Exhibit 8.3 attached hereto. The Commercial Supply Agreement and Commercial Quality Assurance Agreement will each also be consistent with the terms and conditions of this Agreement and contain other terms and conditions customary for agreements of such nature. The Commercial Supply Agreement and Commercial Quality Assurance Agreement will each be executed by the Parties at least [***] prior to the reasonably anticipated date of the First Commercial Sale of any Clinical IRD Product.

8.4. Collaboration on CMC Development.

(a) CMC Development Plan. Subject to the oversight of the JMC and JSC, during the CMC Development Term, Janssen and MeiraGTx will each collaborate with one another to Develop the Manufacturing process used for each clinical program undertaken hereunder in connection with any Clinical IRD Product or Research IRD Product, to identify certain technical Manufacturing process improvements and efficiencies and to share data packages necessary for regulatory filings in connection therewith ("**CMC Development Collaboration**"). The Parties will undertake the CMC Development Collaboration in accordance with this Agreement and one or more CMC Development Plans, which plan will set forth (i) all activities that are necessary or useful to be undertaken by each Party in connection with such CMC Development Collaboration (the "**CMC Development Plan Activities**"), (ii) an estimated timeline for completing such activities, (iii) the respective deliverables for such activities and (iv) the allocation of responsibilities between the Parties for performance of each such activity. In addition, the CMC Development Plan Activities will comprise of: (x) those specific activities listed under "Clinical Development Manufacturing Activities" in Exhibit 8.4(a) attached hereto ("**Clinical Development Manufacturing Activities**") and (y) those specific activities listed under "Process Development Activities" in Exhibit 8.4(a) attached hereto ("**Process Development Activities**"). In the event that any CMC Development Plan Activity does not fall under one of the specific activities listed in Exhibit 8.4(a), the JMC shall discuss and determine whether such CMC Development Plan Activity should be (1) treated as a Clinical Development Manufacturing Activity hereunder and subject to the cost provisions set forth in Section 8.4(d) or (2) treated as a Process Development Activity hereunder and subject to the cost provisions set forth in Section 8.4(e). The JMC shall develop and submit an initial CMC Development Plan to the JSC for its review and approval, [***], and upon the JSC's approval, such initial CMC Development Plan will be automatically attached hereto and form a part of Exhibit 1.51. From time to time, and at least on an annual basis,

the JMC shall propose, review and approve amendments or updates to each CMC Development Plan, subject to the remaining terms and conditions of this Agreement. Each such amended or updated CMC Development Plan will automatically replace the respective CMC Development Plan previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. Each CMC Development Plan shall also be consistent with the terms of this Agreement. The Parties will use Commercially Reasonable Efforts to carry out their respective obligations under each CMC Development Plan.

(b) Clinical Development Manufacturing Budget. The CMC Development Collaboration will be subject to a rolling budget covering Clinical Development Manufacturing Costs associated with the anticipated Clinical Development Manufacturing Activities to be performed during the [***] (such [***] period, the "[***] **Clinical Development Manufacturing Budget Period**"), in each case broken down by Calendar Quarter and broken out on a line item basis to show Out-of-Pocket Costs and FTE Costs of FTEs directly engaged to perform each such Clinical Development Manufacturing Activity and further broken down by Party (the "**Clinical Development Manufacturing Budget**"). [***] after the Effective Date, the Clinical Development Manufacturing Budget will be developed by the JMC, and recommended and proposed to the JSC for review and approval, and upon approval by the JSC, each such Clinical Development Manufacturing Budget will be automatically incorporated into this Agreement by reference and form a part of this Agreement. The Clinical Development Manufacturing Budget will be reviewed by the JMC and approved by the JSC (i) at least [***] (and [***] for the first Calendar Year of the applicable [***] Clinical Development Manufacturing Budget Period) based on: (A) the Parties' good faith estimation of the anticipated Clinical Development Manufacturing Activities to be conducted during the relevant [***] Clinical Development Manufacturing Budget Period; and (B) information prepared by the Parties in good faith for their own internal planning processes relating to anticipated Clinical Development Manufacturing Activities; or (ii) whenever the total Clinical Development Manufacturing Costs for any given Calendar Quarter are reasonably expected to be at least [***] percent ([***]%) higher than the Clinical Development Manufacturing Budget for such Calendar Quarter, whether as a result of any amendments to the CMC Development Plan or increases in costs for the Clinical Development Manufacturing Activities already planned for such Calendar Quarter (*provided, however*, that following the beginning of a Calendar Year, the JSC may not increase the Clinical Development Manufacturing Budget for such Calendar Year by more than [***] percent ([***]%) without the Parties' prior written approval). Each such amended or updated Clinical Development Manufacturing Budget will automatically replace the respective Clinical Development Manufacturing Budget previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. With respect to any Calendar Quarter, in no event will a Party be responsible for any Clinical Development Manufacturing Costs that exceed the respective portion of the then-current Clinical Development Manufacturing Budget recommended and proposed by the JMC and approved by the JSC, except as otherwise provided in Section 10.4(b).

(c) Process Development Budget. The CMC Development Collaboration will be subject to a rolling budget covering Process Development Costs associated with the anticipated Process Development Activities to be performed during the [***] (such [***] period, the "[***] **Process Development Budget Period**"), in each case broken down by Calendar Quarter and broken out on a line item basis to show Out-of-Pocket Costs and FTE Costs of FTEs directly engaged to perform each such Process Development Activity and further broken down by Party (the "**Process Development Budget**"). [***] following the Effective Date, the Process Development Budget will be developed by the JMC, and recommended and proposed to the JSC for review and approval, and upon approval by the JSC, each such Process Development Budget will be automatically incorporated into this Agreement by reference and form a part of this Agreement. The Process Development Budget will be reviewed by the JMC and approved by the JSC (i) at least [***] (and [***] for the first Calendar Year of the applicable [***] Process Development Budget Period) based on: (A) the Parties' good faith estimation of the anticipated Process Development Activities to be conducted during the relevant [***] Process Development Budget Period; and (B) information prepared by the Parties in good faith for their own internal planning processes relating to anticipated Process Development Activities; or (ii) whenever the total Process Development Costs for any given Calendar Quarter are reasonably expected to be at least [***] percent ([***]%) higher than the Process Development Budget for such Calendar Quarter, whether as a result of any amendments to the CMC Development Plan or increases in costs for the Process Development Activities already planned for such Calendar Quarter (*provided*, *however*, that following the beginning of a Calendar Year, the JSC may not increase the Process Development Budget for such Calendar Year by more than [***] percent ([***]%) without the Parties' prior written approval). Each such amended or updated Process Development Budget will automatically replace the respective Process Development Budget previously in effect, and will be automatically incorporated into this Agreement by reference and form a part of this Agreement. With respect to any Calendar Quarter, in no event will a Party be responsible for any Process Development Costs that exceed the respective portion of the then-current Process Development Budget recommended and proposed by the JMC and approved by the JSC, except as otherwise provided in Section 10.5(b).

(d) Clinical Development Manufacturing Costs. Janssen will be responsible for [***] of the documented Clinical Development Manufacturing Costs; *provided*, *however*, that in no event will any Clinical Development Manufacturing Costs for any Calendar Quarter exceed the applicable Clinical Development Manufacturing Budget for such Calendar Quarter, except as otherwise provided in Section 10.4(b). All Clinical Development Manufacturing Costs shall be reconciled and paid in accordance with the procedure described in Section 10.4. Notwithstanding anything herein to the contrary, [***].

(e) Process Development Costs. Janssen and MeiraGTx will each be responsible for [***] of the documented Process Development Costs; *provided*, *however*, that in no event will a Party's portion of any Process Development Costs for any Calendar Quarter exceed its portion of the applicable Process Development Budget for such Calendar Quarter, except as otherwise provided in Section 10.5(b). All Process Development Costs shall be reconciled and paid in accordance with the procedure described in Section 10.5. Notwithstanding anything herein to the contrary, in no event will [***] of any Process Development Costs [***].

8.5. [***] Manufacturing Services.

During the [***] after the Effective Date, if MeiraGTx intends to grant any Third Party the right to reserve for such Third Party one (1) vector Manufacturing suite at the MeiraGTx Facility for Manufacturing of any product other than a Product, then MeiraGTx shall [***]. If Janssen notifies MeiraGTx in writing within [***] of receipt of MeiraGTx's notice that Janssen desires to [***], then the Parties [***] (the "**Manufacturing Services Agreement**"), which agreement will contain customary terms and conditions, including [***], *except* that, if Janssen [***] for Manufacturing. If Janssen either responds to MeiraGTx that [***].

9. COMMERCIALIZATION

Janssen will be solely responsible, at its sole cost and expense, for all aspects of Commercialization in the Territory of each Clinical IRD Product and each Janssen Research IRD Product, including planning and implementation, distribution, booking of sales, pricing and reimbursement, *except* that Janssen shall use Commercially Reasonable Efforts, at its expense, to Commercialize (a) for each Clinical IRD Target, [***] and (b) for each Research IRD Target for which Janssen has exercised an Option, [***], in each case ((a) and (b)) following the obtaining of Regulatory Approval for such Clinical IRD Product or Janssen Research IRD Product (as applicable) and [***] such Regulatory Approval has been obtained.

10. FINANCIAL PROVISIONS

10.1. Upfront Payment.

Provided that this Agreement is not terminated in accordance with Section 14.3, Janssen shall pay to MeiraGTx within [***], a one-time, non-refundable, non-creditable payment of One Hundred Million Dollars ($100,000,000) in partial consideration of the collaborations, options, licenses and rights granted by MeiraGTx to Janssen under this Agreement ("**Upfront Payment**"). For clarity, Janssen shall have no obligation to pay the Upfront Payment in the event that this Agreement is terminated in accordance with Section 14.3.

10.2. Research Costs.

(a) Invoices. For each Research Plan, commencing upon the first Calendar Quarter immediately following the date that such Research Plan is first approved by the JSC pursuant to Section 3.2 and continuing thereafter during the applicable Research Plan Term so long as MeiraGTx incurs Research Costs under this Agreement, MeiraGTx will submit to Janssen within [***] after the conclusion of each Calendar Quarter an invoice setting forth the Research Costs that MeiraGTx incurred in performing its Research Plan Activities under such Research Plan in such Calendar Quarter. Each invoice shall include the nature and amount of Research services rendered or deliverables provided, and each invoice shall provide proper support for expenses included on the invoice. Reasonable support documents for Out-of-Pocket Costs include invoices or pro forma invoices from Third Party vendors. For FTE reimbursement, proper support includes an FTE time report break down by function.

(b) Research Costs. Within [***] after receipt of such invoice, Janssen shall make a reconciliation payment to MeiraGTx equal to [***] percent ([***]%) of all Research Costs incurred during such Calendar Quarter; *provided*, *however*, that if the total Research Costs for such Calendar Quarter exceeds the Research Budget for such Calendar Quarter, then MeiraGTx will provide written notice to Janssen as far in advance as reasonably possible of such cost overrun along with a reasonably detailed explanation of such cost overrun. Janssen shall pay for such excess only if: (i) the JSC approves of such excess; or (ii) such excess exceeds the Research Budget for such Calendar Quarter by [***] percent ([***]%) or less and is attributable to a change in Applicable Law; *provided*, *however*, that in no event will any total Research Costs for a Calendar Year exceed the Research Budget Cap for such Calendar Year, which excess may only be approved by the Parties in writing. Janssen shall not be obligated to pay for any Research Costs in excess of the Research Budget except as otherwise set forth in this Section 10.2(b) or with the Parties' written approval therefor. All such reconciliation payments shall be non-creditable and non-refundable. In addition, subject to the terms and conditions herein, if any Research Plan Activities will exceed the respective portion of the applicable Research Budget or extend beyond the applicable Research Plan Term, then (1) MeiraGTx shall provide written notice to Janssen, as far in advance as reasonably possible, that MeiraGTx intends to not perform such Research Plan Activities (with reasonable detail regarding such Research Plan Activities and Research Costs), (2) MeiraGTx shall afford the JSC or the Parties, as applicable, a reasonable opportunity to approve additional funding or reimburse such Research Plan Activities as permitted in this Section 10.2(b), and (3) MeiraGTx shall not be obligated to undertake such Research Plan Activities unless the JSC or the Parties, as applicable, agree to provide such additional funding as permitted in this Section 10.2(b).

10.3. Clinical Development Costs.

(a) Invoices. For each Clinical IRD Product (and any Janssen Research IRD Product subject to Section 6.2(c)), commencing upon the first Calendar Quarter immediately following the Effective Date and continuing thereafter so long as MeiraGTx incurs Clinical Development Costs under this Agreement, MeiraGTx will submit to Janssen within [***] after the conclusion of such Calendar Quarter an invoice setting forth the Clinical Development Costs MeiraGTx incurred in such Calendar Quarter. Each invoice shall include the nature and amount of Development services rendered or deliverables provided, and each invoice shall provide proper support for expenses included on the invoice. Reasonable support documents for Out-of-Pocket Costs include invoices or pro forma invoices from Third Party vendors. For FTE reimbursement, proper support includes an FTE time report break down by function.

(b) Clinical Development Costs. Within [***] after receipt of such invoice and report, Janssen will pay to MeiraGTx all the Clinical Development Costs set forth in each such invoice; *provided*, *however*, that if the total Clinical Development Costs for such Calendar Quarter exceeds the Clinical Development Budget for such Calendar Quarter, then MeiraGTx will provide written notice to Janssen as far in advance as reasonably possible of such cost overrun along with a reasonably detailed explanation of such cost overrun. Janssen shall pay for such excess only if: (i) the JSC approves of such excess; or (ii) such excess exceeds the Clinical Development Budget for such Calendar Quarter by [***] percent ([***]%) or less and is attributable to (A) a change in Applicable Law, (B) a variation in actual patient enrollment from projected patient enrollment or (C) a change to a clinical trial protocol required or requested by any Regulatory Authority;

provided, *however*, that in no event will total Clinical Development Costs in any given Calendar Year exceed the Clinical Development Budget for such Calendar Year by more than [***] percent ([***]%), which excess may only be approved by the Parties in writing. Janssen shall not be obligated to pay for any Clinical Development Costs in excess of the Clinical Development Budget except as otherwise set forth in this Section 10.3(b). All such payments of MeiraGTx's Clinical Development Costs shall be non-creditable and non-refundable. In addition, subject to the terms and conditions herein, if any Clinical Development Plan Activities will exceed the respective portion of the applicable Clinical Development Budget or extend beyond the applicable Clinical Development Plan Term, then (x) MeiraGTx shall provide written notice to Janssen, as far in advance as reasonably possible, that MeiraGTx intends to not perform such Clinical Development Plan Activities (with reasonable detail regarding such Clinical Development Plan Activities and Clinical Development Costs), (y) MeiraGTx shall afford the JSC or the Parties, as applicable, a reasonable opportunity to approve additional funding or reimburse such Clinical Development Plan Activities as permitted in this Section 10.3(b), and (z) MeiraGTx shall not be obligated to undertake such Clinical Development Plan Activities unless the JSC or the Parties, as applicable, agree to provide such additional funding as permitted in this Section 10.3(b).

10.4. Clinical Development Manufacturing Costs.

(a) Invoices. For each Clinical IRD Product (and any Janssen Research IRD Product subject to Section 6.2(c)), commencing upon the [***] following the Effective Date and continuing thereafter so long as MeiraGTx incurs Clinical Development Manufacturing Costs under this Agreement, MeiraGTx will submit to Janssen within [***] after the conclusion of such Calendar Quarter an invoice setting forth the Clinical Development Manufacturing Costs MeiraGTx incurred in such Calendar Quarter. Each invoice shall include the nature and amount of services rendered or deliverables provided in connection with Clinical Development Manufacturing Activities, and each invoice shall provide proper support for expenses included on the invoice. Reasonable support documents for Out-of-Pocket Costs include invoices or pro forma invoices from Third Party vendors. For FTE reimbursement, proper support includes an FTE time report break down by function.

(b) Clinical Development Manufacturing Costs. Within [***] after receipt of such invoice and report, Janssen will pay to MeiraGTx [***] the Clinical Development Manufacturing Costs set forth in each such invoice; *provided*, *however*, that if the total Clinical Development Manufacturing Costs for such Calendar Quarter exceeds the Clinical Development Manufacturing Budget for such Calendar Quarter, then MeiraGTx will provide written notice to Janssen as far in advance as reasonably possible of such cost overrun along with a reasonably detailed explanation of such cost overrun. Janssen shall pay for such excess only if: (i) the JSC approves of such excess; or (ii) such excess exceeds the Clinical Development Manufacturing Budget for such Calendar Quarter by [***] percent ([***]%) or less and is attributable to (A) a change in Applicable Law, (B) a variation in actual patient enrollment from projected patient enrollment or (C) a change to a clinical trial protocol required or requested by any Regulatory Authority; *provided*, *however*, that in no event will total Clinical Development Manufacturing Costs in any given Calendar Year exceed the Clinical Development Manufacturing Budget for such Calendar Year by more than [***] percent ([***]%), which excess may only be approved by the Parties in writing. Janssen

shall not be obligated to pay for any Clinical Development Manufacturing Costs in excess of the Clinical Development Manufacturing Budget except as otherwise set forth in this Section 10.4(b). All such payments of MeiraGTx's Clinical Development Manufacturing Costs shall be non-creditable and non-refundable. In addition, subject to the terms and conditions herein, if any Clinical Development Manufacturing Activities will exceed the respective portion of the applicable Clinical Development Manufacturing Budget or extend beyond the applicable CMC Development Term, then (x) MeiraGTx shall provide written notice to Janssen, as far in advance as reasonably possible, that MeiraGTx intends to not perform such Clinical Development Manufacturing Activities (with reasonable detail regarding such Clinical Development Manufacturing Activities and Clinical Development Manufacturing Costs), (y) MeiraGTx shall afford the JSC or the Parties, as applicable, a reasonable opportunity to approve additional funding or reimburse such Clinical Development Manufacturing Activities as permitted in this Section 10.4(b), and (z) MeiraGTx shall not be obligated to undertake such Clinical Development Manufacturing Activities unless the JSC or the Parties, as applicable, agree to provide such additional funding as permitted in this Section 10.4(b).

10.5. Process Development Costs.

(a) Invoices. Within [***] after the conclusion of each Calendar Quarter, MeiraGTx and Janssen each will submit to the other Party an invoice setting forth the Process Development Costs it incurred in such Calendar Quarter under the CMC Development Plan. Each invoice shall include the nature and amount of services rendered or deliverables provided in connection with Process Development Activities, and each invoice shall provide proper support for expenses included on the invoice. Reasonable support documents for Out-of-Pocket Costs include invoices or pro forma invoices from Third Party vendors. For FTE reimbursement, proper support includes an FTE time report break down by function.

(b) Process Development Costs. Within [***] after receipt of such invoices, the Parties will confer and agree in writing on whether a reconciliation payment is due from MeiraGTx to Janssen or Janssen to MeiraGTx, and if so, the amount of such reconciliation payment, so that [***] Process Development Costs; *provided*, *however*, that if the Process Development Costs incurred by a Party for such Calendar Quarter exceeds such Party's Process Development Budget for such Calendar Quarter, then such Party will provide written notice to the other Party as far in advance as reasonably possible of such cost overrun along with a reasonably detailed explanation of such cost overrun. The other Party shall pay for its portion of such excess only if: (i) the JSC approves of such excess; or (ii) such excess exceeds the Process Development Budget for such Calendar Quarter by [***] percent ([***]%) or less and is attributable to a change in Applicable Law; *provided*, *however*, that in no event will total Process Development Costs in any given Calendar Year exceed the Process Development Budget for such Calendar Year by more than [***] percent ([***]%), which excess may only be approved by the Parties in writing. The Party owed a reconciliation in connection with any Process Development Costs shall provide an invoice for the amount of such reconciliation payment to the paying Party, and such paying Party will make such reconciliation payment to the other Party, within [***] following receipt of the other Party's invoice for such amount. Janssen shall not be obligated to pay for any portion of Process Development Costs in excess of the applicable portion of the applicable Process Development

Budget except as otherwise set forth in this Section 10.5(b). All such payments shall be non-creditable and non-refundable. In addition, subject to the terms and conditions herein, if any Process Development Activities will exceed the respective portion of the applicable Process Development Budget or extend beyond the applicable CMC Development Term, then (x) MeiraGTx shall provide written notice to Janssen, as far in advance as reasonably possible, that MeiraGTx intends not to perform such Process Development Activities (with reasonable detail regarding such Process Development Activities and Process Development Costs), (y) MeiraGTx shall afford the JSC or the Parties, as applicable, a reasonable opportunity to approve additional funding or reimburse such Process Development Activities as permitted in this Section 10.5(b), and (z) MeiraGTx shall not be obligated to undertake such Process Development Activities unless the JSC or the Parties, as applicable, agree to provide such additional funding as permitted in this Section 10.5(b).

10.6. Development Milestone Payments.

In partial consideration of the cooperation of MeiraGTx in conducting the Clinical Development Plan Activities hereunder, and subject to Section 10.10(b), on a Product-by-Product basis, Janssen shall make milestone payments to MeiraGTx (each, a "**Development Milestone Payment**") upon the first (1st) achievement of each milestone event set forth in this Section 10.6 (each, a "**Development Milestone Event**") as set forth in the applicable tables below. Each Development Milestone Payment shall be non-refundable and non-creditable.

(a) Clinical IRD Products. Subject to the terms and conditions of this Section 10.6, on a Clinical IRD Product-by-Clinical IRD Product basis, Janssen shall make the Development Milestone Payments provided below to MeiraGTx upon the first (1st) achievement of the corresponding Development Milestone Event for the applicable Clinical IRD Product. Each Development Milestone Payment for a Clinical IRD Product will be payable only once with respect to the respective Clinical IRD Product, even if the corresponding Development Milestone Event occurs: (i) more than once; (ii) with respect to more than one (1) Gene Therapy Product that treats the same Clinical IRD Target Indication as such Clinical IRD Product, including if such Clinical IRD Product [***]; or (iii) for Development Milestone Events set forth in the tables below, with respect to more than one (1) Indication. The aggregate total of all Development Milestone Payments made with respect to each Clinical IRD Product shall not exceed the amount identified as the Development Milestone Cap for such Clinical IRD Product in the applicable table below.

(i) *RPGR Product*

Development Milestone Event	Development Milestone Payment (USD)
[***]	[***]
[***]	[***]
[***]	[***]
[***]	**[***]**

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(ii) *CNGB3 Product*

Development Milestone Event	Development Milestone Payment (USD)
[***]	[***]
[***]	[***]
[***]	[***]
[***]	**[***]**

(iii) *CNGA3 Product*

Development Milestone Event	Development Milestone Payment (USD)
[***]	[***]
[***]	[***]
[***]	[***]
[***]	**[***]**

(iv) [***] Product (subject to Section 2.1(b))

Development Milestone Event	Development Milestone Payment (USD)
[***]	[***]
[***]	[***]
[***]	[***]
[***]	**[***]**

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(b) Research IRD Products. Subject to the terms and conditions of this Section 10.6, on a Janssen Research IRD Product-by-Janssen Research IRD Product basis, Janssen shall make the Development Milestone Payments provided below to MeiraGTx upon the first (1st) achievement of the corresponding Development Milestone Event for each Janssen Research IRD Product. Each Development Milestone Payment for a Janssen Research IRD Product will be payable only once, even if the corresponding Development Milestone Event occurs: (i) more than once; (ii) with respect to more than one (1) Gene Therapy Product that treats the same Research IRD Target Indication as such Janssen Research IRD Product, including if such Janssen Research IRD Product [***]; or (iii) for Development Milestone Events set forth in the table below, with respect to more than one Indication. The aggregate total of all Development Milestone Payments made with respect to each Janssen Research IRD Product shall not exceed the amount identified as the Development Milestone Cap for such Janssen Research IRD Product in the table below.

(i) *Each Janssen Research IRD Product*

Development Milestone Event	**Development Milestone Payment (USD)**
[***]	[***]
[***]	[***]
[***]	[***]
[***]	**[***]**

(c) Payment Terms for Development Milestone Payments. Janssen shall provide MeiraGTx with written notice of the achievement of each Development Milestone Event for which payment is due hereunder within [***] after the Calendar Quarter in which such Development Milestone Event has been achieved. After receipt of such notice, MeiraGTx shall submit an invoice to Janssen for the corresponding Development Milestone Payment, and Janssen shall make the corresponding Development Milestone Payment to MeiraGTx within [***] after Janssen's receipt of such invoice.

10.7. Commercial Milestone Payments.

(a) Commercial Milestone Payments and Events for Clinical IRD Products. In partial consideration of the cooperation provided by MeiraGTx to Janssen under this Agreement, and subject to Section 10.10(b), on a Clinical IRD Product-by-Clinical IRD Product basis, Janssen shall make one (1)-time payments of each of the sales milestone payments indicated below (each, a "**Commercial Milestone Payment**") to MeiraGTx when the worldwide, aggregate (*i.e.*, cumulative since First Commercial Sale) Net Sales of a Clinical IRD Product first achieves the

Dollar thresholds indicated in the table below (each, a "**Commercial Milestone Event**"). Each Commercial Milestone Payment will be payable only once with respect to a Clinical IRD Product, notwithstanding the number of times a Dollar threshold indicated in the table below is reached. Each Commercial Milestone Payment shall be non-refundable and non-creditable.

Commercial Milestone Event	Commercial Milestone Payment (USD)
[***]	[***]
[***]	[***]
[***]	[***]

(b) Payment Terms for Commercial Milestone Payments. Janssen shall provide MeiraGTx with written notice of the achievement of each Commercial Milestone Event within [***] after the Calendar Quarter in which such Commercial Milestone Event is achieved. Janssen shall make the corresponding Commercial Milestone Payment to MeiraGTx within [***] following the end of the Calendar Quarter in which such Commercial Milestone Event was achieved.

(c) Commercial Milestone Terms for Janssen Research IRD Products. For the avoidance of doubt, Janssen does not owe MeiraGTx any commercial milestone payments for Research IRD Products.

10.8. Royalties.

In partial consideration of the collaborations, options, licenses and rights granted by MeiraGTx to Janssen under this Agreement, Janssen shall make royalty payments to MeiraGTx, on a Product-by-Product basis, based on Annual Net Sales of the applicable Product within the Field in the Territory, as reported by Janssen or its Affiliates or Sublicensees for each Calendar Quarter, at the applicable rates set forth below during the applicable Royalty Term and subject to Section 10.9, Section 10.10, and where applicable, Section 15.2(a)(ii) (such payments, "**Royalties**").

(a) Clinical IRD Products. Janssen shall pay to MeiraGTx Royalties, on a Clinical IRD Product-by-Clinical IRD Product basis, on Annual Net Sales for each Clinical IRD Product within the Field in the Territory equal to twenty percent (20%) of Annual Net Sales of such Clinical IRD Product in the Territory.

(b) Janssen Research IRD Products. Janssen shall pay to MeiraGTx Royalties, on a Janssen Research IRD Product-by-Janssen Research IRD Product basis, on Annual Net Sales for each such Janssen Research IRD Product within the Field in the Territory equal to [***] percent ([***]%) of Annual Net Sales of such Janssen Research IRD Product in the Territory.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.9. Additional Royalty Provisions.

(a) Royalty Term. Subject to this Section 10.9, on a Product-by-Product and country-by-country basis, the Royalties due under Section 10.8 shall be payable on Annual Net Sales commencing from the First Commercial Sale of such Product in a country until the later of: (i) expiration of the last Valid Claim of: [***] in each case ((A)-(D)) in such country; (ii) ten (10) years from the date of the First Commercial Sale of such Product in such country; or (iii) expiration of the last Data Exclusivity Right applicable to such Product in such country (the "**Royalty Term**").

(b) Royalty Step-Down. For each Product and for any period during the Royalty Term in which the sale of such Product in a given country is neither: (i) Covered by any Valid Claim described in Section 10.9(a)(i); nor (ii) protected by any Data Exclusivity Right applicable to such Product in such country, then the Royalty rate applicable to Net Sales of such Product in such country during such period shall be equal to [***] percent ([***]%) of the applicable Royalty rate set forth in Section 10.8 on Net Sales (*i.e.*, [***] percent ([***]%) for Clinical IRD Products and [***] percent ([***]%) for Janssen Research IRD Products).

(c) Loss of Market Exclusivity of a Product Due to a Biosimilar Product. On a country-by-country basis, if a Loss of Market Exclusivity Due to a Biosimilar Product occurs with respect to a Product in a country, the Royalty rate applicable to Net Sales of such Product in such country shall be equal to [***] percent ([***]%) of the applicable Royalty rate set forth in Section 10.8 on Net Sales from the date such Loss of Market Exclusivity Due to a Biosimilar Product occurs until the end of the Royalty Term (*i.e.*, [***] percent ([***]%) for Clinical IRD Products and [***] percent ([***]%) for Janssen Research IRD Products).

(d) Loss of Market Exclusivity of a Clinical IRD Product Due to a Competing Product. On a country-by-country basis, if a Loss of Market Exclusivity Due to a Competing Product occurs with respect to a Clinical IRD Product in a country, the Royalty rate applicable to Net Sales of such Clinical IRD Product in such country shall be equal to [***] percent ([***]%) of the applicable Royalty rate set forth in Section 10.8 on Net Sales from [***] until [***] (*i.e.*, [***]% for Clinical IRD Products).

(e) Single Royalty. Only a single Royalty shall be due under this Agreement: (i) with respect to the sale of the same unit of Product; and (ii) on the sale of a Product even if the Manufacture or Commercialization of such Product Covered more than one (1) Valid Claim described in Section 10.9(a)(i).

(f) Royalty Minimum. Notwithstanding anything to the contrary in this Agreement, in no event will the applicable Royalty otherwise due to MeiraGTx in a Calendar Quarter be reduced by more than [***] due to the deductions contemplated in this Agreement (*i.e.*, not below [***] for Clinical IRD Products and [***] percent ([***]%) for Janssen Research IRD Products); *provided*, *however*, that [***].

(g) Compulsory Licenses and Other Step-In Rights. If Janssen, its Affiliates or any of its Sublicensees are required to grant any licenses or other rights to a Third Party (including any Governmental Authority) to Develop, Manufacture, or Commercialize a Product because of the actions of any Governmental Authority, then the Royalty rates set forth in Section 10.8 shall not apply, and instead, [***] for each such Product reflecting the applicable market for such Product in such country, subject to [***].

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(h) Combination Products. In the event that a Party reasonably determines that any Product will be incorporated into any other product sold by Janssen or its Affiliates or Sublicensees and such Product will not be priced separately from such other product sold by Janssen or its Affiliates, such Party shall [***] provide written notice thereof to the other Party. Upon receipt of such notice, the Parties shall [***] enter into good faith discussions to determine the reasonable Net Sales value with respect to such Product, which shall be [***].

10.10. Third Party Obligations.

(a) Existing Third Party Obligations. [***] shall remain responsible for the payment of royalty, milestone, and other payment obligations, if any, due to Third Parties in connection with any Third Party License that exists as of the Effective Date and under which MeiraGTx Technology or MeiraGTx Research Technology has been licensed to MeiraGTx and is sublicensed to Janssen under Section 4.1 for Clinical IRD Products and Janssen Research IRD Products (the "**Existing Third Party Obligations**"). All such payments in respect of the Existing Third Party Obligations shall be made [***] in accordance with the terms of its agreements with the applicable Third Party License.

(b) Future Third Party Obligations. If either Party reasonably determines that licensing Future Third Party Obligations would be necessary or useful to Develop, Manufacture, or Commercialize any Product in the Field in the Territory under this Agreement, then MeiraGTx shall have the first right to negotiate and acquire (subject to Janssen's approval; and, for clarity, if MeiraGTx declines to exercise its first right to negotiate and acquire such Future Third Party Obligations or otherwise fails to do so within [***] of any request therefor by Janssen, Janssen shall have the right to do so) a Third Party License to such Future Third Party Obligations. If either Party negotiates and acquires a Third Party License to such Future Third Party Obligations, then: (i) [***]; (ii) [***]; *provided*, *however*, that Janssen shall have the right to [***] for such Product [***] percent ([***]%) of the [***] paid to such Third Party under such Third Party License, and to the extent that Janssen cannot [***] paid to such Third Party exceed any [***] owed to MeiraGTx for such Product at the time of payment to such Third Party, then Janssen may [***] payable for such Product; and (iii) Janssen thereafter shall have the right to [***] up to [***] percent ([***]%) of the [***] subject to the following conditions: (A) Janssen may [***] and [***]; (B) such [***]; (C) if any such Third Party License either: (1) includes additional Intellectual Property Rights other than Intellectual Property Rights that are Controlled by a Third Party and that are necessary or useful to Develop, Manufacture, or Commercialize any Product in the Field in the Territory; or (2) Covers products other than such Product, then any such [***] would be equitably allocated by Janssen in good faith among all products and programs to which such Third Party License applies; and (D) to the extent that Janssen cannot [***], then Janssen may [***].

(c) Breaches, Amendments and Terminations. Neither MeiraGTx nor its Affiliates will breach or default under any Existing Third Party Obligation or any Third Party License for a Future Third Party Obligation. Neither MeiraGTx nor its Affiliates will terminate any such Third Party License in a manner that would terminate any rights that are licensed to Janssen hereunder or otherwise diminish the scope or exclusivity of such Intellectual Property Rights licensed to Janssen hereunder. In the event that MeiraGTx or one of its Affiliates receives notice of a potential dispute in connection with or an alleged breach by MeiraGTx or one of its Affiliates under any such Third Party License, where termination of such Third Party License or any diminishment of the scope or exclusivity of such Intellectual Property Rights licensed to Janssen hereunder is or could be sought by the counterparty, then MeiraGTx will [***], but in no event less than [***] thereafter, provide written notice thereof to Janssen and grant Janssen the right (but not the obligation) to cure such alleged breach, to the extent not prohibited by such Third Party License. In the event that MeiraGTx receives a notice of termination of such a Third Party License by a counterparty, MeiraGTx will [***], but in any event within [***] following receipt of such notice, notify Janssen of such termination in writing. In the event that MeiraGTx or one of its Affiliates intends to materially amend such a Third Party License, then MeiraGTx will [***], but in no event less than [***] before, provide written notice thereof to Janssen. Janssen will have the right (but not the obligation), acting reasonably, to reject any amendment that would increase Janssen's obligations under this Agreement (including any financial obligations) or diminish the licenses or rights granted to Janssen under this Agreement and in the event of any such rejection, neither MeiraGTx nor its Affiliates will enter into any such amendment unless and until Janssen approves such amendment.

10.11. Reports and Royalty Payments.

For as long as Royalties are due under Section 10.8, Janssen shall furnish to MeiraGTx a draft Sales & Royalty Report, within [***] after the end of each Calendar Quarter, showing the estimated amount of Annual Net Sales of Products and Royalties due for such Calendar Quarter. Janssen shall furnish to MeiraGTx a final Sales & Royalty Report and pay such Royalties contained in such final Sales & Royalty Report within [***] following the end of the applicable Calendar Quarter.

10.12. Disclaimer.

MeiraGTx expressly acknowledges and agrees that, despite the efforts and obligations required by this Agreement which may result in the achievement of a Development Milestone Event, Commercial Milestone Event or Net Sales of Product, such event or Net Sales of Product may not be achieved and MeiraGTx, in that case, would not be entitled to receive such further payments hereunder (the Development Milestone Payment, Commercial Milestone Payment or Royalties, as the case may be), other than (i) the Upfront Payment, (ii) Research Costs to be paid as set forth in Section 10.2, (iii) Clinical Development Costs to be paid as set forth in Section 10.3, (iv) Clinical Development Manufacturing Costs to be paid as set forth in Section 10.4, (v) Process Development Costs to be paid as set forth in Section 10.5, (vi) any Option Fee (if and when the respective Option is exercised as set forth in Section 3.9(b)), (vii) such portion of the proceeds of Priority Review Vouchers to be paid to MeiraGTx as set forth in Section 7.1(d) and (viii) such other costs to be paid by Janssen for Manufacturing of Products as set forth in Section 8.1(d). Any Development Milestone Payment, Commercial Milestone Payment or Royalties are contingent upon satisfaction of the conditions provided for herein which may not be satisfied. Except as otherwise expressly provided herein (but without waiving the implied covenant of good faith and fair dealing), Janssen will be under no obligation to use its Commercially Reasonable Efforts, best efforts or any other standard of diligence with respect to satisfying the conditions to any payment hereunder.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

10.13. Payment Terms.

(a) Manner of Payment. All payments to be made by a Party hereunder will be made in Dollars by wire transfer to such bank account as the other Party may timely designate to the other in writing. Any payment which falls due on a date which is not a Business Day in the location from which the payment will be made may be made on the next succeeding Business Day in such location. For the avoidance of doubt, no payment obligations shall be incurred by either Party under or in connection with this Agreement unless and until the Effective Date.

(b) Currency Exchange. With respect to Annual Net Sales invoiced in Dollars, the Annual Net Sales and the amounts due to MeiraGTx under this Agreement shall be expressed in Dollars. When the conversion of payments from any foreign currency is required to be undertaken by Janssen, the Dollar equivalent shall be calculated using Janssen's then-current standard exchange rate methodology as applied in its external reporting for the conversion of foreign currency sales into Dollars.

(c) Invoices. All invoices hereunder to be paid by a Party must reference a valid purchase order ("**PO**") number which the other Party shall provide to such Party within [***] after the Effective Date and invoices shall include the nature and amount of services rendered or deliverables provided and such other terms and conditions specified herein. Invoices to Janssen must be sent to the Johnson & Johnson Accounts Payable Department via [***] if MeiraGTx establishes a web invoice account or sent by postal mail to the address indicated on the PO. Invoices to MeiraGTx must be sent to [***], with a cc to [***]. The other Party reserves the right to return to such Party unprocessed and unpaid those invoices that do not reference the applicable PO number. For clarity, no invoices shall be required with respect to any Royalties due under Section 10.8.

(d) Withholding Taxes. Either Party (the "**Withholding Party**") may withhold from payments due to the other Party (the "**Non-Withholding Party**") amounts for payment of any withholding tax that is required by Applicable Law to be paid to any taxing authority with respect to such payments, which shall be remitted in accordance with Applicable Law. Any such tax required to be withheld will be an expense of and borne by the Non-Withholding Party. If any such tax is assessed against and paid by the Withholding Party, then the Non-Withholding Party will indemnify and hold harmless the Withholding Party from and against such tax, including interest. The Withholding Party shall provide to the Non-Withholding Party all relevant documentation and correspondence, and shall also provide to the Non-Withholding Party any other cooperation or assistance on a reasonable basis as may be necessary to enable the Non-Withholding Party to claim exemption from such withholding taxes and to receive a refund of such withholding tax or claim a foreign tax credit. The Withholding Party shall give proper evidence from time to time as to the payment of any such tax. The Parties shall cooperate with each other in seeking deductions under any double taxation or other similar treaty or agreement from time to time in force. Such cooperation may include the Withholding Party making payments from a single source, where possible.

(e) Tax Forms. On the date of execution of this Agreement, MeiraGTx will deliver to Janssen an accurate and complete Internal Revenue Service Form W-8BEN-E certifying that MeiraGTx is entitled to the applicable benefits under the Income Tax Treaty between the United Kingdom and the United States.

(f) Late Payments. Any undisputed payments or portions thereof due hereunder which are not paid when due will bear interest at the rate per annum equal to the lesser of: [***] or (ii) the highest rate permitted by Applicable Law, calculated on the number of days such payment is paid after the date such payment is due, and compounded monthly (the "**Interest Rate**").

(g) Janssen Entities. [***] acting as paying agent for Janssen, may make certain payments due under this Agreement, and Janssen shall reimburse [***] for all such payments.

10.14. Records and Audits.

(a) Records. Each Party shall keep complete, true, and accurate books and records in accordance with its Accounting Standards in relation to this Agreement, including with respect to Research Costs, Clinical Development Costs, Clinical Development Manufacturing Costs, Process Development Costs, Manufacturing Costs, Net Sales, and such other information contained in Sales & Royalty Reports. Each Party shall keep such books and records for at least [***] following the Calendar Year to which they pertain.

(b) Audits. Each Party (the "**Auditing Party**") may, upon written request, cause an internationally-recognized independent accounting firm (the "**Auditor**"), which is reasonably acceptable to the other Party (the "**Audited Party**"), to inspect the relevant records of such Audited Party and its Affiliates to verify the payments made and amounts reported by the Audited Party and the related reports, statements, and books of accounts, as applicable. Such audit shall be limited to a period of time no more than [***] immediately preceding the year in which the audit is requested, and an audit of the records relating to a particular Calendar Year may be conducted once and not more than once. Before beginning its audit, the Auditor shall execute a written undertaking acceptable to the Audited Party by which the Auditor shall agree to keep confidential all information made available to the Auditor during the audit. Each Party and its Affiliates shall make their records available for inspection by the Auditor during regular business hours at such place or places where such records are customarily kept, upon receipt of reasonable advance notice from the Auditing Party. The records shall be reviewed to verify the accuracy of the Audited Party's Sales & Royalty Report and other payment obligations and compliance with the financial terms of this Agreement. Such inspection right shall not be exercised more than [***], unless the audit reveals a non-compliance by the Audited Party with the terms of this Agreement in which case the audit may be repeated within [***] to confirm compliance. The Auditing Party agrees to hold in confidence all information received and learned in the course of any audit in accordance with Article 12. The Auditor shall provide a draft audit report and basis for any determination to the Audited Party prior to distributing the final report so that the Audited Party can provide comment on the draft report. The final audit report will be provided to the Audited Party at the time such report is provided to the Auditing Party.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(c) Overpayments and Underpayments. If the final result of the inspection reveals an underpayment or an overpayment by either Party, the underpaid or overpaid amount shall be settled within [***] of the Audited Party's receipt of the final report. The Auditing Party shall pay for any audit, as well as its expenses associated with enforcing its rights with respect to any payments under this Agreement, except that, if an underpayment of amounts due by the Audited Party of more than [***] percent ([***]%) of the total payments due under this Agreement for the applicable year is discovered, reasonable and necessary fees and expenses charged by the Auditor shall be paid by the Audited Party, subject to reasonable substantiation thereof.

11. INTELLECTUAL PROPERTY RIGHTS

11.1. Ownership under the Clinical IRD Programs and Research IRD Programs.

(a) Ownership. As between the Parties, and subject to the licenses granted under this Agreement, each Party retains all rights, title, and interests in and to all Intellectual Property Rights that such Party owns or Controls as of the Effective Date or that it develops or otherwise acquires after the Effective Date outside the performance of the activities under this Agreement.

(b) Sole Inventions. Each Party shall solely own any and all Inventions and Know-How developed, conceived, generated or reduced to practice solely by it, its Affiliates or its or their employees, agents or independent contractors in the course of performing activities under any Research Plan or Clinical Development Plan hereunder during the Term ("**Sole Inventions**"). Inventorship shall be determined in accordance with U.S. patent laws.

(c) Joint Inventions. Except as set forth in Section 11.2, the Parties shall jointly own any and all Inventions and Know-How developed, conceived, generated or reduced to practice jointly by a Party, its Affiliates or its or their employees, agents or independent contractors together with another Party, its Affiliates or its or their employees, agents or independent contractors in the course of performing activities under this Agreement during the Term ("**Joint Inventions**"). All Patents Covering Joint Inventions shall be referred to herein as "**Joint Patents**". Except to the extent a Party is expressly limited by the terms of this Agreement, including via exclusive license to the other Party, each Party shall be entitled to practice, license (through multiple tiers), assign (their respective interest only) and otherwise exploit the Joint Inventions and Joint Patents in all countries and jurisdictions without the duty of accounting or seeking consent from the other Party. Upon the reasonable request of either Party, the other Party shall execute documents that evidence or confirm the requesting Party's right to engage in such activities. Inventorship shall be determined in accordance with U.S. patent laws.

11.2. Ownership under the CMC Development Collaboration.

The Parties shall jointly own any and all CMC Development Inventions, CMC Development Know-How and CMC Development Patents regardless of inventorship. Except to the extent a Party is expressly limited by the terms of this Agreement, including by exclusive license to the other Party, each Party shall be entitled to practice, license (through multiple tiers), assign (their respective interest only) and otherwise exploit the CMC Development Inventions, CMC Development Know-How and CMC Development Patents in all countries and jurisdictions without the duty of accounting or seeking consent from the other Party. Upon the reasonable request of either Party, the other Party shall execute documents that evidence or confirm the requesting Party's right to engage in such activities.

11.3. Duties to the Other Party.

(a) Assignment. Each Party hereby assigns to the other Party, one-half of its interest in and to any CMC Development Inventions, CMC Development Know-How and CMC Development Patents, including all rights of action and claims for damages and benefits arising due to past and present infringement of said rights, such that each Party owns an undivided joint interest in and to such CMC Development Inventions, CMC Development Know-How and CMC Development Patents. Each Party shall and shall cause its Affiliates and contractors to, execute and take such further actions reasonably necessary to effectuate such joint ownership in and to such CMC Development Inventions, CMC Development Know-How and CMC Development Patents.

(b) Disclosure. Each Party shall [***] disclose to the JSC all Inventions made by or on behalf of such Party and its Affiliates and subcontractors in the course of performing activities under this Agreement during the Term, including all invention disclosures or other similar documents submitted to such Party by it, its Affiliates or its or their employees, agents or contractors relating to such Invention, and shall also respond to reasonable requests from the other Party for additional information relating to any such Inventions.

(c) Personnel Obligations. Each Party shall cause each employee, agent or contractor of such Party to, and shall cause each of its respective Affiliates or Sublicensees performing work under this Agreement to, prior to commencing such work, be bound by invention assignment obligations, including: (i) [***] reporting any Intellectual Property Right arising from such work; (ii) presently assigning to the applicable Party all of his, her or its rights, title and interests in and to any Intellectual Property Right arising from such work (excluding any agreements with academic universities or other governmental entities, for which a non-exclusive license, or an option for an exclusive license may be obtained); (iii) reasonably cooperating in the preparation, filing, prosecution, maintenance, defense, and enforcement of any Patent; and (iv) performing all acts and signing, executing, acknowledging and delivering any and all documents required for effecting the obligations and purposes of this Agreement.

(d) Common Ownership under Joint Research Agreements. Notwithstanding anything to the contrary in this Agreement, neither Party will have the right to invoke Common Ownership under a Joint Research Agreement pursuant to 35 U.S.C. § 102(c) (AIA) when exercising its rights under this Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned, or delayed. If a Party is permitted to invoke the 35 U.S.C. § 102(c) (AIA) as required by the preceding sentence, the Parties will reasonably cooperate and coordinate their activities with respect to any submissions, filings or other activities in support thereof. The Parties acknowledge and agree that this Agreement is a "joint research agreement" as defined in 35 U.S.C. § 102(c) (AIA).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(e) Updated Exhibit. On each anniversary of the Effective Date during the Term, MeiraGTx shall provide to the JSC an updated copy of Exhibit 1.168, setting forth a true, complete and accurate list of all Patents Controlled by MeiraGTx or any of its Affiliates as of such date, which claim or otherwise Cover any Clinical IRD Products, Clinical IRD Targets or MeiraGTx Know-How in the Field.

11.4. Patent Prosecution and Maintenance.

(a) Sole Patents. Each Party shall have the sole and exclusive right to prosecute and maintain all Patents Covering each Party's Sole Inventions [***], except to the extent the prosecution of such Patents is otherwise covered by another provision of this Section 11.4.

(b) Clinical IRD Product Patents. Subject to the terms of this Section 11.4, the Parties shall jointly prosecute and maintain (i) the MeiraGTx Patents in MeiraGTx's name and (ii) the Joint Patents Covering Clinical IRD Products in both Parties' names (collectively, (i) and (ii), "**Clinical IRD Patents**"). The Parties shall [***]. The Parties shall [***]. Each Party shall be reasonably informed of the status of the Clinical IRD Patents, shall be provided with all material correspondences received from any patent authorities in connection therewith and shall be entitled to consultation and reasonable input with respect to proposed filings and correspondence. In addition, the Parties shall [***] to consult with and consider the reasonable input of [***] with respect to those MeiraGTx Patents licensed from [***] to MeiraGTx. In the event of a dispute, the Parties shall follow the process of Section 5.8.

(c) CMC Development Patents. Subject to the terms of this Section 11.4, as between the Parties, Janssen shall have the first right, but not the obligation, to prosecute and maintain all CMC Development Patents in both Parties' names, subject to consultation and reasonable input from MeiraGTx. The Parties shall [***] and will be joint owners of the CMC Development Patents. The Parties shall [***]. Janssen shall keep MeiraGTx reasonably informed of the status of the CMC Development Patents, shall provide MeiraGTx with all material correspondences received from any patent authorities in connection therewith and shall in good faith consult with and consider reasonable input from MeiraGTx with respect to proposed filings and correspondence. If Janssen decides not to prosecute or maintain any CMC Development Patent, Janssen shall notify MeiraGTx thereof in writing, and MeiraGTx shall thereupon have the right, but not the obligation, to assume the prosecution and maintenance thereof in both Parties' names, [***], and [***]. In the event of a dispute, the Parties shall follow the process of Section 5.8.

(d) Research IRD Product Patents.

(i) *Prior to the Receipt of the Applicable Option Exercise Notice*. Prior to MeiraGTx's receipt of an applicable Option Exercise Notice with respect to a Research IRD Product, subject to the terms of this Section 11.4, as between the Parties, MeiraGTx shall have the first right, but not the obligation, to prosecute and maintain (A) the MeiraGTx Research Patents in MeiraGTx's name and (B) Joint Patents Covering such Research IRD Product in both Parties'

names (collectively, (A) and (B), "**Research IRD Patents**"), [***], subject to consultation and reasonable input from Janssen. MeiraGTx shall keep Janssen reasonably informed of the status of the Research IRD Patents and shall provide Janssen with all material correspondences received from any patent authorities in connection therewith. If MeiraGTx decides not to prosecute or maintain any Research IRD Patent, MeiraGTx shall notify Janssen thereof in writing, and Janssen shall thereupon have the right, but not the obligation, to assume the prosecution and maintenance of such Research IRD Patent [***] (with such MeiraGTx Patents in MeiraGTx's name and such Joint Patents in both Parties' names).

(ii) *After Exercise of the Applicable Option*. On or after Janssen's exercise of an Option with respect to a Research IRD Product, subject to the terms of this Section 11.4, as between the Parties, Janssen shall have the first right, but not the obligation, to prosecute and maintain (A) the MeiraGTx Research Patents Covering such Janssen Research IRD Product in MeiraGTx's name and (B) the Joint Patents Covering such Janssen Research IRD Product in both Parties' names (collectively, (A) and (B), "**Janssen Research IRD Patents**"), subject to consultation and reasonable input from MeiraGTx. [***] shall [***] the cost of and expenses thereof. The Parties shall [***] to facilitate the prosecution and maintenance of such Janssen Research IRD Patents. Janssen shall keep MeiraGTx reasonably informed of the status of the Janssen Research IRD Patents, shall provide MeiraGTx with all material correspondences received from any patent authorities in connection therewith and shall in good faith consult with and consider reasonable input from MeiraGTx with respect to proposed filings and correspondence. If Janssen decides not to prosecute or maintain any Janssen Research IRD Patent, Janssen shall notify MeiraGTx thereof in writing, and MeiraGTx shall thereupon have the right, but not the obligation, to assume the prosecution and maintenance thereof (in MeiraGTx's name with respect to any MeiraGTx Research Patent and both Parties' names with respect to any Joint Patent Covering Janssen Research IRD Products)] [***], and [***] shall [***] the costs and expenses thereof. In addition, each Party shall [***] to consult with and consider the reasonable input of [***] with respect to those MeiraGTx Research Patents licensed from [***] to MeiraGTx and Covering such Janssen Research IRD Product. In the event of a dispute, the Parties shall follow the process of Section 5.8.

(e) Cooperation. Upon a Party's request, the other Party shall provide the prosecuting and maintaining Party with all reasonable assistance and cooperation in connection with its prosecution and maintenance of the applicable Patents, including by providing access to relevant persons and executing all documentation reasonably requested by the prosecuting and maintaining Party.

(f) Patent Term Extension. The Parties shall cooperate with one another in seeking and obtaining patent term extensions (including any pediatric exclusivity extensions as may be available) or supplemental protection certificates or their equivalents in any country with respect to the Clinical IRD Patents, CMC Development Patents, Research IRD Patents and Janssen Research IRD Patents. [***] shall have [***] to obtain patent term extensions or supplemental protection certificates or their equivalents with respect to such Patents that Cover Products and shall report to [***] on the status thereof.

(g) Recordation. To the extent permitted by Applicable Law, if MeiraGTx is responsible for prosecuting any Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent, as applicable, under this Section 11.4, then (i) [***] following the Effective Date, MeiraGTx shall record with each Governmental Authority (where such right is available with such Governmental Authority and such Governmental Authority permits the filing of a form of short notice of license) the licenses granted in Section 4.1 against each such Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent then issued by or filed with such Governmental Authority and being prosecuted by MeiraGTx, as applicable, and (ii) thereafter on a reasonably regular basis as determined by the JSC or upon Janssen's reasonable written request, MeiraGTx shall record with each Governmental Authority (where such right is available with such Governmental Authority and such Governmental Authority permits the filing of a form of short notice of license) the licenses granted in Section 4.1 against each Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent newly filed with such Governmental Authority and being prosecuted by MeiraGTx, as applicable, in each case ((i)-(ii)) by using a form of short notice of license mutually agreed to by the Parties (but shall not, for the avoidance of doubt, file or publicly disclose this Agreement itself for such purposes). To the extent permitted by Applicable Law, if Janssen is responsible for prosecuting any Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent, as applicable, under this Section 11.4, then Janssen shall have the right to record with a Governmental Authority (where such right is available with such Governmental Authority and such Governmental Authority permits the filing of a form of short notice of license) the licenses granted in Section 4.1 against any such Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent then issued by or filed with such Governmental Authority and prosecuted by Janssen, as applicable, by using a form of short notice of license mutually agreed by the Parties (but shall not, for the avoidance of doubt, file or publicly disclose this Agreement itself for such purposes). For clarity, for purposes of this Section 11.4(g), a form of short notice of license may include a short notice form provided by a Governmental Authority or, where permitted by such Governmental Authority, any other short notice of license drafted by the Parties (*e.g.*, a custom short statement that the Parties confirm that certain scheduled Patents are subject to a license grant).

11.5. Third Party Infringement; Patent Actions.

(a) Notice. If either Party becomes aware of: (i) existing or threatened infringement, misappropriation or other violation by a Third Party of any of the MeiraGTx Technology, MeiraGTx Research Technology, Janssen Technology, CMC Development Technology or any Joint Patents ("**Third Party Infringement**"); (ii) request for declaratory judgment, opposition, nullity action, interference, inter partes reexamination, inter partes review, post-grant review, derivation proceeding or similar action alleging the invalidity, unenforceability or non-infringement of any of the MeiraGTx Patents, MeiraGTx Research Patents, Janssen Technology, CMC Development Patents or any Joint Patents (each, a "**Licensed Patent Action**"); or (iii) a BLA for a Biosimilar Product referencing a Product submitted to a Party or a Regulatory Authority, it shall [***] notify the other Party in writing to that effect, and the Parties will consult with each other regarding any actions to be taken.

(b) Enforcement Rights for MeiraGTx Know-How, CMC Development Technology and Clinical IRD Patents.

(i) *Janssen's First Right of Enforcement*. Janssen shall have the first right, but not the obligation, in the case of the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents to bring and control an appropriate suit or other action or defend against any Third Party Infringement or Licensed Patent Action with respect to any MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents. MeiraGTx shall provide reasonable assistance to Janssen in such enforcement or defense, at Janssen's request and expense, including joining such action as a party plaintiff to ensure legal standing if required by Applicable Laws to pursue such action or if requested by Janssen. Janssen shall consult with MeiraGTx and keep MeiraGTx reasonably informed of the status of the enforcement or defense of such MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents. Janssen shall consider MeiraGTx's comments with respect to the enforcement or defense of such MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, as applicable, in good faith. Prior to settling any such suit or action, Janssen shall notify MeiraGTx in writing as to the terms of such proposed settlement to the extent relating to MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents and shall not execute such settlement without MeiraGTx's written consent if MeiraGTx identifies to Janssen in reasonable detail a material risk of a material negative impact on the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, taking into account the potential impact on the value of the Products worldwide as a result of such settlement. If Janssen recovers monetary damages in such claim, suit or action with respect to the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, after Janssen recoups its costs and expenses associated with such litigation, with respect to any portion of such recovery remaining, [***].

(ii) *MeiraGTx's Back-Up Right of Enforcement*. If Janssen does not, within the earlier of [***] after learning about a Third Party Infringement or Licensed Patent Action or [***] before the expiration date for filing an appropriate suit or responding to or taking any action (as applicable), initiate and prosecute any legal action to enforce or defend the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, as applicable, then MeiraGTx shall have the right, but not the obligation, to commence such a suit or take such an action to enforce or defend the applicable MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, as applicable. In such event, Janssen shall take appropriate actions in order to enable MeiraGTx to commence a suit or take the actions set forth in the preceding sentence. Prior to settling any such suit or action, MeiraGTx shall notify Janssen in writing as to the terms of such proposed settlement and shall not execute such settlement without Janssen's written consent if Janssen identifies to MeiraGTx in reasonable detail a material risk of a material negative impact on the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, taking into account the potential impact on the value of the Products worldwide as a result of such settlement. Prior to MeiraGTx commencing such a suit or action, MeiraGTx shall consider in good faith any reasonable Janssen business concerns. If Janssen identifies to MeiraGTx in reasonable detail a material risk of a material negative impact on the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents resulting directly from such a suit or action, taking into account the potential impact on the value of the Products worldwide, then MeiraGTx

shall not commence any such suit or action. If MeiraGTx recovers monetary damages in such claim, suit or action with respect to the MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, any portion of such recovery remaining after MeiraGTx recoups its costs and expenses associated with such litigation, shall be [***].

(c) Enforcement Rights for MeiraGTx Research Know-How and Research IRD Patents.

(i) *Parties' First Right of Enforcement*. Prior to Janssen's exercise of the applicable Option, MeiraGTx shall have the sole right to bring and control an appropriate suit or other action against any Third Party Infringement or Licensed Patent Action with respect to the MeiraGTx Research Know-How or Research IRD Patents; *provided*, *however*, that in the event that any such Third Party Infringement or Licensed Patent Action also affects MeiraGTx Know-How, CMC Development Technology or Clinical IRD Patents, Janssen shall have the first right, but not the obligation, to bring and control such suit or other action pursuant to Section 11.5(b)(i). On or after Janssen's exercise of the applicable Option, Janssen shall have the first right, but not the obligation, in the case of the applicable MeiraGTx Research Know-How and Research IRD Patents to bring and control an appropriate suit or other action against any Third Party Infringement or Licensed Patent Action with respect to such MeiraGTx Research Know-How or Research IRD Patents. MeiraGTx shall provide reasonable assistance to Janssen in such enforcement or defense, at Janssen's request and expense, including joining such action as a party plaintiff to ensure legal standing if required by Applicable Laws to pursue such action or if requested by Janssen. Janssen shall consult with MeiraGTx and keep MeiraGTx reasonably informed of the status of the enforcement or defense of such MeiraGTx Research Know-How and Research IRD Patents. Janssen shall consider MeiraGTx's comments with respect to the enforcement or defense of such MeiraGTx Research Know-How or Research IRD Patents in good faith. Prior to settling any such suit or action, Janssen shall notify MeiraGTx in writing as to the terms of such proposed settlement to the extent relating to MeiraGTx Research Know-How and Research IRD Patents and shall not execute such settlement without MeiraGTx's written consent if MeiraGTx identifies to Janssen in reasonable detail a material risk of a material negative impact on the MeiraGTx Research Know-How or Research IRD Patents, taking into account the potential impact on the value of the Products worldwide as a result of such settlement. If Janssen recovers monetary damages in such claim, suit or action with respect to the MeiraGTx Research Know-How or Research IRD Patents, after Janssen recoups its costs and expenses associated with such litigation, with respect to any portion of such recovery remaining, [***].

(ii) *MeiraGTx's Back-Up Right of Enforcement*. If Janssen does not, within [***] after learning about a Third Party Infringement or Licensed Patent Action or [***] before the expiration date for filing an appropriate suit or responding to or taking any action (as applicable), initiate and prosecute any legal action to enforce or defend the MeiraGTx Research Know-How or Research IRD Patents, as applicable, then MeiraGTx shall have the right, but not the obligation, to commence such a suit or take such an action to enforce the applicable MeiraGTx Research Know-How or Research IRD Patents. In such event, Janssen shall take appropriate actions in order to enable MeiraGTx to commence a suit or take the actions set forth in the preceding sentence. Prior to settling any such suit or action, MeiraGTx shall notify Janssen in

writing as to the terms of such proposed settlement and shall not execute such settlement without Janssen's written consent if Janssen identifies to MeiraGTx in reasonable detail a material risk of a material negative impact on the MeiraGTx Research Know-How or Research IRD Patents, taking into account the potential impact on the value of the Products worldwide as a result of such settlement. Prior to MeiraGTx commencing such a suit or action, MeiraGTx shall consider in good faith any reasonable Janssen business concerns. If Janssen identifies to MeiraGTx in reasonable detail a material risk or a material negative impact on the MeiraGTx Research Know-How or Research IRD Patents resulting directly from such a suit or action, taking into account the potential impact on the value of the Research IRD Products worldwide, then MeiraGTx shall not commence any such suit or action. If MeiraGTx recovers monetary damages in such claim, suit or action with respect to the MeiraGTx Research Know-How or Research IRD Patents, any portion of such recovery remaining after MeiraGTx recoups its costs and expenses associated with such litigation shall be [***].

(d) Cooperation. At the request of the Party bringing and controlling any Third Party Infringement or defending any Licensed Patent Action, as applicable ("**Controlling Party**"), the other Party shall provide reasonable assistance in connection with such action, including by executing reasonably appropriate documents, providing access to such Party's premises and employees, cooperating reasonably in discovery, and joining as a party to the action if requested by the Controlling Party. The Controlling Party will keep the other Party reasonably informed of all material developments in connection with any such suit, and the other Party shall have the right to consult with the Controlling Party and to participate in and, if appropriate, be represented by independent but mutually agreed upon counsel in such litigation at such other Party's own cost and expense.

11.6. Product Infringement.

If a Party becomes aware of any actual or potential Claim alleging that the Research, Development, Manufacture, or Commercialization of any Products under this Agreement infringes, misappropriates, or otherwise violates any Intellectual Property Rights of a Third Party (or would if carried out) ("**Product Infringement**"), then such Party will notify the other Party [***] following the receipt of service of process in such action, suit, or proceeding, or the date on which such Party becomes aware that such action, suit, or proceeding has been instituted, and the Parties will meet as soon as possible to discuss the overall strategy for defense of such matter. Janssen shall have the first right (but not the obligation) to defend any Claims of Product Infringement. If Janssen does not intend to defend such Product Infringement or determines to cease defending any such Product Infringement and informs MeiraGTx that it wishes for MeiraGTx to defend such Product Infringement, then MeiraGTx will have the right, but not the obligation, upon written notice to Janssen, to defend such Product Infringement or take over defense of the Product Infringement from Janssen, as applicable. Notwithstanding anything to the contrary, if either Party has an obligation to indemnify the other Party with respect to such Claim of Product Infringement, then the provisions of Article 17 will apply with respect thereto.

11.7. Patents Licensed From Third Parties.

Each Party's rights under this Article 11 with respect to the prosecution and maintenance, enforcement, and defense of any Patent that is licensed from a Third Party shall be subject to the rights retained by such Third Party with respect to such Patent.

11.8. Trademarks.

Janssen shall have the right to brand any and all Product(s) using Janssen related Trademarks it determines appropriate for such Product(s), which may vary by country or within a country ("**Product Marks**"). Subject to Section 15.4(c)(iii), Janssen shall own all rights in Product Marks and shall have the sole right to register and maintain Product Marks in the countries and regions it determines reasonably necessary, and Janssen shall have the sole right, in its discretion and at its expense, to defend and enforce such Product Marks.

12. CONFIDENTIALITY

12.1. Duty of Confidence.

Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the Parties, the Parties agree that, during the Term and for [***] after the expiration or earlier termination of this Agreement, subject to the other provisions of this Article 12, each Party will, as a receiving Party (the "**Receiving Party**"), and will cause its Affiliates to, maintain in confidence, not publish or otherwise disclose and otherwise safeguard, any and all Confidential Information disclosed by or on behalf of the other Party (the "**Disclosing Party**") or its Affiliates under this Agreement to it, using such degree of care that such Party uses with respect to its own confidential information (which shall in no event be less than a reasonable degree of care). During the Term and for [***] after the expiration or earlier termination of this Agreement, subject to the other provisions of this Article 12, the Receiving Party may only use such Confidential Information for the purposes of this Agreement and in connection with the performance of its obligations or exercise of rights granted or reserved in this Agreement.

12.2. Exceptions.

The obligations under this Article 12 shall not apply to any information to the extent that such information:

(a) is (at the time of disclosure) or becomes (after the time of disclosure) known to the public or part of the public domain through no breach of this Agreement by the Receiving Party or its Affiliates;

(b) was known to, or was otherwise in the possession of, the Receiving Party or its Affiliates, as evidenced by written records of the Receiving Party and its Affiliates kept in the ordinary course of business, prior to the time of disclosure by the Disclosing Party or any of its Affiliates;

(c) is disclosed to the Receiving Party or any of its Affiliates on a non-confidential basis by a Third Party who is entitled to disclose it without breaching any confidentiality obligation to the Disclosing Party or any of its Affiliates; or

(d) is independently developed by or on behalf of the Receiving Party or its Affiliates outside of its performance under this Agreement, as evidenced by written records of the Receiving Party and its Affiliates kept in the ordinary course of business, without the use of the Confidential Information disclosed by the Disclosing Party or its Affiliates to the Receiving Party or its Affiliates under this Agreement.

Specific aspects or details of Confidential Information shall not be deemed to be within the public domain or in the possession of the Receiving Party merely because the Confidential Information is embraced by more general information in the public domain or in the possession of the Receiving Party. Further, any combination of Confidential Information shall not be considered in the public domain or in the possession of the Receiving Party merely because individual elements of such Confidential Information are in the public domain or in the possession of the Receiving Party, unless the combination and its principles are in the public domain or in the possession of the Receiving Party.

12.3. Authorized Disclosures.

(a) Disclosures. Except as expressly permitted by this Agreement, in addition to disclosures allowed under Section 12.2 or Section 12.4, the Receiving Party and its Affiliates may only disclose to Third Parties the Disclosing Party's Confidential Information to the extent such disclosure is necessary in the following instances: (i) in connection with the performance of its obligations or exercise of rights granted or reserved in this Agreement, under non-disclosure and non-use provisions no less restrictive than those in this Agreement; (ii) in connection with the prosecution and maintenance of Patents as permitted by this Agreement; (iii) in connection with Regulatory Filings or audits by Regulatory Authorities for any Product; (iv) in connection with prosecuting or defending litigation as permitted by this Agreement; (v) in complying with applicable court orders or governmental regulations (including securities regulations); (vi) in the case of any Party, in communication with its employees, directors, officers, agents, contractors, consultants, and professional advisers; Affiliates; potential or actual collaborators, partners, and licensees (including potential co-marketing and co-promotion contractors); and potential or actual investment bankers, acquirers, lenders or investors, each of the foregoing whom, on a need-to-know basis and prior to disclosure, must be bound by similar obligations of confidentiality and non-use no less restrictive than those contained in this Article 12; (vii) as permitted in accordance with Article 13; or (viii) as mutually agreed to in writing by the Parties.

(b) Disclosures pursuant to Applicable Law. If the Receiving Party is required to disclose Confidential Information of the Disclosing Party pursuant to Applicable Law (including the rules of the Securities and Exchange Commission or any stock exchange) or in connection with any bona fide legal process, including disclosures of the type contemplated by Section 12.3(a), such disclosure to the extent reasonably necessary shall not be deemed a breach of this Agreement; *provided*, *however*, that the Receiving Party, except where reasonably impracticable or legally impermissible, will: (i) inform the Disclosing Party as soon as reasonably practicable following it becoming aware of the required disclosure; (ii) limit the disclosure to the required purpose; and (iii) at the Disclosing Party's request and reasonable expense, assist in attempting to object to, limit or seek to secure confidential treatment of the required disclosure.

12.4. Terms of this Agreement.

The Parties acknowledge and agree that this Agreement and all of the respective terms of this Agreement shall be treated as Confidential Information of each Party, subject to Section 13.5.

12.5. Use of Residuals.

Residuals shall not be considered Confidential Information of MeiraGTx and the restrictions set forth in this Article 12 shall not apply to the use of Residuals.

12.6. Data Privacy.

(a) Privacy and Data Security Laws. Without limiting a Party's other obligations under this Agreement, each Party shall, and shall cause its Affiliates and permitted subcontractors to, implement and maintain reasonable security procedures and practices appropriate to the nature of Sensitive Information such Party is processing and take such other actions as are reasonably necessary to protect the security and confidentiality of such Sensitive Information against any threats or hazards to the security or integrity of such Sensitive Information in accordance with Privacy and Data Security Laws.

(b) Data Breaches. If a Party learns of any security breach involving Sensitive Information of the other Party collected, prepared or developed in connection with this Agreement, such Party shall immediately notify the owning Party of the same, and shall, at the owning Party's expense, reasonably cooperate with the owning Party in investigating and responding to the foregoing. The Party whose Sensitive Information has been breached (or allegedly breached) shall have the sole right to determine the content, timing and other details of any notices to affected individuals or Governmental Authorities in connection with such breach.

13. PUBLICATIONS AND PUBLICITY

13.1. Use of Names.

Except as otherwise expressly permitted herein or to the extent required pursuant to Section 13.5, neither Party may use the names or Trademarks of the other Party or its Affiliates for any purpose, including in any press release, publication, or other form of public disclosure, without first obtaining, in each case, the prior written consent of the other Party (such consent not to be unreasonably withheld, conditioned, or delayed), except for those disclosures for which consent has already been obtained or which are required by Applicable Law. Notwithstanding the foregoing, Janssen shall be entitled to use the name of MeiraGTx and its Affiliates to the extent necessary under Applicable Law in connection with the Development or Commercialization of any Product.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

13.2. Press Releases and Publicity Related to this Agreement.

The Parties shall agree on a form of a press release that may be issued by each, if desired, on or shortly following the Execution Date. Thereafter, except to the extent required to comply with Applicable Law or otherwise pursuant to Section 13.5, neither Party shall issue any press releases or other public statements, whether oral or written, disclosing the existence of this Agreement, or the terms hereof, without first obtaining, in each case, the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned, or delayed. Notwithstanding the foregoing, to the extent information in connection with this Agreement has already been publicly disclosed in accordance with this Agreement, either Party (or its Affiliates) may subsequently disclose the same information to the public without the consent of the other Party.

13.3. Public Disclosures and Publications Related to the Programs or Products.

Except to the extent required pursuant to Section 13.5, and subject to this Section 13.3, each Party shall not issue any press releases or other public statements, whether oral or written, regarding the Research Plan Activities, Research IRD Targets, Research IRD Products, CMC Development Plan Activities, Clinical Development Plan Activities, Clinical IRD Products and Clinical IRD Targets, without first obtaining, in each case, the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned, or delayed. MeiraGTx shall be permitted to provide public statements with respect to certain Clinical IRD Products pursuant to obligations incurred prior to the Execution Date; *provided* that Janssen has actual knowledge as of the Execution Date of such obligations; and *provided further* that such public statements shall be approved by Janssen prior to their release (such approval not to be unreasonably withheld, conditioned, or delayed). In addition, and notwithstanding the foregoing, on or after the respective Sponsorship Transfer Date with respect to any Clinical IRD Product and on or after the respective Option Election Date with respect to any Janssen Research IRD Product, Janssen may publish a press release or other public statement about such Clinical IRD Product or Janssen Research IRD Product, as applicable; *provided* that Janssen shall provide MeiraGTx with a copy of any such press release or public statement reasonably in advance of such publication and consider in good faith any comments timely provided by MeiraGTx to Janssen with respect to such press release or public statement, as applicable.

13.4. Publications.

(a) Review Process. The Parties may wish to publish or present the results of the programs or Products hereunder. Either Party may publish the results or observations of its work on a program or Product hereunder after providing the other Party [***] to review the proposed publication, abstract or presentation ("**Review Period**") to determine whether the proposed publication, abstract or presentation contains any Confidential Information of the non-submitting Party. If within the Review Period, the non-submitting Party determines that the proposed publication, abstract or presentation contains any such Confidential Information, the non-submitting Party will notify the submitting Party in writing of the presence of the Confidential Information in the proposed publication, abstract or presentation and the submitting Party will delete such Confidential Information from the proposed publication, abstract or presentation. If, within the Review Period, the non-submitting Party identifies material in such publication, abstract

or presentation which, if published, would disclose an Invention of the non-submitting Party that has not yet been protected through the filing of a Patent or would adversely affect the Intellectual Property Rights owned by the non-submitting Party, then at the non-submitting Party's election, (i) the submitting Party will delay submission of its publication, abstract or presentation for an additional period, not to exceed [***] or such other period mutually agreed to by the Parties, in order to allow for the filing of a Patent or other appropriate Intellectual Property Rights protection for such material or (ii) the submitting Party will remove the identified material prior to publication.

(b) Acknowledgments. Proper acknowledgement will be made for contributions, if any, of each Party to the results or other information and material disclosed in any publication, abstract or presentation under this Section 13.4. Authorship of scientific publications will be determined in accordance with customary academic and industry policies and procedures, the scientific contribution, and the standards of the applicable journal.

13.5. Disclosures Required By Law.

Notwithstanding Section 13.1, Section 13.2, and Section 13.3, each Party may make any disclosures required to comply with any duty of disclosure it may have pursuant to Applicable Law or the requirements of any Governmental Authority or Regulatory Authority or pursuant to the rules of any recognized stock exchange (including the rules of the Securities and Exchange Commission). If a disclosure required by Applicable Law, the requirements of any Governmental Authority or Regulatory Authority, or the rules of any recognized stock exchange arises, the Parties shall coordinate with each other with respect to the timing, form, and content of such required disclosure. If so requested by the other Party, except where impracticable or not legally permitted, the Party subject to such obligation shall use commercially reasonable efforts to obtain an order protecting the confidentiality of such provisions of this Agreement, as determined by the Disclosing Party in consultation with its legal counsel. Without limiting the foregoing, MeiraGTx shall use commercially reasonable efforts to provide Janssen with each proposed filing by MeiraGTx with the United States Securities and Exchange Commission (or any recognized stock exchange, including Nasdaq, or any similar regulatory agency in any country other than the United States) describing the terms of this Agreement (including any filings of this Agreement) at least [***] prior to submission of such filing, and shall seek confidential treatment of portions of this Agreement or such terms or information as may be reasonably requested by Janssen in a timely manner.

13.6. Publication and Transparency of Clinical Research Results.

All research results of clinical trials hereunder will be posted on clinicalstudyresults.org and on any other registry with requirements consistent with the registration and publication guidelines of the International Committee of Medical Journal Editors, to the extent required. All data and information posted on clinicaltrial.gov, clinicalstudyresults.org or any other registry will be subject to prior review by the other Party pursuant to Section 13.4(a). All research results generated from clinical trials hereunder may be made available to Third Parties pursuant to data transparency policies and mechanisms of Janssen or its Affiliates then in effect.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

14. EFFECTIVENESS

14.1. Effective Date.

Except for the Parties' obligations under Article 12, Article 13 and this Article 14, which shall be effective as of the Execution Date, this Agreement shall not become effective until the first date (the "**Effective Date**") on which (a) expiration or early termination of all applicable waiting periods under the HSR Act has occurred, (b) each of the representations of MeiraGTx set forth in Section 16 are true and correct in all material respects, (c) an officer of MeiraGTx has delivered a written certificate with respect to (b) to Janssen, (d) an updated copy of Exhibit 1.168, setting forth a true, complete and accurate list of all Patents Controlled by MeiraGTx or any of its Affiliates as of the Effective Date, which claim or otherwise Cover any Clinical IRD Products, Clinical IRD Targets or MeiraGTx Know-How in the Field, has been delivered to Janssen and (e) [***] has entered into a binding commitment with MeiraGTx to provide to Janssen the right to undertake a back-up license under [***]'s license grant to MeiraGTx; *provided*, *however*, that Janssen may waive the conditions described in clause (b), (c), (d) or (e) above.

14.2. Filings.

The Parties shall cooperate with one another in the preparation and execution of all documents that are required to be filed pursuant to the HSR Act. Each Party shall, within [***] following the Execution Date (or such later time as may be agreed to in writing by the Parties), file with the United States Federal Trade Commission and the Antitrust Division of the United States Department of Justice, and/or with equivalent foreign authorities, any HSR Filing required of it in the reasonable opinion of either Party with respect to the transactions contemplated hereby. Neither Party shall seek expedited treatment of any HSR Filing without the other Party's prior written consent. Each Party will use reasonable efforts to do, or cause to be done, all things necessary, proper and advisable to, [***] take all actions necessary to make the filings required of such Party or its Affiliates under the HSR Act. The Parties shall cooperate with one another to the extent necessary in the preparation of any such HSR Filing. Each Party shall be responsible for its own costs, expenses, and filing fees associated with any HSR Filing; *provided*, *however*, that Janssen shall be solely responsible for any fees (other than penalties that may be incurred as a result of actions or omissions on the part of MeiraGTx) required to be paid to any Governmental Authority in connection with making any such HSR Filing.

14.3. Outside Date.

This Agreement shall terminate (a) at the election of either Party, immediately upon written notice to the other Party, if the U.S. Federal Trade Commission or the U.S. Department of Justice, or an equivalent authority in the European Union, seeks a preliminary injunction under applicable antitrust and non-competition laws against Janssen and MeiraGTx to enjoin the transactions contemplated by this Agreement; or (b) at the election of either Party, immediately upon written notice to the other Party, in the event that the HSR Clearance Date shall not have occurred on or prior to one hundred eighty (180) days after the effective date of the HSR Filing. In the event of such termination, this Agreement shall be of no further force and effect.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

15. TERM AND TERMINATION

15.1. Term.

The term of this Agreement shall commence upon the Effective Date and, unless terminated pursuant to Section 15.2, shall continue in full force and effect, on a Product-by-Product and country-by-country basis, until such time as the Royalty Term expires in such country (the "**Term**"). On a Product-by-Product and country-by-country basis, effective upon the expiration of the Royalty Term for such Product in such country (but not for any termination of this Agreement), the licenses granted to Janssen will each become fully paid-up, royalty-free, irrevocable, and perpetual in such country with respect to such Product.

15.2. Termination.

This Agreement may be terminated as follows:

(a) Termination for Breach.

(i) *General*. Subject to Section 15.2(a)(ii), if either Janssen or MeiraGTx is in material breach of this Agreement, the non-breaching Party must give written notice to the breaching Party within [***] following the alleged breach specifying the claimed particulars of such breach in sufficient detail to put the allegedly breaching party on reasonable notice of the nature of the alleged breach, and in the event such material breach is not cured within [***] after such notice (or in the case of any undisputed payment obligations, [***] after such notice), the non-breaching Party shall have the right thereafter to terminate this Agreement immediately, in whole or with respect to the applicable Product (except with respect to a material breach by Janssen arising under Section 6.1(e), Section 6.2(a), Section 8.4(a), Article 9 and Section 19.4, which must be terminated on a Product-by-Product basis), by giving written notice to the breaching Party to such effect; *provided*, *however*, that if such breach is capable of being cured but cannot be cured within the period referenced above and the breaching Party initiates actions to cure such breach within such period and thereafter diligently pursues such actions, the breaching Party shall have such additional period as is reasonable under the circumstances to cure such breach; it being understood that no such extension shall apply with respect to any undisputed payment obligations. If arbitration is commenced with respect to any alleged breach hereunder pursuant to Section 18.1, no purported termination of this Agreement pursuant to this Section 15.2(a)(i) shall take effect until: (A) a final resolution of such arbitration; and (B) the losing Party in such arbitration fails to satisfy the arbitration award within the cure time frames referenced above.

(ii) *Janssen Special Remedy*. If Janssen would have the right to terminate this Agreement under Section 15.2(a)(i), in whole or in part, for an uncured material breach by MeiraGTx in connection with a Product, then Janssen may, in its sole discretion, elect: (A) to either exercise such termination right or (B) in lieu of exercising such termination right, and without limiting Janssen's rights otherwise set under this Agreement, subsequently reduce any payment amounts due to MeiraGTx under [***] for such Product by [***] percent ([***]%) as a result of this Section 15.2(a)(ii).

(b) Termination for Insolvency. This Agreement may be immediately terminated in its entirety by a Party by providing written notice of termination to the other Party in the event of an Insolvency Event of the other Party.

(c) Termination for Patent Challenge. To the extent permitted by Applicable Law, if Janssen or any of its Affiliates Challenges any Patent included in the MeiraGTx Patents, MeiraGTx Research Patents or Joint Patents in any country in the Territory (such Patent, a "**Challenged Patent**"), then MeiraGTx may, following written notice to Janssen and *provided that* Janssen or its Affiliate does not withdraw such Challenge within [***] after receipt of such notice, terminate this Agreement in its entirety by providing written notice of termination to Janssen; *provided that* this Section 15.2(c) will not apply to, and MeiraGTx may not terminate this Agreement with respect to (i) any claim or proceeding that would otherwise be a Challenge hereunder to the extent commenced by a Third Party that after the Effective Date acquires or is acquired by Janssen or its Affiliates or its or their business or assets, whether by stock purchase, merger, asset purchase, or otherwise; *provided that* such proceeding commenced prior to the closing of such acquisition; (ii) any claim or proceeding by a licensor of a product licensed by Janssen for which the licensor has an existing challenge, whether in a court or administrative proceeding, against any MeiraGTx Patent, MeiraGTx Research Patent or Joint Patent; (iii) any Challenge required to be commenced pursuant to an order of a Governmental Authority or Applicable Law; (iv) any Challenge that is commenced by a Sublicensee; *provided that* Janssen demands that such Sublicensee withdraw such Challenge [***] after Janssen becomes aware of such Challenge and terminates the sublicense agreement with the applicable Sublicensee if such Sublicensee does not withdraw such Challenge within [***] after receipt of such demand from Janssen; (v) any proceeding not initiated, directed or controlled by or on behalf of Janssen or one of its Affiliates, for which Janssen or the Affiliate, as the case may be, opposes, or assists any Third Party to oppose, the grant of a MeiraGTx Patent, MeiraGTx Research Patent or Joint Patent pursuant to any application in relation thereto in an administrative proceeding, such as a patent re-examination, *inter partes* review, or other post grant proceeding or opposition; (vi) challenges by an open forum entity or other industry group in which Janssen or its Affiliates or Sublicensees do not direct or control the action of such entity; (vii) general activities not specifically directed to a particular Patent, such as amicus briefs on cases not involving any MeiraGTx Patent, MeiraGTx Research Patent or Joint Patent; (viii) lobbying or other efforts directed to patent issues generally and not to any specific Patent; (ix) any affirmative defense or other validity, enforceability, or non-infringement challenge, whether in the same action or in any other agency or forum of competent jurisdiction, advanced by Janssen, any of its Affiliates or Sublicensees in response to any claim or action brought in the first instance by, or on behalf of, MeiraGTx or any Third Party; (x) providing documents or testimony in response to any discovery requests or court order in a valid legal process not directed to Challenging any Challenged Patent; or (xi) any Challenge related to any Clinical IRD Patent, CMC Development Patent, Research IRD Patent or Janssen Research IRD Patent for which Janssen's reasonable input with respect to such Patent's prosecution, maintenance or enforcement pursuant to Section 11.4 or Section 11.5 was not included in any filings or correspondence with patent authorities or otherwise accepted or pursued by MeiraGTx (as applicable).

(d) Termination by Janssen At Will. Beginning on [***], Janssen may terminate this Agreement at will in its entirety or on a Product-by-Product basis at any time on: (i) [***] prior written notice to MeiraGTx, if prior to the First Commercial Sale of such Product; and (ii) on [***] prior written notice to MeiraGTx, if following the First Commercial Sale of such Product.

15.3. Rights in Insolvency.

The Parties agree that this Agreement constitutes an executory contract under Section 365 of the Code for the license of "intellectual property" as defined under Section 101 of the Code and constitutes a license of "intellectual property" for purposes of any similar laws in any other country in the Territory. The Parties further agree that Janssen, as licensee of such rights under this Agreement, will retain and may fully exercise all of its protections, rights and elections under the Code, including under Section 365(n) of the Code, and any similar laws in any other country in the Territory and that Janssen cannot be compelled to accept a money satisfaction of its interests in the intellectual property licensed pursuant to this Agreement, and that any such sale therefore may not be made to a purchaser "free and clear" of Janssen's rights under this Agreement and Section 365(n) without the express, contemporaneous consent of Janssen. The Parties further agree that, in the event of an Insolvency Event by or against MeiraGTx under the Code and any similar laws in any other country in the Territory, Janssen may be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property (including Materials and Research Results), and the same, if not already in its possession, will be [***] delivered to it: (a) upon any such commencement of an Insolvency Event upon its written request therefor, unless MeiraGTx elects to continue to perform all of its obligations under this Agreement; or (b) if not delivered under clause (a) above, following the rejection of this Agreement by or on behalf of MeiraGTx upon written request therefor by Janssen. Whenever MeiraGTx or any of its successors or assigns provides to Janssen any of the intellectual property licensed hereunder (or any embodiment thereof including Materials and Research Results) pursuant to this Section 15.3, Janssen shall have the right to perform MeiraGTx's obligations hereunder with respect to such intellectual property, but neither such provision nor such performance by Janssen shall release MeiraGTx from liability resulting from rejection of the license or the failure to perform such obligations. MeiraGTx and Janssen each acknowledges and agrees that Option Fees, Development Milestone Payments, Commercial Milestone Payments and Royalties constitute royalties within the meaning of Section 365(n) of the Bankruptcy Code.

All rights, powers and remedies of Janssen provided herein are in addition to and not in substitution for any and all other rights, powers and remedies now or hereafter existing at law or in equity (including the Bankruptcy Code) in the event of the commencement of a case under the Bankruptcy Code with respect to MeiraGTx. The Parties agree that they intend the following rights to extend to the maximum extent permitted by law, and to be enforceable under Bankruptcy Code Section 365(n):

(i) the right of access to any intellectual property rights (including all embodiments thereof) of MeiraGTx licensed to Janssen hereunder, or any Third Party with whom MeiraGTx contracts to perform an obligation of MeiraGTx under this Agreement, and, in the case of the Third Party, which is necessary for the Manufacture, use, sale, import or export of Products; and

(ii) the right to contract directly with any Third Party to complete the contracted work.

15.4. Effects of Expiration or Termination.

(a) Licenses. Upon any expiration or termination of this Agreement after the Effective Date, in whole or in part, any licenses granted by a Party under Section 4.1 with respect to a Terminated Product will terminate, except to the extent necessary for Janssen to perform any of its obligations that survive such termination, to conduct wind-down activities in compliance with applicable legal and ethical obligations, or to the extent set forth in Section 4.1(c) or Section 15.1, and each of the Parties' respective obligations with respect to such Terminated Product shall terminate, except as set forth in Section 15.1, Section 15.4, Section 15.5 and Section 15.6. For clarity, upon any termination in part of this Agreement, the licenses granted under Article 4 shall survive with respect to the Products not affected by such partial termination.

(b) Confidential Information. Upon any expiration or termination of this Agreement, in whole or in part, each Party will destroy all written, electronic, or other materials containing Confidential Information of the other Party, including all copies thereof relating to such Terminated Product within [***] of such termination and provide certification of such destruction to the owning Party; *provided*, that: (i) a Party may retain one (1) copy of any materials containing Confidential Information of the other Party in its archives solely for the purpose of monitoring its ongoing confidentiality obligations hereunder; and (ii) a Party will not be obligated to destroy such materials containing Confidential Information of the other Party that are necessary for the Party to exercise any other license or right of such Party that survives such termination of this Agreement; *provided*, *however*, that the receiving Party's use of such Confidential Information will continue to be subject to the requirements and restrictions set forth in Article 12.

(c) Wind-Down Activities. Upon any termination of this Agreement, in whole or in part, by MeiraGTx for Janssen's uncured material breach under Section 15.2(a), by MeiraGTx under Section 15.2(b) or Section 15.2(c), or by Janssen under Section 15.2(d), then the following shall occur:

(i) Janssen hereby grants to MeiraGTx, with respect to the Terminated Product(s), a non-exclusive, perpetual and irrevocable right and license, with the right to grant sublicenses (through multiple tiers), under Janssen's Sole Inventions as necessary to Develop, Manufacture and Commercialize such Terminated Product(s) in the Field in the Territory;

(ii) Effective as of such termination date, Janssen hereby assigns, and shall [***] transfer (at MeiraGTx's reasonable expense, unless MeiraGTx terminated this Agreement, in whole or in part, for Janssen's uncured material breach under Section 15.2(a), in which case such transfer shall be at Janssen's reasonable expense) on an as-is, where-is basis, to MeiraGTx or MeiraGTx's designee possession and ownership of all governmental or regulatory correspondence, conversation logs, filings and approvals (including all Regulatory Approvals and Pricing Approvals), and global safety database, in each case relating to the Development, Manufacture or Commercialization of the Terminated Product(s) and to the extent permitted under Applicable Law, and Janssen shall reasonably cooperate, at no additional out-of-pocket cost to Janssen, with requests by MeiraGTx for assistance necessary to facilitate MeiraGTx's assumption of regulatory responsibilities for the Terminated Product(s) in the applicable countries in which direct transfer is not permitted during the [***] following such termination date;

(iii) Effective as of such termination date, Janssen hereby assigns (and shall ensure that its Affiliates or Sublicensees hereby assign) to MeiraGTx all Trademarks used solely with respect to the Terminated Product(s) in the Territory (together with the goodwill associated with the foregoing); and

(iv) Effective as of such termination date, Janssen shall [***] provide MeiraGTx with a summary of all Third Party agreements relating to the Development, Manufacture or Commercialization of the Terminated Product(s) to which Janssen is a party (other than those to be assigned to MeiraGTx) and shall assign to MeiraGTx any Third Party agreements solely relating to the Development, Manufacture or Commercialization of the Terminated Product(s), and MeiraGTx shall accept such assignment, including responsibility for all liabilities and obligations accruing under such agreements from and after the date of termination, in each case to the extent permitted thereunder. With respect to each agreement relating to the Development, Manufacture or Commercialization of Terminated Product(s) that is not transferred to MeiraGTx, at MeiraGTx's request, Janssen shall use reasonable efforts to facilitate a direct introduction between MeiraGTx and the Third Party counterparty to such agreement.

(d) Clinical Studies. If Janssen is conducting any Clinical Study on the date of notice of termination for any reason under this Agreement, then MeiraGTx shall notify Janssen within [***] after the notice of termination with regard to any Clinical Study, whether MeiraGTx elects to have Janssen (i) complete such Clinical Study on behalf of MeiraGTx (unless Janssen reasonably believes there is a material safety issue that should prevent the continuation of such Clinical Study), (ii) wind down such Clinical Study as soon as practicable, subject to compliance with ethical and legal requirements, or (iii) transfer such Clinical Study to MeiraGTx as soon as practicable. Notwithstanding the foregoing, if Janssen terminates this Agreement pursuant to Section 15.2(a) or 15.2(b), then this Section 15.4(d) shall not apply and Janssen, at its sole discretion, shall determine whether to continue or wind down any ongoing Clinical Study, subject to compliance with ethical and legal requirements; and each Party shall bear its own expenses incurred pursuant to such wind down. In addition, and subject to the foregoing:

(i) If MeiraGTx notifies Janssen of its election to have Janssen complete a Clinical Study on behalf of MeiraGTx, Janssen and MeiraGTx will, as necessary, negotiate in good faith a separate agreement pursuant to which Janssen would complete such Clinical Study. If the Parties fail to reach agreement within [***] after MeiraGTx makes such election, Janssen may wind down such Clinical Study, subject to compliance with ethical and legal requirements or, if requested by MeiraGTx, transfer such Clinical Study to MeiraGTx.

(ii) If MeiraGTx notifies Janssen of its election to have Janssen wind down such Clinical Study (or fails to provide notice within such [***] period), then Janssen shall wind-down such Clinical Study as soon as practicable, subject to compliance with ethical and legal requirements.

(iii) If MeiraGTx notifies Janssen of its election to have Janssen transfer such Clinical Study to MeiraGTx, then Janssen shall use commercially reasonable efforts to transfer, and MeiraGTx shall use commercially reasonable efforts to assume, such Clinical Study as [***] as practicable (and, in any event, within [***]) after the effective date of termination.

(iv) The costs of ongoing Clinical Studies under this Section 15.4(d) shall be borne as follows:

(A) By [***] after the effective date of termination, if [***]. [***] shall reimburse [***] for any such costs incurred by [***] after the effective date of termination.

(B) By [***] after the effective date of termination, if [***]. [***] shall reimburse [***] after the effective date of termination.

(C) By [***] as follows after the effective date of termination, if [***]: (x) by [***] until the earlier of (i) completion of such wind-down or (ii) [***] after the effective date of termination for such Clinical Study for such Clinical IRD Product; and (y) by [***] until the completion of such wind-down; *provided* that both Parties shall cooperate to wind-down such Clinical Study for such Clinical IRD Product as soon as reasonably practicable, including through discussions with the applicable Regulatory Authorities to minimize the compliance and legal requirement period for such wind-down.

(D) By [***] after the effective date of termination, if [***] until the completion of such wind-down.

(e) Further Actions. Janssen shall take all such further actions within the [***] following such termination of this Agreement, in whole or in part, as may be reasonably requested by MeiraGTx in order to give effect to the foregoing clauses in this Section 15.4, [***].

(f) Rights Terminated. Except as set forth in Section 15.1, Section 15.4, Section 15.5 and Section 15.6, the rights and obligations of the Parties hereunder shall terminate as of the date of such termination of this Agreement, in whole or in part, with respect to the Terminated Product(s). For clarity, termination of this Agreement, in whole or part, will not affect a Party's rights to freely exploit CMC Development Inventions or CMC Development Patents in accordance with Article 11. In addition, upon any expiration or termination of this Agreement after the Effective Date, in whole or in part, each Party shall not undertake any further actions under this Agreement following such expiration or termination except in accordance with this Section 15.4. Subject to Section 15.2(a)(ii), Section 15.4(d) and Section 15.5, in the event of any expiration or termination of this Agreement, in whole or in part, (i) Janssen will pay all amounts then due and owing to MeiraGTx in accordance with Article 10 as of the effective date of such expiration or termination; (ii) each Party will use reasonable efforts to mitigate and avoid any costs incurred by or on behalf of such Party on or after such date of expiration and termination, to the extent to be reimbursed by the other Party in accordance with the express terms herewith (including under Section 15.4(d)(iv)(C)); and (iii) no further payment amounts shall be due to MeiraGTx with respect to the Terminated Product(s) after the effective date of such expiration or termination.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

15.5. Post-Termination Royalties to Janssen.

Following termination of this Agreement, in whole or in part, with respect to a Product, MeiraGTx shall be obligated to pay Janssen a royalty on Net Sales of such Product in the amount of: (a) [***] percent ([***]%) of Net Sales if a Phase 2 Study has not been initiated for such Product as of the effective date of termination; (b) [***] percent ([***]%) of Net Sales if a Phase 2 Study has been initiated as of the effective date of termination but a Pivotal Study has not been initiated for such Product as of the effective date of termination; (c) [***] percent ([***]%) of Net Sales if a Pivotal Study has been initiated for such Product as of the effective date of termination but Pricing Approval in at least one (1) country has not been obtained for such Product as of the effective date of termination; or (d) [***] percent ([***]%) of Net Sales if Pricing Approval in at least one (1) country has been obtained for such Product as of the effective date of termination. The royalty reporting, payment, record-keeping, audit and withholding tax provisions set forth in Sections 10.8, 10.9, 10.11, 10.13(d) and 10.14 and "Royalty Term" definition set forth in Section 10.9(a) shall apply *mutatis mutandis* to royalties payable pursuant to this Section 15.5. For purposes of this Section 15.5, "Net Sales" shall have the meaning given to it in Section 1.176, as if the references to Janssen in such definition were to MeiraGTx.

15.6. Survival.

Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination. Subject to the other terms and conditions regarding the termination and survival of obligations under this Agreement in the event of expiration or termination of this Agreement, upon expiration or termination of this Agreement, all provisions of this Agreement will cease to have any effect, except that the following provisions will survive any such expiration or termination for any reason for the period of time specified therein, or if not specified, then they will survive indefinitely: Article 1 (Definitions), Section 3.5 (Research Records), Section, 3.7(c) (Final Report), Section 4.1(c) (Internal Research License), Section 4.7 (No Other Rights), Section 6.1(g) (Clinical Development Records), Section 10.1 (Upfront Payment) (only with respect to the last sentence), Section 10.11 (Reports and Royalty Payments), Section 10.12 (Disclaimer), Section 10.13 (Payment Terms), Section 10.14 (Records and Audits), Section 11.1 (Ownership under the Clinical IRD Programs and Research IRD Programs), Section 11.2 (Ownership under the CMC Development Collaboration), Section 11.3 (Duties to the Other Party), Section 11.8 (Trademarks) (only with respect to the second sentence), Article 12 (Confidentiality), Section 13.1 (Use of Names), Section 13.2 (Press Releases and Publicity Related to this Agreement), Section 13.4 (Publications) (*provided*, *however*, that Section 13.4 shall not survive in the event of any expiration or termination of this Agreement in its entirety), Section 13.5 (Publications Required by Law), Section 14.3 (Outside Date), Section 15.1 (Term), Section 15.3 (Rights in Insolvency), Section 15.4 (Effects of Expiration or Termination), Section 15.5 (Post-Termination Royalties to Janssen), Section 15.6 (Survival), Section 15.7 (Termination Not Sole Remedy), Article 17 (Indemnification; Liability; Insurance), Article 18 (Dispute Resolution) and Article 19 (General Provisions).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

15.7. Termination Not Sole Remedy.

Termination is not the sole remedy under this Agreement and, whether or not termination is effected and notwithstanding anything contained in this Agreement to the contrary, all other remedies will remain available except as agreed to otherwise herein, and except that termination shall be MeiraGTx's sole remedy with respect to any failure by Janssen to discharge its obligations under Section 6.1(e), Section 6.2(a), Section 8.4(a) and Article 9.

16. REPRESENTATIONS, WARRANTIES AND COVENANTS

16.1. Representations and Warranties by Each Party.

Each Party hereby represents and warrants to the other Party, that as of the Execution Date and the Effective Date:

(a) such Party is a company duly organized, validly existing, and in good standing under the laws of its jurisdiction of formation or incorporation;

(b) such Party has full power and authority to execute, deliver, and perform this Agreement, and has taken all action required by Applicable Law and its organizational documents to authorize the execution and delivery of this Agreement by such Party and the performance of all obligations of such Party as contemplated by this Agreement;

(c) this Agreement constitutes a legal, valid, and binding agreement enforceable against such Party in accordance with its terms;

(d) the person or persons executing this Agreement on such Party's behalf have been duly authorized to do so by all requisite corporate action;

(e) all consents, approvals and authorizations from all Governmental Authorities or other Third Parties required to be obtained by such Party in connection with entering into this Agreement have been obtained, except in the case of the Execution Date as required pursuant to the HSR Act; and

(f) the execution and delivery of this Agreement and all other instruments and documents required to be executed pursuant to this Agreement, the performance of such Party's obligations hereunder and the licenses, options, rights of negotiation and other rights to be granted by such Party pursuant to this Agreement, and the consummation of the transactions contemplated hereby do not and shall not: (i) conflict with or result in a breach of any provision of its organizational documents; (ii) conflict with, violate, result in a breach or constitute a default under any contractual obligations of such Party or any of its Affiliates existing as of the Execution Date or Effective Date, including with respect to applicable Third Party Licenses; or (iii) conflict with or violate any requirements of Applicable Laws existing as of the Execution Date or Effective Date and applicable to such Party.

16.2. Representations, Warranties and Covenants by MeiraGTx.

In addition to the representations and warranties made by MeiraGTx in Section 16.1 above and Section 16.3 below, MeiraGTx hereby represents, warrants and covenants to Janssen that, (1) as of the Execution Date, (2) except as otherwise expressly provided, the Effective Date, and (3) solely with respect to Sections 16.2(a), (b), (d), (f), (g), (i), (k), (l), and (m), and solely with respect to the [***] Product as a Research IRD Product, as of the Option Election Date:

(a) MeiraGTx has, and will have during the Term of this Agreement, the full right, power and authority to grant the licenses, exclusivity, options, right of first negotiation and other rights granted to Janssen hereunder, including under (i) the MeiraGTx Patents and MeiraGTx Know-How; and (ii) to MeiraGTx's knowledge, the MeiraGTx Research Patents and MeiraGTx Research Know-How that each Cover Research IRD Products;

(b) MeiraGTx has not entered, and during the Term, will not enter, into any written agreement that grants or would grant to any Affiliate or Third Party, including any academic organization or agency or other Person, any rights to (i) any Clinical IRD Target or Clinical IRD Product; or (ii) to MeiraGTx's knowledge, any Research IRD Product or related Research IRD Target, or that otherwise conflicts or would conflict with the rights granted to Janssen hereunder or MeiraGTx's ability to fully perform its obligations hereunder, apart from those rights set forth in Exhibit 16.2(b);

(c) Exhibit 1.168 sets forth a true, complete and accurate list of all Patents Controlled by MeiraGTx or any of its Affiliates as of the Execution Date, which claim or otherwise Cover any Clinical IRD Products, Clinical IRD Targets or MeiraGTx Know-How in the Field, and each such updated copy of Exhibit 1.168 provided by MeiraGTx to Janssen as of the Effective Date and to the JSC on each anniversary of the Effective Date thereafter sets forth a true, complete and accurate list of all Patents Controlled by MeiraGTx or any of its Affiliates as of such respective dates, which claim or otherwise Cover any Clinical IRD Products, Clinical IRD Targets or MeiraGTx Know-How in the Field.

(d) the MeiraGTx Technology comprises all of the Intellectual Property Rights used by MeiraGTx and its Affiliates and consultants in the Research, Development and Manufacturing of the Clinical IRD Products prior to the Execution Date, and no MeiraGTx [***] Technology is used by MeiraGTx or its Affiliates or consultants, or is otherwise necessary, for the Research, Development, Manufacture or Commercialization of Clinical IRD Products;

(e) to MeiraGTx's knowledge, (i) the MeiraGTx Research Technology comprises all of the Intellectual Property Rights used by MeiraGTx and its Affiliates and consultants in the Research, Development and Manufacturing of Research IRD Products prior to the Execution Date, and (ii) no MeiraGTx [***] Technology is used by MeiraGTx or its Affiliates or consultants, or is otherwise necessary, for the Research, Development, Manufacture or Commercialization of Research IRD Products;

(f) neither MeiraGTx nor its Affiliates is or was engaged in the Commercialization of any Clinical IRD Product or Research IRD Product on or prior to the Execution Date;

(g) Exhibit 16.2(g) sets forth a complete and accurate list of all Existing Third Party Obligations as of the Execution Date, and besides Existing Third Party Obligations as set forth in Exhibit 16.2(g), neither MeiraGTx nor any of its Affiliates is subject to any royalty, milestone or other payment obligation to any Third Party in connection with the practice, or the grant of rights to Janssen to practice, (i) any of the MeiraGTx Technology, including with respect to the Commercialization of Clinical IRD Products under this Agreement and (ii) to MeiraGTx's knowledge, any of the MeiraGTx Research Technology, including with respect to the Commercialization of Research IRD Products;

(h) MeiraGTx has provided Janssen with complete, true and accurate copies of all agreements in effect between MeiraGTx and [***] relating to this Agreement;

(i) MeiraGTx has not materially breached, or received any notice of breach or default under, any Existing Third Party Obligation, and each Existing Third Party Obligation is valid, binding and in full force and effect;

(j) Exhibit 4.4 sets forth a complete and accurate list of all subcontractors that MeiraGTx has existing relationships with as of the Execution Date, such subcontractors' activities or obligations in connection with this Agreement and if applicable, any MeiraGTx commitments specifically requiring either of the Parties to engage any such subcontractors in connection with any Clinical Development Plan Activities or Research Plan Activities on or after the Effective Date;

(k) to MeiraGTx's knowledge, the Research, Development, Manufacture and Commercialization of the Products, do not, or will not, infringe or misappropriate the Intellectual Property Rights of any Third Party, and MeiraGTx or any of its Affiliates or licensees or Sublicensees of any MeiraGTx Technology or MeiraGTx Research Technology Covering Research IRD Products have not received any written notice alleging such infringement or misappropriation, except that [***];

(l) there are no claims, judgments, orders, decrees, or settlements against or owed by MeiraGTx or any of its Affiliates, and there is no written action or proceeding of any nature, civil, criminal, regulatory or otherwise, pending or, to the knowledge of MeiraGTx, threatened against MeiraGTx or any of its Affiliates, in each case that would prevent MeiraGTx from performing its obligations under this Agreement or from granting the licenses and other rights set forth hereunder;

(m) none of MeiraGTx, its Affiliates, or licensees or Sublicensees of any MeiraGTx Technology, have initiated or been involved in any proceeding or other Claims in which it alleges that any Third Party is or was infringing or misappropriating any (i) MeiraGTx Technology or (ii) to MeiraGTx's knowledge, MeiraGTx Research Technology Covering Research IRD Products, nor have any such proceedings been threatened by MeiraGTx, its Affiliates, or licensees or Sublicensees, nor does MeiraGTx or its Affiliates know of any valid basis for any such proceedings; and

(n) MeiraGTx has provided Janssen with true, accurate and complete copies of all agreements listed in Exhibit 16.2(b) and Exhibit 16.2(g) attached hereto (including amendments and addendums to such agreements).

16.3. Mutual Covenants.

(a) Compliance. Each Party shall comply, and shall cause its Affiliates, subcontractors and Sublicensees to comply, in all material respects with all Applicable Laws applicable to the Development, Manufacture and Commercialization of Products and performance of its obligations under this Agreement, including, to the extent applicable, the statutes, regulations and written directives of the FDA (including cGCP, cGLP, and cGMP), the EMA and any Regulatory Authority having jurisdiction in the Territory, the FD&C Act, the Prescription Drug Marketing Act, the Federal Health Care Programs Anti-Kickback Law, 42 U.S.C. § 1320a-7b(b), the statutes, regulations and written directives of Medicare, Medicaid and all other health care programs, as defined in 42 U.S.C. § 1320a-7b(f), Anti-Corruption Laws, Privacy and Data Security Laws, Trade Control Laws and U.S. Export Control Laws.

(b) No Debarred Person. In the course of the Research, Development, Manufacture and Commercialization of the Products, neither Party nor its Affiliates, Sublicensees or subcontractors shall use any employee, officer, director, agent or consultant who is or has been a Debarred Person, or, to such Party's or its Affiliate's knowledge, is the subject of debarment proceedings by a Regulatory Authority. Each Party shall notify the other Party [***] in writing upon becoming aware that any person performing services under this Agreement has become a Debarred Person or is the subject of debarment proceedings or pending or threatened debarment proceedings by any Regulatory Authority.

(c) Know-How and IP Protection. All of a Party's employees and other Persons acting on such Party's behalf, in each case with access to Know-How and Inventions as contemplated herein, are obligated to maintain the confidentiality of such Know-How and Inventions and not use such Know-How or Inventions for any purpose inconsistent with the terms and conditions of this Agreement. All of a Party's employees and other Persons performing activities hereunder on behalf of such Party in accordance herewith will be obligated to assign all rights, title and interests in and to any Inventions developed by them in connection with such activities, whether or not patentable, to such Party.

16.4. No Other Warranties.

EXCEPT AS EXPRESSLY STATED HEREIN, (A) NO REPRESENTATION, CONDITION OR WARRANTY WHATSOEVER IS MADE OR GIVEN BY OR ON BEHALF OF JANSSEN OR MEIRAGTX; AND (B) ALL OTHER CONDITIONS AND WARRANTIES WHETHER ARISING BY OPERATION OF LAW OR OTHERWISE ARE HEREBY EXPRESSLY EXCLUDED, INCLUDING ANY CONDITIONS AND WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE OR NON-INFRINGEMENT.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

17. INDEMNIFICATION; LIABILITY; INSURANCE

17.1. Indemnification by MeiraGTx.

MeiraGTx shall indemnify, defend and hold Janssen, its Affiliates and Sublicensees, and their respective officers, directors, employees and agents ("**Janssen Indemnitees**") harmless from and against any Damages arising out of or resulting from any Claims of Third Parties against them to the extent arising or resulting from:

(a) the negligence, gross negligence or willful misconduct of any MeiraGTx Indemnitee or contractor in connection with this Agreement;

(b) the breach of any of the covenants, agreements, warranties or representations made by MeiraGTx to Janssen under this Agreement;

(c) MeiraGTx's Research activities with respect to any Research IRD Products prior to the respective Option Election Date, including pursuant to Section 2.2(a) and Section 3.3;

(d) MeiraGTx's Research or Development activities with respect to the [***] Product unless and until Janssen elects to make the [***] Product a Clinical IRD Product pursuant to Section 2.1(b);

(e) MeiraGTx's Research or Development activities with respect to any Clinical IRD Product, including its Clinical Development Plan Activities pursuant to Section 6.1(c) and Section 6.1(e), and, prior to the Sponsorship Transfer Date, regulatory activities pursuant to Section 7.1(a); or

(f) MeiraGTx's Manufacturing activities with respect to any Product, including Manufacturing Products pursuant to Section 8.1, any Supply Agreement or any Quality Agreement, and conducting CMC Development Plan Activities pursuant to Section 8.4(a);

except that MeiraGTx shall not be obliged to so indemnify the Janssen Indemnitees for any Claim to the extent that Janssen has an obligation to indemnify MeiraGTx Indemnitees pursuant to Section 17.2 for such Claim.

17.2. Indemnification by Janssen.

Janssen shall indemnify, defend and hold MeiraGTx, its Affiliates and Sublicensees, and their respective officers, directors, employees and agents ("**MeiraGTx Indemnitees**") harmless from and against any Damages arising out of or resulting from any Claims of Third Parties against them to the extent arising or resulting from:

(a) the negligence, gross negligence or willful misconduct of any Janssen Indemnitee or contractor in connection with this Agreement;

(b) the breach of any of the covenants, agreements, warranties or representations made by Janssen to MeiraGTx under this Agreement;

(c) Janssen's Research, Development or Commercialization activities with respect to any Janssen Research IRD Product pursuant to Section 2.2(a), Section 6.2(a), Section 7.2(a) or Article 9;

(d) Janssen's Development or Commercialization activities with respect to any Clinical IRD Product, including conducting its Clinical Development Plan Activities pursuant to Section 6.1(c) and Section 6.1(e), regulatory activities after [***] pursuant to Section 7.1(a) and Commercialization activities pursuant to Article 9; or

(e) Janssen's Manufacturing activities with respect to any Product, including Manufacturing Products pursuant to the applicable Supply Agreements and conducting its CMC Development Plan Activities pursuant to Section 8.4(a);

except that Janssen shall not be obliged to so indemnify the MeiraGTx Indemnitees for any Claim to the extent that MeiraGTx has an obligation to indemnify Janssen Indemnitees pursuant to Section 17.1 for such Claim.

17.3. Indemnification Procedure.

(a) For the avoidance of doubt, all indemnification claims in respect of a Janssen Indemnitee or MeiraGTx Indemnitee shall be made solely by Janssen or MeiraGTx, respectively.

(b) A Party seeking indemnification hereunder (the "**Indemnified Party**") shall notify the other Party (the "**Indemnifying Party**") in writing reasonably [***] after the assertion against the Indemnified Party of any Claim or fact in respect of which the Indemnified Party intends to base a claim for indemnification hereunder (an "**Indemnification Claim Notice**"); *provided*, that the failure or delay to so notify the Indemnifying Party shall not relieve the Indemnifying Party of any obligation or liability that it may have to the Indemnified Party, except to the extent that the Indemnifying Party demonstrates that its ability to defend or resolve such Claim is adversely affected thereby. The Indemnification Claim Notice shall contain a description of the Claim and the nature and amount of the Claim (to the extent that the nature and amount of such Claim is known at such time). Upon the request of the Indemnifying Party, the Indemnified Party shall furnish [***] to the Indemnifying Party copies of all correspondence, communications, and official documents (including court documents) received or sent in respect of such Claim.

(c) Subject to Section 17.3(d) and Section 17.3(e), the Indemnifying Party shall have the right, upon written notice given to the Indemnified Party, to assume the defense and handling of such Claim, at the Indemnifying Party's sole expense, in which case Section 17.3(d) shall govern. The assumption of the defense of a Claim by the Indemnifying Party shall be construed as acknowledgement that the Indemnifying Party is liable to indemnify any Indemnitee with respect to the Claim, and shall constitute a waiver by the Indemnifying Party of any defenses it may assert against any Indemnified Party's claim for indemnification. Until the Indemnifying Party gives such written notice to the Indemnified Party, Section 17.3(e) shall govern. An Indemnifying Party will not have the right to conduct the defense of any Claim (a) brought by a Governmental Authority, (b) where such Claim involves injunctive relief (which cannot reasonably be severed from the proceeding for damages) or (c) where the Indemnified Party reasonably believes that the Indemnifying Party does not have the financial resources to discharge its indemnification obligation in full, unless the Indemnifying Party posts a bond to cover such potential indemnification obligations.

(d) Upon assumption of the defense of a Claim by the Indemnifying Party: (i) the Indemnifying Party shall have the right to and shall assume sole control and responsibility for defending and handling the Claim; (ii) the Indemnifying Party may, at its own cost, appoint as counsel in connection with conducting the defense and handling of such Claim any law firm or counsel selected by the Indemnifying Party which is reasonably acceptable to the Indemnified Party; (iii) the Indemnifying Party shall keep the Indemnified Party informed of the status of such Claim; and (iv) the Indemnifying Party shall have the right to settle such Claim on any terms the Indemnifying Party chooses; *provided*, *however*, that it shall not, without the prior written consent of the Indemnified Party (such consent not to be unreasonably withheld, conditioned, or delayed), agree to a settlement of any Claim which could lead to liability or create any financial or other obligation on the part of the Indemnified Party for which the Indemnified Party is not entitled to indemnification under this Agreement or which admits any wrongdoing or responsibility for the Claim on behalf of the Indemnified Party or which involves a conduct restriction or obligation of any kind. The Indemnified Party shall cooperate with the Indemnifying Party and shall be entitled to participate in, but not control, the defense of such Claim with its own counsel and at its own expense. In particular, the Indemnified Party shall furnish such records, information, and testimony, provide witnesses, and attend such conferences, discovery proceedings, hearings, trials, and appeals as may be reasonably requested in connection therewith. Such cooperation shall include access during normal business hours by the Indemnifying Party to, and reasonable retention by the Indemnified Party of, records and information that are reasonably relevant to such Claim, and making the Indemnified Party, the Indemnitees, and its and their employees and agents available on a mutually convenient basis to provide additional information and explanation of any records or information provided.

(e) If the Indemnifying Party does not assume the defense of the Indemnified Party in accordance with Section 17.3(c), the Indemnified Party may, at the Indemnifying Party's expense, select counsel reasonably acceptable to the Indemnifying Party in connection with conducting the defense and handling of such Claim and defend or handle such Claim in such manner as it may deem appropriate. In such event, the Indemnified Party shall keep the Indemnifying Party reasonably informed of the status of such Claim and shall not settle such Claim without the prior written consent of the Indemnifying Party, which consent shall not be unreasonably withheld, conditioned, or delayed. If the Indemnified Party defends or handles such Claim, the Indemnifying Party shall cooperate with the Indemnified Party, at the Indemnified Party's request but at no expense to the Indemnified Party, and shall be entitled to participate in the defense and handling of such Claim with its own counsel and at its own expense.

(f) Neither Party shall have the obligation to indemnify the other Party in connection with any settlement made without the Indemnifying Party's written consent, which consent shall not be unreasonably withheld, conditioned or delayed.

17.4. Mitigation of Loss.

Each Indemnified Party will take and will procure that the other Janssen Indemnitees (where Janssen is the Indemnified Party) or the MeiraGTx Indemnitees (where MeiraGTx is the Indemnified Party) take all such reasonable steps and actions in order to mitigate any potential Damages under this Article 17.

17.5. Limited Liability.

NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY FOR ANY SPECULATIVE OR PUNITIVE DAMAGES OF ANY KIND ARISING FROM OR RELATING TO THIS AGREEMENT, THE NEGOTIATION OF THIS AGREEMENT OR THE ACTIVITIES CONDUCTED HEREUNDER, OR ANY OTHER DAMAGES NOT REASONABLY FORESEEABLE AS A PROXIMATE RESULT OF THE BREACH OF A PARTY OF THIS AGREEMENT, ON ANY THEORY OF LIABILITY (WHETHER IN CONTRACT, TORT, STRICT LIABILITY OR OTHERWISE), REGARDLESS OF ANY NOTICE, AWARENESS OR ADVICE OF THE LIKELIHOOD OF SUCH DAMAGES. NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS SECTION 17.5 IS INTENDED TO OR SHALL LIMIT OR RESTRICT INDEMNIFICATION FOR DAMAGES OF THE NATURE DESCRIBED ABOVE THAT ARE PAYABLE TO A THIRD PARTY FOLLOWING A FINAL ADJUDICATION OF THE MATTER IN QUESTION OR A SETTLEMENT EFFECTED IN ACCORDANCE WITH THIS AGREEMENT.

17.6. Insurance Obligations

Each Party, at its own expense, shall procure and maintain during the Term and for a period of [***] thereafter product liability insurance adequate to cover the activities to be conducted by such Party and its obligations under this Agreement that are consistent with normal business practices of prudent companies similarly situated; *provided*, *however*, that in no event shall such product liability insurance be written in amounts less than [***] per claim or per occurrence and annual aggregate. All such insurance shall include worldwide coverage. Prior to the initiation of any Clinical Study of a Product, the Party responsible for such Clinical Study shall secure, and maintain in full force and effect, clinical trial insurance as required by Applicable Law in those territories where such Clinical Study shall be conducted. It is understood that such insurance shall not be construed to create a limit of either Party's liability with respect to its indemnification obligations under Section 17.1 and Section 17.2. Each Party shall provide the other Party with written evidence of such insurance upon request. Each Party shall provide the other Party with written notice at least [***] prior to the cancellation, non-renewal or material change of such insurance that could materially adversely affect the rights of such other Party hereunder. Notwithstanding the foregoing, either Party's failure to maintain adequate insurance shall not relieve that Party of its obligations set forth in this Agreement. The Parties acknowledge and agree that Janssen may meet its obligations under this Section 17.6 through self-insurance consistent with the levels set forth herein with prior written notice to MeiraGTx. In such event, Janssen shall provide a written certification of such self-insurance to MeiraGTx upon request.

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18. DISPUTE RESOLUTION

18.1. Dispute Resolution.

(a) Escalation; Decision-Making Authority. In the case of any dispute, claim or controversy between the Parties arising from or related to this Agreement, or the interpretation, application, breach, termination or validity of this Agreement and which are not subject to Section 5.8(c) (a "**Dispute**"), the Parties will discuss and negotiate in good faith a solution acceptable to the Parties and in the spirit of this Agreement. If, after negotiating in good faith pursuant to the foregoing sentence, the Parties fail to reach agreement within [***] (or such longer period as agreed in writing by the Parties), then the Dispute may be referred to the Executive Officers for resolution at the request of either Party. If, after negotiating in good faith, the Executive Officers fail to reach agreement within [***] of submission to the Executive Officers (or such longer period as agreed in writing by the Parties), then either Party may upon written notice to the other submit the Dispute to non-binding mediation pursuant to Section 18.1(b).

(b) Mediation.

(i) If the Parties fail to resolve the Dispute pursuant to Section 18.1(a), the Parties shall attempt in good faith to resolve any Dispute by confidential mediation in accordance with the then-current Mediation Procedure of the International Institute for Conflict Prevention and Resolution ("**CPR Mediation Procedure**") (www.cpradr.org) before initiating arbitration. The CPR Mediation Procedure shall control, except where it conflicts with these provisions, in which case these provisions control. The mediator shall be chosen pursuant to CPR Mediation Procedure. The mediation shall be held in New York, New York.

(ii) Either Party may initiate mediation by written notice to the other Party of the existence of a Dispute. The Parties agree to select a mediator within [***] of the notice and the mediation will begin [***] after the selection. The mediation will continue until the mediator, or either Party, declares in writing, no sooner than after the conclusion of [***] of a substantive mediation conference attended on behalf of each Party by a senior business person with authority to resolve the Dispute, that the Dispute cannot be resolved by mediation. In no event, however, shall mediation continue more than [***] from the initial notice by a Party to initiate meditation unless the Parties agree in writing to extend that period.

(iii) Any period of limitations that would otherwise expire between the initiation of mediation and its conclusion shall be extended until [***] after the conclusion of the mediation.

(c) Arbitration. If the Parties fail to resolve the Dispute pursuant to Section 18.1(a) or Section 18.1(b), and a Party desires to pursue resolution of the Dispute, subject to Section 18.5, the Dispute shall be resolved by final and binding arbitration as follows:

(i) The Dispute shall be finally resolved by arbitration in accordance with the then-current CPR Rules for Non-Administered Arbitration Rules ("**CPR Rules**") (www.cpradr.org), except where they conflict with these provisions, in which case these provisions control. The place of arbitration shall be New York, New York. All aspects of the arbitration shall be treated as confidential.

(ii) Whenever a Party decides to institute such arbitration proceedings, such Party shall as [***] as practicable, give written notice to that effect to the other Party. The law of this arbitration clause shall be the law of New York, USA, the seat and venue of the arbitration shall be New York, USA, and the arbitration proceedings shall be conducted in the English language. The arbitrators will be chosen from the CPR Panel of Distinguished Neutrals, unless a candidate not on such panel is approved by both Parties. Each arbitrator shall be a lawyer with at least fifteen (15) years' experience with a law firm or corporate law department of over twenty-five (25) lawyers or who was a judge of a court of general jurisdiction. There shall be three (3) arbitrators for the Dispute, selected as follows: each Party shall select an arbitrator in accordance with the "screened" appointment procedure provided in CPR Rule 5.4, and the chair will be chosen in accordance with CPR Rule 6.4. If, however, the aggregate award sought by the Parties is less than [***] and equitable relief is not sought, a single arbitrator shall be appointed in accordance with the CPR Rules. Candidates for the arbitrator position(s) may be interviewed by representatives of the Parties in advance of their selection, *provided* that all Parties are represented.

(iii) The hearing on the merits shall be concluded within [***] after the initial prehearing conference and the award shall be rendered within [***] of the conclusion of the hearing, or of any post-hearing briefing, which briefing shall be completed by both sides within [***] after the conclusion of the hearing, unless the arbitrator(s) determine(s), in a reasoned decision, that the interest of justice or the complexity of the case requires that the time limit for concluding the hearing on the merits or rendering the award be extended. In the event the Parties cannot agree upon a schedule, then the arbitrator(s) shall set the schedule following the time limits set forth above as closely as practical.

(iv) The hearing on the merits will be concluded in [***] or less, unless the arbitrator(s) determine(s), in a reasoned decision, that the interest of justice or the complexity of the case requires that the time limit for concluding the hearing on the merits or rendering the award be extended. Multiple hearing days will be scheduled consecutively to the greatest extent possible. A transcript of the testimony adduced at the hearing shall be made and shall be made available to each Party.

(v) The Parties shall allow and participate in discovery in accordance with the United States Federal Rules of Civil Procedure. Unresolved discovery disputes shall be submitted to the arbitrator(s).

(vi) The arbitrator(s) shall decide the merits of any Dispute in accordance with the law governing this Agreement, without application of any principle of conflict of laws that would result in reference to a different law. The arbitrator(s) may not apply principles such as "*amiable compositeur*" or "*natural justice and equity*."

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

(vii) The arbitrator(s) are expressly empowered to decide dispositive motions in advance of any hearing and shall endeavor to decide such motions as would a United States District Court Judge sitting in the jurisdiction whose substantive law governs.

(viii) The arbitrator(s) shall render a written opinion stating the reasons upon which the award is based. The Parties consent to the entry of judgment on any award rendered hereunder. Judgment on the award may be entered in any court of competent jurisdiction.

(ix) The award of the arbitral tribunal shall be final and binding upon the Parties, and the prevailing Party may apply to a court of competent jurisdiction for enforcement of such award. When any Dispute occurs and when any Dispute is under arbitration, except for the matters in Dispute, the Parties shall continue to fulfill their respective obligations and shall be entitled to exercise their rights under this Agreement. Except in a proceeding to enforce the results of the arbitration or as otherwise required by Applicable Law, neither Party nor any arbitrator may disclose the existence, content or results of any arbitration hereunder without the prior written consent of both Parties. Notwithstanding anything to the contrary contained in this Agreement, each Party to the Dispute shall be entitled to seek provisional remedies, interim measures of protection and emergency relief, if possible, such as attachment, preliminary injunction, replevin or other equitable relief, from the arbitral tribunal or any court of competent jurisdiction in accordance with the Applicable Laws of that jurisdiction in order to avoid irreparable harm, maintain the status quo, preserve its status and priority as a creditor or preserve the subject matter of the Dispute.

(d) Waivers. EACH PARTY HERETO WAIVES: (I) ITS RIGHT TO TRIAL OF ANY ISSUE BY JURY AND (II) ANY CLAIM FOR ATTORNEY FEES, COSTS AND PREJUDGMENT INTEREST; *PROVIDED, HOWEVER*, THAT THE FOREGOING WILL NOT LIMIT A PARTY'S OBLIGATIONS IN RESPECT OF DAMAGES CLAIMED BY A THIRD PARTY.

18.2. Governing Law.

This Agreement shall be governed by and construed under the laws of the State of New York, without giving effect to the conflicts or choice of laws provision thereof ("**Governing Law**"). The United Nations Convention on Contracts for the International Sale of Goods (1980) shall not apply to the interpretation of this Agreement.

18.3. Exclusions.

Nothing in this Article 18 shall preclude a Party from: (a) seeking and obtaining in any competent court injunctive or equitable relief to preserve the status quo or prevent immediate harm to the Party; or (b) submitting any dispute, controversy or Claim relating to the scope, validity, enforceability or infringement of any Patents or Trademarks to adjudication in accordance with the Applicable Laws of the country or jurisdiction in which the relevant patent is pending or has been issued, including before any patent or trademark administrative body in the country in which such Patent or Trademark was granted or arose. The Parties agree that the venue of any such adjudication involving a patent pending in or issued by the United States will be a U.S. federal

district court sitting in New York, New York or an appellate body of such court, and for a patent pending in or issued by any other country, any competent court having jurisdiction over the subject of the Patent controversy or Claim sitting in the capital of such country (or if there is not any such competent court in the capital, a location reasonably proximate to the capital), and each Party hereby irrevocably submits to the jurisdiction of such courts or administrative bodies.

18.4. Cumulative Remedies.

Unless expressly stated otherwise in this Agreement, no remedy referred to in this Agreement is intended to be exclusive, but each shall be cumulative and in addition to, and not in lieu of, any other remedy referred to in this Agreement or otherwise available to either Party under Applicable Law.

18.5. Injunctive Relief.

Notwithstanding anything to the contrary set forth in this Agreement, the Parties each stipulate and agree that given the nature of the Confidential Information and Intellectual Property Rights and the competitive damage and irreparable harm that may result to a Party upon unauthorized disclosure, use or transfer of its Confidential Information or Intellectual Property Rights to any Third Party, monetary damages may not be a sufficient remedy for any breach of Section 4.4, Article 11, Article 12 or Article 13 by a Party. In the case of any such breach or threatened breach, in addition to all other remedies, the non-breaching Party will be entitled to seek equitable relief (including temporary or permanent restraining orders, specific performance or other injunctive relief) for any such breach or threatened breach from any court of competent jurisdiction without first submitting to the dispute resolution procedures set forth in Section 18.1.

19. GENERAL PROVISIONS

19.1. Assignment.

Subject to Section 4.6(d) and Section 4.6(e), neither Party may assign its rights or obligations under this Agreement, in whole or in part, without the other Party's prior written consent, except that either Party may assign this Agreement, in whole or in part, without the other Party's consent to: (a) an Affiliate of the assigning Party; or (b) any Third Party successor or purchaser of all or substantially all of its business or assets to which this Agreement relates, whether in a merger, sale of stock, sale of assets or other similar transaction. In addition, Janssen may, without the prior written consent of MeiraGTx, assign its rights and obligations, in whole or in part, under this Agreement to a Third Party, where Janssen or its Affiliate is required, or makes a good faith determination based on advice of counsel, to divest any Products in order to comply with Applicable Law or the order of any Governmental Authority as a result of a merger or acquisition or similar transaction. In any such case, Janssen shall provide MeiraGTx with advance notice of any such assignment. Any permitted assignee will assume all obligations of its assignor under this Agreement (or related to the assigned portion in case of a partial assignment). For clarity: (i) an assignment to an Affiliate will terminate, and all rights so assigned will revert to the assigning Party, if and when such Affiliate ceases to be an Affiliate of the assigning Party; and (ii) sublicensing of any licenses granted under this Agreement will be governed by Section 4.1(c) and Section 4.3. Any attempted assignment in contravention of the foregoing will be null and void. Subject to the terms of this Agreement, this Agreement will be binding upon and inure to the benefit of the Parties and their respective successors and permitted assigns.

19.2. Extension to Affiliates.

Each Party may discharge any obligations and exercise any rights under this Agreement through delegation of its obligations or rights to any of its Affiliates. Each Party hereby guarantees the performance by its Affiliates of such Party's obligations under this Agreement, and will cause its Affiliates to comply with the provisions of this Agreement in connection with such performance. Any breach by a Party's Affiliate of any of such Party's obligations under this Agreement will be deemed a breach by such Party. Any reference to performance by a Party hereunder includes any performance by its Affiliates.

19.3. Severability.

If any of the provisions of this Agreement become void or unenforceable as a matter of law, then this Agreement shall be construed as if such provision were not contained herein, the remainder of this Agreement shall be in full force and effect, and the Parties will negotiate in good faith to substitute any invalid or unenforceable provision with a valid and enforceable provision such that this Agreement conforms as nearly as possible with the original intent of the Parties.

19.4. Force Majeure.

If either Party is prevented from performing its obligations under this Agreement due to any contingency beyond its reasonable control ("**Force Majeure**"), including acts of Governmental Authorities, any war, terrorism, hostilities between nations, civil commotions, riots, national industry strikes, sabotage, fire, floods and acts of nature such as typhoons, hurricanes, earthquakes, or tsunamis, the Party so affected shall not be responsible to the other Party for any delay or failure of performance of its obligations hereunder, for so long as and to the extent that such Force Majeure prevents such performance. If a Force Majeure arises, the Party immediately affected thereby shall give prompt written notice to the other Party specifying the Force Majeure event complained of, and shall use Commercially Reasonable Efforts to avoid or remove such causes of non-performance and to mitigate the effect of such occurrence to resume performance of its obligations with reasonable dispatch.

19.5. Waivers and Amendments.

The delay or failure of any Party to assert a right hereunder or to insist upon compliance with any term or condition of this Agreement shall not constitute a waiver of that right, term or condition or excuse a similar subsequent failure to perform any such term or condition by the other Party, and no waiver shall be effective unless it has been given in writing and signed by the Party giving such waiver. No waiver by either Party of any condition or of the breach of any term contained in this Agreement, in any one (1) or more instances, will be deemed to be, or considered as, a further or continuing waiver of any such condition or of the breach of such term or any other term of this Agreement. No provision of this Agreement may be amended or modified other than by a written document signed by authorized representatives of each Party.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

19.6. Relationship of the Parties.

Nothing contained in this Agreement shall be deemed to constitute a partnership, joint venture, or legal entity of any type between MeiraGTx and Janssen or their respective Affiliates, or to constitute one as the agent of the other. Moreover, each Party agrees not to construe this Agreement, or any of the transactions contemplated hereby, as a partnership for any tax purposes. Each Party shall act solely as an independent contractor, and nothing in this Agreement shall be construed to give any Party the power or authority to assume or create any obligations on behalf of or in the name of the other Party or to bind the other Party to any contract, agreement or undertaking with any Third Party.

19.7. Notices.

All notices, consents, waivers, and other communications under this Agreement must be in writing, in the English language, and will be deemed to have been duly given when: (a) delivered by hand; (b) when sent by an internationally recognized overnight delivery service (receipt requested); or (c) when sent by confirmed email, in each case, to the appropriate addresses set forth below (or to such other addresses as a Party may designate by notice in accordance with this Section 19.7):

If to MeiraGTx:

MeiraGTx UK II Limited
25 Provost Street
London N1 7NH Email: [***]
Attn: Chief Operating Officer

with a copy to:

MeiraGTx - US
430 East 29th Street, 10th Floor
New York, NY 10016
Attn: Chief Operating Officer

If to Janssen:

Janssen Pharmaceuticals, Inc.
1125 Trenton-Harbourton Road
Titusville, NJ 08560
Attention: President

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

with a copy to:

Johnson & Johnson
One Johnson & Johnson Plaza
New Brunswick, New Jersey 08933
Attn: General Counsel, Pharmaceuticals

Any such notice shall be deemed to have been given on the date delivered. A Party may add, delete (so long as at least one (1) person is remaining) or change the person or address to which notices should be sent at any time upon written notice delivered to the other Party in accordance with this Section 19.7.

19.8. Further Assurances.

Janssen and MeiraGTx hereby covenant and agree without the necessity of any further consideration, to execute, acknowledge and deliver, and to cause to be executed, acknowledged, and delivered, any and all such other documents and take any such other action as may be reasonably necessary to carry out the intent and purposes of this Agreement.

19.9. No Third Party Beneficiary Rights.

The provisions of this Agreement are for the sole benefit of the Parties and their successors and permitted assigns, and they shall not be construed as conferring any rights to any Third Party (including any third party beneficiary rights), except with respect to certain Janssen Indemnitees and certain MeiraGTx Indemnitees, who are Third Parties, solely with respect to Article 17.

19.10. English Language.

This Agreement was prepared and executed in the English language, which language shall govern the interpretation of, and any disputes regarding, the terms of this Agreement. Any translation into any other language shall not be an official version of this Agreement and in the event of any conflict in interpretation between the English version and such translation, the English version shall prevail.

19.11. Interpretation.

Unless the context of this Agreement otherwise requires:

(a) references to an Article, Section or Exhibit means an Article or Section of, or Exhibit to, this Agreement, unless another agreement is specified;

(b) the term “including” (in its various forms) means “including without limitation”;

(c) a particular statute or statutory instrument, regulation or any of their provisions shall include all rules and regulations thereunder and shall be construed as a reference to that statute or statutory instrument, regulation or such provision as the same may have been or may from time to time hereafter be modified, amended or re-enacted;

(d) words denoting the singular shall include the plural and vice versa, and words denoting any gender shall include all genders;

(e) references to a particular Person include such Person's successors and assigns to the extent not prohibited by this Agreement;

(f) the Exhibits and other attachments form part of the operative provisions of this Agreement and references to this Agreement shall, unless the context otherwise requires, include references to the Exhibits and attachments;

(g) the headings in this Agreement, in any Exhibit to this Agreement and in the table of contents to this Agreement are for information and convenience only and shall not in any way affect the construction of or be considered in the interpretation of this Agreement;

(h) the word "notice" means notice in writing (whether or not specifically stated) and will include notices, consents, approvals and other written communications contemplated under this Agreement;

(i) the word "or" will be interpreted in the inclusive sense commonly associated with the term "and/or";

(j) "days" refers to calendar days;

(k) the terms "hereof", "herein", "hereby", and derivative or similar words refer to this entire Agreement;

(l) general words shall not be given a restrictive interpretation by reason of their being preceded or followed by words indicating a particular class of acts, matters or things;

(m) the words "shall" and "will" have the same meaning; and

(n) the Parties agree that each Party has been represented by legal counsel in connection with this Agreement, the terms and conditions of this Agreement are the result of negotiations between the Parties, each Party has participated in the drafting hereof, and the terms and provisions of this Agreement shall not be construed and applied in favor of or against any Party by reason of the extent to which any Party participated in the preparation of this Agreement.

19.12. Entire Agreement.

This Agreement, together with its Exhibits, which are incorporated by reference herein, sets forth the entire agreement and understanding between the Parties as to the subject matter hereof and supersedes all prior discussions, representations, understandings, agreements, proposals, oral or written, and all other prior communications between the Parties with respect to such subject matter; *provided*, *however*, that the Prior CDA shall survive in accordance with its terms, except that any Information (as defined in the Prior CDA) that was disclosed by MeiraGTx LLC under the Prior CDA and that relates to the subject matter of this Agreement will be deemed Confidential

Information of MeiraGTx under this Agreement and treated in accordance with the terms and conditions of Article 12 and Article 13. If any conflict between a substantive provision of this Agreement and any Exhibit hereto arises, the substantive provisions of this Agreement shall prevail.

19.13. Counterparts.

This Agreement may be executed in one or more counterparts, each of which shall be deemed an original, and all of which taken together shall be deemed to constitute one and the same single instrument. Signature pages of this Agreement exchanged by facsimile or other electronic transmission will be deemed to be as effective as an original executed signature page.

[*Signature Pages Follow*]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives.

MEIRAGTX UK II LTD

By: /s/ Alexandria Forbes
Name: Alexandria Forbes
Title: Chief Executive Officer

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives.

MEIRAGTX HOLDINGS PLC

By: /s/ Alexandria Forbes
Name: Alexandria Forbes
Title: Chief Executive Officer

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives.

JANSSEN PHARMACEUTICALS, INC.

By: /s/ Jeffrey N. Smith
Name: Jeffrey N. Smith
Title: Vice President

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.34

Clinical Development Plans, Including Anticipated Costs and Timelines

See attached for anticipated costs and timelines.

Initial Clinical Development Plans to be attached upon approval as Exhibits 1.34-4, -5, -6 and (as applicable) -7 in accordance with Section 6.1(c).

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.34-1

Anticipated Costs and Timelines – CNGA3 Product

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.34-2

Anticipated Costs and Timelines – CNGB3 Product

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.34-3

Anticipated Costs and Timelines – RPGR Product

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.51

CMC Development Plans

To be attached upon approval.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.103

Final Report Requirements

Required

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.136

J&J Universal Calendar for 2019 and 2020

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.168

MeiraGTx Patents

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.221

IRD Genes

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 1.223

Research Plans

To be attached upon approval.

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 3.4

Research Budget Cap

Calendar Year	2019	2020	2021
Research Budget Cap*	[***]	[***]	[***]

* Total budget to be [***] in accordance with Section 3.8. Includes all pre-clinical work required to [***].

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 3.5

Janssen Data Policies

JANSSEN DATA GENERATION, PROCESSING AND STORAGE POLICIES

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 4.4

MeiraGTx Subcontractors

Subcontractor	Activities
[***]	

MeiraGTx Commitments to Engage Scheduled Subcontractors: [***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 8.2

Key Terms of Clinical Supply Agreement and Clinical Quality Assurance Agreement

The Clinical Supply Agreement will substantially contain the following provisions:

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 8.3

Key Terms of Commercial Supply Agreement and Commercial Quality Assurance Agreement

The Commercial Supply Agreement will substantially contain the following provisions:

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 8.4(a)

CMC Development Plan Activities – Breakdown

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 16.2(b)

Third Party Rights

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Confidential Treatment Requested by MeiraGTx Holdings plc

Exhibit 16.2(g)

MeiraGTx Existing Third Party Obligations

[***]

Confidential Portions of this Exhibit marked as [***] have been omitted pursuant to a request for confidential treatment and have been filed separately with the Securities and Exchange Commission.

Exhibit 21

SUBSIDIARIES OF MEIRAGTX HOLDINGS PLC

Legal Name of Subsidiary	**Jurisdiction of Organization**
BRI-Alzan, Inc.	Delaware
MeiraGTx B.V	Netherlands
MeiraGTx Limited	England and Wales
MeiraGTx, LLC	Delaware
MeiraGTx UK Limited	England and Wales
MeiraGTx UK II Limited	England and Wales
MeiraGTx Neurosciences, Inc.	Delaware

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in Registration Statement (Form S-8 No. 333-225535) pertaining to the 2016 Equity Incentive Plan, 2018 Incentive Award Plan and 2018 Employee Share Purchase Plan of MeiraGTx Holdings plc of our report dated March 26, 2019, with respect to the consolidated financial statements of MeiraGTx Holdings plc included in this Annual Report (Form 10-K) of MeiraGTx Holdings plc for the year ended December 31, 2018.

/s/ Ernst & Young LLP

Stamford, Connecticut
March 26, 2019

Exhibit 31.1

CERTIFICATION

I, Alexandria Forbes, certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2018 of MeiraGTx Holdings plc;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) [intentionally omitted];

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 26, 2019

By: /s/ Alexandria Forbes

Alexandria Forbes
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Katherine Breedis, certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2018 of MeiraGTx Holdings plc;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) [intentionally omitted];

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 26, 2019

By: /s/ Katherine Breedis

Katherine Breedis
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of MeiraGTx Holdings plc (the "Company") on Form 10-K for the fiscal year ended December 31, 2018, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 26, 2019

By: /s/ Alexandria Forbes

Alexandria Forbes
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of MeiraGTx Holdings plc (the "Company") on Form 10-K for the fiscal year ended December 31, 2018, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 26, 2019

By: /s/ Katherine Breedis

Katherine Breedis
Chief Financial Officer
(Principal Financial Officer)