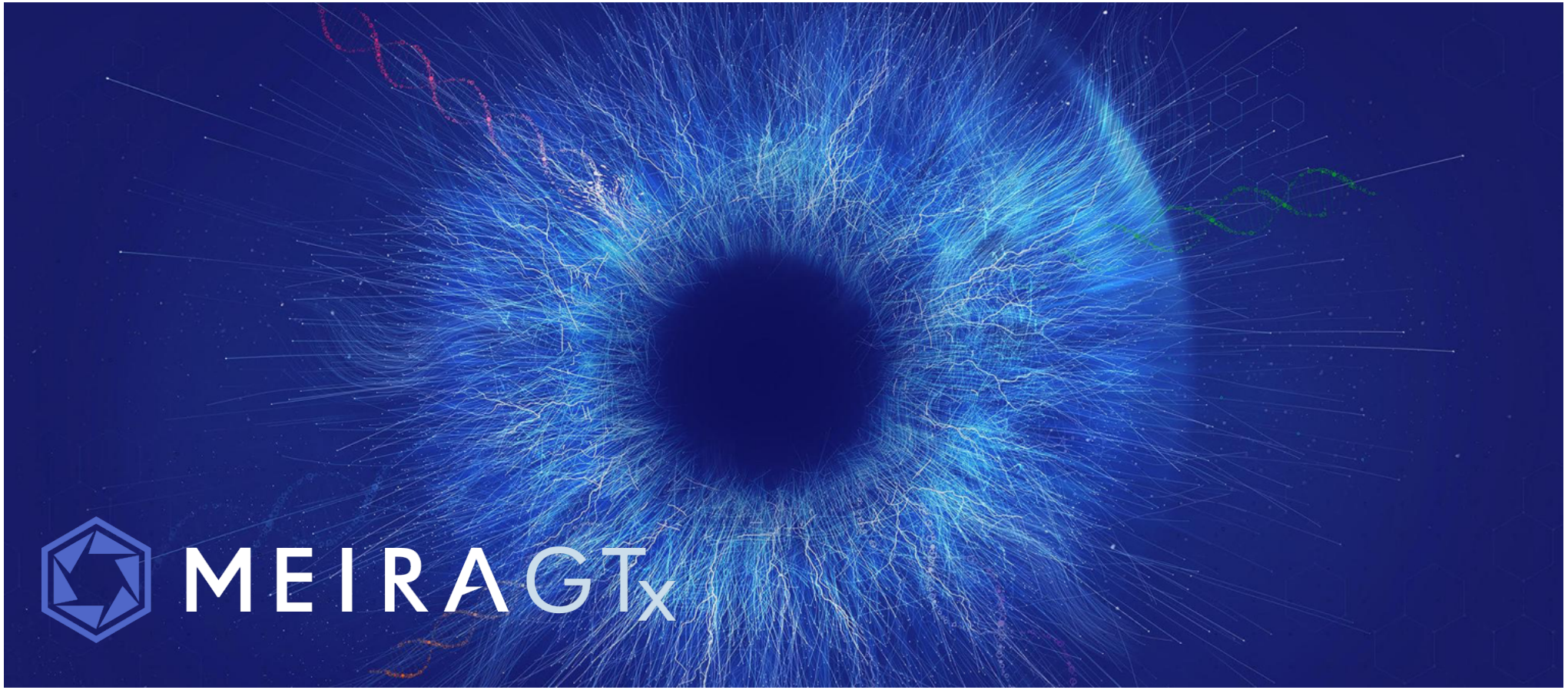


February 11, 2019

Forward Looking Statements

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this presentation that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the success of the research to be performed under the collaboration agreement, the development of our leading IRD product candidates and the development of our AAV manufacturing technology, as well as statements that include the words “expect,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “should,” “anticipate” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, acquire additional capital, identify additional and develop existing product candidates, continue operating as a going concern, successfully execute strategic priorities, bring product candidates to market, build-out the manufacturing facility and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2018, as such factors may be updated from time to time in our other filings with the Securities and Exchange Commission. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this presentation. Any such forward-looking statements represent management’s estimates as of the date of this presentation. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this presentation. Unless otherwise stated or the context otherwise requires, the information herein is as of Feb 1, 2019.

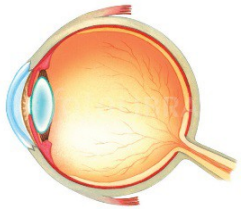
A Vertically Integrated, Clinical Stage Gene Therapy Company

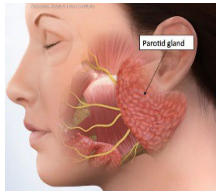
Developing a new pharmaceutical modality for the cost effective treatment of a broad range of serious disorders

Diversified Pipeline of Gene Therapy Candidates

5 ongoing clinical programs:

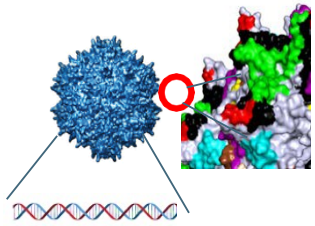
- Inherited retinal diseases
- Salivary gland
- Parkinson's Disease

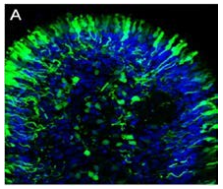




Platform of Core Viral Vector Engineering Capabilities

Viral vector design, promoters, capsid, transgene optimization, **process development** expertise



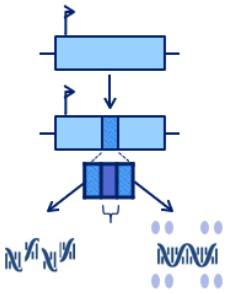


Manufacturing Capacity & Know-How

Flexible and **scalable cGMP manufacturing facility** with capacity for commercial supply for all our programs

Next Generation Gene Therapy Riboswitch based gene regulation

Proprietary technology that may allow for innovative gene therapy treatments whose **expression can be turned on and off** with an easily administered small molecule



Gene Therapy Pipeline

Ocular, Neurodegenerative, Salivary Gland Programs

Broad Clinical Pipeline

Product	Indication	Preclinical	Phase I/II	Status
Ocular Programs				
AAV-CNGB3	Achromatopsia (CNGB3)	RPDD, PRIME, Fast Track, Orphan Drug		Topline data from Phase I/II dose escalation study anticipated Q3 2019
AAV-CNGA3	Achromatopsia (CNGA3)	RPDD, Orphan Drug		Phase I/II trial initiation expected 1H 2019 (pediatric patients)
AAV-RPGR	X-linked RP (RPGR)	Fast Track, Orphan Drug		Phase I/II trial ongoing, preliminary data anticipated mid-2019
AAV-RPE65	RPE65-Deficiency (RPE65)	RPDD, Orphan Drug		Phase I/II trial complete, topline data anticipated Q1 2019
AAV-AIPL1	LCA4 (AIPL1)	Orphan U.S. & EU; Compassionate Use		Specials License approved October 2017
A006	Wet AMD (anti-VEGFR2)			IND expected 2019
Neurodegenerative Disease Programs				
AAV-GAD	Parkinson's Disease (GAD)			45 patient Phase II trial complete, regulatory path to be discussed with FDA 2019
AAV-UPF1	ALS/FTD (UPF1)			IND expected 2019
Salivary Gland Programs				
AAV-AQP1	Xerostomia (hAQP1)			6 patients treated in Phase I study Phase I/II trial initiation expected 1H 2019
AAV-AQP1	Sjögren's Syndrome (hAQP1)			IND expected 2019

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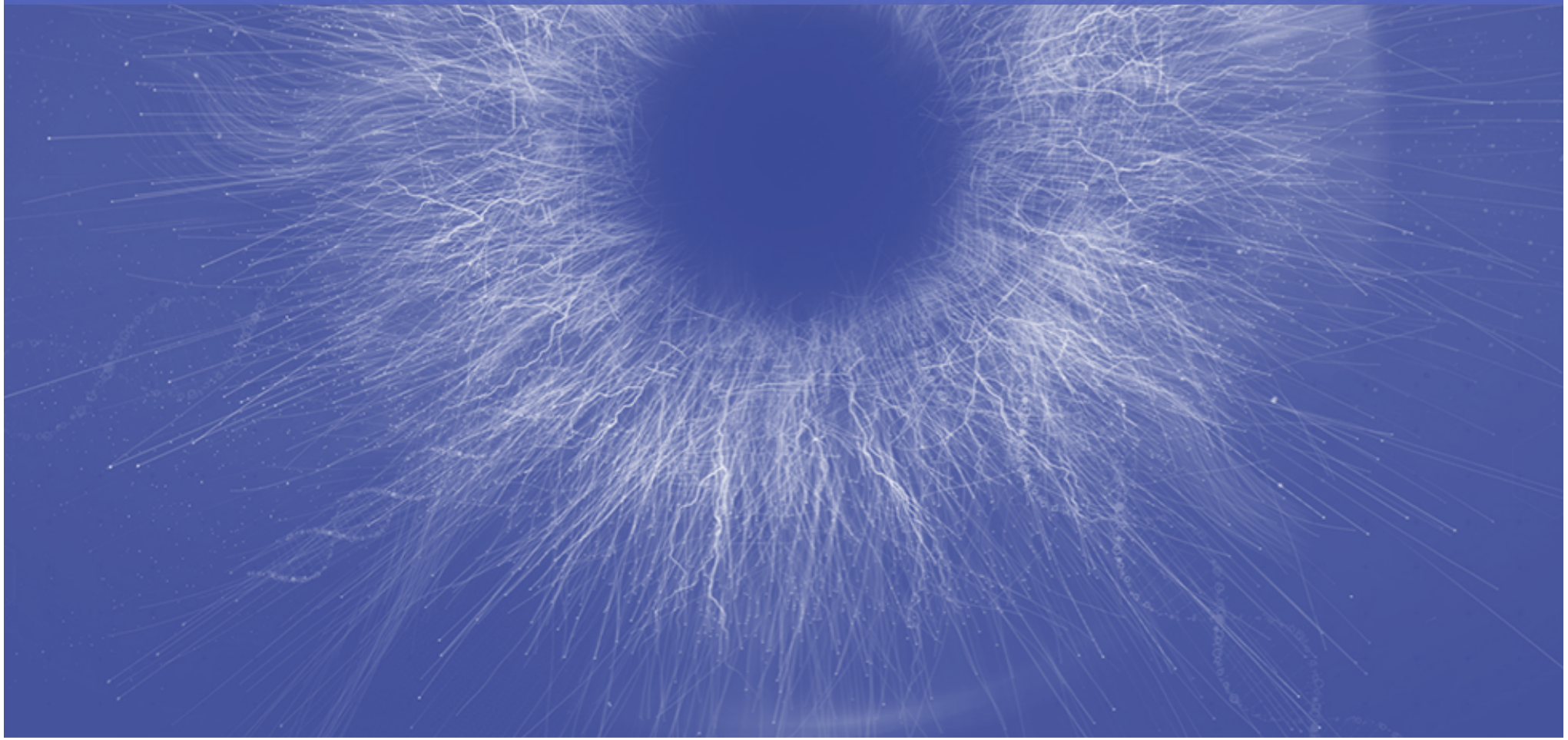
Ocular Franchise

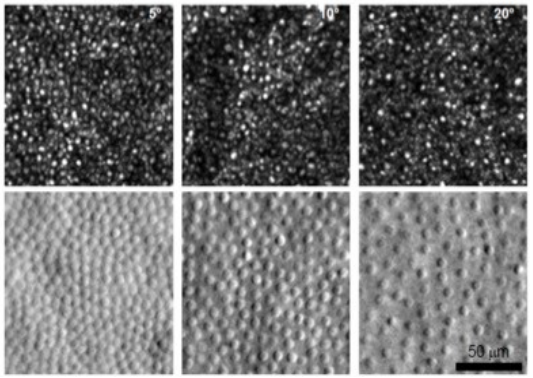


MeiraGTx Ocular Franchise

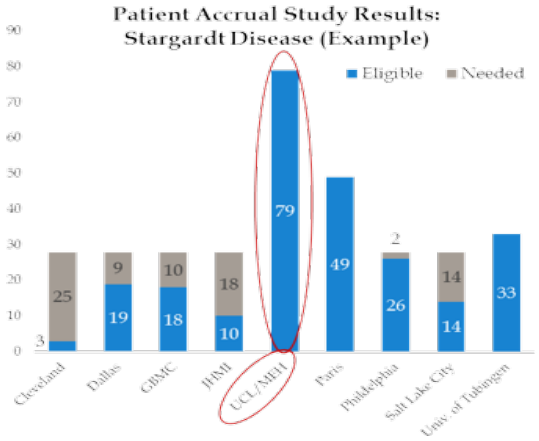
Ophthalmology Partners and Centers of Excellence

- **UCL Institute of Ophthalmology**
  One of the world's leading research institutes pioneering ophthalmology gene therapy
- **Moorfields Eye Hospital**
  Provides access to the world's largest catchment of patients with inherited retinal diseases, well characterized patients, prospective Natural History studies
- US footprint through links with **University of Michigan Kellogg Eye Center, Massachusetts Eye and Ear (MEEI)**, **Casey Eye Institute** and other leading centers
- Partnership with **Foundation Fighting Blindness (FFB)**
- Global expertise in imaging and validated endpoints in each of our target diseases

World Leading Imaging, Endpoint Development and Validation



Patient Access



Worldwide Strategic Collaboration

Janssen
PHARMACEUTICAL COMPANIES OF
Johnson & Johnson

Clinical development

Janssen and MeiraGTx to collaborate to develop a portfolio of potential gene therapy products to address Inherited Retinal Diseases (IRDs)

Janssen commercial infrastructure

- Janssen to receive worldwide exclusive commercial rights to AAV-CNGB3, AAV-CNGA3, AAV-RPGR and future IRD programs
- Janssen pays 100% of clinical development and commercialization costs
- Untiered 20% royalty on net sales
- MeiraGTx to supply clinical and commercial product

Pre-clinical IRD research

- Research collaboration to develop potential gene therapy treatments for IRDs
- Janssen to receive exclusive rights to develop & commercialize programs
- Janssens pays the majority of costs of research collaboration
- Janssen pays 100% of clinical development and commercialization costs of optioned programs
- Untiered high teens royalty on net sales

Manufacturing and process development

- Joint development of novel AAV manufacturing technologies to expedite and optimize development
- Janssen and MeiraGTx share costs of manufacturing research collaboration

AAV-CNGB3 & AAV-CNGA3 for the Treatment of Achromatopsia

Achromatopsia (ACHM)

Retinal defect

- Complete absence of cone photoreceptor function from birth

Disease characterization

- Very poor visual acuity from birth (typically 20/200)
- Photophobia (disabling aversion to light)

Prevalence and genes

- Overall ACHM prevalence is around 1/30,000 in US
- Total patients in US, EU5 and Japan:~24,000
- CNGB3 (~50% of cases), CNGA3 (~40% of cases)

Patient Experience

Normal

Achromatopsia

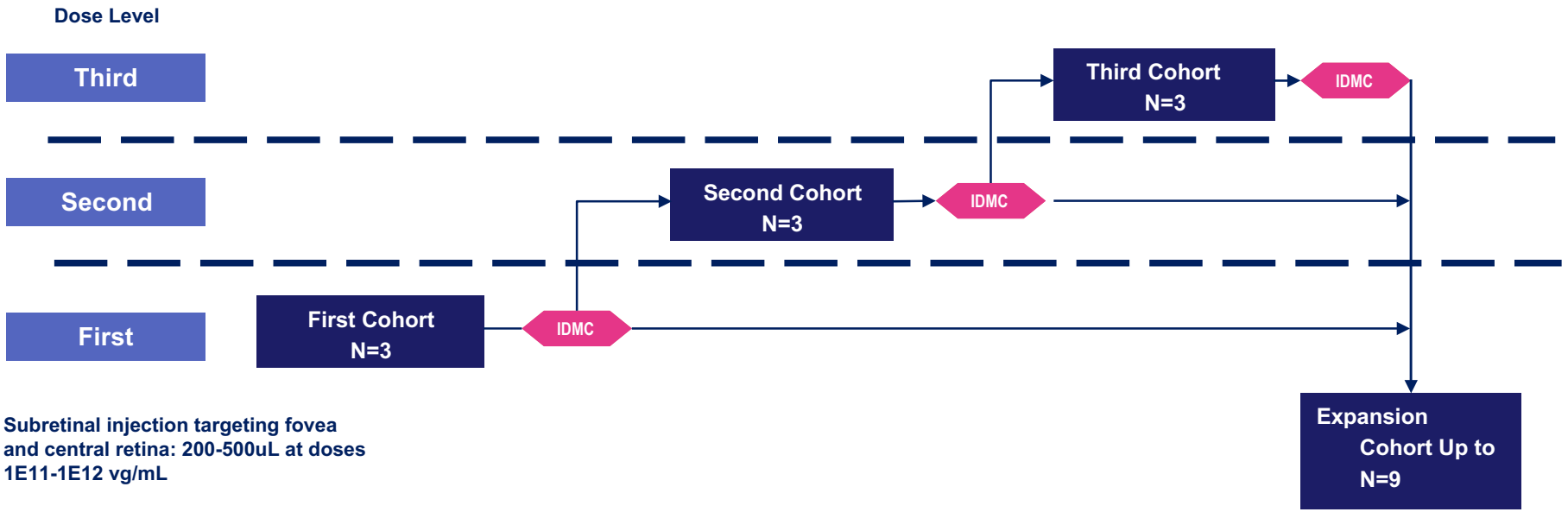
AAV-CNGB3

- Human cone arrestin promoter (hCARp)
- AAV2/8
- Human CNGB3 cDNA

AAV-CNGA3

- Novel synthetic cone specific promoter (hG1.7p)
- AAV2/8
- Codon optimized human CNGA3 cDNA

Phase I/II Trial of AAV-CNGB3



- **Primary endpoint:** safety
- **Secondary endpoints:** static perimetry (Octopus 900 and visual field modeling & analysis), microperimetry, fundus imaging, optical coherence tomography, electroretinography, adaptive optics, contrast sensitivity, visual acuity, reading speed, color vision, visual mobility, quality of life, nystagmus, and photoaversion
- **Trial ongoing:** 20 patients treated (11 adult, 9 pediatric)
- **Topline data anticipated Q3 2019:** 6 month follow up on all patients treated
- **ACHM Natural History study ongoing:** > 90 patients well characterized

AAV-RPGR for the Treatment of X-Linked Retinitis Pigmentosa due to RPGR-Deficiency

XLRP

Retinitis Pigmentosa (RP)

- Group of IRDs which represents the most common genetic cause of blindness
- X-linked RP is the most severe form of RP and accounts for 10-15% of RP patients

Disease progression

- Loss of night vision
- Progressing into tunnel vision
- Blindness in 4th decade

Prevalence and genes

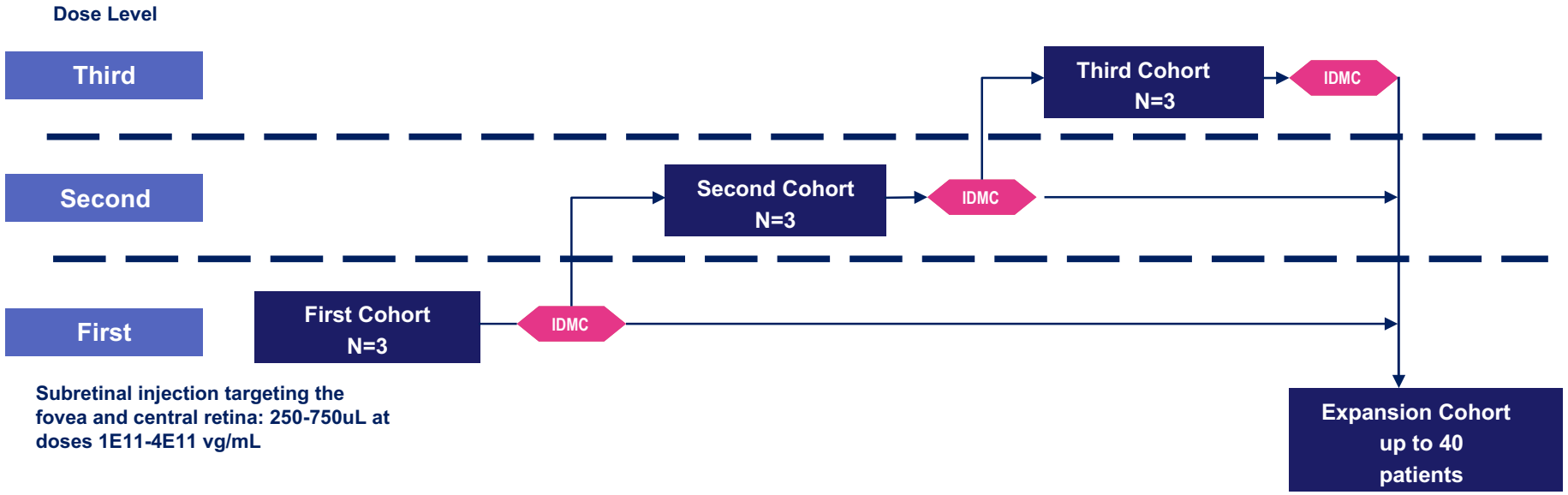
- ~1/40,000
- Total patients in US, EU5, Japan: ~20,000

AAV-RPGR

- Human rhodopsin kinase promoter (RKp)
- AAV5
- $RPGR^{ORF15}$

Patient Experience

Phase I/II Trial of AAV-RPGR

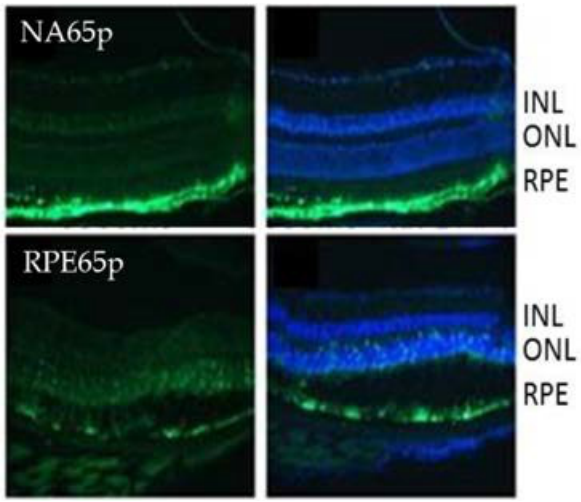


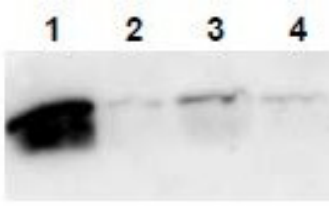
- **Primary endpoint:** safety
- **Secondary endpoints:** static perimetry (Octopus 900 and visual field modeling & analysis), microperimetry, fundus imaging, optical coherence tomography, electroretinography, adaptive optics, contrast sensitivity, visual acuity, reading speed, mobility, quality of life
- **Dose escalation complete:** 10 adults (including young adults) treated in dose escalation, expansion cohort enrolling (1 pediatric patient treated)
- **Topline data anticipated mid-2019:** 6 month follow up on dose escalation cohorts
- **Natural History study ongoing:** > 100 XLRP patients well characterized

AAV-RPE65 Vector Construct Optimization

Optimization Details

- **Capsid changed from AAV2 to AAV5:** 4x improvement in transfection efficiency of RPE
- **Promoter optimization:** 20x increase in protein expression
- **Codon optimization:** 7x increase in protein expression in human cells
- **SV40 intron increases mRNA stability:** 2.5x increased mRNA levels



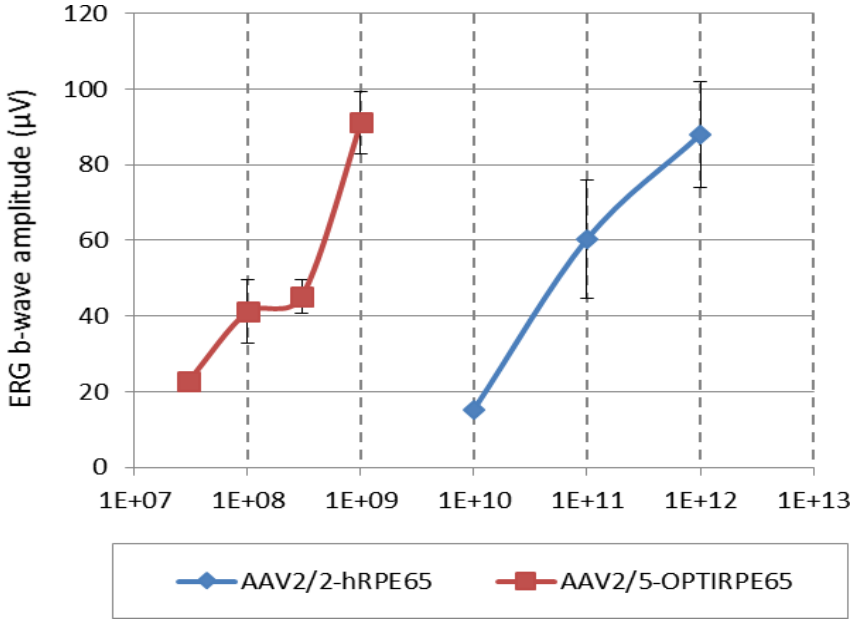


1: AAV. RPE65OPT.GFP
2: AAV. RPE65OPT.GFP (1:20 dilution)
3: AAV.BGL65p.GFP (alt promoter fragment)
4: AAV.hRPE65.GFP

Head-to-Head Comparison

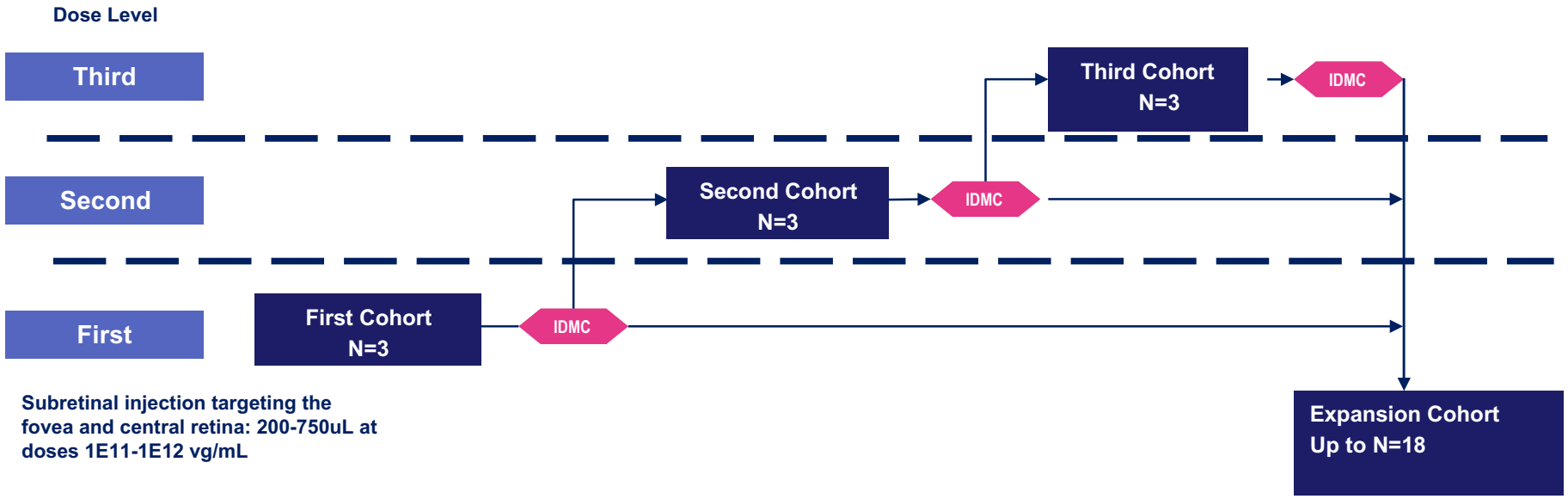
Head-to-head comparison in vivo of AAV2/5-OPTIRPE65 and AAV2/2-hRPE65, the vector used in the first UCL clinical trial

- After subretinal injection into RPE65-deficient mice, AAV2/5-OPTIRPE65 can restore retinal function at 300-1,000 fold lower doses than AAV2/2-hRPE65



Subretinal injection targeting the central retina
Volume of injection may be up to 1ml
Multiple retinotomies permitted

Phase I/II Trial of AAV-RPE65



- **Primary endpoint:** safety
- **Secondary endpoints include:** static perimetry (Octopus 900 and visual field modeling & analysis), microperimetry, fundus imaging, optical coherence tomography, electroretinography, adaptive optics, contrast sensitivity, visual acuity, reading speed, mobility, quality of life
- **Dosing complete:** 15 patients treated (9 adult, 6 pediatric)
- **Topline data anticipated Q1 2019:** 6 month follow up on all patients treated
- **Natural History study ongoing:** >30 RPE65-deficiency patients well characterized

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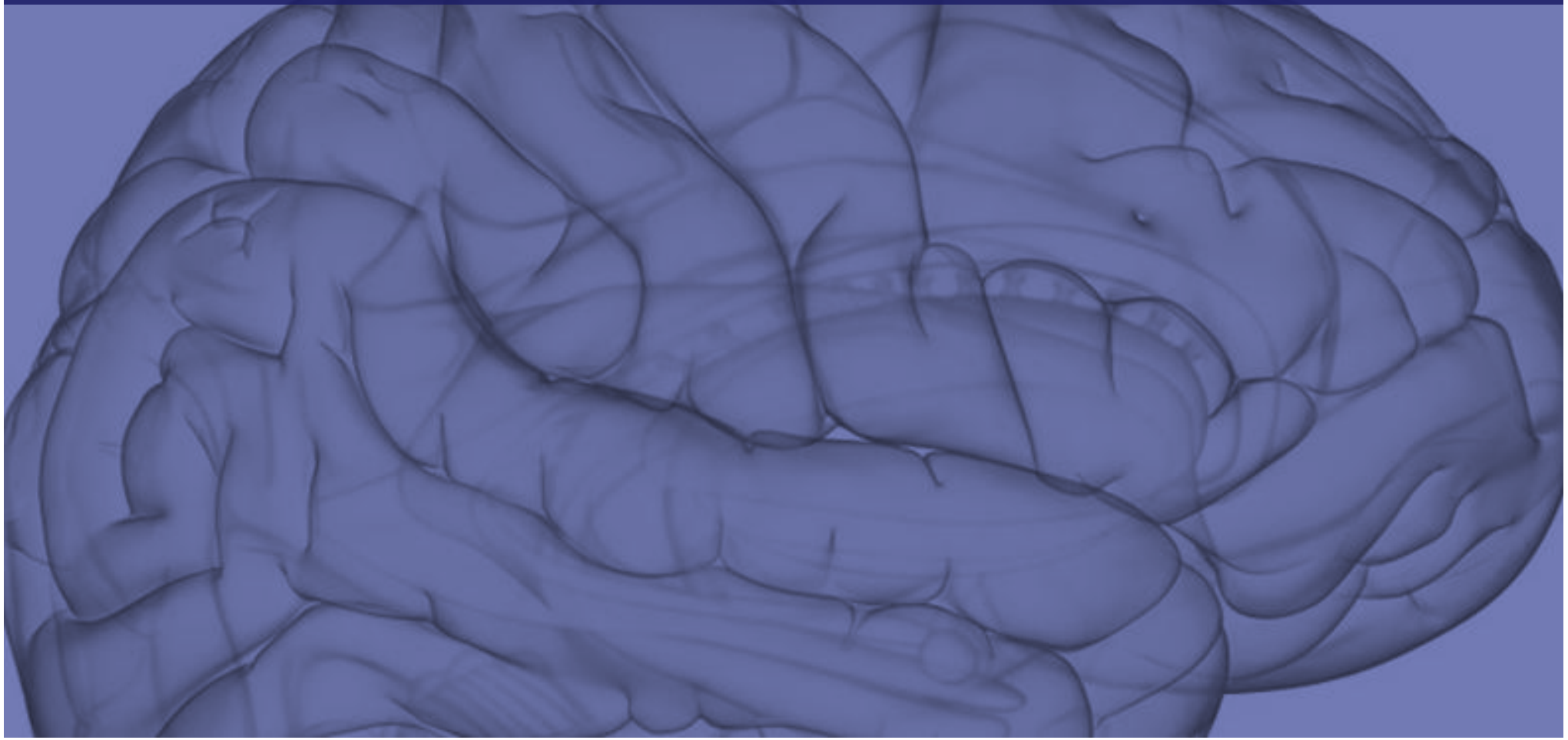
Neurodegenerative Diseases



AAV-GAD for the Treatment of Parkinson's Disease

1.5 million U.S. PD patients

Most patients become refractory to SoC dopamine treatment after 5-10 years

- 300,000 U.S. PD patients refractory to pharmacological intervention

Few options for refractory patients

- Deep Brain Stimulation
- Surgical lesioning

PD manifests as a movement disorder

A cascade of changes in basal ganglia circuitry occurs

Hyperactivity of the subthalamic nucleus (STN) key output

- Decreased GABAergic inflow (inhibition)
- Increased glutamatergic outflow (excitation)

Our approach: AAV-GAD gene therapy rebalances the basal ganglia output to the motor cortex

- **G**lutamic **A**cid **D**ecarboxylase converts Glutamate into GABA
- Vector: **AAV2-CMV-CBA-GAD**
- Delivered directly into the STN
- Bypasses circuitry disrupted by dopamine loss to restore normal basal ganglia outflow and reduce STN hyperactivity

AAV-GAD Met Primary Endpoint in Randomized, Controlled Phase II Trial

Randomized, double-blind, sham-controlled, multi-center Phase II trial in advanced PD patients

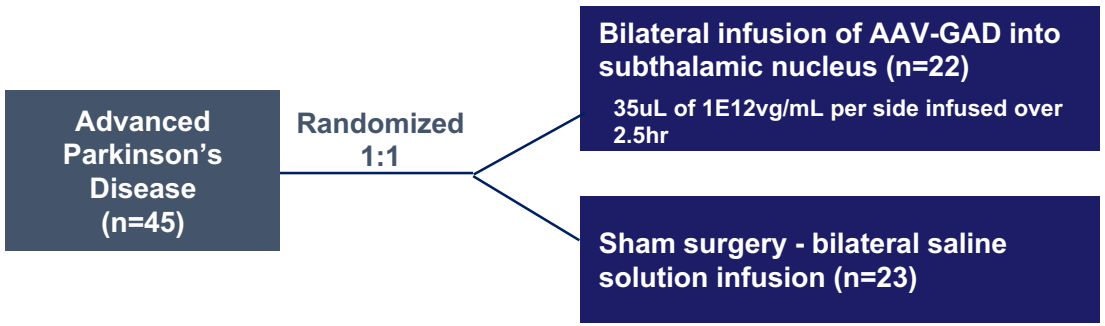
AAV-GAD safe and well tolerated
- No treatment-related SAEs

Met primary endpoint: UPDRS Part 3 improvement at 6 months, maintained at 12 months
- 8.1 point improvement for AAV-GAD vs 4.7 point improvement for sham (p=0.003)

Responders with 9 point or greater UPDRS Part 3 improvement
- 50% AAV-GAD response at 6 months vs 14% Sham response
- 62% AAV-GAD response at 12 months vs 24% Sham response

Significant reduction in medication complications
- At 6 months and 12 months for AAV-GAD patients

Changes in basal ganglia metabolism and reorganization of connectivity correlated w/ improved symptoms
- Decreased metabolism in striatum and thalamus, increased metabolism in premotor cortex
- New polysynaptic pathways connecting STN to motor cortex



Kaplitt MG. AAV2-GAD gene therapy for advanced Parkinson's Disease: a double-blind, sham-surgery controlled, randomized trial. *Lancet Neurology*, 2011, vol. 10.

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Radiation-Induced Xerostomia

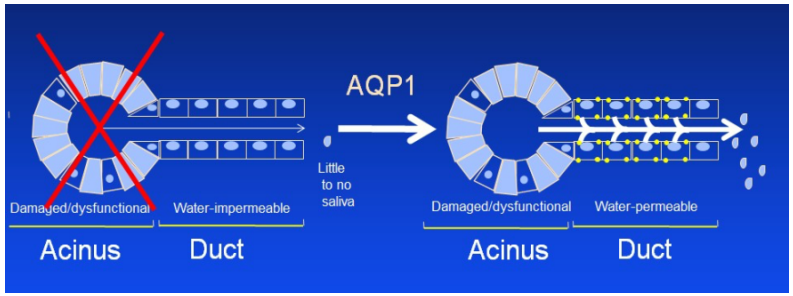
Radiation-Induced Xerostomia (RIX)

Significant unmet medical need: xerostomia persisting >2 years after radiation therapy for oral cancer

- 170,000 existing patients in the U.S.
- 50,000 new cases of head and neck cancer/yr treated in US
- 85% of radiation-treated patients experience reduced saliva production, of whom 40% have persistent Grade 2/3 RIX
- Serious, debilitating complications: dental caries, enamel erosion, oral infections, sleep disturbances, difficulty talking, chewing, swallowing, weight loss and malnutrition

Strategy for the repair

- Water-impermeable duct cells generate an osmotic gradient (lumen > interstitium)
- Introduction of human aquaporin 1 gene (hAQP1) into duct cells via viral vector, making duct cells permeable to water
- Allows water to flow into the salivary duct and out to moisten the mouth



Salivary gland as target for gene therapy

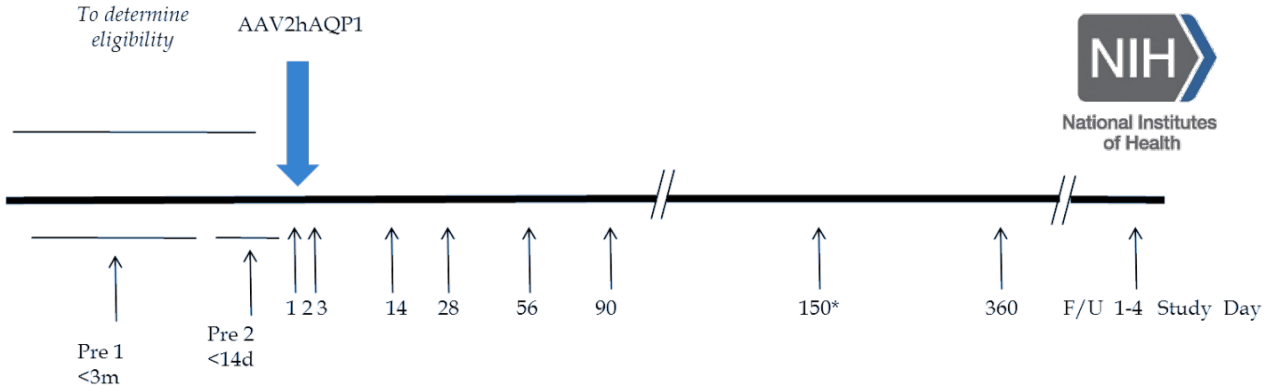
- Non-invasive: allows local administration and avoids systemic exposure
- Isolated and encapsulated
- Small volume of vector

AAV-AQP1

- CMV enhancer CBA promoter
- AAV2
- Human aquaporin 1 (hAQP1)

Phase I Trial of AAV2-hAQP1

AAV2-hAQP1 in patients with grade 2/3 xerostomia following IR for oral cancer



Dose Group	Vector Dose (Viral Particles per Gland)
*0	3×10^9
1	1×10^{10}
2	3×10^{10}
3	1×10^{11}
4	3×10^{11}
5	6×10^{11}

- **Primary endpoint:** safety
- **Secondary endpoint:** parotid gland salivary output
- **6 patients treated to date:** Dosing complete in cohorts 1 and 2, trial to enroll up to 18 patients
- **Multi-site, Phase I/II trial to be initiated 1H 2019**
- **AAV2-hAQP1 for Sjogren's syndrome:** IND filing anticipated 2019

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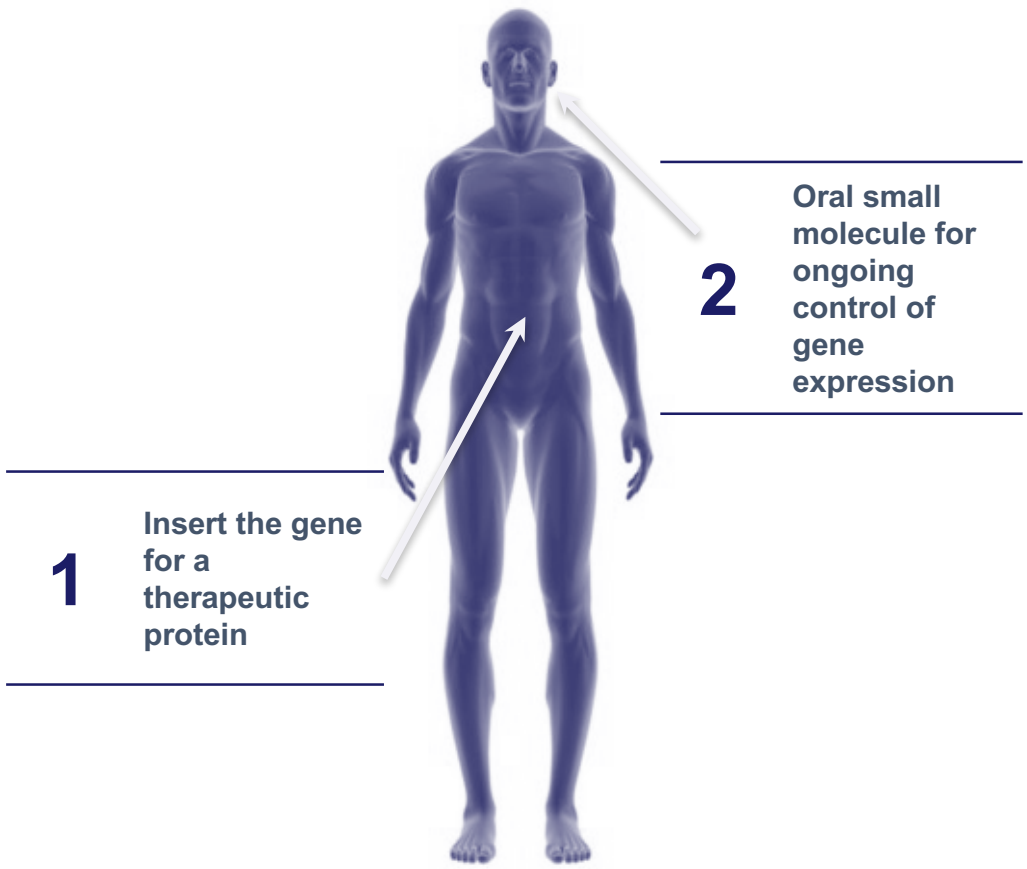
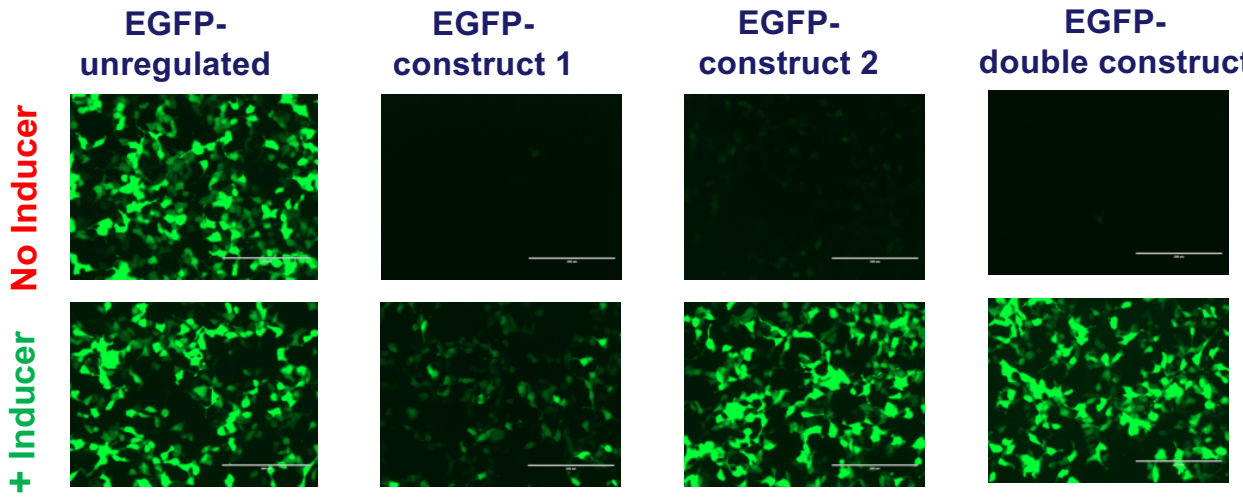
Gene Regulation

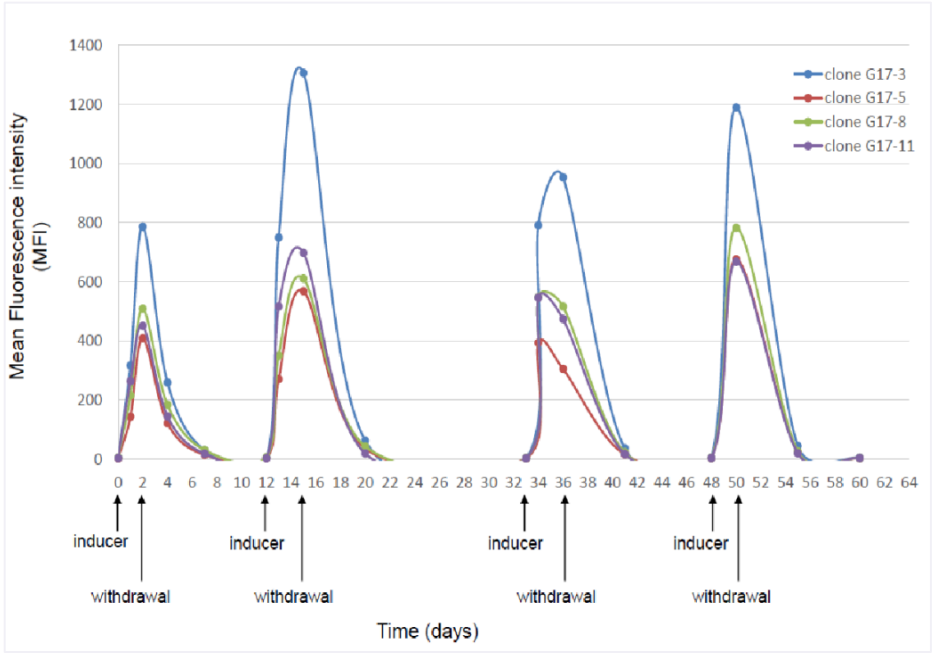
Next Generation Gene Therapy: Gene Regulation Platform

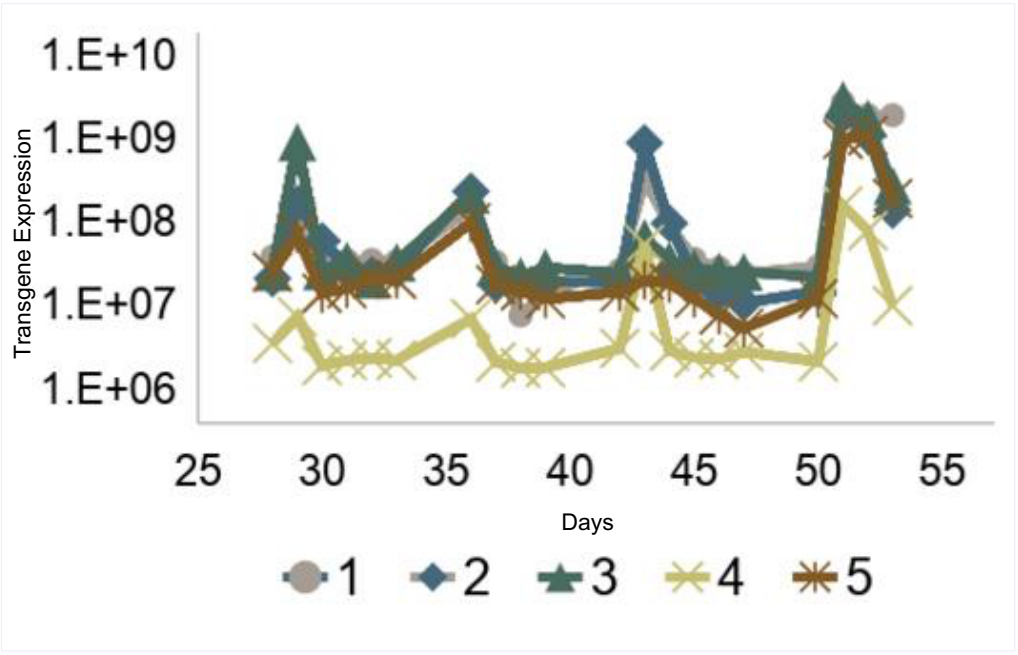
- **Modular switch cassette based on RNA shape**
- Regulate a chosen transgene in vivo using a different **small molecule** for each transgene
- **Platform can regulate multiple genes:** antibodies, hormones, cytokines
- **Demonstrated regulation in vivo in the liver of AAV delivered genome**



Regulation of Transgene Expression







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Manufacturing and Process Development

cGMP Certified Manufacturing Facility: Flexible and Scalable

Key Attributes

- cGMP certified 29,000 sq ft multi-product, multi-viral vector manufacturing facility
- Designed to meet MHRA, EMA and FDA regulatory requirements
- Single use philosophy / fully enclosed technologies
- 2 cell suites; 3 viral vector suits
- Independent air handling
- Designed for minimal downtime and maximum flexibility
- Adherent / non-adherent cell lines – HEK293
- Support laboratories: Quality Control
- Adjacent MSAT (Manufacturing Science and Technology) area/pilot plant

Plan

Highlights and Anticipated 2019 Clinical Milestones

Diversified pipeline – rare genetic disorders & common non-genetic disorders

5 clinical programs with 18 regulatory designations and at least **3 data readouts** over the next 9 months

Platform of Core Viral Vector Engineering Capabilities

Viral vector design, promoter, capsid, transgene optimization, **process development** expertise across therapeutic franchises

Manufacturing Infrastructure & Expertise

Capacity to manufacture **clinical and commercial supply** across all programs

Riboswitch- Based Gene Regulation Platform

Developing next-generation gene therapies whose **expression can be turned on and off**

RPE65 Phase I/II trial topline data (Q1 2019)

CNGA3 Phase I/II trial initiation (H1 2019)

Initiate multi-center xerostomia Phase I/II trial (H1 2019)

RPGR Phase I/II trial preliminary data (mid-2019)

CNGB3 Phase I/II trial topline data (Q3 2019)

Update on AAV-GAD path to regulatory approval (2019)

$88.6 million cash as of September 30, 2018