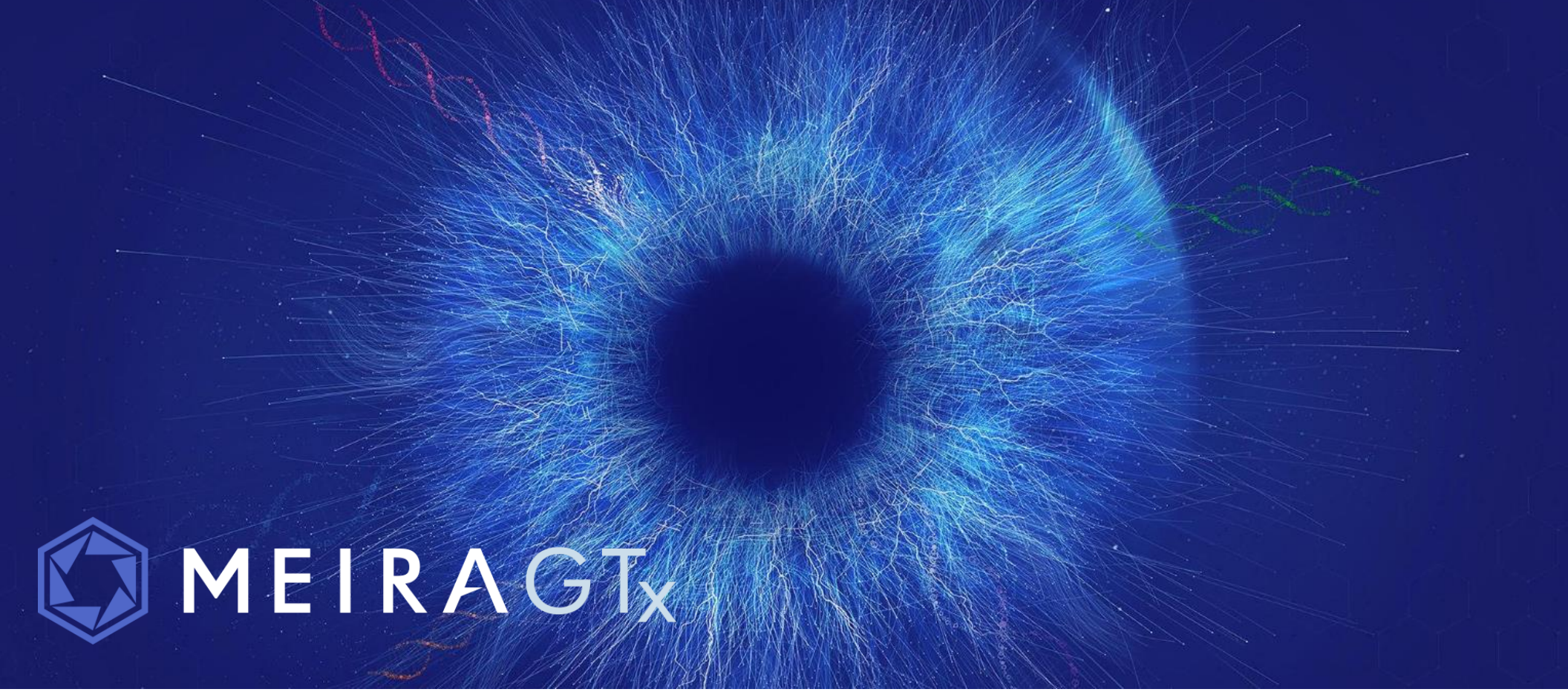


Collaboration with Janssen Pharmaceuticals, Inc.

January 31, 2019

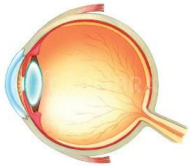
Forward Looking Statements

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 press release 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 SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. 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 press release.

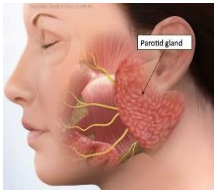
Vertically Integrated, Clinical Stage Gene Therapy Company

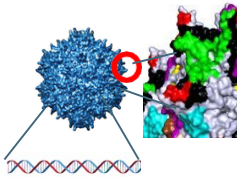
MEIRAGTx



Diversified pipeline of gene therapy candidates

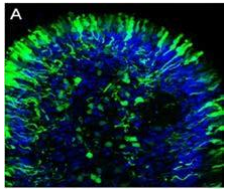
5 ongoing clinical programs:
- Inherited retinal diseases
- Salivary gland
- Parkinson's Disease





Platform of core viral vector engineering capabilities

- Viral vector design
- Promoters, capsid, transgene optimization
- Process development expertise

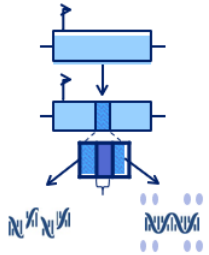


Manufacturing capacity & know-how

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

Next generation gene therapy: riboswitch-based gene regulation platform

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



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

Worldwide Strategic Collaboration

Pre-clinical IRD research

- **Collaboration to leverage MeiraGTx vector design and optimization technology to develop potential gene therapy treatments for multiple IRDs**
- **Janssen to receive exclusive rights to develop & commercialize programs from IRD research collaboration**

Clinical development

Janssen and MeiraGTx to collaborate to expedite the advancement of AAV-CNGB3, AAV-CNGA3, AAV-RPGR through clinical development

Manufacturing and process development

- **Janssen to access MeiraGTx advanced manufacturing capabilities with clinical and commercial supply agreements**
- **Joint development of novel AAV manufacturing technologies to expedite and optimize development**

Janssen commercial infrastructure

- **Janssen to receive worldwide exclusive commercial rights to AAV-CGB3, AAV-CGA3, AAV-RPGR and future IRD programs**
- **IRD portfolio to benefit from worldwide reach of Janssen commercial infrastructure**

Key Financial Terms

MeiraGTx to collaborate with Janssen to advance current clinical IRD programs through regulatory approval

- $100 million upfront payment upon closing
- $340 million in potential development and sales milestones related to AAV-CNGB3, AAV-CNGA3 and AAV-RPGR
- Janssen to fund 100% of clinical development and commercialization costs
- 20% royalties (untiered) on global annual net sales of AAV-CNGB3, AAV-CNGA3, AAV-RPGR
- MeiraGTx to supply clinical and commercial material

Research collaboration to develop a pipeline of IRD gene therapy candidates

- Janssen to cover significant portion of pre-clinical research costs
- MeiraGTx to receive opt-in payments and development milestones on each program
- Janssen to fund 100% of clinical development and commercialization costs of programs arising from collaboration
- High teens royalties (untiered) on global annual net sales of programs arising from collaboration

MEIRAGTx

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 Designation		Topline data from Phase I/II dose escalation study anticipated Q3 2019
AAV-CNGA3	Achromatopsia (CNGA3)	RPDD		Phase I/II trial initiation expected 1H 2019 (pediatric patients)
AAV-RPGR	X-linked RP (RPGR)	Fast Track Designation		Phase I/II trial ongoing, preliminary data anticipated mid-2019
AAV-RPE65	RPE65-Deficiency (RPE65)	RPDD		Phase I/II trial complete, topline data anticipated 1H 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 I/II trial complete Data published 2018
AAV-UPF1	ALS/FTD (UPF1)			IND expected 2019
Salivary Gland Programs				
AAV-AQP1	Xerostomia (hAQP1)			5 patients treated in Phase I study Additional Phase I/II trial initiation expected 1H 2019
AAV-AQP1	Sjögren's Syndrome (hAQP1)			IND expected 2019

Multiple Therapeutic Targets

OCULAR

Clinical Development
- Gene IRD franchise: XLRP, achromatopsia, RPE-65 deficiency, LCA4

Pre-IND
- Wet AMD

NEURODEGENERATIVE

Clinical Development
- Parkinson's Disease

Pre-IND
- Amyotrophic Lateral Sclerosis (ALS)

SALIVARY GLAND

Clinical Development
- Radiation-induced xerostomia (Grade 2/3)

Pre-IND
- Sjögren's Syndrome

Human proof of concept demonstrated across ocular, salivary gland and neurodegenerative pipelines

Vector development & optimization technology create opportunities to treat broader indications beyond rare, inherited genetic disorders

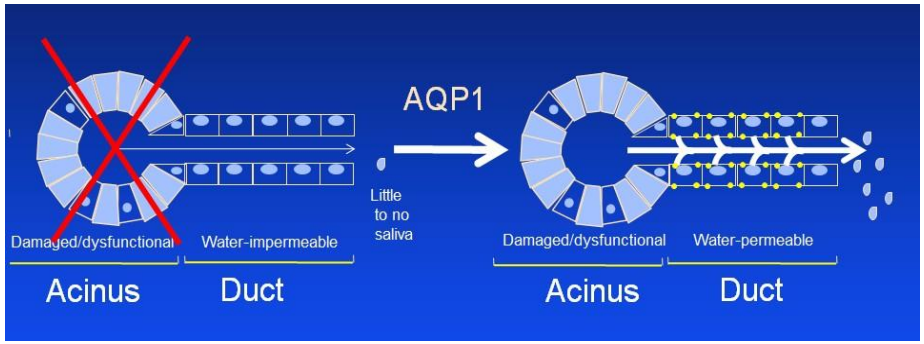
Radiation-Induced Xerostomia

Significant Unmet Medical Need

- **Xerostomia persisting >2 years after radiation therapy for oral cancer**
  - 170,000 existing patients in the U.S.; orphan status
  - 50,000 new cases of head and neck cancer/yr treated in U.S.
  - 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
- **Salivary gland as a target for gene therapy**
  - Non-invasive: allows local administration and avoids systemic exposure
  - Isolated and encapsulated
  - Small volume of vector

Strategy for the Repair

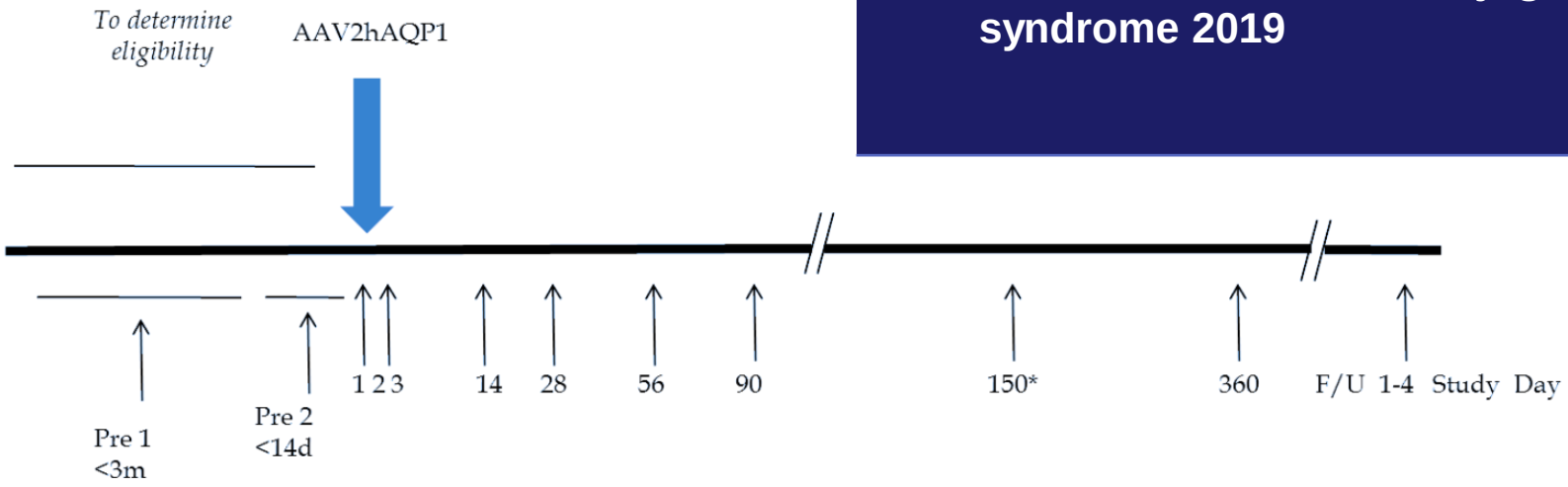
- Water-impermeable duct cells generate an osmotic gradient (lumen > interstitium)
- Introduction of human aquaporin 1 gene (hAQP1) into duct cells via viral vector, making duct cells permeable to water
- Allows water to flow into the salivary duct and out to moisten the mouth



Radiation-Induced Xerostomia Phase I Study

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

- AAV2-hAQP1 in patients with grade 2/3 Xerostomia following IR for oral cancer
- Assess safety and effectiveness to increase parotid gland salivary output
- Up to 18 patients
- Dose escalation cohorts 3 patients at monthly intervals
- **5 patients treated**
- **3 in cohort 1; 2 in cohort 2**
- **Initiate additional Phase I/II Study at MSKCC and up to 5 other sites in the US - 1H 2019**
- **IND for new indication: Sjogren's syndrome 2019**



AAV-GAD: Summary

- **Positive safety and efficacy data from two clinical studies**
  - Phase I: unilateral treatment 12 patients
  - Phase II: double blind sham controlled 45 patients
- Significant improvement in motor symptoms in AAV-GAD treated patients
- Significant reduction in medication complications up to 12 months
- Changes in basal ganglia metabolism and reorganization of connectivity correlated with improved symptoms

- AAV-GAD delivery is into the same brain region as DBS, has no hardware or programming
- Convenient for patient, neurologist, neurosurgeon, hospital
- No speech complications, neuropsychological or cognitive declines

- **1H 2019:** Discuss clinical data with the FDA with the aim of determining a path to regulatory approval of AAV-GAD gene therapy for the treatment of advanced Parkinson's disease

Large Target Indication:

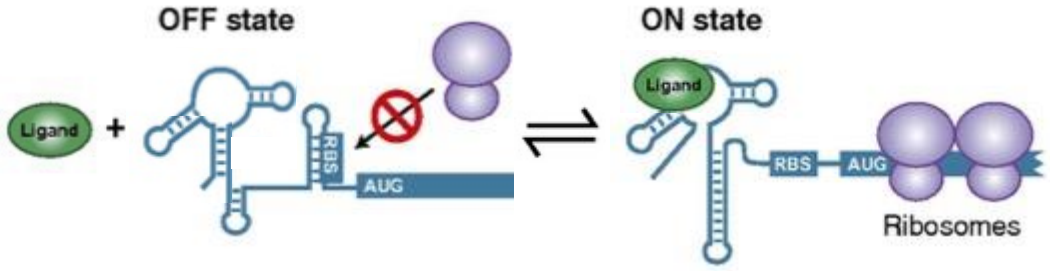
- **300,000 PD patients that are refractory to oral medications**

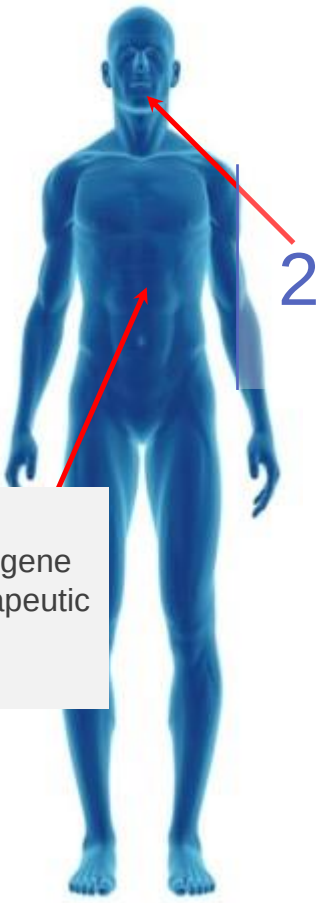
Novel mechanism of action:

- **to rebalance the basal ganglia output to the motor cortex**

Next Generation Gene Therapy:
Gene Regulation Platform

- Modular switch cassette based on RNA shape – not transcription
- Regulate a chosen transgene in vivo using a different small molecule for each transgene
- We have regulated multiple genes: antibodies, hormones, cytokines
- Demonstrated regulation in vivo in the liver of AAV delivered genome





2 Oral small molecule for ongoing control of gene expression

1 Insert the gene for a therapeutic protein

Manufacturing and Process Development

Research collaboration to further develop AAV manufacturing technology

Allows us to manufacture all of the material needed to expedite our clinical development

Highlights

MeiraGTx and Janssen to share costs

Facility has the capacity to run:

- 3 viral vector suites in parallel
- each with a different viral vector
- multiple consecutive batches in each suite per year
- 2 cell lines for continual cell supply
- Minimal down time between batches

Clinical and manufacturing supply agreements for the partnership programs

Manufacturing flexibility and capacity to produce material for 4 potential pivotal programs and several Phase I/II clinical studies over the next 18 months

cGMP Certified Manufacturing Facility:
Flexible and Scalable

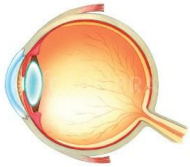
Key Attributes

- 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

Vertically Integrated, Clinical Stage Gene Therapy Company

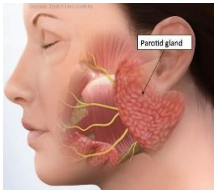
MEIRAGTx

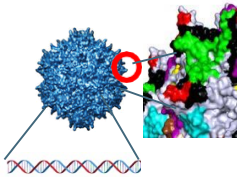


Diversified pipeline of gene therapy candidates

5 ongoing clinical programs:

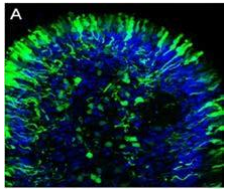
- Inherited retinal diseases
- Salivary gland
- Parkinson's Disease





Platform of core viral vector engineering capabilities

- Viral vector design
- Promoters, capsid, transgene optimization
- Process development expertise

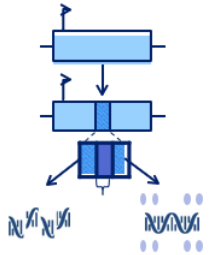


Manufacturing capacity & know-how

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

Next generation gene therapy: riboswitch-based gene regulation platform

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



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