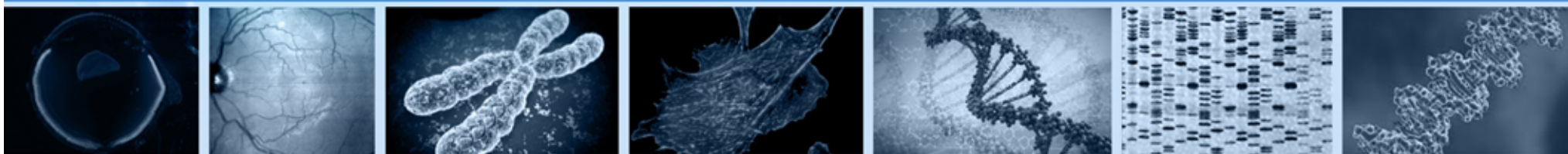




Corporate Overview

January 2019



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 product pipeline, anticipated product benefits, goals and strategic priorities, product candidate development, growth expectations or targets and pre-clinical and clinical data, 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 December 18, 2018.

Company Overview



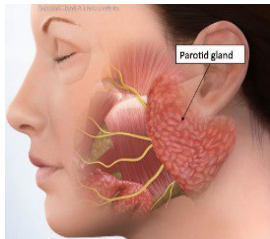
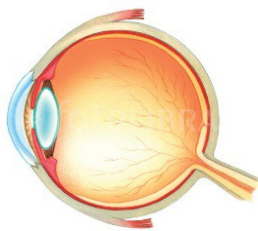
A Vertically Integrated, Clinical Stage Gene Therapy Company

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

Diversified Pipeline of Gene Therapy Candidates

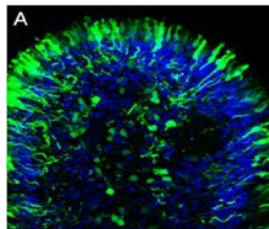
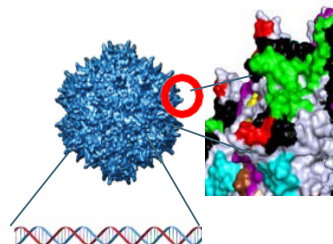
5 ongoing clinical programs:

- 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



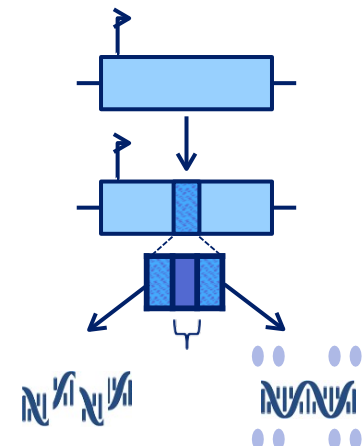
Manufacturing Capacity & Know-How

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



Broad Clinical Pipeline

Product Candidate	Indication	Research	Preclinical	Phase I/II	Status
Ophthalmology Programs					
AAV-CNGB3	Achromatopsia (CNGB3)	Orphan U.S. & EU; RPDD; PRIME Fast Track Designation			20 patients treated Ph I/II Study 91 patient natural history study
AAV-CNGA3	Achromatopsia (CNGA3)	Orphan U.S. & EU; RPDD			Phase I/II expected initiation in 1H 2019
AAV-RPGR	X-linked RP (RPGR)	Orphan U.S. & EU; Fast Track Designation			11 patients treated Ph I/II Study 100+ patient natural history study
AAV-RPE65	RPE65-Deficiency (RPE65)	Orphan U.S. & EU; RPDD			15 patients treated Study complete
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 Study complete Data published 2011, 2014, 2017 2018
AAV-UPF1	ALS / FTD (UPF1)				IND expected 2019

Salivary Gland Programs

AAV-AQP1	Xerostomia (hAQP1)	Orphan U.S.			5 patients treated Ph I Study Additional Ph I/II initiation 1H 2019
AAV-AQP1	Sjogren's Syndrome (hAQP1)				IND expected 2019

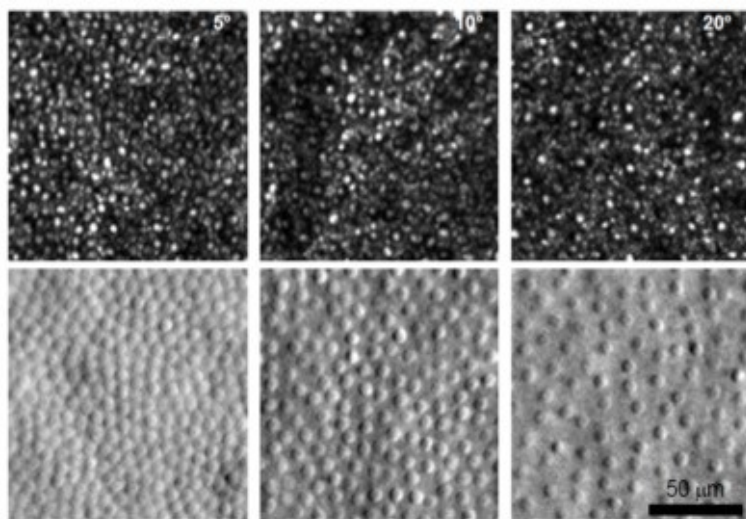
Ocular Franchise



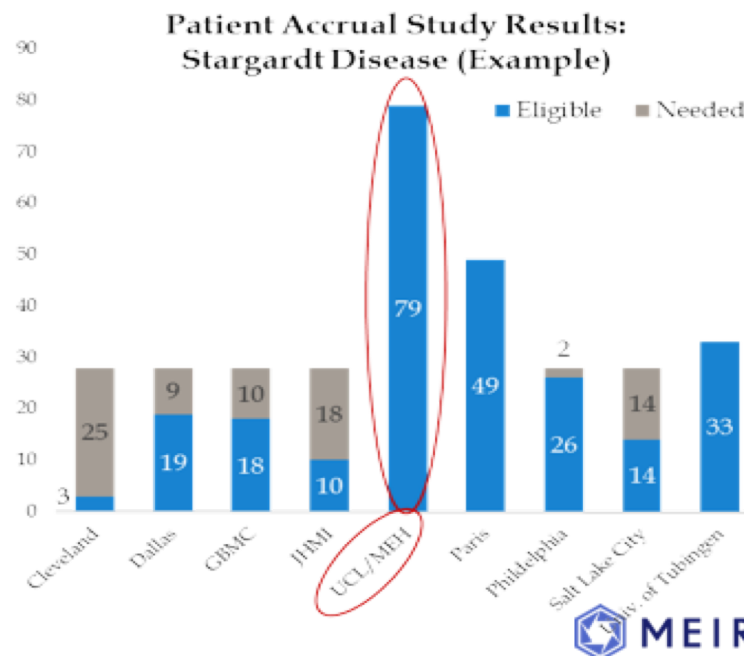
MeiraGTx Ophthalmology Franchise

- **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



Achromatopsia

Disease Overview

- **Achromatopsia Retinal defect:**
 - Complete absence of cone photoreceptor function from birth
- **Achromatopsia characterized by:**
 - Very poor visual acuity from birth (typically 20/200)
 - Photophobia (disabling aversion to light)
- **Achromatopsia prevalence and genes:**
 - Overall achromatopsia 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)

Normal

Day Vision



Achromatopsia

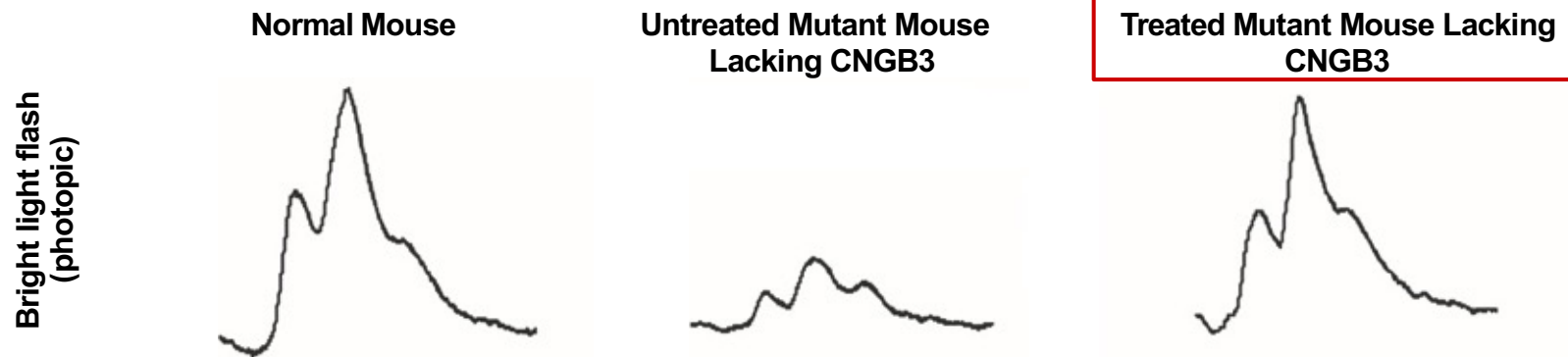


Construct Detail

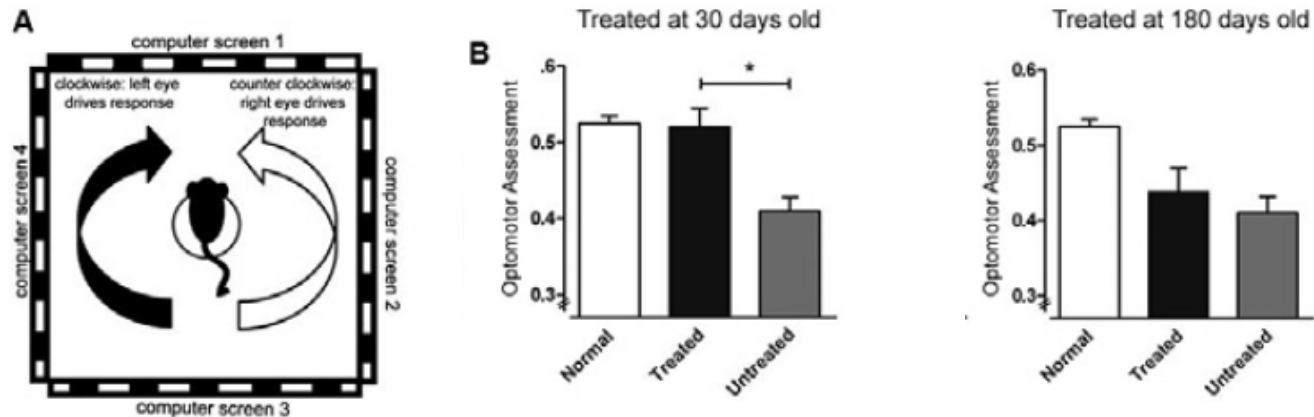
- **CNGB3 Construct**
 - **Promoter:** Human Cone Arrestin promoter (hCARp)
 - **Capsid:** AAV2/8
 - **Gene:** human CNGB3 cDNA
- **CNGA3 Construct**
 - **Promoter:** Novel synthetic cone specific (hG1.7p)
 - **Capsid:** AAV2/8
 - **Gene:** codon optimized human CNGA3 cDNA

ACHM Preclinical Data: CNGB3

Restoration of Cone Electrical Function

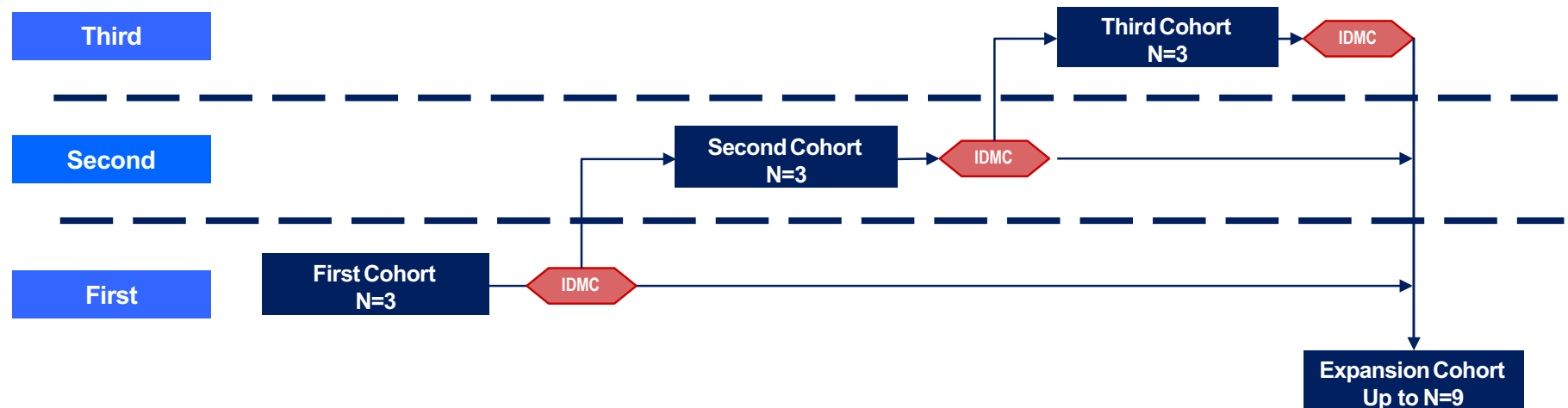


Restoration of Visual Function



ACHM caused by CNGB3: Phase I/II Study Design

Dose Level



- **Primary endpoint is 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, color vision, visual mobility, quality of life, nystagmus, and photoaversion
 - Treated 20 patients: 11 adults and 9 pediatrics treated – in UK and US with 3 surgeons
 - Subretinal injection targeting fovea and central retina: 200-500uL at doses 1E11-1E12 vg/mL
 - Top line data on all patients 6 months after the last patient treated – Q3 2019
- **ACHM Natural History Study:** ongoing with >90 patients
- **Regulatory designations:** Orphan Status US and EU; Rare Pediatric Designation; PRIME Designation; Fast Track Designation
- **CNGA3:** Phase I/II pediatric study to start in 1H 2019; Orphan Status in US and EU, Rare Pediatric Designation

X-Linked Retinitis Pigmentosa due to RPGR-Deficiency

Disease Overview

- Retinitis pigmentosa (RP) is a group of IRDs which represent 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
- Around 70% of XLRP patients have mutations in the RPGR gene
- Loss of night vision
- Progressing to tunnel vision
- Blindness in 4th decade

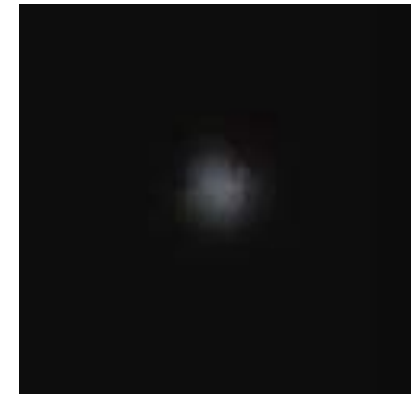
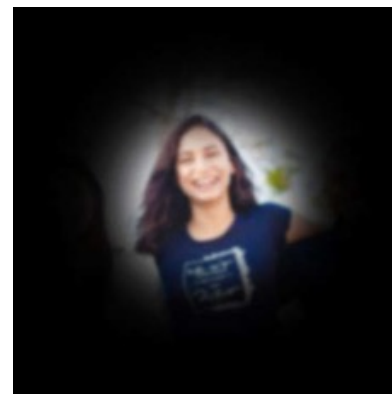
XLRP Patient Numbers

Prevalence	~1/40,000
Total Patients in US, EU5 & Japan	~20,000

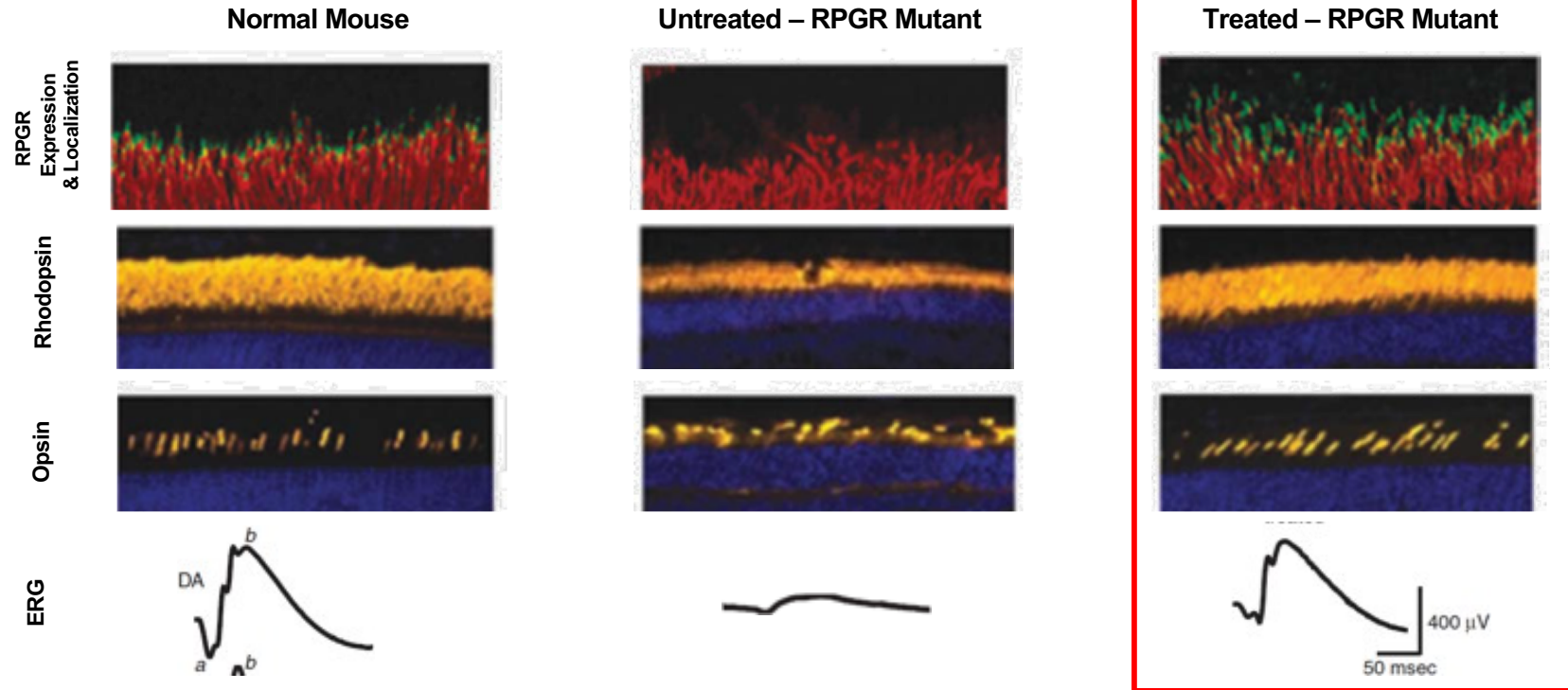
AAV2/5.RKp.hRPGR^{ORF15}

- **Promoter:** RKp (Human rhodopsin kinase promoter)
- **Capsid:** AAV2/5
- **Gene:** Stabilized RPGRORF15 cDNA

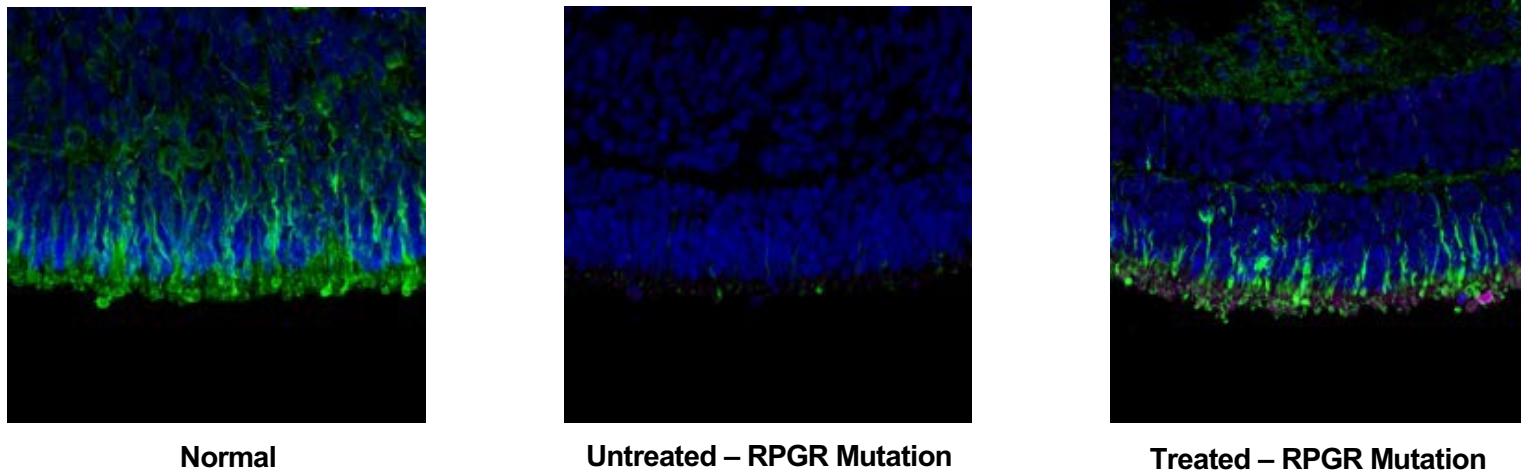
Disease Progression



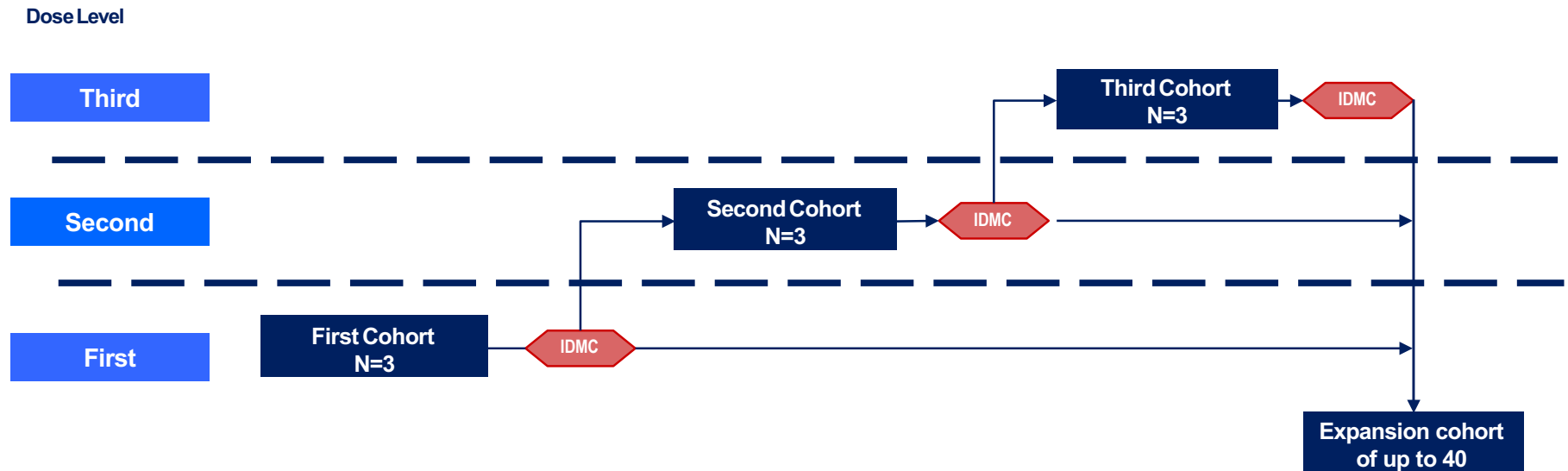
X-Linked Retinitis Pigmentosa: Preclinical Data



Human Pluripotent Stem Cell Retinal Organoids



X-linked Retinitis Pigmentosa: Phase I/II Study Design

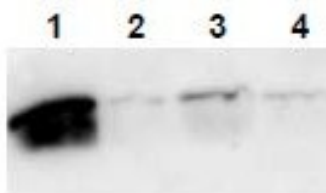
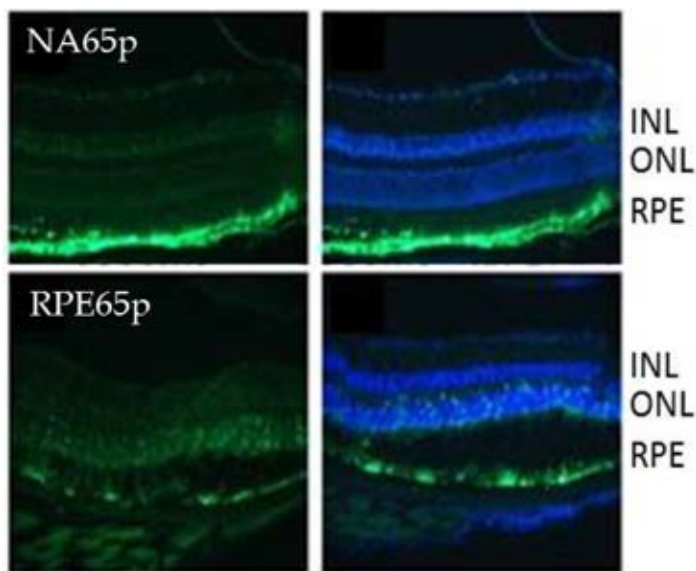


- **Primary endpoint is safety**
- **Secondary endpoints include:** static perimetry (Octopus 900 and visual field modeling & analysis), microperimetry, fundus imaging, optical coherence tomography, electroretinography, adaptive optics, contrast sensitivity, visual acuity, reading speed, mobility, quality of life,
 - 11 patients treated: 10 in dose escalation, 1 in pediatric cohort
 - Expansion cohort consisting of up to 40 patients randomized into 3 groups
 - Subretinal injection targeting the fovea and central retina: 250-750uL at doses 1E11-4E11 vg/mL
 - Preliminary data release around middle of 2019
- **Prospective Natural History** study ongoing with > 100 XLRP patients well characterized
- **Regulatory Designations:** Orphan Status US and EU; Fast Track Designation

RPE65-Deficiency - Optimized Vector Construct

Optimization Details

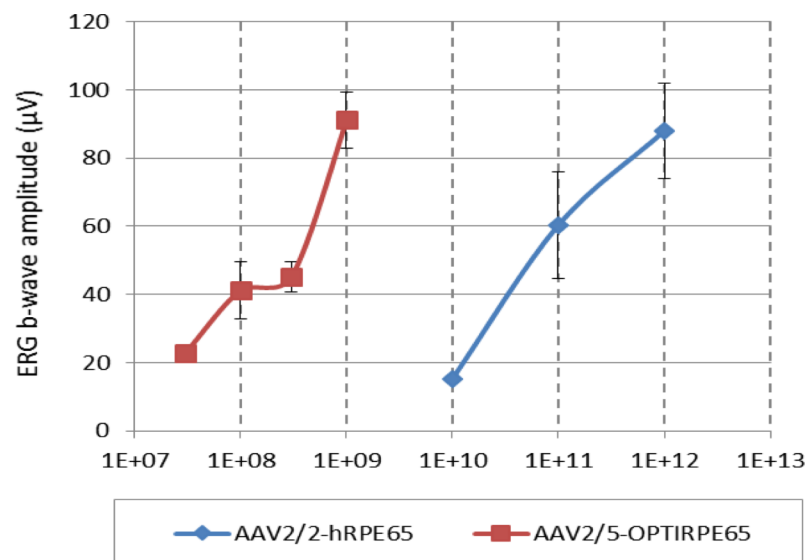
- **Capsid changed from AAV2 to AAV5:** 4x improvement in transfection efficiency of RPE
- **Promoter optimization:** 20x increase in protein expression
- **Codon optimization:** 7x increase in protein expression in human cells
- **SV40 intron increases mRNA stability:** 2.5x increased mRNA levels



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

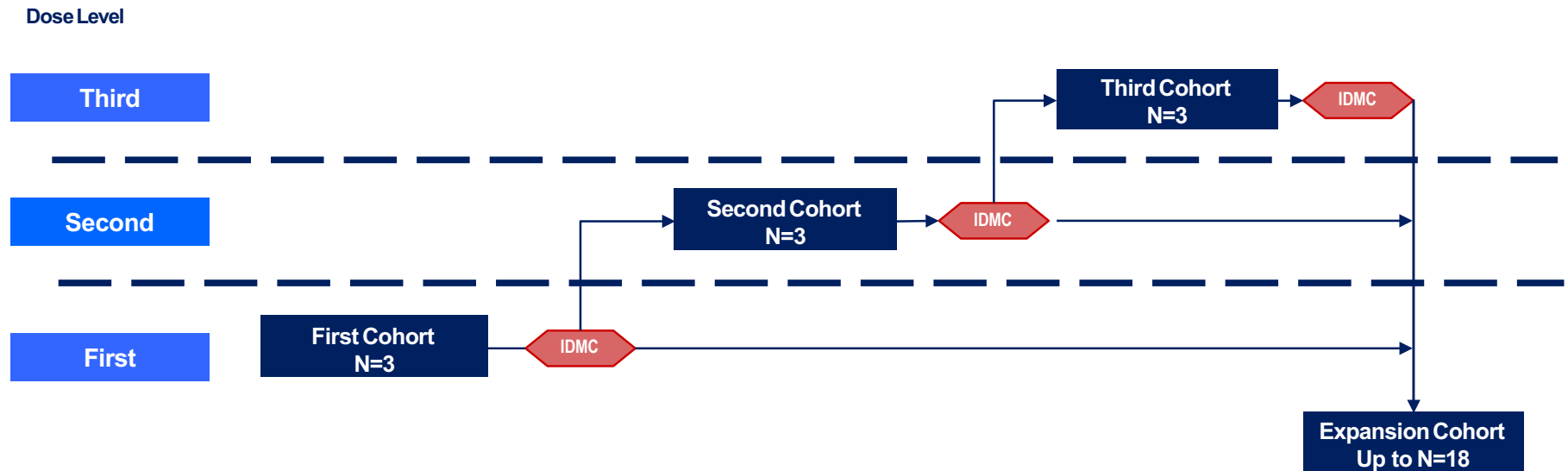
Head-to-Head Comparison

- 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



- Surgery and area of retina targeted
 - Subretinal injection targeting the central retina
Volume of injection may be up to 1ml
 - Multiple retinotomies permitted

RPE65-Deficiency: Phase I/II Study Design



- **Primary endpoint is 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 Q2 2018 in UK and US
 - 15 patients treated: 9 adults, 6 pediatrics
 - Subretinal injection targeting the fovea and central retina: 200-750uL at doses 1E11-1E12 vg/mL
 - Top line data on all full study 1H 2019 with a minimum of 6 months on every pediatric patient
- **Prospective Natural History** study ongoing with >30 patients well characterized
- **Regulatory Designations:** Orphan Status US and EU; Rare Pediatric Designation

Clinical Summary and 2019 Milestones

CNGB3:

- Phase I/II dose escalation in adults complete: 11 patients
- 9 pediatrics treated in UK and US
- Top line data 6 months after last patient treated – 3Q 2019

CNGA3:

- Phase I/II to start with dose escalation in pediatrics 1Q 2019

RPGR:

- 10 young adults treated in dose escalation
- 1 pediatric treated Q4 2018
- Extension study 1H 2019
- Preliminary data – mid 2019

RPE65:

- Completed dosing June 2018
- 9 adults, 6 pediatric
- Top line data on 6 month endpoints for full study - Q1 2019

Neurodegenerative Diseases



AAV-GAD Parkinson's Program



Parkinson's Disease

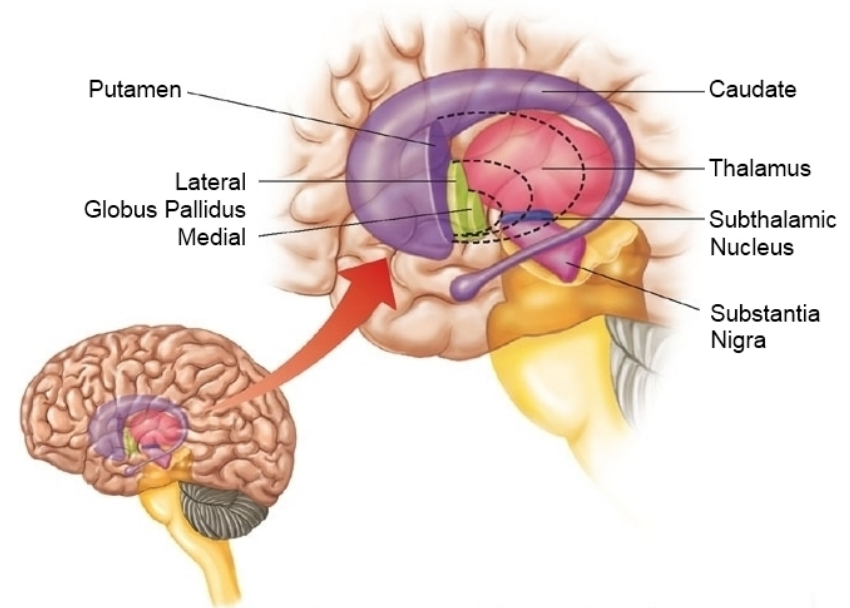
300,000 patients are refractory to pharmacological intervention

- Second most common neurodegenerative disease after Alzheimer's
- Estimated 1.5 million US PD patients according to NPF
- 60,000 new patients per year
- Cost to US healthcare system >\$6 billion
- Pharmacological treatment for dopamine responsive patients
 - L-DOPA
 - Dopamine agonists
 - COMT/MAO inhibitors (prevents breakdown of L-DOPA/Dopamine)
- After 5-10 years most patients become refractory to dopamine treatment
- Few options for refractory patients
 - Best Pharmacological Therapy - poor efficacy and severe side effects (dyskinesias, etc)
 - Deep Brain Stimulation (Deep Brain Stimulation) - effective but high SAE rate, permanent hardware, ongoing adjustment and tuning at highly specialized centers
- 300,000 PD patients in the US refractory to drug treatment

Parkinson's Disease: Principally Manifest as a Movement Disorder

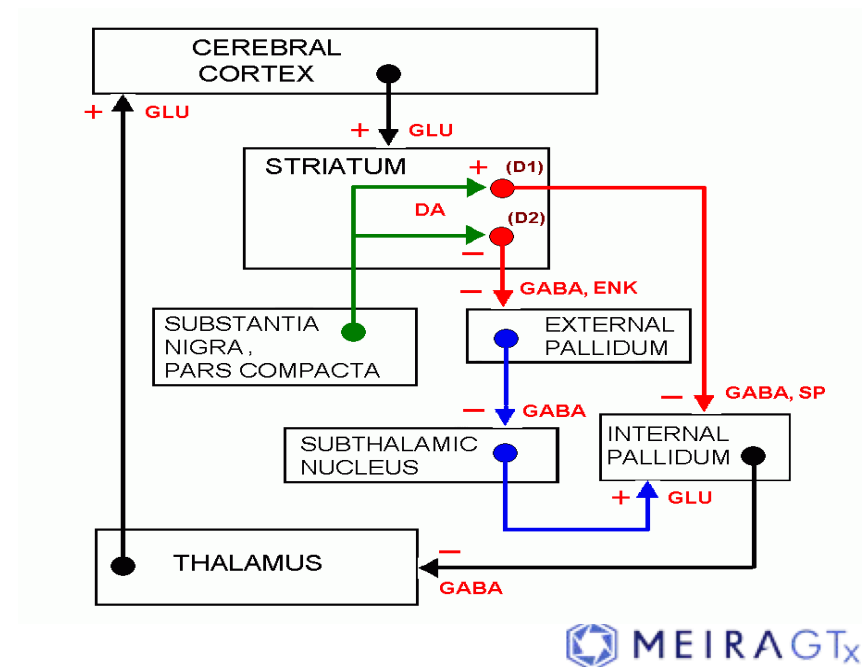
Voluntary Movement:

- Controlled by a group of interconnected nuclei in the brain called the **basal ganglia**



Normal Brain:

- Complex interaction of inhibitory and excitatory circuits regulate movement
 - GABA** – inhibitory
 - Glutamate** – excitatory



AAV-GAD Gene Therapy: A Novel Strategy To Rebalance Excitation and Inhibition in the Basal Ganglia

■ Parkinson's:

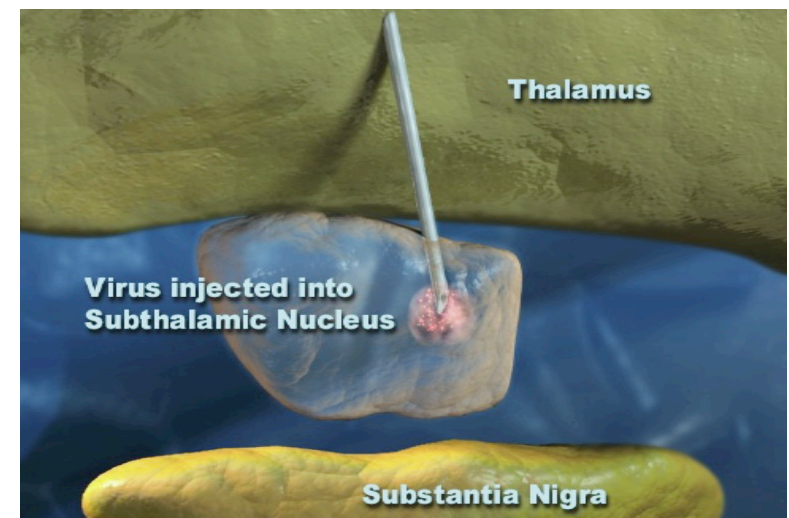
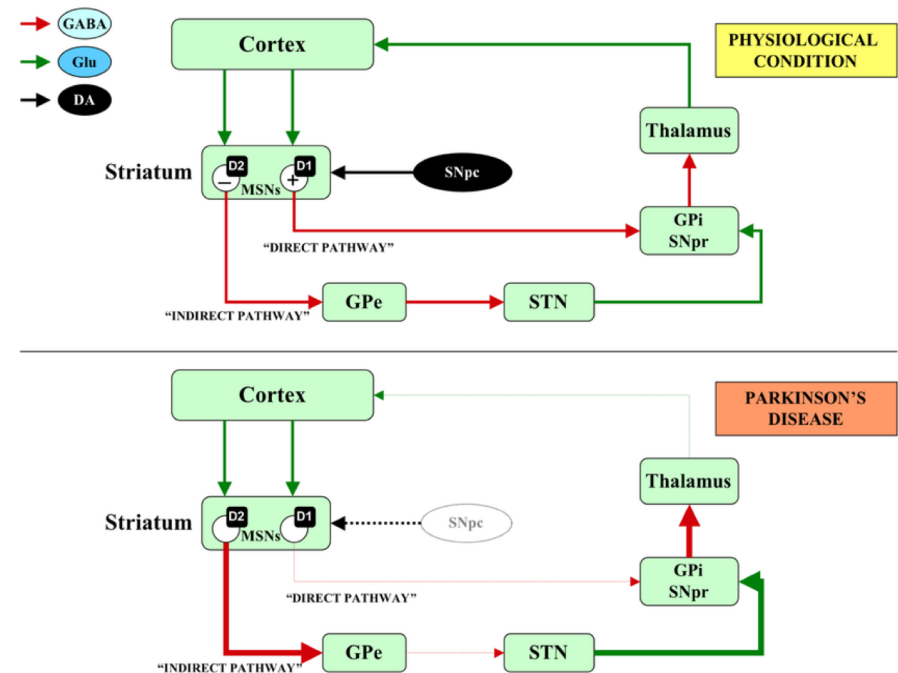
- dopaminergic neurons of the **substantia nigra** die
- A cascade of changes in basal ganglia circuitry occurs
- **Hyperactivity of the subthalamic nucleus (STN)** key output
 - Decreased GABAergic inflow (inhibition)
 - Increased glutamatergic outflow (excitation)

■ Invasive therapies to reduce hyperactivity of STN:

- Surgical lesioning
- Deep Brain Stimulation

■ AAV-GAD:

- **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: Phase I Clinical Trial Summary

Design:

- 12 advanced PD patients
- Unilateral injection into subthalamic nucleus
- 3 dose cohorts: 45uL viral vector solution at titers of 1E11vg/mL, 3E11vg/mL, 1E12vg/mL

Results:

- No SAE or toxic effects of GAD at 12mths
- No immune response to AAV2 capsid or transgenic protein
- UPDRS Part 3 motor rating: on and off states improved at 3, 6 and 12 months post surgery
- Consistent benefit restricted to limbs opposite to treated side
- PET scans at 12 months:
 - Improvement in abnormal Thalamus metabolism observed on treated side compared to baseline
 - Increased metabolism in supplementary motor areas on treated side
 - Changes highly correlated with UPDRS improvements

AAV-GAD: Phase II Clinical Trial Summary

Design

- Randomized, double blind, sham controlled study - 7 sites in the US
- 45 patients - 23 randomized to sham: 22 to AAV-GAD
- UPDRS Part 3 >25, PD for >5 years, levodopa responsive for >12mths, PD compatible PET scan
- Bilateral treatment: 35uL of 1E12vg/mL per side infused over 2.5hr

Results:

- AAV-GAD injection safe and well tolerated, no SAE related to treatment
- Met primary endpoint: UPDRS Part 3 improvement in per protocol group 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 and 12 months for AAV-GAD patients
- PET scans:
 - Decreased metabolism in striatum and thalamus, increase metabolism in premotor cortex
 - New polysynaptic pathways connecting STN to motor cortex

AAV-GAD: Summary

- **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
- **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

Radiation-Induced Xerostomia



Radiation-Induced Xerostomia

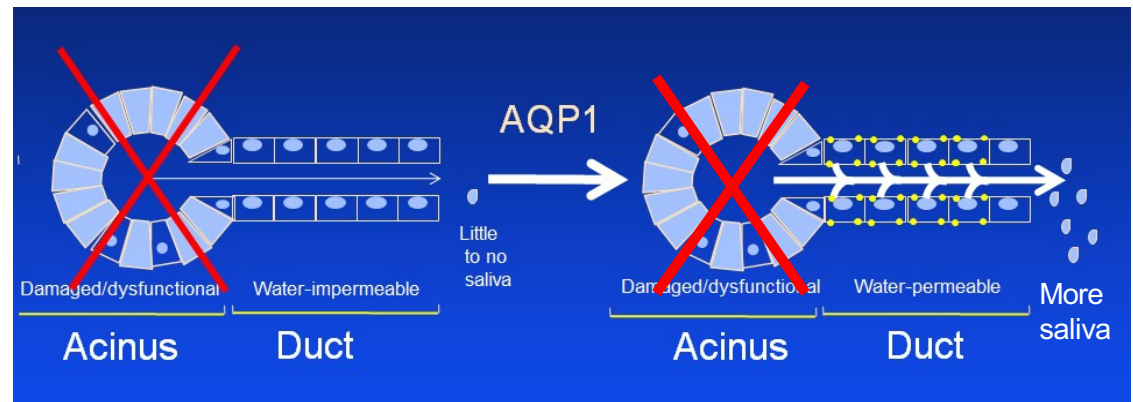
Significant Unmet Medical Need

- **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**

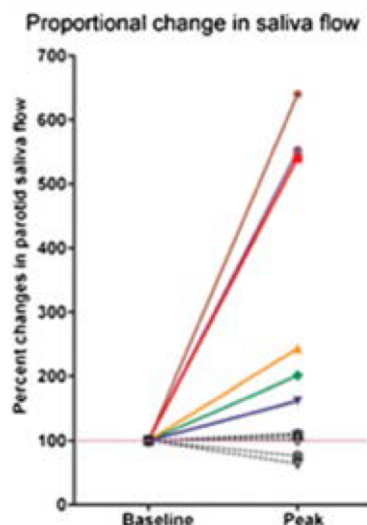
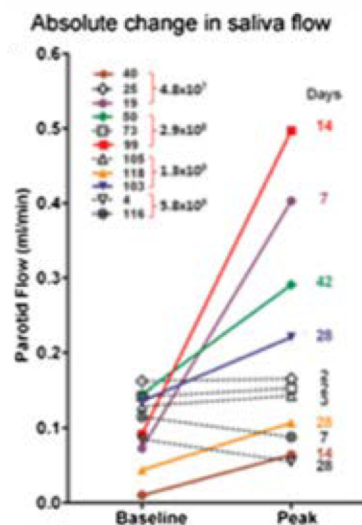


Human Proof-of-Concept in Xerostomia Patients using AdhAQP1

Change in Parotid Salivary Flow Rate

Absolute

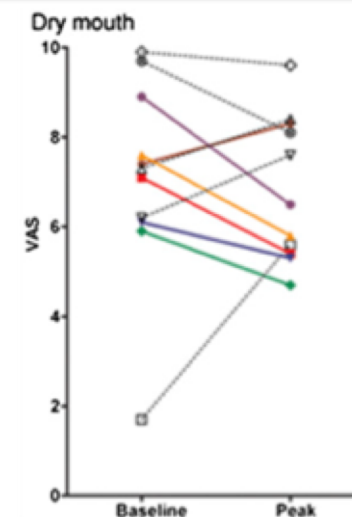
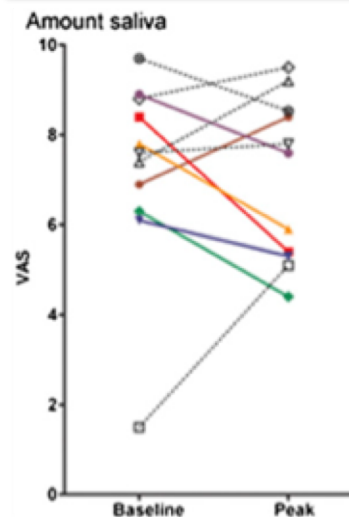
Proportional



Visual Analogue Scale (VAS) Results

Rate Amount of Saliva in Your Mouth

Rate Dryness of Your Mouth



Note that lower visual analogue scale (VAS) results indicate reduced perception of dry mouth

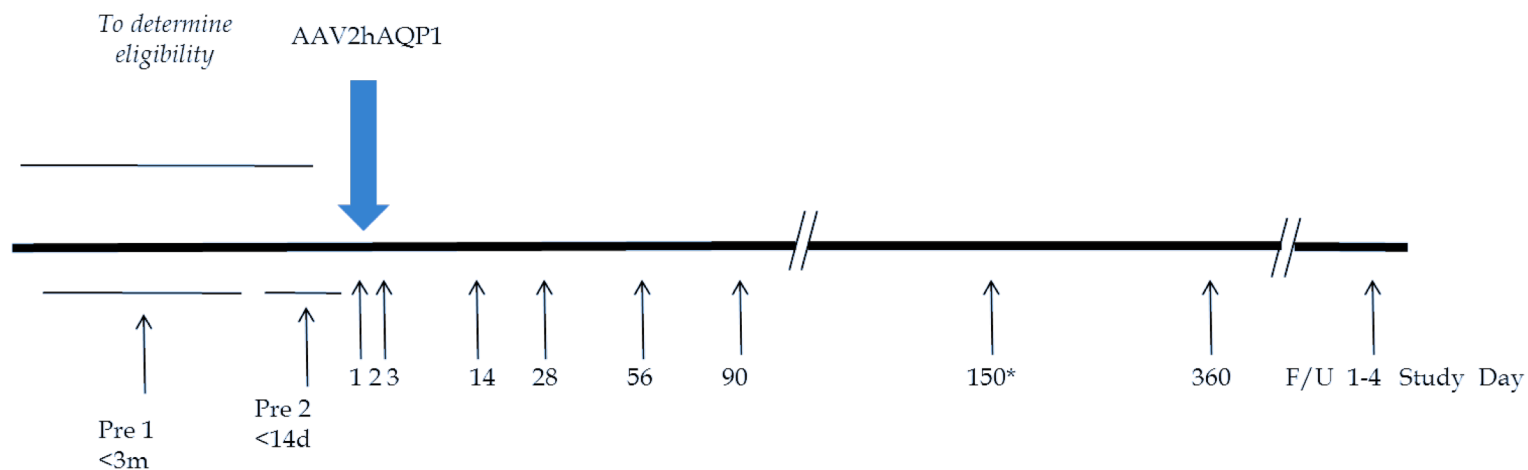
- AdhAQP1 treatment was reported to be well tolerated: no SAEs
- Responses seen in 2 of 3 patients in each of the first 3 cohorts
- 60% to 540% increase in parotid flow in responding patients
- 5 of 6 patients also reported subjective improvement in symptoms

Baum BJ et al. *PNAS*, 2012.

Radiation-Induced Xerostomia Phase I Study

- 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**

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}

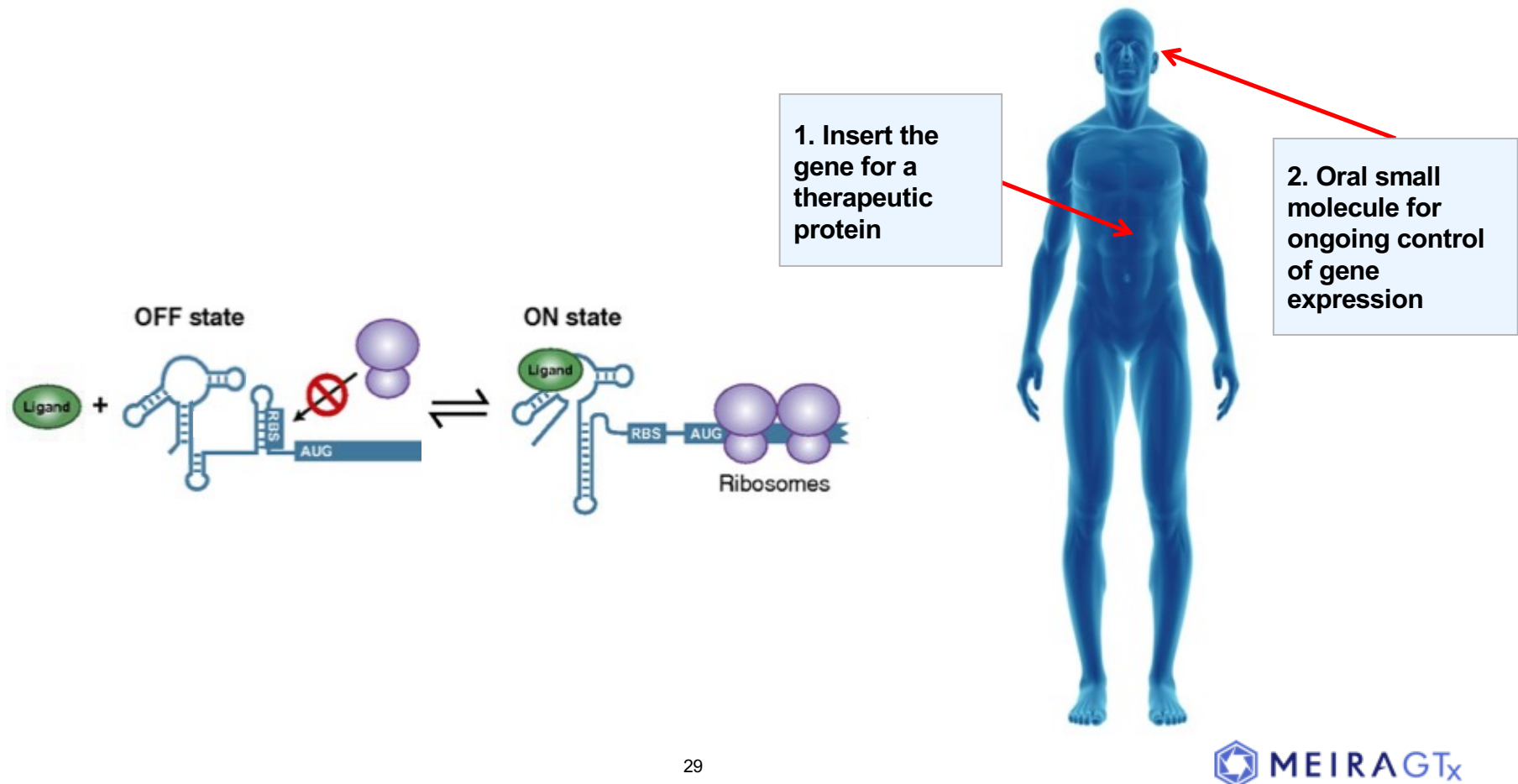


Gene Regulation



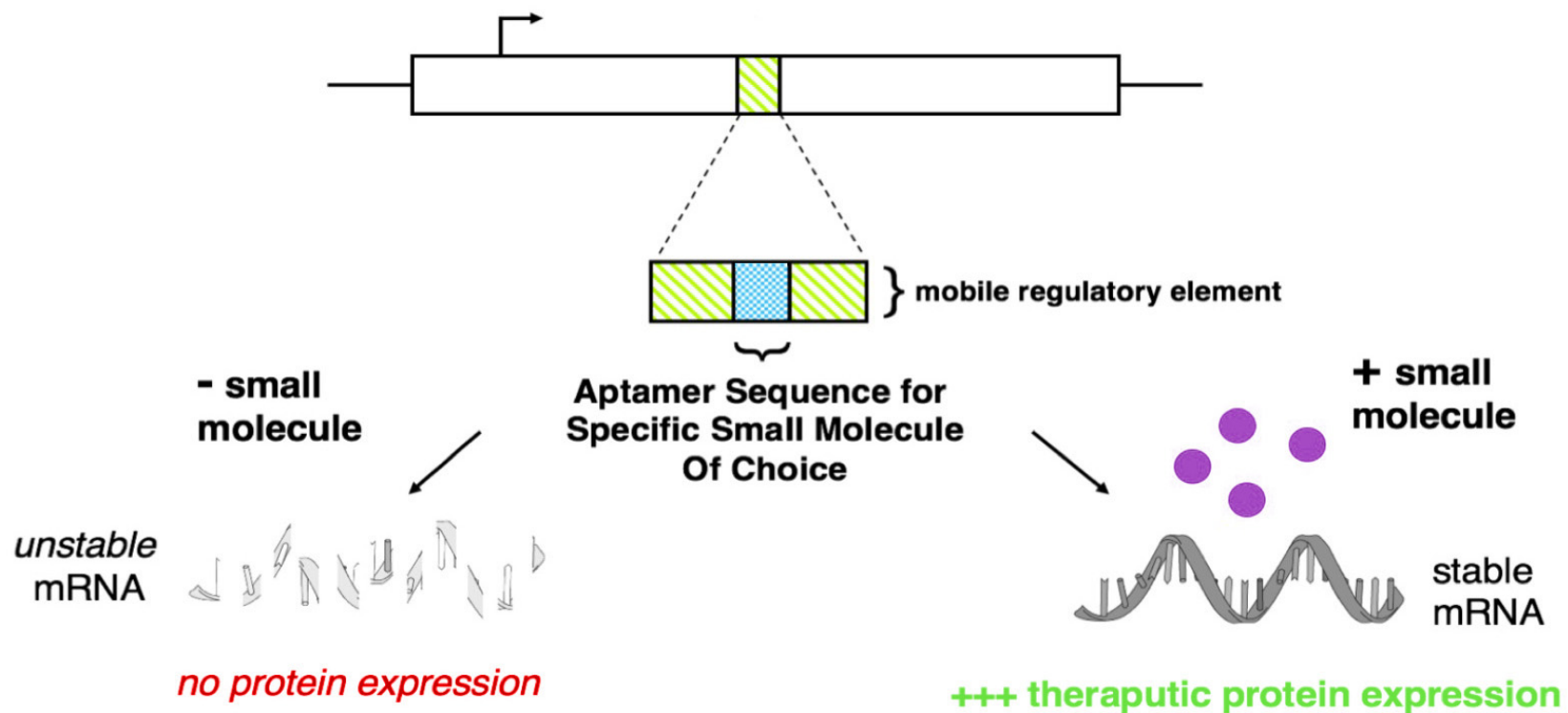
Next Generation Gene Therapy: Gene Regulation Platform

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

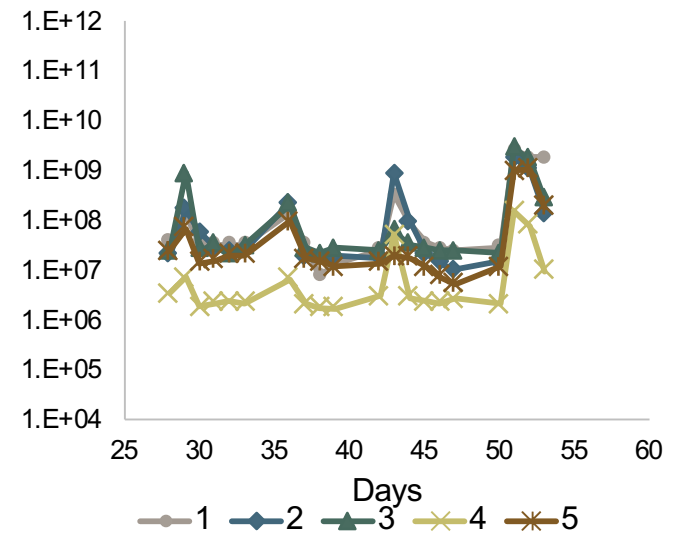
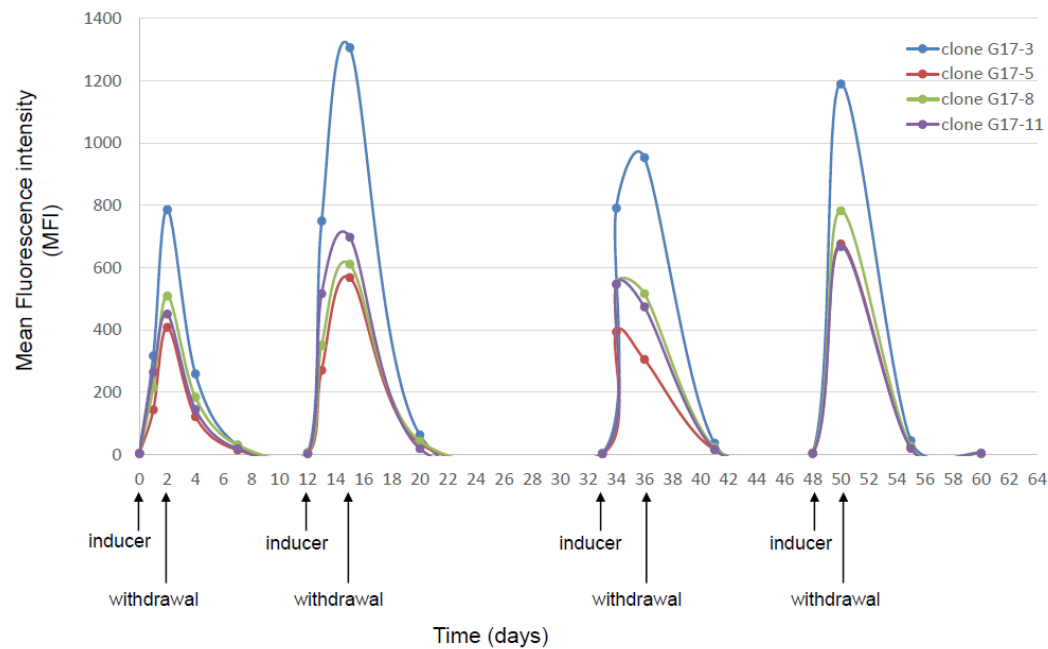
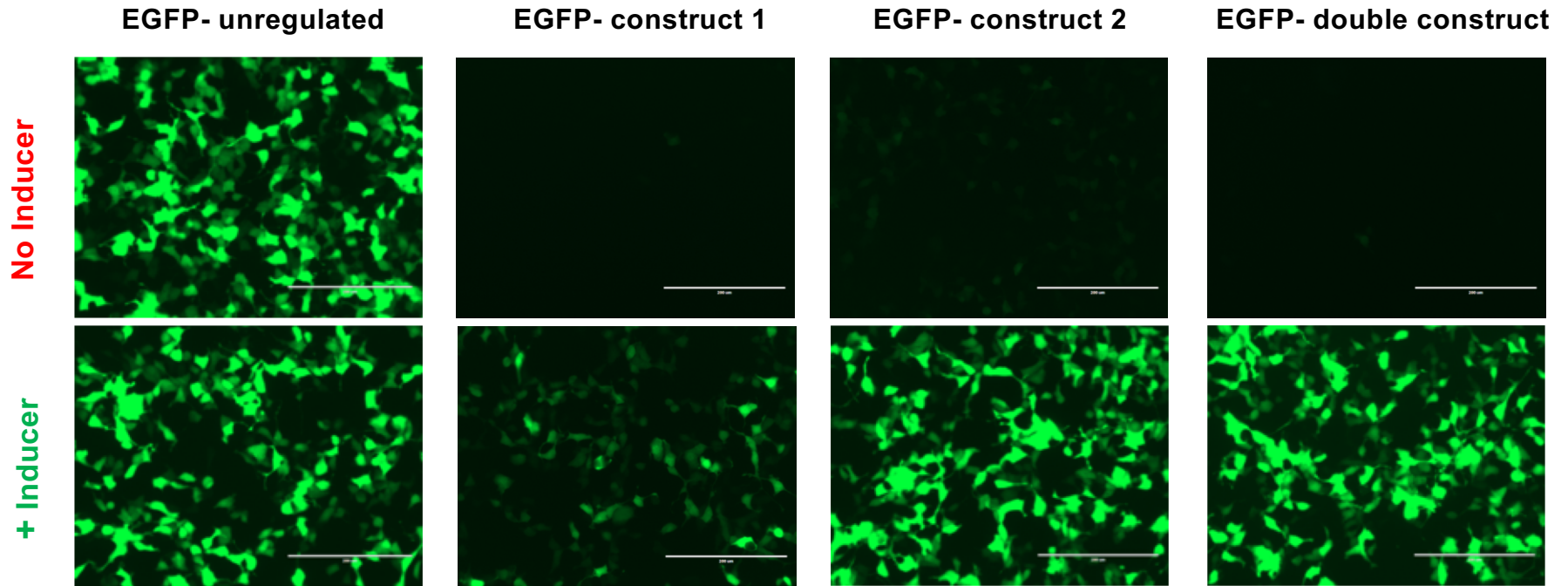


Regulation of Transgenes using Proprietary Riboswitch Technology

Regulation driven by specific small molecule / RNA binding driving functional changes in RNA configuration, splicing and translation



Regulation of Transgene Expression



Manufacturing and Process Development



Manufacturing Leadership Team

James Christie, MBA, BSc SVP, Manufacturing and Supply Chain

- Ex GSK, CellTech, Centocor, Protherics, BTG, Oxford BioMedica, UK Industry Associations: ABPI and BIA
- Government policy lead for the Medicines Manufacturing Industry Partnership
- Led development of gene therapy manufacturing & supply chain capability for OxfordBioMedica
- Appointed as MeiraGTx VP Manufacturing and Supply Chain January 2016

Ian Delmage, MBA, Cbiol, MRSB Head of Quality

- Prior to MeiraGTx, he was a Senior Compliance Professional, Global Pharma QA with Novartis
 - Responsible for the performance of state-of-the-art GxP audits per the Novartis Quality System Standards
- Prior to Novartis, he worked with Chorus Europe, Eli Lilly & Company, Emergent Biosolutions, UCB, Celltech and Glaxo Wellcome

Francois-Xavier Toubhantz Production Manager

- Prior to MeiraGTx, he held various roles at Oxford BioMedica including Senior Biotechnologist and Production Manager
 - Part of the teams involved in the design and build of Oxford BioMedica's facilities in Yarnton and Cowley
 - Oversaw production of internal and third party clinical grade materials
 - Active role in regulatory (FDA & MHRA) and client audits
- Engineering degree (Ingénieur diplômé) MSc in Food Production Systems at Lille University (France), with a specialization in Fermentation processes

Bastiaan Leewis MSAT Manager – Industrialization

- Prior to MeiraGTx, was part of leading Cell and Gene Therapy CMOs
- Development and implementation of PM systems for Technology transfers
- Led technology transfer of Provenge to the EU from CMO side
- Led implementation large scale systems for allogeneic therapies
- Led development of gene therapy manufacturing platform

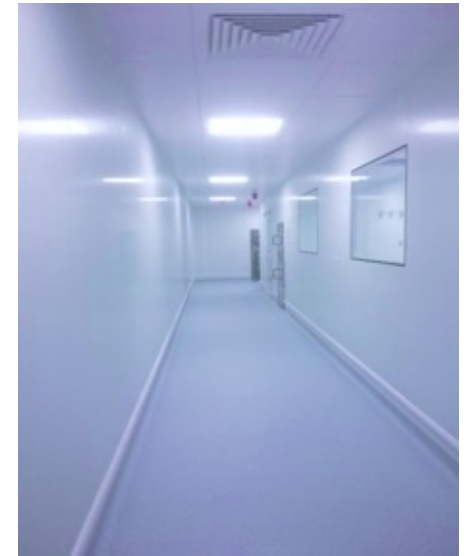
cGMP Certified Manufacturing Facility: Flexible and Scalable

■ 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



MeiraGTx Manufacturing: The Facility



Strategic Importance of Flexible cGMP Manufacturing Facility

Allows Us to Manufacture all of the Material Needed to Expedite Our Clinical Development

- **If positive data positive from our IRD programs we may have 3 pivotal programs by end 2020 RPE65, RPGR and CNGB3 – all require commercial cGMP material**
- **A pivotal program for AAV-GAD would also require commercial cGMP material**
- **We have 3 pre-IND programs in Sjogren's, ALS and wet AMD – 3 additional Phase I/II clinical programs require cGMP manufactured material**
- **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
- **Manufacturing flexibility and capacity to produce material for 4 potential pivotal programs and 3 potential Phase I/II clinical studies over the next 18 months**

Highlights and Milestones

- *Diverse Pipeline of rare genetic disorders and common non-genetic disorders*
- *6 Clinical Programs with 18 Regulatory Designations and at least 3 data readouts over the next 9 months*
 - *RPE65 – data 1Q 2019*
 - *RPGR – data mid 2019*
 - *CNGB3 – data 3Q 2019*
 - *CNGA3 – Phase I/II study in pediatrics to start 1H2019*
 - *RIX – Phase I/II open label preliminary data 2019*
 - *AAV-GAD – path to regulatory approval - mid 2019*
- *Internal Flexible and Scalable cGMP Manufacturing Facility*
- *Transformative Gene Regulation Technology Platform*
- *Cash \$88.6 million as of September 30, 2018*

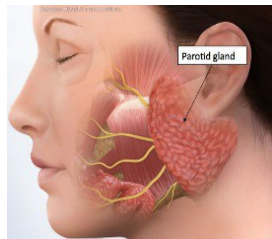
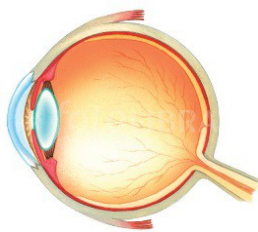
A Vertically Integrated, Clinical Stage Gene Therapy Company

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

Diversified Pipeline of Gene Therapy Candidates

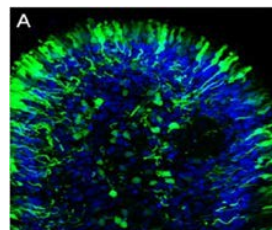
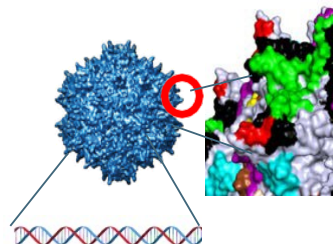
5 ongoing clinical programs:

- 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



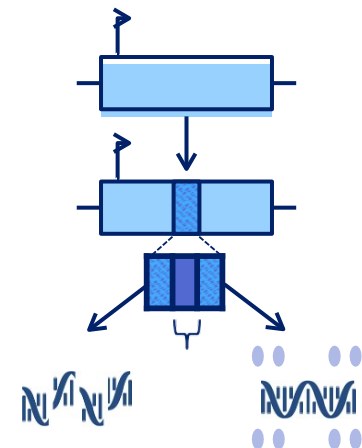
Manufacturing Capacity & Know-How

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





Corporate Overview

January 2019

