MEIRAGTx

Jefferies Healthcare conference
June 2026

MeiraGTx: late-stage clinical pipeline and comprehensive end-to-end capabilities & technologies in genetic medicine

Diverse Program Pipeline

Broad pipeline across neuro, salivary gland, and ophthalmology

4 pivotal and BLA ready programs:

- Radiation-induced xerostomia
- X-linked retinitis pigmentosa
- Parkinson's disease
- AIPL1 retinal dystrophy (Eli Lilly)

Diverse preclinical pipeline:

- ALS, intractable neuropathic pain, obesity & diabetes, large ophthalmology indications Stargardt's, wet and dry AMD

Unlimited potential of transformative Riboswitch-platform

End-to-End GMP Manufacturing

In-house manufacturing and industry-leading process

- 2 cGMP viral vector manufacturing facilities
- cGMP plasmid production
- QC facility for release and stability
- Fill & Finish, warehouse and supply chain
- Dedicated MSAT facility
- Commercial ready Platform Production Process

Commercial licenses for viral vector production and QC

Next-Generation Vector Optimization

Improved potency & safety, lower dose and lower COGS

- >250k promoter library
- AI enhanced promoter optimization
- Proprietary intravitreal capsids
- Capsid development: muscle, CNS
- Human organoids

Improve potency up to 3 to 4 logs, reducing dose 3-4 logs, reducing Cost of Goods and improving safety

Transformative *in vivo* production Technology

Proprietary Riboswitch platform for precise control of therapeutic proteins

***in vivo* production of any therapeutic protein via bespoke small molecule activation of transgene.**

- **Gene agnostic**: multiple antibodies, peptides, hormones, nucleases, cell therapy validated in animal models
- **Delivery agnostic:** AAV, Lentivirus and CRISPR all demonstrated equivalent tight control
- **Leptin**: first into the clinic, 2026
- **Neuropathic Pain:** Clinic 2027

Broad Pipeline of Transformative Genetic Medicines

Advanced clinical programs across multiple therapeutic areas

Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3 / Registrational
Salivary Gland					
AAV-AQP1	**Radiation-induced xerostomia**	*Breakthrough Designation, RMAT, Orphan*			
	Sjögren's disease	*IND ready*			
	PSMA radioligand xerostomia prophylaxis and treatment				
Neurodegenerative Disease					
AAV-GAD[1]	**Parkinson's disease**	*RMAT*			*Phase 3 ready*
AAV-UPF1, AAV-CNTFR	**ALS**				
Ophthalmology					
Botaretigene sparoparvovec	**X-linked RP (RPGR)**	*PRIME, Fast Track, Orphan Drug*			
AAV-AIPL1	**LCA4 congenital blindness** Lilly	*RPDD, Orphan Drug, MHRA Specials License*			
AAV-ABCA4	**Stargardt's disease**				
AAV-VEGFR2	**Wet AMD**				
Undisclosed	**Dry AMD/GA**				
BBS10	**Bardet-Biedl syndrome**	*RPDD, Orphan Drug*	*Developed under MHRA Specials License*		
Riboswitch Regulated Therapies					
RiboLeptin	**Lipodystrophies**				
Undisclosed	**Intractable neuropathic pain**				
GLP-1, GIP, incretin combinations	**Obesity/MASH/Metabolic Disease**				
Ribo-CAR-T	**Oncology, autoimmune disease**				
Genetic Obesity					
AAV-BDNF[2]	**MC4R/BDNF genetic obesity**				

[1] Joint venture with Hologen AI (see press release here)

Four pivotal stage programs in prevalent and rare indications

Radiation-Induced Xerostomia

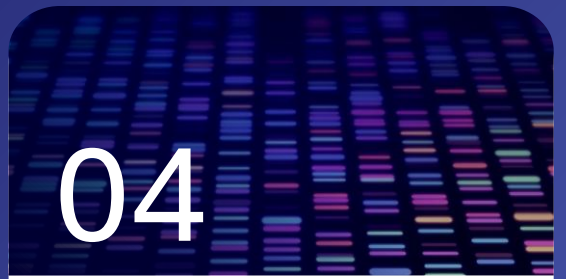
- Pivotal Phase 2
- **Potential BLA filing mid-2027**
- **Target launch early 2028**
- Large patient population with no effective therapies available
- ‘Pipeline in a product’

X-Linked Retinitis Pigmentosa (RPGR)

- Completed Phase 3
- BLA and MAA ready – PPQ complete
- MeiraGTx manufactures commercial product

AIPL1-Associated Congenital Blindness

- Developed under ‘specials’ license
- **Near term BLA and MAA filings– FDA and MHRA**
- **Potential approvals 2026**
- Transformative effect - 11/11 blind to seeing children under 4 years



Parkinson’s Disease

- Phase 3 ready
- **Potential BLA filing in 2028**
- Large patient population inadequately controlled by dopamine therapy

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Strong industry partnerships

In November 2025, MeiraGTx entered into a broad strategic collaboration with Eli Lilly to develop and commercialize genetic medicines in ophthalmology

MeiraGTx to receive $75 million in upfront cash, as well as up to $135 million in near-term milestone payments related to AAV-AIPL1.

MeiraGTx is also eligible to receive additional milestone payments and tiered royalties on licensed products.

Under the terms of the agreement, Lilly obtained:

- Worldwide exclusive rights to MeiraGTx's AAV-AIPL1 product for LCA4,
- Exclusive license to certain MeiraGTx proprietary intravitreal capsids as well as certain proprietary promoters for use with up to five ocular disease targets
- A Right of First Negotiation (ROFN) to MeiraGTx's proprietary Riboswitch Technology in the field of gene editing in the eye

Link to press release

In October 2023, MeiraGTx received a $30 million strategic investment from Sanofi through sale of 4 million ordinary shares at $7.50 per share

Sanofi received a Right of First Negotiation (ROFN) for MeiraGTx's phase 2 xerostomia program, as well as for the use of MeiraGTx's Riboswitch gene regulation technology in certain targets:

- Immunology and Inflammation (I&I), including IL-4 and IL-13
- GLP-1 and other gut peptides for metabolic disease and obesity
- Central Nervous System (CNS)

In August 2024, Sanofi made an additional $30 million equity investment in MeiraGTx as part of a $50 million offering of ordinary shares

Link to press release

MeiraGTx entered into a strategic collaboration with Hologen AI to expedite Phase 3 development of AAV-GAD and industrialize MeiraGTx's proprietary manufacturing process

- **MeiraGTx to receive $200 million in upfront cash consideration**
- MeiraGTx and Hologen will form a JV with an additional $230 million committed capital from Hologen to fund 100% of AAV-GAD program through to commercialization, as well as other potential pipeline products
- Hologen will also fund a portion of MeiraGTx's manufacturing operations and will own a minority stake in MeiraGTx's manufacturing subsidiary

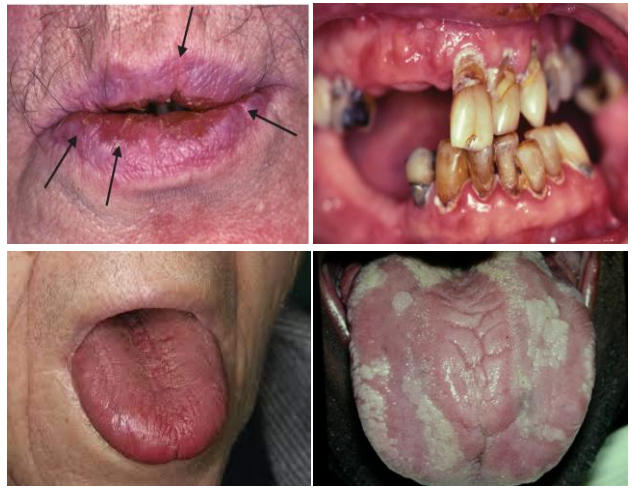
Link to press release

AAV-AQP1 for Persistent Grade 2/3 moderate to severe Radiation Induced Xerostomia (RIX)

Persistent Grade 2/3 Moderate to Severe Radiation Induced Xerostomia (RIX)

Persistent, late, RIX is a severe, untreatable, lifelong condition with devastating consequences for >30% of survivors of head and neck cancer.

- Extreme dry mouth, inability to swallow or chew, lose sense of taste
- Major diet restrictions, ongoing weight loss, need for invasive tube feeding
- Oral health complications, frequent oral infections, sores, persistent pain.
- Uncontrolled dental caries, accelerated loss of dentition requiring major reconstruction
- Impaired speech, difficulty sleeping, inability to exercise
- Social isolation and refusal to interact with others.



Poor nutrition, lack of sleep, inability to exercise, continual pain, loss of social interaction have a significant, life-changing impact and may lead to frailty and premature death

*"… **People can't have a normal life**. They go around with these sprays to moisturize the mouth … when **they wake up in the morning and try to open their mouth, the skin tears** and they have mouth ulcers…" Medical Oncologist, AMC (IT)*

"It was like I had paper cut my tongue 100 times and then you suck on a lemon." **JANET**

"If I start choking, I can't get the food back out of my mouth which is really terrifying." **CARRIE**

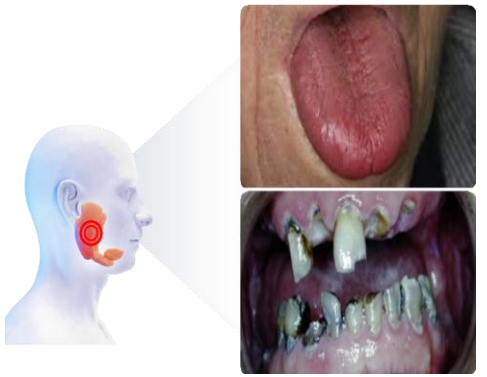
AAV-AQP1 for treatment of persistent grade 2/3 moderate and severe radiation induced late xerostomia (RIX)

Disease Mechanism

- Salivary glands are particularly vulnerable to radiation
- Damage to gland during radiation leads to xerostomia in almost all patients with H&NC treated with radiation
- **In 30-40% of patients cured of HNC Grade 2/3 moderate to severe xerostomia persists for life.**

Therapeutic Mechanism

- AAV-hAQP1 is instilled into the duct of damaged glands and transduces the remaining gland epithelium
- Vector genome encodes Aquaporin 1 (AQP1), a non-polarized water channel
- **Expression of AQP1 makes the epithelium permeable to water and allows water to flow down the concentration gradient into the salivary duct and into the mouth**



AAV-AQP1 one-time delivery

- **Small dose delivered locally directly to salivary gland**
- **Simple in-office procedure**
- **No general anesthesia**
- **One-time therapy**
- **Low cost of goods**

Transformative improvements demonstrated in Phase 1 dose escalation study

- **Unprecedented improvements in both PROs and saliva flow observed at 12 months**
- **Durability of benefit demonstrated out to 3 years post one-time treatment**

AAV-AQP1 has the potential to be a disease modifying therapy with durable, transformative benefits for this otherwise severe, lifelong, untreatable condition

Clinicians view the strong benefit, curative treatment, and durability of effect as highly meaningful

- **Clinicians consider this a one-time treatment with disease modifying effect on a severe, otherwise untreatable lifelong condition**
- **Clinician Preference Share: ~78% global adoption based on clinician preference**

Physicians highlight:

- **Transformative benefit in pivotal endpoints** (PRO and water flow)
- **Good safety profile**
- **Minimally invasive one-time dosing**
- **Strong 3-year durability**

*"… **I will say if the data shows that it is providing durable responses, maybe I will try to do it for all [Grade 2 or Grade 3 (moderate or severe)] patients** …" Medical Oncologist (U.S.)*

*"… **In the course of my career, I have had four or five experiences where I saw something and I said, 'That is freaking awesome'. This is something that I have to integrate into my practice, and this is one of them …"** Oral and Maxillofacial Surgeon, (U.S.)*

*"… **This is the first disease-modifying treatment for xerostomia, and I am very excited that it might be available**…" Medical Oncologist (U.S.)*

*"… **This is the first disease-modifying treatment for xerostomia, and I am very excited that it might be available**…" Medical Oncologist (U.S.)*

*"…**if this product is effective for 5 years**….**especially if it is a one-time administration that is pretty attractive**" Medical Oncologist (U.S.)*

***"Patients will be motivated to get that product, otherwise the status quo is that the xerostomia they get from radiation will be permanent. That would be a big motivator for patients to get this one-time procedure"** Radiation oncologist (US)*

***"A strength is, of course, the way of application. It's relatively easy in the outpatient setting without severe surgery and anesthesia"** Medical oncologist (DE)*

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Source: Based on survey and interview feedback with U.S., UK+EU4, JP physicians (N=75); * Overstatement adjustments were applied to individual responses as follows – 80% for "Definitely would", 60% for "Probably would", 25% for "Might or might not", and 0% for "Probably would not" and "Definitely would not"

Significant commercial opportunity for a first-in-class treatment for large population of persistent RIX with severely debilitating unmet need

AAV-AQP1 has the potential to provide meaningful durable benefit for xerostomia patients and to be the first and only disease-modifying therapy for RIX

SEVERE UNMET MEDICAL NEED

- Almost all patients treated with radiation for H&N cancer get xerostomia
- **30-40%** of RIX patients **do not recover and xerostomia persists for life**
- **83%** of treated grade 2 & 3 late RIX patients do not adequately benefit from available therapy and have no treatment options
- **Severe permanent condition with no effective treatment**
- **165k patients in the US ; 20k each year**

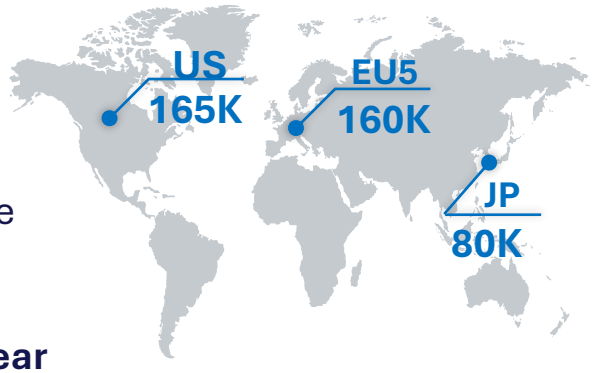
LOW DOSE, LOW COGS

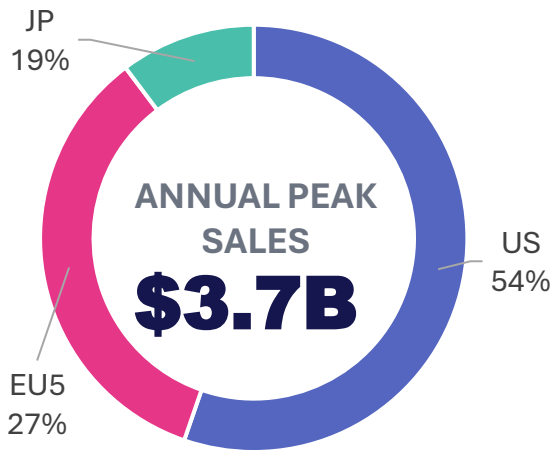
- **Small dose**
- **Locally delivered**
- **In-house manufacturing**
- **Low COGS**

SIGNIFICANT PATIENT POPULATION

405K

405k patients currently living with persistent grade 2/3 late RIX in the 7 major markets, with **48K new cases diagnosed each year**





LARGE POTENTIAL MARKET

Annual peak sales (**late RIX only**) of **$3.7 billion globally**

Opportunities for label expansion and increased revenues:

- PSMA radioligand therapy
- Sjogren's disease

Access to >60% of the U.S. population >55 years old with HNC by targeting a concentrated set of 15 major metro areas with a 3-hour driving catchment radius

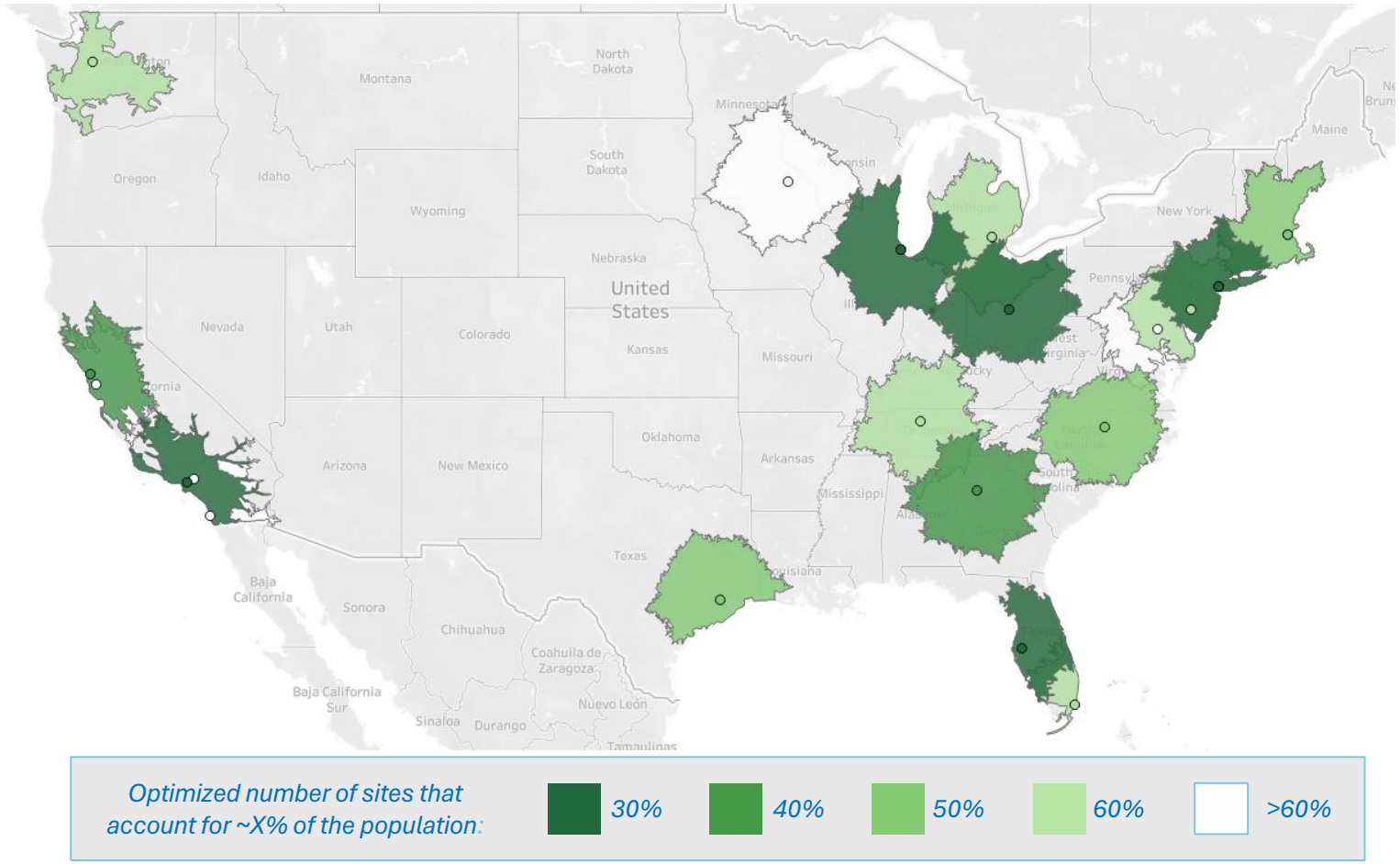
U.S. Population and HNC hospitals

Key assumptions and inputs

- HNC is **distributed evenly** across the U.S. population
- Population **>55 years** considered*
- Catchment areas include a **drive radius of 3 hours; NYC was prioritized over others in radius** due to hospital strength
- **Top 25 HNC hospitals** were identified using overall **cancer ranking (e.g., U.S. News), ENT specialty ranking, surgical volume, clinical trial leadership, and NIH research funding**

Key conclusions

Access to >60% of the population would require **15 sites**



Note: *>80% of HNC is in population >55 years old
Source: U.S. Census; Hospital websites

Of the leading head and neck cancer centers, MeiraGTx can optimize reach based on the cumulative population addressed; diminishing incremental reach is seen after ~17 hospitals in the top 25

Optimized number of sites that account for ~X% of the population: 30% 40% 50% 60% >60%

	Metro area	Key HNC Hospital	Catchment population (% of total pop.)	Cumulative population (% of total pop.)
1	New York, NY	Memorial Sloan Kettering Cancer Center	10.8%	10.8%
2	Los Angeles, CA	UCLA Jonsson Comprehensive Cancer Center	6.3%	17.1%
3	Chicago, IL	Northwestern Medicine / Robert H. Lurie Cancer Center	5.4%	22.4%
4	Columbus, OH	Ohio State University / James Cancer Hospital	5.0%	27.4%
5	Tampa, FL	Moffitt Cancer Center	4.6%	31.9%
6	Atlanta, GA	Winship Cancer Institute / Emory University	4.0%	35.9%
7	San Francisco, CA	UCSF Helen Diller Family Comprehensive Cancer Center	3.9%	39.8%
8	Boston, MA	Massachusetts General Hospital	4.7%	43.2%
9	Durham, NC	Duke Cancer Institute	3.7%	46.6%
10	Houston, TX	MD Anderson Cancer Center	3.1%	49.7%
11	Philadelphia, PA	University of Pennsylvania / Abramson Cancer Center	11.9%	52.6%
12	Nashville, TN	Vanderbilt-Ingram Cancer Center	2.8%	55.1%
13	Ann Arbor, MI	University of Michigan Rogel Cancer Center	4.8%	57.5%
14	Miami, FL	Sylvester Comprehensive Cancer Center / Univ. of Miami	3.1%	59.5%
15	Seattle, WA	University of Washington / Fred Hutchinson Cancer Ctr	2.0%	61.5%
16	Rochester, MN	Mayo Clinic Cancer Center	1.9%	63.3%
17	Baltimore, MD	Johns Hopkins / Sidney Kimmel Comprehensive Cancer Ctr	7.5%	64.5%
18	Stanford, CA	Stanford Cancer Institute	3.8%	64.5%
19	San Diego, CA	UC San Diego Moores Cancer Center	5.9%	64.6%
20	Duarte, CA	City of Hope Comprehensive Cancer Center	6.3%	64.6%

Note: *>80% of HNC is in population >55 years old; Remaining hospitals in the top 25 are NewYork-Presbyterian / Weill Cornell & Columbia (10.8%), Mount Sinai Hospital – Head and Neck Institute (10.8%), Dana-Farber / Brigham and Women's Cancer Center (4.7%), NYU Langone Perlmutter Cancer Center (10.8%), University of Chicago Medicine (5.4%), however, because catchment areas have significant overlap, they do not add significant population to the cumulative number of patients reached

Source: U.S. Census; Hospital websites

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AAV-AQP1: for treatment of xerostomia

- Granted Breakthrough Therapy Designation, RMAT and Orphan Drug designations
- Alignment with FDA on pivotal clinical trial design and CMC for BLA filing

AQUAx: Phase 1 Clinical Study Design

- **Open-label, multi-center, dose-escalation study (4 sites, US/Canada)**
- **One-time administration of AAV-AQP1 to one (unilateral) or both (bilateral) parotid glands**
- **Four dose-escalating cohorts with 3 participants per cohort (n=12 for unilaterally treated and n=12 for bilaterally treated)**
- **All participants are followed for 1-year post-treatment and then invited to enroll in a long-term follow-up study for a total of 5 years**

Primary endpoint

- Safety

Secondary endpoint

- Patient reported measures of xerostomia symptoms
  - Xerostomia Questionnaire (XQ)
  - MD Anderson Symptom Inventory – Head and Neck
  - Global Rate of Change Questionnaire (GRCQ)
- Unstimulated whole saliva flow rate

Cohort	Dose
Unilateral treatment	
1	1×10^{11} vg/gland
2	3×10^{11} vg/gland
3	1×10^{12} vg/gland
4	3×10^{12} vg/gland
Bilateral treatment	
1b	3×10^{10} vg/gland
2b	1×10^{11} vg/gland
3b	3×10^{11} vg/gland
4b	1×10^{12} vg/gland

Improvements in Xerostomia Questionnaire (XQ) were maintained out to 3 years, demonstrating significant durability of AAV-AQP1

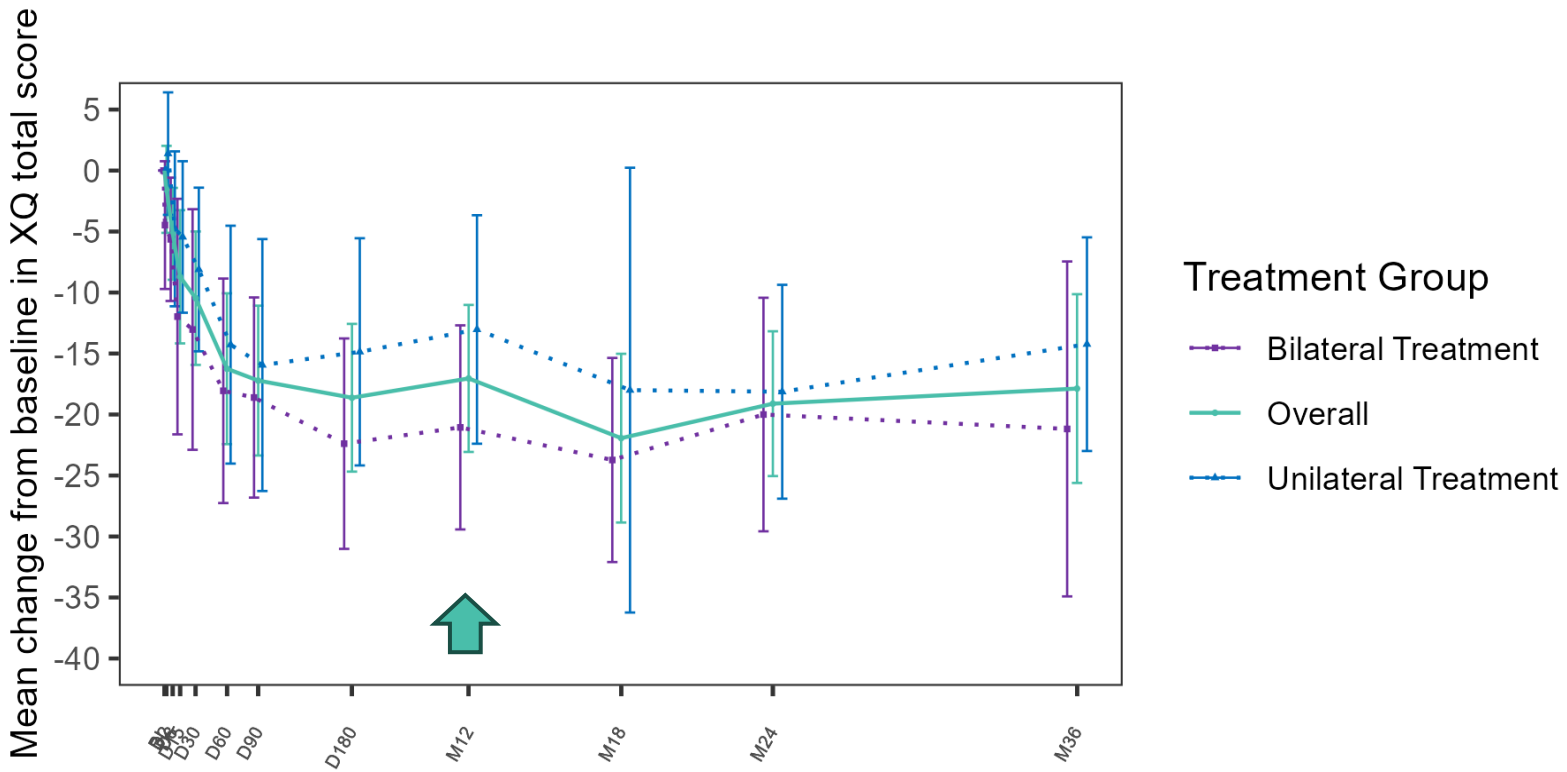
- 8 symptom-specific xerostomia PRO answered by patients with a total **maximum score of 80 points** (higher is worse)
- An improvement (decrease) of **≥8 points** is considered clinically meaningful
- An improvement of **≥10 points** is considered transformative

Transformative improvement: Average XQ score improved by **17 points** (39.5%) at Month 12

Bilaterally-treated participants reported greater improvement than those treated unilaterally, 21 points vs 13 points, with **75% of bilaterally-treated patients reporting transformative (≥10 point) improvement at Month12**

Responses were durable up to 3 years (latest visit)

Average change in XQ score from baseline



Visit	Baseline	Day 2	Day 8	Day 15	Day 30	Day 60	Day 90	Day 180	Month 12	Month 18	Month 24	Month 36
N_Overall	24	24	23	24	23	23	23	24	24	16	21	21
N_Bilateral	12	12	11	12	11	12	11	12	12	11	11	11
N_Unilateral	12	12	12	12	12	11	12	12	12	5	10	10

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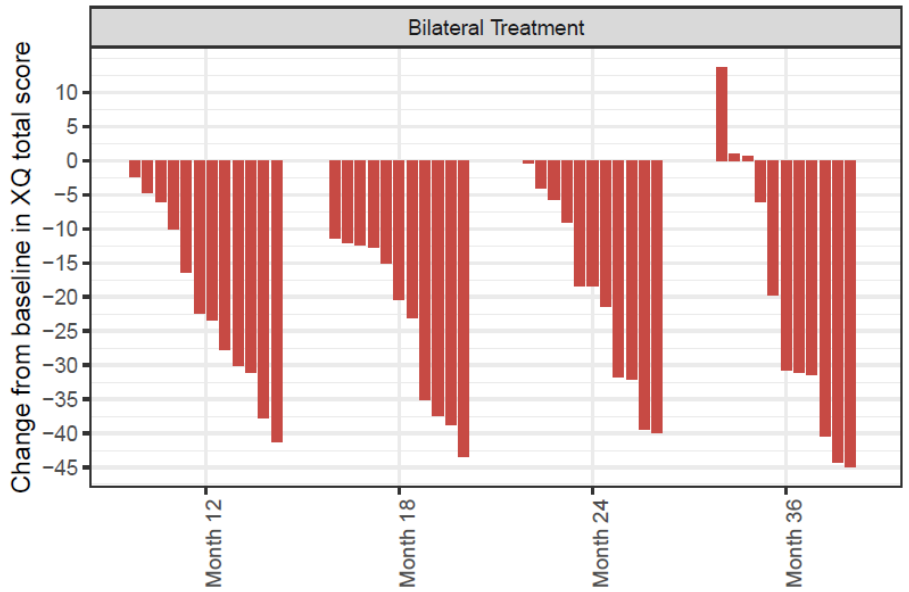
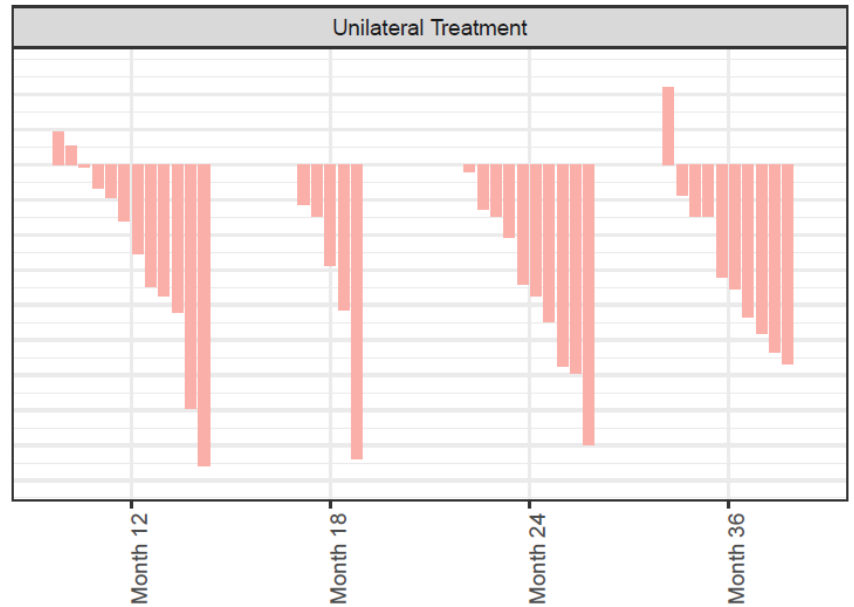
Individual patient data demonstrate durable clinical response over 3 years

Bilateral cohort: Change from baseline (XQ)



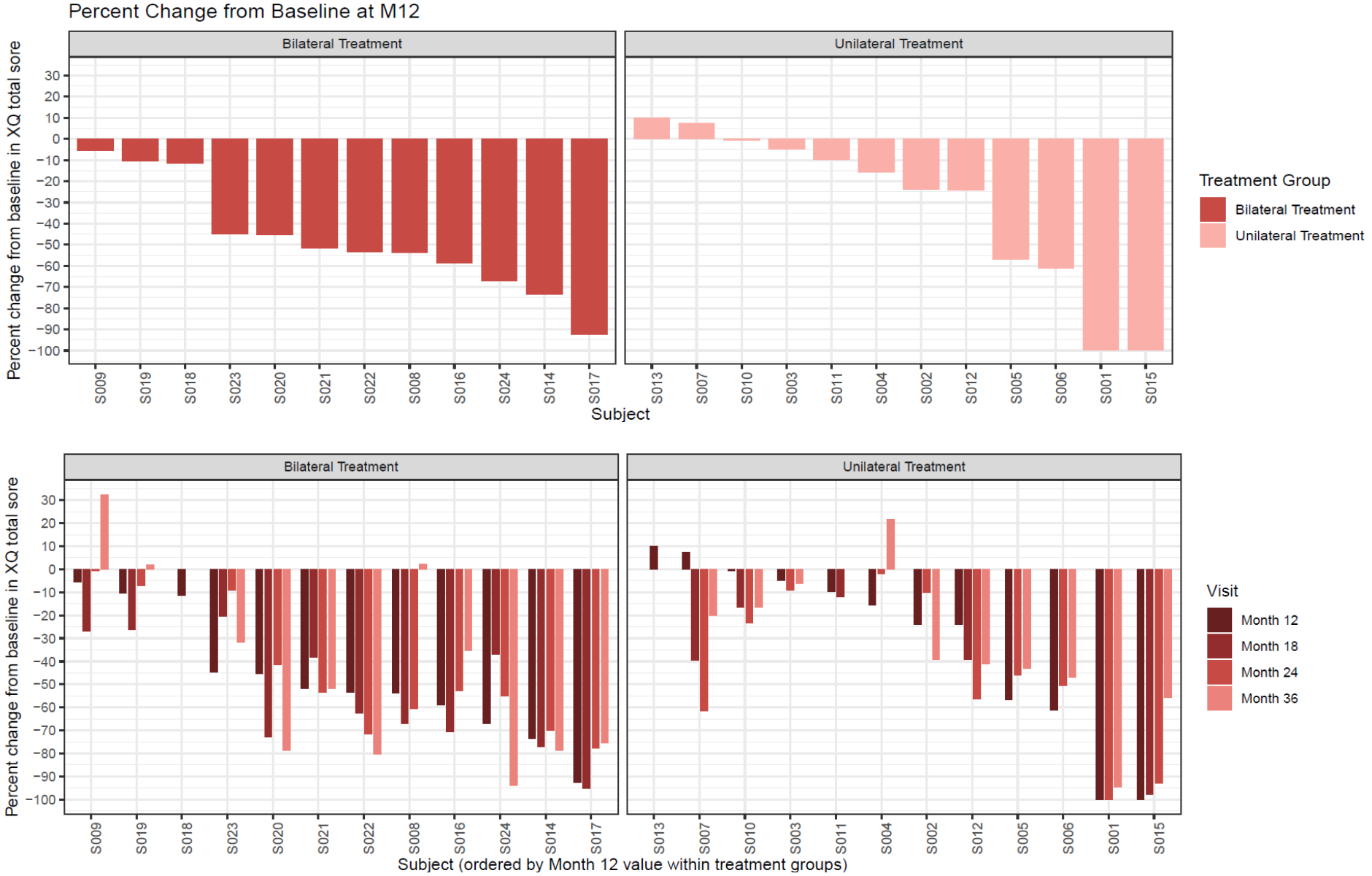
Unilateral cohort: Change from baseline (XQ)



Robust Clinical Response: almost all patients experienced a significant improvement in xerostomia symptoms, with some patients reporting complete **resolution of their xerostomia symptoms**

Sustained Long-Term Durability: most subjects maintained or **further improved** their response over the three-year follow-up period

Waterfall Plot of individual subject XQ score at each visit over 36 months showing the consistency of individual patient responses



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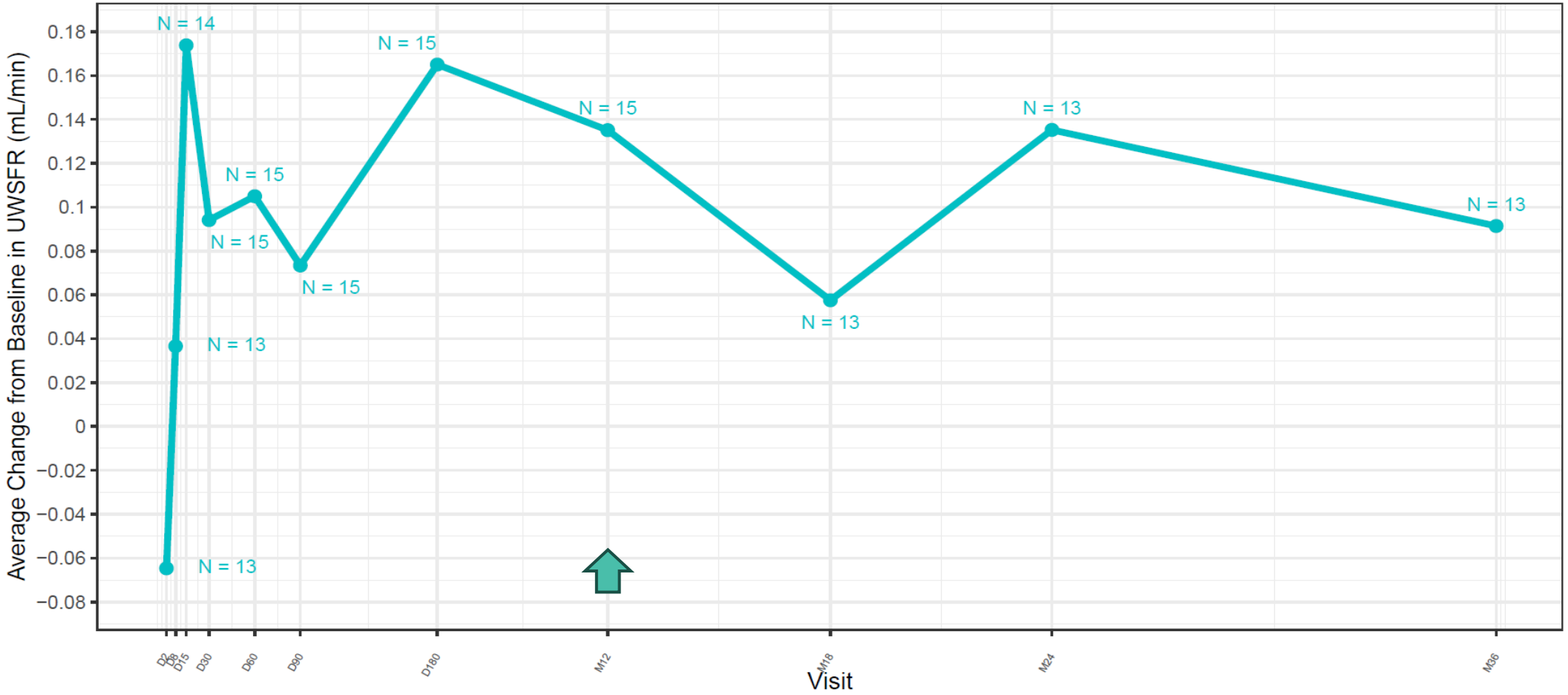
Unstimulated Whole Saliva Flow Rate (UWSFR) : Average Change from Baseline out to Month 36 in all treated patients

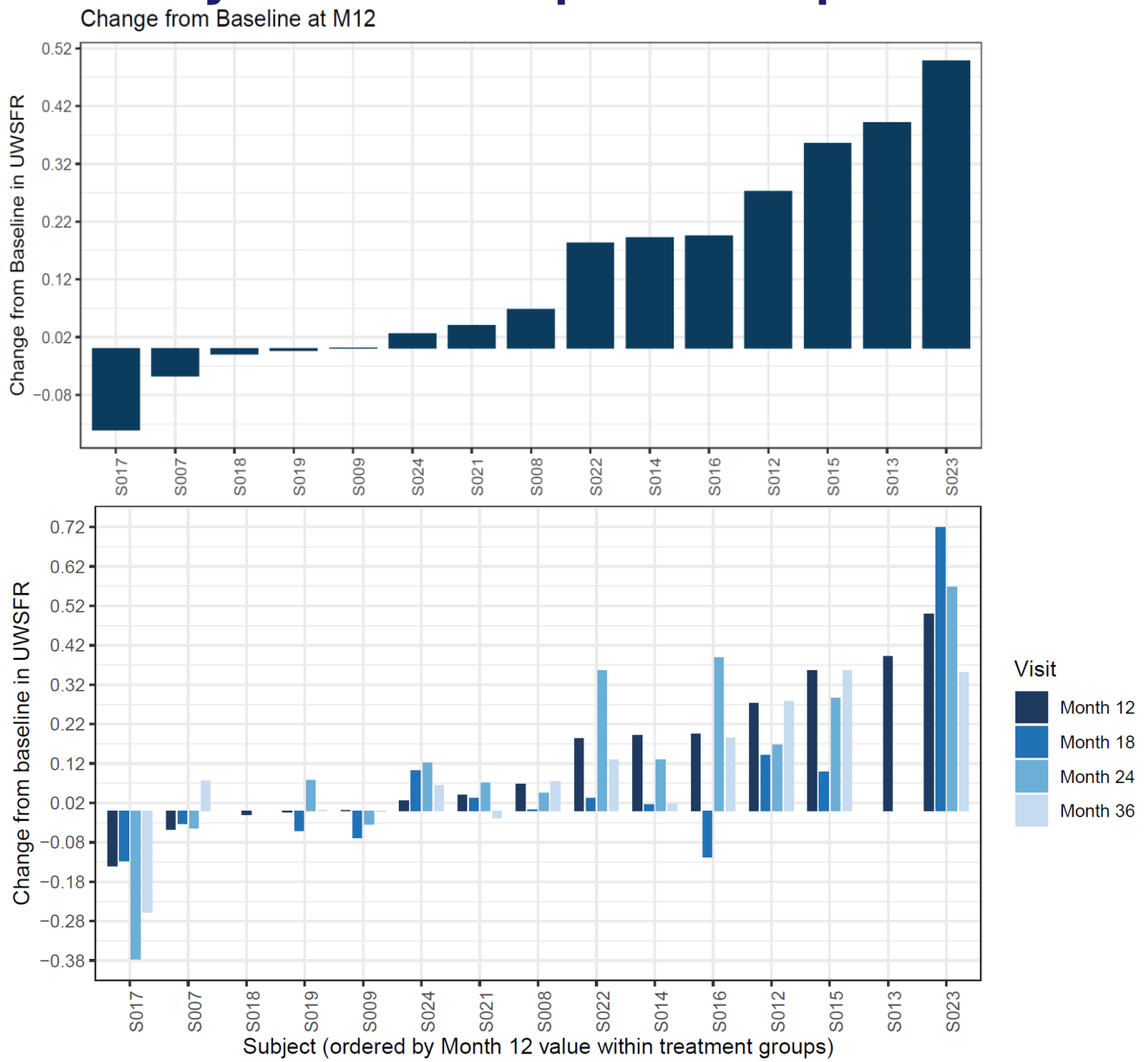


Visit	Baseline	Day 2	Day 8	Day 15	Day 30	Day 60	Day 90	Day 180	Month 12	Month 18	Month 24	Month 36
N_Overall	15	13	13	14	15	15	15	15	15	13	13	13
N_Bilateral	11	11	10	10	11	11	11	11	11	10	10	10
N_Unilateral	4	2	3	4	4	4	4	4	4	3	3	3

Waterfall Plot of each individual subject UWSFR at each visit out to 36 months with subjects showing the overall consistency of individual patient responses

- **Individual Patient UWSFR score at each visit out to 36 months:** the graphs shows the UWSFR for each patient r at 12,18, 24 and 36 months and illustrates the consistency in response for each patient over 3 years.

- Those patients with the the strongest response at 12 months tended to maintain the strongest responses over 36 months, and those with the worst response at 12 months tended to have the worst response throughout the study to 36 months.

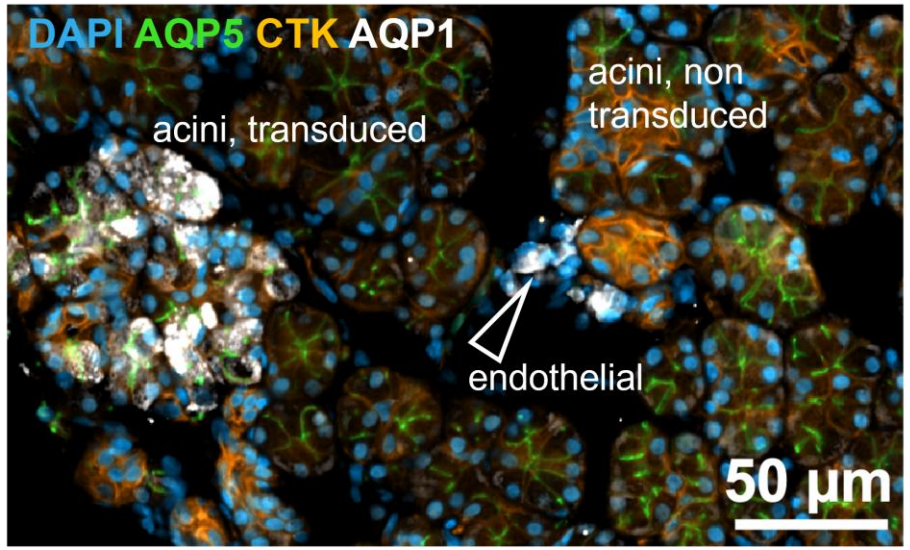


Biopsies indicate that AAV2-hAQP1 persists in the salivary gland

- Core needle biopsies were obtained in 7 participants who enrolled in a NIH Phase 1 study of AAV2-hAQP1 (MGT001).
- **6/7 biopsies showed AAV2-hAQP1 genomes 12-30 months post-treatment**
- There was a trend of increasing copy number of vector genomes with increasing viral vector dose

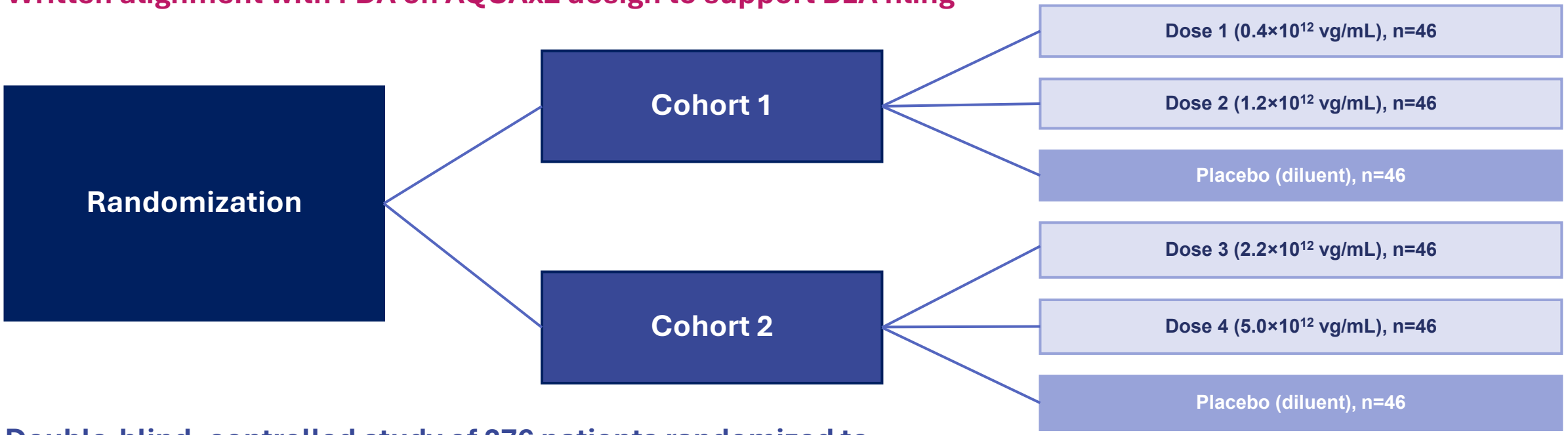
Participant	Cohort	Dose per gland	Visit of Biopsy	Copy #/ng DNA	Copy #/Cell
AAV001	1	1E10	18 Months	160	0.96
AAV005	1	1E10	24 Months	122	0.73
AAV002	2	3E10	18 Months	236	1.4
AAV019	3	1E11	24 Months	5393	32
AAV020	4	3E11	30 Months	ND	ND
AAV021	4	3E11	12 Months	87390	524
AAV031	5	6E11	12 Months	7313	43

- The image on the right shows a core needle biopsy from a participant in the NIH Phase 1 study
- **AQP1 protein expression was observed in parotid gland cells at 24 months post-treatment**
- Acinar cells in this section express AQP1 (shown in white), whereas they normally express only AQP5 – here shown in green
- Levels of AQP1 protein in transduced acinar cells appear similar to the endogenous levels seen in non-parotid endothelial cells



Pivotal MGT-AQP1-201 Study (AQUAx2)

Written alignment with FDA on AQUAx2 design to support BLA filing



Double-blind, controlled study of 276 patients randomized to one of 4 active doses or placebo

- Primary Endpoint - Change from Baseline to Month 12 in modified Xerostomia-specific Questionnaire Total Score
- Key Secondary Endpoint - Change from Baseline to Month 12 in unstimulated whole saliva flow rate (mL/min)
- Other Secondary Endpoints
  - Change from Baseline to Month 12 in Average Dry Mouth Index
  - The Global Rating of Change Questionnaire Score at Month 12
  - Number of participants with treatment-emergent adverse events and serious adverse events

AAV-AQP1: Program highlights

- **AAV-AQP1 has the potential to become the standard of care for patients with late, grade 2/3 radiation-induced xerostomia**
- **disease-modifying mechanism**
- **meaningful improvements objective and subjective outcome measures**

- **One-time, minimally-invasive, local delivery** of a single, small dose delivered through an outpatient cannulation procedure with which ENTs and dentists trained in oral medicine are familiar with
- **Unprecedented improvements** in PRO (XQ) and Objective endpoints (UWSFR) in Phase 1 treated patients
- Expected to provide **durable long-term benefit** in severely affected patients with no other effective current treatment options
- AAV-AQP1 treatment for grade 2/3 xerostomia is **a large commercial opportunity,** very concentrated
- AAV-AQP1 uses a small dose with low associated COGS
- Granted Orphan Drug, **RMAT and Breakthrough Therapy** designations by FDA
- **Written alignment with FDA** on clinical and CMC and requirements of BLA supportive Phase 2 study
- Pivotal Phase 2 data in Q2 2027, BLA filing and approval y/e 2027, targeting early 2028 launch in the US
- **Data from the long-term follow up study for all cohorts shows durable and consistent intra-patient responses in both PROs and saliva production out to 36 months**

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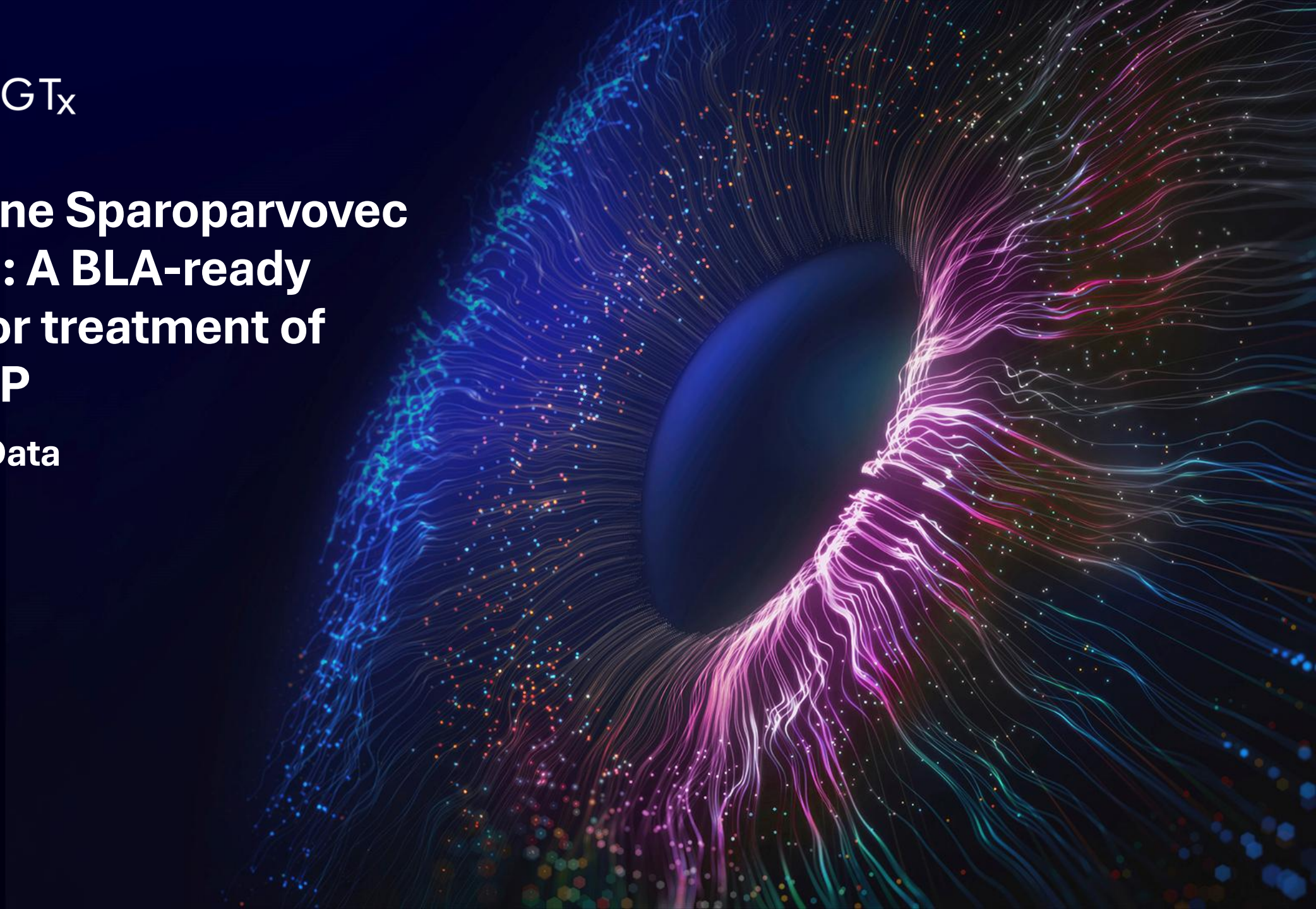
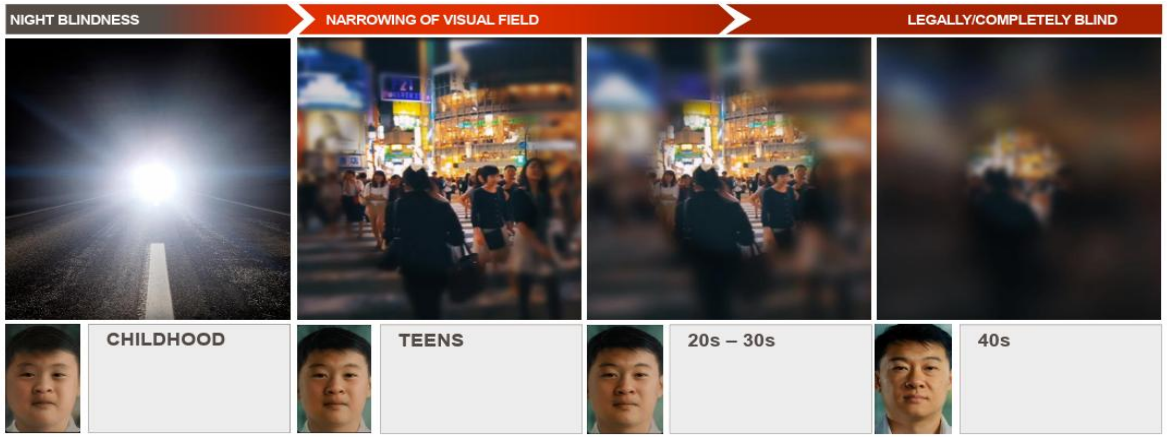
Botaretigene Sparoparvovec (bota-vec): A BLA-ready Program for treatment of RPGR XLRP

Summary of Data



XLRP: Severe, Progressive Vision Loss with High Unmet Need

X-linked retinitis pigmentosa (XLRP) is one of the most severe forms of RP, characterized by early onset night blindness and progressive constriction of the visual field



>20,000 patients in US and EU

XLRP represents a significant portion of the broader Retinitis Pigmentosa population being the cause of **up to 20% of all RP**

Inexorably progressive to blindness

Loss of night vision in pre-teen children
Progressive retinal degeneration reaching complete blindness in the 4th decade of life

Halting vision loss is the #1 priority for patients

High Unmet Need

There are currently NO approved treatments for XLRP, creating a significant urgency for disease-modifying therapies.

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Bota-vec status: awaiting BLA and MAA filings

Largest global development program in Inherited Retinal Disease (IRD) completed with statistically significant and clinically meaningful benefits across every domain of vision :

- **Phase 3 complete (n= 95)** conducted at 32 of the leading global sites for IRD sites in N. America UK EU and Japan
  - Novel primary endpoint (VMA, Maze) while not achieving statistical significance showed a positive trend with **treated patients 2.4x more likely to respond** than untreated
  - **Statistically significant and clinically meaningful improvements** demonstrated in secondary endpoints in **all domains of vision**
  - **Significant clinically meaningful responder analysis**
  - **Significant PRO data** – particularly mobility in low light
  - **Strong safety profile -** expected and manageable across both Phase 1 and Phase 3 studies **(n= 137)**
- **Japan** four Phase 3 patients treated in Japan with 100% response rate, PI reported an extraordinary 40-letter gain in one patient LLVA
- **Phase1/2 study (n=42)** supportive of safety as well as efficacy

Commercial Manufacturing Status:

- **PPQ completed:** MeiraGTx is the commercial manufacturer; several hundred vials are available for commercial release.
- **Commercial CMC/QC licenses in hand:** MeiraGTx granted commercial licenses for vector manufacturing and QC facilities.

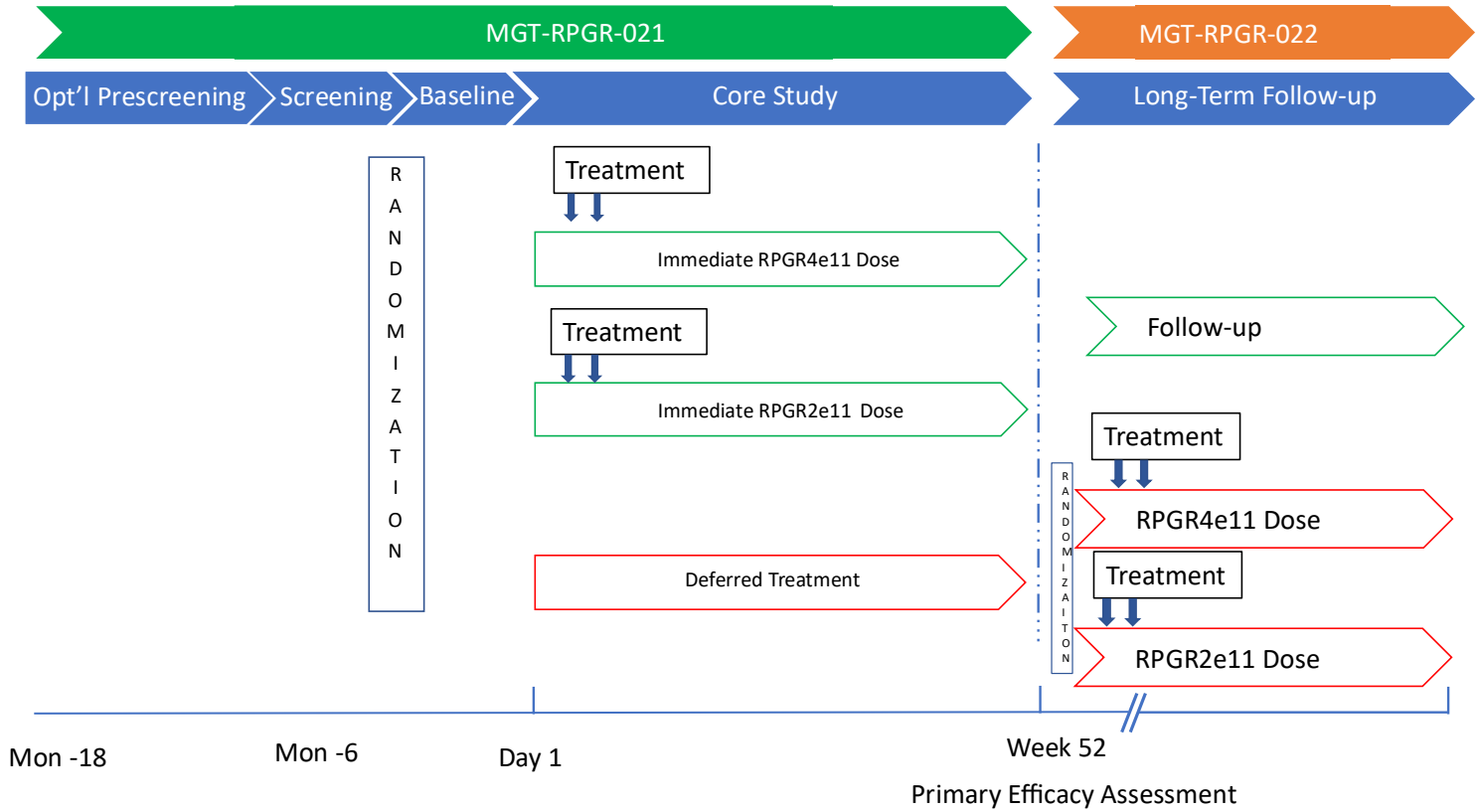
Regulatory Status:

- **EMA:** MAA filing continues to be requested by EMA to be reviewed on totality of data
- **FDA:** Discussions about path to approval based on the current dataset

Phase 3 Randomized, Controlled Study of Bota-Vec Followed by Long-Term Follow-Up

Phase 3 MGT021

- **95 participants** were randomized to **low dose, high dose or deferred treatment** with bota-vec
- **Primary objective:** to assess the effect of bilateral treatment with bota-vec on functional vision as measured by VMA*
- **Secondary objectives:** to assess changes after treatment administration in retinal function, functional vision, visual function, assess the safety and tolerability
- **Read-out at Week 52**



*VMA is a novel assessment first used in this study.
bota-vec, botaretigene sparoparvovec; RPGR, retinis pigmentosa GTPase regulator; VMA, vision-guided mobility assessment.

Bota-vec Phase 3 Pivotal Study Results

Consistent and Robust Efficacy Across Objective & Subjective Measures
These Endpoints Reflect Significant and Clinically Meaningful Improvement in Every Domain of Vision

Category	Functional Vision		Retinal Function			Visual Function	Safety
Assessment	VMA* (binocular) PRIMARY	PRO: LLQ Extreme lighting domain score	Static perimetry responder (central 30 degrees)	Static perimetry responder (full field)	Retinal sensitivity (central 10 degrees)	Low luminance visual acuity (LLVA, ETDRS letters)	Intraocular inflammation
Results	↑ †	↑ ‡	↑ ‡	↑ ‡	↑ ‡	↑ ‡	Expected, acceptable, and manageable
Treatment difference (±95% CI)	13.4% (-3.6%, 30.5%) *P value = 0.247*	7.29 (2.20, 12.38) *P value = 0.006*	38.1% (21.4%, 54.7%) *P value = 0.001*	36.6% (18.2%, 54.9%) *P value = 0.001*	1.24 dB (0.54, 1.94) *P value = 0.001*	4.75 ETDRS letters (1.64, 7.86) *P value = 0.003*	

*VMA is a novel assessment first used in this study. †Results trending in anticipated direction.‡Treated - Concurrent Control Difference, adjusted for baseline ± 95% CI.
Full analysis set population (observed data). Includes participants randomized to both doses.
BCVA, best-corrected visual acuity; bota-vec, botaretigene sparoparvovec; ETDRS, Early Treatment Diabetic Retinopathy Study; LLQ, low luminance questionnaire; LLVA, low luminance visual acuity; PRO, patient-reported outcomes; VMA, vision-guided mobility assessment.

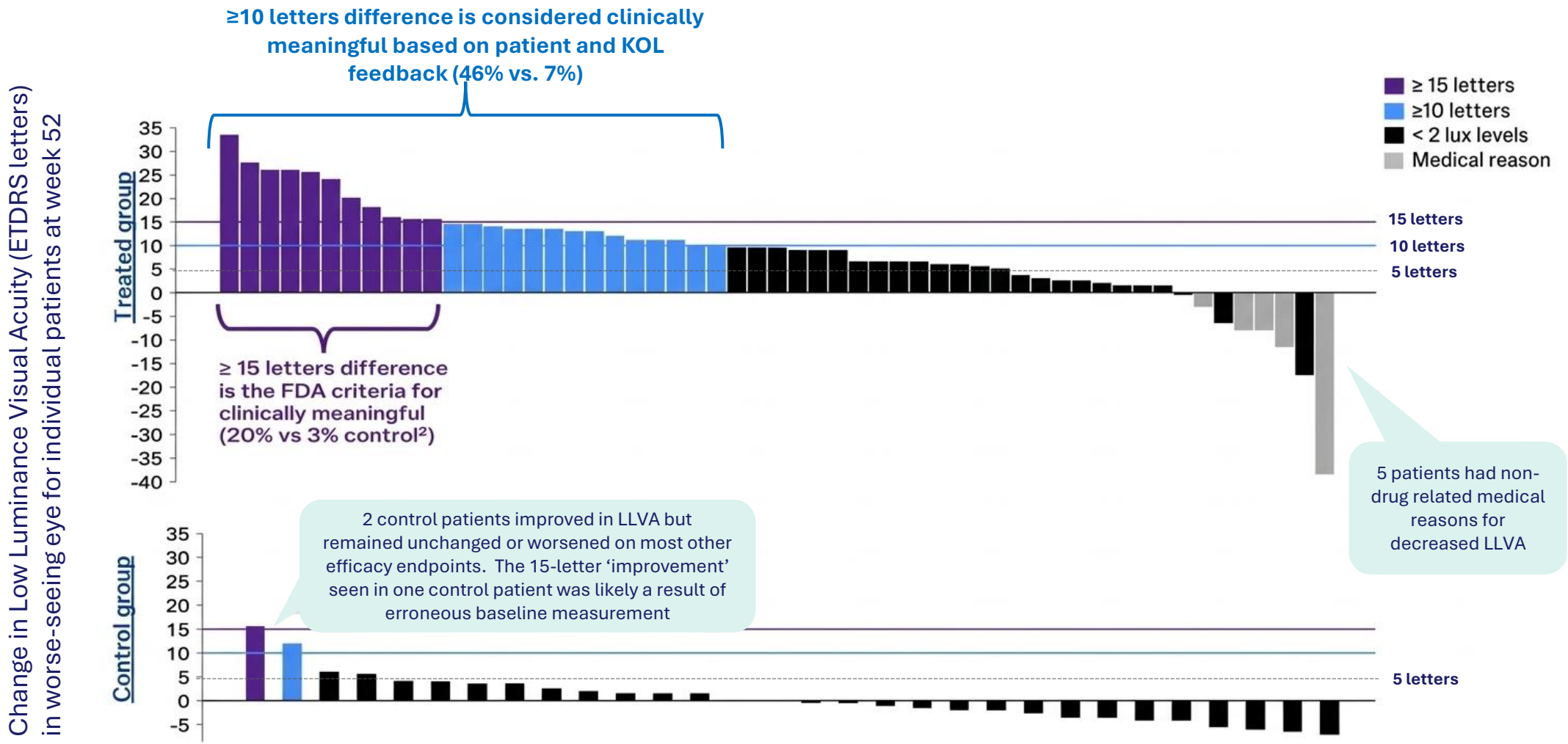
Bota-vec Phase 3 Pivotal Study Results: Highly statistically significant and clinically meaningful benefit in multiple secondary endpoints every domain of vision

	Pooled treated (n = 55)	Control group (n = 30)	Between-group difference (pooled treated group minus control) (95% CI)	P-value
Primary Endpoint				
VMA responders[1] (binocular), %	27.3%	13.3%	13.4% (-3.6%, 30.5%)	0.247†
Secondary Endpoints				
Change in MRS10[4], LS mean (SE)	0.88 (0.22)	-0.36 (0.29)	1.24 (0.54; 1.94)	**0.001**
Pointwise responders in full visual field[2], %	58.2%	23.3%	36.6% (18.2%, 54.9%)	**0.001**
Pointwise responders in the central 30 degrees[2], %	47.3%	10.0%	38.1% (21.4%, 54.7%)	**0.001**
Change in mLLQ Extreme lighting domain score, LS mean (SE)	1.80 (1.50)	-5.49 (2.05)	7.29 (2.20; 12.38)	**0.006**
Change in LLVA[4], LS mean (SE)	6.86 (0.94)	2.11 (1.26)	4.75 (1.64; 7.86)	**0.003**
Change in MRS90[4], LS mean (SE)	-0.00 (0.19)	-0.93 (0.26)	0.92 (0.29; 1.55)	**0.004**

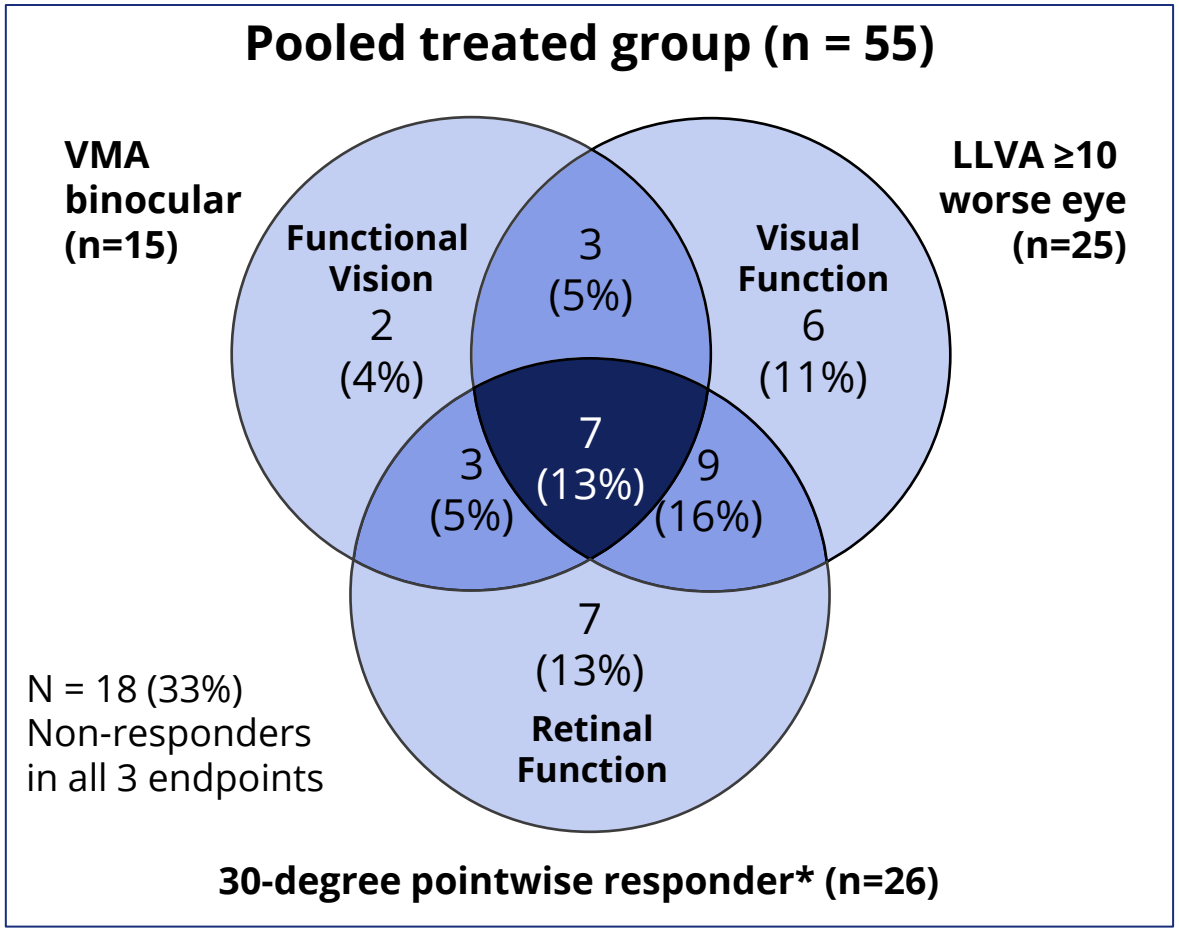
Significant improvements in Patient Reported Outcomes (PRO) questions related to everyday function and quality of life:

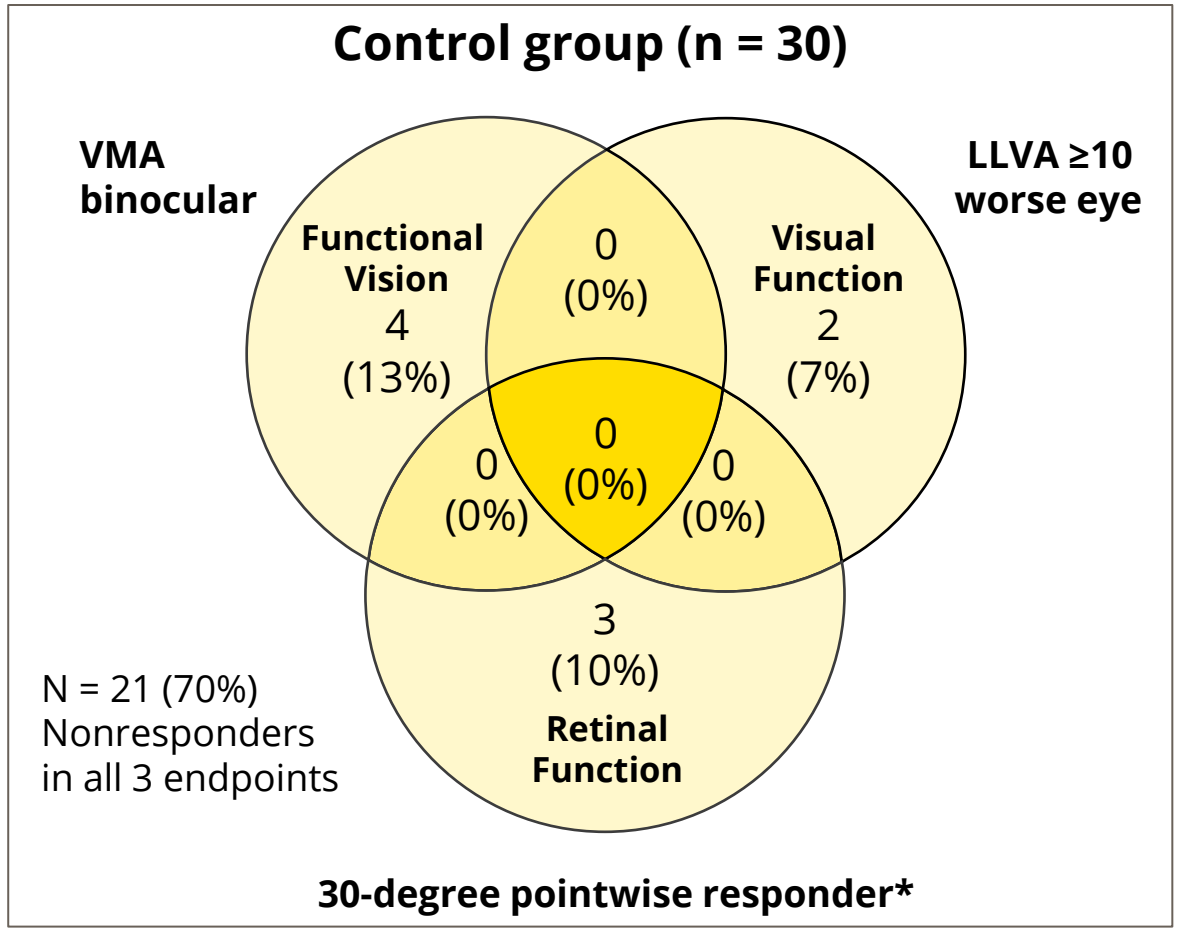
- **IVI-A:** significant improvement in total score vs. control at week 52 (**p=0.024)** with greater significance in the emotional well being questions (**p=0.005**)
- **Modified Low Luminance Questionnaire MLLQ** showed statistically significant improvement (**p=0.006)** with improvements in questions relating to mobility (**p= 0.001**), general dim lighting (**p= 0.007**) and emotional distress (**p= 0.019**) being highly significant
- **Notable concordance** between PRO interviews and other clinical data

Impressive results for Visual Acuity measured in low light levels (LLVA) with 46% of treated patients achieving ≥10 letter gain on ETDRS chart compared to 7% for untreated controls



40% (22/55) of Treated Patients Show Improvement in ≥2 Endpoints Compared to 0% in Controls





*Pointwise responder based on both eyes modeled analysis
LLVA, low luminance visual acuity; n, number; VMA, vision-guided mobility assessment.

Multi-Endpoint Responder Analysis: Comprehensive Results

FF = Full Field; Pt = Pointwise; binoc = binocular.

Consistent 25–40% treatment benefit across ALL endpoint combinations with most combinations showing 0% response in control arm

SECTION 1: VMA + LLVA COMBINATIONS

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
VMA binoc. + LLVA binoc. (≥10)	40.0%	0%	+40.0%
VMA binoc. + LLVA worse eye (≥10)	27.3%	0%	+27.3%
VMA binoc. + LLVA binoc. (≥15)	29.1%	0%	+29.1%
VMA binoc. + LLVA worse eye (≥15)	25.5%	0%	+25.5%

SECTION 2: VMA + STATIC PERIMETRY

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
VMA binoc. + Pointwise 30° binoc.	32.7%	3.3%	+29.4%
VMA binoc. + Pointwise 30° worse eye	25.5%	0%	+25.5%
VMA worse eye + Pointwise FF binoc.	30.9%	0%	+30.9%
VMA worse eye + Pointwise FF worse eye	29.1%	0%	+29.1%

SECTION 3: LLVA + STATIC PERIMETRY

ENDPOINT COMBINATION	TREATED	CONTROL	DIFF
LLVA worse eye (≥10) + Pt 30° binoc.	43.6%	3.3%	+40.3%
LLVA binoc. (≥10) + Pt 30° binoc.	36.4%	6.7%	+29.7%
LLVA worse eye (≥10) + Pt FF binoc.	41.8%	3.3%	+38.5%
LLVA binoc. (≥15) + Pt 30° worse eye	29.1%	0%	+29.1%

Bota-vec commercial opportunity:

Launch into concentrated, educated KOL market with commercial supply manufactured in-house at MeiraGTx

Projected cost-effective launch into a concentrated group of educated physicians and patients globally

- **>20,000 patients EU and US**
- **small community of connected KOLs**
- **~ 80% of IRD patients** under the care of ~ **40-50 sites US, EU, and Japan**
- **32 of these sites who participated in the Bota-vec Phase 3 study,**
- **> 60 surgeons were trained in bota-vec** delivery for the Phase 3

- **KOLs and patient advocates globally are lobbying for approval**
- **Several hundred patients already identified**
- **Commercial material available for release:** PPQ successful with several hundred vials at MeiraGTx for potential commercial launch
- **MeiraGTx is the commercial manufacturer** with end-to-end **control of drug supply and guaranteed capacity**

Conclusions

- Large Phase 3 study, 32 leading global sites (n=95), trend in novel primary, strong secondary endpoints in every domain of vision.
- Good safety profile
- Supported by Phase 1/2 dose escalation and expansion study (n=43) for safety and efficacy.
- Key Leadership from MeiraGTx and JNJ engaged in global approval
- Strong global KOL and patient advocacy support for approval globally
- EMA: Continue to request MAA filing to be reviewed based on totality of data
- FDA: Discussions on potential for approval based on current data
- PDMA: Strong support for filing in Japan

"The vision improvements in LUMEOS have been life changing for many patients. Both objective measures and subjective reports from patients demonstrate clear and meaningful efficacy," says Todd Durham, PhD, senior vice president of clinical outcomes and research at the Foundation Fighting Blindness. "The Foundation and trial investigators feel it is incumbent upon J&J to urgently pursue regulatory approval to make the vision-restoring treatment available to the global XLRP community." FFB letter to JNJ

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bota-vec, botaretigene sparoparvovec; RPGR, retinis pigmentosa GTPase regulator; VMA, vision-guided mobility assessment.

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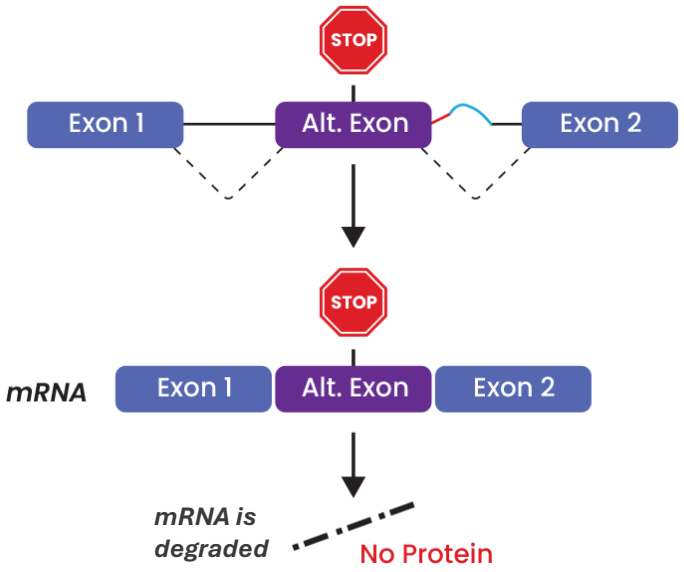
Riboswitch Gene Regulation Platform

In vivo production of vectorized therapeutic proteins and peptides with oral inducers

Riboswitch platform: precise *in vivo* production of therapeutic proteins via oral small molecule inducers

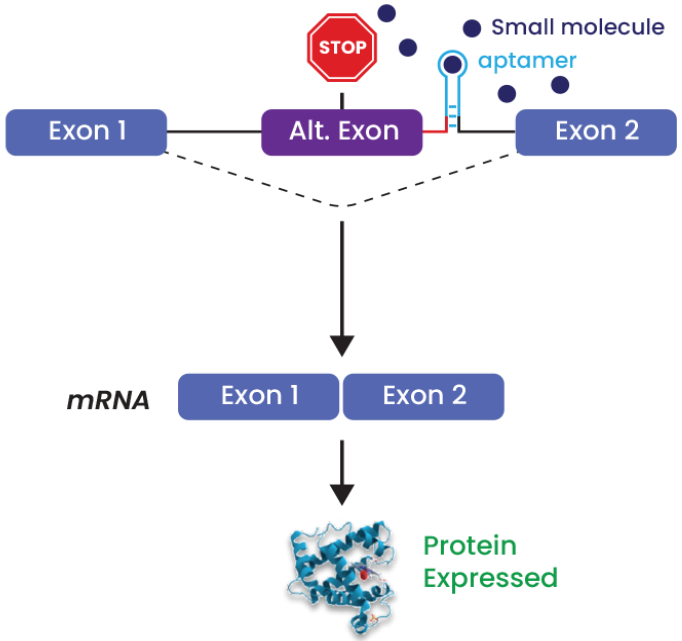
mRNA formation is controlled by alternative splicing cassette via binding of a small molecule inducer to the Riboswitch cassette

No small molecule (OFF state)



In the absence of the small molecule inducer ("OFF state"), the incorporation of an alternative exon with a premature stop codon drives degradation of the mRNA transcript via the ubiquitous process of nonsense-mediated decay (NMD)

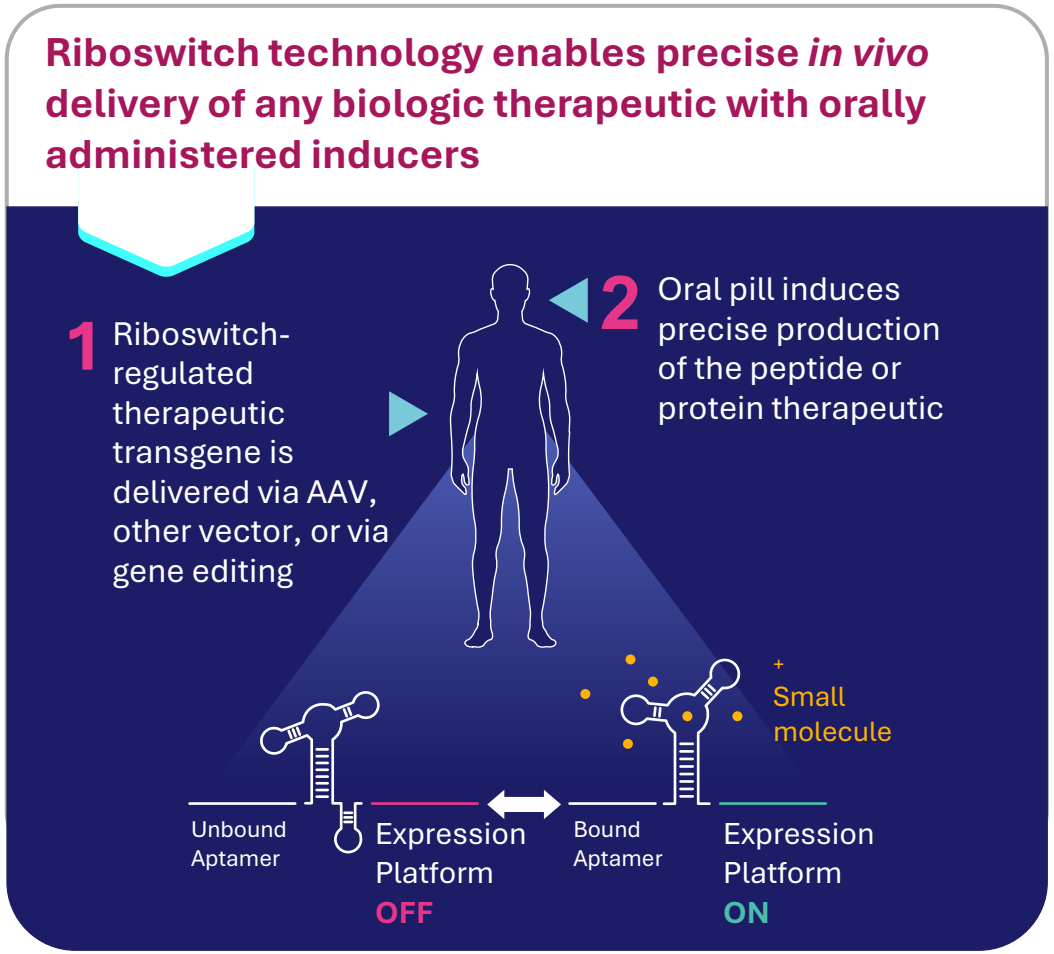
With small molecule (ON state)



Binding of the small molecule inducer to the Riboswitch aptamer drives stabilization of a hairpin structure which incorporates the 5' splice site of the second intron, triggering splicing out of the alternative exon and production of a stable, protein-encoding transcript

Riboswitch platform: precise *in vivo* production of therapeutic proteins using oral small molecule inducers

Riboswitch technology enables precise *in vivo* delivery of any biologic therapeutic with orally administered inducers



Riboswitch technology can be applied across many therapeutic areas and modalities, providing titratable control of transgene expression with an oral pill

Optimized Vectorized Antibody Drugs

Cell Therapy
- Autoimmune
- Oncology

Gene Editing
- DNA nucleases
- CasRX

Hormones & peptides
- CV & Metabolism
- Inflammation
- Longevity

Controlled delivery CNS & PNS
- Pain
- BBB penetrant inducers

Precise Control of Ocular Therapies

A broad range of therapeutic proteins encoded by Riboswitch-containing transgenes show tight control via oral small molecule dosing, *in vivo*

Therapeutic Antibodies

- Anti-PCSK9
- Anti-VEGFR2 (eye)
- Anti-Amyloid
- Anti-IL-17
- Anti-PD1
- Anti-HER2
- Anti-IL4Ra
- Anti-Myostatin

Cell Therapy

RiboCAR:

- Anti-CD19
- Anti-PSMA
- Anti-mesothelin
- Anti-HER2
- Cytokines

- ProTcell (progenitor T cell) derived riboCAR-T

Therapeutic Hormones/Cytokines/ Peptides

- Epo
- hGH
- PTH
- Insulin
- GLP-1R agonists
- Gut peptide combinations: GLP1- GIP; GLP1, GIP, PYY, Glucagon, Amylin, Oxyntomodulin
- Myokines
- Adipokines e.g: leptin

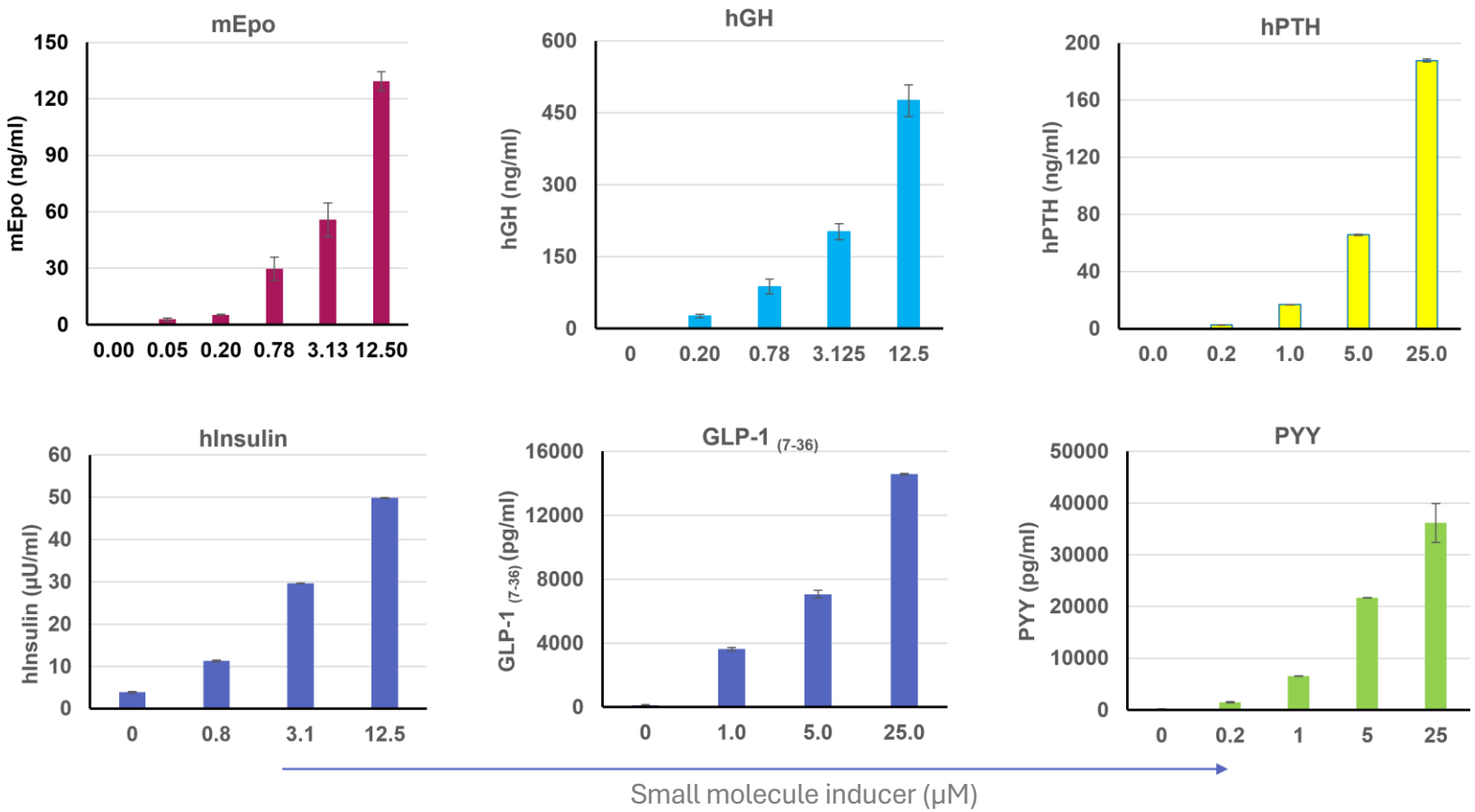
Gene/RNA Editing Nucleases

- Cas9
- CasRx

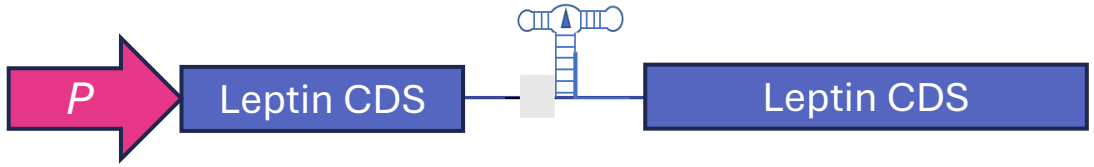
Precise regulation of multiple therapeutic hormones and peptides with riboswitch, *in vitro*

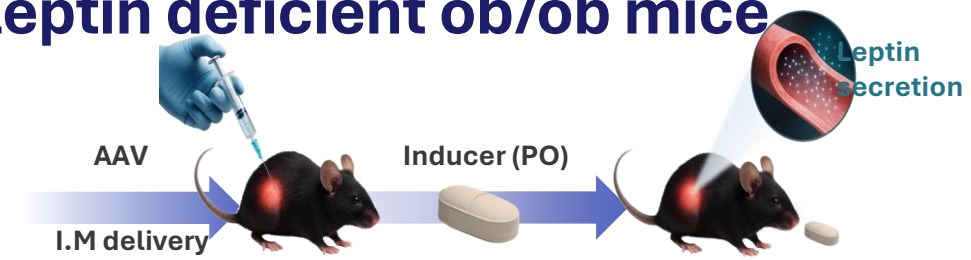
The riboswitch platform can be applied to any transgene and delivered by any vector - achieving *in vivo* production of the therapeutic protein or peptide in a precise dose response to a bespoke orally administered small molecule inducer.

- Graphs to the right show examples of regulation of human hormones in response to dosing with a small molecule riboswitch inducer, *in vitro*
- Many of these targets have been validated in relevant animal models, showing precise control of therapeutic protein serum levels and therapeutic effect driven by the dose of the oral small molecule inducer

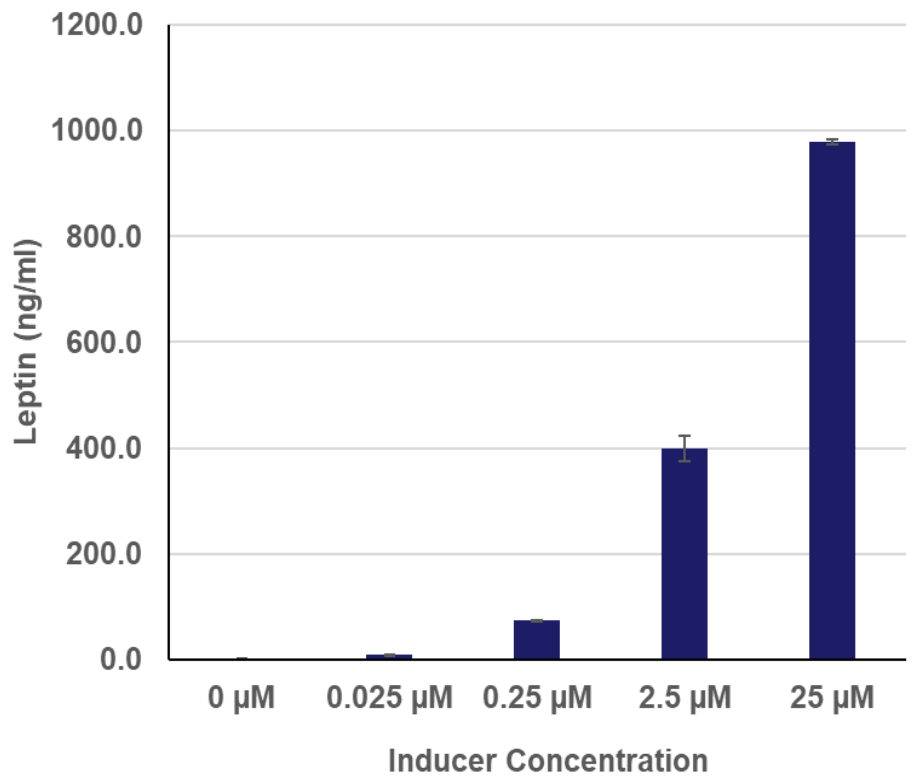


Riboswitch-controlled leptin demonstrates oral inducer dose-dependent expression of leptin in mammalian cells and in leptin deficient ob/ob mice



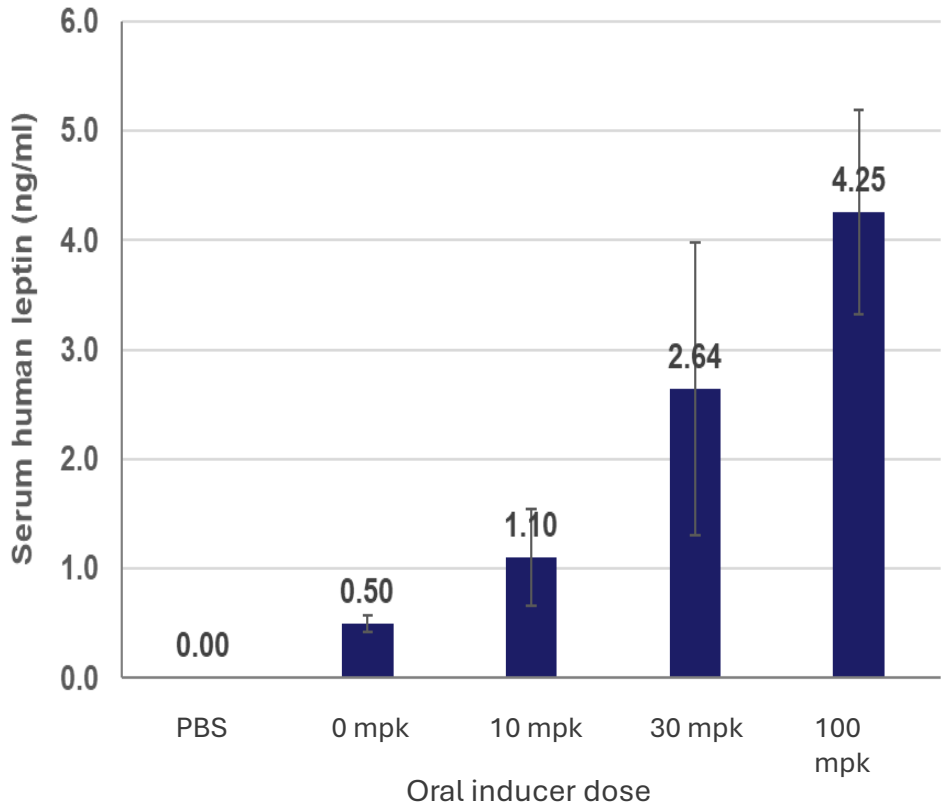


Dose-dependent leptin expression in HEK 293 cells



Leptin expression levels directly corresponded to the dose of the orally administered inducer M310 in vitro and in vivo

Dose-dependent leptin expression in ob/ob mice



Leptin reaches physiological levels in response to 30 mg/kg dose of inducer

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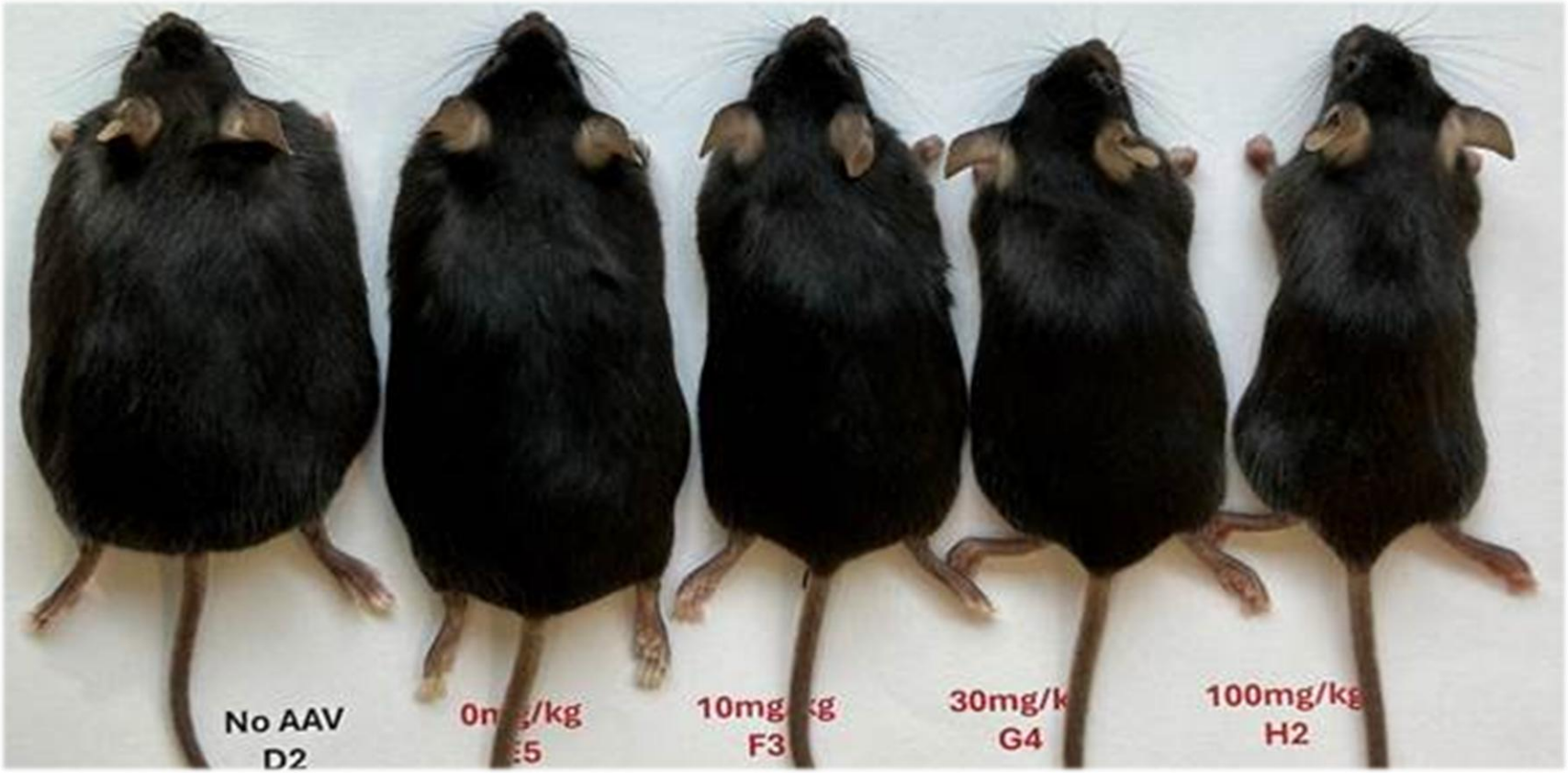
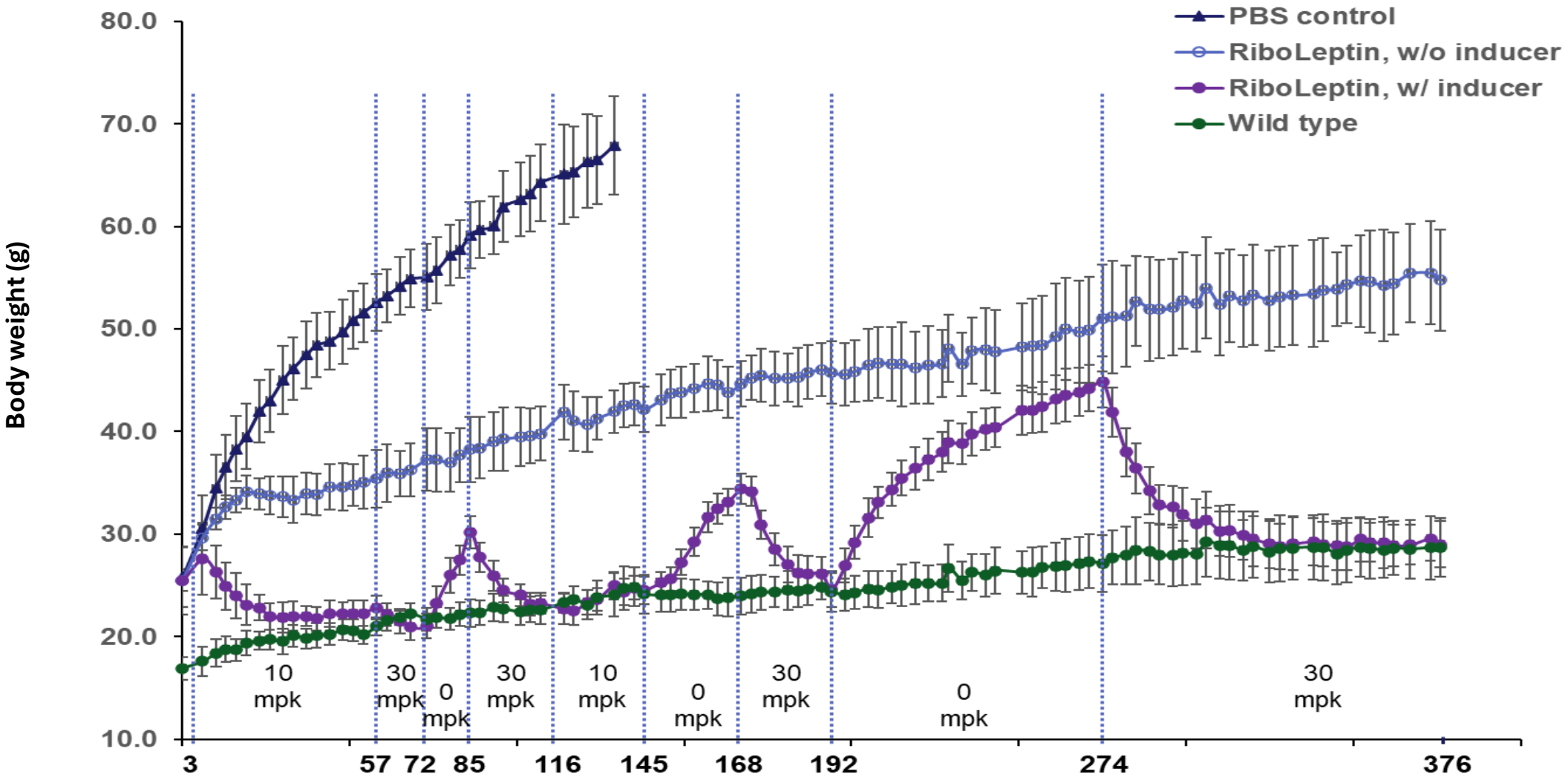
riboLeptin demonstrates dose-dependent weight loss in response to oral inducer dosing, preventing morbid obesity

	I.M.	PBS	AAV9.RS.Leptin			
Oral M310		0 mg/kg	0 mg/kg	10 mg/kg	30 mg/kg	100 mg/kg



Durable riboswitch-controlled production of leptin in ob/ob mice effectively treats leptin deficiency with daily oral small molecule dosing over a year after one-time IM injection of RiboLeptin



Riboswitch-regulated leptin therapy (RiboLeptin) - Summary

RiboLeptin resolved leptin deficiency following oral inducer treatment in ob/ob mice:

- Dose dependent expression of leptin to physiological levels
- Significant reduction in excessive food intake behavior (hyperphagia)
- Significant and durable weight loss - to normal levels
- Complete correction of body fat levels to normal levels
- Complete correction of glucose tolerance and serum glucose levels
- One-time local injection of gene vector followed by daily dose of oral small molecule
- Much decreased cost of goods, increasing the accessibility of leptin replacement, while replacing injection with a daily oral pill, and reducing toxicity and increasing safety

MeiraGTx is leading the next wave of genetic medicines

Advanced and diverse pipeline of genetic medicines

- **4 pivotal stage programs:** Radiation-induced xerostomia, Parkinson's disease,, AIPL1 retinal dystrophy, X-linked retinitis pigmentosa
- **Diverse preclinical pipeline:** ALS, intractable neuropathic pain, obesity & diabetes, large ophthalmology indications such as Stargardt's, wet AMD and dry AMD
- **Multiple potential near-term BLA filings**

Powered by best-in-class genetic medicine technologies and unique industry leading end-to-end in-house GMP manufacturing:

Vector Design & Optimization Technologies

- ☑ Novel intravitreal capsids
- ☑ Proprietary promoters
- ☑ Sequence optimization
- ☑ Proprietary non-coding elements for increased potency

Riboswitch Platform

- ☑ Precise control of transgene expression with orally administered pills
- ☑ New approach to cell therapy, gene editing, metabolic disease

End-to-end GMP Manufacturing

- ☑ In-house plasmid and viral vector GMP manufacturing
- ☑ Commercially-licensed QC facility
- ☑ Fill & Finish
- ☑ Single use – flexible & scalable production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Riboswitch regulation of metabolic peptides, alone or in combination

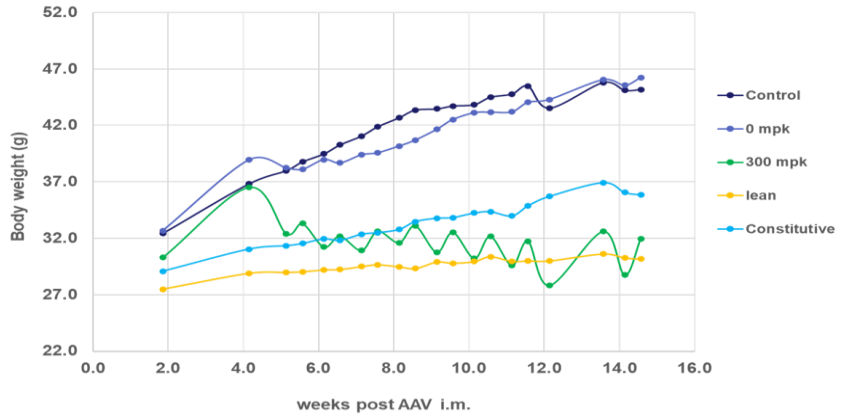
In vivo production of natural gut peptides

- Delivery of high effective levels of active peptides can be challenging. MeiraGTx has achieved high expression of natural gut peptides, alone or in combination produced by the oral dosing of a small molecule activator
- The Riboswitch platform provides tight and controlled expression of unmodified, wild-type peptides
- Delivery of multiple combinations of peptides can be achieved using a single vector

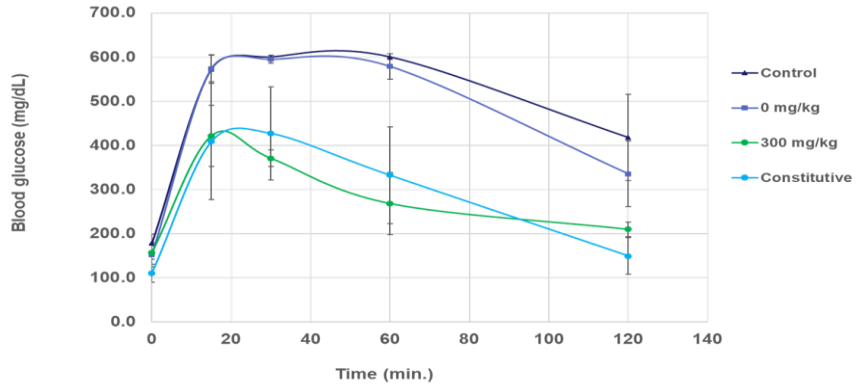
These can be constructed and tested rapidly head-to-head to provide fast *in vivo* proof of concept of efficacy and benefit on **muscle mass, metabolism, and feeding as well as behavior and CNS impact**

In vivo delivery of GLP1-GIP via daily oral inducer dosing significantly improves weight loss and glucose control compared to constitutively expressed GLP1-GIP

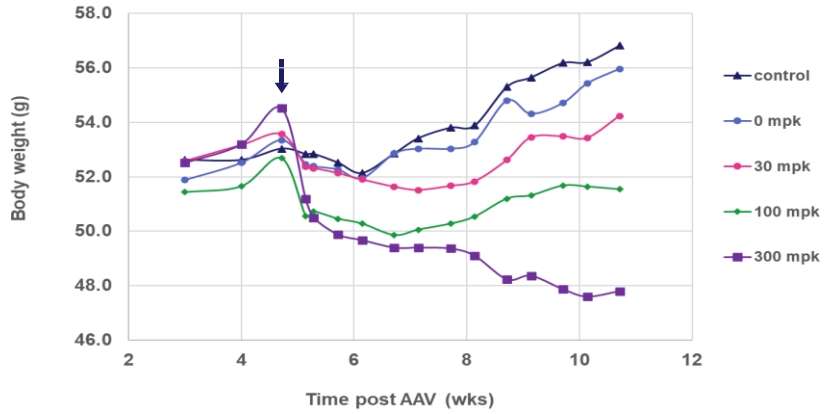
Weight loss in DIO mice

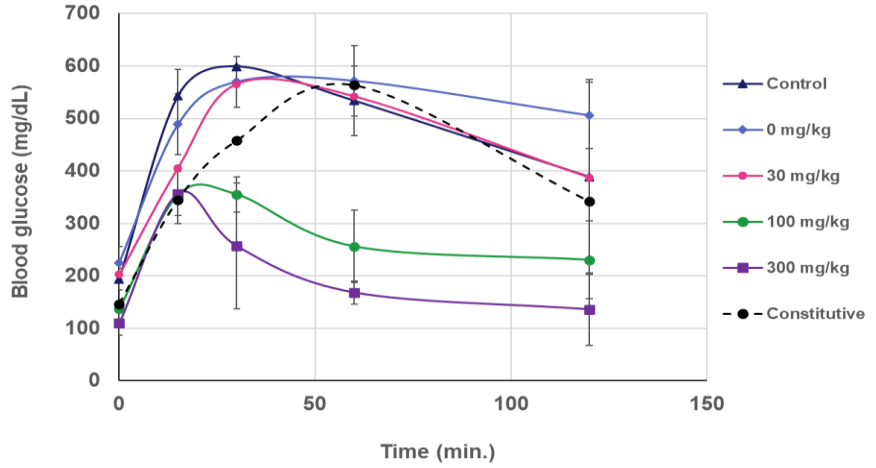


Glucose Control in DIO mice



In vivo delivery of GLP1-GIP-Glucagon via daily oral inducer dosing significantly improves weight loss & glucose control vs. constitutive GLP1-GIP-Glucagon



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