



MEIRAGTx

Gene Therapy for the Treatment of Radiation-Induced Xerostomia: AAV2-hAQP1 Program Update

June 27, 2023

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1. Introduction

Zandy Forbes, PhD
President & CEO MeiraGTx

2. Radiation-Induced Xerostomia: Disease Background and Unmet Medical Need

Robert K. Zeldin, MD
Chief Medical Officer MeiraGTx

3. AQUAx Phase 1 Study Data

Zandy Forbes, PhD
President & CEO MeiraGTx

4. AQUAx 2: Phase 2 Study

Robert K. Zeldin, MD
Chief Medical Officer MeiraGTx

5. Question and Answer Session

Salivary Gland Gene Therapy for Radiation-Induced Xerostomia

Robert K. Zeldin, MD



Serious, debilitating complications as a result of reduced saliva production

- ❖ RIX is one of the most frequent complications of radiation treatment for head and neck cancer
- ❖ 85% of radiation-treated patients experience reduced saliva production, of whom 40% have persistent Grade 2/3 RIX
- ❖ Persistent Grade 2/3 RIX is a common, durable and severely debilitating condition
- ❖ Patients' experience:
 - Difficulty eating, chewing and swallowing; taste alterations
 - Speech difficulties and abnormalities
 - Difficulty sleeping; difficulty exercising
 - Uncontrollable dental caries with severe tooth decay/periodontal disease
 - Inability to wear dentures
 - Oral pain and throat pain
 - Burning mouth sensation in 40% of patients
 - Harmful changes in oral flora



Currently therapies only treat symptoms, with limited efficacy and poor tolerability, leaving a significant unmet need in grade 2/3 RIX

- ❖ Providers generally recommend lifestyle interventions first (e.g., extra water consumption) followed by topical agents (e.g., artificial saliva) for RIX patients. Saliva substitutes such as carboxymethyl cellulose and mucin have short term benefit and are disliked by patients
- ❖ 75% of grade 2 & 3 RIX patients are treated with oral sialogogues - pilocarpine or cevimeline
 - Do not improve salivary gland functionality
 - Not well tolerated
 - Majority of patients experience side effects including flushing, upset stomach, and sweating
 - Contraindicated in a variety of conditions
- ❖ Sialogogues have decreasing efficacy with increasingly severe RIX
- ❖ ~83% of treated grade 2 & 3 patients either experience no response or don't tolerate the drugs
- ❖ AAV2-hAQP1 is the only known treatment in the clinical pipeline

Large indication for a gene therapy: both prevalence and incidence

- ❖ >170,000^{1,2,3} long term (2 years post successful treatment) grade 2/3 RIX patients currently in the US alone
- ❖ 54,000^{1,2} new cases of head and neck cancer per year in the US with >15,000 new long-term grade 2/3 RIX patients
- ❖ No effective treatment for long term grade 2/3 RIX
- ❖ Patients are in the healthcare system in remission for head and neck cancer and seeing physicians at least annually
- ❖ Low dose, low cost of goods, large market for gene therapy = strong commercial opportunity

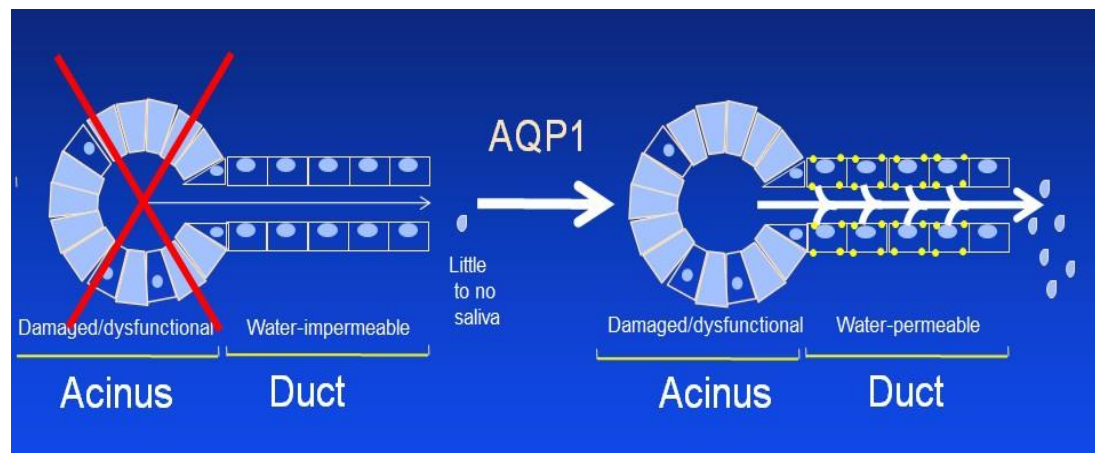
¹ SEER, Cancer.net

² Marta GN et al (2014). Intensity-modulated radiation therapy for head and neck cancer: systematic review and meta-analysis. *Radiother Oncol.* 110(1):9-15

³ Jensen S.B., et al. (2010). A systematic review of salivary gland hypofunction and xerostomia induced by cancer therapies: prevalence, severity and impact on quality of life. *Support Care Cancer.* 18(8):1039-1060

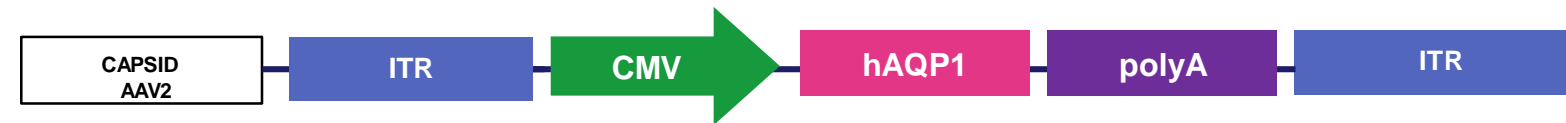
Mechanism of Action:

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



¹Rouleau, Tanya S. et al, A retrospective, cohort study of the prevalence and risk factors of oral burning in patients with dry mouth Oral Surg Oral Med Oral Pathol Oral Radiol Endod 2011;111:720-725

Viral Vector Configuration:



Capsid - AAV2:

- Transduces human primary salivary gland tissue more effectively than any other known capsids
- Local transduction: AAV2 stickiest of capsids. The target is the single layer of duct cells and remaining acinar cells

Promoter – CMV promoter:

- Drives strong, durable expression in salivary glands

Gene – hAQP1:

- Human water channel that provides membranes with high permeability to water, permitting water to move in the direction of an osmotic gradient

Local Non-invasive Delivery:

- Delivered via catheter into the opening of the parotid duct

AQUAx Phase 1 Study Data

Zandy Forbes, PhD



Study Design

- Open label, multi-center, dose escalation study at 4 sites in USA and Canada
- One-time administration of AAV2-hAQP1 to one (unilateral) or both (bilateral) parotid glands
- Four dose escalating cohorts with 3 participants per cohort for both unilaterally-treated and bilaterally-treated participants
- All participants are followed for 1-year post-treatment and then enrolled in long-term follow-up study for a total of 5 years

Cohort	Dose
1	1×10^{11} vg/gland (single gland)
2	3×10^{11} vg/gland (single gland)
3	1×10^{12} vg/gland (single gland)
4	3×10^{12} vg/gland (single gland)

Primary Endpoint

- Safety

Secondary Endpoints

- Patient reported measures of xerostomia symptoms
 - Global Rate of Change Questionnaire (GRCQ)
 - Xerostomia Questionnaire (XQ)
- Whole saliva flow rate

1b	3×10^{10} vg/gland (both glands)
2b	1×10^{11} vg/gland (both glands)
3b	3×10^{11} vg/gland (both glands)
4b	1×10^{12} vg/gland (both glands)

Study Status: COMPLETED

- Four unilateral treated cohorts (n=12)
- Four bilateral treated cohorts (n=12)
- Study completed, database locked
- Subjects continue to be followed for up to 5 years in the long term follow up study

Data Presented Today:

- Data from all unilateral (n=12) and bilateral cohort subjects (n=12) out to 12 months post treatment
- Data from those long term follow up subjects who have reached 2 and 3 years post treatment

Safety:

- AAV2-hAQP1 treatment appears safe and well tolerated at each dose tested
- No dose limiting toxicity or drug related serious adverse events

Activity:

- Improvements observed in both of the patient reported assessments of xerostomia symptoms, GRCQ and XQ, in both unilateral and bilateral treated cohorts at 12 months post treatment
- Improvements in salivary flow were seen in unilateral as well as bilateral cohorts
- Durability out to 2 years in 4 participants and 3 years in 3 participants

- ❖ **Hyposalivation: Objective measure of saliva production – assessed by collecting whole saliva**
- ❖ **Xerostomia: Subjective feeling of dry mouth – assessed using patient reported outcome measures (PROs)**
- ❖ **Relationship between Xerostomia and Saliva Production**
 - Xerostomia symptoms are associated with reduction in saliva production
 - Xerostomia severity (or PRO score) is not directly correlated with an absolute volume of saliva production

ASCO GUIDELINES

TABLE 1. Definitions Related to Dysfunction of the Salivary Glands

Salivary gland hypofunction	Reduced salivary flow rate as measured objectively. Saliva flow rate is considered low when < 0.2 ml/min for unstimulated whole saliva ¹¹
Hyposalivation	Pathologic low saliva secretion, commonly defined as an unstimulated whole saliva flow rate of ≤ 0.1 ml/min or a stimulated whole saliva flow rate of ≤ 0.7 ml/min measured by sialometry ¹¹
Xerostomia	Patient-reported, subjective sensation of oral dryness. Although xerostomia most frequently occurs when the unstimulated whole saliva flow rate is reduced by about 45%-50% of the normal secretion of that person, ¹² there are no specific threshold levels of salivary flow rate that characterize xerostomia. The degree of xerostomia may be affected by factors other than salivary flow rates
Whole saliva	Derives from the major salivary glands (the parotid, submandibular, and sublingual glands, which account for 90% of the saliva secretion) and the minor salivary glands (which account for the remaining 10%)

- ❖ Patients are asked if there is a change in their symptom of Dry Mouth
- ❖ They may reply, “Better”, “Worse”, or “About the Same”
- ❖ If patients reply “Better” or “Worse”, they are asked to quantify the change on a 7-point scale with **the maximum score of 7** and “a very important change”, and 1 being the minimum
- ❖ A 2-point change is “large enough to be important” to the patient
- ❖ Anything 3 points or greater is considered a substantial improvement over standard of care and “transformative” by KOLs
- ❖ This questionnaire is very similar to the “Global Improvement” tool accepted by the FDA to approve Cevimeline

GLOBAL RATINGS OF CHANGE

- 1.0 Overall, has there been any change in your **Dry Mouth** since you received study treatment? Please indicate if there has been any change in your **Dry Mouth** by choosing one of the following response options:
(Place an “X” in the appropriate box below)
1. WORSE (if **WORSE**, go to question 2.0)
 2. ABOUT THE SAME (if **SAME**, go to question 4.0)
 3. BETTER (if **BETTER**, go to question 3.0)
- 2.0 How much **WORSE** would you say your **Dry Mouth** has been since you received study treatment? Please choose one of the following response options: **(Record the appropriate response option in the box below)**
1. almost the same, not important
 2. a little worse, but large enough to be important
 3. somewhat worse, still a small change, but large enough to be important
 4. moderately worse, an important change for the worse
 5. a good deal worse, an important change for the worse
 6. a great deal worse, a very important change for the worse
 7. a very great deal worse, a very important change for the worse
- (go to question 4.0)
- 3.0 How much **BETTER** would you say your **Dry Mouth** has been since you received study treatment? Please choose one of the following response options: **(Record the appropriate response option in the box below)**
1. almost the same, not important
 2. a little better, but large enough to be important
 3. somewhat better, still a small change, but large enough to be important
 4. moderately better, an important improvement
 5. a good deal better, an important improvement
 6. a great deal better, a very important improvement
 7. a very great deal better, a very important improvement
- (go to question 4.0)

GRCQ: Strong, Durable Improvements in Severity of Xerostomia was Demonstrated 12 Months After Unilateral Treatment

GRCQ UNILATERAL

Dry Mouth Symptoms?
Better (+), Worse (X),
or Same (=), How Much Better/Worse?

Cohort	Participant	Day 90	Day 180	Day 360	Month 18	Year 2	Year 3
1	1-1	+, 5	+, 6	+, 7	N/A	+, 7	+, 7
	1-2	+, 3	+, 3	+, 6	N/A	+, 2	+, 3
	1-3	+, 3	+, 3	=	N/A	+, 4	+, 4
2	2-1	=	=	=	N/A	=	
	2-2	+, 2	+, 4	+, 4	N/A	+, 4	
	2-3	+, 6	+, 6	+, 6	N/A		
3	3-1	+, 4	+, 3	+, 3	+, 3		
	3-2	=	=	=	=		
	3-3	NA	=	+, 5	=		
4	4-1	+, 4	+, 4	+, 4	+, 4		
	4-2	=	=	=			
	4-3	+, 4	+, 4	+, 6			

GRCQ Score for Unilateral Treatment (n=12) All participants to 12 months or more

- ❖ 8/12 participants at 12 months reported symptoms of dry mouth as 'better' following treatment
- ❖ Each of the 8 participants reported a score of 2 or more ie: "an important change"
- ❖ At 12 months, 4 participants rated the change in xerostomia symptoms with the highest improvement scores of 6 or 7 denoting "a very important improvement"
- ❖ Improvement in xerostomia symptoms can be seen persisting through 2 years in 4 patients and 3 years in 3 patients
- ❖ No participant reported any worsening of xerostomia symptoms

N/A: Month 18 data collection was included in a protocol amendment, data was not collected for these patients

GRCQ: Strong Improvements in Severity of Xerostomia At 12 Months Following Bilateral Treatment

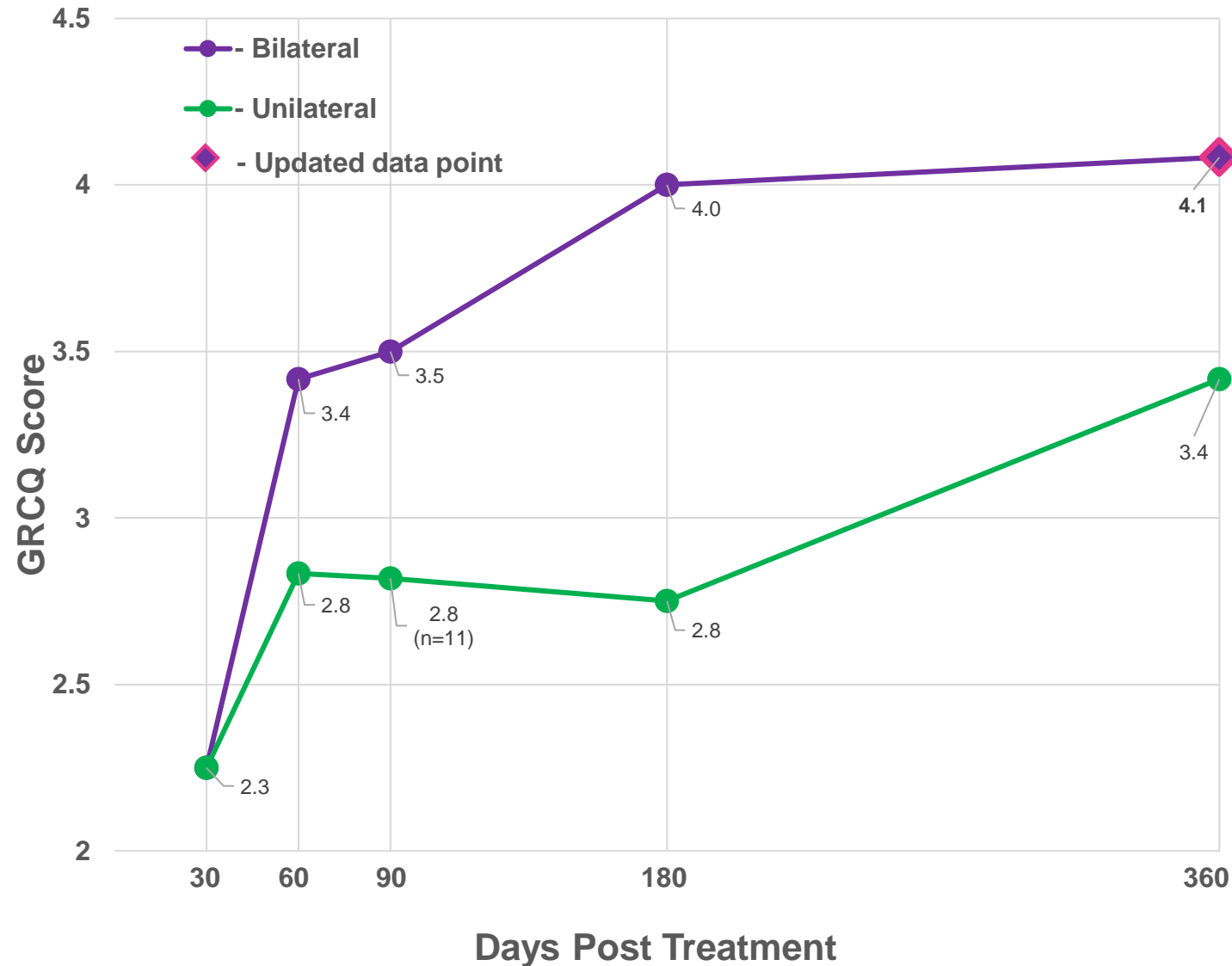
GRCQ BILATERAL		Dry Mouth Symptoms? Better (+), Worse (X), or Same (=), How Much Better/Worse?		
Cohort	Participant	Day 90	Day 180	Month 12
1b	1b-1	+, 4	+, 4	+, 4
	1b-2	+, 4	+, 5	+, 6
	1b-3	+, 5	+, 6	+, 5
2b	2b-1	+, 1	=	=
	2b-2	+, 5	+, 5	+, 5
	2b-3	+, 1	+, 2	=
3b	3b-1	+, 2	=	+, 2
	3b-2	+, 6	+, 7	+, 7
	3b-3	+, 6	+, 6	+, 6
4b	4b-1	+, 4	+, 4	+, 6
	4b-2	+, 4	+, 5	+, 2
	4b-3	=	+, 4	+, 6

GRCQ Score for Bilateral Treatment (n=12) All participants to 12 months

- ❖ 10/12 (83%) participants at 12 months reported symptoms of dry mouth as ‘better’ following treatment
- ❖ Each of these participants rated changes in xerostomia scores that were important or very important with a score of 2 or more at 12 months
- ❖ 5 participants rated the change in xerostomia symptoms with scores of 6 or 7 denoting “a very important improvement”
- ❖ No participant reported any worsening of xerostomia symptoms
- ❖ For all patients including bilateral and unilateral (n=24), 18/24 or 75% reported dry mouth as ‘better’ with a clinically meaningful score of 2 or more

GRCQ: Overall Improvement Greater in Bilateral compared to Unilateral treatment group; Unprecedented 4-point Improvement at 12 Months

GRCQ improvements for Bilateral and Unilateral and Treated Cohorts



n=12 unless otherwise indicated

- ❖ In the overall cohorts, the average improvement score in GRCQ was greater in bilateral compared to unilateral
- ❖ Overall improvements were maintained and increased over time in both unilateral and bilateral cohorts
- ❖ A 2-point change in GRCQ compared to placebo is considered significant by KOLs
- ❖ Anything 3 points or greater is considered a substantial improvement over standard of care and “transformative” by KOLs
- ❖ Unilateral cohort achieved overall improvement of >3 points at 12 months
- ❖ Bilateral cohort achieved overall improvement of >3 points at 2 months and an overall improvement of 4 points by 6 months, this 4-point improvement is maintained at 12 months

- A Patient Reported Outcome measure
- 8 symptom-specific questions wherein the patient rates each symptom from 0 (not present) to 10 (worst possible)
- Responses are summed (0-80), providing an overall measure of disease burden
- **An improvement (decrease) of 8 points (or 10%) or more is considered clinically meaningful**
- **A decrease in score of 10 or greater is considered a substantial improvement over standard of care and “transformative” by KOLs**

Xerostomia-Specific Questionnaire (XQ)

Objective: To measure patient-reported xerostomia (decreased saliva flow) associated with radiation therapy (RT) for head-and-neck cancer

Instructions: Patients are to rate (circle) each of the eight items on a scale from 0 to 10; the higher the score, the worse the xerostomia

1. Rate your difficulty in talking due to dryness
0 1 2 3 4 5 6 7 8 9 10
2. Rate your difficulty in chewing due to dryness
0 1 2 3 4 5 6 7 8 9 10
3. Rate your difficulty in swallowing solid food due to dryness
0 1 2 3 4 5 6 7 8 9 10
4. Rate the frequency of your sleeping problems due to dryness
0 1 2 3 4 5 6 7 8 9 10
5. Rate your mouth or throat dryness when eating food
0 1 2 3 4 5 6 7 8 9 10
6. Rate your mouth or throat dryness while not eating
0 1 2 3 4 5 6 7 8 9 10
7. Rate the frequency of sipping liquids to aid swallowing food
0 1 2 3 4 5 6 7 8 9 10
8. Rate the frequency of sipping liquids for oral comfort when not eating
0 1 2 3 4 5 6 7 8 9 10

Xerostomia Questionnaire (XQ): Very Strong Improvements (Decrease in Score) Compared to Baseline Observed in Both Unilateral and Bilateral Cohorts



Change From Baseline Unilateral

Cohort	Participant	Day 90	Day 180	Day 360	Month 18	Year 2	Year 3
1	1-1	-14.7	-14.7	-18.7	N/A	-19	-17.7
	1-2	-8.3	-0.3	-17.3	N/A	-7.3	-28.3
	1-3	-6.3	-6.3	-3.3	N/A	-6.3	-4.3
2	2-1	-14.0	-9.0	-8.0	N/A	-1.0	
	2-2	-23.0	-24.0	-21.0	N/A	-17.0	
	2-3	-38.7	-29.7	-34.7	N/A		
3	3-1	-19.3	-20.3	2.7	-14.3		
	3-2	7.7	1.7	-0.3	-7.3		
	3-3	5.3	-1.7	-4.7	-5.7		
4	4-1	-37.7	-34.7	-12.7	-20.7		
	4-2	-3.3	0.7	3.7			
	4-3	-39.0	-41.0	-43.0			

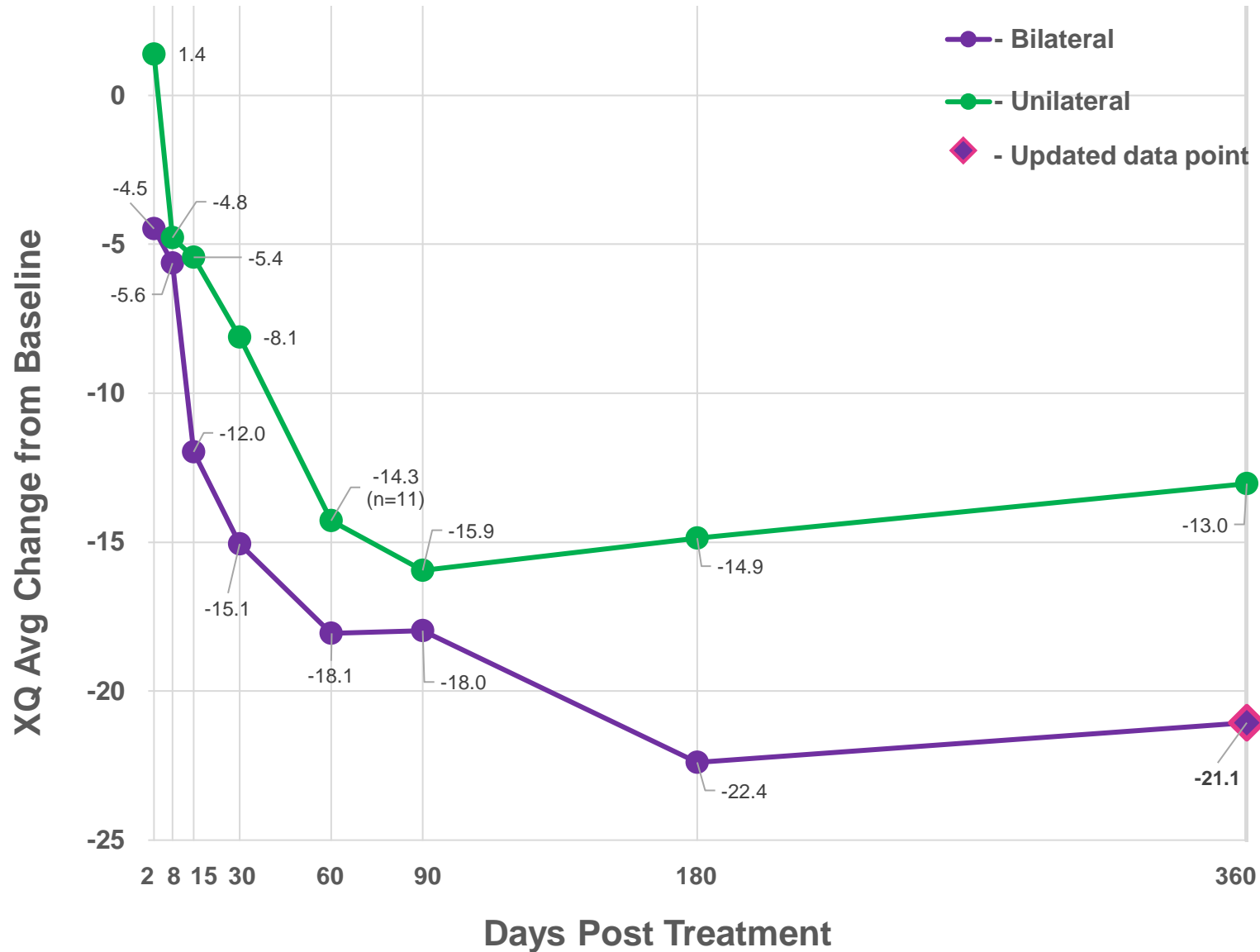
Change From Baseline Bilateral

Cohort	Participant	Day 90	Day 180	Month 12
1b	1b-1	-15.3	-17.3	-16.3
	1b-2	-31.3	-26.3	-41.3
	1b-3	-11.0	-10.0	-10.0
2b	2b-1	-7.3	-11.3	-2.3
	2b-2	-34.7	-33.7	-37.7
	2b-3	-15.7	-23.7	-4.7
3b	3b-1	-4.0	-5.0	-6.0
	3b-2	-26.3	-30.3	-23.3
	3b-3	-29.7	-44.7	-27.7
4b	4b-1	-27.0	-35.0	-31.0
	4b-2	-16.0	-31.0	-30.0
	4b-3	2.7	-0.3	-22.3

- ❖ **Unilateral:** 7/12 had score improvements (decrease) ≥ 8 at 12 months
- ❖ **Bilateral:** 9 /12 had score improvements ≥ 8 at 12 months
- ❖ **Overall:** 16 /24 (66%) had an improvement following treatment of ≥ 8 points
- ❖ A decreased score of 10 is considered transformative
- ❖ 6/12 or 50% of unilateral at 12 months and 9/12 or 75% of bilateral at 12 months achieved at least a 10-point improvement
- ❖ There was good concordance with the individual patients who responded in XQ and GRCQ

N/A: Month 18 data collection was included in a protocol amendment, data was not collected for these patients

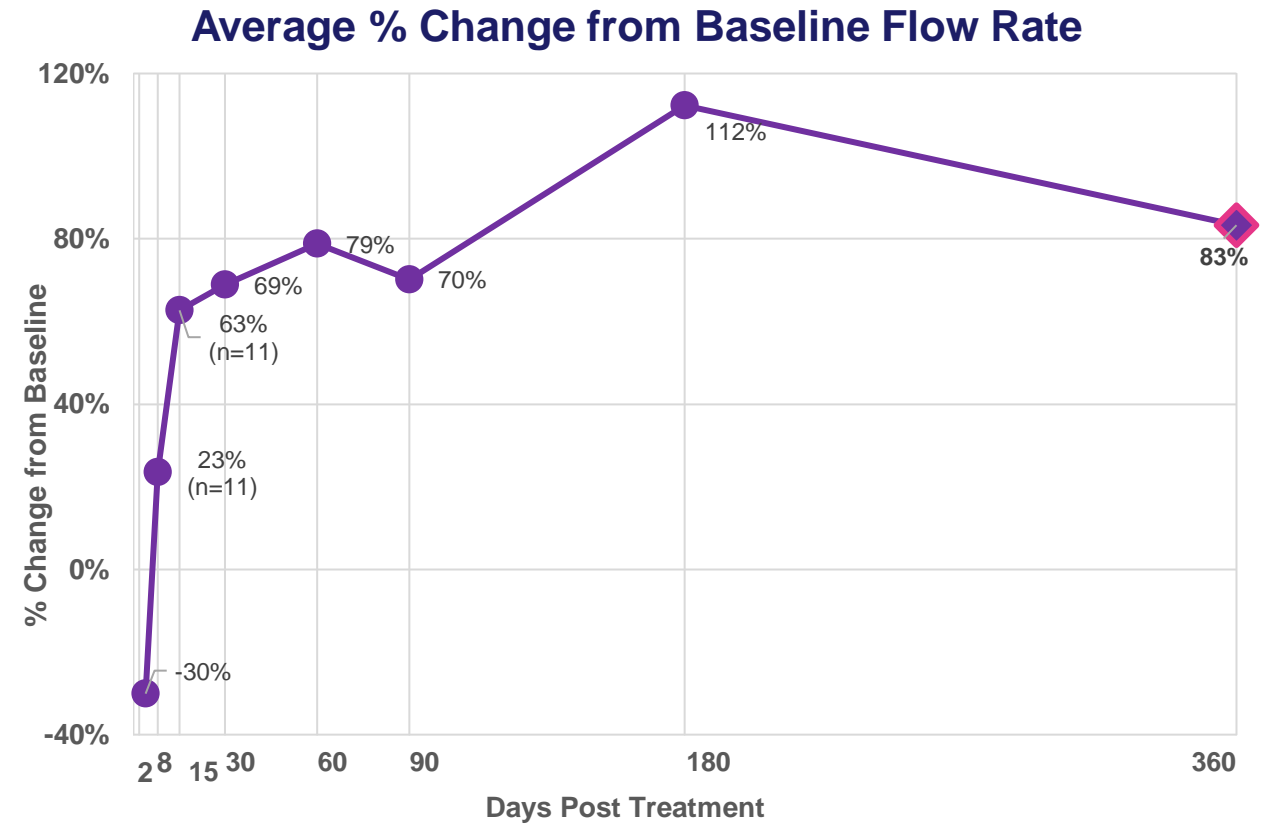
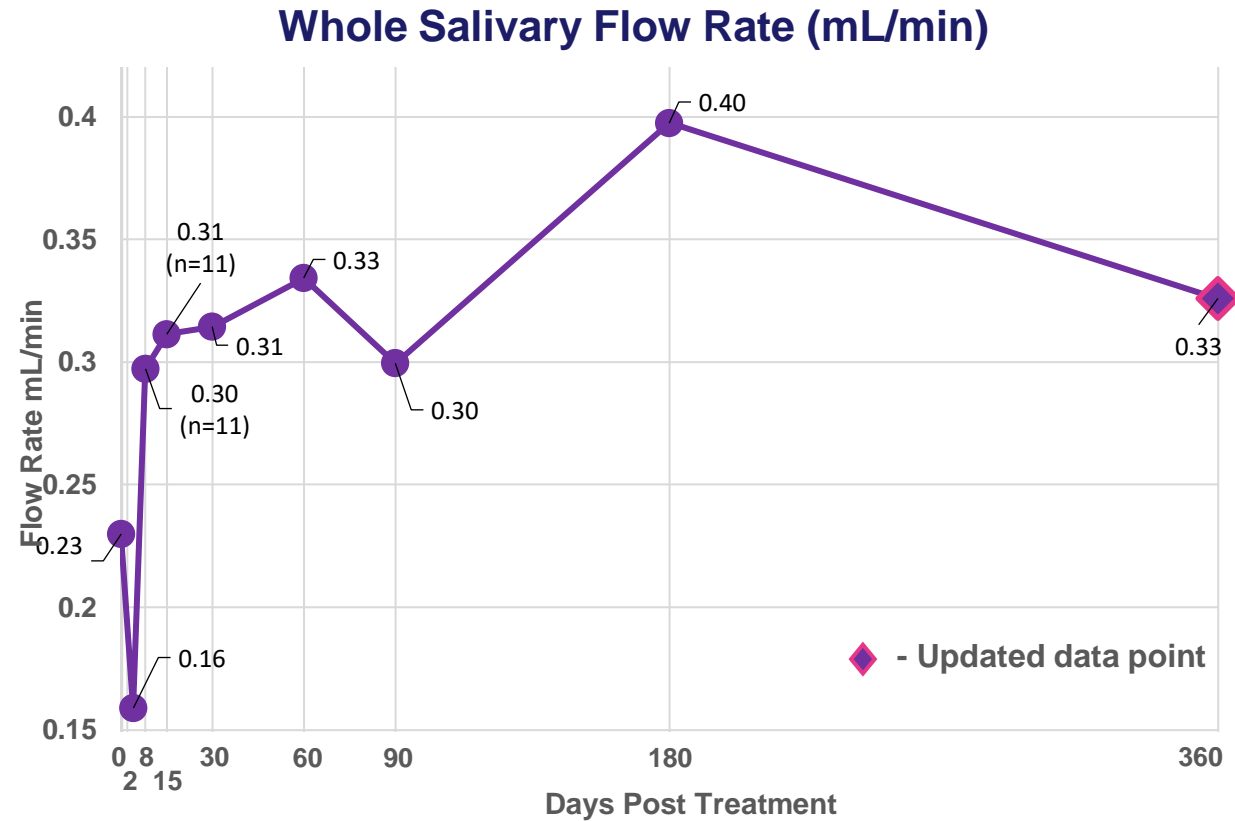
XQ: Substantial Clinically Meaningful Improvements From Baseline in XQ in both Unilateral and Bilateral treated Cohorts



- ❖ **Unilateral:** 13-point improvement from baseline at 12 months
- ❖ **Bilateral:** 21-point improvement from baseline at 12 months
- ❖ Improvement in XQ was observed rapidly post treatment
- ❖ In both groups XQ scores improved (declined) >8 points soon after treatment, and >10 points within 2 months after treatment
- ❖ **This level of benefit is considered transformative by KOLs**
- ❖ As with the GRCQ, the degree of improvement was greater in bilateral compared to unilateral treated cohorts

n=12 unless otherwise indicated

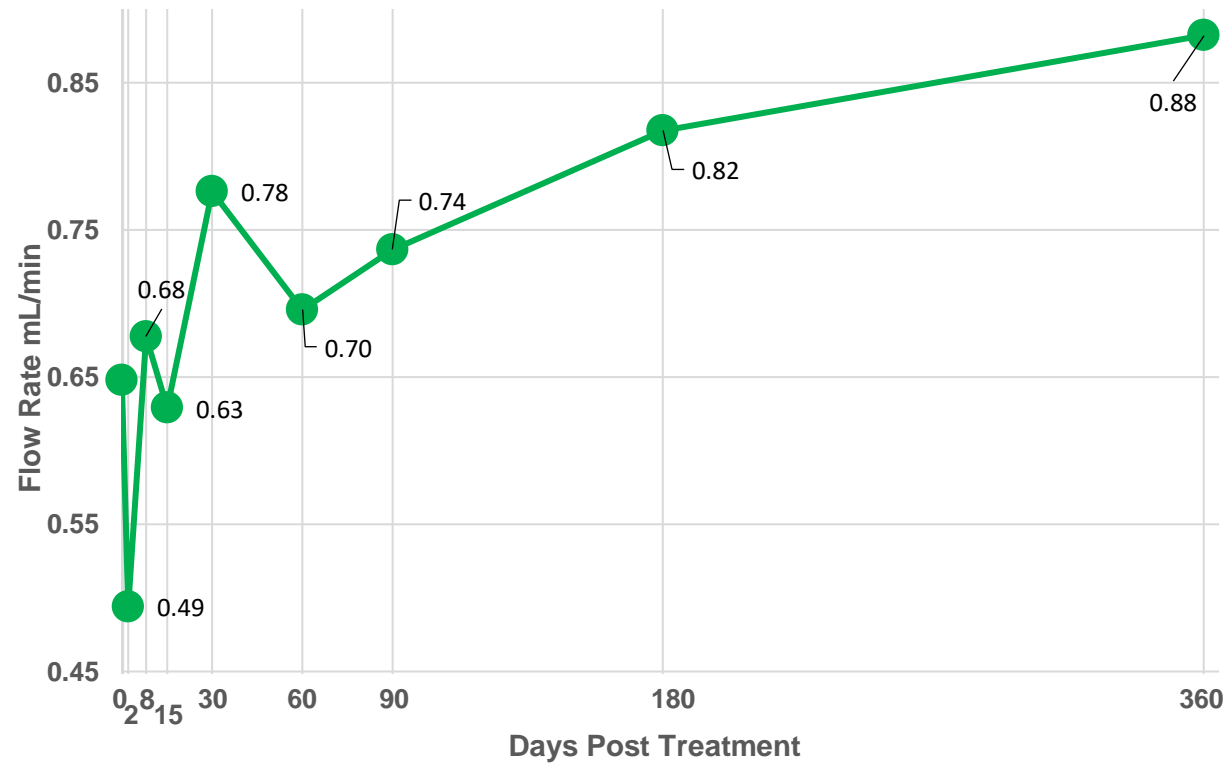
Bilateral Cohorts: Meaningful Improvement in Unstimulated Whole Saliva Production Achieved Reaching Normal Levels Following AAV2-hAQP1 Treatment



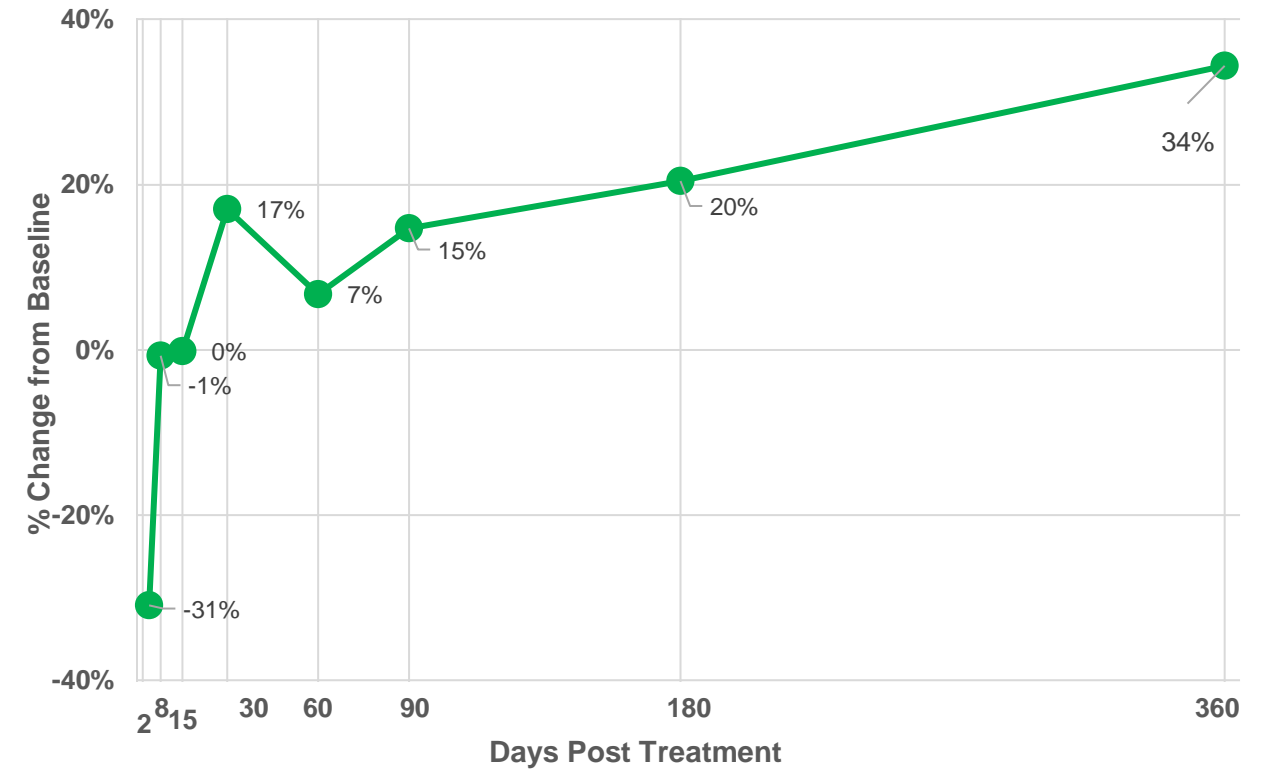
- Meaningful increase in whole salivary flow was seen in bilateral treated patients
- The overall flow rate improved to an average of 0.33 mL/min which is in the normal range for unstimulated whole saliva production
- Normal unstimulated salivary flow rate averages 0.3-0.4 mL/min
- If flow rate of unstimulated saliva is >0.1-0.2mL/min, then salivary hypofunction is diagnosed with associated xerostomia likely
- The average % change from baseline was 83% at 12 months
- This is clinically meaningful as a 50% reduction in whole saliva volume is associated with xerostomia symptoms
- Based on both absolute whole resting saliva as well as the overall % change from baseline – the improvement in unstimulated salivary flow in the bilateral appear to be of clinically meaningful size that could result in improvement in xerostomia symptoms

Unilateral Treated Subjects Also Showed Improvement in Absolute Whole Saliva Measures (Stimulated)

Absolute Salivary Flow Rate (mL/min)



Average % Change from Baseline Flow Rate



- Increase in whole salivary flow was seen in unilateral treated patients
- Whole saliva was collected using gum stimulation, however this was directly following citric acid stimulation for extended periods with manipulations to attempt collection from individual glands
- Normal stimulated salivary flow rate averages 1.5–2.0 mL/min
- A diagnosis of hyposalivation is made with flow rate ≤ 0.5 – 0.7 mL/min

Improvements in Both Xerostomia Severity Scores and Saliva Production Demonstrated Following AAV2-hAQP1 Treatment



SUMMARY

- ❖ Meaningful improvements in xerostomia symptoms were reported across both unilateral and bilateral treated cohorts
- ❖ As assessed by the GRCQ in unilateral and bilateral treated patients, 18/24 (75%) reported a clinically meaningful score of 2 or more
- ❖ Using the XQ severity scale, 16/24 (66%) had an improvement of ≥ 8 points and 15/24 (63%) had an improvement of 10 or more points
- ❖ Increases in whole saliva flow rates observed post-treatment, providing objective evidence supporting biological activity
- ❖ Unstimulated whole saliva flow increased meaningfully in the bilaterally treated cohorts with improvement to normal levels being achieved 6 months post treatment
- ❖ Greater improvements were observed in bilaterally treated patients across every assessment compared to unilateral
- ❖ Early long-term follow-up data suggest durability of improvement 3 or more years post-treatment
- ❖ Biopsy data shows transduction of cells of parotid glands treated AAV-hAQP1, expression of hAQP1 protein, and persistence to at least 24 months post treatment

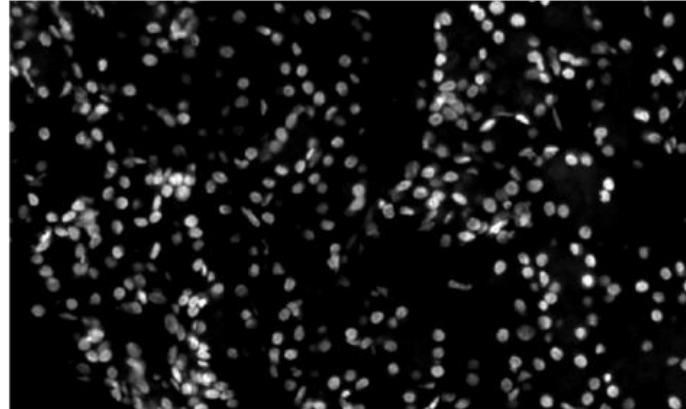
Study MGT001: AAV2-hAQP1 Persists in Parotid Gland for at Least 24 Months After Treatment

Participant	Cohort	Dose per gland	Dose Concentration	Visit of Biopsy	Copy #/ng DNA	Copy #/Cell
AAV001	1	1E10	1.43E10	18 Months	160	0.96
AAV005	1	1E10	5.00E9	24 Months	122	0.73
AAV002	2	3E10	1.76E11	18 Months	236	1.4
AAV019*	3	1E11	1.11E11	24 Months	5393	32
AAV020	4	3E11	1.50E11	30 Months	ND	ND
AAV021*	4	3E11	1.15E11	12 Months	87390	524
AAV031	5	6E11	3.16E11	12 Months	7313	43

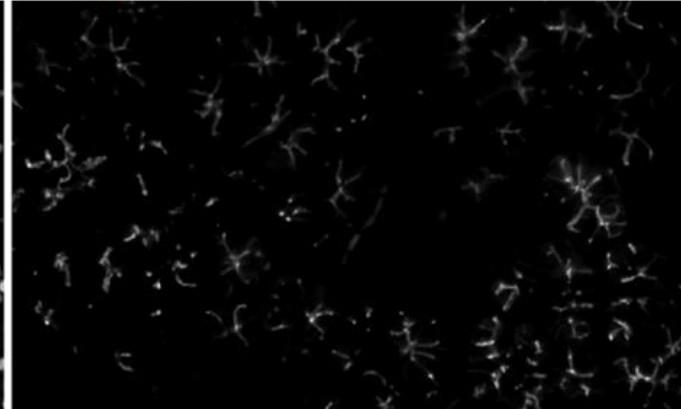
- Biopsies were obtained in 7/15 participants enrolled in MGT001
- 6/7 biopsies showed AAV2-hAQP1 genomes ≥ 12 months post-treatment
- There is a trend of increasing copy number of transduced vector genomes with increasing viral vector dose

Study MGT001: AAV2-hAQP1 Persists in Parotid Gland for at Least 24 Months After Treatment

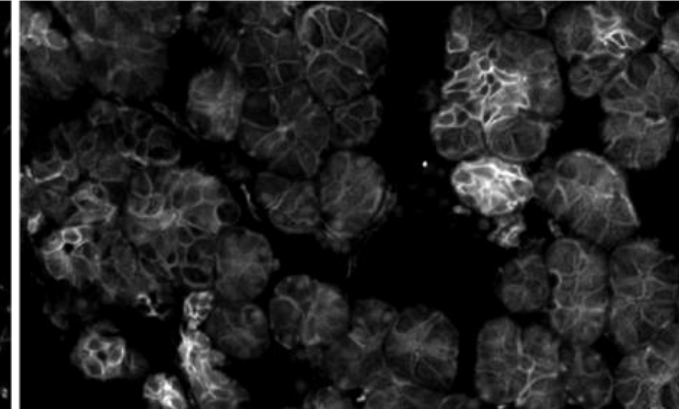
DAPI



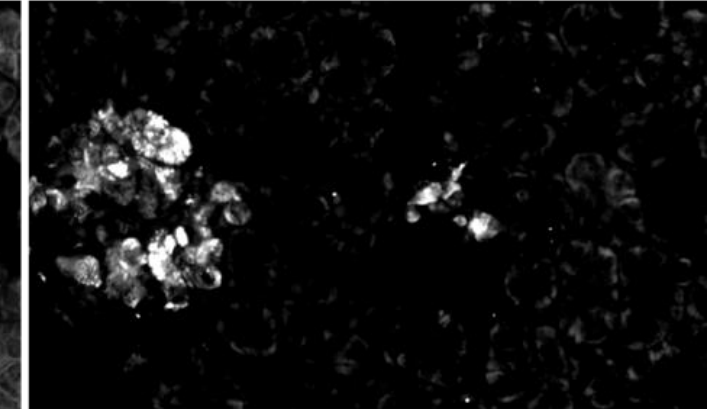
AQP5



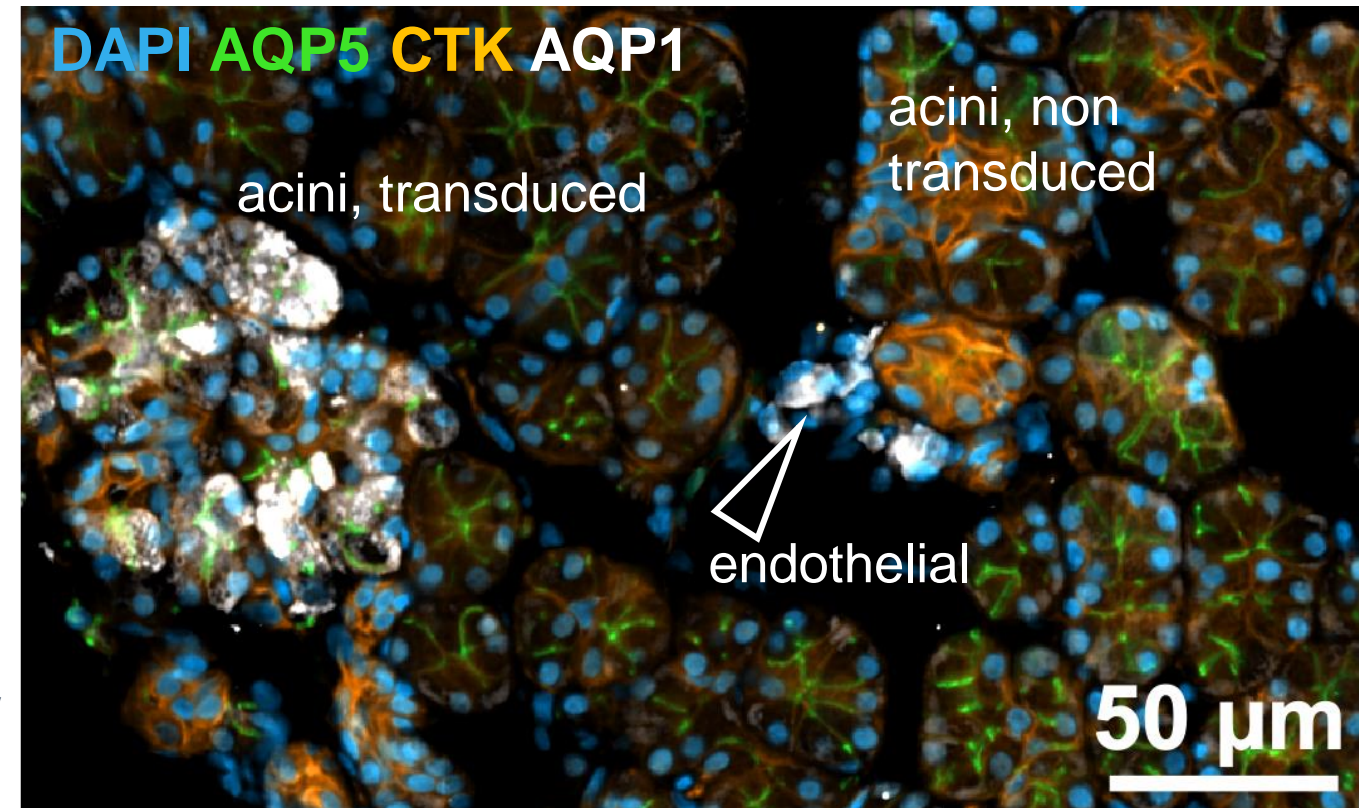
CTK



AQP1



- The image in this slide comes from a core needle biopsy from participant AAV019 in the NIH Phase 1 study
- AQP1 protein expression is 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



Most comprehensive viral vector manufacturing infrastructure in the industry, supported by robust know-how and patent estate

- In-house, commercial scale cGMP viral vector manufacturing – 2 facilities, London and Shannon, support commercial production
- In-house cGMP plasmid manufacturing - overcoming a significant supply chain bottleneck in the industry
- In-house QC, full analytics for commercial release and stability – essential to overcome global CRO deficiency in this area
- On-site Fill/Finish and Central Warehouse
- Experienced global regulatory team supporting 6 clinical programs Phase 2 and Phase 3
- Preparing for global commercial supply supporting BLA filing 2024



Phase 2 randomized, double-blind, placebo-controlled study is now open

Study Design

- Randomized, double-blind, placebo-controlled
- 120 participants: Two active doses of AAV2-hAQP1 vs Placebo 1:1:1
- Active Doses: 0.4E12 and 1.2E12 (n=40 for each arm)
- A third higher dose of 3.6E12 may be added to the blinded design at a future date

Primary Efficacy Endpoints

- Change from Baseline to 12 Months in Symptom-specific Xerostomia Questionnaire (XQ)

Key Secondary Endpoints

- Change from Baseline to 12 Months in Whole Saliva Flow Rate
- Safety and tolerability of AAV2-hAQP1 treatment
- GRCQ is also being assessed as a secondary endpoint

AAV-hAQP1 is a Potentially Transformative Treatment for Grade 2/3 Radiation-Induced Xerostomia

- AAV2-hAQP1 has potential to become a standard of care for long term grade 2/3 radiation induced xerostomia patients based on its disease-modifying mechanism of action and meaningful improvements in both objective and subjective assessments of disease
- Salivary gland cells are very slowly dividing, and a single administration is anticipated to have a durable effect
- The one-time treatment is a minimally invasive, non-systemic administration delivered through an outpatient cannulation procedure that ENTs are already familiar with. It is a small locally delivered dose of AAV2-hAQP1 with excellent safety profile.
- AAV2-hAQP1 treatment for grade 2/3 xerostomia is a large commercial opportunity given the high unmet need, large prevalent population as well as very large incidence population globally – with no current effective treatments and no other known treatment in the clinic
- AAV2-hAQP1 uses a small locally delivered dose, COGS are low, with plasmid production, viral vector manufacturing and QC analytics all in-house at Meira, providing flexibility to support a range of sustainable price points for patients and payors
- Data to be presented at a scientific meeting in the second half of 2023



MEIRAGTx

Audience Q&A