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AAV-AQP1 Long Term Data and Market Review

April 16, 2026

Forward Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this presentation that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the development and efficacy of AAV-hAQP1, reporting of such data and the timing of results of data and regulatory matters, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate,” “eligible” 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, raise additional capital, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic transactions or priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug or rare pediatric disease designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; 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 or other manufacturing issues; 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; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2025, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this 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 April 16, 2026.

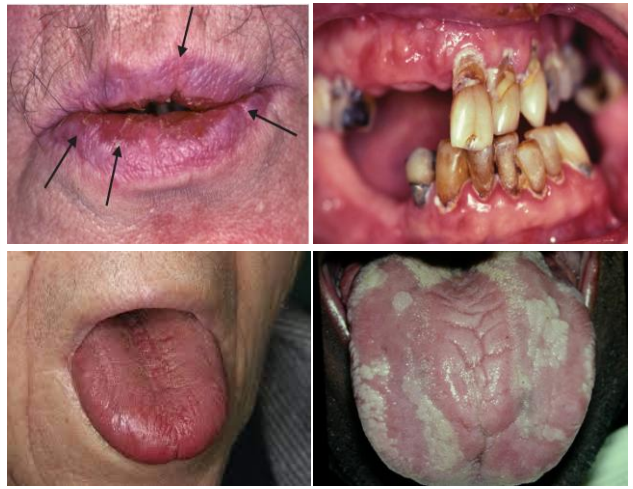
Agenda

1. **Introduction:** Zandy Forbes, PhD. President & CEO MeiraGTx
   - Persistent Radiation Induced Xerostomia (RIX) is a severe, devastating, untreatable lifelong condition
   - Etiology of persistent RIX following radiation for Head and Neck Cancer
   - Mechanism of Action of AAV-AQP1
   - Phase 1 endpoints and data summary
2. **AQUAx Phase 1 Study Data:** Zandy Forbes, PhD President & CEO MeiraGTx
   - Phase 1 AQUAx 12-month data recap
   - Phase 1 AQUAx 3-year data showing robust 3-year durability and intra-patient consistency
3. **Study Investigator discussion of disease burden, patient experience, and treatment administration**
   - David Owens, MBCHB, FRCS, MPHIL, PGDME, FFST(Ed) (University Hospital of Wales)
   - Michael Brennan, DDS, MHS (Wake Forest University School of Medicine)
4. **AAV-hAQP1 Commercial Opportunity:** Zandy Forbes, PhD President & CEO MeiraGTx
   - Clinician Response to Durability demonstrated in 3-year data
   - Global and US market
   - Payor response to Durability Data
   - Market Concentration in the US
5. **Question and Answer Session**

Patient experience and disease burden: How bad is RIX really? Very bad indeed.

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

- Extreme persistent dry mouth, inability to swallow or chew, loss of sense of taste
- Major diet restrictions, ongoing weight loss, need for invasive tube feeding
- Oral health complications and frequent oral infections, sores and persistent pain.
- Uncontrolled dental caries, accelerated loss of dentition requiring major reconstruction
- Impaired speech, difficulty sleeping, inability to exercise as faster breathing may lead to choking
- 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**

Etiology of Persistent RIX and Population Size

Persistent RIX is the most frequent and severe consequence of curative radiation treatment for head & neck cancer

- Almost all patients treated with radiation for H&N cancer experience acute xerostomia at the time of radiation
- In 60%-70% of patients, acute radiation induced xerostomia resolves or becomes manageable by 12 to 18 months after radiation
- However, in **~ 30%-40% of patients xerostomia does not resolve** even 2 years after radiation
- **85%** of these patients do not respond to any available therapy
- **Persistent RIX is a lifelong condition that only gets worse with time, with no effective therapies**

Persistent RIX following treatment for H&N cancer is a large population and a completely unmet need:

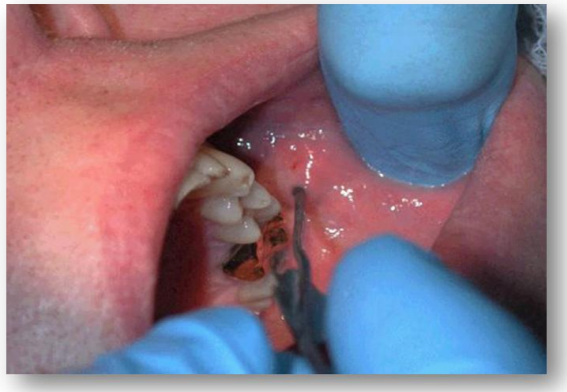
- In the US: there are **165k prevalent patients** and >20k incidents per year
- Globally (US, EU, Japan) there are **435k prevalent patients** and 48k incidents per year
- A large, commercial opportunity of up to **$3.8bl annual sales** globally and ~ $2bl in US

Mechanism of Action of AAV-AQP1 for treatment of Late Radiation-induced Xerostomia (RIX)

- Salivary glands are particularly vulnerable to radiation
- Damage to salivary glands during radiation leads to acute xerostomia in almost all patients
- Over 12-18 months, damaged glands may remodel, saliva flow restored and xerostomia alleviated or becomes manageable (60%-70%).
- **In 30-40% of patients the damage to salivary glands is irreversible**, the glands fail to recover and xerostomia persists for life.
- AAV-hAQP1 is instilled into the duct of damaged glands and transduces the remaining gland epithelium with the gene encoding the Aquaporin 1 (AQP1), a non-polarized water channel.
- **Expression of the 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 Delivery Procedure:

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



Phase1 (n=24): Transformative Clinical Improvements in Xerostomia PRO (XQ) as well as Objective Saliva Flow (UWSFR) Endpoints

Xerostomia Clinical Definition:

- Xerostomia is a purely patient reported condition
- The level of xerostomia symptoms is not correlated to the absolute amount of saliva produced by an individual (ASCO Guidelines)
- However, xerostomia is the result of too little saliva available to wet the mouth and retain normal oral health and function.

Xerostomia Endpoints:

- Xerostomia Questionnaire (XQ) is the standard patient-reported measure of xerostomia
- Unstimulated Whole Saliva Flow Rate (UWSFR) is an objective measure of the change in salivary function

Phase 1 Clinical Data:

- **Compelling 12 month data:**
  - **XQ score** 12-month data demonstrate **"unprecedented" and "transformative"** improvements in xerostomia
  - **USWFR** also showed large increases in water flow into the mouth – the objective measure of AAV-AQP1 MOA
- **Durability of effect :** Phase 1 study shows these **transformative improvements in XQ and UWSFR are maintained out to 3 years**

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

Granted Breakthrough Therapy designation in addition to RMAT and ODD

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Phase 1 AQUAx Clinical study of AAV-AQP1 for treatment of radiation induced xerostomia

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

SAFETY: Primary Endpoint of Phase 1 Study

- AAV2-hAQP1 was generally safe and well-tolerated at all doses tested
- No treatment-related serious adverse events
- No dose-limiting toxicities
- No participant discontinued from the study
- 6 mild, treatment-emergent treatment-related adverse events (TEAEs). All resolved without sequelae.

AQUAx: Xerostomia Questionnaire (XQ) 12 month change from baseline

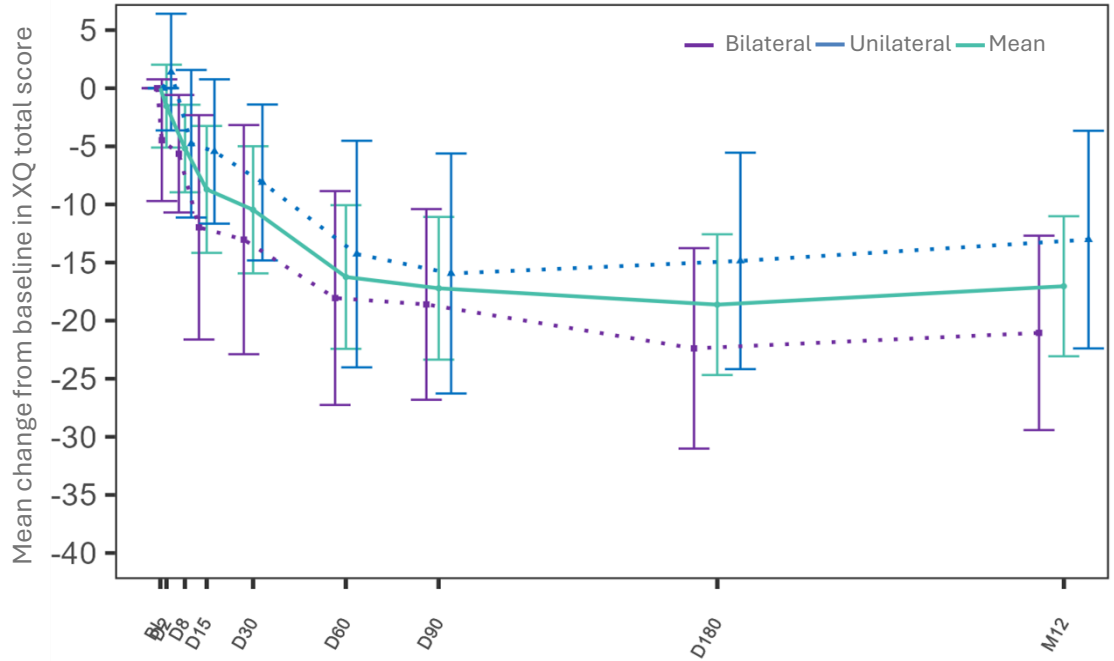
- 8 symptom-specific xerostomia PRO answered by patients with a total **maximum score of 80 points** (higher is worse)
- An improvement (decrease) of **≥8 points** is considered clinically meaningful
- An improvement of **≥10 points** is considered transformative

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)

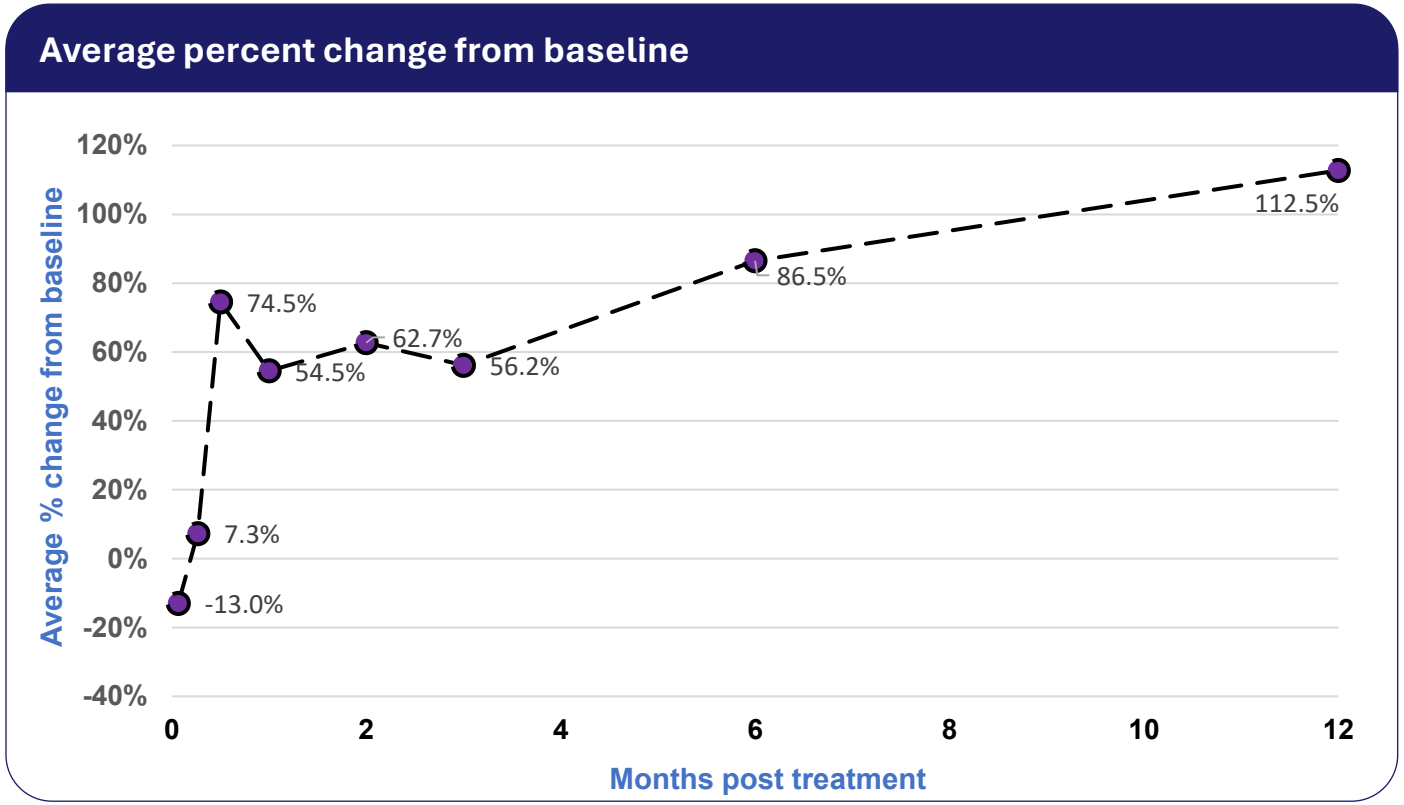
Average change in XQ score



1. Eisbruch A et al. Xerostomia and its predictors following parotid-sparing irradiation of head-and-neck cancer. Int J Radiat Oncol Biol Phys. 2001 Jul 1;50(3):695-704. 2. Jabbari S et al. Matched Case–Control Study of Quality of Life and Xerostomia after Intensity-Modulated Radiotherapy or Standard Radiotherapy for Head-and-Neck Cancer: Initial Report. Int. J. Radiat. Oncol. Biol. Phys. 2005;63:725–731.

AQUAx: Unstimulated whole saliva flow rate average percent change from baseline at 12 months pooled unilateral and bilateral cohorts

At Month 12, the Unstimulated Whole Saliva Flow Rate increased by 112.5% from baseline

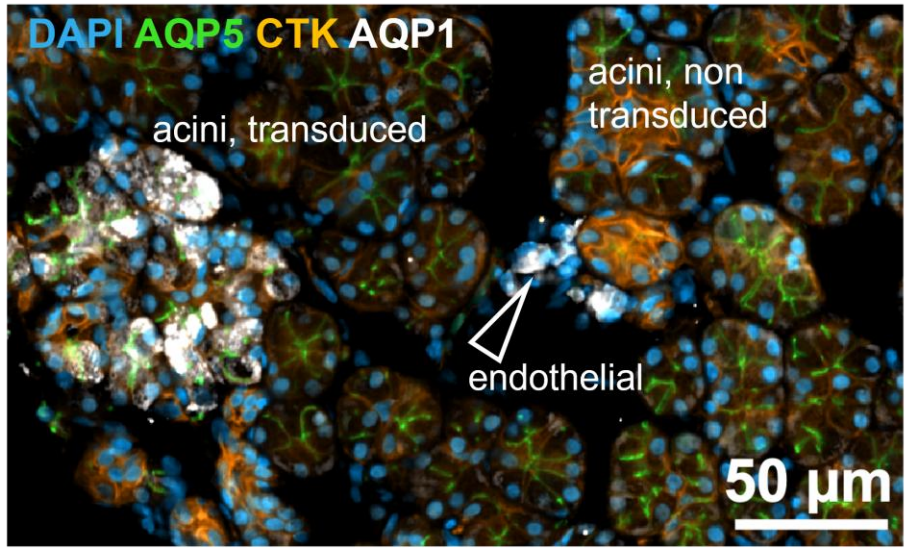


Biopsies indicate that AAV2-hAQP1 persists in the salivary gland

- 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



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AAV-AQP1: for treatment of xerostomia

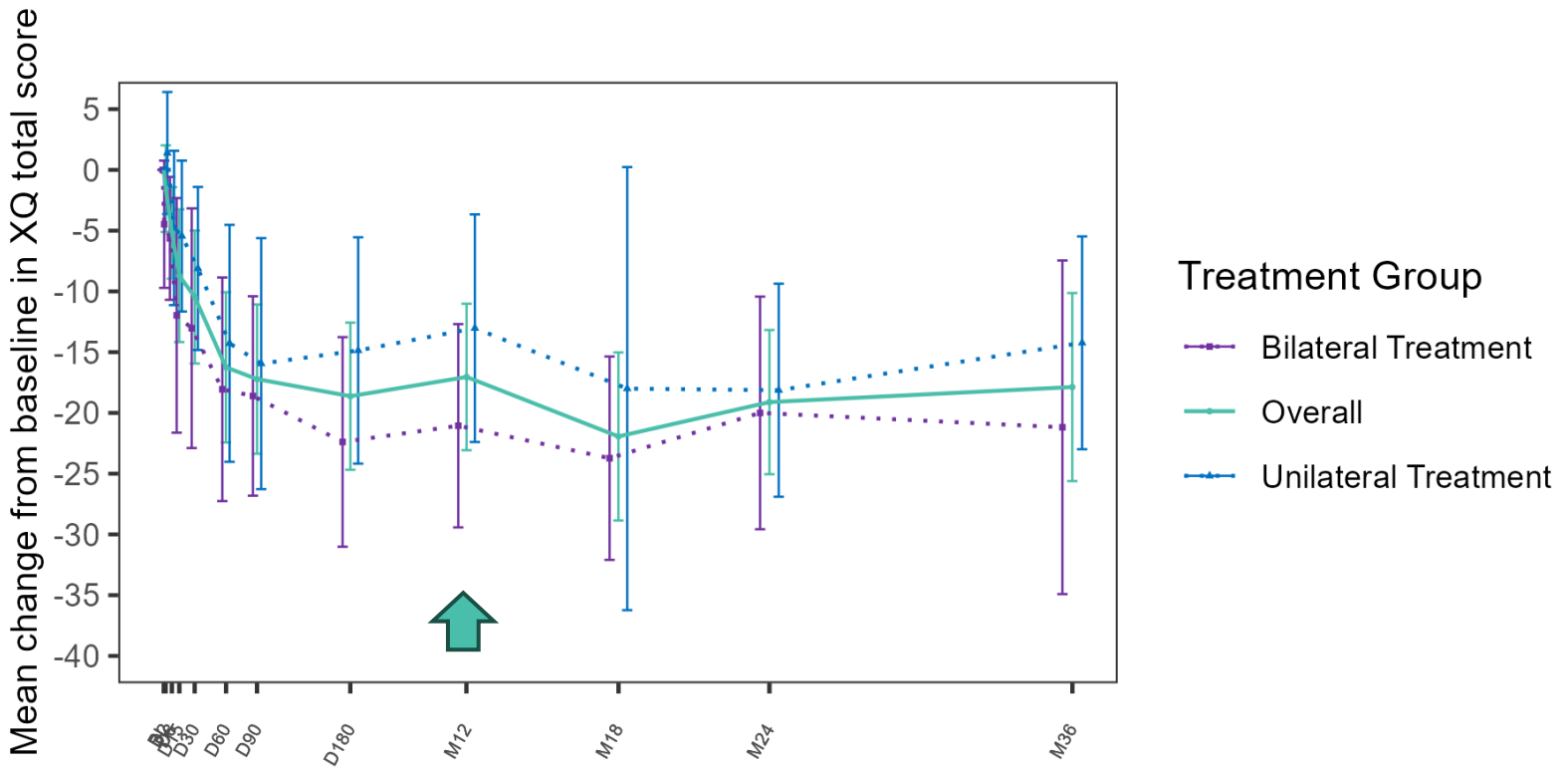
Phase 1 data out to 3 years on all cohorts

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

The transformative improvement in the XQ PRO observed in Month 12 were maintained through Month 36

Bilaterally-treated participants reported greater improvement than those treated unilaterally

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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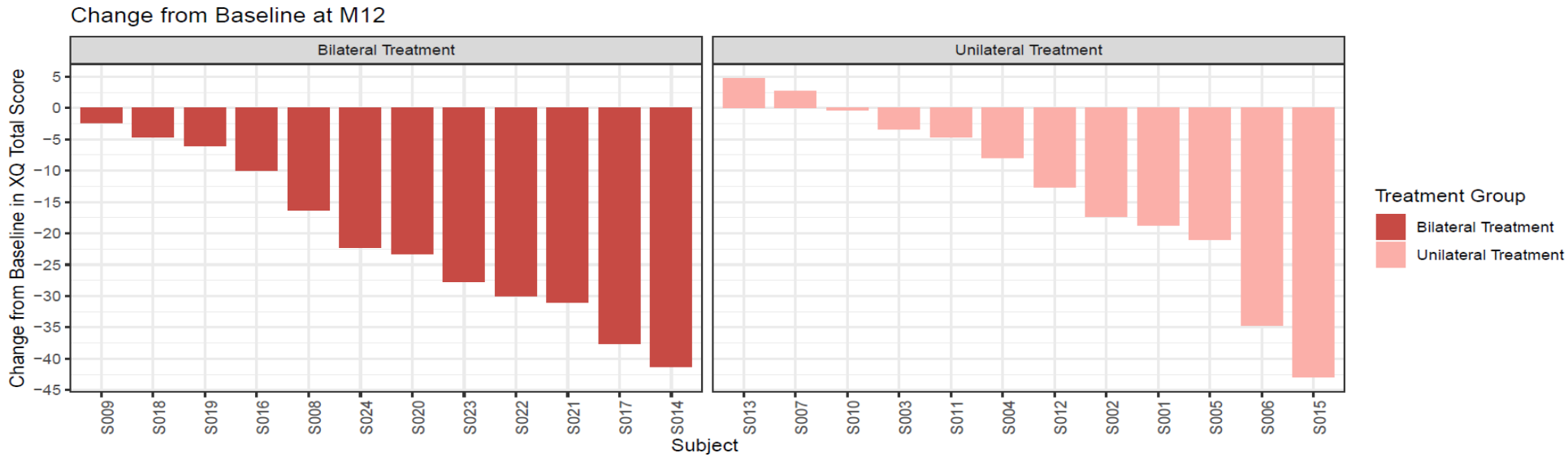
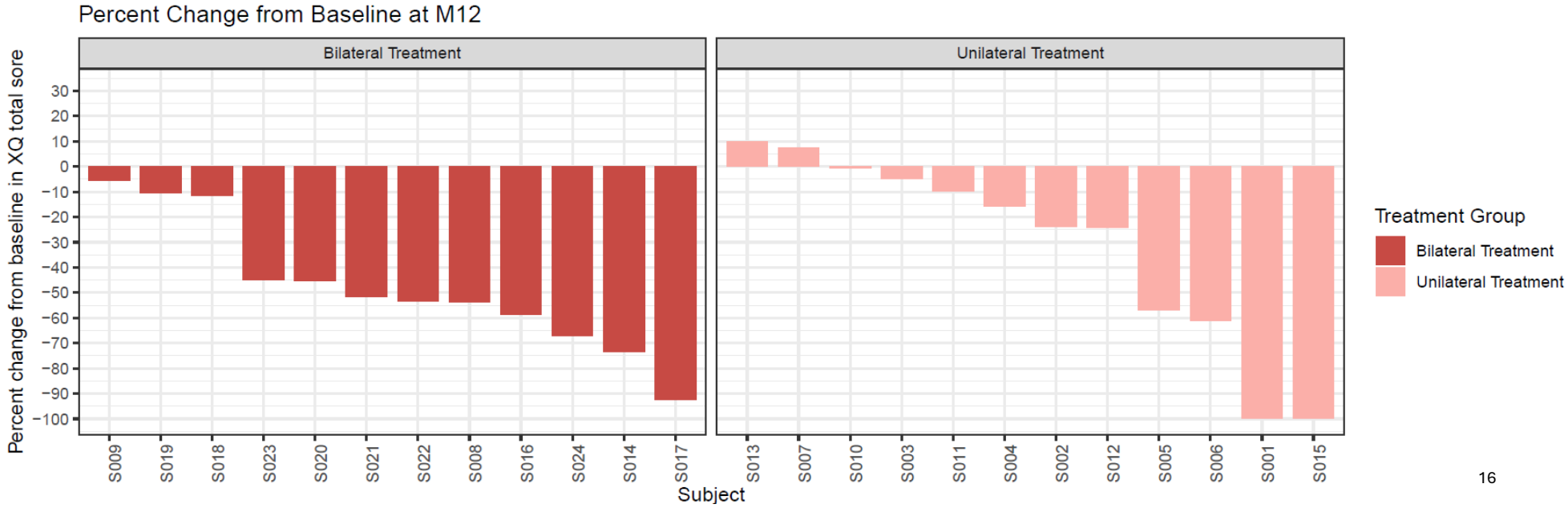
Waterfall Plots of individual subject XQ scores at 12 months: Bilateral and Unilateral: Absolute change and Percentage change

Absolute Change from Baseline



Percent Change from Baseline



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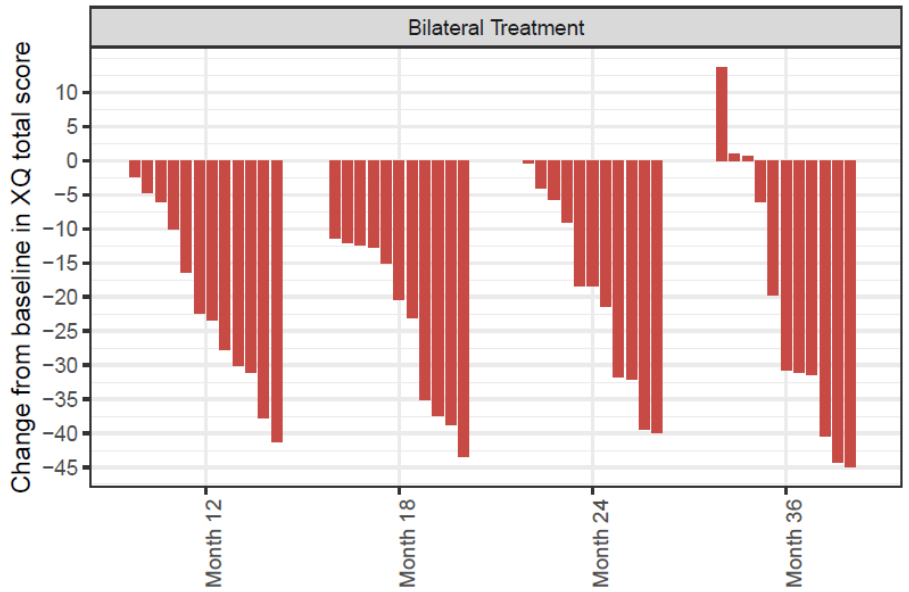
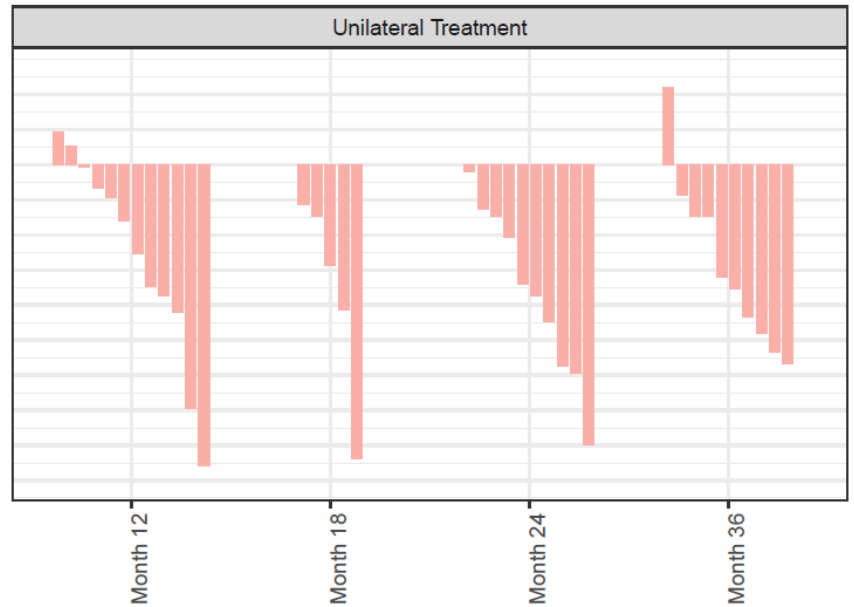
Individual patient data demonstrate durable clinical response over 3 years

Bilateral cohort: Change from baseline (XQ)



Unilateral cohort: Change from baseline (XQ)



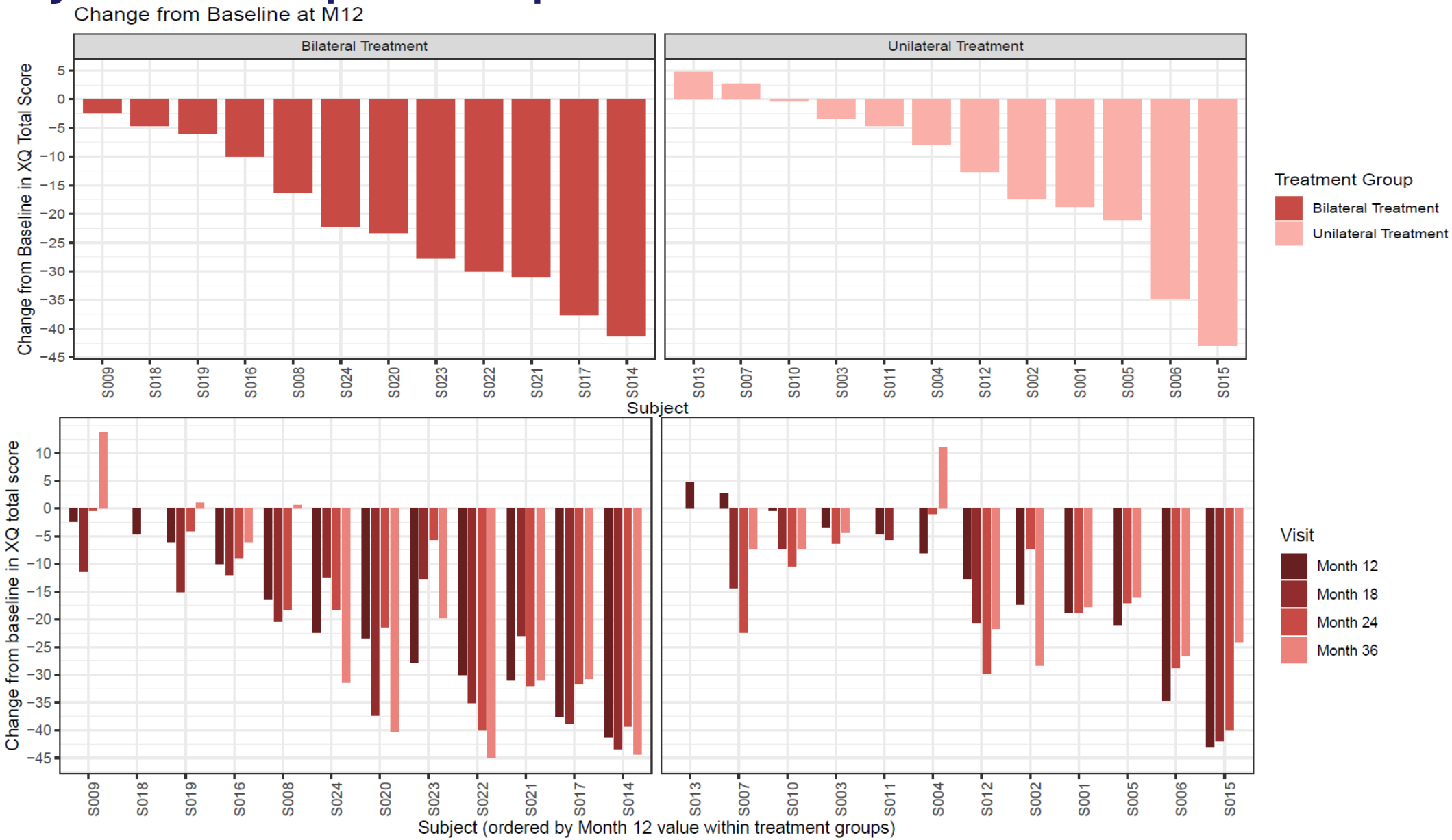
Robust Clinical Response: almost all patients experienced a significant improvement in xerostomia symptoms, with some patients reporting complete **resolution of their xerostomia symptoms**

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

Individual Patient XQ score at each visit out to 36 months: the graphs shows the XQ scores for each patient from left to right in the order of magnitude of XQ in the 12 month waterfall plot grouped together at 12,18, 24 and 36 months and illustrates the consistency in XQ 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 scores at 12 months tended to have the worst scores throughout the study to 36 months.



Waterfall Plots of individual subject XQ scores at 12 months: Bilateral and Unilateral Combined

Individual Patient XQ score at each visit out to 36 months: the graphs shows the XQ scores for each patient grouped together at 12,18, 24 and 36 months and illustrates the consistency in XQ response for each patient over 3 years.

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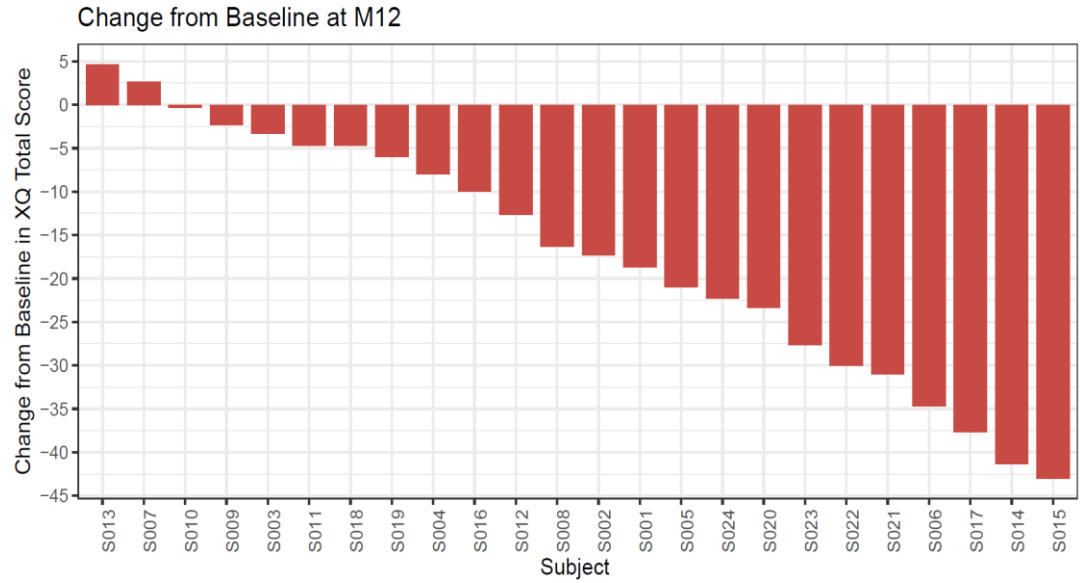
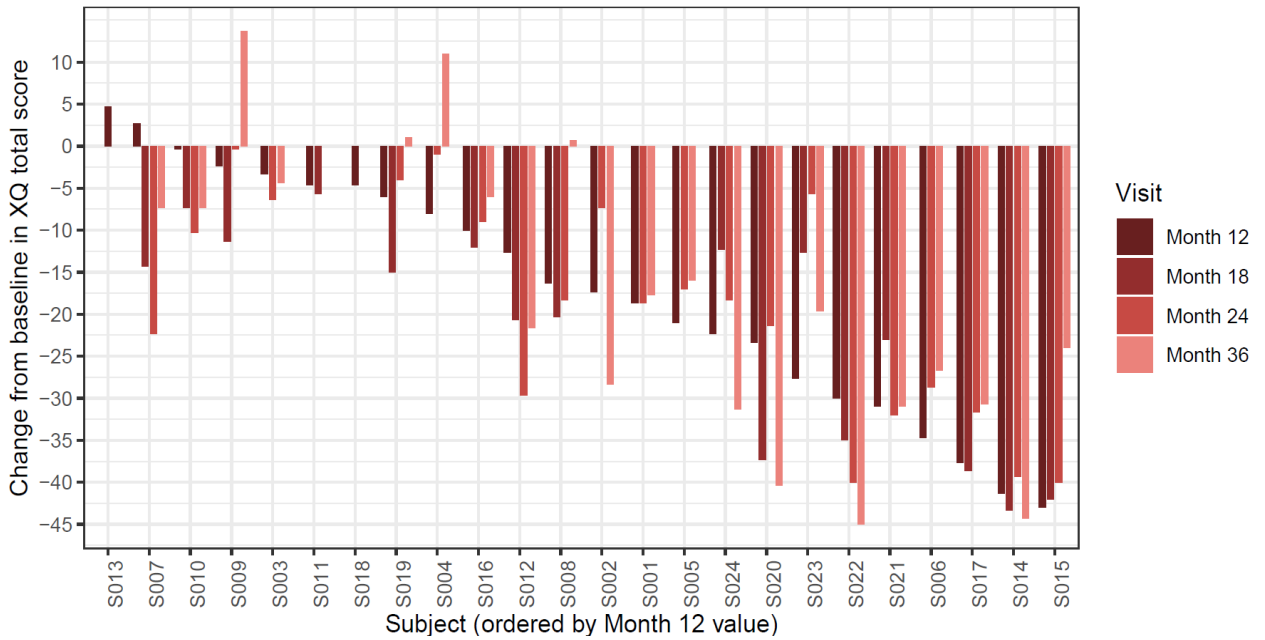
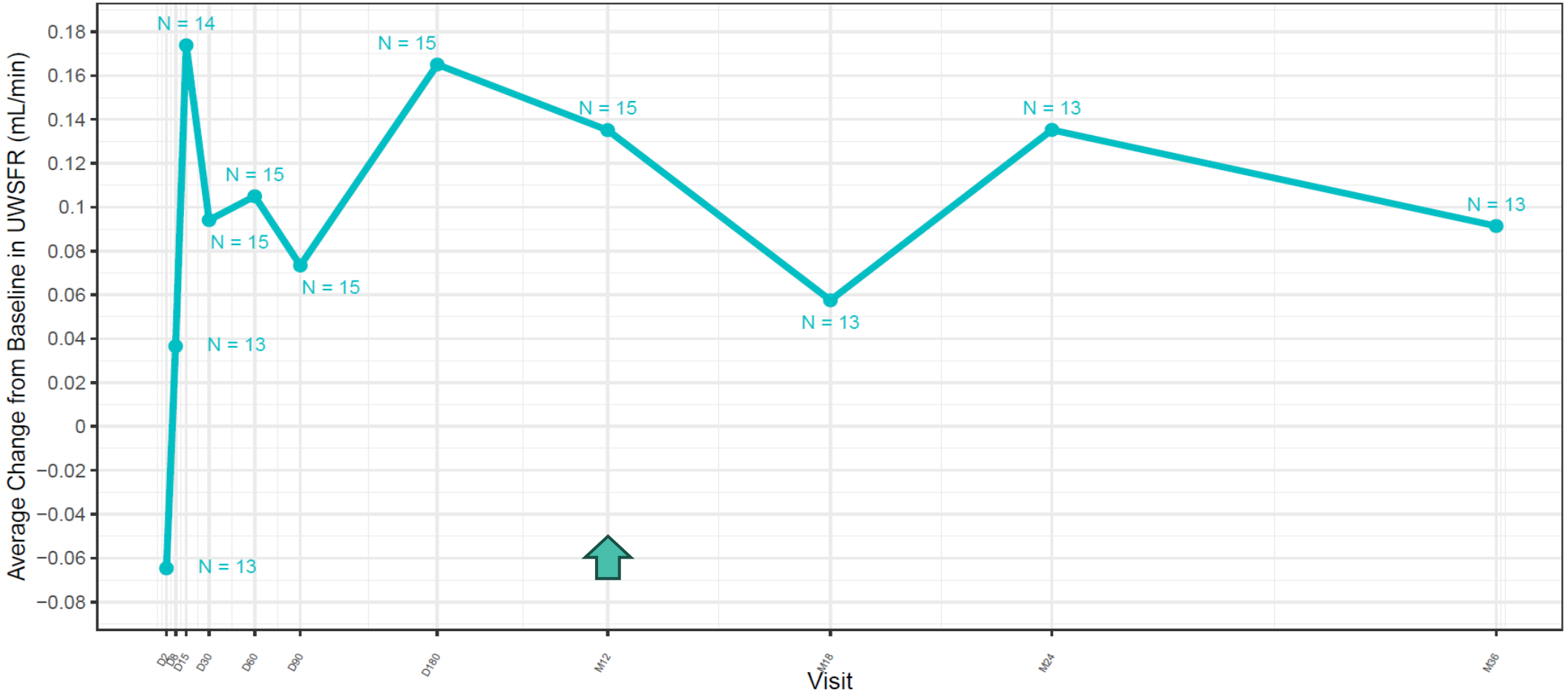




Unstimulated Whole Saliva Flow Rate (UWSFR) : Average Change from Baseline out to Month 36 in all treated patients

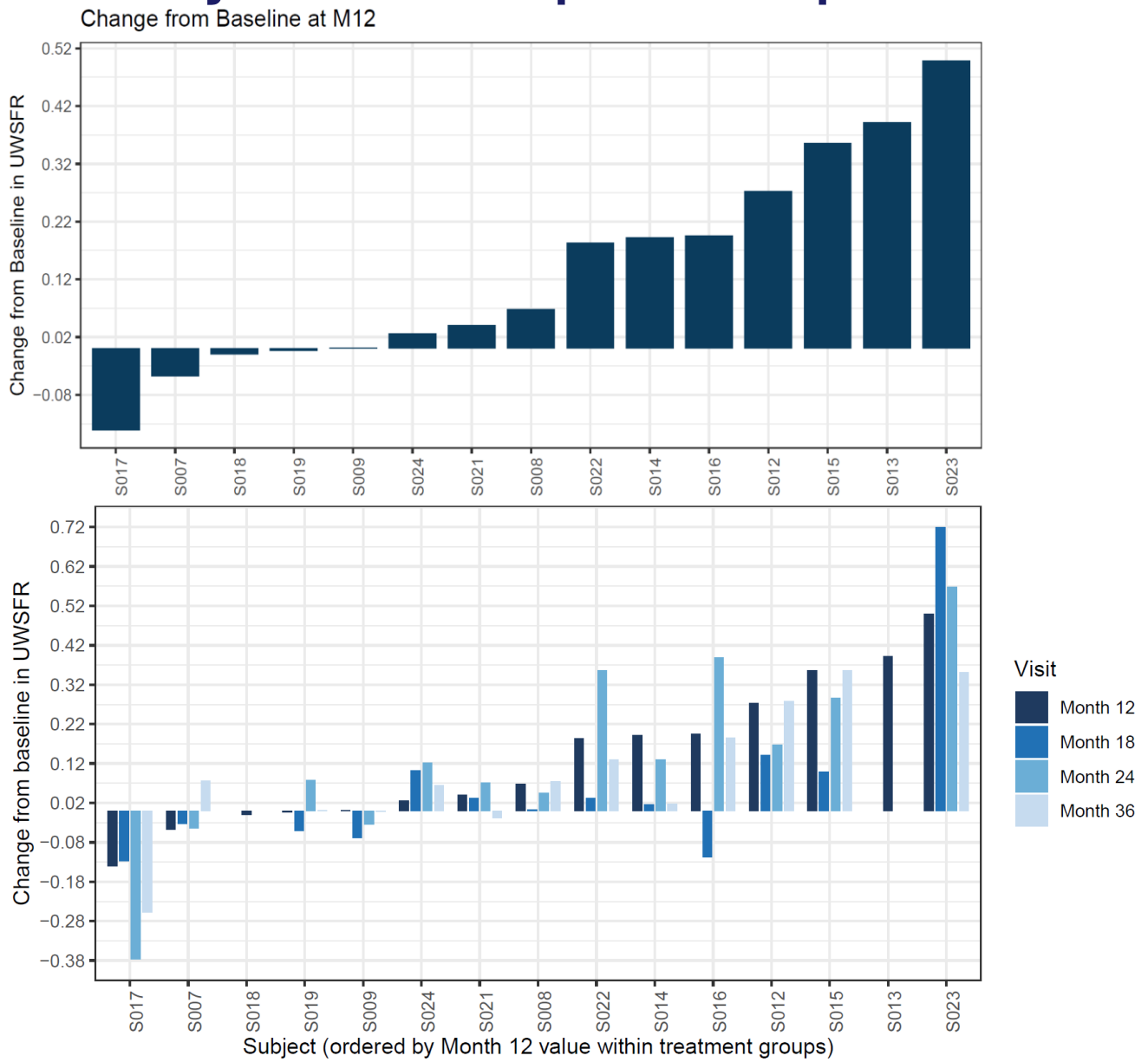


Visit	Baseline	Day 2	Day 8	Day 15	Day 30	Day 60	Day 90	Day 180	Month 12	Month 18	Month 24	Month 36
N_Overall	15	13	13	14	15	15	15	15	15	13	13	13
N_Bilateral	11	11	10	10	11	11	11	11	11	10	10	10
N_Unilateral	4	2	3	4	4	4	4	4	4	3	3	3

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

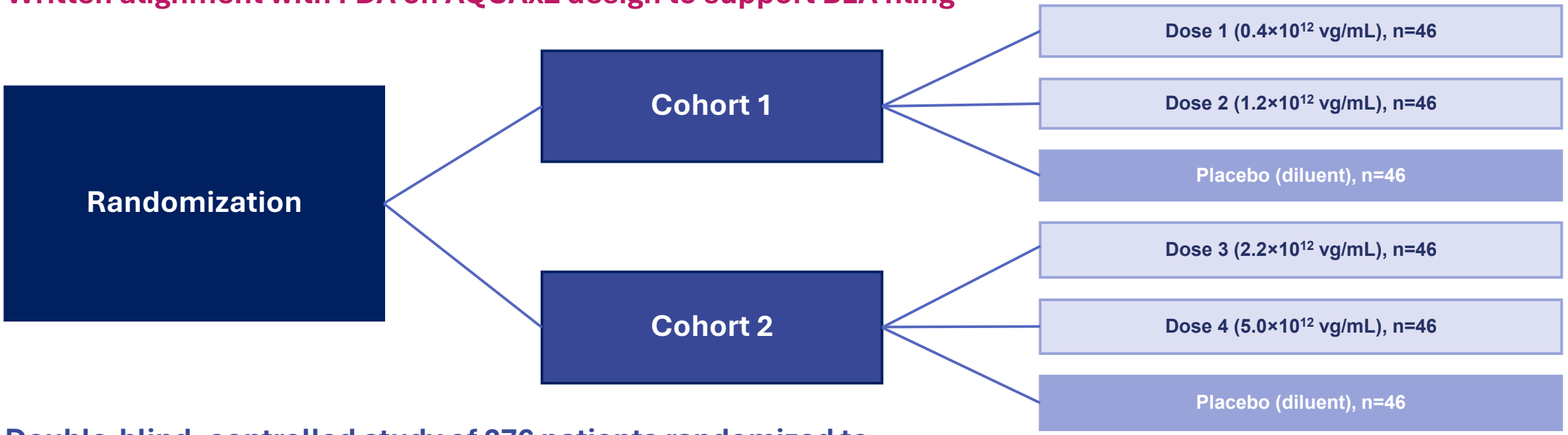
- **Individual Patient UWSFR score at each visit out to 36 months:** the graphs shows the UWSFR for each patient r at 12,18, 24 and 36 months and illustrates the consistency in response for each patient over 3 years.

- 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.



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

Study Investigator Discussion of Disease Burden, Patient Experience, and Treatment Administration

David Owens, MBCHB, FRCS, MPHIL, PGDME, FFST(Ed)

Consultant Otolaryngologist,
University Hospital of Wales, Cardiff, UK.

David Owens: Impact of Radiation-induced Xerostomia and Limitations of Current Therapies

Severe Clinical Consequences

Grade 2/3 radiation-induced xerostomia causes constant dryness, pain, sores, swallowing difficulties, and altered taste.

- Lifelong condition with profound impact on both patient and caregiver
- Severely affects eating, swallowing, speaking, taste, sleep, and social interaction

Negative Health Effects

This condition can cause weight loss & frailty, malnutrition, communication difficulties, and emotional distress

Dental Complications

Loss of saliva's antimicrobial protection accelerates dental decay, complicating patient management

Limitations of Current Therapies

- Gels and washes provide minimal, short-lived symptomatic relief
- Currently, there are no effective disease-modifying therapies

Clinical Experience, Feasibility, and Implications for Practice

Clinical Trial Outcomes

AQUAx Phase 1 trial showed promising efficacy, safety, and long-term durable benefits for patients.

Clinical Integration

Procedure fits existing clinical skills, requires minimal training, standard outpatient equipment, and is quick.

- Procedure aligns with existing skills of: ENT Surgeons, Oral medicine specialists and oral and maxillofacial surgeons
- No operating theatre or complex infrastructure procedure performed in a standard outpatient clinic chair
- Bilateral treatment in <1hr enabling routine clinic integration
- Multiple procedures a day possible

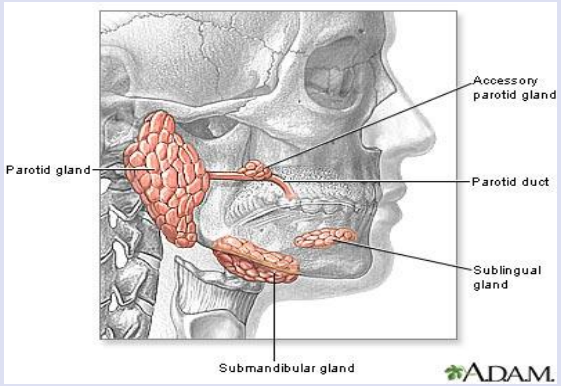
Potential to change standard of care

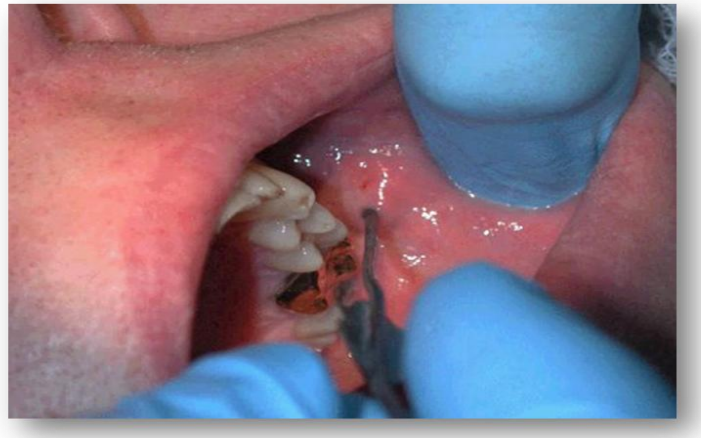
AAV-hAQP1 therapy could become the standard care for moderate-severe xerostomia in head and neck cancer survivors.

- One-time treatment with high tolerability
- Durable clinical benefit and ease of administration

Minimally Invasive Procedure

Retrograde intraductal delivery of AAV-hAQP1 is straightforward, tolerable, and typically pain-free for patients.





Study Investigator discussion of disease burden, patient experience, and treatment administration

Michael Brennan, DDS, MHS

Professor and Chair, Department of Oral Medicine/Oral & Maxillofacial Surgery, Wake Forest University School of Medicine

Atrium Health Carolinas Medical Center, Charlotte, NC

Dr Brennan: Transformative, Disease-Modifying Potential for A Serious, Lifelong Debilitating Disorder

Debilitating Side Effect of Radiotherapy

Radiation-induced xerostomia occurs in virtually all patients receiving radiation treatment for head and neck cancer

- In 30-40% of patients, xerostomia persists as a lifelong condition
- Severely affects eating, swallowing, speaking, taste, sleep, and social interaction

Meaningful Decline in Overall Health and Well-being

- Patients suffer from oral infections, uncontrolled dental caries, pain & mouth sores, and difficulty speaking
- Difficulty eating and swallowing result in nutritional challenges and the potential need for a feeding tube
- Choking because of faster breathing can limit exercise
- Social Isolation

Lifelong Burdensome Consequence of Successful Treatment for Cancer

- Despite prevalence and significant impact on patients lives, there is no effective treatment

One-Time Gene Therapy Targeting the Root Cause of the Disorder

AAV-hAQP1 designed to restore activity within salivary tissue

- Re-establishes function at a biological level
- Meaningful shift in therapeutic intent

Meaningful Improvement

- Dramatic, consistent, and clinically meaningful improvements in daily functioning
- Patients are able to eat, speak, exercise, and re-engage socially

Transformative Observed Benefits

- Durable responses last > 3 years after treatment with AAV-hAQP1
- Persistence of the transformative benefits from changes to the underlying biology of the condition
- Benefits are transformative, durable and potentially disease modifying

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Global Survey of Physicians and Payors with 3 year durability data

Commercial Opportunity

Clinicians view the strong response rates, curative treatment, and durability of effect as highly meaningful in this unmet need

Clinician Preference Share: ~78% global adoption based on clinician preference

Clinician-stated preference share weighted by patient volume

~78%

*Overstatement-adjusted preference share**

~52%

Physicians highlight:

- **Transformative benefit in meaningful endpoints:** Both PRO and water flow impact unprecedented
- **Minimally invasive onetime** dosing fits easily into clinical practice
- **Strong 3-year durability** seen as evidence of possibly permanent response
- **Good safety profile**
- Viewed as **a simple, one-time treatment leading to a disease modifying effect on a severe, otherwise untreatable lifelong condition.**

*"… **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.)*

*"**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"

Late moderate to severe RIX: A completely unmet need and large potential market

Peak global revenue is estimated to be ~$3.7B, with a steady state of ~$3.2B

Clinician perspectives	• **Strong responses in meaningful measures:** PRO and salivary flow, **long-lasting benefit** following a **simple one-time treatment** • HCPs view AAV-AQP1 as a **highly differentiated, disease-modifying treatment** • **Physician-stated preference share** is **~78%** and leading to **~ 52%** usage with overstatement adjustment
Epidemiology and disease landscape	• **Persistent RIX** three or more years post radiation treatment is **severe, lifelong disorder** with no effective treatment • **Prevalence:** 165k US only ; 435k Global • **Annual Incidence:** ~20K US only ; ~ 48k Global
Payer perspectives	• U.S. list price of **~$150K** and **15-20% GTN** • Market access coverage is estimated at **~90%**
Projected RIX revenue	• **Global potential net revenue**: **peak at ~$3.7B** reaching a **steady state of ~$3.2B** in the late 2030s • **U.S. net revenue**: **peak at ~$2.0B** reaching a **steady state of ~$1.8B** in the late 2030s • **Cumulative treated globally:** 250K/730k, **32%-37%** over 10 years

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Source: Primary market research commissioned by MeiraGTx

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

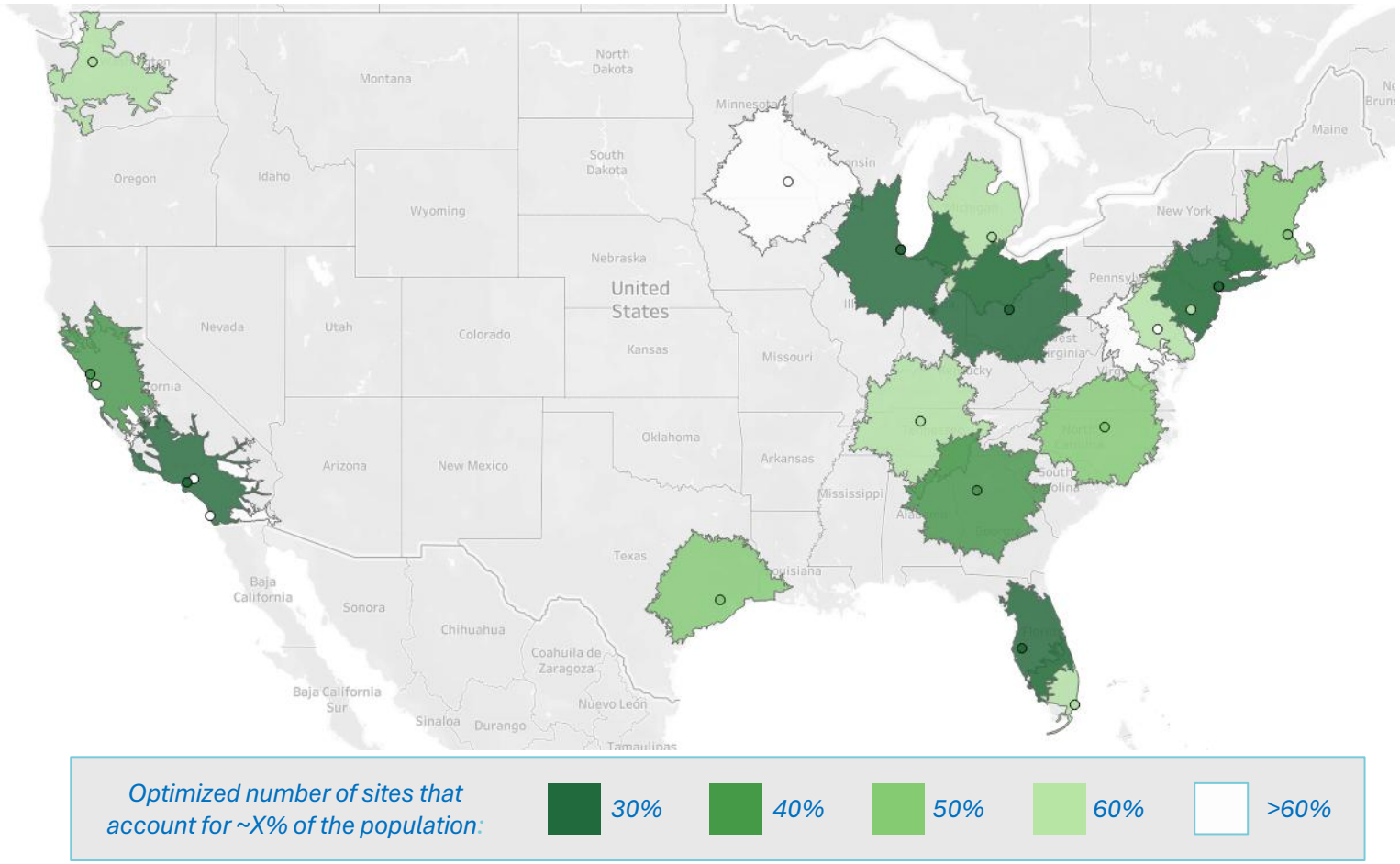
U.S. Population and HNC hospitals

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: Program highlights

AAV-AQP1 has the potential to become the standard of care for patients with late, grade 2/3 radiation-induced xerostomia based on its disease-modifying mechanism and meaningful improvements in both 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 improvement 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 study data and BLA filing expected in Q2 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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