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

December 17, 2020

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Agenda

AAV-hAQP1 Program Overview

Zandy Forbes, PhD

President & CEO MeiraGTx

Radiation-Induced Xerostomia: Current Treatment and Unmet Need

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AAV-hAQP1 Clinical Development Program: Next Steps

Robert K. Zeldin, MD

Chief Medical Officer MeiraGTx

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Salivary Gland Gene Therapy

Radiation-Induced Xerostomia

Radiation-Induced Xerostomia (RIX):
A Condition with a High Unmet Medical Need

Target Indication: Treatment of Xerostomia persisting >2 years after radiation therapy for head and neck cancer

- 85% of radiation-treated patients experience reduced saliva production, of whom 40% have persistent Grade 2/3 RIX[1]
- >170,000 existing patients in the US (orphan drug designation)[2]
- 58,000 new cases of head and neck cancer per year in the US; 650,000 worldwide[3]
- Serious, debilitating complications as a result of reduced saliva
  - Lack of lubrication
  - Loss of antimicrobial and antifungal effects of saliva
  - Negative impact on patient quality of life
- Current treatment options for this serious condition are limited

[1]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.

[2]Cox J.D., *et al.* (1995). Toxicity criteria of the Radiation Therapy Oncology Group (RTOG) and the European Organization for Research and Treatment for Cancer (EORTC). *Int. J. Radiation Oncology Biol. Phys.* 31(5):1341-1346.

[3] Bray F, Ferlay J, Soerjomataram I, et al. Global cancer statistics 2018: GLOBOCAN estimates of incidence and mortality worldwide for 36 cancers in 185 countries. CA Cancer J Clin 2018; 68:394.

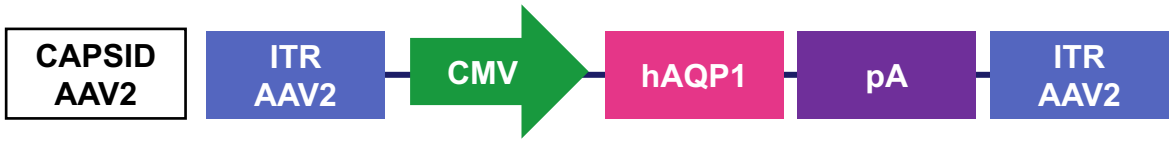
AAV-hAQP1 for Radiation-Induced Xerostomia (RIX)

Strategy for the repair

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

Salivary gland as target for gene therapy

- Non-invasive: allows local administration and avoids systemic exposure
- Isolated and encapsulated
- Small volume of vector



- **Potential Additional Indications**
  - Sjogren's Syndrome – dry mouth and dry eye
  - Dry Eye

Xerostomia: MGT016 Phase 1 AQUAx Study

Study Design

- Open label, multi-center, dose escalation study of a single administration of AAV-hAQP1 to one parotid gland in patients with radiation-induced parotid salivary hypofunction and xerostomia

Target Enrollment: Up to 30 subjects

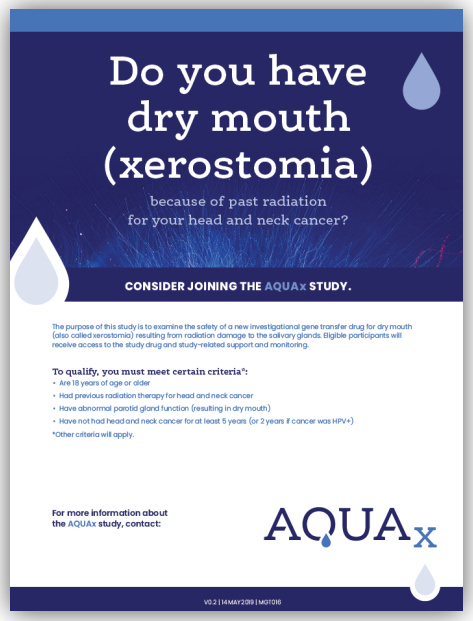
- Four dose cohorts with minimum of 3 subjects per cohort
- May treat up to 9 subjects in dose expansion cohorts
- 5 centers (4 in US, 1 in Canada)
- All subjects to be followed for 1-year post-treatment

Primary Endpoint

- Safety

Secondary Endpoints

- Patient reported measures of xerostomia symptoms
- Unstimulated and stimulated salivary volume



MGT016 Phase 1 AQUAx Study

Study Status

- 2 centers currently open for enrollment
- All 5 centers to be open by 1Q 2021
- Cohort 2 recruitment ongoing

Cohort 1 (n=3 subjects) completed treatment

- Treatment well tolerated
- No dose limiting toxicity
- No serious adverse events
- Improvements observed in patient reported assessments and measures of salivary volume output
- Complete symptom resolution in the subject who has reached the 12-month timepoint

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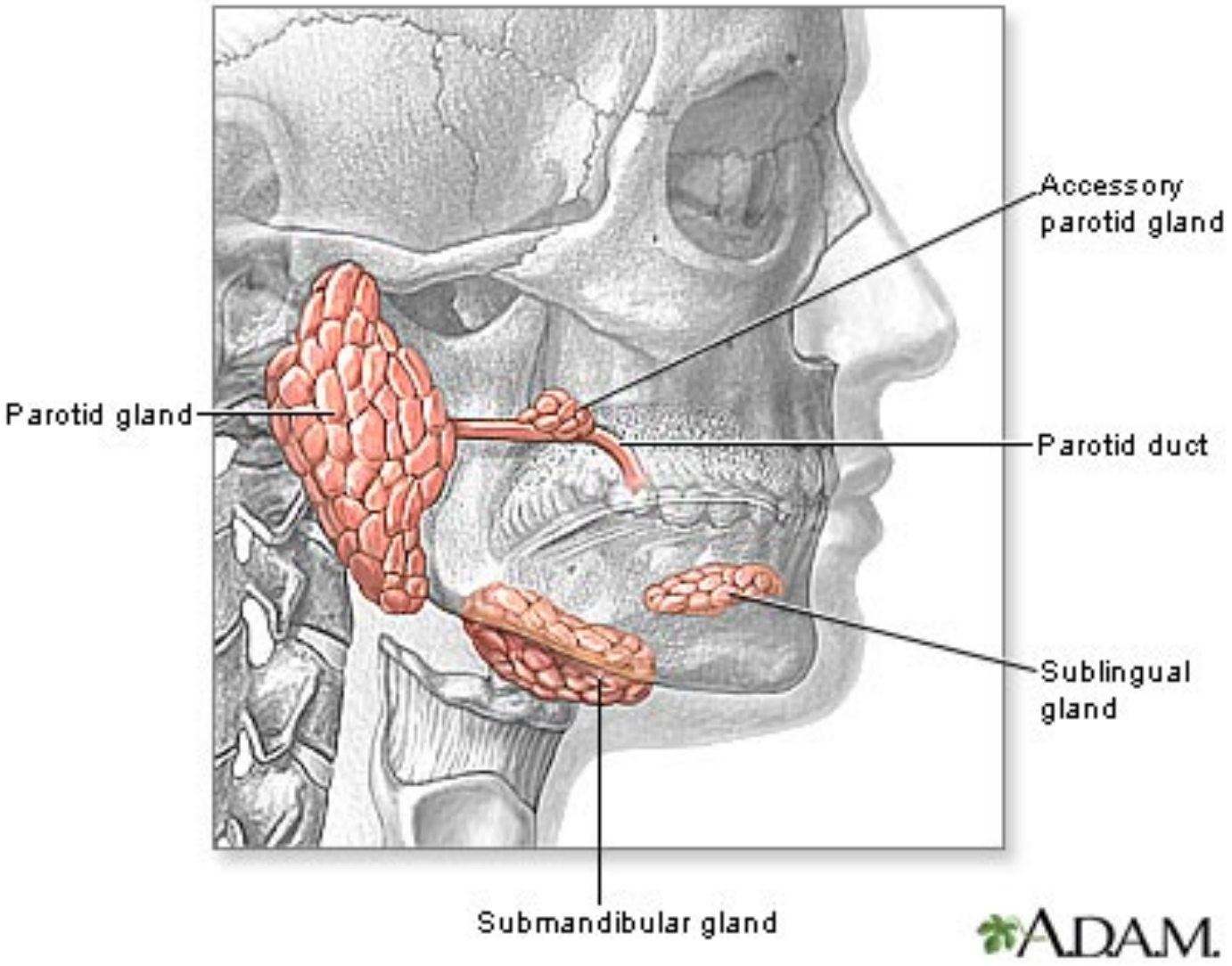
Clinical Perspective

Michael Brennan, DDS, MHS, FDS RCSEd

Salivary Glands



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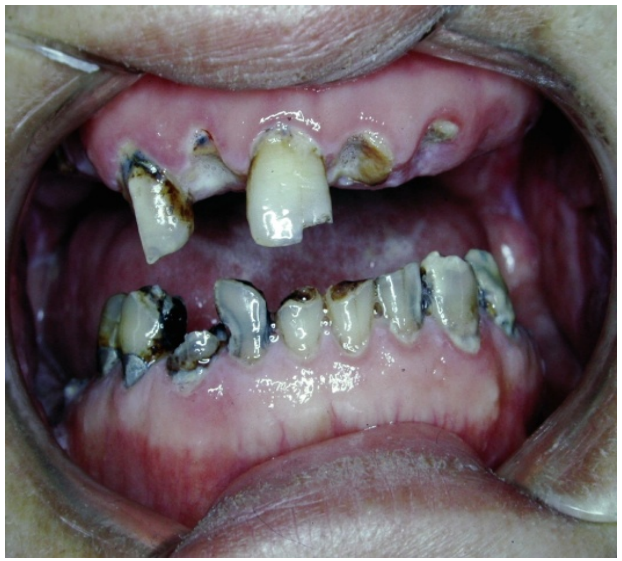
Radiation-Induced Xerostomia

Xerostomia (Dry Mouth)

- One of the most common complications of treatment for head and neck cancer
- Progressive, irreversible, significantly impairs quality of life of potentially cured cancer patients
- Changes in quantity and quality of saliva occur, impacting lubrication, cleansing, antimicrobial effect, digestion and taste
- Often leads to severe and lasting oral issues

Clinical Signs and Symptoms

- Dryness of mouth and lips make it difficult to eat, chew, swallow
- Sore throat and changes in vocal quality
- Burning present in 40% of patients with dry mouth[1]
- Unable to wear/tolerate dentures
- Increased risk of dental cavities and tooth loss
- Increased risk of fungal infection
- Taste changes - decreased or food tastes metallic/salty

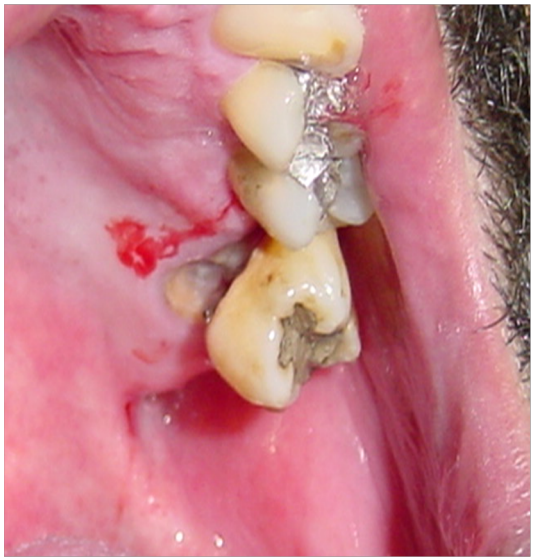


[1]Rouleau, Tanya S. et al, A retrospective, cohort study of the prevalence and risk factors of oral burning in patients with dry mouth Oral Surg Oral Med Oral Pathol Oral Radiol Endod 2011;111:720-725

Limitations in Current Management of Xerostomia

Current treatment options

- Over the counter mechanical and gustatory stimulants
  - Not all patients tolerate frequent gum chewing
  - May exacerbate temporomandibular disorder symptoms
- Parasympathomimetics
  - Cevimeline and Pilocarpine
  - Not well tolerated
    - Side effects - flushing, upset stomach, sweating
    - Ineffective in addressing lower salivary function
- Saliva substitutes
  - Carboxymethyl cellulose and mucin
  - Short term benefit



Current options do not address symptoms of reduced salivary output

Physician and Patient Experience with AAV-hAQP1

AQUAx 1st patient cohort (n=3)

- Administration of AAV-hAQP1
  - Non-invasive procedure
  - Easy to perform
  - Well tolerated by patients
- No serious adverse events
- Improvements in patient reported quality of life measures
  - Less pain
  - Less burning
  - Better sleep
  - Fewer throat symptoms
- Increase in salivary output
- At 12-months the first patient saw a complete resolution of symptoms

AAV-hAQP1 Clinical Development Program: Next Steps

Robert K. Zeldin, MD

Phase 2 Study: Design & Efficacy Endpoints

Design

- Double-blind, sham-control study
- Two active doses of AAV-hAQP1
- Doses, sample size, and timing based on Phase 1 results

Efficacy Endpoints

- Patient reported measure of xerostomia symptoms
- Salivary output

Phase 2 Study: Inclusion & Exclusion Criteria

Inclusion Criteria

- At least 18 years of age
- History of radiation therapy for head and neck cancer
- Grade 2 (moderate) or Grade 3 (severe) Xerostomia
- Accessible Stensen's duct
- No evidence of recurrence of primary malignancy

Exclusion Criteria

- History of salivary gland malignancy
- History of a systemic autoimmune disease affecting the salivary glands

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Audience Q&A