



## MeiraGTx To Present Clinical Program Update for AAV-hAQP1 Treatment of Radiation-Induced Xerostomia

December 16, 2020

**Webcast and conference call to take place on December 17, 2020 at 8:00 a.m. ET**

LONDON and NEW YORK, Dec. 16, 2020 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced the Company will host a webcast and conference call to provide an update on the Company's AAV-hAQP1 clinical program for the treatment of radiation-induced xerostomia.

The webcast will include discussion of the first cohort of patients (n=3) treated in the Company's Phase 1 AQUAx clinical trial. Of the three patients treated in Cohort 1, one patient has reached the 12-month assessment and two have passed the six-month assessment. In these patients, the investigational gene therapy AAV-hAQP1 has been well tolerated with no dose limiting toxicity and no serious adverse events reported. Encouraging responses have been seen in both patient-reported measures of xerostomia symptoms and in salivary output in the patients treated in Cohort 1, with complete resolution of symptoms in the patient who has reached the 12-month timepoint. AQUAx trial investigator Dr. Michael Brennan, DDS, from Atrium Health's Carolinas Center for Oral Health, will speak about his experience treating patients with radiation-induced xerostomia, and discuss the encouraging results he has observed in the patients he has treated to date in the AQUAx trial.

Details of the webcast are listed below:

**Title:** MeiraGTx Xerostomia Clinical Program Update

**Presenters:**

- Alexandria Forbes, Ph.D., President and CEO of MeiraGTx
- Michael Brennan, DDS, MHS, FDS RCSEd, Director of the Sjögren's Syndrome and Salivary Disorders Center, Atrium Health's Carolinas Center for Oral Health
- Robert K. Zeldin, M.D., Chief Medical Officer of MeiraGTx

**Date:** Thursday, December 17, 2020

**Time:** 8:00 a.m. ET

A live webcast will be available on the Investors page of the Company's website at <https://investors.meiragtx.com/events-presentations>. Please connect at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call 1 (866) 796-1272 (U.S.) or 1 (409) 937-8924 (International) to listen to the live conference call. The conference ID number for the live call is 8582943. A replay of the webcast and accompanying presentation materials will be available on the Company's website for 30 days following the live conference call.

### About Xerostomia

Xerostomia is a chronic and debilitating disorder of the salivary glands in which saliva production is impaired. Xerostomia has a number of causes, including radiation therapy for head and neck cancer and certain autoimmune diseases. In the U.S., there are more than 170,000 patients with chronic grade 2/3 radiation-induced xerostomia. In these patients, reduced salivary output results in a lack of lubrication and a loss of the antimicrobial and antifungal properties of saliva with consequent morbidities and significant negative impact on patient quality of life. Current options for the management of xerostomia are few and are of limited benefit so there is a high unmet medical need for a safe and effective treatment.

### About the Phase 1 AQUAx Clinical Trial

The Phase 1 AQUAx clinical trial is an open-label, non-randomized, dose escalation trial designed to evaluate the safety of MeiraGTx's investigational gene therapy AAV-hAQP1 when administered via Stensen's duct to a single parotid gland in patients who have been diagnosed with grade 2 or 3 radiation-induced xerostomia and who have remained cancer free for at least five years (or at least two years if HPV+) after receiving radiation treatment for head and neck cancer. Primary endpoint of the trial is safety, with efficacy endpoints including the evaluation of the change in parotid gland salivary output after treatment with AAV-hAQP1 and patient reported measures of xerostomia symptoms.

### About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical stage gene therapy company with six programs in clinical development and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, as well as a potentially transformative gene regulation technology. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: inherited retinal diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system and salivary gland, MeiraGTx intends to expand its focus in the future to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

For more information, please visit [www.meiragtx.com](http://www.meiragtx.com).

### Forward Looking Statement

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements

contained in this press release 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 and anticipated milestones regarding our clinical data and reporting of such data and the timing of results of data, including in light of the COVID-19 pandemic, as well as statements that include the words “expect,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “should,” “anticipate” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, raise additional capital, identify additional and develop existing product candidates, successfully execute strategic 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 designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of the COVID-19 pandemic 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, 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](http://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 press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. 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 press release.

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