MeiraGTx Announces Positive Three-year Data from the Phase 1 AQUAx Clinical Study of AAV-hAQP1 for the Treatment of Grade 2/3 Late Radiation-Induced Xerostomia

April 16, 2026

- *Clinically meaningful improvements in xerostomia symptoms measured by PRO Xerostomia Questionnaire (XQ) maintained out to 3 years post treatment with AAV-hAQP1 for both bilateral and unilateral cohorts*
- *Increases in Unstimulated Whole Saliva Flow Rate (UWSFR) maintained out to 3 years*
- *AAV-hAQP1 was safe and well tolerated at each dose tested*
- *AAV-hAQP1 Data presentation webcast to be held today, April 16, 2026, at 8:00 a.m. ET*

LONDON and NEW YORK, April 16, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced positive three-year data from the completed Phase 1 AQUAx study of AAV-hAQP1 for the treatment of grade 2/3 late radiation-induced xerostomia (RIX).

“Today we are very pleased to be releasing 3-year data from the Phase 1 AQUAx study which includes the full 36-month data from all cohorts,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “The 3-year data shows remarkable maintenance of the unprecedented improvements seen in response to AAV-hAQP1 treatment at 12 months. XQ responses are maintained over 3 years as is unstimulated saliva flow – the objective measure of the therapy’s mechanism of action. The durability we are seeing with AAV-hAQP1 is extraordinary, particularly following the simple one-time treatment. In addition to the durability of the population data over three years, the consistency of responses within individual patients is also encouraging. This three-year durability data is changing the way AAV-hAQP1 treatment is viewed by physicians, as it is now being regarded as a simple one-time treatment that has disease modifying impact on patients with this lifelong, severely debilitating condition that has no other treatments.”

Dr. Forbes continued, “Persistent radiation induced xerostomia is a severe unmet need with a large, well defined patient population, strong physician enthusiasm, and a simple one-time in-office procedure. We believe AAV-hAQP1 has the potential to be clinically transformative and a very meaningful commercial opportunity.”

MeiraGTx’s conference call and webcast details are as follows:

- Thursday, April 16, 2026, at 8:00 a.m. ET.
- To register and attend the event, please click here

A live webcast of the event, as well as a replay, will be available on the Investors page of the Company’s website at www.investors.meiragtx.com/.

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 one or both parotid glands 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 patient-reported measures of xerostomia symptoms and the evaluation of the change in salivary output after treatment with AAV-hAQP1. Patients treated in the Phase 1 AQUAx study are followed for 5 years after the one-time administration of AAV-hAQP1.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline with four late-stage clinical programs. Each of these programs uses local delivery of small doses, resulting in disease-modifying effects in both inherited and more common diseases, in the eye, Parkinson’s disease, and radiation-induced xerostomia. MeiraGTx uses its innovative technology in optimization of capsids, promoters, and novel translational control elements to develop best-in-class, potent, safe viral vectors. MeiraGTx’s broad pipeline is supported by end-to-end in-house manufacturing. MeiraGTx has built the most comprehensive manufacturing capabilities in the industry, including two that are licensed for GMP viral vector production and a GMP QC facility with clinical and commercial licensure. In addition, MeiraGTx has developed a proprietary manufacturing platform process over 9 years based on more than 20 different viral vectors with leading yield and quality aspects and commercial readiness. Uniquely, MeiraGTx has developed a novel technology for in vivo delivery of any biologic therapeutic using oral small molecules. This transformative riboswitch gene regulation technology allows precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the regulated in vivo delivery of metabolic peptides, including GLP-1, GIP, Glucagon, Amylin, PYY, and Leptin, as well as cell therapy, CAR-T for liquid and solid tumors and autoimmune diseases, and additionally, PNS targets addressing long-term intractable pain. MeiraGTx has developed the technology to apply genetic medicine to common diseases, increasing efficacy,

addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

For more information, please visit 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 our product candidate development and anticipated milestones regarding our pre-clinical and clinical data, 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 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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