MeiraGTx Announces Oral Presentation at the 2024 American Academy of Oral Medicine (AAOM) Annual Conference

April 18, 2024

LONDON and NEW YORK, April 18, 2024 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced the Company gave an oral presentation at the American Academy of Oral Medicine Annual Conference, being held from April 16-20, 2024, at the Hyatt Regency Grand Cypress in Orlando, FL.

The details of the oral presentation are below:

Session: Oral Abstract Session I
Presentation ID #196
Title: Results of a Phase 1, Open-label, Dose-escalation Study of Gene Therapy with AAV2-hAQP1 as Treatment for Grade 2 and 3 Radiation-induced Late Xerostomia and Parotid Gland Hypofunction – The AQUAx Study
Presenting Author: Dr. Michael Brennan
Time: 4:20pm ET

Abstract:
Results of a Phase 1, Open-label, Dose-escalation Study of Gene Therapy with AAV2-hAQP1 as Treatment for Grade 2 and 3 Radiation-induced Late Xerostomia and Parotid Gland Hypofunction

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Objectives
Grade 2/3 late xerostomia is a chronic, debilitating complication of radiotherapy for head and neck cancers. We assessed the safety and efficacy of AAV2-hAQP1 gene therapy as a treatment for this condition.

Methods
Twenty-four participants with Grade 2/3 xerostomia at least five years after completing radiotherapy (2 years if HPV+) were enrolled in this multi-center, open-label, dose-escalation study. AAV2-hAQP1 was delivered to the parotid gland(s) via cannulation of Stensen's duct. Twelve participants received AAV2-hAQP1 in one gland and 12 in both glands. Participants were followed for 12 months post-treatment.

Safety parameters included adverse events, physical examinations, laboratory tests, and electrocardiograms. Efficacy assessments included the Xerostomia-specific Questionnaire (XQ), MD Anderson Symptom Inventory-Head and Neck Module (MDASI-HN), Global Rate of Change Questionnaire (GRCQ), and measurement of unstimulated and stimulated whole saliva flow rates (UWSFR, SWSFR).

Results
No treatment-related serious adverse events or dose-limiting toxicities were reported, and all participants completed the study.

Statistically significant improvements were seen in the patient-reported outcome (PRO) instruments by Day 30 and were maintained through Month 12, with greater improvement in the bilateral versus unilateral cohorts.

At Month 12, the mean percent change from baseline (%CFB) was -39.5% and -42.2% for the XQ Total Score and the Dry Mouth question of the MDASI-HN, respectively, and the mean GRCQ symptom score was 3.8. Overall, 16/24 participants reported an improvement of ≥8 points in the XQ Total Score, and 19/24 participants reported important improvements in xerostomia symptoms based on the GRCQ. The improvement reported across PRO instruments, measuring different aspects of xerostomia symptoms, provides compelling evidence of treatment effectiveness.

The mean %CFB in UWSFR at Month 12 was 112.5%, and a trend toward improved SWSFR was observed.

Conclusion
Treatment with AAV2-hAQP1 was safe and well-tolerated at all doses and resulted in meaningful improvements in xerostomia symptoms and unstimulated whole saliva flow rate.

The presentation will be available on the Posters and Publications page of the Company's website after the respective presentation session has concluded.

About MeiraGTx
MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage gene therapy company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has an internally developed manufacturing platform process, internal plasmid production for GMP, two GMP viral vector production facilities as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. MeiraGTx has core capabilities in viral vector design and optimization and a potentially transformative riboswitch gene regulation platform technology that allows for the precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the

riboswitch platform on delivery of metabolic peptides including GLP-1, GIP, Glucagon and PYY using oral small molecules, as well as cell therapy for oncology and autoimmune diseases. Although initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx has developed the technology to apply genetic medicine to more common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains great.

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 our pre-clinical and clinical data and reporting of such data and the timing of results of data, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “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, 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 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, 2023, 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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