MeiraGTx to Present 12-month Data from All Cohorts of the Completed Phase 1 AQUAx Clinical Study and an Update on the Company's Recently Initiated Phase 2 Study of AAV-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia on Tuesday, June 27,

June 22, 2023

LONDON and NEW YORK, June 22, 2023 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ: MGTX), a vertically integrated, clinical stage gene therapy company, today announced it will host a conference call and webcast to present an update on the Company's AAV-hAQP1 clinical program for the treatment of grade 2/3 radiation-induced xerostomia (RIX) on Tuesday, June 27, 2023, at 8:00 a.m. ET.

The presentation will include:

1) Data from the completed Phase 1 AQUAx clinical study for the 24 patients treated with AAV-hAQP1 in the unilateral and bilateral cohorts:

- Safety and tolerability
- 12-month data for PRO assessments of xerostomia symptoms in the unilateral cohorts (n=12)
- 12-month data for PRO assessments of xerostomia symptoms in the bilateral cohorts (n=12)
- Change over time in the objective measure of saliva flow out to 12 months for both bilateral and unilateral cohorts
- Long-term follow up data to 2 or 3 years for the participants who have reached those timepoints

2) Summary of biopsy data from a subset of patients in the Phase 1 National Institutes of Health (NIH) study of AAV-hAQP1 in RIX demonstrating parotid transduction and durability out to at least 24-months post-treatment.

3) Overview of the recently initiated randomized, double blind, placebo-controlled Phase 2 study of AAV-hAQP1 for the treatment of RIX, now enrolling patients.

A question-and-answer session will follow the formal presentation.

To register and attend the event, please click here.

A live webcast of the call, 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 parotid gland salivary output after treatment with AAV-hAQP1.

About the Phase 1 NIH Study
The Phase 1 NIH study is an open-label, dose-escalation study evaluating the safety of a single administration of an adeno-associated virus vector encoding human aquaporin-1 to one parotid salivary gland in individuals with irradiation-induced parotid salivary hypofunction.

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, and a transformative gene regulation platform technology which allows tight, dose responsive control of gene expression by oral small molecules with dynamic range that can exceed 5000-fold. 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: ocular, including inherited retinal diseases and large degenerative ocular diseases, neurodegenerative diseases, and severe forms of xerostomia. Though initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx plans to expand its focus to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

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 the development of AAV-hAQP1 and our 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 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 most recent quarterly report on Form 10-Q or annual report on Form 10-K or subsequent 8-K reports, as filed with the Securities and Exchange Commission. 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.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com

Media:
Jason Braco, Ph.D.
LifeSci Communications
jbraco@lifescicomms.com