MeiraGTx Announces Upcoming Presentation on Achromatopsia Gene Therapy Candidate AAV-CNGA3 at ARVO 2019

April 25, 2019

LONDON and NEW YORK, April 25, 2019 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced a pre-clinical poster on safety and efficacy of the Company's gene therapy product candidate AAV-CNGA3 for the treatment of patients with achromatopsia (ACHM) due to mutations in the *CNGA3* gene will be presented at the Association for Research in Vision and Ophthalmology (ARVO) 2019 Annual Meeting in Vancouver, British Columbia.

Details of MeiraGTx's poster are as follows:

Title: Development and efficacy assessment of AAV2/8-hG1.7p.coCNGA3, a *CNGA3* gene therapy vector
Abstract Number: 3426 - A0197
Session Name: Gene therapy for ocular disorders
Type: Poster
Date: Tuesday, April 30, 2019, 11:45am – 1:30pm PT (2:45pm – 4:30pm ET)
Location: West Exhibition Hall

Data Summary: AAV-CNGA3 utilizes a proprietary engineered promoter (hG1.7p) that was associated with strong, pan-cone gene expression in induced pluripotent stem cell (iPSC)-derived retinal organoids driving a codon-optimized CNGA3 cDNA. In pre-clinical models, treatment with AAV-CNGA3 resulted in long-term visual improvements and cone photoreceptor survival at titers planned for use in a Phase 1/2 clinical trial of AAV-CNGA3.

About AAV-CNGA3
AAV-CNGA3 is a gene therapy treatment being developed as a potential treatment for patients with achromatopsia (ACHM) due to mutations in the *CNGA3* gene. Delivered via subretinal injection to a patient's cone receptors at the back of the eye, AAV-CNGA3 is designed to restore cone function by delivering a normal copy of the *CNGA3* gene. AAV-CNGA3 has been granted orphan drug designation and rare pediatric disease designation by the United States Food and Drug Administration (FDA), as well as orphan drug designation by the European Medicines Agency (EMA).

AAV-CNGA3 is being developed in collaboration with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson.

In 2019, MeiraGTx and Janssen expect to initiate a Phase 1/2 dose escalation study of AAV-CNGA3 in children with ACHM due to mutations in the *CNGA3* gene.

About Achromatopsia
Achromatopsia is an inherited retinal disorder that specifically prevents cone photoreceptors from functioning. ACHM is characterized by severely reduced visual acuity of 20/200 or worse, disabling light sensitivity (photoaversion) and involuntary back and forth eye movements (nystagmus). ACHM occurs in approximately one in 30,000 people in the United States, with 92 percent of cases caused by mutations in CNGB3 and CNGA3 genes. Currently, there are no effective treatments for this disease.

About MeiraGTx
MeiraGTx (NASDAQ:MGTX) is a vertically integrated, clinical stage gene therapy company with five 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 and xerophthalmia. 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.

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 2019 milestones regarding its pre-clinical and clinical data, 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, acquire additional capital, identify additional and develop existing product candidates, continue operating as a going concern, successfully execute strategic priorities, bring product candidates to market, build-out the manufacturing facility 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; 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; 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; 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.

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