MeiraGTx To Present Clinical Data from Phase 1/2 Trial of AAV-RPE65 at Upcoming Scientific Conferences

September 10, 2019

LONDON and NEW YORK, Sept. 10, 2019 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced that clinical data updates from the Company's Phase 1/2 trial of AAV-RPE65 will be presented at two upcoming medical meetings.

The Phase 1/2 open-label, multi-center, dose-finding trial evaluated AAV-RPE65 in patients with retinal dystrophy associated with disease-causing variants in the *RPE65* gene, a condition that causes severe sight impairment beginning at birth. AAV-RPE65 is a second-generation gene therapy candidate developed specifically to treat RPE65-deficiency, and optimized for transduction efficiency, potency and stability. MeiraGTx previously reported positive topline data from this trial demonstrating safety and tolerability, as well as statistically significant improvement across several endpoints assessing clinical activity.

Michel Michaelides, BSc MB BS MD(Res) FRCOphth FACS, trial investigator, Consultant Ophthalmologist, Moorfields Eye Hospital and Professor of Ophthalmology, University College London, will present updated data at the following meetings:

52nd Annual Scientific Meeting of The Retina Society

- Podium Presentation: *Results of a Phase 1/2 Trial of an Optimized Gene Therapy in Adults and Children with Retinal Dystrophy Associated with Bi-allelic Variants in RPE65*
- Session Date and Time: Sunday, September 15, 2019, 11:01 a.m. GMT
- Session Title: Late Breaking Presentations
- Room: Grand Ballroom

Retina Subspecialty Day of the American Academy of Ophthalmology (AAO) 2019 Annual Meeting

- Podium Presentation*: Results of a Phase 1/2 Trial of an Optimized Gene Therapy in Adults and Children with Retinal Dystrophy Associated with Bi-allelic Variants in RPE65*
- Session Date and Time: Saturday, October 12, 2019, 9:17 a.m. PDT
- Session Title: RET15- Section XI: Late Breaking Developments, Part II
- Room: West 3002

MeiraGTx will host a conference call and live webcast following Professor Michaelides' presentation at AAO to review the data from the Phase 1/2 trial.

About AAV-RPE65
AAV-RPE65 is a novel second-generation gene therapy candidate in development for the treatment of patients with RPE65-deficiency, a condition that causes severe sight impairment beginning at birth. Delivered via subretinal injection, AAV-RPE65 is designed to deliver a normal copy of the *RPE65* gene, which is essential for photoreceptor function in the eye. AAV-RPE65 has been granted orphan designation by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of Leber congenital amaurosis (LCA) caused by disease-causing variants in the *RPE65* gene. The FDA has also granted AAV-RPE65 rare pediatric disease designation for the treatment of inherited retinal dystrophy due to biallelic RPE65 disease-causing variants.

About RPE65-Deficiency
RPE65-deficiency is a rare, genetic disorder caused by disease-causing variants in the *RPE65* gene. Due to rod photoreceptor dysfunction, RPE65-deficiency causes impaired vision from birth and results in the degeneration of the entire retina over time. Most RPE65-deficient patients experience poor vision in low-light conditions from a young age and suffer from central vision loss that progresses to complete blindness by early adulthood.
RPE65-deficiency is often characterized as a specific subtype of LCA caused by disease-causing variants in the *RPE65* gene (LCA2), or a specific subtype of Retinitis Pigmentosa (RP) caused by disease-causing variants in the *RPE65* gene (RP20).

RPE65-deficiency occurs in approximately one in 125,000 people in the U.S.[1] There are estimated to be approximately 6,000 RPE65-deficient patients in the U.S., Japan and EU5, with almost 30% of those patients under the age of 30 years old. Approximately 50 new cases are diagnosed annually.

[1] Based on an estimated prevalence of approximately one in 500,000 people in the U.S. with LCA related to disease-causing variants in the *RPE65* gene, and approximately one in 70,000 people in the U.S. with RP due to disease-causing variants in the *RPE65* gene.

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 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 the timing and results of data from the Phase 1/2 trial of AAV-RPE65, 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, 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2019, 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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