MeiraGTx Announces Data at EURETINA 2021 Virtual Meeting Demonstrating Reversal of Disease Progression Following Treatment with AAV5-RPGR in X-Linked Retinitis Pigmentosa

September 9, 2021

LONDON and NEW YORK, Sept. 09, 2021 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced new data from subjects treated in the Phase 1/2 dose escalation phase of Study MGT009 indicating AAV5-RPGR, an investigational gene therapy in development for the treatment of X-linked retinitis pigmentosa (XLRP), reverses course of disease progression when retinal function assessed 12 months following treatment with AAV5-RPGR is compared with retinal function in these same subjects up to 48 months prior to treatment. These data were presented today in an oral session at the EURETINA 2021 Virtual Meeting.

Details of the presentation are listed below.

Title: AAV5-RPGR Gene Therapy for RPGR-Associated X-Linked Retinitis Pigmentosa Reverses Natural Disease Progression
Abstract: 8412
Presenter: Michel Michaelides, BSc MB BS MD(Res) FRCOphth FACS
Date and Time: Thursday, September 9, 8:00am CEST (2:00am ET)
Session: Retinal Dystrophies FP Narration

MeiraGTx and Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, are jointly developing AAV5-RPGR as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases.

EURETINA 2021 Data Summary:

- Ten adult males aged 18-30 years with RPGR-associated XLRP from the natural history study MGT011 were identified as suitable for gene therapy intervention and were subsequently enrolled in the dose escalation phase of MGT009, the Phase 1/2 Trial of AAV5-RPGR Gene Therapy
  - Retinal function was assessed through 12 months post-treatment in MGT009
  - Changes in mean retinal sensitivity (MS) and the volumetric analysis of the central 30 degrees of the retinal field (V30), were examined up to 48 months pre-intervention (MGT011) and 12 months post-intervention (MGT009)
- For the intermediate dose escalation cohort (N=4), intervention with AAV5-RPGR therapy in the poorer- seeing eye altered the course of natural disease progression
- At 12 months post-intervention, MS and V30 in the treated eye were similar to levels observed 24 months pre-intervention, while the untreated eye showed a continued downward trajectory

In November 2020, MeiraGTx announced 12-month data from the ongoing Phase 1/2 MGT009 clinical trial of AAV5-RPGR, which demonstrated that statistically significant vision improvement was sustained one year after treatment in the dose escalation phase of the trial. MeiraGTx and Janssen are preparing to initiate the pivotal Phase 3 Lumeos clinical trial of AAV5-RPGR for the treatment of patients with XLRP.

About AAV5-RPGR
AAV5-RPGR is an investigational gene therapy for the treatment of patients with XLRP caused by disease-causing variants in the eye-specific form of the *RPGR* gene (*RPGR* ORF15). AAV5-RPGR is designed to deliver functional copies of the *RPGR* gene to the subretinal space in order to improve and preserve vision. MeiraGTx and development partner Janssen are currently conducting a Phase 1/2 clinical trial of AAV5-RPGR in patients with XLRP with disease-causing variants in *RPGR* ORF15 (MGT009). AAV5-RPGR has been granted Fast Track and Orphan Drug designations by the U.S. Food and Drug Administration (FDA) and PRIME, ATMP and Orphan designations by the European Medicines Agency (EMA).

About Phase 1/2 Study MGT009
Study MGT009 is a Phase 1/2, first-in-human, open-label, multi-center study of a recombinant adeno-associated virus (AAV) vector AAV5-RPGR for gene therapy of adults and children with XLRP caused by mutations in the *RPGR* gene (*RPGR*-XLRP). Study MGT009 includes 10 adults in a dose-escalation phase, three children in a pediatric dose-confirmation phase, followed by a randomized expansion phase which includes adults randomized 1:1:1 to receive immediate treatment at one of two doses or deferred treatment six months after randomization. MGT009 is currently ongoing having completed enrollment and treatment of all subjects.

About Long Term Natural History Study MGT011
Study MGT011 is a prospectively designed non-interventional long-term natural history study of participants with *RPGR*-XLRP with design elements matching those of the therapeutic study MGT009. MGT011 has enrolled over 135 participants with retinal function data available and in some patients with almost 5.5 years of follow up data. Demographic and baseline characteristics for the participants in MGT011 and MGT009 studies are similar.

About X-Linked Retinitis Pigmentosa (XLRP)
XLRP is the most severe form of retinitis pigmentosa (RP), a group of inherited retinal diseases characterized by progressive retinal degeneration and vision loss. In XLRP, both rods and cones function poorly, leading to degeneration of the retina and total blindness. The most frequent cause of XLRP is disease-causing variants in the *RPGR* gene, accounting for more than 70% of cases of XLRP, and up to 20% of all cases of RP. There are currently no approved treatments for XLRP.

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: ocular, including inherited retinal diseases and large degenerative diseases, neurodegenerative diseases and severe forms of xerostomia. 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 the development and efficacy of AAV5-RPGR, plans to initiate the pivotal Phase 3 Lumeos clinical trial of AAV5-RPGR and anticipated milestones regarding our clinical data and reporting of such data and the timing of results of data, including in light of the COVID-19 pandemic, 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, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, 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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