MeiraGTx Announces AAV-CNGA3 Granted Rare Pediatric Disease Designation by the U.S. FDA for the Treatment of Achromatopsia

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LONDON and NEW YORK, Aug. 27, 2018 (GLOBE NEWSWIRE) -- MeiraGTx Holdings Plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced that the Offices of Orphan Products Development and Pediatric Therapeutics of the U.S. Food and Drug Administration (FDA) have granted rare pediatric disease designation to the Company's gene therapy product candidate AAV-CNGA3 for the treatment of patients with achromatopsia (ACHM) due to mutations in the *CNGA3* gene.

ACHM is an inherited retinal disease that severely limits a person's sight by preventing cone photoreceptors in the eye from functioning. Individuals with ACHM are often legally blind from birth, have extreme sensitivity to light, and experience involuntary eye movements. AAV-CNGA3 is an investigational gene therapy treatment designed to restore cone function, delivered to the cone receptors at the back of the eye via subretinal injection.

"We are very pleased that the FDA and EMA continue to recognize the important research MeiraGTx is doing for people living with rare inherited disorders such as ACHM," said Zandy Forbes, Ph.D., chief executive officer of MeiraGTx. "Our focus remains on patient benefit and developing products that offer a cure to people living with serious diseases. Working closely with expert clinicians and the FDA, we look forward to bringing impactful therapies to those in need."

In 2018, AAV-CNGA3 was granted orphan drug designation (ODD) by the FDA and received a positive opinion from the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products recommending orphan medicinal product designation. Both the ODD and EMA positive opinion for AAV-CNGA3 are indicated for the treatment of ACHM caused by mutations in the *CNGA3* gene.

A rare pediatric disease designation may be granted by the FDA to drugs and biologics intended to treat certain orphan diseases affecting fewer than 200,000 patients in the U.S., the serious or life-threatening manifestations of which primarily affect individuals aged 18 years or younger. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor that receives approval for a biologics license application for a rare pediatric disease may be eligible to receive a voucher for a priority review of a subsequent marketing application for a different product.

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 four ongoing clinical programs 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, severe forms of xerostomia and neurodegenerative diseases. Though initially focusing on the eye, salivary gland and central nervous system, 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 Statements
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 product pipeline, anticipated product benefits, goals and strategic priorities, product candidate development, growth expectations or targets and 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2018 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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