



MeiraGTx Announces AAV-CNGB3 Granted Fast Track Designation by U.S. FDA for Treatment of Achromatopsia

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LONDON and NEW YORK, Aug. 20, 2018 (GLOBE NEWSWIRE) -- MeiraGTx Holdings Plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for its AAV-CNGB3 gene therapy product candidate for the treatment of achromatopsia (ACHM) caused by mutations in the *CNGB3* 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-CNGB3 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 thrilled by this designation and the continued recognition by the FDA of AAV-CNGB3's potential to become a much-needed treatment option for ACHM patients suffering from this debilitating disease," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "Fast Track designation is not just a milestone for the company, but also for the ACHM community. With the ability to communicate closely and often with the FDA, we are hopeful that we can expedite our commitment to bring a safe and effective therapy to patients in need."

AAV-CNGB3 has been granted orphan drug designation (ODD) by the FDA and orphan medicinal product designation by the European Medicines Agency (EMA), as well as rare pediatric disease designation by the FDA and PRIME designation by the EMA, for the treatment of ACHM caused by mutations in the *CNGB3* gene. MeiraGTx is currently conducting a Phase 1/2 clinical trial of AAV-CNGB3 in both adult and pediatric patients, with 14 patients treated to date. Eleven adult patients have been treated in three dose escalation cohorts and three pediatric patients have now been treated in the extension phase of the study. The company anticipates completing dosing of up to eight pediatric patients in the second half of 2018.

The FDA's Fast Track process is designed to expedite the development and review of drugs used to treat serious conditions and fill an unmet medical need. Fast Track designation enables the company to have early and frequent communication with the FDA throughout the drug development and review process, with the potential for faster drug approval and patient access. Today's announcement marks the second Fast Track designation granted to MeiraGTx in 2018. In April 2018, the company was also granted Fast Track designation for AAV-RPGR for the treatment of another debilitating ocular disorder, X-linked retinitis pigmentosa (XLRP) due to defects in the *RPGR* 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 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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