



MeiraGTx Announces AAV-CNGA3 Granted Fast Track Designation by U.S. FDA for Treatment of Achromatopsia

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LONDON and NEW YORK, Jan. 26, 2021 (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 to its AAV-CNGA3 gene therapy product candidate for the treatment of achromatopsia (ACHM) caused by 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 and have extremely debilitating sensitivity to light. 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.

MeiraGTx and Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, are jointly developing AAV-CNGA3 as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases. MeiraGTx is currently conducting an open-label, dose-escalation Phase 1/2 clinical trial of AAV-CNGA3 in patients with ACHM due to mutations in the *CNGA3* gene. ([NCT03758404](https://clinicaltrials.gov/ct2/show/study/NCT03758404))

"We are very pleased to have received Fast Track designation for AAV-CNGA3 and that the FDA has recognized a significant need exists to quickly advance new therapies for those with ACHM," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "ACHM is a serious and debilitating disease and we look forward to communicating closely with the FDA as we continue the clinical advancement of AAV-CNGA3."

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.

AAV-CNGA3 has already been granted Orphan Drug designation by the FDA and European Medicines Agency (EMA). In addition, AAV-CNGA3 for the treatment of ACHM caused by mutations in the *CNGA3* gene has been designated as a drug for a rare pediatric disease by the FDA.

This is the third Fast Track designation MeiraGTx has received for its inherited retinal disease product candidates, having previously received Fast Track designation for AAV-RPGR and AAV-CNGB3, as well as having received PRIME designation for AAV-RPGR and AAV-CNGB3 from the EMA. AAV-CNGB3 for the treatment of ACHM caused by mutations in the *CNGB3* gene has also been designated as a drug for a rare pediatric disease by the FDA.

MeiraGTx also recently received orphan drug designation from the FDA as well as orphan medicinal product designation from the EMA for its investigational gene therapy product AAV-RDH12 for the treatment of retinol dehydrogenase 12 (*RDH12*) mutation-associated retinal dystrophy.

Disease-causing sequence variants in *RDH12* cause severe retinal dystrophy most often resulting in the clinical diagnosis of Leber congenital amaurosis (LCA) and early onset severe retinal dystrophy (EOSRD); although *RDH12* variants have also been associated with a clinical diagnosis of retinitis pigmentosa (RP). Sequence variants in *RDH12* account for 3.4%–10.5% of LCA/EOSRD. Individuals with *RDH12* deficiency exhibit widespread retinal degeneration impacting both rods and cones, with early macular involvement. Most people with *RDH12*–LCA/EOSRD experience marked central visual loss by their late teens to twenties. AAV-*RDH12* is an AAV based gene therapy designed to deliver a functional copy of the *RDH12* gene to the retina of patients with genetically defined *RDH12* deficiency.

About Achromatopsia

Achromatopsia (ACHM) 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, reduced or complete loss of color vision, 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 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. 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.meiraqtx.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 AAV-CNGA3 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," "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, 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 September 30, 2020, 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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