MeiraGTx Receives Rare Pediatric Disease Designation for A002 for the Treatment of Achromatopsia Due to Mutations in the CNGB3 Gene

February 1, 2018

London, UK, New York, NY, February 1, 2018 (PR NEWSWIRE) – MeiraGTx, a London and New York-based 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 AAV2/8-hCARp.hCNGB3 (A002) for the treatment of patients with achromatopsia (ACHM) due to mutations in the *CNGB3* gene. Achromatopsia is an inherited eye disorder that severely limits a person's sight as well as causing extreme light sensitivity, involuntary eye movement, and complete color blindness. A002 previously received orphan drug designation from the FDA and the European Medicines Agency (EMA) for the treatment of ACHM in 2016.

A002 is an adeno-associated virus (AAV) investigational gene therapy designed to deliver a codon-optimized hCNGB3 cDNA to the back of the eye for expression in cone photoreceptors. A002 is delivered via a sub-retinal injection to cover the central region of the retina, including the fovea where the majority of cones are located.

MeiraGTx has dosed eight patients in an open label, multi-center Phase I/II dose escalation trial of A002 in individuals diagnosed with achromatopsia due to biallelic mutations in *CNGB3*. The trial is currently treating patients in the highest of three dose cohorts. The primary endpoint of the study is to determine the safety of the treatment. Secondary endpoints include improvement in visual function including assessment of photophobia/photoaversion, retinal function, retinal structure and quality of life measures.

"With the receipt of our second rare pediatric disease designation in just two months, we have significant momentum for our ocular gene therapy pipeline as we begin 2018," said Zandy Forbes, Ph.D., President and CEO of MeiraGTx. "Achromatopsia is a severe and debilitating genetic disorder of the retina for which there are no effective treatment options. We are excited to be making such good progress in dosing patients in our ongoing Phase I/II *CNGB3* clinical trial."

About Rare Pediatric Disease Designation

A rare pediatric disease designation may be granted by the FDA to drugs and biologics intended to treat orphan diseases affecting fewer than 200,000 patients in the U.S., primarily aged 18 years or younger. The designation provides incentives to advance the development of rare disease treatments, including access to the FDA's expedited review and approval programs. In addition, 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 a severe inherited retinal disorder characterized by markedly reduced visual acuity (legal blindness), extreme light sensitivity, nystagmus, and absence of color discrimination from birth. Achromatopsia is genetically heterogeneous, with mutations in *CNGB3* and *CNGA3* accounting for 70-80% of patients. Currently, there are no effective treatments for this disease.

About MeiraGTx

MeiraGTx is committed to the development of novel gene therapies to transform the lives of patients suffering from acquired and inherited disorders. The company is developing treatments for ocular diseases, including rare inherited blindness and age-related macular degeneration (AMD). MeiraGTx is also establishing treatments for xerostomia, a frequent and debilitating side effect of radiation treatment used in head and neck cancers, as well as certain neurodegenerative diseases. In addition, MeiraGTx is developing novel gene regulation platforms that promise to transform the way gene therapy can be applied and create new paradigms for biologic therapeutics.

For more information, please visit www.meiragtx.com

Forward-Looking Statements

This press release contains forward-looking statements. These forward-looking statements are based on management's expectations and are subject to certain factors, risks and uncertainties that may cause actual results, outcome of events, timing and performance to differ materially from those expressed or implied by such statements. The information contained in this press release is believed to be current as of the date of original issue. MeiraGTx expressly disclaims any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations with regard thereto or any change in events, conditions or circumstances on which any such statements are based.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com
or
Media:
W2O pure
Kelly Boothe, 206-349-3010
kboothe@w2ogroup.com