MeiraGTx Receives EMA PRIME Designation for Achromatopsia Gene Therapy Candidate

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London, UK, New York, NY March 2, 2018 (Businesswire) – MeiraGTx, a London and New York based gene therapy company, announced today the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) designation to MeiraGTx's gene therapy product candidate A002 for the treatment of patients with achromatopsia (ACHM) due to mutations in the *CNGB3* gene. Achromatopsia is a chronically debilitating inherited eye disorder that severely limits a person's sight. Patients are legally blind from birth with very poor visual acuity as well as extreme light sensitivity, involuntary eye movement, and complete color blindness. The PRIME application was based on non-clinical *in vivo* data and early clinical safety data from MeiraGTx's ongoing Phase I/II dose escalation study.

"With receipt of this important designation, we are excited to be begin working closely with the EMA to accelerate A002 as a potential treatment option for patients living with this challenging and painful condition," said Zandy Forbes, Ph.D., President and CEO of MeiraGTx. "Having just received Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) earlier this month, we are very pleased with the momentum of our ACHM program and the recognition by regulatory agencies of its potential to help those in need of effective treatment options."

A002 is an adeno-associated virus (AAV) investigational gene therapy designed to deliver a codon-optimized CNGB3 cDNA under the control of the cone arrestin (CAR) promoter to photoreceptors in the back of the eye. 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 completed dosing of 8 CNGB3 patients in the Phase I/II study and is currently treating patients in the third dose escalation cohort. A002 received rare pediatric disease designation for the treatment of ACHM in January 2018 and has also received orphan drug designation from the FDA and the EMA.

About PRIME
PRIME is an initiative launched by EMA in 2016 to enhance support for the development of medicines that target an unmet medical need, and may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. PRIME aims to strengthen clinical trial designs to facilitate the generation of high quality data for the evaluation of an application for marketing authorization. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on preclinical and/or early clinical data.

About Achromatopsia

Achromatopsia (ACHM) 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, we believe that 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 potential treatments for ocular diseases, including rare inherited blindness and age-related macular degeneration (AMD). MeiraGTx is also developing potential 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 it believes will 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.

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