MeiraGTx Receives Rare Pediatric Disease Designation for A001 for the Treatment of Leber's Congenital Amaurosis and Treats First Pediatric Patient in Phase 1/2 Clinical Trial

December 7, 2017

London, UK, New York, NY, December 7, 2017 (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 AAV2/5-OPTIRPE65 (A001) for the treatment of patients with Leber's Congenital Amaurosis due to mutations in the RPE65 gene (LCA2). A001 previously received orphan drug designation from the FDA for the treatment of LCA2 in 2016.

A001 is an adeno-associated virus (AAV2/5) investigational gene therapy designed to deliver a codon-optimized RPE65 cDNA under the control of a synthetic RPE-specific promoter to the back of the eye. A001 is delivered via subretinal injection of up to 1 ml to cover the largest possible area of viable retina.

"The FDA's decision to award rare pediatric disease designation to A001, along with the previously received orphan drug designation, underscores the urgency of developing effective therapies for rare pediatric diseases like LCA2," said Zandy Forbes, Ph.D., President and CEO of MeiraGTx. "We are pleased with the rapid progress in our LCA2 clinical study and we are excited to be including pediatric patients with this severe genetic disorder in both Europe and the U.S."

MeiraGTx is conducting an open label, multi-center Phase I/II dose escalation trial of A001 in up to 27 patients, aged 3 years and older, diagnosed with severe early onset LCA2. The primary endpoint of the study is to determine the safety of the treatment. Secondary endpoints include improvement in visual function including a mobility maze, retinal function, retinal structure and quality of life measures. MeiraGTx has completed the three dose escalation cohorts of adult patients in this study and in November 2017 the first pediatric patient was treated at the Moorfields Eye Hospital in London.

In October 2017, the FDA accepted the Investigational New Drug (IND) application for A001 opening the path for the treatment of pediatric patients in the U.S.

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 LCA

Leber's Congenital Amaurosis (LCA) is a group of autosomal recessive, early-onset retinal dystrophies that cause severe sight impairment in childhood. The underlying deficit in up to 16 percent of all LCA cases lies in the *RPE65* gene, which plays a key role in the regeneration of visual pigment following exposure to light. There is currently no approved treatment available that can improve sight or protect against progressive sight impairment for people with LCA.

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.

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