



MeiraGTx Provides Clinical Trial Updates for Achromatopsia and Leber's Congenital Amaurosis Ocular Gene Therapy Programs

February 8, 2017

New York, NY/London, UK, February 8, 2017 /PR NEWSWIRE/ — MeiraGTx, a New York and London based gene therapy company, announced today the first patient in its clinical study for achromatopsia due to mutations in the CNGB3 gene was treated at the Moorfields Eye Hospital in London. The AAV-mediated gene therapy study design for CNGB3 deficiency is an open label, multi-center, Phase I/II dose escalation trial.

Achromatopsia is a recessively inherited condition characterized by a lack of cone photoreceptor function resulting in impairment of visual acuity and color vision, central scotoma often with eccentric fixation, disabling hypersensitivity to light (photophobia) and involuntary eye movements (pendular nystagmus). Children with CNGB3-related achromatopsia have profound sight impairment from birth or early infancy, and the condition is currently untreatable.

In addition, MeiraGTx has successfully completed dosing of patients in the second cohort in its clinical trial for Leber's Congenital Amaurosis with RPE65 mutations, also at the Moorfields Eye Hospital in London.

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% 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 for LCA-RPE65 deficiency.

The timely patient accrual and study initiation in both MeiraGTx inherited retinal disease studies reflects the value of the Company's partnership with Moorfield's Eye Hospital and its access to a world leading patient catchment, as well as the extensive natural history and imaging data gathered on these rare patients.

"We are very excited to be treating inherited blindness by addressing the genetic defect itself. Our aim is to improve the outcome for people with eye diseases that are currently untreatable," said James Bainbridge, MD Ph.D., of Moorfields and UCL.

"MeiraGTx is very pleased to have started a clinical trial in our second genetic ocular program," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx. "Our commitment to treating genetic diseases of the eye is a major area of effort for MeiraGTx. We look forward to continuing our work not only in LCA2 and achromatopsia, but in our other rare eye disease programs as well."

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.

About Moorfields Eye Hospital

Moorfields Eye Hospital NHS Foundation Trust is the leading provider of eye health services in the UK and a world-class center of excellence for ophthalmic research and education. We have a reputation, developed over two centuries, for providing the highest quality of ophthalmic care. Our 1,800 staff are committed to sustaining and building on our pioneering legacy and ensuring we remain at the cutting edge of developments in ophthalmology.

About UCL

The UCL Institute of Ophthalmology conducts cutting-edge science, attracting research workers of the highest international caliber. The most recent national Research Excellence Framework has once more confirmed that they have the strongest Clinical Medicine research of any British university. Independent auditors have shown that their partnership with Moorfield's Eye Hospital is the most productive in the world.

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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