MeiraGTx Provides Clinical Trial Updates for X-Linked Retinitis Pigmentosa and Achromatopsia Gene Therapy Programs

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New York, NY, London, UK, August 24, 2017 (PR NEWSWIRE) – MeiraGTx, a New York and London based gene therapy company, announced today the first patient in its clinical study for X-Linked Retinitis Pigmentosa (XLRP) was treated at Moorfields Eye Hospital in London. This clinical study is the first-in-man Phase I/II dose escalation study of AAV2/5-hRKp.RPGR, MeiraGTx's AAV-mediated gene therapy for XLRP caused by mutations in the RP GTPase regulator gene (*RPGR*).

In addition, the first patient in the 2nd cohort in the company's clinical study of AAV-mediated gene therapy AAV2/8-hCARp.hCNGB3 for Achromatopsia patients with mutations in the *CNGB3* gene has also been treated at Moorfields Eye Hospital. This follows the safe completion of treatment of the first three patients in the low dose cohort in the *CNGB3* clinical trial.

MeiraGTx has also treated 7 patients in the PhaseI/II clinical study of AAV2/5-OPTIRPE65 in LCA2 patients with RPE65 deficiency, with the final two patients in the high dose cohort to be treated during Q3 2017.

In addition to these therapeutic clinical studies, MeiraGTx is conducting ongoing Natural History studies in each of these indications. In the Natural History study of XLRP caused by mutations in *RPGR*, 70 subjects are enrolled, all of whom are deep phenotyped, including 50 patients with more than 2 years natural history data.

Retinitis Pigmentosa (RP) is the commonest inherited retinal dystrophy, and is characterized by progressive constriction of visual field with eventual central vision loss, and legal blindness. An estimated 10% to 20% of cases are X-linked, and more than 70% of X-linked cases are caused by mutations in the *RPGR* gene. *RPGR* encodes a protein located in the cilium that connects the inner and outer segments of photoreceptors, which is required to maintain directional protein transport between the inner and outer segments. Mutations in *RPGR* result in disrupted transport and lead to photoreceptor cell death. Currently, there are no treatments available for this condition.

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.

MeiraGTx has received Orphan Drug Designation (ODD) from the FDA and Orphan Medicinal Product Designation from the EMA Committee for Orphan Medical Products (COMP) for each of their proprietary AAV-mediated ocular gene therapy product candidates with on-going clinical trials.

"MeiraGTx is pleased to have initiated its third clinical trial in rare inherited retinal disease," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx. "We continue our commitment to treating genetic diseases of the eye for patients globally."

"We are delighted that the first patient has had successful surgery in our clinical trial for XLRP, and also with the rapid advancement of our two other ocular gene therapy clinical studies. We are excited to be making such progress in the development of treatments for inherited retinal blindness which collectively account for the commonest cause of blindness in the working age population and the second commonest in children" said Dr. Michel Michaelides, a founding member of MeiraGTx, and Professor of Ophthalmology, UCL Institute of Ophthalmology in the Department of Genetics, and Consultant Ophthalmic Surgeon in the Departments of Medical Retina, Genetics and Pediatric Ophthalmology at Moorfields Eye Hospital.

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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