MeiraGTx Granted Orphan Drug Designation for its A004 Gene Therapy Treatment for Retinitis Pigmentosa

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NEW YORK, Dec 20, 2016/PRNewswire/ — MeiraGTx, a New York and UK based gene therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) for its proprietary AAV-mediated gene therapy product candidate containing the retinitis pigmentosa GTPase regulator (RPGR) gene for treatment of X-linked retinitis pigmentosa (XLRP).

Retinitis pigmentosa (RP) is one of the most common forms of inherited blindness, with three general modes of inheritance: autosomal dominant, autosomal recessive and X-linked. Over 70% of XLRP and up to 20% of all RP cases are caused by mutations in the RPGR gene. Males with XLRP caused by mutations in RPGR (RPGR-XLRP) typically have night blindness in their first decade of life followed by progressive reduction of their visual field and loss of visual acuity. By the end of their fourth decade, most patients are legally blind.

MeiraGTx has developed a gene therapy product designed to target the RPGR gene to photoreceptor cells to restore function in RPGR-XLRP patients. MeiraGTx will be initiating a Phase 1/2 clinical study in these patients in the first half of 2017 at the Moorfields Eye Hospital in London.

"We are pleased to receive Orphan Drug Designation for our XLRP gene therapy product. It is further verification of the importance of our ocular gene therapy pipeline," said Alexandria Forbes, Ph.D., President and CEO of MeiraGTx.

This marks the third gene therapy product in the MeiraGTx pipeline targeting a rare inherited retinopathy to receive Orphan Drug Designation from the FDA and Orphan Medicinal Product Designation from the European Medicines Agency (EMA) Committee for Orphan Medical Products (COMP). MeiraGTx has previously received ODD from the FDA and COMP for its gene therapy product candidates for Leber congenital amaurosis with RPE65 mutations and for achromatopsia due to mutations in the CNGB3 gene.

MeiraGTx also has ODD from the FDA for its gene therapy treatment for Xerostomia using AAV-hAQP1 gene.

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