



MeiraGTX Announces AAV-RPGR Granted Fast Track Designation by U.S. FDA for Treatment of X-Linked Retinitis Pigmentosa Due to RPGR Deficiency

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LONDON & NEW YORK—(BUSINESS WIRE)— MeiraGTX Limited, a vertically integrated, clinical stage gene therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for AAV-RPGR for the treatment of X-linked retinitis pigmentosa (XLRP) due to defects in the retinitis pigmentosa GTPase regulator (*RPGR*) gene.

"XLRP is a devastating condition that causes rapid progression to blindness and currently has no approved treatment options. This Fast Track designation is an important milestone for both patients living with XLRP and MeiraGTX, allowing our team to communicate closely and often with the FDA as we work to bring a much-needed therapy to patients," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTX. "Marking the third regulatory milestone our ocular program has received this year, we look forward to continuing this momentum in our commitment to develop innovative gene therapy treatments for patients."

MeiraGTX is currently conducting an open label, Phase 1/2 dose escalation clinical trial of AAV-RPGR in adult and pediatric patients diagnosed with XLRP caused by mutations in the eye-specific form of the *RPGR* gene called RPGR open reading frame 15 (*RPGR-ORF 15*). AAV-RPGR has received Orphan Drug designation from the U.S. FDA and Orphan Medicinal Product designation from the European Medicines Agency (EMA). In addition to the Phase 1/2 study that MeiraGTX is conducting, MeiraGTX is also continuing to enroll and conduct an ongoing natural history study of patients with XLRP.

The FDA's Fast Track process is designed to expedite the development and review of drugs used to treat serious conditions and fill an unmet medical need. Fast Track designation enables the company to have early and frequent communication with the FDA throughout the drug development and review process, which may facilitate faster drug approval and patient access.

About X-linked Retinitis Pigmentosa (XLRP)

XLRP represents the most severe forms of retinitis pigmentosa (RP), a group of inherited retinal diseases characterized by progressive retinal degeneration and vision loss that ends in complete blindness. There are currently no approved treatments for RP. The most frequent mutation causing XLRP is in the *RPGR* gene accounting for more than 70% of cases of XLRP and up to 20% of all cases of RP.

About AAV-RPGR

MeiraGTX has developed a gene therapy product candidate, AAV-RPGR, for the treatment of XLRP which is designed to treat the most common form of XLRP caused by mutations in the eye specific form of the *RPGR* gene called *RPGR-ORF 15*. We are conducting a Phase 1/2 clinical trial of AAV-RPGR in both adult and pediatric XLRP patients with mutations in *RPGR-ORF 15*.

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 potentially 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.

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