INVESTORS & **MEDIA**

MeiraGTx Announces Priority Medicines (PRIME) and Advanced Therapy Medicinal Product (ATMP) Designations Granted by the European Medicines Agency to AAV-RPGR Gene Therapy for the Treatment of X-Linked Retinitis Pigmentosa

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PDF Version

- PRIME designation granted based on clinical data from ongoing Phase 1/2 trial of AAV-RPGR
- AAV-RPGR is the only XLRP treatment in development to be awarded PRIME designation

LONDON and NEW YORK, March 02, 2020 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced that the European Medicines Agency (EMA) has granted Priority Medicines (PRIME) and Advanced Therapy Medicinal Product (ATMP) designations to AAV-RPGR, MeiraGTx's investigational gene therapy for the treatment of x-linked retinitis pigmentosa (XLRP) caused by mutations in the *RPGR* gene.

MeiraGTx and Janssen Pharmaceuticals, Inc. (Janssen), part of the Janssen Pharmaceutical Companies of Johnson & Johnson, are jointly developing AAV-RPGR as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases.

PRIME designation was granted based on clinical data from MeiraGTx and Janssen's ongoing Phase 1/2 trial of AAV-RPGR in patients with XLRP (NCT03252847). AAV-RPGR is the only gene therapy in development for the treatment of XLRP to receive PRIME designation.

"XLRP is a severe disease which causes rapid progression to blindness and total loss of vision in most patients by the fourth decade," said Alexandria Forbes, Ph.D., president and CEO, MeiraGTx. "People suffering from this devastating disease are currently living without treatment options, and

To be eligible for PRIME, medicinal products must be of major public health interest and target conditions where there is an unmet medical need. To determine PRIME eligibility, the EMA considers whether the available data suggest that the product may potentially offer a major therapeutic advantage to patients in a given indication through a clinically meaningful improvement, such as having an impact on the prevention, onset and duration of the condition, or improving the morbidity or mortality of the disease. To be accepted for PRIME, early clinical data must demonstrate the medicine's potential to benefit patients with unmet medical needs. Features of PRIME designation include guidance and interaction with the EMA on the drug development plan, including early interactions to discuss the regulatory pathway and potential ways to generate data packages designed to address EMA Marketing Authorization Application (MAA) requirements.

ATMP status is granted to medicines that are based on genes, tissues or cells and can offer groundbreaking new opportunities for the treatment of disease. All advanced therapy medicines are authorized centrally via the EMA and may benefit from a single evaluation and authorization procedure.

For more information about the ongoing clinical trial, please visit https://clinicaltrials.gov/ct2/show/NCT03252847.

About AAV-RPGR
AAV-RPGR is an investigational gene therapy for the treatment of patients with XLRP caused by mutations in the eye specific form of the *RPGR* gene (*RPGR* ORF15). AAV-RPGR is designed to deliver functional copies of the *RPGR* gene to the subretinal space in order to improve and preserve visual function. MeiraGTx and development partner Janssen are currently conducting a Phase 1/2 clinical trial of AAV-RPGR in patients with XLRP with mutations in *RPGR* ORF15. AAV-RPGR has been granted Fast Track and Orphan Drug designations by the U.S. Food and Drug Administration (FDA) and PRIME, ATMP and Orphan designations by the European Medicines Agency (EMA).

About X-Linked Retinitis Pigmentosa (XLRP)
XLRP is the most severe form of retinitis pigmentosa (RP), a group of inherited retinal diseases characterized by progressive retinal degeneration and vision loss. In XLRP, both rods and cones function poorly, leading to degeneration of the retina and total blindness. 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. There are currently no approved treatments for XLRP.

About MeiraGTx
MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical stage gene therapy company with six programs in clinical development and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, as well as a potentially transformative gene regulation technology. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: inherited retinal diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system and salivary gland, MeiraGTx intends to expand its focus in the future to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

For more information, please visit www.meiragtx.com.

Forward Looking Statement
This press release contains forward-looking statements within the meaning of the Private Securities

similar statements of a future or forward-looking nature. These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, acquire additional capital, identify additional and develop existing product candidates, successfully execute strategic priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; litigation risks; and the other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2019, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC's website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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