MeiraGTx Receives Clinical Development Milestone Payment from Janssen

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-MeiraGTx Receives $30 Million Cash Milestone Payment

-MeiraGTx, in Collaboration with Janssen Pharmaceuticals, Inc., is Now Dosing Patients in the Phase 3 Lumeos Clinical Trial in X-linked Retinitis Pigmentosa

-MeiraGTx Remains Eligible for Further Development and Commercial Milestones Related to botaretigene sparoparvovec (AAV-RPGR), as well as for AAV-CNGB3 and AAV-CNGA3

LONDON and NEW YORK, Jan. 27, 2022 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced that it has received a $30 million payment from Janssen Pharmaceuticals, Inc. (Janssen), part of the Janssen Pharmaceutical Companies of Johnson & Johnson, for a clinical milestone in the Phase 3 Lumeos trial of botaretigene sparoparvovec (AAV-RPGR), an investigational gene therapy for the treatment of X-linked retinitis pigmentosa (XLRP). The Phase 3 Lumeos trial is a global study of botaretigene sparoparvovec which is now dosing participants.

MeiraGTx and Janssen are jointly developing botaretigene sparoparvovec as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases. MeiraGTx remains eligible to receive additional development and commercial milestones for botaretigene sparoparvovec as well as for other programs as part of the collaboration agreement.

“XLRP is one of the most common and severe forms of retinitis pigmentosa, with no approved treatment options, and with most patients completely losing sight by the fourth decade of their lives,” said Alexandria Forbes, Ph.D., President and Chief Executive Officer of MeiraGTx. “We are very pleased with the progress made in the Lumeos study and are working closely with our collaboration partner Janssen, the regulatory agencies, and patient and clinical communities to advance botaretigene sparoparvovec toward regulatory approval.”

About Botaretigene Sparoparvovec
Botaretigene sparoparvovec, formerly referred to as AAV-RPGR, is an investigational gene therapy for the treatment of patients with XLRP caused by disease-causing variants in the eye-specific form of the *RPGR* gene (RPGR ORF15). Botaretigene sparoparvovec is designed to deliver functional copies of the *RPGR* gene to the retina in order to improve and preserve vision. The Lumeos trial (NCT04671433) is a Phase 3 randomized, controlled study of botaretigene sparoparvovec for the treatment of XLRP associated with variants in the *RPGR* gene. Botaretigene sparoparvovec has been granted Fast Track and Orphan Drug designations by the FDA and PRIority MEdicines (PRIME), Advanced Therapy Medicinal Product (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 cause of XLRP is disease-causing variants 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, and a transformative gene regulation platform technology which allows tight, dose responsive control of gene expression by oral small molecules with dynamic range that may exceed 5000-fold. 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: ocular, including inherited retinal diseases and large degenerative ocular diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx plans to expand its focus 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 Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the development and efficacy of botaretigene sparoparvovec, the Phase 3 Lumeos clinical trial of botaretigene sparoparvovec and the achievement of milestones or regulatory approvals, including in light of the COVID-19 pandemic, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate” and 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, raise 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; the impact of the COVID-19 pandemic on the status, enrollment, timing and results of our clinical trials and on

our business, results of operations and financial condition; 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 or other manufacturing issues; 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; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, 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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