MeiraGTx Granted FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for AAV-GAD for the Treatment of Parkinson's Disease

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- *This RMAT designation is based on data from 3 clinical studies demonstrating the potential benefit of AAV-GAD as a one-time treatment for Parkinson's disease*

- *RMAT designation includes the benefits of the Fast Track and Breakthrough Therapy designations, allows frequent regulatory interactions with the FDA, and potential routes to accelerated approval and Priority Review*

LONDON and NEW YORK, May 09, 2025 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to AAV-GAD for the treatment of Parkinson's disease not adequately controlled with anti-Parkinsonian medications.

This RMAT was awarded following the presentation to the FDA of positive data from 3 clinical studies demonstrating the benefit of AAV-GAD when administered in a one-time stereotactic infusion to the subthalamic nucleus in the brain. A Phase 1 dose escalating clinical study (n=12) was conducted, followed by a double-blind, sham-controlled Phase 2 study (n=45) and a second randomized, double-blind, sham-controlled dose ranging clinical bridging study (n=14).

"We are very pleased to have been awarded this RMAT designation for our AAV-GAD product in Parkinson's disease," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "The data from our double-blind, sham-controlled Phase 2 studies show significant clinically meaningful benefit on the standard motor endpoint in Parkinson's disease, UPDRS Part 3, as well as other validated measures of Parkinson's symptoms. What is most exciting is the analysis of the data from these Phase 2 studies using the AI technology of our partner Hologen. Through the use of Hologen's technology applied to the data from our double-blind, sham-controlled studies, we have demonstrated disease modifying changes in the circuitry of the brain of patients treated with AAV-GAD as well as potentially protective changes in the substantia nigra and regions of the brain involved in cognition and mood. This is the first time sham-controlled gene or cell therapy Phase 2 studies have shown significant benefit in UPDRS and now, to our knowledge, the only demonstration of disease modification in a sham or placebo-controlled study in Parkinson's disease."

Dr. Forbes continued, "We are excited to have been granted RMAT designation for our AAV-GAD program in Parkinson's disease and we look forward to working closely with the FDA to bring this potential life changing therapy to this large population of Parkinson's patients in need of effective and disease modifying treatments for this serious neurodegenerative disorder."

The requirements for receiving an RMAT designation include that the drug candidate is an advanced regenerative medicine, in this case a gene therapy; that the therapy is targeting a serious condition, in this case, Parkinson's disease; and that the applicant has presented clinical evidence demonstrating that the drug candidate has the potential to address an unmet need in the serious condition. The RMAT requirement for clinical data supporting a benefit in an unmet need is a high hurdle, with less than half of all RMAT designation applications granted.

The RMAT designation is aimed to expedite the development and review of promising Regenerative Medicine therapeutic candidates, including human gene therapies, that treat, modify, reverse or cure serious or life-threatening diseases. Similar to Breakthrough Therapy designation, RMAT designation allows for increased interaction with the FDA and immediate multidisciplinary comprehensive discussions of the ongoing product development program, clinical trials and plans for expediting the manufacturing development strategy. RMAT designation includes the benefits of Fast Track and Breakthrough Therapy designations with rolling review and potential Priority Review of a product's biologics license application (BLA).

About AAV-GAD

Parkinson's disease (PD) is the second most common neurodegenerative disease after Alzheimer's, with nearly one million people in the U.S. currently living with Parkinson's disease and approximately 90,000 new patients diagnosed annually in the U.S. There are more than 10 million people worldwide currently living with PD. Most individuals with PD initially respond to dopamine replacement therapy, yet for a large percentage of patients, over time, this type of treatment is no longer sufficiently helpful while adverse effects of medication can also occur, leading to a considerable reduction in quality of life and the ability to function effectively. The cause of Parkinson's disease is unknown for a majority of patients, while a much smaller percentage have a known genetic cause, but in all cases, there is dysfunction of the key circuits that control movement. AAV-GAD is an investigational gene therapy designed to reprogram these dysfunctional brain circuits through the local production of GABA, a chemical neurotransmitter that can help restore more normal activity to these critical cells in any form of PD. AAV-GAD is delivered via a one-time infusion through a minimally invasive procedure, using a MeiraGTx proprietary device that allows infusion of the equivalent of one drop of gene therapy solution into the subthalamic nucleus, a key regulator of the circuits responsible for normal movement.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline with four late-stage clinical programs. Each of these programs use local delivery of small doses resulting in disease modifying effects in both inherited and more common diseases, in the eye, Parkinson's disease and radiation-induced xerostomia. MeiraGTx uses its innovative technology in optimization of capsids, promoters and novel translational control elements to develop best in class, potent, safe viral vectors. MeiraGTx's broad pipeline is supported by end-to-end in-house manufacturing. MeiraGTx has built the most comprehensive manufacturing capabilities in the industry, with 5 facilities globally, including two that are licensed for GMP viral vector production and a GMP QC facility with clinical and commercial licensure. In addition, MeiraGTx has developed a proprietary manufacturing platform process over 9 years based on more than 20 different viral vectors with leading yield and quality

aspects and commercial readiness. Uniquely, MeiraGTx has developed a novel technology for *in vivo* delivery of any biologic therapeutic using oral small molecules. This transformative riboswitch gene regulation technology allows precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the regulated *in vivo* delivery of metabolic peptides, including GLP-1, GIP, Glucagon, Amylin, PYY and Leptin, as well as cell therapy, CAR-T for liquid and solid tumors and autoimmune diseases, and additionally PNS targets addressing long term intractable pain. MeiraGTx has developed the technology to apply genetic medicine to common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

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 our product candidate development and anticipated milestones regarding our pre-clinical and clinical data, reporting of such data and the timing of results of data and regulatory matters, statements regarding the collaboration with Hologen, including the anticipated timing for its closing and funding thereunder, the success of the activities to be performed under the collaboration, the efficacy of Hologen's AI technology, the development of our AAV-GAD and other CNS product candidates and the development of our manufacturing technology, 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, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic transactions or 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 or rare pediatric disease designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases 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 Annual Report on Form 10-K for the year ended December 31, 2024, 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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