MeiraGTx Granted FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for AAV2-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia

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- *RMAT designation recognizes the preliminary clinical evidence of the potential benefit of AAV2-hAQP1 as a one-time treatment for this debilitating condition*

- *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, Dec. 09, 2024 (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 AAV2-hAQP1 for the treatment of Grade 2/3 radiation-induced xerostomia (RIX).

“This RMAT designation underscores the strength of our data indicating the potential of our AAV2-hAQP1 therapy to significantly improve the lives of patients who suffer from xerostomia following radiation treatment. 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, Grade 2 and Grade 3 late xerostomia caused by radiotherapy for cancers of the upper aerodigestive tract; and that the applicant has presented preliminary 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. We are therefore very excited to have been awarded this designation for our AAV-hAQP1 program and we look forward to working closely with the FDA to bring this potential life changing therapy to these patients with no alternative treatments as quickly as possible,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx.

The RMAT designation was established under the 21st Century Cures Act 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).

AAV2-hAQP1 was previously granted Orphan Drug Designation by the FDA.

AAV2-hAQP1 for the Treatment of Xerostomia:

- Data from the Company’s Phase 1 AQUAx clinical trial were presented in an oral session at the American Academy of Oral Medicine (AAOM) 2024 annual meeting in April 2024, demonstrating that treatment with AAV2-hAQP1 resulted in significant improvements across three different patient-reported outcomes and in saliva production, with no treatment-related serious adverse events or dose-limiting toxicities reported. These data underpinned this successful RMAT designation.
- The Phase 2 AQUAx2 (NCT05926765) randomized, double-blind, placebo-controlled study continues to enroll and dose participants at multiple sites in the U.S., Canada and the U.K.
- The Company has gained alignment with the FDA on requirements for the ongoing Phase 2 AQUAx2 clinical trial for Grade 2/3 radiation-induced xerostomia to be considered a pivotal trial in support of a potential BLA filing based on the use of material manufactured using MeiraGTx’s proprietary production process and in-house manufacturing facilities.
- The RMAT designation will allow the Company to benefit from increased interactions with the FDA to further accelerate the development pathway and BLA approval.

About AAV2-hAQP1

Grade 2/3 radiation-induced xerostomia (RIX) is a severely debilitating consequence of radiation treatment for head and neck cancer that affects approximately 30-40% of all patients treated with radiation for head and neck cancer. This is a completely unmet need with no treatment options, and a large addressable market with over 170,000 patients currently in the U.S., and an additional 15,000 new patients in the U.S. each year. Treatment with AAV2-hAQP1, an investigational genetic medicine, involves a small dose locally delivered to the salivary gland via a non-invasive procedure, that can be delivered in a dental office or oncology center where these patients are seen at least annually following radiation treatment. The small local dose of AAV2-hAQP1 manufactured in-house at MeiraGTx allows for a low cost of goods, and the potential long-term durability and ease of delivery make this large addressable market a compelling commercial opportunity.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has internal plasmid production for GMP, two GMP viral vector production facilities as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. In addition, MeiraGTx has developed a

proprietary manufacturing platform with leading yield and quality aspects and commercial readiness, core capabilities in viral vector design and optimization and a transformative riboswitch gene regulation platform technology that allows for the precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the delivery of metabolic peptides, including GLP-1, GIP, Glucagon, and PYY, using oral small molecules, as well as cell therapy for oncology and autoimmune diseases. MeiraGTx has developed the technology to apply genetic medicine to more 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, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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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