



MeiraGTx Enters into Strategic Collaboration with Eli Lilly and Company to Develop and Commercialize Genetic Medicines in Ophthalmology

November 10, 2025

LONDON and NEW YORK, Nov. 10, 2025 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage genetic medicines company, today announced a broad strategic collaboration in the area of ophthalmology with Eli Lilly and Company ("Lilly").

MeiraGTx will grant Lilly worldwide exclusive rights to its AAV-AIPL1 program for treatment of one of the most severe inherited retinopathies, Leber congenital amaurosis 4 (LCA4) owing to genetic deficiency of Aryl-hydrocarbon-interacting protein-like 1 (AIPL1). Clinical data from 11 children under the age of 4 who were born legally blind as a result of mutations in the *AIPL1* gene were unprecedented, with all 11 blind children gaining vision after treatment with AAV-AIPL1. Beyond the effects on vision, treatment with AAV-AIPL1 resulted in life-changing benefits in vital areas of development including communication, behavior, learning, mood, psychological benefits and social integration.

Lilly will also receive worldwide exclusive access rights to MeiraGTx's innovative gene therapy technologies for use in ophthalmology with certain targets named by Lilly, including novel intravitreal capsids developed in-house at MeiraGTx and bespoke promoters including AI-generated promoters for specific cells in the retina. MeiraGTx also grants Lilly certain rights to its proprietary riboswitch technology for use in gene editing in the eye. MeiraGTx's riboswitch technology platform is broadly applicable to any therapeutic protein. It allows precise, titratable *in-vivo* production of the therapeutic protein or gene editing nuclease from a gene template controlled by oral dosing of a small molecule inducer.

Under the terms of the agreement, MeiraGTx will receive an upfront payment of \$75 million and will be eligible to receive over \$400 million in total milestone payments. MeiraGTx is also eligible to receive tiered royalties on licensed products.

"We are excited to be entering into this collaboration with Lilly in ophthalmology," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "MeiraGTx has been dedicated to treating patients with serious eye conditions since the company's inception, and this collaboration with Lilly is a testament to our leadership in this field and the power of our broad toolkit of proprietary gene therapy technologies for both rare and prevalent ocular disease."

Dr. Forbes continued, "I am very proud of the team at MeiraGTx who over the past decade has built one of the industry's most comprehensive genetic medicine companies - from capsid screening, promoter engineering and vector optimization, to clinical development and in-house GMP manufacturing, allowing us to accelerate the development of next-generation genetic medicines from concept to clinic with exceptional speed and quality. We are particularly pleased that Lilly, a global leader in development and commercialization of innovative medicines, has chosen to partner with us in this area of high unmet need, and shares our dedication to bringing truly life changing therapies to patients with otherwise intractable conditions."

"Ophthalmology is an emerging area of interest for Lilly," said Andrew Adams, Lilly group vice president, Molecule Discovery. "We are excited to partner with MeiraGTx to bring transformative treatments to patients around the world suffering from eye diseases, starting with AAV-AIPL1, which has shown the unprecedented ability to restore vision in children who were born legally blind."

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

About AIPL1

AAV-AIPL1 is an investigational genetic medicine for the treatment of one of the most severe forms of Leber congenital amaurosis 4 (LCA4) owing to genetic deficiency of Aryl-hydrocarbon-interacting protein-like 1 (*AIPL1*). It is delivered via subretinal injection to children, and through a one-time administration, AAV-AIPL1 is designed to deliver functional copies of the *AIPL1* gene to cone and rod photoreceptors in the central retina, to restore vision. AAV-AIPL1 has been granted orphan drug as well as rare pediatric disease designation (RPDD) by the FDA and orphan designation by the European Commission.

About Riboswitch

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.

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 success of the research to be performed under the collaboration agreement, the development of our ophthalmology product candidates and the development of our manufacturing technology, the reporting of data relating to our pre-clinical and clinical programs and the timing of results of data and regulatory matters, potential milestone and royalty payments and the achievement of such milestones and royalties, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate,” “eligible” 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 June 30, 2025, 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.

Contacts

Investors:

MeiraGTx

investors@meiragtx.com

or

Media:

Jason Braco, Ph.D.

LifeSci Communications

jbraco@lifescicomms.com