MeiraGTx Announces Two Posters at the European Society of Gene and Cell Therapy (ESGCT) 2025 Annual Congress

October 07, 2025

Multiple Posters Highlight the Breadth of Company's Novel Genetic Medicine and Cell Therapy Platforms

LONDON and NEW YORK, Oct. 07, 2025 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced the Company will exhibit two posters at the European Society of Gene and Cell Therapy (ESGCT) 2025 Annual Congress, which is being held from October 7-10, 2025, in Seville, Spain.

The posters are available on the Posters and Publications page of the Company's website.

The details of the poster presentations are as follows:

Poster Number: P0089
Abstract Title: *Novel AAV Capsids for Intravitreal Delivery Developed by Directed Evolution in Non-human Primate Eyes and Validated in Human Retinal Organoids*
Poster Session: Wednesday 8 October from 14:00 to 15:30 CEST
Abstract:
We performed an *in vivo* directed evolution capsid screen in non-human primates to identify novel capsids that target the back of the eye. This involved administering a diverse library of AAV capsid variants intravitreally into the eyes of NHPs.

The library was constructed by generating a variety of AAV capsid variants through the insertion of random peptides at a specific site in the capsid. Specifically, a 7-mer peptide display library based on AAV2 was created, with a 7-mer peptide inserted at amino acid 588, flanked by a 5' AAA linker and a 3' AA linker.

After administration, the fovea was excised, and retinal cells were harvested. By excluding the fovea, which most capsids readily transduce, we aimed to identify capsids with a broader transduction profile, extending to the perimacular region and beyond. Viral genomes were recovered and sequenced to identify the most efficient variants. By analysing the recovered viral genomes, we identified the top-performing capsids for further development.

From a library of ~ 1E7 variants, the top-performing capsids were selected. These candidates were validated and compared head-to-head against AAV2.7m8 and the parental AAV2 in our human retinal organoid platform using live cell imaging, flow cytometry, histology, and single-cell RNA sequencing. We identified variants with significantly higher transduction efficiency compared to the parental serotype, AAV2, and AAV2.7m8. Deep transcriptional profiling using single-cell RNA sequencing demonstrated over 2-fold higher transduction efficiency of our novel capsids compared to AAV2.7m8. Top performers were further validated and characterized in human iPSC-derive RPE where our novel capsids showed higher transduction efficiency compared to other natural and engineered serotypes. In addition, *in vivo* studies in mice using absolute quantification of total retinal expression in intravitreally injected retinas, overall expression levels of our novel capsids were higher than AAV2.7m8.

These novel AAV capsids have potential applications in treating various ocular diseases, including inherited retinal disorders and age-related macular degeneration. Their enhanced efficiency makes them exceptional candidates for gene therapy approaches requiring precise targeting of retinal cells and high overall levels of retinal transduction and expression. The ability to transduce a broad range of retinal cell types with high efficiency opens up new possibilities for treating complex retinal diseases that affect multiple cell types in the retina.

Poster Number: P0170
Abstract Title: *Scale-up of a Perfusion-Based AAV Manufacturing Process*
Poster Session: Thursday 9 October from 14:00 to 15:30 CEST
Abstract:
Building upon prior development of MeiraGTx's adeno-associated viral vector (AAV) upstream manufacturing platform, this work presents the scale-up of our perfusion-based AAV production process to 40L bioreactors. MeiraGTx's perfusion-based upstream process was optimized and modulated through the choice of transfection reagents, AAV production enhancers, and transfection parameters, in 250mL stirred tank reactors. Operating a perfusion-based process at bench-scale was found to increase volumetric VG yield by up to 120% and reduce plasmid DNA usage by up to 50%, without compromising on AAV productivity and product quality, resulting in a reduction in the cost-of-goods per dose of up to 2.2-fold. The work presented here focuses on the translation of bench-scale process parameters to the 40L manufacturing scale with a focus on maintaining process efficiency and product quality, to demonstrate a perfusion-based process as a viable approach for clinical and commercial AAV manufacturing.

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, , 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 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.

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