



MeiraGTx Announces Four Posters at the European Society of Gene and Cell Therapy (ESGCT) 2026 Annual Congress and the Network of Excellence for ALS (NEALS) Annual Meeting

October 27, 2026

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

LONDON and NEW YORK, Oct. 27, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTx), a vertically integrated, clinical-stage genetic medicines company, today announced the Company will exhibit three posters at the European Society of Gene and Cell Therapy (ESGCT) 2026 Annual Congress, which is being held from October 27-30, 2026, in Hamburg, Germany and one poster at the Network of Excellence for ALS (NEALS) Annual Meeting which is being held from October 27-30, 2026, in Clearwater, FL.

The posters will be available on [the Posters and Publications page](#) of the Company's website after their respective conference presentation times.

The details of the poster presentations are as follows:

ESGCT Annual Congress:

Poster Number: P0096

Abstract Title: *A commercial in-vitro biological activity potency assay for AAV-AQP1 treatment of Xerostomia*

Poster Session: Thursday, October 29, from 14:00 to 16:00 CET

Abstract:

Xerostomia, resulting from ionizing radiation treatment for head and neck cancer or Sjögren's syndrome is associated with irreversible salivary gland damage, oral dryness, impaired chewing and swallowing, and reduced quality of life. MeiraGTx's rAAV-AQP1 gene therapy product is being developed to restore localized water movement in damaged salivary glands through expression of human aquaporin 1 (hAQP1), a constitutively active membrane water channel that promotes osmotic water transport.

This study describes the development and optimization of a biological activity potency assay to quantify the water movement in a surrogate cell line after treatment with our product rAAV-AQP1 via fluorescence-based plate reader. The assay is based on calcein quenching, in which fluorescently labelled cells are exposed to a hyperosmotic sugar gradient, resulting in a cell volume change by water efflux. The rate of water transport reaction can be calculated by measuring and analyzing the changes in fluorescence. Compared with the previously used methods, the plate reader approach provides a rapid kinetic readout and enables quantitative assessment with improved throughput, precision, and robustness.

Preliminary optimization studies identified T98G cells as the most suitable assay model due to lower endogenous AQP1 activity, broad linear range, and compatibility with complementary expression studies, despite higher transducibility observed in HeLa cells. Samples transduced with rAAV-AQP1 demonstrated a significantly faster reduction in cell volume compared with untransduced controls, consistent with increased AQP1-mediated water transport. Observed rate constants remained consistent across different sugar concentrations at equivalent multiplicities of infection, supporting assay robustness across osmotic conditions. Intra-assay variability remained below 20% CV throughout assay evaluation. The assay is being validated for key analytical performance characteristics to ICH Q2(R2) standards including linearity, precision, specificity, robustness, and stability.

Together, this work establishes a scalable and biologically relevant *in-vitro* mode of action potency assay to support rAAV-AQP1 product development, Quality Control release and future commercial licensing.

Poster Number: P0099

Abstract Title: *Denaturing size exclusion chromatography-MS for characterisation of AAV and assessment of CQAs*

Poster Session: Wednesday, October 28, from 13:30 to 15:30 CET

Abstract:

Adeno-associated virus (AAV) is a gene therapy vector composed of a single-stranded DNA genome (~4.7 kb) encapsulated within a protein capsid. The characterization of the viral proteins (VP1–VP3) forming the capsid is crucial for understanding potential alterations in stoichiometry and post-translational modifications (PTMs) which may occur during manufacturing. These changes can impact critical quality attributes (CQAs) and critical process parameters (CPP's) such as transduction efficiency, genome packaging and vector stability. At MeiraGTx, this is currently assessed using reverse-phase LC-MS; however, the shallow gradient employed limits separation of viral proteins. Capillary Electrophoresis–SDS (CE-SDS) is also used to assess VP stoichiometry, although it reveals a pre-peak hypothesized to arise from VP3 truncation during sample preparation. Denaturing size exclusion chromatography–MS (dSEC-MS) has emerged as a promising alternative assay that may improve viral protein separation while minimizing artefacts introduced during sample preparation.

This study describes the development of a dSEC-MS method for characterization of AAV drug product at MeiraGTx and its comparison with reverse-phase LC-MS. Buffer optimization was performed to achieve baseline separation of VP1/VP2/VP3 and improve analytical sensitivity. The results demonstrate that dSEC-MS provides improved resolution of viral proteins for AAV2, AAV5, and AAV8 relative to reverse-phase LC-MS while maintaining comparable deconvolution performance for identification of PTMs. The method also exhibited strong sensitivity and linearity. In addition, it was applied to characterize the CE-SDS pre-peak, confirming a cleavage site within the C-terminal region of capsid proteins. Furthermore, analysis of VP protein cross-linking via disulfide bonding revealed that VP3–VP3 disulfide formation is predominant during capsid assembly, suggesting that variations in disulfide bonding may influence AAV capsid stability.

Overall, dSEC-MS provides a more robust, artefact-reduced approach for AAV capsid protein analysis, enabling improved assessment of VP stoichiometry and structural heterogeneity. Its platform applicability across multiple serotypes supports its use as a valuable analytical tool in gene

therapy process development and product characterization.

Poster Number: P0508

Abstract Title: *AAV-NRF2 rescues RPE cell function and viability in human iPSC-derived and mouse models of oxidative stress*

Poster Session: Thursday, October 29, from 14:00 to 16:00 CET

Abstract:

Dry AMD accounts for 85-90% of all AMD cases with few therapeutic options available to patients. While a variety of environmental and genetic risk factors contribute to the onset of AMD, oxidative stress is a major underlying pathology of early AMD and its progression. Here we present evidence for the therapeutic efficacy of an AAV vector expressing NRF2 in iPSC-retinal pigmented epithelium (RPE) and a mouse model of oxidative stress.

NRF2 (nuclear factor erythroid 2-related factor 2) is a master transcription factor regulating cellular defense against oxidative stress by controlling the expression of over 250 antioxidant and detoxification genes. We have designed AAV vectors expressing NRF2 under the control of a ubiquitous CAG promoter or novel tissue-specific RPE (retinal pigmented epithelium) promoters and packaged them into engineered AAV capsids with high transduction efficiency in human RPE cells.

Mature iPSC-RPE cells treated with tert Butyl hydroperoxide (tBH), upregulate oxidative stress genes and undergo dose-dependent loss of function – quantified here by a reduction in trans epithelial resistance (TER) and internalization of bovine photoreceptor outer segments (POS). At high tBH doses, RPE undergo apoptosis.

Bulk RNAseq data show how AAV-NRF2 transduced RPE cells are able to mount a significantly stronger antioxidative stress response to tBH. As a result, NRF2 transduced RPE has significantly increased resilience with a complete rescue of viability, phagocytic function and barrier integrity (Trans-Epithelial Resistance; TER) following tBH doses which induce significant loss of function in non-transduced cells.

Our AAV-NRF2 vectors were also tested in a mouse model of dry AMD. Subretinal delivery of AAV-NRF2 did not induce any significant structural changes in the retina four weeks post treatment – indicating a clean safety profile. AAV-NRF2 significantly reduced sodium iodate induced RPE atrophy (F-actin staining) and completely prevented sodium iodate induced loss of electrophysiological responses in the retina.

Together, our results show that our AAV-NRF2 vectors have the ability to protect RPE from oxidative stress and as such show significant therapeutic potential for the treatment of dry AMD.

NEALS Annual Meeting:

Poster Number: 53

Abstract Title: *The Development and Optimization of AAV-mediated Gene Medicine as Potential ALS Therapeutics*

Poster Session 1: Wednesday, October 28, from 16:30 to 18:30 ET

Abstract:

Background:

UPF1, a central regulator in nonsense-mediated RNA decay (NMD), and CNTFR α , a cytokine receptor enriched in skeletal muscle that is upregulated after denervating injuries, are therapeutic candidates that may be agnostic to ALS patients' genetic background.

Objective:

MeiraGTx presents the development and optimization of AAV-hUPF1 and AAV-hCNTFR α , two pre-clinical gene therapies.

Methods:

We optimized the construct sequence to improve AAV packaging and increase expression to lower therapeutic dose requirements for clinical translation. We tested for efficacy in both *in-vitro* and *in-vivo* models of ALS.

Results/Impact:

For AAV-hUPF1, we reduced the size of the original construct by 1.5kb while improving therapeutic efficacy in both *in vitro* and *in vivo* models. The optimized AAV-hUPF1 effectively mitigated toxicity in TDP-43 and C9orf72 iNeuron models and restored function of neurons derived from patient at lower doses than used for the original. Mechanistically, UPF1 target engagement was confirmed in transduced C9orf72 patient-derived neurons by the downregulation of known NMD targets. In a FUS mouse model of ALS, optimized AAV-hUPF1 significantly improved motor neuron survival to levels comparable to wild-type controls at approximately 36% transduction.

For AAV-hCNTFR α , we implemented vector optimization that included unique combinations of ubiquitous and muscle-specific promoters, enhancers, and key 3'-UTR regulatory elements. We established effective *in vitro* transgene expression in HEK293T and C2C12 cells with our optimized constructs and demonstrated that CNTFR α is expressed in both the cell lysate and conditioned media, indicating both membrane-bound and soluble CNTFR α are present. We utilized IM injection of AAV-CNTFR α into the mouse gastrocnemius to establish *in vivo* protein expression, relative solubility, and biodistribution of locally administered AAV-hCNTFR α .

Conclusion/Next Steps:

Collectively, the data from *in vitro* and *in vivo* studies using FUS, TDP-43, and C9orf72 models demonstrate that hUPF1 holds significant potential for treating the majority of patients with ALS. We continue to develop both programs with additional efficacy validation and the development of biological activity assays to support the manufacturing of the AAV gene medicine.

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, radiation-induced xerostomia, and Parkinson's disease. 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, 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 10 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 our pre-clinical and clinical data, reporting of such data and the timing of results of data, 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, 2026, 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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