MeiraGTx Announces Oral Presentation at ObesityWeek 2026

November 16, 2026

Presentation highlights the Company's AAV-BDNF program for the treatment of severe early-onset obesity

LONDON and NEW YORK, Nov. 16, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced the Company will have an oral presentation at ObesityWeek, in Washington, DC, at 8:30 am ET at the Gaylord National Resort & Convention Center. Hosted annually by The Obesity Society (TOS), ObesityWeek is a premier international scientific conference focused on the multi-disciplinary science, treatment, and prevention of obesity.

The presentation will be available on the Posters and Publications page of the Company's website.

Session Details: Brain Biology, Gene Therapy, and Treatment Outcomes Oral Session
Monday November 16, 2026
8:30 AM- 9:30 AM ET
Presentation Title: *Restoration of the Leptin-Melanocortin Pathway via Hypothalamic AAV-BDNF Rescues Syndromic Obesity*
Speaker: Josefa Sullivan
Location: Gaylord National Resort & Convention Center: Potomac AB, Ballroom Level, National Harbor, Maryland
Abstract:
Restoration of the Leptin-Melanocortin Pathway via Hypothalamic AAV-BDNF Rescues Syndromic Obesity
Chenjin Jin, Jihye Kim, Betty Bai, Jared Sills, Jeroen Bastiaans, Alisa Panichkina, Gavin Lagani, Ann Lettko, Casey Lutnick, Molly Lutolf, Karen Wei, T.D. Barbara Nguyen-Vu, Francis S. Lee, Josefa M. Sullivan
Background: Brain-derived neurotrophic factor (BDNF) is a critical mediator of the leptin-melanocortin signaling pathway, acting downstream of the melanocortin-4 receptor (MC4R) to regulate energy expenditure and satiety. BDNF haploinsufficiency is an ultra-rare pathology defined by the loss of one functional copy of the *BDNF* gene, separate from common polymorphisms such as Val66Met. While complete BDNF deletion is lethal in mice and unobserved in humans, haploinsufficiency causes highly penetrant clinical manifestations. These mutations impair MC4R-mediated signaling, resulting in severe rapid-onset obesity and intractable hyperphagia. Because BDNF is a pleiotropic neurotrophin, its deficiency also impacts broader neuronal plasticity which leads to a distinct neurobehavioral profile including variable intellectual disability, increased nociceptive thresholds, and significant physical aggression. There is a critical unmet need for a mechanistically targeted therapy for BDNF haploinsufficiency that restores metabolic and behavioral homeostasis.
Methods: AAV-BDNF was delivered via a single unilateral injection into the left ventromedial hypothalamus (VmH) of diet-induced (DIO) and genetic $BDNF^{+/-}$ obesity mouse models. The experimental design controlled for genotype, diet, and AAV treatment. To optimize clinical translation, we investigated capsid dose-expression interactions using in situ hybridization (ISH), correlating vector spread within the hypothalamus to efficacy and safety outcomes. Systemic safety was evaluated via complete blood count (CBC) and serum toxicity analysis. Metabolic effects were assessed via longitudinal bodyweight and glucose tolerance, while neurobehavioral phenotypes were quantified using automated pose estimation and neural network-based clustering.
Results: In the DIO model, a single administration of AAV-BDNF established metabolic homeostasis for over 14 months following initial weight loss. In $BDNF^{+/-}$ mice, treatment led to significant weight stabilization and normalized glucose tolerance compared to controls ($p < 0.05$). ISH analysis confirmed that ultra-low unilateral doses were sufficient for therapeutic rescue. This physiological recalibration appeared self-limiting, as weights plateaued at normal levels without uncontrolled cachexia. CBC and serum toxicity results demonstrated a favorable safety profile. Importantly, this intervention significantly decreased aggressive and perseverative chasing behaviors without impairing naturalistic pro-social interactions.
Conclusion: A single, unilateral VmH administration of AAV-BDNF provides a potent, durable rescue of metabolic and neurobehavioral phenotypes in models of BDNF haploinsufficiency. Addressing both life-threatening obesity and debilitating neurobehavioral symptoms with a single, low-dose intervention could fundamentally improve quality of life and long-term outcomes for patients with these rare genetic disorders.

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 facilities 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 September 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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