MeiraGTx Announces the Acquisition of Botaretigene Sparoparvovec (bota-vec) for the Treatment of X-linked Retinitis Pigmentosa (XLRP)

April 16, 2026

- *Company entered into an asset purchase agreement with Johnson & Johnson (J&J) to acquire all interests in botaretigene sparoparvovec (bota-vec) for the treatment of X-linked retinitis pigmentosa (XLRP)*
- *MeiraGTx intends to immediately pursue global regulatory filings for approval of bota-vec*

LONDON and NEW YORK, April 16, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced that it has entered into an asset purchase agreement with Johnson & Johnson* (J&J) to acquire all interests in bota-vec for the treatment of XLRP.

“We are extremely pleased to have reacquired bota-vec for the treatment of XLRP,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “This is a unique opportunity to gain an asset at this stage in development with data supporting a meaningful benefit in patients with no alternative treatment, many of whom are waiting for this life changing therapy and hoping for expeditious approval.”

Dr. Forbes continued, “We are intimately familiar with AAV-RPGR, having collaborated with J&J during the development of the program from Phase 1 onward. Importantly, from a regulatory CMC perspective, as the commercial manufacturer of the product, we have completed PPQ with the CMC datasets for filing with global regulators. We intend to start filing BLA and MAA in the U.S., EU and Japan as soon as possible.”

Jason Menzo, CEO of Foundation Fighting Blindness, stated, “For patients living with X-linked retinitis pigmentosa, the need for treatment options is clear and urgent. The data from the LUMEOS Phase 3 study of bota-vec, reflected in both objective measures and patient-reported outcomes, point to real improvements in vision. Our focus is on advancing safe and effective therapies that matter to patients, and we are excited to be working with MeiraGTx and regulators to bring this potential treatment to the global XLRP community.”

Rachel Huckfeldt, M.D., Ph.D., director of Inherited Retinal Disorders Clinical Trials at Mass Eye and Ear and a site principal investigator who has led multiple botaretigene sparoparvovec clinical trials at the hospital, added, “There is clear unmet need for individuals with X-linked retinitis pigmentosa. The Phase 3 trial demonstrated meaningful improvements across multiple outcome measures with 10- and 15-letter gains in low luminance visual acuity as one example. Many participants were able to provide examples from their daily lives of the real-world impact of these gains. These results provide hope for individuals with XLRP, and they warrant further consideration by regulatory agencies.”

The IRD community is a concentrated one with 40-50 centers of excellence in the EU, U.S. and Japan caring for approximately 80% of IRD patients. Through the initial formation of MeiraGTx in 2015 in collaboration with University College London (UCL) and the Moorfields Eye Hospital, MeiraGTx has close relationships with most of the KOLs at these leading sites, with 32 of these sites participating in the Phase 3 LUMEOS study of bota-vec.

Since the release of the LUMEOS Phase 3 data, MeiraGTx has heard from numerous investigators about the clinically meaningful benefit that bota-vec has afforded a significant number of patients who participated in the study, with unprecedented improvements demonstrated in each of the 3 domains of vision. Investigators around the world are enthusiastically supporting filing for regulatory approval of bota-vec in order to allow access to treatment for the patients they are seeing in their clinic today who are waiting for this potentially life-changing therapy.

In re-acquiring bota-vec, MeiraGTx intends to expeditiously file for approval in the U.S. and EU with the aim of a potential launch in 2027. With the data from the AQUAx 2 pivotal study of AAV-hAQP1 for the treatment of grade 2/3 radiation-induced xerostomia expected in the second quarter of 2027, the Company’s intent is to become a commercial stage company with two potential products launching over the next 2 years into concentrated markets, both addressing severe unmet needs and both being disease modifying in areas where patients have no treatment options.

Bota-vec Asset Purchase Terms:
MeiraGTx will pay J&J a $25 million upfront cash payment and a one-time regulatory and commercial milestone tied to U.S. approval and U.S. sales performance of bota-vec for the treatment of XLRP, as well as a high double-digit royalty on global net sales starting in mid-2029.

Bota-vec for the Treatment of X-linked Retinitis Pigmentosa (XLRP):

- XLRP is a rare inherited retinal disease with early onset and progressive degeneration to complete blindness in the third decade of life. There are currently no treatment options.
- There are >20,000 XLRP-RPGR patients in the U.S. and EU.
- The Phase 3 LUMEOS study was a global randomized study (n=95). All patients were treated bilaterally.
- Data from the Phase 3 LUMEOS trial of botaretigene sparoparvovec (bota-vec) for the treatment of XLRP was presented at the Foundation Fighting Blindness 2025 Retinal Therapeutics Innovation Summit.
- Following the release of the compelling Phase 3 data at their summit, the Foundation Fighting Blindness issued a public letter to J&J strongly supporting the filing and ultimate approval of this treatment for XLRP and stating that it had a remarkable benefit for many of the patients treated.

Phase 3 LUMEOS Study Data:

- The novel primary endpoint to assess the effect of bilaterial treatment with bota-vec on functional vision as measured by a Visual Mobility Assessment (VMA), or maze, did not meet statistical significance. However, it was directionally supportive with treated subjects 2.4x more likely to respond than untreated subjects.
- LLQ PRO showed significant benefit in mobility and dim light function, which is what the VMA tested, indicating the maze was not sensitive enough to capture these benefits.
- The data from the secondary endpoints were very strong, with clinically meaningful and statistically significant improvements demonstrated in each of the three domains of vision.

Additional Functional Vision Endpoints:

- Significant change in the LLQ Extreme lighting domain score, LS mean **p=0.006;** statistically significant improvements in questions relating to mobility (**p= 0.001),** general dim lighting **(p= 0.007)** and emotional distress **(p= 0.019)**
- IVI-A: significant improvement in total score vs control at week 52 **p=0.024** with greater significance in the emotional wellbeing questions **(p=0.005)**

Retinal Function:

- All measures of retinal sensitivity showed highly significant difference between treated and untreated groups
- Pointwise responders (repeated 5-point 7-decibel) in the Central 30 degrees **p=0.001**
- Pointwise responders (repeated 5-point 7-decibel) in the Full visual field **p=0.001**
- Change in Mean retinal sensitivity in the central 10 degrees, **p=0.001**
- Change in Mean retinal sensitivity full field 90 degrees **p= 0.004**

Visual Function:

- Change in Low luminance visual acuity (LLVA, EDTRS letters) LS mean **p=0.003**
- 45% of treated patients gained >10 letters in LLVA
- 20% of treated patients achieved >15 letters in LLVA

Multi-endpoint Responder Analysis:

- 40% (22/55) of treated patients showed improvement in ≥2 endpoints each in different domains of vision compared to 0% in the control group. This was consistent whichever endpoints were tested.

Safety:

- Safety profile of bota-vec was as expected and manageable, no new safety signals in the Phase 3 with improved inflammatory profile compared to the Phase 1/2.

CMC:

- MeiraGTx is the commercial manufacturer of bota-vec and have successfully completed PPQ. The Company has received a commercial license from the MHRA for its London manufacturing facility, as well as a commercial license for the Company's QC facility that conducts the release and stability assays for the product in Shannon, Ireland. The Company currently has several hundred vials of product in hand that on QP release can be used to treat patients immediately following approval.

The FDA has granted Fast Track and Orphan Drug Designations to bota-vec, and the regulatory authorities in the EU have granted Priority Medicines, or PRIME, advanced therapy medicinal product, or ATMP, and Orphan Drug Designations to bota-vec.

*Janssen Pharmaceuticals, Inc., a Johnson & Johnson company

MeiraGTx has a licensing agreement with Mass Eye and Ear. Dr. Huckfeldt does not have a personal financial interest in bota-vec or MeiraGTx.

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 uses 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, 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 anticipated payments under the Asset Purchase Agreement, execution of the obligations under the Asset Purchase Agreement and estimates regarding the market size for bota-vec, 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 Annual Report on Form 10-K for the year ended December 31, 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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