



REGENXBIO Announces Regulatory Update on RGX-121 for MPS II

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ROCKVILLE, Md., Aug. 24, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today provided an update on its investigational gene therapy, RGX-121 (clevidomine lanparvovec), for the treatment of Mucopolysaccharidosis type II (MPS II), also known as Hunter Syndrome. The U.S. Food and Drug Administration (FDA) placed a clinical hold on RGX-121 following the discovery of asymptomatic spine MRI findings in five participants in the CAMPSITE® study; REGENXBIO does not expect to resubmit the RGX-121 Biologics License Application (BLA) in the near term.

"We believe these findings are unique and limited to our Hunter Syndrome program, and require longer-term follow-up and additional data analysis to assess the benefit-risk profile of RGX-121," said Curran Simpson, President and CEO of REGENXBIO. "We remain focused on our Duchenne and retinal disease candidates, which utilize a different capsid and routes of administration, with near-term catalysts that are on track, including the planned submission of the Duchenne BLA this quarter and the wet AMD topline pivotal data announcement in the fourth quarter."

All five participants continue to do well clinically and have demonstrated overall stability to improvement on neurocognitive and neurobehavioral assessments. The findings were identified through an expanded MRI monitoring plan, implemented by REGENXBIO a few months ago, following the clinical hold related to RGX-111. The enhanced monitoring included both brain and spine MRI and identified asymptomatic findings of either a small nodule or a small cystic mass in spine MRIs of five participants who received intracisternal or intraventricular RGX-121 approximately three to six years ago. Investigators deemed these findings to be nonserious and radiologists believe they are likely benign. There is no clinical or pathological evidence to confirm the nature or causation of the spine MRI findings. No brain nodules or masses were identified on any brain MRIs.

Because spine MRI is not normally conducted for MPS in clinical practice or trials, the underlying prevalence and clinical significance of these types of asymptomatic findings in this patient population is unknown. Investigators plan to continue to observe these patients with periodic imaging only.

"Boys with neuropathic MPS II experience a multitude of neurodevelopmental and systemic effects. While imaging natural history is limited for this ultra-rare disease, I believe that asymptomatic, likely benign findings like these may be inherent to the impact of Hunter Syndrome throughout the body," said Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, Porto Alegre, Brazil. "I am pleased that these patients are doing well and remain asymptomatic."

The Company and its partner NS Pharma are evaluating additional patient imaging and longer term follow up data, and will incorporate FDA feedback, including the full clinical hold letter once received, into next steps for RGX-121.

About Mucopolysaccharidosis Type II (MPS II)

MPS II, or Hunter Syndrome, is a rare, X-linked recessive disease caused by a deficiency in the lysosomal enzyme I2S leading to an accumulation of glycosaminoglycans (GAGs), including heparan sulfate (HS) in tissues which ultimately results in cell, tissue, and organ dysfunction, including in the CNS. Approximately 2,000 patients worldwide are diagnosed with MPS II, with more than 500 babies born annually around the world with the disease. The majority of MPS II patients have severe forms of the disease, with which early developmental milestones may be met, but developmental delay is readily apparent by 18 to 24 months. CSF HS is a key disease biomarker in MPS II patients. Among its quantified disaccharides, D2S6 has been shown to correlate with neurocognitive manifestations, highlighting its role as a clinically relevant biomarker of disease severity and therapeutic response.

About RGX-121 (clevidomine lanparvovec)

RGX-121 is a one-time investigational gene therapy for the treatment of boys with MPS II, designed to deliver the iduronate-2-sulfatase (*IDS*) gene to the central nervous system (CNS). Delivery of the *IDS* gene within cells in the CNS could provide a permanent source of secreted iduronate-2-sulfatase (I2S) protein beyond the blood-brain barrier, allowing for long-term cross correction of cells throughout the CNS. RGX-121 expressed protein is structurally identical to normal I2S.

RGX-121 has received Orphan Drug Product, Rare Pediatric Disease, Fast Track and Regenerative Medicine Advanced Therapy (RMAT) designations from the U.S. Food and Drug Administration and advanced therapy medicinal products (ATMP) classification from the European Medicines Agency.

ABOUT REGENXBIO Inc.

REGENXBIO is a biotechnology company on a mission to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the field of AAV gene therapy. REGENXBIO is advancing a late-stage pipeline of one-time treatments for rare and retinal diseases, including RGX-202 for the treatment of Duchenne; surabgene lanparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie, and RGX-121 (clevidomine lanparvovec) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA®. REGENXBIO's investigational gene therapies have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.REGENXBIO.com.

FORWARD-LOOKING STATEMENTS

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "assume," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform

with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, FDA's review process, the success of clinical trials conducted by REGENXBIO, the ability to obtain and maintain regulatory approval of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2025, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the SEC and are available on the SEC's website at WWW.SEC.GOV. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. Except as required by law, REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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