REGENXBIO Announces Regulatory Update on Ultra Rare MPS Programs

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ROCKVILLE, Md., Jan. 28, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the U.S. Food and Drug Administration (FDA) placed a clinical hold on its investigational gene therapy, RGX-111, for the treatment of MPS I, also known as Hurler syndrome, following preliminary analysis of a single case of neoplasm (intraventricular CNS tumor) in a participant treated in its Phase I/II study. The FDA also placed a clinical hold on RGX-121, for the treatment of MPS II, also known as Hunter Syndrome, citing the similarities in products, study populations, and shared risk between the clinical studies.

The case was identified during a routine brain MRI of an asymptomatic five-year-old participant who received intracisternal RGX-111 four years prior. Preliminary genetic analysis of the resected tumor detected an AAV vector genome integration event associated with overexpression of a proto-oncogene (PLAG1), which is known to be susceptible to chromosomal rearrangements. The investigation to determine if this SAE is drug related is ongoing. The causality has not been established. The participant continues to be asymptomatic, with positive developmental advancements noted by the treating physician. No evidence of neoplasm has been reported in the nine other participants treated with RGX-111 nor in the 32 participants treated with RGX-121.

"We are surprised by FDA's decision to place our RGX-121 program on hold while the investigation of this single, inconclusive incident in RGX-111 continues," said Curran Simpson, President and CEO of REGENXBIO. "These are separate therapies, and the positive safety profile of RGX-121 in more than 30 patients treated, including those dosed nearly seven years ago, remains unchanged. Patient safety is our top priority, and we, our investigators, and the patient community remain confident in the benefit-risk ratio of RGX-121 and are highly encouraged by the meaningful efficacy profile demonstrated in the pivotal trial. RGX-121 presents an opportunity to address the urgent, significant unmet medical need in this ultra-rare disease community, and continued delay means continued neurodevelopmental decline in boys with MPS II."

REGENXBIO has not yet received the full clinical hold letter and awaits additional details from the FDA.

About RGX-121 (clemidsogene lanparvovec)
RGX-121 is a potential one-time 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 FDA and advanced therapy medicinal products (ATMP) classification from the European Medicines Agency.

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 central nervous system (CNS). In severe forms of the disease, early developmental milestones may be met, but developmental delay is readily apparent by 18 to 24 months. Specific treatment to address the neurological manifestations of MPS II remains a significant unmet medical need. Key biomarkers of I2S enzymatic activity in MPS II patients include its substrate CSF HS D2S6, which has been shown to correlate with neurocognitive manifestations of the disorder.

About RGX-111
RGX-111 is designed to use the AAV9 vector to deliver the α-l-iduronidase (IDUA) gene to the central nervous system (CNS). Delivery of the IDUA gene within the cells in the central nervous system (CNS) could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. By providing rapid IDUA delivery to the brain, RGX-111 could potentially help prevent the progression of cognitive deficits that otherwise occurs in MPS I patients. RGX-111 has received orphan drug product, rare pediatric disease and Fast Track designations from the FDA.

About Mucopolysaccharidosis Type I (MPS I)
MPS I is a rare autosomal recessive genetic disease caused by a deficiency in the lysosomal enzyme alpha-L-iduronidase (IDUA), 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 central nervous system (CNS). This can include excessive accumulation of fluid in the brain, spinal cord compression, and cognitive impairment. MPS I is estimated to occur in 1 in 100,000 births. Current disease modifying therapies for MPS I include hematopoietic stem cell transplant (HSCT) and enzyme replacement therapy with a recombinant form of human IDUA administered intravenously. However, intravenous enzyme therapy does not treat the CNS manifestations of MPS I, and HSCT can be associated with clinically significant morbidity and mortality. Key biomarkers of IDUA enzymatic activity in MPS I patients include its substrate heparan sulfate (HS), which has been shown to correlate with neurocognitive manifestations of the disorder.

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; NAVSUNLI for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku; and surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie. 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, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timing or likelihood of payments from AbbVie or Nippon Shinyaku, the monetization of any priority review voucher, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance 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, 2024, 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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