



REGENXBIO Announces FDA Review Extension of BLA for RGX-121 to Treat Patients with MPS II

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- *RGX-121 would be the first and only potential one-time commercially-available therapy designed to directly address the underlying genetic cause of Hunter syndrome, if approved*
- *Commercial launch plans remain on track*
- *REGENXBIO plans to present updated pivotal data during the ICIEM meeting in September 2025*

ROCKVILLE, Md., Aug. 18, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the U.S. Food and Drug Administration (FDA) extended its review timeline of the Biologics License Application (BLA) for clemidsogene lanparvovec (RGX-121) for the treatment of Mucopolysaccharidosis II (MPS II), also known as Hunter syndrome. The Prescription Drug User Fee Act (PDUFA) goal date has been extended from November 9, 2025 to February 8, 2026.

The extension follows the Company's submission of longer-term clinical data for all patients in the pivotal study of RGX-121 (n=13) in response to an FDA information request. These positive 12-month clinical data are consistent with biomarker and neurodevelopmental data previously submitted on the same patients in the BLA and will be presented during the International Congress of Inborn Errors of Metabolism (ICIEM) in September 2025.

In August 2025, the FDA completed a pre-license inspection and bioresearch monitoring information inspection for the RGX-121 BLA with no observations. No safety-related concerns have been raised by the FDA during the BLA review.

"Boys with this rare, devastating disease have no treatment options to address neurodevelopmental decline, and the Hunter syndrome community is in urgent need for a therapeutic option with the potential to improve these patients' lives," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "We promptly provided the FDA with the information requested and expect the commercial launch plans remain on track."

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 RGX-121 (clemidsogene lanparvovec)

RGX-121 is a potential one-time AAV therapeutic 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.

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. 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 heparan sulfate (HS) D2S6, 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; clemidsogene lanparvovec (RGX-121) 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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