REGENXBIO Announces FDA Acceptance and Priority Review of the BLA for RGX-121 for MPS II

May 13, 2025 07:05 AM EDT

- *FDA assigns PDUFA target action date of November 9, 2025*
- *RGX-121 on track to be the first gene therapy and one-time treatment for MPS II*
- *Partner Nippon Shinyaku to lead commercialization upon potential approval*
- *REGENXBIO to lead commercial manufacturing and supply chain*

ROCKVILLE, Md., May 13, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the U.S. Food and Drug Administration (FDA) has accepted for review the Biologics License Application (BLA) seeking accelerated approval for clemidsogene lanparvovec (RGX-121) for the treatment of Mucopolysaccharidosis II (MPS II), also known as Hunter syndrome.

The FDA granted the BLA Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of November 9, 2025.

"Acceptance of the RGX-121 BLA marks an exciting milestone on our path to bring the MPS II patient community a one-time treatment with the potential to address both the neurodevelopmental and systemic effects of Hunter syndrome," said Curran M. Simpson, President and Chief Executive Officer of REGENXBIO. "Supported by positive biomarker data and long-term outcomes, RGX-121 has the potential to be a first-in-class gene therapy that could dramatically transform the MPS II treatment landscape and reduce the significant burden patients and families currently face with weekly enzyme replacement therapy."

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.

Under the strategic partnership announced in January 2025, following potential FDA approval, RGX-121 will be commercialized by NS Pharma, Inc., a wholly-owned subsidiary of Nippon Shinyaku, in the U.S. Approval of RGX-121 could result in receipt of a Priority Review Voucher (PRV). REGENXBIO retains all rights to, and 100 percent of any proceeds related to the potential sale of, the PRV for RGX-121.

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.

Zolgensma® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

CONTACTS:
Dana Cormack
Corporate Communications
Dcormack@regenxbio.com

George E. MacDougall
Investor Relations
IR@regenxbio.com

View original content to download multimedia:https://www.prnewswire.com/news-releases/regenxbio-announces-fda-acceptance-and-priority-review-of-the-bla-for-rgx-121-for-mps-ii-302453436.html

SOURCE REGENXBIO Inc.