REGENXBIO Announces Alignment with FDA on Path Forward for NAVSUNLI™ BLA Resubmission for Accelerated Approval; First Potential Gene Therapy for MPS II

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- *FDA confirmed no additional studies required, existing longer-term data from the CAMPSIITE® study will be reviewed on an expedited basis for approval via the accelerated approval pathway*
- *The Company expects to resubmit the BLA in Q3 2026*

ROCKVILLE, Md., June 22, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced it has aligned with the U.S. Food and Drug Administration (FDA) regarding the next steps needed for a potential accelerated approval of NAVSUNLI™ (clemidsogene lanparvovec-sngl, RGX-121), the only potential one-time treatment and gene therapy for Mucopolysaccharidosis II (MPS II), an ultra-rare neurodegenerative disease also known as Hunter syndrome.

Through a recent collaborative discussion, as part of the Company's appeal of the February 2026 NAVSUNLI Complete Response Letter, the FDA acknowledged the existing NAVSUNLI clinical data is sufficient to be considered for the accelerated approval pathway and that the Company does not need to enroll additional patients or conduct additional studies, including the FDA's previously recommended incorporation of an untreated control arm. The FDA asked the Company to request a Type A meeting to review existing longer-term biomarker and clinical data and to resubmit the Biologics License Application (BLA) following this meeting. The FDA stated that it would review REGENXBIO's resubmission on an expedited basis, with labeling discussions to begin shortly following the resubmission. The FDA emphasized its commitment to advancing new therapies for rare diseases under the accelerated approval pathway.

REGENXBIO expects the Type A meeting to take place in July and to resubmit the BLA rapidly following the meeting in Q3 2026.

"We are encouraged by recent signals from the new FDA leadership reinforcing a commitment to address the unique nature of rare diseases and use the accelerated approval pathway to bring transformative therapies to patients with serious, unmet medical needs," said Curran Simpson, President and CEO of REGENXBIO. "We will continue to work closely with the FDA and remain focused on bringing this important therapy to boys living with Hunter syndrome as quickly as possible."

"Hunter syndrome is a devastating, progressive disease with significant unmet medical needs and we are thrilled to see this progress for NAVSUNLI," said Scott Loiler, Ph.D., Chief Scientific Officer National MPS Society. "The MPS community urgently needs new treatment options, and we appreciate the FDA's willingness to use the accelerated approval pathway for rare diseases and expedite the review of NAVSUNLI so that the Hunter syndrome community may soon have access to a potentially transformative one-time treatment."

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 NAVSUNLI (clemidsogene lanparvovec- sngl)
NAVSUNLI 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. NAVSUNLI expressed protein is structurally identical to normal I2S.

NAVSUNLI 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 NAVSUNLI 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.

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 lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie, and NAVSUNLI™ (clemidsogene lanparvovec-sngl, RGX-121) 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. Zolgensma® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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