REGENXBIO Presents Positive Twelve-Month Pivotal Data from Phase I/II/III CAMPSIITE® Trial of RGX-121 for Treatment of MPS II

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- *12-month pivotal data further demonstrate the ability of one-time RGX-121 treatment to improve outcomes for patients with MPS II*
  - *>80% reduction in CSF levels of HS D2S6, key biomarker of MPS II brain disease, sustained through 1 year*
  - *Pivotal patients demonstrate continued skill acquisition or stability, stratified by baseline function, through 1 year*
- *Primary endpoint of CSF HS D2S6 reduction at week 16 met; strong correlation to neurodevelopmental outcomes at 1 year, supporting HS D2S6 as surrogate biomarker reasonably likely to predict clinical benefit*
- *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*

ROCKVILLE, Md., Sept. 5, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced new, positive data from the Phase I/II/III CAMPSIITE® trial of clemidsogene lanparvovec (RGX-121) for the treatment of patients with Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, at the International Congress of Inborn Errors of Metabolism (ICIEM) 2025. REGENXBIO submitted these longer-term pivotal results to the U.S. Food and Drug Administration (FDA) in response to an information request in the ongoing Biologics License Application (BLA) review of RGX-121.

"These positive biomarker and functional data provide further evidence of the long-term potential of RGX-121 to change the trajectory of Hunter syndrome for boys with this devastating, degenerative disease," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "The sustained reductions in CSF HS D2S6 and evidence of the strong correlation between biomarker level and neurodevelopmental improvement are highly encouraging as we look toward potential accelerated approval early next year."

"I am highly encouraged by the 12-month pivotal data and continued safety and efficacy profile of RGX-121," said Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, Porto Alegre, Brazil. "The vast majority of Hunter syndrome patients have no current treatment options to address the neurodevelopmental decline of this disease and are in urgent need of new therapies, and a one-time treatment option, like RGX-121, could make a meaningful impact on their lives."

Data Summary
In the pivotal phase of the CAMPSIITE trial (n=13), participants through one year sustained an 82% median reduction of cerebrospinal fluid (CSF) levels of heparan sulfate (HS) D2S6, a key biomarker of MPS II brain disease that is reasonably likely to predict clinical benefit. These longer-term data are consistent with previously reported topline pivotal results from the CAMPSIITE trial, which met its primary endpoint of proportion of participants with CSF HS D2S6 below maximum attenuated levels at week 16 with statistical significance ($p < 0.0001$). Similar results were previously reported for the pivotal dose in the dose-finding phase of the study, with 85% reductions of CSF HS D2S6 sustained through two years.

Positive neurodevelopmental outcomes were observed in the pivotal and dose-finding phases of the CAMPSIITE trial. Pivotal participants demonstrated neurodevelopmental skill acquisition or stability on all sub-scales of the Bayley Scales of Infant and Toddler Development, 3rd Edition (BSID-III) at one year.

New data from both the dose-finding and pivotal phases of the CAMPSIITE trial demonstrate a strong correlation between measured CSF HS D2S6 levels at week 16 and neurocognitive outcomes at one year. This correlation supports the use of CSF HS D2S6, a type of glycosaminoglycan (GAG), as a surrogate endpoint reasonably likely to predict clinical benefit under the accelerated approval pathway, as the buildup of GAGs in MPS II leads to clinical manifestations including neurodevelopmental deficits.

RGX-121 BLA
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, and RGX-121 has been well tolerated in all 26 patients dosed across all phases of the CAMPSIITE trial as of August 20, 2024. The FDA is expected to make a decision on the application by February 8, 2026.

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.

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 the CAMPSIITE® Trial
CAMPSIITE is a Phase I/II/III multicenter, open-label trial for boys aged four months up to five years with neuronopathic MPS II. The primary endpoint of the trial is measurement of CSF GAGs. Accurate and sensitive measurements of CSF GAGs, such as HS D2S6, have the potential to be considered a surrogate endpoint that is reasonably likely to predict clinical benefit in MPS II disease under the accelerated approval pathway, as buildup of GAGs in the CSF of MPS II patients correlates with clinical manifestations including neurodevelopmental deficits.

The pivotal program uses commercial-scale cGMP material from REGENXBIO's proprietary, high-yielding suspension-based manufacturing process, named NAVXpress®. In addition to measuring GAGs in the CSF, the trial will continue to collect neurodevelopmental data and caregiver-reported outcomes.

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 and clinical trials. 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 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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