REGENXBIO Announces Pivotal Trial of RGX-121 for the Treatment of MPS II Achieves Primary Endpoint

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- *Results support BLA submission in 2024 using the accelerated approval pathway*
- *Primary endpoint of patients achieving reduction in CSF biomarker of MPS II disease was met with statistical significance (p value of 0.00016)*
- *Patients treated with RGX-121 have showed continued improvement in neurodevelopmental skill acquisition up to four years and discontinued intravenous enzyme therapy*
- *Company plans to discuss these results as part of a full rare disease program update on its conference call today, Wednesday, February 7, 4:30 p.m. ET*

ROCKVILLE, Md., Feb. 7, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced topline results from the Phase I/II/III CAMPSIITE® trial of RGX-121 for the treatment of patients up to 5 years old diagnosed with Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, demonstrating that the pivotal phase of the trial met its primary endpoint with statistical significance.

The results were presented at the 20th Annual WORLD*Symposium* ™ by Paul Harmatz, M.D., UCSF Benioff Children's Hospital and trial investigator.

"The data from this pivotal trial supports that RGX-121 changes the course of disease by restoring the gene missing in boys with Hunter syndrome and has the potential to significantly improve vital brain function for patients living with this debilitating disease," said Kenneth T. Mills, President and CEO of REGENXBIO. "We are excited about these results and working quickly to complete activities to file the BLA this year. We have shared CAMPSIITE results with FDA leadership, and they have confirmed that, based on the totality of the evidence, they are open to accelerated approval if supported by review of the full data."

"There is currently no treatment to address fatal neuronopathic CNS disease in MPS II, and I am encouraged by the topline data from the pivotal trial of RGX-121," said Dr. Harmatz. "A one-time gene therapy that can help these boys develop beyond the natural history of the disease and may allow them to discontinue enzyme replacement therapy or remain ERT-naïve represents a meaningful breakthrough."

Data Summary
In the pivotal phase, MPS II patients treated with RGX-121 achieved decreased cerebrospinal fluid (CSF) levels of D2S6, a key biomarker of brain disease activity, below maximum attenuated disease levels at 16 weeks (p value of 0.00016). Patients receiving RGX-121 demonstrated an 86% median reduction in D2S6, approaching normal levels.

Pivotal results were consistent with data from the dose-finding phase of CAMPSIITE. In the dose-finding phase, the majority of patients are exceeding expectations in neurodevelopmental function compared to natural history data up to four years. New long-term follow-up of patients treated with RGX-121 in the dose-finding phase also showed there was a high rate of patients for whom trial investigators chose to discontinue standard-of-care intravenous enzyme replacement therapy (ERT) or were allowed to remain ERT-naïve. At the pivotal dose level, 80% of patients were ERT-free at last time point.

As of January 3, 2024, RGX-121 continues to be well tolerated in 25 patients dosed across all phases of the CAMPSIITE trial.

Following an RMAT meeting held with FDA at the end of 2023, REGENXBIO continues with plans to use CSF levels of D2S6 as a surrogate endpoint for accelerated approval and is completing remaining activities in order to file a BLA in the second half of 2024. Based on an expected priority review, potential approval of the planned BLA could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025.

Data presented is available on the "Publications" section of the REGENXBIO website at WWW.REGENXBIO.COM.

Conference Call
As part of a full rare disease program update, REGENXBIO will host a conference call today, Wednesday, February 7 at 4:30 p.m. ET and will be joined by Dr. Harmatz and Raymond Wang, M.D., Children's Hospital of Orange County to discuss the CAMPSIITE trial pivotal phase topline results and the expedited plan for filing a Biologics License Application using the accelerated approval pathway in 2024.

Listeners can register for the webcast via this link. Analysts wishing to participate in the question and answer session should use this link. A copy of the slides being presented will be available via the Company's investor website. Those who plan on participating are advised to join 15 minutes prior to the start time. A replay of the webcast will also be available via the Company's investor website approximately two hours after the call's conclusion.

About the CAMPSIITE® Trial
CAMPSIITE is a Phase I/II/III multicenter, open-label trial enrolling boys with neuronopathic MPSII, aged four months up to five years of age. The primary endpoint of the trial is measurement of CSF GAGs. Heparan sulfate (HS) and D2S6, a component of HS closely correlated with severe MPS II, are GAGs that are key biomarkers of I2S enzyme activity and are being measured in the CSF at baseline and after administration of RGX-121. Accurate and sensitive measurements of CSF GAGs, such as 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 is using 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 RGX-121
RGX-121 is an investigational, one-time gene therapy designed to deliver the iduronate-2-sulfatase gene (IDS) that encodes the iduronate-2-sulfatase enzyme (I2S) using the NAV® AAV9 vector. RGX-121 expressed protein is structurally identical to normal I2S. RGX-121 is administered directly to the central nervous system (CNS) using intracisternal or intracerebroventricular delivery. Delivery of the IDS gene within cells in the CNS could provide a permanent source of secreted I2S beyond the blood-brain barrier, allowing for long-term cross correction of cells throughout the CNS.

RGX-121 has received Orphan Drug Product, Rare Pediatric Disease, Fast Track and Regenerative Medicine Advanced Therapy designations from the U.S. Food and Drug Administration 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 iduronate-2-sulfatase (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). MPS II is estimated to occur in 1 in 100,000 to 170,000 births. 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 leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8 and AAV9. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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 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, 2022, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (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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