REGENXBIO Announces Successful Pre-BLA Meeting with FDA to Support Accelerated Approval Pathway for RGX-121 for the Treatment of MPS II

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- *Aligned with FDA on content of BLA and plans for submission:*
  - *Submission of a rolling BLA using the accelerated approval pathway expected to start in Q3 2024*
  - *Confirmatory trial expected to begin in H2 2025*
  - *FDA confirmed RGX-121 commercial bulk drug is comparable to clinical material*
- *Positive biomarker, neurocognitive and systemic data will be part of BLA submission*

ROCKVILLE, Md., June 18, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced it completed a successful Pre-Biologics License Application (BLA) meeting for RGX-121 for the treatment Mucopolysaccharidosis Type II (MPS II), where it finalized details of its BLA with the U.S. Food and Drug Administration (FDA).

The FDA continues to be aligned with REGENXBIO's plan to use cerebrospinal fluid (CSF) levels of heparan sulfate (HS) D2S6, a key biomarker of brain disease activity, as a surrogate endpoint reasonably likely to predict clinical benefit to support accelerated approval of RGX-121. Additionally, REGENXBIO and the FDA discussed manufacturing, non-clinical, device delivery system and other critical elements of the BLA, including a confirmatory study designed to verify and describe the predicted clinical benefit.

REGENXBIO completed its database lock for the pivotal program and expects to initiate submission of a rolling BLA in the third quarter of 2024. REGENXBIO expects an FDA inspection of its Manufacturing Innovation Center in the first half of 2025. Commercial bulk drug and clinical trial material, both manufactured using REGENXBIO's proprietary NAVXpress™ platform process, were confirmed to be comparable.

A confirmatory study of RGX-121 is expected to initiate enrollment in the second half of 2025, prior to potential FDA approval. 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.

"This positive engagement with the FDA marks an important milestone for REGENXBIO and RGX-121 on the path towards potential approval of the first gene therapy for Hunter syndrome," said Curran Simpson, Chief Operating Officer of REGENXBIO and President and CEO-elect. "Alignment with the FDA on important elements of this BLA, like our proprietary NAVXpress™ platform process and inspection of our commercial-ready manufacturing facility, provide a solid foundation as we advance to pivotal stage for our other rare program, RGX-202, and work to diligently to expedite its development for the Duchenne community."

"The MPS II community is in need of new treatment options that have the potential to address the neurocognitive impacts of this devastating disease," said Dr. Matthew Ellinwood, Chief Scientific Officer of the National MPS Society. "We are pleased with REGENXBIO's continued positive discussions with the FDA and are encouraged by the totality of the biomarker, neurocognitive and systemic data for RGX-121 and the impact its potential approval may have for the families we serve."

Topline results from the Phase I/II/III CAMPSIITE® trial of RGX-121 presented earlier this year demonstrated that the pivotal phase of the trial met its primary endpoint with statistical significance. Pivotal results were consistent with data from the dose-finding phase of CAMPSIITE, in which the majority of patients were shown to be exceeding expectations in neurodevelopmental function compared to natural history data up to four years. Long-term follow-up of patients treated with RGX-121 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. As of January 3, 2024, RGX-121 continues to be well tolerated in 25 patients dosed across all phases of the CAMPSIITE trial. REGENXBIO expects to share additional safety and efficacy data from the CAMPSIITE trial in the second half of 2024.

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 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 a potential one-time AAV therapeutic for the treatment of boys with MPS II. RGX-121 expressed protein is structurally identical to normal I2S. RGX-21 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 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. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for retinal and rare diseases, including ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, RGX-202 for the treatment of Duchenne and RGX-121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics 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 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, 2023, 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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