REGENXBIO Announces Positive Data from Pivotal Dose Level of RGX-121 Demonstrating Long-Term Systemic Effect

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- *Data from pivotal dose level demonstrates long-term, sustained reductions in CSF levels of HS D2S6, a key biomarker of brain disease in MPS II*
- *80% of patients who received the pivotal dose discontinued intravenous enzyme replacement therapy or remained treatment-naïve*
- *Submission of a rolling BLA using the accelerated approval pathway on track for Q3 2024*

ROCKVILLE, Md., Sept. 3, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced positive results from the Phase I/II/III CAMPSIITE® trial of RGX-121 for the treatment of patients with Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome. The results were presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium 2024.

The totality of evidence from the CAMPSIITE trial continues to support RGX-121 as the potential first gene therapy and one-time treatment for MPS II. In the United States, RGX-121 is also on track to be the first treatment that addresses the neurocognitive decline associated with MPS II, with the potential to be the first-line treatment for patients with neuronopathic disease.

"As we quickly approach the BLA filing for RGX-121, we are very pleased with the data presented at SSIEM demonstrating encouraging evidence of systemic activity and long-term reductions of CSF D2S6," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "The data continue to support that by restoring the gene missing in boys with Hunter syndrome, RGX-121 changes the course of disease and has the potential to significantly improve both vital brain function and the systemic manifestations of this devastating disease."

Data Summary

In new, long-term data from the Phase I/II/III CAMPSIITE® trial, patients receiving RGX-121 at the pivotal dose level demonstrated an 85% median reduction of cerebrospinal fluid (CSF) levels of heparan sulfate (HS) D2S6, a key biomarker of brain disease activity, approaching normal levels and sustained for up to two years. Topline results presented earlier this year from the CAMPSIITE trial 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.

In the dose-finding part of the trial, investigators chose to discontinue standard-of-care intravenous enzyme replacement therapy (ERT) or to remain ERT-naïve for a majority of patients. At the pivotal dose level (dose level 3), 80% of patients were ERT-free at last time point, up to more than 18 months post-dosing. At dose level 2, 71% of patients were ERT-free at last time point, up to almost three years.

As of January 5, 2024, RGX-121 continues to be well tolerated in 25 patients dosed across all phases of the CAMPSIITE trial.

"A potential one-time treatment that can allow these boys to exceed the natural history of this disease in their neurocognitive development, as well as the ability to remain off enzyme replacement therapy for multiple years represents a meaningful option for patients and their families," said Roberto Giugliani, M.D., Ph.D., Professor, Department of Genetics, UFRGS, Medical Genetics Service, HCPA, Porto Alegre, Brazil. "I continue to be very encouraged by the data supporting RGX-121 and look forward to the seeing this program advance towards potential approval for this community."

REGENXBIO is on track to initiate a rolling Biologics License Application (BLA) submission using the accelerated approval pathway in the third quarter of 2024 using CSF D2S6 as a surrogate endpoint reasonable likely to predict clinical benefit. Approval of the planned BLA could result in receipt of a Priority Review Voucher in 2025.

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

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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