REGENXBIO Announces Completion of Dosing in the Phase I/II Trial of RGX-111 for the Treatment of Severe MPS I

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- *RGX-111 is an investigational AAV Therapeutic for the treatment of severe MPS I that is part of REGENXBIO's clinical-stage pipeline of neurodegenerative disease programs.*
- *Expanded Cohort 2 enrollment is complete; eight patients have received RGX-111 in the trial*
- *Company intends to manufacture commercial-scale cGMP material to support the continued development of RGX-111*
- *Company expects additional interim updates from the RGX-111 trial in the first half of 2023*

ROCKVILLE, Md., Dec. 8, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced it has completed dosing in the expanded Cohort 2 of the Phase I/II trial of RGX-111 for the treatment of severe Mucopolysaccharidosis Type I (MPS I). The trial has now completed dosing, with eight patients enrolled across two dose cohorts. As previously reported, RGX-111 has also been administered to a patient with MPS I through a single-patient IND.

RGX-111 is an investigational one-time gene therapy designed to deliver the gene that encodes the α-l-iduronidase (IDUA) enzyme using the NAV AAV9 vector. RGX-111 is administered directly to the CNS. The primary endpoint of the trial is to evaluate the safety of RGX-111. Secondary and exploratory endpoints include biomarkers of IDUA enzyme activity in the cerebrospinal fluid (CSF), serum and urine, neurodevelopmental assessments, and caregiver reported outcomes. Patients were treated across two dose cohorts: $1.0x10^{10}$ genome copies per gram (GC/g) of brain mass (n=2) and $5.0x10^{10}$ GC/g of brain mass (n=6). In the single-patient IND for RGX-111, a severe MPS I patient was dosed with $1x10^{10}$ GC/g of brain mass.

"We have made great progress this year advancing our pipeline of neurodegenerative disease programs, and completing the dosing in this MPS I trial is another important clinical milestone as we continue to develop potential one-time gene therapies for patients," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "Earlier, at this year's WORLD*Symposium* meeting, we presented data on biomarker and neurodevelopmental assessments that indicated an encouraging CNS profile in patients dosed with RGX-111. We intend to advance the program for RGX-111 with the aim of providing a much-needed new treatment option for the MPS I community as quickly as possible."

The Phase I/II trial of RGX-111 is a global trial, which includes sites in the United States and Brazil. Many of these trial sites are also participating in REGENXBIO's on-going CAMPSIITE ™ trial, the pivotal program for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II).

"Our '5x'25' strategy is to have five gene therapies either on the market or in late-stage development by 2025. Earlier this year, we announced that the pivotal program for RGX-121 is active and enrolling patients, making this our second internal program to enter late-stage development. RGX-111 is our next most-advanced clinical candidate in our neurodegenerative disease pipeline and over 30 children with severe MPS I and MPS II have been dosed in our trials using the NAV AAV9 vector," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "I would like to express my deep gratitude to our employees and clinical partners, as well as patients and their families in the MPS community, for their ongoing commitment and support in the development of both RGX-111 and RGX-121."

REGENXBIO plans to continue having early and frequent communication with regulatory agencies about pathways to expedite development of its neurodegenerative disease pipeline. In the first half of 2023, REGENXBIO expects to use its Manufacturing Innovation Center to produce RGX-111 commercial-scale cGMP material from its proprietary, high-yielding suspension-based manufacturing process, named NAVXPress ™. The pivotal program for RGX-121 is using the NAVXPress manufacturing process.

Additional interim data from the Phase I/II trial of RGX-111 is also expected to be reported in the first half of 2023.

About RGX-111

RGX-111 is designed to use the AAV9 vector to deliver the α-l-iduronidase (IDUA) gene to the central nervous system (CNS). Delivery of the IDUA gene within the cells in the central nervous system (CNS) could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long-term cross-correction of cells throughout the CNS. By providing rapid IDUA delivery to the brain, RGX-111 could potentially help prevent the progression of cognitive deficits that otherwise occurs in MPS I patients. RGX-111 has received orphan drug product, rare pediatric disease and Fast Track designations from the U.S. Food and Drug Administration.

About Mucopolysaccharidosis Type I (MPS I)

MPS I is a rare autosomal recessive genetic disease caused by a deficiency in the lysosomal enzyme alpha-L-iduronidase (IDUA), 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). This can include excessive accumulation of fluid in the brain, spinal cord compression, and cognitive impairment. MPS I is estimated to occur in 1 in 100,000 births. Current disease modifying therapies for MPS I include hematopoietic stem cell transplant (HSCT) and enzyme replacement therapy with a recombinant form of human IDUA administered intravenously. However, intravenous

enzyme therapy does not treat the CNS manifestations of MPS I, and HSCT can be associated with clinically significant morbidity and mortality. Key biomarkers of IDUA enzymatic activity in MPS I patients include its substrate heparan sulfate (HS), 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, AAV9 and AAVrh10. 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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, 2021, 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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