REGENXBIO Announces Dosing of First Patient in Phase I/II Trial of RGX-111 for the Treatment of Mucopolysaccharidosis Type I

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- Trial will evaluate one-time, direct-to-CNS treatment for MPS I

- Second novel gene therapy for neurodegenerative disease program to be actively enrolling patients

ROCKVILLE, Md., Dec. 1, 2020 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that the first patient has been dosed in the Phase I/II trial of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I). RGX-111 is an investigational one-time gene therapy designed to deliver the α-l-iduronidase (IDUA) gene directly to the central nervous system (CNS) using the NAV AAV9 vector.

MPS I is a rare, autosomal recessive genetic disease caused by deficiency of IDUA, an enzyme required for the breakdown of the polysaccharides in lysosomes. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS I patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms including in the central nervous system (CNS), which can include excessive accumulation of fluid in the brain, spinal cord compression, and cognitive impairment.

"We are leaders in the development of one-time treatments using our NAV Technology Platform and proprietary delivery procedure which is designed to administer our gene therapy candidates directly to the central nervous system (CNS). RGX-111 is our second product candidate for the treatment of a rare, neurodegenerative disease to be dosed in patients, following RGX-121, which is in clinical development for MPS II," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "We believe one-time treatment with RGX-111 can provide sustainable IDUA enzyme for patients, potentially preventing the progression of disease. The dosing of the first patient in the clinical trial for RGX-111 marks an important milestone in our neurodegenerative disease program and our commitment to the MPS community."

The Phase I/II trial is a multi-center, open-label, dose escalation trial that will evaluate the safety, tolerability and pharmacodynamics of RGX-111 delivered to patients with MPS I via injection directly into the cerebrospinal fluid (CSF). Up to five patients will be enrolled at two dose levels: $1.0x10^{10}$ GC/g of brain mass and $5.0x10^{10}$ GC/g of brain mass. The primary endpoint of the study is safety and tolerability of RGX-111. Other endpoints include the effect of RGX-111 on biomarkers of IDUA enzyme activity in the CSF, serum and urine, neurocognitive development and other outcome measures.

About RGX-111

RGX-111 is a product candidate for the treatment of Mucopolysaccharidosis Type I (MPS I), also known as Hurler syndrome. RGX-111 is designed to use the AAV9 vector to deliver the α-l-iduronidase (IDUA) gene. Delivery of the enzyme that is deficient within 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. This strategy could also provide rapid IDUA delivery to the brain, potentially preventing 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 deficiency of IDUA, an enzyme required for the breakdown of the polysaccharides in lysosomes. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS I patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms including in the central nervous system (CNS), which 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.

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 in multiple therapeutic areas.

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," "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, 2019, 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. 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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