REGENXBIO Announces Continued Progress and Expansion of Clinical Development Program for RGX-121 for the Treatment of Mucopolysaccharidosis Type II (MPS II)

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- Ongoing Phase I/II trial to enroll up to 6 additional patients in Cohort 2 while planning for next steps in program; further trial updates expected by the end of 2020

- New IND for Phase I/II trial cleared by FDA to evaluate RGX-121 in patients with severe MPS II ages 5-18 years old and expected to initiate in 2H 2020

- Initiation of a new prospective observational study expected to provide detailed characterization of neurocognitive development and key biomarkers in patients with severe MPS II

ROCKVILLE, Md., Sept. 30, 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 the expansion of the RGX-121 program for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome, to gain additional insight into the neurodegenerative manifestations of the disease and evaluate RGX-121 in a broader patient population. RGX-121 is an investigational one-time gene therapy, designed to use the AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase (I2S) enzyme directly to the central nervous system (CNS) through intracisternal administration.

"Today's update reflects significant forward progress in our clinical program for the treatment of MPS II, as we expand the program to gain additional insight into the potential treatment effects of RGX-121 in more patients. REGENXBIO is committed to advancing potential gene therapy treatment options for MPS II, as there remains a significant unmet medical need to address the neurological manifestations and prevent or stabilize cognitive decline for patients," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "In addition, we are announcing the initiation of an important prospective natural history study to provide critical data about the neurocognitive development of young pediatric patients with MPS II. We plan to share the data from this observational study with the patient community and look forward to working closely with researchers and advocates in order to enhance awareness of this study."

"MPS II is a serious and debilitating lysosomal disease that affects 1 in 100,000 children, and available treatments are inadequate to treat the neurodegenerative manifestations of the disease. Initiating a natural history study will increase the understanding of neurocognitive effects and key biomarkers of severe MPS II, and is critical to advancing the development of new treatment options. We are grateful for REGENXBIO's dedication to MPS and commitment to share the learnings from this observational study with the community," said Terri Klein, President and Chief Executive Officer of the National MPS Society.

An ongoing Phase I/II study is evaluating a single intracisternal administration of RGX-121 in severe MPS II patients under the age of 5 years old, and six patients have been dosed across two dose levels. The study is evaluating the safety and tolerability of RGX-121, as well as the effects of RGX-121 on biomarkers of I2S enzyme activity, neurocognitive development and other clinical measures. As of September 16, 2020, RGX-121 is reported to be well-tolerated in all six patients with no drug-related serious adverse events (SAEs). REGENXBIO plans to immediately expand enrollment of patients in Cohort 2 based on support from MPS II treating physicians and the Independent Data Monitoring Committee. Up to six additional patients will be dosed with RGX-121 at the second dose level, $6.5x10^{10}$ genome copies per gram (GC/g) of brain mass. REGENXBIO anticipates further updates from this trial by the end of 2020.

In addition, REGENXBIO announced that the U.S. Food and Drug Administration (FDA) has cleared a new Investigational New Drug application (IND), and plans to initiate a second Phase I/II multicenter, open-label trial of RGX-121 for the treatment of pediatric patients with severe MPS II ages 5-18 years old. Up to six patients will be enrolled, and RGX-121 will be administered at a dose level of $6.5x10^{10}$ GC/g of brain mass, delivered directly to the cerebrospinal fluid (CSF) through intracisternal or intracerebroventricular injection. The trial is designed to evaluate the safety of a single administration of RGX-121; the effects of RGX-121 on biomarkers of I2S enzyme activity; and changes in cognitive function, adaptive behavior, daily function, and quality of life.

A new observational natural history trial designed to provide detailed characterization of neurocognitive development and key biomarkers in patients with severe MPS II is expected to open for enrollment in the second half of 2020. In severe forms of MPS II, early developmental milestones in a child may be met, but delays become apparent by 18 to 24 months, with neurologic deficits and cognitive impairment appearing before the age of 6 years. This trial is intended to prospectively document the changes in neurodevelopmental parameters of cognitive, behavioral and adaptive function over time, in addition to biomarker activity in the CSF, serum, and urine. Up to 40 patients between the ages of 1 month and 8 years with genetic diagnosis of severe MPS II will be enrolled in this trial and evaluated for up to two years.

For additional information on these trials, please visit clinicaltrials.gov.

About RGX-121

RGX-121 is a product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome. RGX-121 is designed to use the AAV9 vector to deliver the human iduronate-2-sulfatase (IDS) gene which encodes the iduronate-2-sulfatase (I2S) enzyme to the central nervous system (CNS). 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 and Fast Track designations from the U.S. Food and Drug Administration.

About Mucopolysaccharidosis Type II (MPS II)

MPS II 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, including heparan sulfate (HS) in tissues which ultimately results in cell, tissue, and organ dysfunction. 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 and prevent or stabilize cognitive decline remains a significant unmet medical need. Key biomarkers of I2S enzymatic activity in MPS II patients include its substrate 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 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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