REGENXBIO Receives FDA Fast Track Designation for RGX-121 Gene Therapy for the Treatment of Mucopolysaccharidosis Type II

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-- Novel, one-time, direct-to-CNS investigational treatment for MPS II designed to prevent the progression of cognitive deficits

-- Phase I/II clinical trial expected to enroll children with MPS II

-- Expect to initiate patient recruitment and dosing in mid-2018

ROCKVILLE, Md., May 2, 2018 /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 U.S. Food and Drug Administration (FDA) has granted Fast Track designation for RGX-121. RGX-121 is a novel, one-time investigational treatment for Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, that is designed to deliver the human iduronate 2-sulfatase (I2S) gene directly to the central nervous system (CNS) using the NAV AAV9 vector.

The FDA Fast Track program is designed to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. Fast Track-designated drugs often qualify for priority review, thereby expediting the FDA review process.

"Children living with MPS II have limited treatment options, making this Fast Track designation tremendously important," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are honored to support the MPS community and committed to offering innovative solutions to people with MPS II and their families. We look forward to working closely with the FDA to facilitate the development of RGX-121 and will begin the Phase I/II trial in the coming months."

"My family has seen firsthand the unmet needs of children with MPS II," said Jeanette Henriquez, who founded the Hunter Syndrome Foundation after her son Dominic was diagnosed with MPS II in 2011. "As we prepare to recognize International MPS Awareness Day on May 15, we are encouraged to see recognition from the FDA on important research exploring new treatment options for children with MPS II."

Leading international gene therapy and lysosomal storage disease centers will participate in the Phase I/II clinical trial for RGX-121 for the treatment of MPS II.

About the Phase I/II Clinical Trial of RGX-121

RGX-121 will be evaluated in a Phase I/II, multi-center, open-label, multiple-cohort, dose-escalation study in pediatric subjects with MPS II. Eligible patients must have documented evidence of early-stage neurocognitive deficit due to MPS. Approximately six male subjects with MPS II greater than or equal to four months old and less than five years old will be treated in two dose cohorts (1.3×10^{10} GC/g brain mass and 6.5×10^{10} GC/g brain mass), and will receive a single dose of RGX-121 administered by an injection directly in the cerebrospinal fluid (CSF). Patients will receive immunosuppression for the first year after RGX-121 is administered. The primary purpose of the clinical study is to assess the safety and tolerability of RGX-121 at 24 weeks. The primary endpoint will be a safety assessment. The secondary and exploratory endpoints include the effect of RGX-121 on biomarkers of I2S activity in the CSF, serum and urine and effect of RGX-121 on neurocognitive deficits, as well as other outcome measures. Following completion of the primary study period, subjects will continue to be assessed for a total of 104 weeks following treatment with RGX-121 and then be asked to participate in a long-term follow-up.

About RGX-121

RGX-121 is being developed as a novel, one-time, direct-to-CNS treatment for MPS II that includes the NAV AAV9 vector encoding for human I2S. Delivery of the enzyme that is deficient 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. This strategy could also provide rapid I2S delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS II patients.

Treatment with RGX-121 has been shown to restore I2S enzyme activity in animal models of MPS II to levels equivalent to or greater than those in non-affected animals. The extent of CNS correction in animal studies suggests that RGX-121 has the potential to be an important and suitable therapeutic option for MPS II patients.

About Mucopolysaccharidosis Type II (MPS II)

MPS II is a rare X-linked recessive genetic disease caused by deficiency of I2S, an enzyme required for the breakdown of polysaccharides in the lysosomes. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS II patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms including in the central nervous system (CNS), which can include neural cell death, excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. MPS II is estimated to occur in approximately 1 in 100,000 to 1 in 170,000 births. The current disease-modifying therapy for MPS II is enzyme replacement therapy with a recombinant form of human I2S administered intravenously. However, intravenous enzyme therapy does not treat the CNS manifestations of MPS II.

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 of REGENXBIO's clinical trials; the timing and success of preclinical studies and clinical trials 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, 2017 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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