REGENXBIO Receives FDA Fast Track Designation for RGX-111 Gene Therapy for the Treatment of Mucopolysaccharidosis Type I

June 12, 2018 9:36 PM EDT

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

- Phase I clinical trial expected to enroll children and adults with MPS I

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

ROCKVILLE, Md., June 12, 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-111. RGX-111 is a novel, one-time investigational treatment for Mucopolysaccharidosis Type I (MPS I), that is designed to deliver the human iduronidase (IDUA) 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.

"Fast Track designation represents another positive step for the development of RGX-111 as we seek to address the unmet needs of people living with MPS I," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We continue to engage the MPS community as we seek to provide innovative solutions to people with MPS I and their families. We look forward to working closely with the FDA to facilitate the development of RGX-111 and expect to begin the Phase I trial in the coming months."

"There are limited treatment options that address the CNS symptoms of MPS I," said Steve Holland, long-time board member of the National MPS Society and father to three children with MPS I. "Having experienced firsthand the debilitating effects that MPS I can have on children living with this condition, we are encouraged to see the FDA recognize important research that explores new treatment options for people with MPS I."

Leading international gene therapy and lysosomal storage disease centers will participate in the Phase I clinical trial for RGX-111 for the treatment of MPS I.

About the Phase I Clinical Trial of RGX-111

RGX-111 will be evaluated in a Phase I, multi-center, open-label, multiple-cohort, dose-escalation study in children and adult subjects with MPS I. Eligible patients must have documented evidence of early-stage neurocognitive deficit due to MPS. Approximately five subjects with MPS I (initial subject ≥18 years of age, subsequent subjects can be ≥6 years of age) will be treated in two dose cohorts (2×10^9 GC/g brain mass and 1×10^{10} GC/g brain mass) and will receive a single dose of RGX-111 administered by an injection directly in the cerebrospinal fluid (CSF). Patients will receive immunosuppression for the first year after RGX-111 is administered. The primary purpose of the clinical study is to assess the safety and tolerability of RGX-111 at 24 weeks. Primary endpoints include adverse events, certain laboratory measures (including immunologic parameters), and neurological examinations. The study will also assess biomarkers related to IDUA protein activity within the CSF, serum, and urine. Following completion of the primary study period, subjects will continue to be assessed for a total of 104 weeks following treatment with RGX-111.

About RGX-111

RGX-111 is being developed as a novel, one-time, direct-to-CNS treatment for MPS I that includes the NAV AAV9 vector encoding a gene for human IDUA. Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDUA, which is otherwise untreated in intravenous-only therapy due to 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.

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 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 bone marrow transplant (BMT) 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 BMT can be associated with clinically significant morbidity and mortality.

AboutREGENXBIO 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, 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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