REGENXBIO Announces IND Active for Phase I Trial of RGX-111 to Treat Mucopolysaccharidosis Type I

August 08, 2017 03:06 PM EDT

- *Clinical trial expected to enroll children and adults with MPS I*
- *Anticipate beginning trial enrollment during the first half of 2018*

ROCKVILLE, Md., August 8, 2017 (GLOBE NEWSWIRE) -- 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 Investigational New Drug application (IND) is active for the planned multi-center, open-label, multiple-cohort, dose-escalation Phase I clinical trial of RGX-111 for the treatment of children and adult subjects with Mucopolysaccharidosis Type I (MPS I).

“The goal of the RGX-111 program is to develop a single-dose treatment for MPS I that can prevent the progression of neurocognitive decline experienced by children and some adults, and that can address the shortcomings of the current standard of care, which includes enzyme replacement therapy and bone marrow transplant,” said Olivier Danos, Ph.D., Chief Scientific Officer of REGENXBIO. “We are on track to meet our next program objectives for RGX-111, beginning with trial enrollment in the first half of 2018, and we look forward to working with leading gene therapy researchers and the broader MPS I community on this novel clinical program.”

RGX-111 is being developed under a collaboration with world-renowned gene therapy expert James M. Wilson, M.D., Ph.D., director of the Orphan Disease Center and Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania (Penn). Wilson is also a professor of Medicine and Pediatrics.

“In animal studies, treatment with RGX-111 has been shown to restore iduronidase, or IDUA, expression to levels equivalent to or greater than in non-affected animals. The extent of central nervous system correction in our studies was greater than that observed in a previous study of animals treated with bone marrow transplant at similar ages, thus demonstrating that RGX-111 has the potential to be an important and suitable therapeutic option in MPS I,” said Dr. Danos.

RGX-111 has received orphan drug designation as well as rare pediatric disease designation from the U.S. Food and Drug Administration (FDA). Leading international gene therapy and lysosomal storage disease centers are expected to participate in the Phase I trial of RGX-111.

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 iduronidase (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 Mucopolysacchardosis Type I (MPS I)

MPS I is a rare autosomal recessive genetic disease caused by deficiency of iduronidase (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 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.

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 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.

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.

Note Regarding Penn

Penn has licensed certain Penn-owned AAV technologies to REGENXBIO, including rights related to RGX-111. Dr. Wilson is an advisor to REGENXBIO, and is a founder of, holds equity in, and receives sponsored research funding from REGENXBIO.

Forward Looking Statements

This press release contains “forward-looking statements,” within the meaning of the Private Securities Litigation Reform Act of 1995, regarding, among other things, REGENXBIO’s research, development and regulatory plans in connection with its NAV Technology Platform and gene therapy treatments. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could cause actual results to differ materially from those projected by such forward-looking statements. All of REGENXBIO’s development timelines could be subject to adjustment depending on recruitment rate, regulatory agency review and other factors that could delay the initiation and completion of clinical trials. Meaningful factors which could cause actual results to differ include, but are not limited to, 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 ability to obtain and maintain regulatory approval of REGENXBIO’s product candidates, and the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing REGENXBIO’s product candidates; REGENXBIO’s ability to obtain and maintain intellectual property protection for REGENXBIO’s product candidates and technology; REGENXBIO’s growth strategies; REGENXBIO’s competition; trends and challenges in REGENXBIO’s business and the markets in which REGENXBIO operates; REGENXBIO’s ability to attract or retain key personnel; the size and growth of the potential markets for REGENXBIO’s product candidates and the ability to serve those markets; the rate and degree of market acceptance of any of REGENXBIO’s product candidates; REGENXBIO’s ability to establish and maintain development partnerships; REGENXBIO’s expenses and revenue; regulatory developments in the United States and foreign countries; the sufficiency of REGENXBIO’s cash resources and needs for additional financing; and other factors discussed in 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, which have been filed with the Securities and Exchange Commission (SEC) and are available on the SEC’s website at www.sec.gov. Additional factors may be set forth in those sections of REGENXBIO’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, which will be filed with the SEC in the third quarter of 2017. In addition to the risks described above and in REGENXBIO’s filings with the SEC, other unknown or unpredictable factors also could affect REGENXBIO’s results. There can be no assurance that the actual results or developments anticipated by REGENXBIO will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, REGENXBIO. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

All forward-looking statements contained in this press release are expressly qualified by the cautionary statements contained or referred to herein. REGENXBIO cautions investors not to rely too heavily on the forward-looking statements REGENXBIO makes or that are made on its behalf. These forward-looking statements speak only as of the date of this press release (unless another date is indicated). REGENXBIO undertakes no obligation, and specifically declines any obligation, to publicly update or revise any such forward-looking statements, whether as a result of new information, future events or otherwise.

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