# REGENXBIO Announces IND Active for Phase I/II Trial of RGX-121 to Treat Mucopolysaccharidosis Type II

December 19, 2017 08:00 AM EST

- *Novel, one-time, direct-to-CNS treatment for MPS II focused toward potentially preventing the progression of cognitive deficits*
- *Clinical trial expected to enroll children with MPS II*
- *Anticipate beginning trial enrollment during the first half of 2018*

ROCKVILLE, Md., Dec. 19, 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/II clinical trial of RGX-121 for the treatment of pediatric subjects with Mucopolysaccharidosis Type II (MPS II).

“The goal of the RGX-121 program is to develop a single-dose treatment for MPS II that can prevent the progression of neurocognitive decline experienced by children with the disease, which addresses one of the shortcomings of the current standard of care, enzyme replacement therapy,” said Stephen Yoo, M.D., Chief Medical Officer of REGENXBIO. “We expect to commence trial enrollment in the first half of 2018, and we look forward to working with leading gene therapy researchers and the broader MPS II community on this novel clinical program.”

RGX-121 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/II trial of RGX-121.

**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 4 months old and less than 5 years old will be treated in two dose cohorts ($1.3 \times 10^{10}$ GC/g brain mass and $6.5 \times 10^{10}$ 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. Primary endpoints include adverse events, certain laboratory measures (including immunologic parameters) and neurological examinations. The study will also assess biomarkers related to iduronate 2-sulfatase (I2S) 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-121.

**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 1 in 200,000 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 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 expression 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 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 research, development and regulatory plans in connection with its NAV Technology Platform and gene therapy treatments. 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, 2016 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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