FDA Grants Rare Pediatric Disease Designation to REGENXBIO's RGX-111 Gene Therapy for the Treatment of Mucopolysaccharidosis Type I (MPS I)

December 30, 2015 09:00 AM EST

ROCKVILLE, Md., Dec. 30, 2015 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (Nasdaq:RGNX), a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to RGX-111, the Company's investigational gene therapy product candidate for the treatment of mucopolysaccharidosis Type I (MPS I).

"The Rare Pediatric Disease Designation builds upon the Orphan Drug Designation granted earlier this year by the FDA to RGX-111 for MPS I, underscoring the therapy's potential to provide meaningful benefit to children struggling with this severely debilitating disease," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We plan to file an Investigational New Drug application for RGX-111 for the treatment of MPS I in the first half of 2016, and look forward to working with the FDA to advance this important gene therapy to patients in need."

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S., primarily aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for any subsequent marketing application.

MPS I is a rare neurodegenerative disease caused by deficiency of the a-l-iduronidase (IDUA) gene. Over 1,000 individuals with MPS I are estimated to be born each year worldwide. Symptoms include excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. RGX-111 uses an AAV9 vector to deliver the IDUA gene to the central nervous system.

About REGENXBIO

REGENXBIO is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. REGENXBIO's NAV Technology Platform, a proprietary AAV gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO's mission is to transform the lives of patients suffering from severe diseases with significant unmet medical need by developing and commercializing *in vivo* gene therapy products based on REGENXBIO's NAV Technology Platform. REGENXBIO seeks to accomplish this mission through a combination of internal development efforts and third-party NAV Technology Platform licensees. As of September 30, 2015, REGENXBIO's NAV Technology Platform is being applied in the development of 23 product candidates for a variety of diseases, including five internally developed candidates and 18 partnered candidates developed by REGENXBIO's licensees.

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 for RGX-111 and other gene therapies. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual results to differ materially from those projected in its forward-looking statements. Meaningful factors which could cause actual results to differ include, but are not limited to, 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 our product candidates; REGENXBIO's ability to establish and maintain development partnerships; REGENXBIO's expectations regarding federal, state and foreign regulatory requirements; regulatory developments in the United States and foreign countries, as well as other factors discussed in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission (SEC) on September 17, 2015 (the Prospectus) and available on the SEC's website at www.sec.gov. Additional factors are described in those sections of REGENXBIO's quarterly report on Form 10-Q for the quarter ended September 30, 2015, filed with the SEC and available on the SEC's website at www.sec.gov. In addition to the risks described above and in the Prospectus, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other 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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