REGENXBIO Announces IND Active for Phase I Trial of RGX-314 to Treat Wet Age-Related Macular Degeneration

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- *Six leading U.S. retinal surgery centers expected to participate as enrollment sites in the trial*
- *Anticipate beginning trial enrollment by mid-2017; interim trial update expected by the end of 2017*

ROCKVILLE, Md., Feb. 14, 2017 (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 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-314 for the treatment of wet age-related macular degeneration (wet AMD).

“The goal of the RGX-314 program is to develop a single-dose treatment for wet AMD that prevents future disease recurrence while reducing or eliminating the need for regular injections that are the current standard of care in wet AMD,” said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. “We are on track to meet our next program objectives for RGX-314, beginning with trial enrollment by mid-2017 and an interim trial update by the end of the year, and we look forward to working with leading U.S. researchers and retina surgeons on this novel clinical program.”

RGX-314 is being developed under a multi-institutional collaboration with world-renowned gene therapy and ophthalmology experts James M. Wilson, M.D., Ph.D., Jean Bennett, M.D., Ph.D. and Albert Maguire, M.D. from the University of Pennsylvania’s Gene Therapy Program and Center for Advanced Retinal and Ocular Therapeutics (Penn), respectively, and Peter Campochiaro, M.D. at the Johns Hopkins Wilmer Eye Institute (Johns Hopkins).

“In animal studies, treatment with RGX-314 gene therapy led to rapid and sustained anti-VEGF protein detected in the eyes of treated animals. Preclinical studies have shown anti-VEGF mRNA and protein distributed widely throughout the retina. This high protein expression observed using RGX-314’s NAV AAV8 vector may make this approach suitable for an ocular therapeutic in wet AMD,” said Dr. Maguire.

Six leading retinal surgery centers across the United States, including Penn and Johns Hopkins, are expected to participate in the Phase I trial of RGX-314.

About the Phase I Clinical Trial of RGX-314

RGX-314 will be evaluated in a Phase I, multi-center, open-label, multiple-cohort, dose-escalation study in adult subjects with wet AMD in the United States. The study is expected to include approximately eighteen previously treated wet AMD subjects that are responsive to anti-vascular endothelial growth factor (anti-VEGF) therapy and are 50 years of age or older. The study is designed to evaluate three doses of RGX-314 (3 × 10^9 genome copies (GC)/eye, 1 × 10^10 GC/eye, and 6 × 10^10 GC/eye). Primary endpoints include adverse events, certain laboratory measures (including immunological parameters) and ocular examinations and imaging (including BCVA and SD-OCT). The primary purpose of the clinical study is to evaluate the safety and tolerability of RGX-314 at 24 weeks after a single dose of RGX-314 administered by subretinal delivery. Following completion of the primary study period, it is expected that subjects will enter the follow-up period and will continue to be assessed until week 106 to assess long term safety and durability of effect.

About Wet AMD

Wet AMD is characterized by loss of vision due to excess blood vessel formation between two layers of cells in the retina, which results in fluid leakage that can result in physical changes in the structure of the retina and changes in vision. Wet AMD is a leading cause of total and partial vision loss in the United States, Europe and Japan and there may be over two million individuals living with wet AMD in these geographies alone. Current anti-VEGF therapies have significantly changed the landscape for treatment of wet AMD, becoming the standard of care due to their ability to halt or significantly impede the loss of vision in the majority of patients with wet AMD. All of these therapies, however, require repetitive and inconvenient intraocular injections, typically ranging from every four to eight weeks in frequency, to maintain efficacy. Patients often experience vision loss with reduced frequency of treatment.

About RGX-314

RGX-314 is being developed as a novel, one-time subretinal treatment for wet AMD that includes the NAV AAV8 vector encoding a gene for a monoclonal antibody fragment. The expressed protein is designed to neutralize VEGF activity, modifying the pathway for formation of new leaky blood vessels and retinal fluid accumulation. In preclinical animal models with conditions similar to macular degeneration, significant and dose-dependent reduction of blood vessel growth and prevention of disease progression was observed after a single subretinal dose of RGX-314.

About REGENXBIO Inc.

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. REGENXBIO and its 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-314. Dr. Wilson is an advisor to REGENXBIO, and is a founder of, holds equity in, and receives grants 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 and objectives for its RGX-314 program, including REGENXBIO’s Phase I clinical trial of RGX-314. 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, including for RGX-314, 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 to conduct clinical trials and to commercialize REGENXBIO’s product candidates, the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing REGENXBIO’s product candidates; competitive products; REGENXBIO’s ability to obtain and maintain intellectual property protection for REGENXBIO’s product candidates and technology; 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, the sufficiency of REGENXBIO’s cash resources and needs for additional financing, 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 Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, which are available on the SEC’s website at www.sec.gov. Additional factors may be set forth in those sections of REGENXBIO’s Annual Report on Form 10-K for the year ended December 31, 2016, to be filed in the first 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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