REGENXBIO Announces Completion of Dosing of Third Cohort in Phase I Clinical Trial of RGX-314 Gene Therapy for Wet AMD

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- *Clinical trial evaluates one-time treatment for wet AMD using NAV® AAV8 gene therapy*
- *18 patients have been dosed at leading retinal surgery centers in the United States*
- *Anticipate reporting topline data in late 2018*

ROCKVILLE, Md., Feb. 08, 2018 (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 it has completed dosing of the third cohort of six patients in a Phase I clinical trial evaluating RGX-314 for the treatment of patients suffering from wet age-related macular degeneration (wet AMD).

"We are excited about the progress that we have made in the clinical development of our lead product candidate, RGX-314," said Stephen Yoo, M.D., Chief Medical Officer of REGENXBIO. "Completing enrollment of the third cohort in the Phase I clinical trial brings us one step closer toward delivering on the promise of a one-time treatment with rapid and sustained therapeutic effects for patients with wet AMD, one of the largest indications for which gene therapy is being developed."

Six leading retinal surgery centers across the United States are participating in the Phase I trial of RGX-314. This multi-center, open-label, multiple-cohort, dose-escalation clinical trial is designed to assess the safety and tolerability of RGX-314 as a one-time therapy for patients with previously treated wet AMD. For further details on the trial, enrollment criteria and eligibility, please contact patientadvocacy@regenxbio.com or visit https://clinicaltrials.gov/ct2/show/NCT03066258.

REGENXBIO plans to share topline results from the Phase I trial in late 2018.

About the Phase I Clinical Trial of RGX-314

RGX-314 is currently being 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). The primary purpose of the clinical study is to evaluate the safety and tolerability of RGX-314 at 24 weeks after a single dose administered by subretinal delivery. Primary endpoints include safety and tolerability and secondary endpoints include ocular examinations and imaging (including BCVA and SD-OCT) and the need for additional anti-VEGF therapy. Following completion of the primary study period, subjects will enter a follow-up period and will continue to be assessed until week 106 for long term safety and durability of effect.

About RGX-314

RGX-314 is being developed as a one-time subretinal treatment for wet AMD. It includes the NAV AAV8 vector encoding an antibody fragment which inhibits VEGF, modifying the pathway for formation of new leaky blood vessels which lead to retinal fluid accumulation and vision loss. 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 Wet AMD

Wet AMD is characterized by loss of vision due to new leaky blood vessel formation in the retina. This results in fluid leakage that can manifest in physical changes in the structure of the retina and loss of vision. Wet AMD is a significant cause of vision loss in the United States, Europe and Japan. There may be more than 2 million people 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 improve vision and retinal fluid in the majority of patients. 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 a decline in the initial vision gain from therapy with reduced frequency of treatment over time.

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.

REGENXBIO 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 product candidates, clinical trials and future operations. 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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