REGENXBIO Announces Initiation of Phase I Clinical Trial of RGX-314 Gene Therapy for Wet Age-Related Macular Degeneration

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- *Clinical trial evaluates one-time treatment for wet AMD using NAV® AAV8 gene therapy; interim trial update expected late 2017*

ROCKVILLE, Md., May 31, 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 that the first patient was dosed in a phase I clinical trial evaluating RGX-314 for patients with wet age-related macular degeneration (wet AMD). This multi-center, open-label, multiple-cohort, dose-escalation clinical trial will assess the safety and tolerability of RGX-314 as a one-time therapy for patients with previously treated wet AMD.

“Reaching the clinic and dosing our first patient is an important milestone as we strive to improve the lives of patients through the development of one of the first gene therapies for long-term delivery of a therapeutic protein,” said Stephen Yoo, MD, Chief Medical Officer at REGENXBIO. “RGX-314 is designed to be a one-time treatment for wet AMD that is administered sub-retinally and results in the local production of an anti-VEGF antibody fragment, potentially eliminating the burden of additional anti-VEGF administrations. Our goal with this trial is to confirm prior observations in preclinical models that treatment with RGX-314 can induce a rapid and sustained reduction of VEGF protein in the eyes to provide long-lasting treatment.”

“I am excited by the potential of a one-time therapy for the many patients affected by wet AMD who require frequent and often burdensome anti-VEGF injections in the eye,” said Dr. Jeffrey S. Heier, principal investigator of the RGX-314 phase I trial, and co-president and medical director and director of retina research at Ophthalmic Consultants of Boston. “We know that as we deviate from regular injections, or at a minimum, regular examinations, that visual outcomes suffer. In many cases, such a schedule is unsustainable. A significant unmet need exists for a therapeutic option with long-lasting anti-VEGF potential. A one-time treatment, such as RGX-314, has the potential to be life changing for these patients.”

Six leading retinal surgery centers across the United States are expected to participate in the Phase I trial of RGX-314. 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 an interim trial update by the end of the year.

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 sub-retinal 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 RGX-314

RGX-314 is being developed as a one-time sub-retinal treatment for wet AMD. It includes the NAV AAV8 vector encoding an antibody fragment designed to neutralize VEGF activity, 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 sub-retinal 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 total and partial 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

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 Licensees are applying the NAV Technology Platform in the development of a broad pipeline of product candidates in multiple therapeutic areas.

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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 filed with the Securities and Exchange Commission (SEC). 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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