REGENXBIO Announces Completion of Dosing for Phase I/IIa Clinical Trial of RGX-314 in Wet AMD

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- Dosing complete for 42 subjects in five cohorts at eight leading U.S. retinal surgery centers
- Top-line data for RGX-314 Phase I/IIa trial for wet AMD expected by end of 2019
- On track to initiate RGX-314 Phase IIb trial for wet AMD by end of 2019

ROCKVILLE, Md., May 30, 2019 /PRNewswire/ -- 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 completed dosing across all five cohorts in the Phase I/IIa clinical trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD).

"We are excited to announce this important clinical milestone as we continue to drive the development of RGX-314 as a potential one-time gene therapy for patients with wet AMD," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "Patients with wet AMD require intravitreal injections every four to 12 weeks, on average, with the current standard of care. We were pleased to report durable treatment response from Cohort 3 of the RGX-314 Phase I/IIa trial for wet AMD at one year after a single administration of RGX-314 in a heavily pre-treated patient population in our interim trial update earlier this month," added Dr. Pakola. "We look forward to providing top-line data, including from Cohorts 4 and 5, in the Phase I/IIa trial by the end of the year."

Eight leading retinal surgery centers across the United States are participating in the Phase I/IIa trial of RGX-314, designed to evaluate the safety and tolerability of RGX-314 as a one-time therapy for patients with wet AMD who were previously treated with anti-vascular endothelial growth factor (VEGF) injections. The trial includes 42 dosed subjects across five escalating dose cohorts. Each subject received a single dose of RGX-314 administered by subretinal delivery.

"The sustained clinical durability of effect seen one year after one-time administration of RGX-314 in Cohort 3 demonstrates the potential of RGX-314 to provide foundational anti-VEGF therapy that may sustain vision gains and alleviate treatment burden for millions of patients suffering from wet AMD," added Robert Avery, M.D., trial investigator and retina surgeon from California Retina Consultants.

REGENXBIO is planning to initiate a Phase IIb trial in wet AMD by the end of 2019 based on the Phase I/IIa trial data and expand clinical development of RGX-314 by filing an Investigational New Drug (IND) application for diabetic retinopathy (DR) in the second half of 2019.

About the Phase I/IIa Clinical Trial of RGX-314

RGX-314 is being evaluated in a Phase I/IIa, multi-center, open-label, multiple-cohort, dose-escalation study in adult subjects with wet AMD in the United States. The study includes subjects previously treated for wet AMD who are responsive to anti-VEGF therapy. The study is designed to evaluate five escalating doses of RGX-314, with six subjects in the first three dose cohorts and 12 subjects in the fourth and fifth dose cohorts. Secondary endpoints include visual acuity, retinal thickness on spectral domain optical coherence tomography (SD-OCT), ocular RGX-314 protein expression, and the need for additional anti-VEGF therapy. Following completion of the primary study period, subjects enter a follow-up period and will continue to be assessed until week 106 for long-term safety and durability of effect.

About Wet AMD

Wet AMD is characterized by loss of vision due to new, leaky blood vessel formation in the retina. Wet AMD is a significant cause of vision loss in the United States, Europe and Japan, with up to 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 prevent progression of vision loss in the majority of patients. These therapies, however, require life-long intraocular injections, typically repeated every four to 12 weeks in frequency, to maintain efficacy. Due to the burden of treatment, patients often experience a decline in vision with reduced frequency of treatment over time.

About RGX-314

RGX-314 is being developed as a potential one-time subretinal treatment for wet AMD and DR. It includes the NAV AAV8 vector encoding an antibody fragment which inhibits VEGF, preventing the proliferation of leaky blood vessels which lead to retinal fluid accumulation and vision loss. In preclinical animal models to evaluate anti-VEGF therapies, prevention of disease progression was observed after a single subretinal dose of RGX-314.

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 future operations and clinical trials. 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 and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies 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, 2018 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.

CONTACT:

Investors
Heather Savelle, 212-600-1902
heather@argotpartners.com

Media
David Rosen, 212-600-1902
david.rosen@argotpartners.com

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