REGENXBIO Announces Dosing of First Patient in Phase II AAVIATE™ Trial of RGX-314 for the Treatment of Wet AMD Using Suprachoroidal Delivery

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- Interim data update from first cohort expected by end of 2020

- Targeted, in-office suprachoroidal delivery of RGX-314 using the SCS Microinjector® may provide additional administration options for patients with wet AMD

ROCKVILLE, Md., Sept. 9, 2020 /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 that the first patient has been dosed in the AAVIATE trial, a Phase II trial to evaluate the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet age-related macular degeneration (wet AMD). The Company expects to report interim data from the first cohort of this trial by the end of 2020.

"The initiation of this trial is an important milestone for REGENXBIO, as it is the first clinical trial to evaluate the delivery of any gene therapy, including RGX-314, to the suprachoroidal space. RGX-314 has shown evidence of potentially meaningful treatment effect in patients with wet AMD when delivered subretinally in the ongoing Phase I/IIa trial, and we are excited about the potential of the targeted, in-office suprachoroidal approach, which may provide additional RGX-314 delivery options for patients. We look forward to providing additional updates from this trial later this year," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO.

AAVIATE is a multi-center, open-label, randomized, active-controlled, dose-escalation trial that will evaluate the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314 using the SCS Microinjector, a targeted, in-office route of administration. The trial is expected to enroll approximately 40 patients with severe wet AMD across two cohorts. Patients in each cohort will be randomized to receive RGX-314 versus monthly 0.5 mg ranibizumab intravitreal injection at a 3:1 ratio, and two dose levels of RGX-314 will be evaluated: $2.5x10^{11}$ GC/eye and $5x10^{11}$ GC/eye. Patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

The primary endpoint of the trial is mean change in vision in patients dosed with RGX-314, as measured by best corrected visual acuity (BCVA), at Week 40 from baseline, compared to patients receiving monthly injections of ranibizumab. Other endpoints include mean change in central retinal thickness (CRT) and number of anti-VEGF intravitreal injections received following administration of RGX-314.

About RGX-314

RGX-314 is being developed as a potential one-time treatment for wet AMD, diabetic retinopathy, and other chronic retinal conditions. RGX-314 consists of the NAV AAV8 vector, which encodes an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). RGX-314 is believed to inhibit the VEGF pathway by which new, leaky blood vessels grow and contribute to the accumulation of fluid in the retina.

REGENXBIO is advancing two separate routes of administration of RGX-314 to the eye, through a standardized subretinal delivery procedure as well as delivery to the suprachoroidal space. REGENXBIO has licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. to deliver gene therapy treatments to the suprachoroidal space of the eye.

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 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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, 2019, 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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