REGENXBIO Announces Enrollment Complete in Cohort 5 of Phase II AAVIATE® Trial

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- ***85 subjects dosed across five cohorts in AAVIATE® trial evaluating RGX-314 for the treatment of wet AMD using suprachoroidal delivery***
- ***Company expects to report additional suprachoroidal data later this year***

ROCKVILLE, Md., July 8, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced it has completed enrollment in Cohort 5 of the Phase II AAVIATE® trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) using in-office suprachoroidal delivery.

"We are encouraged by the excellent progress we have made advancing RGX-314 suprachoroidal delivery in both our AAVIATE and ALTITUDE ™ trials for the treatment of wet AMD and diabetic retinopathy," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "RGX-314, has the potential through delivery to the suprachoroidal space to offer the millions of patients facing vision loss from these retinal diseases with a one-time, in-office treatment option. We plan to present additional suprachoroidal data this year."

Results from Cohort 1 of the Phase II ALTITUDE trial of RGX-314 for the treatment of diabetic retinopathy were presented at the Angiogenesis, Exudation, and Degeneration 2022 conference and will be encored at the upcoming American Society of Retina Specialists Annual Meeting.

AAVIATE Clinical Trial

The multi-center, open-label, randomized, active-controlled, dose-escalation Phase II AAVIATE trial is evaluating the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314 in patients with wet AMD using the Clearside SCS Microinjector®. Twenty patients in Cohort 1 were randomized to receive RGX-314 at a dose level of $2.5x10^{11}$ genomic copies per eye (GC/eye) through one injection versus monthly 0.5 mg ranibizumab intravitreal injection at a 3:1 ratio. Twenty patients in Cohort 2 were randomized to receive RGX-314 at a dose level of $5x10^{11}$ GC/eye through two injections versus monthly 0.5 mg ranibizumab intravitreal injection at a 3:1 ratio. Cohort 3 is evaluating RGX-314 at the same dose level as Cohort 2 in 20 patients who are neutralizing antibody (NAb) positive. Cohort 4 is evaluating RGX-314 in 15 patients at a dose level of $1x10^{12}$ GC/eye and Cohort 5 is evaluating the same dose level of RGX-314 in 20 patients who are NAb positive. Patients in these cohorts did not receive prophylactic immune suppressive corticosteroids before or after administration of RGX-314.

About RGX-314

RGX-314, being developed in collaboration with AbbVie, is being investigated 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 research in 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 repeated intraocular injections, 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, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

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," "assume," "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, 2021, 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. Except as required by law, 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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