REGENXBIO Announces Dosing of First Patient in Phase II ALTITUDE™ Trial of RGX-314 for the Treatment of Diabetic Retinopathy Using Suprachoroidal Delivery

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-Second Phase II trial to evaluate RGX-314 using the SCS Microinjector®
-Initial data from ALTITUDE expected in 2021

ROCKVILLE, Md., Dec. 10, 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 ALTITUDE, a Phase II trial to evaluate the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of diabetic retinopathy (DR).

"We are pleased to announce the first patient dosed in our ALTITUDE trial, an important milestone as we continue to evaluate the overall clinical profile of RGX-314 for the treatment of chronic retinal conditions. This is our second Phase II trial using the in-office suprachoroidal delivery approach, which may allow physicians to treat patients with diseases like DR earlier in the disease course," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "Long-term treatment with anti-VEGF injections has been shown to significantly reduce disease progression and severity of DR, even in patients who are asymptomatic, as well as prevent vision threatening complications. We believe that one-time treatment with anti-VEGF gene therapy can have a meaningful impact for patients with DR, and we look forward to providing additional updates from this trial next year."

"The SCS Microinjector is designed to provide targeted delivery of the gene therapy to the suprachoroidal space, with broad distribution to the back of the eye and into the retina through a one-time, in-office procedure, which could be an important alternative to current standard of care," said Charles Wykoff, M.D., Ph.D., trial investigator, Retina Specialist and Director of Research with Retina Consultants of Houston.

"DR is the most common cause of vision loss among patients with diabetes, with an average age of disease onset between 45-50 years old. Anti-VEGF injections have been approved by the FDA for diabetic retinopathy, but treatment with anti-VEGF agents requires frequent clinic visits and injections. RGX-314 may provide sustainable, long-term anti-VEGF protein production in the eye, which could be a one-time treatment option for working-age adults," said Arshad M. Khanani M.D., M.A., trial investigator and director of clinical research at Sierra Eye Associates.

ALTITUDE is a multi-center, open label, randomized, controlled dose-escalation trial that will evaluate the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314. The trial is expected to enroll approximately 40 patients with DR across two cohorts. Patients will be randomized to receive RGX-314 versus observational control at a 3:1 ratio, and two dose levels of RGX-314 will be evaluated: $2.5x10^{11}$ GC/eye and $5.0x10^{11}$ 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 the proportion of patients that improve in DR severity based on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS) at 48 weeks. Other endpoints include safety and development of DR-related ocular complications.

The Company expects to report initial data from this trial in 2021.

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 Diabetic Retinopathy

Diabetic retinopathy (DR) is the leading cause of vision loss in adults between 24 and 75 years of age worldwide. DR affects approximately eight million people in the United States alone. The spectrum of DR severity ranges from non-proliferative diabetic retinopathy (NPDR) to proliferative diabetic retinopathy (PDR) and as DR progresses, a large proportion of patients develop vision threatening complications, including diabetic macular edema (DME) and neovascularization that can lead to blindness. Current treatment options for patients with DR include "watchful waiting", anti-VEGF treatment, retinal laser or surgical treatment. Anti-VEGF treatments have been shown to reduce the severity of DR and prevent vision threatening complications by at least 75% in patients with moderately severe to severe NPDR.

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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