REGENXBIO Presents Positive Initial Data from Phase II ALTITUDE™ Trial of RGX-314 for the Treatment of Diabetic Retinopathy Using Suprachoroidal Delivery at American Society of Retina Specialists Annual Meeting

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- *Suprachoroidal delivery of RGX-314 well tolerated in 15 patients in Cohort 1 with no drug-related serious adverse events*
  - *No intraocular inflammation observed*
- *Positive interim data update from Cohort 1 at three months after one-time treatment of RGX-314*
  - *33% of patients demonstrated a ≥2 step improvement from baseline on the ETDRS-DRSS compared to 0% of patients in observational control*
- *Cohorts 2 and 3 continue to enroll patients at a dose level of $5x10^{11}$ GC/eye*

REGENXBIO Inc. (Nasdaq: RGNX) today announced initial data from the ongoing Phase II ALTITUDE™ trial of RGX-314 for the treatment of diabetic retinopathy (DR) without center-involved diabetic macular edema (CI-DME) using in-office suprachoroidal delivery. The data is being presented at the American Society of Retina Specialists (ASRS) Annual Meeting by Dennis Marcus, M.D., F.A.S.R.S., President, Southeast Retina Center. RGX-314 is a potential one-time gene therapy in clinical development for the treatment of wet age-related macular degeneration and DR.

"We are pleased to share initial data from the Phase II ALTITUDE trial, and we are encouraged to see treatment effect particularly at this early time point of three months after the one-time, in-office administration of RGX-314," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "RGX-314 is the first gene therapy in clinical trials for DR using suprachoroidal delivery, and has the potential to provide sustainable, long-term anti-VEGF protein production in the eye for the treatment of DR, which affects approximately eight million people in the United States alone. We look forward to providing additional updates on this trial next year."

"DR can start in young adulthood and often progresses quickly, leading to vision-threatening complications, including diabetic macular edema (DME) and neovascularization that can lead to vision loss," said Dr. Marcus. "Current treatment options include 'watchful waiting,' treatment requiring repeated anti-VEGF injections, retinal laser or surgical treatment. This first look at the ALTITUDE trial data is promising, as it shows not only clinical improvement in disease severity as measured by the ETDRS-DRSS, but also that this treatment is well tolerated in patients."

Study Design and Safety Update from Phase II ALTITUDE Trial of RGX-314 for the Treatment of DR Using Suprachoroidal Delivery

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 using the SCS Microinjector® in patients with a DR diagnosis of moderately severe or severe nonproliferative diabetic retinopathy (NPDR) or mild proliferative diabetic retinopathy (PDR). 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) versus observational control at a 3:1 ratio. Cohort 2 will include 20 patients randomized to receive RGX-314 at an increased dose level of $5x10^{11}$ GC/eye versus observational control at a 3:1 ratio. Cohort 3 is designed to evaluate RGX-314 at the same dose level as Cohort 2 in 20 patients who are neutralizing antibody (NAb) positive. Enrollment is ongoing in Cohorts 2 and 3. Patients in this trial do not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

As of September 29, 2021, RGX-314 was reported to be well tolerated in the 15 patients dosed with RGX-314 in Cohort 1. One serious adverse event was reported in one patient dosed with RGX-314, which occurred in the patient's untreated fellow eye and is considered not related to RGX-314. Among patients in Cohort 1 dosed with RGX-314, no intraocular inflammation was observed on slit-lamp examination. One patient experienced a mild case of episcleritis that resolved with topical corticosteroids. Common ocular treatment emergent adverse events in the study eye were not considered drug-related and were predominantly mild. These included conjunctival hemorrhage and conjunctival hyperemia.

Summary of Data for Cohort 1 at Three Months

Of the 15 patients dosed with RGX-314 in Cohort 1, five patients (33%) demonstrated a two-step or greater improvement from baseline on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS) at three months, compared to zero of the five patients (0%) in the observational control group. One patient dosed with RGX-314 had a four-step improvement.

In the seven patients who had NPDR (DR severity level 47-53) at baseline, three patients (43%) demonstrated a two-step or greater improvement at three months after administration of RGX-314. In the eight patients who had PDR (DR severity level ≥ 61) at baseline, two patients (25%) demonstrated a two-step or greater improvement at three months after administration of RGX-314.

Data presented today is available on the "Presentations and Publications" section of the REGENXBIO website at www.regenxbio.com.

About RGX-314

RGX-314 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 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.

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," "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, 2020 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.

Contacts:
Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

Investors:
Brendan Burns, 212-600-1902
brendan@argotpartners.com

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

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