REGENXBIO Presents Additional Positive Interim Data from Trials of RGX-314 in Wet AMD and Diabetic Retinopathy Using Suprachoroidal Delivery at AAO 2021

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- *Suprachoroidal delivery of RGX-314 in Phase II AAVIATE® trial for the treatment of wet AMD continues to be well tolerated in 50 patients from Cohorts 1-3 with no drug-related serious adverse events*
- *Positive initial data from Cohort 2 in AAVIATE trial at six months after one-time treatment of RGX-314*
  - *Treatment effect observed with stable visual acuity and retinal thickness*
  - *Demonstrated meaningful reduction (>70%) in anti-VEGF treatment burden; 40% of patients in Cohort 2 were anti-VEGF injection-free*
- *Additional data from Cohort 1 in Phase II ALTITUDE™ trial for the treatment of DR demonstrated stable visual acuity at three months after one-time treatment of RGX-314*

REGENXBIO Inc. (Nasdaq: RGNX) today announced additional positive interim data from the ongoing Phase II AAVIATE® trial and the ongoing Phase II ALTITUDE™ trial of RGX-314 using in-office suprachoroidal delivery for the treatment of wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR) without center-involved diabetic macular edema (CI-DME), respectively. The results were presented at the American Academy of Ophthalmology (AAO) 2021 Annual Meeting by Robert L. Avery, M.D., Founder of California Retina Consultants and Research Foundation.

"We are pleased to share this initial data from Cohort 2 of the AAVIATE trial which provides encouraging evidence of the emerging clinical profile of RGX-314 for the treatment of wet AMD using suprachoroidal delivery. In the data reported today, RGX-314 was observed to be well tolerated in Cohort 2, with stable visual acuity and retinal thickness as well as a meaningful reduction in anti-VEGF treatment burden at six months," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "We look forward to providing additional updates from the program."

"These initial results from patients in Cohort 2 of the AAVIATE trial at six months after suprachoroidal administration of RGX-314 reinforce the potential impact that RGX-314 could have on the overall clinical management of patients with wet AMD," said Dr. Avery. "I am encouraged by the six-month data in Cohort 2 and look forward to reviewing further data from Cohorts 1-3 and from the higher dose level in Cohorts 4 and 5."

Study Design and Safety Update in Phase II AAVIATE Trial of RGX-314 for the Treatment of Wet AMD Using Suprachoroidal Delivery

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®. 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 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. Enrollment is ongoing in two additional cohorts (Cohorts 4 and 5) to evaluate RGX-314 at a third dose level of $1x10^{12}$ GC/eye. Cohort 4 will enroll 15 patients who will be dosed with RGX-314 and Cohort 5 will evaluate the same dose level of RGX-314 in 20 patients who are NAb positive. Patients do not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

As of November 4, 2021, RGX-314 was reported to be well tolerated across 50 patients dosed in Cohorts 1-3. Four serious adverse events (SAEs) were reported in four patients, all of which were considered not related to RGX-314.[1] For the total group of Cohorts 1 and 2, all common treatment emergent adverse events (TEAEs) through 6 months in the study eye were mild, and included conjunctival hemorrhage, worsening of wet AMD, dry eye, episcleritis, and conjunctival hyperemia. Mild intraocular inflammation observed on slit-lamp examination was reported at similar incidence across both dose levels in Cohorts 1 and 2, with four out of 15 patients in Cohort 1 and three out of 15 patients in Cohort 2. All cases of inflammation in both cohorts were resolved within days to weeks on topical corticosteroids.

Summary of Data for Cohort 2 in Phase II AAVIATE Trial of RGX-314 for the Treatment of Wet AMD at Six Months

In Cohort 2, patients dosed with RGX-314 demonstrated stable Best Corrected Visual Acuity (BCVA) and central retinal thickness (CRT) at 6 months. Fifteen patients in Cohort 2 dosed with RGX-314 had a mean BCVA change of -0.1 letters (95% Confidence Interval: -3.8, 3.6) when measured from Day 1 (at Screening) and +0.2 letters (-2.7, 3.1) when measured from Week 1 (prior to Randomization). These patients also demonstrated stable central retinal thickness (CRT), with a mean change of -33 µm (-71, 5) at six months from Day 1. Ten control patients receiving monthly injections of ranibizumab in Cohorts 1 and 2 had a mean BCVA change at six months of +4.0 letters (-0.5, 8.5) when measured from Day 1 and +1.3 letters (-2.2, 4.8) when measured from Week 1. Patients receiving monthly injections of ranibizumab had a mean change of CRT of -12 µm (-33, 8) at six months from Day 1.

There was a meaningful reduction in anti-vascular endothelial growth factor (anti-VEGF) treatment burden in patients following administration of RGX-314 compared to the mean annualized injection rate during the 12 months prior to administration. Patients in Cohort 2 received a mean of 1.3 injections over six months following administration of RGX-314, which represents a 71.8% reduction in anti-VEGF treatment burden. Six out of 15 patients (40%) in Cohort 2 received no anti-VEGF injections over six months following RGX-314 administration. In these patients, visual acuity and CRT was observed to be stable from Day 1 over six months, with a mean change of BCVA of +1.0 letters (-3.8, 5.8), and a mean change of CRT of +8 µm (-9.2, 24.2).

Summary of Visual Acuity Data for Cohort 1 in Phase II ALTITUDE Trial of RGX-314 for the Treatment of Diabetic Retinopathy at Three Months

REGENXBIO shared additional data from Cohort 1 of the ongoing ALTITUDE trial for the treatment of DR without CI-DME using in-office suprachoroidal delivery, supporting the positive initial data previously reported at the American Society of Retina Specialists (ASRS) Annual Meeting in October 2021. At three months, 15 patients dosed with $2.5x10^{11}$ GC/eye of RGX-314 demonstrated stable BCVA of +2.6 letters, while five patients in the observational control arm demonstrated stable BCVA of -0.4 letters.

Data presented today are 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 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 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.

[1] One patient in Cohort 1 discontinued the study after Week 12 as a result of death, which was assessed to be unrelated to RGX-314. At the time of the death, the subject was free of anti-VEGF injections.

SCS Microinjector® is a trademark of Clearside Biomedical, Inc. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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