REGENXBIO Presents Positive Interim Data from and the Expansion of Phase II ALTITUDE® Trial of RGX-314 for the Treatment of Diabetic Retinopathy Using Suprachoroidal Delivery

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- *RGX-314 continues to be well tolerated in 50 patients from Cohorts 1-3 with no drug-related serious adverse events*
  - *Patients treated with RGX-314 in Cohorts 1-3 demonstrated clinically meaningful improvements in disease severity and less disease worsening versus observation control at six months*
  - *20% of patients achieved ≥2-step DRSS improvement vs. 10% in control*
  - *54% of patients achieved any DRSS improvement vs. 20% in control*
  - *0% of patients worsened ≥2 steps vs. 20% in control*
- *No meaningful differences in safety outcomes at six months for patients who are NAb positive*
- *Phase II trial expanded to include higher third dose level, with patients stratified by DRSS levels across cohorts and all receiving short-course prophylactic ocular steroids following RGX-314 administration*

ROCKVILLE, Md., Nov. 3, 2022 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced additional positive interim 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 were presented at the 55th Annual Retina Society meeting in Pasadena, CA by Lejla Vajzovic, MD, FASRS, Associate Professor of Ophthalmology and Director of Duke Vitreoretinal Fellowship Program, Vitreoretinal Surgery and Disease, Department of Ophthalmology, Duke University School of Medicine. RGX-314 is being investigated as a potential one-time gene therapy for the treatment of wet age-related macular degeneration, DR, and other chronic retinal conditions.

"We are pleased with these positive interim results which demonstrate a clinically meaningful improvement in disease severity versus observational control, with more than 50% of patients dosed with RGX-314 in Cohorts 1-3 seeing improvement from baseline in their DRSS scores," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "RGX-314 continues to be well-tolerated across cohorts, and we look forward to the expansion of this trial to further evaluate the potential of RGX-314 for patients with DR."

"I am encouraged by the positive results observed in the ALTITUDE trial of RGX-314," said Dr. Vajzovic. "A one-time, in-office injection of RGX-314 gene therapy could potentially provide critical improvement in DR severity and reduce the risk of vision threatening complications. Early intervention is key in slowing disease progression for DR patients, and RGX-314 has the potential to provide an important treatment option to address the current unmet need for these patients. I look forward to seeing additional results from Cohorts 4 and 5 of the ALTITUDE trial."

"DR is the leading cause of blindness in working-age adults globally, and there is a significant need for new treatment options," said Michael Robinson, MD, Vice President, Global Therapeutic Area Head Eye Care at AbbVie. "Progressing RGX-314 and the ALTITUDE trial with the aim of addressing unmet needs for patients with retinal diseases is part of AbbVie's commitment to advancing vision care."

Data Summary 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 evaluating 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). Patients in Cohort 1 received RGX-314 at a dose level of $2.5x10^{11}$ genomic copies per eye (GC/eye) (D1). Patients in Cohorts 2 and 3 received RGX-314 at an increased dose level of $5x10^{11}$ GC/eye (D2). Patients in Cohorts 1-3 did not receive prophylactic corticosteroid therapy before or after administration of RGX-314.

As of October 17, 2022, RGX-314 was reported to be well tolerated in Cohorts 1-3. Five serious adverse events were reported, none of which were considered drug related. For the total group of Cohorts 1-3 (n=50), common ocular treatment-emergent adverse events in the study eye through six months were predominantly mild and included conjunctival hemorrhage, conjunctival hyperemia and episcleritis. In addition, three patients had intraocular inflammation (IOI), all mild and resolved on topical corticosteroids. There were no meaningful differences in safety outcomes observed for patients who are neutralizing antibody (NAb) positive. Best Corrected Visual Acuity remained stable in Cohorts 1-3 through six months.

At six months, patients treated with RGX-314 demonstrated clinically meaningful improvements in disease severity versus observation control as measured by the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS). Specifically:

- 20% (D1: 40%, D2: 11%) achieved ≥2-step DRSS improvement vs. 10% in control
- 54% (D1: 60%, D2: 51%) achieved any DRSS improvement vs. 20% in control
- 0% (D1: 0%, D2: 0%) worsened ≥2 steps vs. 20% in control

A ≥2-step improvement in DRSS at 12 months has been accepted as a pivotal endpoint by the U.S. Food and Drug Administration for DR clinical trials.

Trial Expansion

REGENXBIO announced today that the ALTITUDE trial has been expanded to include a higher third dose level of $1x10^{12}$ GC/eye. The trial is currently enrolling two new cohorts (Cohorts 4 and 5) at the third dose level. Cohorts 4 and 5 are enrolling patients stratified by DRSS levels, with patients in Cohort 4 having moderately severe or severe NPDR DRSS levels of 47-53 and patients in Cohort 5 having mild to moderate PDR DRSS levels of 61-65. Patients in these cohorts will receive short-course, prophylactic ocular corticosteroids following RGX-314 to evaluate the ability to prevent or reduce the occurrence of the mild intraocular inflammation seen to date. Patients will be enrolled in these cohorts regardless of baseline AAV8 NAb status.

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

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 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 10 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. Chronic, frequent treatment with anti-VEGF agents has been shown to improve DR severity and reduce risk of progression to vision threatening complications by > 70%. However, the majority of DR patients without vision threatening complications are not treated with anti-VEGF due to the unsustainable treatment burden of frequent injections in the eye.

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.

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

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