REGENXBIO Presents Positive One Year Data from Phase II ALTITUDE® Trial of ABBV-RGX-314 for Treatment of Diabetic Retinopathy Using Suprachoroidal Delivery

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- *ABBV-RGX-314 continues to be well tolerated in 50 patients from dose levels 1 and 2 (Cohorts 1-3) with no drug-related serious adverse events*
- *Dose level 2 prevented disease progression and reduced vision-threatening events in non-proliferative diabetic retinopathy patients at 1 Year*
  - *70.8% of patients achieved Diabetic Retinopathy Severity Scale improvement vs. 25.0 % in control*
  - *0% of patients worsened ≥2 steps vs. 37.5 % in control*
  - *ABBV-RGX-314 reduced vision-threatening events by 89% compared to control*
- *Conference call Monday, November 6, 8:30 a.m. ET*

ROCKVILLE, Md., Nov. 3, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced additional positive data from the ongoing Phase II ALTITUDE® trial of ABBV-RGX-314 for the treatment of diabetic retinopathy (DR) without center-involved diabetic macular edema (CI-DME) using in-office suprachoroidal delivery. The data will be presented at the American Academy of Ophthalmology meeting in San Francisco, CA by Mark Barakat, M.D., Retinal Consultants of Arizona. ABBV-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 that ABBV-RGX-314 at dose level 2 continues to be well tolerated and demonstrate clinically meaningful improvements for patients with non-proliferative DR," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "A one-time, in-office injection of ABBV-RGX-314 gene therapy has the potential to stabilize and improve DR severity score and reduce the long-term risk of vision-threatening events."

"DR is a leading cause of vision loss in working-age adults and is a global public health concern, and I am encouraged by the one-year positive results observed in the ALTITUDE trial of ABBV-RGX-314," said Dr. Barakat. "There is an unmet need for sustainable treatment options that prevent the progression of early diabetic retinopathy to proliferative diabetic retinopathy and development of vision threatening complications over the long term. I look forward to seeing additional data from the ALTITUDE trial of a one-time, in-office treatment of ABBV-RGX-314."

Data and Safety Summary

ALTITUDE is a multi-center, open-label, randomized, controlled, dose-escalation trial evaluating the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 using the SCS Microinjector® in patients with moderately severe or severe non-proliferative diabetic retinopathy (NPDR) or mild proliferative diabetic retinopathy (PDR). Patients in Cohort 1 received ABBV-RGX-314 at a dose level of $2.5x10^{11}$ genomic copies per eye (GC/eye) (dose level 1). Patients in Cohorts 2 and 3 received ABBV-RGX-314 at an increased dose level of $5x10^{11}$ GC/eye (dose level 2). Patients in Cohorts 1-3 did not receive prophylactic corticosteroid therapy before or after administration of ABBV-RGX-314.

As of September 25, 2023, ABBV-RGX-314 was reported to be well tolerated at dose levels 1 and 2. Seven serious adverse events were reported, none of which were considered drug related. For patients in dose levels 1 and 2 (n=50), common ocular treatment-emergent adverse events in the study eye through one year included conjunctival hemorrhage and conjunctival hyperemia. Three patients had mild intraocular inflammation (IOI), which resolved on topical corticosteroids. Six patients had mild to moderate episcleritis and have resolved on topical corticosteroids.

No cases of chorioretinitis, vasculitis, occlusion, or hypotony were reported. Best Corrected Visual Acuity remained stable through one year.

At one year, dose level 2 in NPDR patients prevented disease progression as measured by the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (DRSS). Importantly, dose level 2 reduced the risk of developing vision-threatening events by 89% in these patients.

NPDR patients treated with ABBV-RGX-314 at dose levels 1 and 2 demonstrated clinically meaningful improvements in disease severity and reduction of vision-threatening events. In dose level 2, patients with baseline NPDR:

- 100% demonstrated stable to improved disease severity
  - 70.8% achieved ≥1 step improvement vs. 25.0% in control
  - 0% worsened ≥2 steps vs. 37.5% in control
- 4.2% developed vision-threatening events vs. 37.5% in control

In connection with this announcement, REGENXBIO will host a conference call to discuss the Phase II ALTITUDE trial data with Dr. Barakat and Dr. Peter Kaiser, Chaney Family Endowed Chair in Ophthalmology Research and Professor of Ophthalmology, Cleveland Clinic Lerner College of Medicine and Cole Eye Institute, and Dr. Pakola on Monday, November 6 at 8:30 a.m. ET. Listeners can register for the webcast via this link. Analysts

wishing to participate in the question and answer session should use this link. A replay of the webcast will be available via the company's investor website approximately two hours after the call's conclusion. Those who plan on participating are advised to join 15 minutes prior to the start time.

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

About ABBV-RGX-314
ABBV-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. ABBV-RGX-314 consists of the NAV® AAV8 vector, which encodes an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). ABBV-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 ABBV-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 and AAV9. 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, 2022, 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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