REGENXBIO Presents Positive Initial Data from Phase II AAVIATE® Trial of RGX-314 for the Treatment of Wet AMD Using Suprachoroidal Delivery and Provides Trial Update at Retina Society 54th Annual Scientific Meeting

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- *Suprachoroidal delivery of RGX-314 well tolerated in 50 patients in Cohorts 1-3 with no drug-related serious adverse events*
- *Positive initial data from Cohort 1 at six months after one-time treatment of RGX-314*
  - *Treatment effect observed with stable visual acuity and retinal thickness*
  - *Demonstrated meaningful reduction in anti-VEGF treatment burden*
- *Phase II trial expanded to include third dose level of RGX-314 ($1x10^{12}$ GC/eye)*
- *Company to host conference call and webcast on Friday, October 1 at 5:00 p.m. ET, featuring wet AMD Key Opinion Leaders Nikolas London, M.D., and Peter Campochiaro, M.D.*

REGENXBIO Inc. (Nasdaq: RGNX) today announced initial data from the ongoing Phase II AAVIATE® trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) using in-office suprachoroidal delivery. The data is being presented at the Retina Society 54th Annual Scientific Meeting by Nikolas London, M.D., M.S., F.A.C.S., Partner and Director of Research, Retina Consultants of San Diego, Chief of Ophthalmology, Scripps Memorial Hospital. RGX-314 is a potential best-in-class, one-time gene therapy for the treatment of wet AMD.

"We are pleased to share initial data from the Phase II AAVIATE trial, and we are encouraged by the emerging clinical profile of RGX-314 for the treatment of wet AMD using suprachoroidal delivery, a delivery mechanism which we believe could provide access to gene therapy treatment in all settings of patient care," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "The observed stability of visual acuity and retinal thickness in the backdrop of reduced anti-VEGF injections is encouraging at this first dose level. We look forward to further enhancing our understanding of the potential of RGX-314 when delivered to the suprachoroidal space, as we continue to evaluate two higher dose levels in this trial."

"These are the first data ever reported from a gene therapy delivered to the suprachoroidal space of the eye in a clinical trial," said Dr. London. "The data from patients in Cohort 1 at six months after RGX-314 administration demonstrate the potential benefit of one-time administration of RGX-314 to the suprachoroidal space for the treatment of wet AMD. I look forward to seeing additional data from the AAVIATE trial."

Study Design and Safety Update from 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 an increased dose level of $5x10^{11}$ GC/eye versus monthly 0.5 mg ranibizumab intravitreal injection 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. Patients in all three cohorts did not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314. Enrollment is complete across these three cohorts.

As of September 13, 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]

Summary of Data for Cohort 1 at Six Months

Fourteen patients dosed with RGX-314 in Cohort 1 demonstrated stable visual acuity at six months with a mean Best Corrected Visual Acuity (BCVA) change of -2.8 letters (95% Confidence Interval: -7.0, 1.4) when measured from Day 1 (at Screening) and -0.6 letters (-5.2, 4.0) when measured from Week 1 (prior to Randomization). These patients also demonstrated stable central retinal thickness (CRT), with a mean change of -2.5 µm (-27.1, 22.0) at six months from Day 1. Five patients receiving monthly injections of ranibizumab had a mean BCVA change at six months of +6.8 letters (-3.3, 16.9) when measured from Day 1 and +3.0 letters (-4.7, 10.7) when measured from Week 1. Patients receiving monthly injections of ranibizumab also demonstrated stable CRT, with a mean change of -22.2 µm (-41.6, -2.8) 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 1 received a mean of 1.2 injections over six months following administration of RGX-314, which represents a 75.9% reduction in anti-VEGF treatment burden. Four out of fourteen patients 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 +1.3 letters (-5.7, 8.2), and a mean change of CRT of -5.8 µm (-49.5, 38.0).

As of September 13, 2021, among patients in Cohort 1 dosed with RGX-314, common treatment emergent adverse events (TEAE) in the study eye were generally mild, and none were severe. These included conjunctival hemorrhage, worsening of wet AMD, conjunctival hyperemia, and dry eye. In addition, mild intraocular inflammation was observed in four out of 15 patients based on slit-lamp examination. All cases were resolved within days to weeks on topical corticosteroids, which have been discontinued.

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

AAVIATE Trial Expansion to Dose Level 3

REGENXBIO announced today that the AAVIATE study has expanded, and two additional cohorts (Cohorts 4 and 5) will be enrolled 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. As in previous cohorts, patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

Conference Call

In connection with this announcement, REGENXBIO will host a webcast and conference call with accompanying slides today at 5:00 p.m. ET. This event will feature Nikolas London, M.D., M.S., F.A.C.S., Partner and Director of Research, Retina Consultants of San Diego, Chief of Ophthalmology, Scripps Memorial Hospital, and Peter Campochiaro, M.D., Director, Retinal Cell and Molecular Laboratory, Professor of Ophthalmology, The Wilmer Eye Institute, The Johns Hopkins University School of Medicine.

To access a live or recorded webcast of the call and accompanying slides, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international) and enter the passcode 4577338. The recorded webcast will be available for approximately 30 days following the call.

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 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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