



## REGENXBIO Announces Positive Interim Data from Phase II AAVIATE® Trial of ABBV-RGX-314 for the Treatment of Wet AMD Using Suprachoroidal Delivery

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- *A single ABBV-RGX-314 gene therapy treatment has the potential to become a new standard-of-care option among anti-VEGF treatments by sustaining vision health long term and overcoming the clinical challenges of managing wet AMD due to the treatment burden of chronic anti-VEGF injections*
- *ABBV-RGX-314 continues to be well tolerated in over 100 patients from three dose levels with no drug-related serious adverse events*
- *ABBV-RGX-314 in over 50 patients at third dose level demonstrated highest reduction in treatment burden:*
  - *80% reduction in annualized injection rate*
  - *50% injection-free*
- *Zero cases of intraocular inflammation observed in patients that received short-course prophylactic topical steroid eye drops*
- *Conference call Tuesday, January 16 at 4:30 p.m. EST*

ROCKVILLE, Md., Jan. 16, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced positive interim data from the Phase II AAVIATE® trial of ABBV-RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) using suprachoroidal delivery. Wet AMD is a chronic, life-long disease with available anti-VEGF treatment options that may reduce the risk of blindness, but require frequent injections. Investigational ABBV-RGX-314 using suprachoroidal delivery is designed to be a one-time, in office treatment that has the potential to sustain constant anti-VEGF therapy and stabilize or improve vision long-term for wet AMD patients. The new data presented at the Hawaiian Eye and Retina meeting in Maui, HI by John Pitcher, M.D., Eye Associates of New Mexico, includes 6-month results from two additional dose level 3 cohorts (Cohorts 5 and 6). ABBV-RGX-314, being developed in collaboration with AbbVie, is also being investigated as a potential one-time gene therapy for the treatment of diabetic retinopathy and other chronic retinal conditions.



"The potential of ABBV-RGX-314 to transform the way chronic retinal conditions are treated has been demonstrated in multiple trials using both subretinal and suprachoroidal delivery, and these latest interim results from the AAVIATE trial continue to support this outlook," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "One-time, in-office treatment with ABBV-RGX-314 continues to be well tolerated in patients with wet AMD. We are pleased that dose level 3 in this trial has shown the highest reduction in treatment burden with zero cases of inflammation observed in patients who received prophylactic short-course topical steroids. We are encouraged by this data as part of the global ABBV-RGX-314 program."

"Real world evidence shows that patients with wet AMD are often undertreated due to the unsustainable treatment burden of frequent injections. As a result, many wet AMD patients experience significant vision loss over time," said Dr. Pitcher. "I am encouraged by the safety profile of ABBV-RGX-314 using suprachoroidal delivery and the potential of a one-time, in-office injection that can sustain constant anti-VEGF therapy to address a major unmet need for patients."

### Data and Safety Summary

AAVIATE is a Phase II, multi-center, open-label, randomized, active-controlled, dose-escalation trial that is evaluating the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 in patients with wet AMD. The primary endpoint of the trial is mean change in vision in patients dosed with ABBV-RGX-314, as measured by best corrected visual acuity (BCVA) at Week 40 from baseline, compared to patients receiving monthly injections of ranibizumab. Other endpoints include mean change in central retinal thickness (CRT) and number of anti-vascular endothelial growth factor (anti-VEGF) intravitreal injections received following administration of ABBV-RGX-314.

As of November 6, 2023, ABBV-RGX-314 suprachoroidal delivery was well tolerated across 106 patients from three dose levels. No drug-related SAEs were reported. All treatment emergent adverse events (TEAEs) through 6 months in the study eye were mild or moderate and included conjunctival hemorrhage, increased intraocular pressure, episcleritis, and conjunctival hyperemia. Mild intraocular inflammation was reported at similar incidence rate in the first and second dose levels, with mild to moderate intraocular inflammation seen at the third dose level in Cohort 4 and 5. All intraocular inflammation resolved with topical corticosteroids. Notably, there were zero cases of intraocular inflammation in dose level 3, Cohort 6 (n=21), in which patients received a short-course of prophylactic topical steroids following administration of ABBV-RGX-314.

Patients treated with ABBV-RGX-314 continue to demonstrate stable BCVA and CRT at six months. In addition, a meaningful reduction in anti-VEGF treatment burden was observed following administration of ABBV-RGX-314. The highest reduction was seen in dose level 3, demonstrating an 80% reduction in annualized injection rate with 50% of patients remaining injection-free.

Data presented is available on the "Publications" section of the REGENXBIO website at [www.regenxbio.com](http://www.regenxbio.com).

#### Conference Call

In connection with this announcement, REGENXBIO will host a conference call to discuss the Phase II AAVIATE trial data with Dr. Pitcher and Allen Ho, M.D., FACS, FASRS, Co-Director, Wills Eye Retina Service and Director, Retina Research, on Tuesday, January 16 at 4:30 p.m. EST. 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.

#### About the AAVIATE<sup>®</sup> Trial

The multi-center, open-label, randomized, active-controlled, dose-escalation Phase II AAVIATE trial is evaluating the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 in patients with wet AMD using the Clearside SCS Microinjector<sup>®</sup>. Patients have been dosed across three dose levels:  $2.5 \times 10^{11}$  (Cohort 1),  $5 \times 10^{11}$  (Cohorts 2 and 3), and  $1 \times 10^{12}$  (Cohorts 4-6) genomic copies per eye (GC/eye). Cohort 5 is evaluating ABBV-RGX-314 in 20 patients who are NAB positive. Cohort 6 includes a short course of prophylactic steroids following administration of ABBV-RGX-314.

#### 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<sup>®</sup> 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<sup>®</sup> 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, it is difficult for patients to adhere to frequent injections, which can lead to a decline in vision 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 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 "5x25" 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, 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](http://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<sup>®</sup> is a trademark of Clearside Biomedical, Inc. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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