REGENXBIO Presents Positive Data from the Phase II Study of Subretinal ABBV-RGX-314 in Patients with Bilateral Wet AMD at AAO 2024

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- *97% reduction in treatment burden at nine months after treatment with ABBV-RGX-314*
- *Data consistent with that from multiple previous studies demonstrating favorable safety and efficacy profile*
- *Well tolerated with zero cases of intraocular inflammation in a setting of no prophylactic steroids*
- *Data highlight the potential of ABBV-RGX-314 to treat both eyes in wet AMD*

ROCKVILLE, Md., Oct. 21, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced positive data from the Phase II fellow eye sub-study evaluating the subretinal delivery of ABBV-RGX-314 in patients with bilateral wet age-related macular degeneration (wet AMD). The new data were presented at the American Academy of Ophthalmology (AAO) meeting by Arshad Khanani, M.D., M.A., FASRS, Director of Clinical Research at Sierra Eye Associates, Reno, NV.

"The results presented at AAO from the Phase II sub-study, the first to evaluate a gene therapy in fellow eyes for wet AMD, demonstrate that ABBV-RGX-314 could be a treatment for patients with bilateral disease and add to the already-robust data demonstrating ABBV-RGX-314's potential to impact the treatment paradigm for patients with wet AMD," said Curran Simpson, President and Chief Executive Officer of REGENXBIO. "With more treated patients and the longest-term data of any gene therapy program for wet AMD, REGENXBIO, with our partner AbbVie, are well-positioned to bring the first gene therapy to market with the hope of preserving long-term vision for millions of patients globally with wet AMD."

"The majority of our patients with wet AMD eventually have bilateral disease and face a substantial treatment burden with frequent lifelong injections in both eyes. This leads to suboptimal real-world vision outcomes with current standard of care," said Dr. Khanani. "The fellow eye dosing data with ABBV-RGX-314 is a milestone for the field of gene therapy for common retinal diseases, as this is the first time we have performed bilateral treatment for wet AMD patients. These results, combined with the durable treatment effect up to four years shown in long-term follow up, highlight the potential of ABBV-RGX-314 as a one-time effective treatment option for patients with wet AMD."

Data and Safety Summary

The fellow eye sub-study was designed to evaluate the safety and efficacy of a single dose ($1.3x10^{11}$ GC/eye) of ABBV-RGX-314 using subretinal delivery in the fellow eye of previously treated patients. The second eye was treated with ABBV-RGX-314 approximately one year or more after administration of ABBV-RGX-314 in the first eye. This dose is being evaluated in the ongoing pivotal trials of ABBV-RGX-314 and is similar to one of the doses evaluated in the Phase I/IIa trial, which demonstrated durable treatment effect up to four years in a long-term follow up study.

The fellow eye sub-study data at nine months includes nine patients who received ABBV-RGX-314 using subretinal delivery in the Phase I/IIa or bridging studies and elected to receive treatment in their second eye. Prior to ABBV-RGX-314 administration, these patients had a high treatment burden in the fellow eye and had received an average of nine anti-VEGF injections in the year prior to entering the study, including anti-VEGF injections intended to be longer-lasting treatments.

At nine months post-administration of ABBV-RGX-314, key findings from the Phase II fellow eye sub-study include:

- 97% reduction in annualized anti-VEGF treatment burden
- 100% of patients required either zero or one supplemental injection
- 78% of patients were completely injection-free

Additionally, patients demonstrated sustained best-corrected visual acuity (BCVA) and central retinal thickness (CRT) at nine months. ABBV-RGX-314 produced similar levels of aqueous protein in both treated eyes.

As of September 11, 2024, ABBV-RGX-314 was well tolerated in the treated fellow eye with no drug related serious adverse events. No cases of intraocular inflammation, chorioretinitis, vasculitis, occlusion or hypotony were observed. No prophylactic steroids were used in this trial, other than those typically used in vitrectomy surgery. Common adverse events included mild retinal pigmentary changes occurring in periphery and post-operative conjunctival hemorrhage, which all resolved within days to weeks.

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 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 frequent, 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. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for retinal and rare diseases, including ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, RGX-202 for the treatment of Duchenne and RGX-121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.regenxbio.com.

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, 2023, 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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