REGENXBIO Announces Lancet Publication of Phase I/IIa Study Evaluating ABBV-RGX-314 as a One-Time Gene Therapy for Wet AMD

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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*
- *Patients who received therapeutic doses resulted in stable or improved vision and retinal anatomy up to 2 years*
- *Additional long-term follow-up data has demonstrated durable treatment effect, with stable or improved vision, up to 4 years*
- *Enrollment is on track in pivotal trials of ABBV-RGX-314 in wet AMD that are expected to support global regulatory submissions in late 2025 through the first half of 2026*

ROCKVILLE, Md., March 28, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the publication of results from the Phase I/IIa trial evaluating the safety and tolerability of a single dose of subretinal ABBV-RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). Two-year data were published in *The Lancet* in a paper titled "Gene therapy for neovascular age-related macular degeneration by subretinal delivery of RGX-314: a phase 1/2a dose-escalation study." These positive study results informed the ongoing pivotal trials of ABBV-RGX-314, a potential one-time gene therapy, for the treatment of wet AMD.

"We have started 2024 with strong, positive new data from the ABBV-RGX-314 program, and we believe that there is multi-billion-dollar potential for ABBV-RGX-314 to become a first-in-class gene therapy for wet AMD and the standard of care to treat and prevent progression of diabetic retinopathy," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "To have these Phase I/IIa data published in *The Lancet* highlights the groundbreaking work of our scientists and investigators, and further validates the clinically transformative nature of ABBV-RGX-314 as a potential one-time gene therapy for wet AMD that may help patients maintain or improve their vision."

The findings highlighted in *The Lancet* demonstrated that a single administration of ABBV-RGX-314 was generally well tolerated. Stable or improved visual acuity and retinal thickness was observed with few or no supplemental anti-VEGF injections in most patients at two years. Patients who received therapeutic doses demonstrated sustained levels of ABBV-RGX-314 protein and stable or improved vision and retinal anatomy with few, to no, supplemental anti-VEGF injections in most participants up to two years. REGENXBIO has also reported additional positive interim data from a long-term follow-up study of ABBV-RGX-314 supporting that treatment continues to be well-tolerated and demonstrates long-term, durable treatment effect up to four years.

"The publication of the ABBV-RGX-314 Phase I/IIa trial results in *The Lancet* reinforces the encouraging long-term clinical data observed using subretinal delivery and underscores the potential of ABBV-RGX-314 gene therapy to offer a new approach to the clinical management of wet AMD," said Jeffrey S. Heier, M.D., Director of the Vitreoretinal Service and Director of Retina Research at Ophthalmic Consultants of Boston and primary investigator for the trial. "Wet AMD is a chronic, life-long disease and real-world evidence shows patients are losing significant vision over time, and the burden of frequent anti-VEGF injections needed to manage their wet AMD is a major reason why. A single treatment of ABBV-RGX-314 that can potentially provide long-lasting treatment outcomes and a strong safety profile would offer a novel approach to treating this serious and blinding disease."

ABBV-RGX-314 is currently being evaluated in patients with wet AMD in two pivotal trials called ATMOSPHERE® and ASCENT™. Enrollment is on track and these trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration and the European Medicines Agency in late 2025 through the first half of 2026.

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.

Contacts:

Dana Cormack
Corporate Communications
dcormack@regenxbio.com

Investors:
Chris Brinzey, ICR Westwicke
339-970-2843
chris.brinzey@westwicke.com

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