REGENXBIO Announces Completion of Enrollment in Pivotal Trials of Subretinal Surabgene Lomparvovec for Wet AMD

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- *Over 1,200 participants enrolled in ATMOSPHERE® and ASCENT® pivotal trials, representing largest global gene therapy program ever conducted*
- *Subretinal surabgene lomparvovec on track to be first gene therapy for wet AMD*
- *Topline pivotal data expected in Q4 2026*

ROCKVILLE, Md., Oct. 6, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the completion of enrollment in the ATMOSPHERE® and ASCENT® pivotal studies evaluating surabgene lomparvovec (sura-vec, ABBV-RGX-314) in wet age-related macular degeneration (wet AMD) using subretinal delivery.

"Completing enrollment in this large, global pivotal program is an exciting milestone in our efforts to deliver sura-vec to patients as the potential first gene therapy for wet AMD," said Steve Pakola, M.D., Chief Medical Officer, REGENXBIO. "The millions of patients worldwide with wet AMD are in need of a treatment option that can preserve vision, prevent disease progression, and reduce the significant burden of frequent, life-long eye injections required with today's standard of care. We are highly encouraged by the safety and long-term durability seen in multiple earlier-stage trials. We look forward to sharing the topline data next year and advancing global registration of this potentially transformative treatment."

ATMOSPHERE and ASCENT are multi-center, randomized, active-controlled trials designed to support the global regulatory submissions of sura-vec. ATMOSPHERE, conducted in the U.S., is evaluating sura-vec versus ranibizumab; ASCENT, conducted in the U.S. and 13 other countries, is evaluating sura-vec versus aflibercept. The primary endpoint is non-inferiority based on change from baseline in Best Corrected Visual Acuity (BCVA) at 54 weeks and one year, respectively. Secondary endpoints include safety and tolerability, change in central retinal thickness (CRT) and need for supplemental anti-VEGF injections in the treatment arms. Together, these pivotal studies have enrolled over 1,200 participants across more than 200 sites.

In a long-term follow up study, sura-vec was well tolerated and demonstrated durable treatment effect with stable or improved vision up to four years. An additional Phase II pharmacodynamic study evaluated sura-vec at the same dose levels as the pivotal trials. At one year, sura-vec was well tolerated in 60 treated participants with no drug-related serious adverse events, stable to improved vision and anatomy, and meaningful reduction in anti-VEGF injections in participants with high treatment burden.

Topline data is expected in the fourth quarter of 2026.

About Surabgene Lomparvovec (sura-vec, ABBV-RGX-314)
Sura-vec is being investigated as a potential one-time treatment for wet AMD, diabetic retinopathy and other chronic retinal conditions. Sura-vec consists of the NAV® AAV8 vector, which encodes an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). Sura-vec is believed to inhibit the VEGF pathway by which new, leaky blood vessels grow and contribute to the accumulation of fluid in the retina.

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 has been shown to lead to a decline in vision over time.

ABOUT REGENXBIO Inc.
REGENXBIO is a biotechnology company on a mission to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the field of AAV gene therapy. REGENXBIO is advancing a late-stage pipeline of one-time treatments for rare and retinal diseases, including RGX-202 for the treatment of Duchenne; clemidsogene lanparvovec (RGX-121) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku; and surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie. Thousands of patients have been treated with REGENXBIO's AAV platform, including those receiving Novartis' ZOLGENSMA®. REGENXBIO's investigational gene therapies 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 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, 2024, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the 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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