REGENXBIO Presents Positive Three-Year Durability Data for Surabgene Lomparvovec in Diabetic Retinopathy at Retina Society Annual Scientific Meeting

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- *Majority of Dose Level 3 participants with NPDR demonstrated a > 2-step DRSS improvement at 3 years post-one-time, in-office sura-vec gene therapy treatment, with no vision-threatening events and no additional treatment for diabetic retinopathy*
- *No IOI observed with short-course prophylactic topical steroids*

ROCKVILLE, Md., Sept. 24, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced positive long-term follow-up data from the Phase II ALTITUDE® study of investigational surabgene lomparvovec (sura-vec, ABBV-RGX-314) in diabetic retinopathy (DR) using suprachoroidal delivery. These new data were presented at the Retina Society 59th Annual Scientific Meeting in Los Angeles.

"These latest long-term results are highly encouraging and reinforce the potential of sura-vec to change the treatment landscape for the millions of patients with non-proliferative diabetic retinopathy," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "Building on the positive data seen at two and two and a half years after a single treatment, the three-year data continues to show meaningful improvements in DRSS alongside a reduction in disease progression. The durability of these results strengthens our conviction in sura-vec as a potentially transformative, one-time treatment."

"For patients with non-proliferative diabetic retinopathy, the goal is to intervene before the disease progresses to vision-threatening complications and irreversible vision loss. However, current preventive treatment requires repeat injections, which is a significant burden for a population that is largely working-age adults," said Nikolas London, M.D., FACS, FASRS, Retina Consultants San Diego, ALTITUDE study investigator. "These three-year ALTITUDE results are encouraging and support continued study of sura-vec as a one-time, in-office treatment with the potential to change the course of the disease. A single treatment that reduces or eliminates the need for ongoing intraocular injections, and reduces the risk of vision-threatening complications, would be a meaningful change for our diabetic patients."

Phase II ALTITUDE Long-term follow up data (as of August 17, 2026)

In long-term follow-up from the Phase II ALTITUDE® trial, a single in-office administration of sura-vec using suprachoroidal delivery in participants with non-proliferative DR (NPDR) at Dose Level 3 (1.0×10^{12} GC/eye) with short-course prophylactic topical steroids demonstrated a durable efficacy profile at three years with no new sura-vec-related safety signals observed during long-term follow up. 60% of all Dose Level 3 participants with three-year visits (n=6/10) achieved ≥2-step improvement on the Diabetic Retinopathy Severity Scale (DRSS) without additional treatment for diabetic retinopathy.[1] These participants did not experience any vision-threatening events.

Additionally, the majority of participants (n=3/4) who achieved a 1-step DRSS improvement at one year without supplemental anti-VEGF injections went on to achieve a ≥2-step DRSS improvement by three years without additional treatment for diabetic retinopathy.

There were no new sura-vec-related safety signals and no intraocular inflammation observed through three years (n=17) with short-course prophylactic topical steroids.

These data are consistent with previously presented two and a half-year Dose Level 3 NPDR data from the ALTITUDE trial and support the potential of one-time in-office sura-vec to modify the underlying disease and decrease risk of vision-threatening events. Dose Level 3 is being evaluated in the Phase IIb/III NAAVIGATE trial of sura-vec in NPDR.

About Surabgene Lomparvovec (sura-vec, ABBV-RGX-314)
Sura-vec is a one-time investigational gene therapy designed to deliver sustained treatment effect in wet AMD, diabetic retinopathy and other chronic retinal conditions. Sura-vec uses the NAV® AAV8 vector to encode an antibody fragment designed to inhibit vascular endothelial growth factor (VEGF). The sura-vec transgene protein inhibits the VEGF pathway by which new, leaky blood vessels grow and contribute to the accumulation of fluid in the retina.[2]

About Diabetic Retinopathy
Diabetic retinopathy (DR) is the leading cause of vision loss in adults between 24 and 75 years of age worldwide.[3] DR affects nearly 10 million people in the United States alone.[4] The spectrum of DR severity ranges from non-proliferative diabetic retinopathy (NPDR) to proliferative diabetic retinopathy (PDR).[4] As DR progresses, a large proportion of patients develop vision-threatening events, including diabetic macular edema (DME) and neovascularization that can lead to blindness.[5] Current treatment options for patients with NPDR typically include "watchful waiting" or anti-VEGF treatment. For patients with PDR, current treatment options include anti-VEGF treatment or retinal laser; surgical treatment may be required for advanced PDR.[2]

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® and ITVISMA®. 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, the timing, availability and interpretation of clinical data. 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 risks related to the FDA's review process, the timing of enrollment, commencement, completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of any milestone payments, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, 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, 2025, 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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[1] One participant who achieved a ≥2-step DRSS improvement at Year 2 received two supplemental anti-VEGF injections after Year 2 despite no evidence of disease progression or center-involved DME in the study eye; investigator elected to treat the study eye concurrently with treatment of the fellow eye for center-involved DME. Participant was lost to follow-up and had no Year 3 data.
*These are interim results from analyses performed by REGENXBIO for an ongoing trial.
[2] Penn JS, Madan A, Caldwell RB, et al. Vascular endothelial growth factor in eye disease. *Prog Retin Eye Res.* 2008;27(4):331-71.
[3] Cheung N, Mitchell P, Wong TY. Diabetic retinopathy. Lancet. 2010;376(9735):124–36.
[4] Lundeen EA, Burke-Conte Z, Rein DB, Wittenborn JS, Saaddine J, Lee AY, Flaxman AD. Prevalence of Diabetic Retinopathy in the US in 2021. JAMA Ophthalmology. 2023;141(8):747-754.
[5] Berrocal MD, Alexandra Acabá. Current Management of Diabetic Retinopathy, 2018