REGENXBIO Announces First Patient Dosed in Phase IIb/III NAAVIGATE Trial of Surabgene Lomparvovec in Diabetic Retinopathy; Company to Receive $100 Million Milestone

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- *New long-term data from diabetic retinopathy and wet AMD programs to be presented at American Society of Retina Specialists (ASRS) 2026*

ROCKVILLE, Md., June 29, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the first patient has been dosed in the Phase IIb/III NAAVIGATE clinical trial of investigational surabgene lomparvovec (sura-vec, ABBV-RGX-314) in diabetic retinopathy (DR) using suprachoroidal delivery. REGENXBIO will receive $100 million from AbbVie for this milestone.

"Dosing the first patient in the Phase IIb/III study is a significant milestone in our commitment with AbbVie to bring a one-time gene therapy to patients who continue to face progressive and vision-threatening complications across multiple chronic retinal diseases," said Steve Pakola, M.D., Chief Medical Officer, REGENXBIO. "We are excited to advance sura-vec for DR, following the two-year data that reinforced the durable and disease-modifying impact that sura-vec has the potential to deliver with a single administration. The opportunity to prevent disease progression before vision damage occurs is critical for this population, and we are collaborating closely with the investigators and AbbVie to progress this innovative treatment option."

"Diabetic retinopathy is a leading cause of vision loss among working adults, and the goal as a physician is always to prevent the disease from progressing and treat patients before they lose their vision. Current treatment options often require ongoing, repeated interventions, which can lead to undertreatment and progressive vision-threatening complications," said Arshad Khanani, M.D., M.A., FASRS, Director of Clinical Research at Sierra Eye Associates, Reno, NV. "A one-time, in-office treatment has the potential to transform DR management by improving long-term outcomes while making earlier intervention both practical and scalable for patients worldwide."

NAAVIGATE is a Phase IIb/III multicenter, randomized, masked, sham-controlled study to evaluate the safety and efficacy of sura-vec in subjects with non-proliferative DR (NPDR) without center-involved diabetic macular edema (CI-DME). Subjects will receive sura-vec at $1.0x10^{12}$ genome copies (GC)/eye, which was evaluated as dose level 3 in the Phase II ALTITUDE® trial of sura-vec, and short-course topical prophylactic steroids. The primary endpoint is ≥ 2-step improvement on the diabetic retinopathy severity scale (DRSS) at one year. The Phase IIb portion, operationalized by REGENXBIO, is expected to enroll approximately 135 participants in the United States.

REGENXBIO plans to present two and a half-year data from the long term follow up study of the ALTITUDE trial at the ASRS 44th Annual Meeting in July 2026. In two-year dose level 3 data from the ALTITUDE trial, sura-vec demonstrated a durable efficacy profile and was well tolerated with no intraocular inflammation observed (n=15), supporting the potential of an in-office gene therapy approach.

Five-year long-term follow-up data from the Phase I/IIa study of subretinal sura-vec for wet AMD will also be presented at ASRS. REGENXBIO expects to announce topline data with AbbVie from the ATMOSPHERE® and ASCENT® pivotal trials of sura-vec using subretinal delivery in Q4 2026.

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). 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.[1]

About Diabetic Retinopathy
Diabetic retinopathy (DR) is the leading cause of vision loss in adults between 24 and 75 years of age worldwide.[2] DR affects nearly 10 million people in the United States alone.[3] 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 complications, including diabetic macular edema (DME) and neovascularization that can lead to blindness.[4] 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; surabgene lomparvovec (ABBV-RGX-314) for the treatment of wet AMD and diabetic retinopathy, in collaboration with AbbVie, and NAVSUNLI™ (clemidsogene lanparvovec-sngl, RGX-121) for the treatment of MPS II and RGX-111 for the treatment of MPS I, both in partnership with Nippon Shinyaku. 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, 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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*These are interim results from analyses performed by REGENXBIO for an ongoing trial.

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