



REGENXBIO Announces Pivotal Program for Surabgene Lomparvovec in Diabetic Retinopathy

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- *A pivotal Phase IIb/III clinical trial using suprachoroidal delivery for treatment of diabetic retinopathy will be initiated*
- *New Phase II ALTITUDE[®] trial data demonstrate a durable safety and efficacy profile observed through two years with a single, in-office injection of surabgene lomparvovec in subjects with non-proliferative diabetic retinopathy*
- *REGENXBIO to receive \$100 million upon first subject dosed in the Phase IIb/III clinical trial and an additional \$100 million upon first subject dosed in a second Phase III clinical trial*

ROCKVILLE, Md., Aug. 7, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced it will initiate a pivotal Phase IIb/III clinical trial for investigational surabgene lomparvovec (sura-vec, ABBV-RGX-314) in diabetic retinopathy (DR) using suprachoroidal delivery and a corresponding amendment to its eyecare collaboration with AbbVie.

This clinical advancement follows new, positive two-year data from the Phase II ALTITUDE[®] trial and long-term follow-up study, which enables the initiation of a global clinical program for DR.

"Advancing our DR program to late-stage development brings sura-vec closer to being a potentially transformative new treatment for the millions of people living with DR," said Steve Pakola, M.D., Chief Medical Officer, REGENXBIO. "We remain committed to advancing this program to maximize its value and impact for patients worldwide."

"DR is a progressive disease, with most patients eventually developing vision threatening events (VTEs) and is the leading cause of blindness among working age adults," said Primal Kaur, M.D., Senior Vice President, Global Development of Immunology, Neuroscience, Eye Care and Specialty, AbbVie. "We are excited to advance this clinical development program with the goal of helping to address these unmet needs and bring an additional option to patients living with DR."

Phase II / Long-Term Follow-Up Clinical Data and Pivotal Program Update*

In the Phase II ALTITUDE trial, sura-vec was well tolerated in subjects with non-proliferative diabetic retinopathy (NPDR) at dose levels 1, 2, and 3. As of June 9, 2025, there were no drug-related serious adverse events. No intraocular inflammation was observed through two years at dose level 3 (1.0×10^{12} GC/eye) (n = 15) with short-course topical prophylactic steroids.

Updated Phase II ALTITUDE results will be presented at a future medical meeting.

A pivotal two-part placebo-controlled Phase IIb/III trial will be initiated. Supported by the Phase II dose level 3 data, the primary endpoint will be $\geq$ 2-step DRSS improvement at 1 year. Site selection is in progress.

Collaboration Update

AbbVie and REGENXBIO executed an amendment to the collaboration and license agreement established between the two companies on September 10, 2021. The amendment includes an updated milestone structure for the DR program, under the terms of which AbbVie will pay REGENXBIO \$100 million upon first subject dosed in the Phase IIb/III trial and an additional \$100 million upon first subject dosed in a second Phase III clinical trial. REGENXBIO will pay for all costs for Phase IIb of the Phase IIb/III trial.

The amendment also reflects AbbVie's continued investment across the broader sura-vec program. AbbVie will independently advance and pay all costs for a new Phase III ACHIEVE trial in wet AMD. This randomized controlled trial will assess the potential reduction in injection burden and preservation of long-term vision of sura-vec compared to standard of care.

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.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; 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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*These are interim results from analyses performed by REGENXBIO for an ongoing trial.

¹ Penn JS, Madan A, Caldwell RB, et al. Vascular endothelial growth factor in eye disease. *Prog Retin Eye Res.* 2008;27(4):331-71.

² Cheung N, Mitchell P, Wong TY. Diabetic retinopathy. *Lancet.* 2010;376(9735):124-36.

³ 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.

⁴ Berrocal MD, Alexandra Acabá. *Current Management of Diabetic Retinopathy*, 2018



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