REGENXBIO Presents Positive Long-Term Data for Surabgene Lomparvovec in Wet AMD and Diabetic Retinopathy at American Society of Retina Specialists Annual Meeting

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- *One-time gene therapy treatment with sura-vec demonstrated long-term efficacy at similar doses to those being evaluated in ongoing late-stage studies:*
  - *Stable to improved vision through 5 years in wet AMD patients who required frequent anti-VEGF injections prior to one-time sura-vec; no drug-related IOI observed in a setting of no prophylactic steroids*
  - *Prevention of vision-threatening events without any additional treatment and ≥2-step DRSS improvement in majority of NPDR participants at 2.5 years; no IOI observed with short-course prophylactic topical steroids*
- *Topline data from wet AMD pivotal studies, ATMOSPHERE® and ASCENT®, expected in Q4 2026*

ROCKVILLE, Md.,, July 18, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced positive data from long-term follow-up studies of investigational surabgene lomparvovec (sura-vec, ABBV-RGX-314) in wet AMD using subretinal delivery and diabetic retinopathy (DR) using suprachoroidal delivery. These new data were presented at the American Society of Retina Specialists (ASRS) 44th Annual Meeting in Montreal, Canada.

"Sura-vec continues to demonstrate a durable safety and efficacy profile across multiple routes of administration, reinforcing its potential to preserve vision long-term and change the treatment paradigm for patients with chronic retinal disease," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "These long-term data support the potential for a single treatment to provide lasting disease control while reducing the burdensome need for chronic anti-VEGF injections, which too often leads to undertreatment and subsequent vision loss. We are especially encouraged by the wet AMD five-year data as sura-vec was evaluated in patients with harder-to-treat disease requiring more frequent injections, unlike other studies that have evaluated treatment-naïve patients. The durable efficacy observed through five years supports the potential of sura-vec to treat a significant unmet need in this community."

Sura-vec for the treatment of wet AMD: 5-year data summary

Participants in Cohorts 3 and 4 of the Phase I/IIa trial received subretinal sura-vec at doses similar to those being studied in the ATMOSPHERE® and ASCENT® pivotal trials and demonstrated stable to improved visual acuity and had meaningful reductions in anti-VEGF treatment burden through five years, apart from a polypoidal participant in Cohort 4 with polypoidal choroidal vasculopathy, refractory to anti-VEGF.

Real-world outcomes data show that decline is expected long-term on today's standard of care intravitreal treatments.[1]

No new safety signals were identified in long-term follow-up, including no intraocular inflammation observed in participants who completed the Phase I/IIa study.

Data from an external control study of sura-vec compared to real-world anti-VEGF intravitreal injections in wet AMD were also presented at ASRS. At one year, vision was preserved for sura-vec participants compared to real-world anti-VEGF external control-matched participants. Participants who received sura-vec also maintained or improved BCVA gains, had less central retinal thickness fluctuation, and received significantly fewer injections.

REGENXBIO expects to announce topline data with AbbVie from the ATMOSPHERE and ASCENT pivotal trials of sura-vec in wet AMD using subretinal delivery in Q4 2026.

ATMOSPHERE and ASCENT are multi-center, randomized, active-controlled trials evaluating sura-vec versus ranibizumab and aflibercept, respectively. The primary endpoint is non-inferiority based on change from baseline in 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.

Sura-vec for the treatment of DR: 2.5-year data summary

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, the same dose being evaluated in the Phase IIb/III NAAVIGATE trial, demonstrated a durable safety and efficacy profile through 2.5 years. 55% of participants achieved ≥2 step improvement on the Diabetic Retinopathy Severity Scale (DRSS) without additional treatment, and 70% of participants experienced no vision-threatening events.

Additionally, the majority of patients who achieved a 1-step DRSS improvement at one year post-treatment went on to achieve a 2-step DRSS improvement by 2.5 years.

As of May 25, 2026, sura-vec was well tolerated with no intraocular inflammation observed through 2.5 years (n=17) with short-course prophylactic topical steroids.

These data are consistent with previously presented two-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.

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.[2]

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 lifelong 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 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; 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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