REGENXBIO Completes Dosing in Confirmatory Study of RGX-202, Marking Completion of Registrational Development Program and Supporting Planned BLA Submission in Q3 2026

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- *Confirmatory study completed ahead of schedule due to strong patient demand and robust investigator interest*
- *On track to initiate BLA in Q3 2026 under the accelerated approval pathway supporting potential approval in 2H 2027*

ROCKVILLE, Md., June 24, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the successful completion of dosing in the confirmatory study of RGX-202, a potential best-in-class gene therapy for Duchenne muscular dystrophy. This milestone positions REGENXBIO to initiate a Biologics License Application (BLA) under the accelerated approval pathway in Q3 2026 for a potential approval by the U.S. Food and Drug Administration (FDA) in the second half of 2027.

The BLA submission will include a substantial safety dataset from the AFFINITY DUCHENNE® study of RGX-202 pivotal and confirmatory studies (n=63) and efficacy data from the pivotal portion (n=30). The BLA is expected to include 12-month functional data for at least half of the total participants in the pivotal study.

"Completing the enrollment of our confirmatory study ahead of schedule is a significant milestone that underscores the urgent unmet need in Duchenne and brings us another step closer to advancing RGX-202 towards approval via the accelerated approval pathway," said Curran Simpson, President and Chief Executive Officer of REGENXBIO. "We are encouraged by the recent FDA trends in rare disease development, including our collaborative discussion with FDA regarding our Hunter Syndrome program, demonstrating that the accelerated approval pathway remains available and supported for rare diseases. The RGX-202 pivotal dataset directly aligns with the established accelerated approval criteria: the magnitude of clinical effect seen in functional improvement from baseline, correlation between the biomarker and functional outcomes, and a differentiated safety profile. For all of these reasons, we believe we are well positioned to deliver RGX-202 as the next approved gene therapy for Duchenne patients."

In the recent topline pivotal dataset, RGX 202 met the primary endpoint of 10% microdystrophin expression in >93% of patients at Week 12. Additionally,

- Functional data from patients who reached 12-month assessments (n=9) showed a large magnitude of effect in multiple timed function tests and the North Star Ambulatory Assessment (NSAA).
- RGX-202 was well tolerated and demonstrated a favorable safety profile.
- RGX-202 demonstrated strong correlation between microdystrophin expression at Week 12 and interim functional improvement at one year, supporting the use of RGX-202 microdystrophin as a surrogate endpoint reasonably likely to predict clinical benefit.

In anticipation of potential FDA approval, the Company is preparing for launch, leveraging its fully end-to-end, commercial-ready in-house manufacturing at the REGENXBIO Manufacturing Innovation Center in Rockville, Md., where production intended for commercial supply was initiated last year.

About RGX-202
RGX-202 is designed to address the underlying cause of Duchenne by enabling targeted expression of a novel microdystrophin that is closest to naturally occurring dystrophin. It is the only microdystrophin that includes the C-Terminal domain, which has been shown to protect and preserve muscle function. The differentiated therapeutic approach behind RGX-202 includes a novel construct, a proactive immune suppression regimen, and a suspension-based manufacturing process that delivers industry-leading product purity levels. These factors enable RGX-202 to be delivered at the highest dose among gene therapy programs, with the goal of providing maximum functional benefit and durability while maintaining a favorable safety profile for patients.

About Duchenne Muscular Dystrophy
Duchenne is a severe, progressive, degenerative muscle disease, affecting 1 in 3,500 to 5,000 boys born each year worldwide. Duchenne is caused by mutations in the Duchenne gene which encodes for dystrophin, a protein involved in muscle cell structure and signaling pathways. Without dystrophin, muscles throughout the body degenerate and become weak, eventually leading to loss of movement and independence, required support for breathing, cardiomyopathy and premature death.

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 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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