REGENXBIO REPORTS POSITIVE BIOMARKER DATA FROM AFFINITY DUCHENNE® TRIAL OF RGX-202 GENE THERAPY

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- *Positive biomarker data in patient aged 1-3 add to consistent, robust microdystrophin and transduction levels across all treated ages*
  - *Patient aged 3 years at dosing had expression level at 122.3% compared to control*
- *With a differentiated novel construct and proactive short course immune modulation regimen, RGX-202 continues to demonstrate encouraging safety profile with no SAEs or AESIs*
- *Phase III portion of AFFINITY DUCHENNE® trial enrolling ambulatory patients aged 1 and above, on track for BLA submission mid-2026*

ROCKVILLE, Md., March 19, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported new, positive interim data from two additional patients in the Phase I/II portion of the AFFINITY DUCHENNE® trial of RGX-202, a differentiated investigational gene therapy for Duchenne muscular dystrophy (Duchenne). Results were presented at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference.

"RGX-202 is the only next generation gene therapy for Duchenne in a pivotal phase trial. The new data from the age 1-3 cohort builds on the favorable safety and efficacy profile seen in ages 4 and older and reinforces the potential of RGX-202 to serve a wide age range of patients," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "The consistent, robust microdystrophin levels seen across the age range as well as the functional improvements previously reported support RGX-202's potential to alter the course of this devastating disease. We look forward to sharing additional Phase I/II functional data in the first half of 2025. We also continue to rapidly advance the pivotal trial towards completing enrollment this year and BLA submission mid-2026."

"Patients with Duchenne continue to be in need of treatment options that could meaningfully impact the course of disease," said Carolina Tesi-Rocha, M.D., Stanford Children's Hospital. "The microdystrophin expression and biomarker data presented represent key indicators of potential therapeutic effect. Combined with the safety and functional data to date, I am highly encouraged by the profile of RGX-202."

AFFINITY DUCHENNE Phase I/II Interim Data Updates

Biomarker Data
New biomarker data from two patients who received the pivotal dose of RGX-202 were presented at MDA and continue to support consistent, robust expression and transduction of RGX-202 microdystrophin across all ages.

In a patient aged 3 at dosing, microdystrophin expression was measured to be 122.3% compared to control. Patients under 4 years old have no access to gene therapy, and REGENXBIO is the only gene therapy sponsor recruiting patients in this age group in the U.S.

In a patient aged 7 years old, RGX-202 microdystrophin expression was measured to be 31.5% compared to control.

In all patients, RGX-202 was appropriately localized to the sarcolemma, demonstrating the differentiated construct with the CT-Domain is appropriately targeting the muscle. RGX-202 microdystrophin expression results in ambulatory patients aged 8+ are the highest reported microdystrophin levels across approved or investigational gene therapies. To support a Biologics License Application (BLA) using the accelerated approval pathway, the primary endpoint in the pivotal phase of AFFINITY DUCHENNE is the proportion of participants whose RGX-202 microdystrophin expression is ≥10% at Week 12.

RGX-202 also continues to demonstrate the highest reported vector genome copies (4.9-55.4) measured by qPCR across approved or investigational gene therapies.

Safety and Tolerability Data
As of February 21, 2025, RGX-202 was well tolerated with no serious adverse events (SAEs) and no AEs of special interest (AESIs). Common drug-related AEs included nausea, vomiting and fatigue. All resolved and are typically anticipated with gene therapy administration. A thorough, proactive, short-course immune modulation regimen in combination with industry-leading product purity levels of more than 80% full capsids may contribute to the favorable safety profile seen in patients receiving RGX-202 to date.

RGX-202 Treatment Emergent Adverse Events	**Dose Level 1 Dose Evaluation ($1x10^{14}$ GC/kg)**	**Dose Level 2 Younger Boys ($2x10^{14}$ GC/kg)**	**Dose Level 2 Dose Evaluation / Expansion ($2x10^{14}$ GC/kg)**	**Total n=11**
Age Range (number dosed)	**4-11** (n=3)	**1-3** (n=1)	**4-11** (n=7)	**All Ages**
SAE	0	0	0	0
Central or peripheral neurotoxicity	0	0	0	0

AESI	Drug-induced liver injury	0	0	0	0
	Thrombocytopenia	0	0	0	0
Myocarditis		0	0	0	0
Myositis		0	0	0	0

As reported in November 2024, the first five participants in the Phase I/II portion of the AFFINITY DUCHENNE trial all showed functional improvements that exceeded external natural history controls, demonstrating evidence of RGX-202 positively impacting disease trajectory. (press release). Patients demonstrated stable or improved function on the North Star Ambulatory Assessment (NSAA) and timed function tests. REGENXBIO plans to share additional interim functional data in the first half of 2025.

About RGX-202
RGX-202 is a potential best-in-class investigational gene therapy designed for improved function and outcomes in Duchenne. RGX-202 is the only gene therapy approved or in late-stage development for Duchenne with a differentiated microdystrophin construct that encodes key regions of naturally occurring dystrophin, including the C-Terminal (CT) domain. In preclinical studies, the CT domain has been shown to protect the muscle from contraction-induced stress and improve its ability to repair itself.

Additional design features may potentially improve gene expression, increase protein translation efficiency and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of microdystrophin throughout skeletal and heart muscle using the NAV® AAV8 vector and a well-characterized muscle-specific promoter (Spc5-12). RGX-202 is manufactured using REGENXBIO's proprietary, high-yielding NAVXpress™ suspension-based platform process.

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; 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, clinical trials, costs and cash flow. 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 timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timing or likelihood of payments from AbbVie or Nippon Shinyaku, the monetization of any priority review voucher, 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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 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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