REGENXBIO Announces Positive Topline Results from Pivotal Phase III AFFINITY DUCHENNE® Study of RGX-202

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- *Achieved primary endpoint with high statistical significance; 93% of patients achieved microdystrophin expression above 10%* (p<0.0001)
- *Statistically significant correlation between RGX-202 microdystrophin expression and functional improvement (NSAA n=9), supporting validity of surrogate endpoint*
- *Well-tolerated, differentiated safety profile*
- *Company preparing for potential accelerated approval in 2027*
- *Webcast to be held at 8:00 a.m. ET today*

ROCKVILLE, Md., May 14, 2026 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) announced positive topline and interim functional data from the pivotal Phase III portion of the Phase I/II/III AFFINITY DUCHENNE® trial of RGX-202, a potential best-in-class gene therapy for Duchenne Muscular Dystrophy. The trial met its primary endpoint with high statistical significance (p<0.0001), with 93% of participants reaching at least 10% microdystrophin expression at Week 12 (n=30). Additionally, RGX-202 demonstrated statistically significant correlation between microdystrophin expression and interim functional improvement.

"These topline results are exciting for the Duchenne community," said Pat Furlong, Founding President of Parent Project for Muscular Dystrophy. "For decades, our community has pushed for therapies that can change the trajectory of this disease, and today's news gives us renewed optimism. Our families cannot wait; regulatory flexibility for innovative medicines to treat rare disease remains an urgent priority. We applaud the dedication of the patients and families who participated in this research and look forward to continued progress toward delivering stronger futures for people with Duchenne."

"Duchenne muscular dystrophy is a rare, progressive neuromuscular disease characterized by worsening muscle weakness and loss of function, and there continues to be a critical unmet need for therapies that can reliably alter the course of the disease", said AFFINITY DUCHENNE principal investigator Aravindhan Veerapandiyan, M.D., Arkansas Children's Hospital. "It's encouraging to see robust microdystrophin expression, correlation with functional outcomes, and a manageable safety profile. These data give us hope and reinforce the potential of RGX-202 to positively impact disease progression in individuals with Duchenne."

"RGX-202 is the first gene therapy in development for Duchenne to demonstrate strong, statistically significant correlation between microdystrophin expression and functional improvement, a landmark distinction in the field," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "Today's topline results underscore how our novel construct and differentiated therapeutic approach support a favorable safety profile and potential clinical benefit, including in older patients where progressive decline is expected. These data support the potential of RGX-202 to become a best-in-class gene therapy for Duchenne patients."

AFFINITY DUCHENNE Topline Pivotal Interim Data
As of April 16, 2026

The pivotal portion of the Phase I/II/III AFFINITY DUCHENNE trial evaluated RGX-202 at $2x10^{14}$ GC/kg in 31 ambulatory boys aged 1 year of age and older. Key topline interim results include safety (n=31), biomarker (n=30), and functional data (n=9 aged ≥4 years, 12 months post-treatment).[1]

More than 20 additional participants have been enrolled in the confirmatory trial of RGX-202 (n=30), and the Company expects to have completed dosing in all 60 patients across the pivotal and confirmatory trials by mid-year.

Primary Endpoint and Biomarker Data
93% of participants achieved >10% RGX-202 microdystrophin expression at Week 12 (p<0.0001). Microdystrophin expression averaged 71.1% across all participants, and 41.6% in older boys, aged ≥8 years. Additionally, 80% of participants achieved >40% microdystrophin expression.

RGX-202 was appropriately localized to the sarcolemma, demonstrating that the differentiated construct with the inclusion of the C-Terminal (CT) domain is appropriately targeting the muscle. Additionally, robust vector copies per nucleus and percent positive fibers observed support the potential for sustained microdystrophin expression.

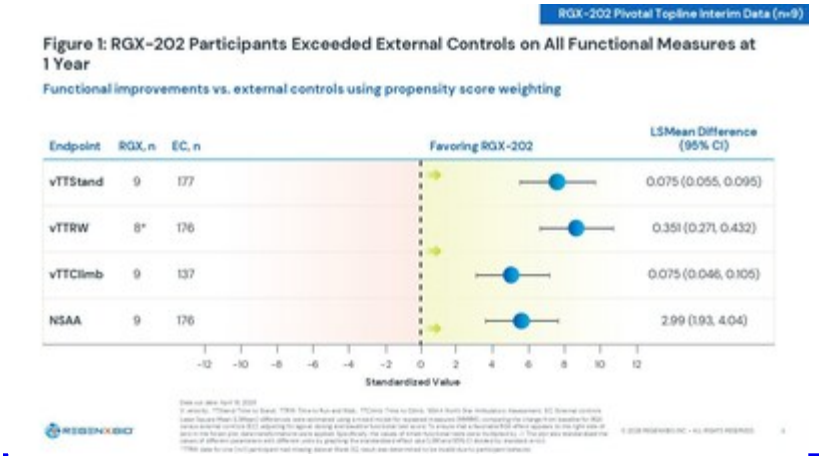
Interim Safety and Tolerability Data
RGX-202 was well tolerated and demonstrated a favorable safety profile as of last data cut. A proactive, short-course immune suppression regimen in combination with a differentiated construct and industry-leading product purity levels of more than 80% full capsids may contribute to a favorable safety profile for RGX-202. Mean gamma-glutamyl transferase (GGT) and total bilirubin, recognized markers of liver inflammation in Duchenne, did not exceed the upper limit of normal up to one year post-treatment (n=9).

Two serious adverse events were reported; both were easily managed and resolved within weeks without sequelae. One case of subacute myocarditis was reported in an 8-year-old participant. The participant's most recent cardiac MRI confirmed no heart muscle fibrosis and no change in ejection fraction. One case of asymptomatic liver injury was reported in a 10-year-old participant. This patient's GGT peak elevation was 123 U/L, and his

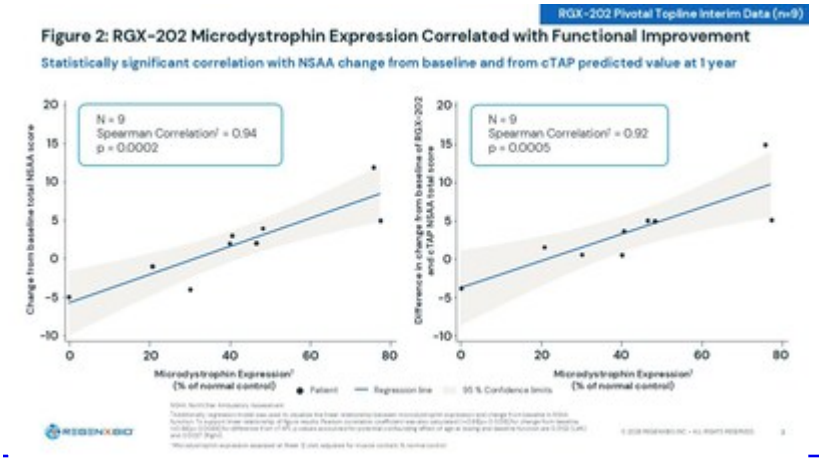
abdominal ultrasound and bilirubin levels were normal. Common drug-related adverse events included vomiting, fatigue, and nausea, all considered mild or moderate, and resolved without sequelae.

Interim One Year Functional Data
In interim functional results from nine participants aged approximately 5 to 12 years at dosing, RGX-202 demonstrated functional improvement and evidence of positively impacting disease trajectory at one year post-treatment, as measured by North Star Ambulatory Assessment (NSAA) and timed function tests (Time to Stand, 10 Meter Walk-run, Time to Climb).

Participants demonstrated statistically significant improved performance across NSAA and all timed function tests when compared to external control using propensity score weighting, which is the primary analysis method specified in the SAP for the pivotal trial. [Figure 1]



RGX-202 microdystrophin expression at Week 12 demonstrated a statistically significant correlation with functional improvement at one year as measured by NSAA change from baseline (correlation= .094, p = 0.0002) and NSAA change from baseline compared to the cTAP predictive model (correlation= .092, p = 0.0005). [Figure 2]



Primary Endpoint and Interim Data Support Potential Accelerated Approval
In recent discussions with FDA, the agency shared that the use of RGX-202 microdystrophin expression as a surrogate endpoint will be based on the correlation analysis with clinical outcomes, which has been clearly demonstrated in the interim data. While the FDA has recommended a randomized controlled trial, it has guided that externally controlled trials may be adequate for demonstrating substantial evidence of effectiveness, especially when the treatment effect is sufficiently large enough to overcome limitations of externally controlled trials. FDA offered to review the RGX-202 data and alternative proposals. REGENXBIO plans to discuss this data with the FDA at a future meeting. The Company is also finalizing the trial design for an ex-U.S. study to support global regulatory submissions.

Given the positive topline pivotal data, continued favorable safety profile, and statistically significant correlation between microdystrophin and functional improvement, REGENXBIO plans to pursue accelerated approval for RGX-202 and is preparing for a potential commercial launch in 2027.

Webcast Details
REGENXBIO will host a webcast featuring REGENXBIO management and leading Duchenne physicians Dr. Veerapandiyan, Carolina Tesi-Rocha, M.D., Clinical Professor, Neurology, Stanford School of Medicine, Stanford Children's Health, and Diana Castro, M.D., Founder and Director of the Neurology & Neuromuscular Care Center and Neurology Rare Disease Center, to discuss today's developments at 8:00 a.m. ET.

The live webcast can be accessed **HERE** and in the Investors section of REGENXBIO's website at **WWW.REGENXBIO.COM**. An archived replay of the webcast will be available for approximately 30 days following the presentation.

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. RGX-202 is designed for improved muscle function, durability and safety outcomes 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; 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, the timing, availability and interpretation of clinical data, including interim, preliminary or updated data readouts. 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 availability, timing, completeness and interpretation of clinical and preclinical data; the possibility that interim, preliminary or early data may not be indicative of final results; that additional data, longer follow-up or subsequent analyses may materially change previously reported results; and that regulatory authorities may interpret data differently than the REGENXBIO, 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, 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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[1] 30 of 31 total participants have Week 12 biopsy available for evaluation; one participant refused muscle biopsy.

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