REGENXBIO REPORTS NEW POSITIVE FUNCTIONAL DATA FROM PHASE I/II AFFINITY DUCHENNE® TRIAL OF RGX-202

June 05, 2025 07:05 AM EDT

- *RGX-202 demonstrating consistent evidence of positively changing disease trajectory for Duchenne*
  - *All dose level 2 participants exceeded external natural history controls on all functional measures*
- *Biomarker data demonstrate consistent, robust microdystrophin expression and transduction levels across all treated ages*
  - *One new participant aged 2 years at dosing had expression level at 118.6% compared to control*
- *Favorable safety profile continues with no serious adverse events or adverse events of special interest observed*
- *Webcast to be held at 8:00 a.m. today*

ROCKVILLE, Md., June 5, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced new positive interim data from the Phase I/II AFFINITY DUCHENNE trial. Updates include positive functional, safety and biomarker data for RGX-202, REGENXBIO's potential best-in-class, investigational gene therapy for Duchenne muscular dystrophy. The functional data demonstrate consistent benefit among dose level 2 participants at 9 and 12 months following treatment with RGX-202.

"Today's findings support the potential of RGX-202 to positively change the disease course for Duchenne and meaningfully benefit patients living with this degenerative disease. At the same dose being used in the pivotal trial, RGX-202 participants exceeded natural history across all key measures, including the North Star Ambulatory Assessment, which is striking," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "We are particularly encouraged by the outperformance observed in older patients. The continued, positive data further strengthen our commitment to rapidly bring this potentially transformative therapy to market and support our planned Biologics License Application submission under accelerated approval in mid-2026."

"These findings suggest that the microdystrophin expression observed with RGX-202 is leading to meaningful functional improvements, even in individuals with DMD who are expected to experience functional decline," said Aravindhan Veerapandiyan, M.D., of Arkansas Children's Hospital. "These Phase I/II results, demonstrating functional improvements and favorable safety profile, underscore the potential of RGX-202 as a treatment option for individuals with DMD. It is both encouraging and essential to have innovative therapies that can help preserve muscle integrity and substantially delay disease progression. I'm enthusiastic about the continued development of RGX-202 and the promise it holds for the Duchenne community."

AFFINITY DUCHENNE Phase I/II Interim Data Updates (data cut: May 7, 2025)

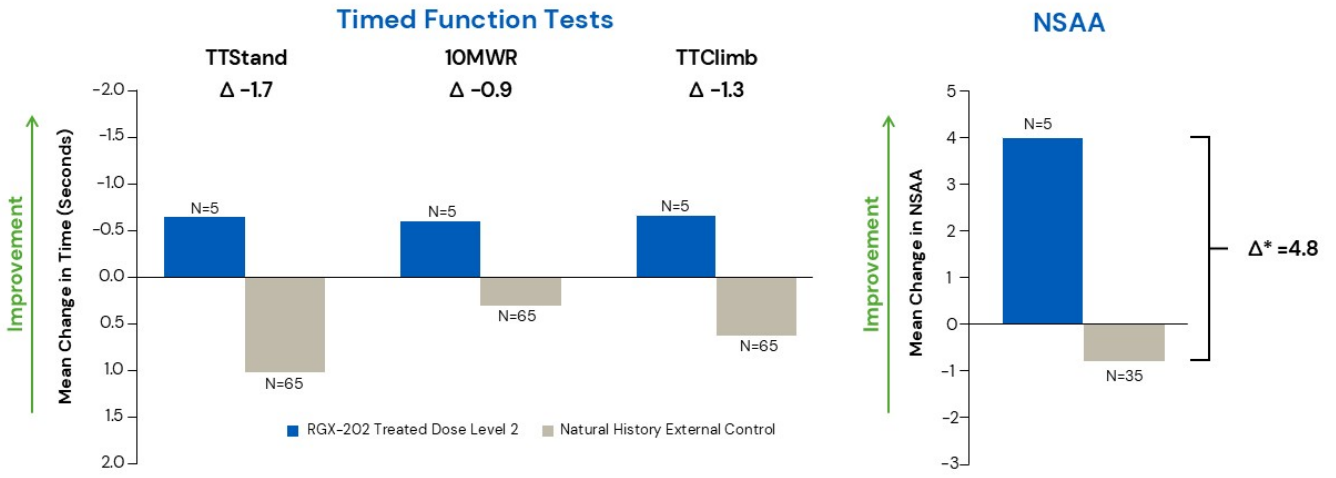
Functional Data

Today, REGENXBIO announced positive interim functional results from the first five participants, aged approximately 6 to 12 years at dosing, receiving RGX-202 at dose level 2 ($2x10^{14}$ GC/kg). Based on these patients' age at dosing and baseline function, four out of five patients are expected to be in the decline phase of their disease trajectory. Results were measured against external natural history controls that were strictly matched for age and baseline function[1].

RGX-202 continues to demonstrate evidence of positively impacting disease trajectory with dose level 2 participants demonstrating improved performance on North Star Ambulatory Assessment (NSAA) and timed function tests (Time to Stand, 10 Meter Walk-run, Time to Climb), exceeding external natural history controls.

At 9 months, RGX-202 participants demonstrated improvement in function and exceeded external controls on all measures. On NSAA, RGX-202 recipients improved an average of 4 points from baseline and 4.8 points compared to natural history. [Figure 1]

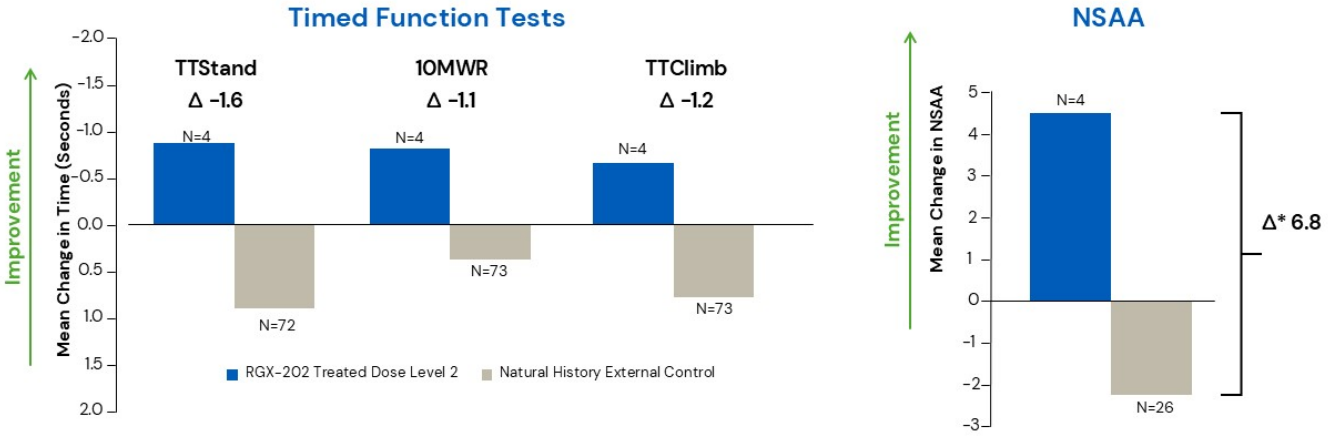
Figure 1: RGX-202 Dose Level 2 9-Month Functional Data



Data cut date May 7, 2025
Time to Stand (TTStand); 10M Walk Run (10MWR); Time to Climb (TTClimb)
Mean of changes from baseline for EC was stratum-based, i.e., the values of individual matched EC subjects to a RGX-202 subject were averaged first before calculating the mean.
* For NSAA, the EC matched subjects of one treated subject did not have data at Month 9. The delta was based on the mean of RGX-202 participants' changes from baseline minus stratum-based mean change from baseline of EC matched participants.

Four out of the five participants reached 12-months post dosing. Results at 12 months are similar to those seen at 9 months. RGX-202 participants demonstrated improved performance on timed function tests and NSAA, exceeding external natural history controls at 12 months. All participants within this cohort demonstrated improvement on all timed function tests compared to baseline. On NSAA, RGX-202 recipients improved an average of 4.5 points from baseline and 6.8 points compared to natural history. [Figure 2]

Figure 2: RGX-202 Dose Level 2 12-Month Functional Data



Data cut date May 7, 2025
Time to Stand (TTStand); 10M Walk Run (10MWR); Time to Climb (TTClimb)
Mean of changes from baseline for EC was stratum-based, i.e., the values of individual matched EC subjects to a RGX-202 subject were averaged first before calculating the mean. [2]
*For NSAA, the EC matched subjects of one treated subject did not have data at Month 12. The delta was based on the mean of RGX-202 participants' changes from baseline minus stratum-based mean change from baseline of EC matched participants.

Additionally, dose level 2 participants' timed task velocity changes exceeded minimal clinically important difference (MCID) benchmarks at 12 months, a measure referenced by the FDA in the approval of an available gene therapy.

Biomarker Data
Biomarker data from the Phase I/II study continues to support consistent, high expression and transduction of RGX-202 microdystrophin. New data from an additional patient, aged 2 at dosing, had a microdystrophin expression level of 118.6% compared to control. 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 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.

Mean at 12 Weeks (min, max)	**Dose Level 1 $1x10^{14}$ GC/kg**		**Dose Level 2 $2x10^{14}$ GC/kg**		
Age range (number with data)	**4-7** (2)	**8-11** (1)	**1-3** (2)	**4-7** (2)	**8-11** (5)
RGX-202 Microdystrophin % normal control (Western Blot)	**60.6** (37.8, 83.4)	**10.4**	**120.5** (118.6, 122.3)	**54.3** (31.5, 77.2)	**39.7** (20.8, 75.7)
VCN copies/nucleus (qPCR)	**9.8** (7.4,12.1)	**5.4**	**24.8** (20.4, 29.1)	**30.1** (4.9, 55.4)	**17.8** (12.0,30.7)
Positive Fibers % (Immunofluorescence)	**79.3**[2]	**34.6**	**82.1**[2]	**50.3** (29.4, 71.1)	**45.7** (21.3,70.6)

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
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 are typically anticipated with gene therapy administration. A proactive, short-course immune modulation 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.

Phase I/II AFFINITY DUCHENNE Trial: RGX-202 Treatment Emergent Adverse Events		**Dose Level 1 Dose Evaluation ($1x10^{14}$ GC/kg)**	**Dose Level 2 Dose Younger Boys ($2x10^{14}$ GC/kg)**	**Dose Level 2 Evaluation / Expansion ($2x10^{14}$ GC/kg)**	**Total n=13**
Age Range (number dosed)		**4-11** (n=3)	**1-3** (n=3)	**4-11** (n=7)	**All Ages**
SAE		0	0	0	0
AESI	Central or peripheral neurotoxicity	0	0	0	0
	Drug-induced liver injury	0	0	0	0
	Thrombocytopenia	0	0	0	0
Myocarditis		0	0	0	0
Myositis		0	0	0	0

AFFINITY DUCHENNE Pivotal Trial
REGENXBIO is enrolling participants in the pivotal portion of the Phase I/II/III AFFINITY DUCHENNE trial of RGX-202. The trial is expected to enroll approximately 30 patients aged 1+ in the U.S. and Canada by 2025, with more than half already enrolled to support the pivotal dataset.

The pivotal trial is expected to support a Biologics License Application (BLA) submission using the accelerated approval pathway in mid-2026. REGENXBIO expects to share top-line data in the first half of 2026 and plans to include biomarker, functional, and safety data in its submission.

Webcast Details
REGENXBIO will host a webcast featuring REGENXBIO management and Dr. Veerapandiyan to discuss today's developments at 8:00 a.m. EST.

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 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 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.

Contacts:
Dana Cormack
Corporate Communications
dcormack@regenxbio.com

Investors:
George E. MacDougall
Investor Relations
IR@regenxbio.com

1 *For NSAA, the EC matched subjects of one treated subject did not have data at Month 9 or Month 12. The delta was based on the mean of RGX-202 participants' changes from baseline minus stratum-based mean change from baseline of EC matched participants.*

2 *One sample could not be evaluated*

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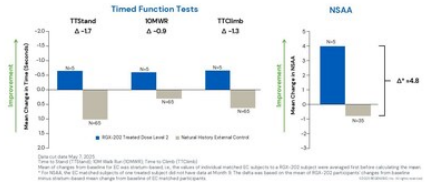

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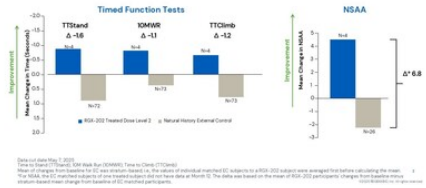

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