



# REGENXBIO®

## REGENXBIO Announces Presentation at the World Muscle Society

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ROCKVILLE, Md., Sept. 29, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced Chief Medical Officer, Steve Pakola, M.D., will present at the International Congress of the World Muscle Society taking place in Vienna, Austria, October 7-11, 2025.

The podium presentation will share new analysis of previously presented 12-month functional data from the Phase I/II trial of RGX-202, including individual patient improvement on the North Star Ambulatory Assessment (NSAA) using the established cTAP disease progression model from the Collaborative Trajectory Analysis Project. As reported, RGX-202 demonstrated a favorable safety profile with no serious adverse events or adverse events of special interest observed in the Phase I/II study. Pivotal dose participants exceeded baseline-matched external natural history controls on all functional measures.

Using multiple models of natural history disease progression, these results further demonstrate the potential of RGX-202 to serve as a differentiated, best-in-class gene therapy for the treatment of Duchenne muscular dystrophy.

**Presentation:** RGX-202, An Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy: Interim Clinical Data

**Presenter:** Steve Pakola, M.D., Chief Medical Officer, REGENXBIO

**Session:** Short Oral Presentations 1: Updates on SMA and DMD Trials

- Accompanying Poster: P425

**Date/Time:** October 8, 3:30 CET

The presentation will be available on the Publications page of REGENXBIO's website, [www.REGENXBIO.com](http://www.REGENXBIO.com).

### 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.

Additional design features such as codon optimization 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 (Sp5-12). RGX-202 is manufactured by REGENXBIO using its proprietary, high-yielding NAVXpress® suspension-based platform process.

### 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; clemidogene 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](http://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 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 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](http://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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