REGENXBIO Announces Completion of Pivotal Enrollment and Initiates Commercial Production in Duchenne Gene Therapy Program

October 30, 2025 07:05 AM EDT

- *Patients treated with RGX-202 demonstrate consistent, robust microdystrophin expression and functional improvement compared to natural history in Phase I/II portion of AFFINITY DUCHENNE® trial supporting potential approval via the accelerated approval pathway*
- *REGENXBIO continues to enroll patients in the confirmatory trial*
- *First batches intended for commercial supply manufactured at in-house Manufacturing Innovation Center*
    - *Capacity to produce up to 2,500 RGX-202 doses per year*
- *Topline pivotal data now expected in early Q2 2026 and BLA submission in mid-2026*

ROCKVILLE, Md., Oct. 30, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the completion of enrollment in the AFFINITY DUCHENNE® pivotal trial of RGX-202, an investigational gene therapy for the treatment of Duchenne muscular dystrophy, as well as the successful production of the first batches intended for commercial supply.

"The Duchenne community urgently needs new treatment options that provide durable, safe outcomes and can meaningfully change the course of this degenerative disease. Completing this pivotal trial milestone and manufacturing in-house our first doses intended for commercial use bring us even closer to delivering RGX-202 as a potential best-in-class gene therapy for Duchenne patients with limited options," said Curran Simpson, President and Chief Executive Officer, REGENXBIO. "The differentiated therapeutic approach behind RGX-202, including our industry-leading product purity levels and novel construct with the C-Terminal domain, has resulted in the positive safety and efficacy profile, with consistent functional benefit seen in Phase I/II. With these highly encouraging results, we are committed to expanding our commercial supply and sharing topline pivotal data in early Q2 of next year."

REGENXBIO continues enrolling ambulatory participants aged 1 year and above in the confirmatory trial.

AFFINITY DUCHENNE® TRIAL
The pivotal portion of the multi-center, open-label Phase I/II/III AFFINITY DUCHENNE trial completed enrollment of 30 participants in October 2025. To support accelerated approval, the primary pivotal endpoint is the proportion of participants whose RGX-202 microdystrophin expression is ≥10% at Week 12. Secondary endpoints include change from baseline on timed function tests in participants aged 4 years and older. Participants aged 1 to < 4 years will be evaluated using the Peabody Developmental Motor Scale-Third Edition (PDMS-3) and SV95C.

In the Phase I/II portion of the trial, microdystrophin levels ranged from 20% to 122% in participants who received the pivotal dose. As of May 7, 2025, RGX-202 was well tolerated, with no serious adverse events (SAEs) or adverse events of special interest (AESIs) reported in the Phase I/II trial. Pivotal dose participants exceeded baseline-matched external natural history controls on all functional measures.

Commercial Readiness
REGENXBIO has manufactured the first batches of RGX-202 intended for commercial supply, supporting the company's expected approval and commercial launch in 2027, when the vast majority of the prevalent market is expected to be available. The company has also manufactured full supply of RGX-202 for the confirmatory trial.

RGX-202 is manufactured at the REGENXBIO Manufacturing Innovation Center at the company's headquarters in Rockville, Md., using its NAVXpress® suspension-based manufacturing process. This proprietary, high-yielding, commercial-ready process has consistently enabled industry-leading product purity levels of more than 80% full capsids, the highest in Duchenne gene therapy. REGENXBIO can produce 2,500 doses of RGX-202 per year.

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 (Spc5-12). 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; 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

View original content to download multimedia:https://www.prnewswire.com/news-releases/regenxbio-announces-completion-of-pivotal-enrollment-and-initiates-commercial-production-in-duchenne-gene-therapy-program-302599520.html

SOURCE REGENXBIO Inc.