# REGENXBIO Announces Dose Escalation in AFFINITY DUCHENNE® Trial

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- *First patient received dose level 2 of RGX-202, a potential one-time AAV Therapeutic for the treatment of Duchenne that includes an optimized transgene for a novel microdystrophin*
- *On track for pivotal dose determination and initiation of pivotal program in 2024*

ROCKVILLE, Md., Nov. 29, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the first patient received RGX-202 at dose level 2 in the Phase I/II AFFINITY DUCHENNE® trial. RGX-202 is an investigational one-time AAV Therapeutic for Duchenne muscular dystrophy (Duchenne), using the NAV® AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

"Progressing to dose level 2 is an important milestone in our updated strategic plans and for accelerating the development of RGX-202," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "There is a large unmet need for new therapies for boys with Duchenne, and the market is capable of supporting multiple gene therapies. We believe RGX-202 has unique, differentiating features that support its potential to be a best-in-class product."

Initial biomarker data in two patients who received RGX-202 at dose level 1 who completed three-month assessment demonstrated robust RGX-202 microdystrophin expression with localization to the muscle cell membrane. In recently completed preclinical efficacy studies, RGX-202 at dose level 2 showed improvement in functional performance, compared to dose level 1, as determined by forelimb muscle strength and treadmill exhaustion in *mdx* mice.

REGENXBIO expects to share initial strength and functional assessment data for both dose levels and anticipates pivotal dose determination and the initiation of a pivotal program in 2024. The Company plans to use RGX-202 microdystrophin expression as a surrogate endpoint to support a Biologics License Application (BLA) filing using the accelerated approval pathway.

**AFFINITY DUCHENNE Trial Design**
The Phase I/II AFFINITY DUCHENNE trial is a multicenter, open-label dose escalation and dose expansion clinical study to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in patients with Duchenne. In the dose evaluation phase of the trial, four ambulatory, pediatric patients (ages 4 to 11 years old) are expected to enroll in two cohorts with doses of $1x10^{14}$ (GC)/kg body weight (n=2) and $2x10^{14}$ GC/kg body weight (n=2). After an independent safety data review for each cohort, a dose expansion phase of the trial may allow for additional patients to be enrolled.

The trial design was informed by the Duchenne community and engagement with key opinion leaders, including a comprehensive, short-term, prophylactic immunosuppression regimen to proactively mitigate potential complement-mediated immunologic responses, and inclusion criteria based on dystrophin gene mutation status, including DMD gene mutations in exons 18 and above. Trial endpoints include safety, immunogenicity assessments, pharmacodynamic and pharmacokinetic measures of RGX-202, including microdystrophin protein levels in muscle, and strength and functional assessments, including the North Star Ambulatory Assessment (NSAA) and timed function tests.

**About RGX-202**
RGX-202 is designed to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin. Presence of the CT domain has been shown in preclinical studies to recruit several key proteins to the muscle cell membrane, leading to improved muscle resistance to contraction-induced muscle damage in dystrophic mice. Additional design features, including codon optimization and reduction of CpG content, may potentially improve gene expression, increase translational efficiency and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of genes throughout skeletal and heart muscle using the NAV AAV8 vector, a vector used in numerous clinical trials, and a well-characterized muscle-specific promoter (Spc5-12).

**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 leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8 and AAV9. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates, including late-stage and commercial programs, in multiple therapeutic areas. REGENXBIO is committed to a "5x'25" strategy to progress five AAV Therapeutics from our internal pipeline and licensed programs into pivotal-stage or commercial products by 2025.

**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 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, 2022, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (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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