REGENXBIO Receives FDA Fast Track Designation for RGX-202, a Novel Gene Therapy Candidate for the Treatment of Duchenne Muscular Dystrophy

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- *RGX-202 is a potential one-time AAV Therapeutic for the treatment of Duchenne and includes an optimized transgene for a novel microdystrophin and REGENXBIO's proprietary NAV® AAV8 vector*
- *Commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center to be used in the clinical trial*
- *AFFINITY DUCHENNE™ Phase I/II trial of RGX-202 is active and recruiting patients; anticipated to report initial trial data in the second half of 2023*

ROCKVILLE, Md., April 11, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for RGX-202, a potential one-time gene therapy for the treatment of Duchenne muscular dystrophy (Duchenne).

Fast Track designation aims to facilitate the development and expedite the review of new therapeutics that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. Therapies granted this designation are given the opportunity for more frequent interactions with the FDA and may qualify for priority review. The FDA has granted RGX-202 Orphan Drug Designation and Rare Pediatric Disease Designation.

"Fast Track designation, along with our capabilities to conduct our clinical trials using commercial-scale cGMP material, will further support the efficient development of RGX-202 from clinic to commercial readiness," said Kenneth T. Mills, President, and Chief Executive Officer of REGENXBIO. "RGX-202 is a key part of our '5x25' strategy, and we look forward to continuing to work closely with the FDA and the Duchenne community as we advance a highly differentiated product candidate developed with the potential to make a meaningful difference for patients. We look forward to reporting initial data from our clinical trial of RGX-202 in the second half of this year."

"We are pleased that the FDA has granted Fast Track designation for RGX-202," said Debra Miller, Founder and CEO of CureDuchenne. "Accelerating the development of medicines for Duchenne, especially potential one-time gene therapies like RGX-202, is critical for this community."

RGX-202 Clinical Program
In January, REGENXBIO announced that the Phase I/II AFFINITY DUCHENNE ™ trial of RGX-202 for the treatment of Duchenne is now active and recruiting patients. AFFINITY DUCHENNE is a multicenter, open-label dose evaluation 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. Six ambulatory, pediatric patients (ages 4 to 11 years old) with Duchenne are expected to enroll in two cohorts with doses of $1x10^{14}$ genome copies (GC)/kg body weight (n=3) and $2x10^{14}$ GC/kg body weight (n=3). After an independent safety data review for each cohort, a dose expansion phase of the trial may allow for up to six additional patients to be enrolled at each dose level (for a total of up to nine patients in each dose cohort).

Additionally, REGENXBIO is recruiting patients in the AFFINITY BEYOND™ trial, an observational screening study to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

REGENXBIO Gene Therapy Manufacturing
REGENXBIO has manufactured additional clinical supply of RGX-202 in its in-house Manufacturing Innovation Center using the NAVXpress™ platform process. Located in REGENXBIO's headquarters in Rockville, MD, the Manufacturing Innovation Center is designed to meet global clinical and commercial regulatory standards, and includes two independent bulk drug substance production suites, a final drug product suite and integrated quality control labs. REGENXBIO is one of only a few gene therapy companies worldwide with a cGMP facility capable of production at scales up to 2,000 liters.

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). RGX-202 has been granted Fast Track, Orphan Drug and Rare Pediatric Disease designations by the FDA.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (Duchenne) is a rare genetic disorder, caused by mutations in the gene responsible for making dystrophin, a protein of central importance for muscle cell structure and function. Duchenne primarily affects males with approximately 1 in 3,500 to 1 in 5,000 males affected worldwide. The absence of functional dystrophin protein in individuals with Duchenne results in cell damage during muscle contraction, leading to cell

death, inflammation, and fibrosis in muscle tissues. Initial symptoms of Duchenne include muscle weakness that is often noticeable at an early age, with diagnosis typically occurring by 5 years of age. Over time, individuals with Duchenne experience progressive muscle weakness and eventually lose the ability to walk. Respiratory and heart muscles are also affected, leading to difficulty breathing and the need for ventilator assistance, along with the development of cardiomyopathy. There is presently no cure for Duchenne.

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 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 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, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, 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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