REGENXBIO Announces Phase I/II Trial of RGX-202, a Novel Gene Therapy Candidate for Duchenne Muscular Dystrophy, is Active and Recruiting Patients

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- *Company has initiated Phase I/II AFFINITY DUCHENNE ™ trial of RGX-202*
- *Company also enrolling newly active observational screening study, AFFINITY BEYOND, evaluating AAV8 antibody prevalence in boys with Duchenne*
- *Commercial-scale cGMP material from the REGENXBIO Manufacturing Innovation Center to be used in the clinical trial*
- *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*

ROCKVILLE, Md., Jan. 23, 2023 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced that the Phase I/II AFFINITY DUCHENNE ™ trial of RGX-202 for the treatment of Duchenne muscular dystrophy (Duchenne) is now active and recruiting patients. RGX-202 is designed to deliver a transgene for a novel microdystrophin protein that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin. RGX-202 uses REGENXBIO's proprietary NAV® AAV8 vector.

AFFINITY DUCHENNE is a multicenter, open-label dose evaluation and dose expansion clinical trial to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in patients with Duchenne.

Additionally, REGENXBIO is recruiting patients in the AFFINITY BEYOND ™ trial, an observational screening study. The primary objective is 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.

"I am pleased that we are now able to initiate the trial for RGX-202 and also begin enrollment activities in our AAV8 antibody screening study," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "The RGX-202 program is a key piece of our '5x'25' strategy to have five AAV Therapeutics either on the market or in late-stage development by 2025. We look forward to continuing to work closely with the Duchenne community as we advance a highly differentiated product candidate developed with the potential to improve muscle strength and motor function in boys with Duchenne."

"Duchenne muscular dystrophy is a devastating disease and there are still unmet therapeutic needs," said Aravindhan Veerapandiyan, M.D., a principal investigator in the study and Director of the Comprehensive Neuromuscular Program, PPMD Certified Duchenne Care Center, and Co-Director of the Muscular Dystrophy Association Care Center at Arkansas Children's Hospital. "Gene therapies, like RGX-202, have the potential to impact the progressive nature of Duchenne."

REGENXBIO has manufactured additional clinical supply of RGX-202 in its in-house Manufacturing Innovation Center using the NAVXpress ™ process platform. Located in REGENXBIO's 132,000 square foot 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.

Additional information can be found on clinicaltrials.gov for AFFINITY DUCHENNE and AFFINITY BEYOND.

AFFINITY DUCHENNE ™ Trial Design

In the dose evaluation phase of the trial, 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).

The trial design also consists of thorough safety measures 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. Initial trial sites are located in the U.S., with additional sites in Canada and Europe expected to follow.

AFFINITY BEYOND ™ Observational Study

AFFINITY BEYOND is an observational screening study. The primary objective is to evaluate the prevalence of anti-adeno-associated serotype 8 (AAV8) antibodies in participants with Duchenne muscular dystrophy. AAV gene therapies are delivered via viral vectors that are not known to cause disease in humans. For AAV gene therapies delivered systemically, it's important to screen patients for antibodies to the vector. This observational study is intended to help inform future clinical research in Duchenne.

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 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, AAV9 and AAVrh10. 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, 2021, 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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