REGENXBIO Announces Orphan Drug Designation Granted to RGX-202, a Novel Gene Therapy Candidate for the Treatment of Duchenne Muscular Dystrophy

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- *Potential one-time gene therapy for the treatment of Duchenne, includes a novel, optimized microdystrophin transgene and REGENXBIO's proprietary NAV® AAV8 vector*
- *Commercial-scale cGMP material to be used in clinical development*
- *Company on track to submit IND by end of 2021*

REGENXBIO Inc. (Nasdaq: RGNX) today announced the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for RGX-202, a potential one-time gene therapy for the treatment of Duchenne muscular dystrophy (Duchenne). RGX-202 is designed to deliver a novel, optimized microdystrophin transgene with a unique C-terminal domain and a muscle specific promoter to support targeted therapy for improved resistance to muscle damage associated with Duchenne. RGX-202 uses REGENXBIO's proprietary NAV® AAV8 vector.

"This important designation is a milestone in the development of RGX-202 and highlights the need for potential new treatment options for patients with Duchenne," said Olivier Danos, Ph.D., Chief Scientific Officer of REGENXBIO. "The novel microdystrophin transgene in RGX-202 includes coding regions that retain essential functional elements of naturally occurring dystrophin to potentially improve muscle strength and resistance in patients with Duchenne. We look forward to advancing this one-time gene therapy into the clinic."

REGENXBIO expects to submit an Investigational New Drug (IND) application to the FDA for RGX-202 by the end of 2021. Commercial-scale cGMP material has already been produced at 1,000 liter capacity using REGENXBIO's suspension cell culture manufacturing process, and the Company's internal cGMP facility is expected to allow for production up to 2,000 liters for the clinical development of RGX-202.

FDA Orphan Drug Designation is granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States. Orphan drug status provides benefits to drug developers, including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of post-approval marketing exclusivity.

About RGX-202

RGX-202 is designed to deliver a novel microdystrophin transgene which retains key elements of the dystrophin protein, including an extended coding region of the C-Terminal (CT) domain found in naturally-occurring dystrophin, as well as other fundamental improvements to the transgene. Presence of the CT domain has been shown 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 targeting promoter (Spc5-12).

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 in multiple therapeutic areas.

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, 2020 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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