REGENXBIO Announces Dosing of First Patient in Cohort 3 of Phase I/II Trial of RGX-121 for the Treatment of MPS II (Hunter Syndrome)

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- *First patient dosed at third dose level in ongoing trial of RGX-121, a one-time gene therapy for MPS II; total of nine patients have been dosed at four leading clinical centers in the U.S. and Brazil*
- *Previously reported positive interim data from Cohorts 1 and 2 demonstrated consistent reductions in CNS biomarkers, continued neurocognitive development, and evidence of systemic effects*

REGENXBIO Inc. (Nasdaq: RGNX) today announced that it dosed the first patient in Cohort 3 of the ongoing Phase I/II trial of RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome, in patients up to five years old. RGX-121 is an investigational one-time gene therapy designed to deliver the gene that encodes the iduronate-2-sulfatase (I2S) enzyme using the AAV9 vector. RGX-121 is administered directly to the central nervous system (CNS).

"We are pleased with our continued progress in the Phase I/II trial of RGX-121 as we increase the dose level to further our understanding of the potential treatment effects, including potential systemic benefit for patients," said Steve Pakola, M.D., Chief Medical Officer of REGENXBIO. "We are encouraged by the positive interim data we've reported previously from the first two cohorts, including signals of I2S enzyme activity in the CNS, continued neurocognitive development, and evidence of I2S enzyme activity in plasma and urine following administration of RGX-121. We look forward to further program updates later this year."

The Phase I/II trial of RGX-121 in patients with MPS II up to five years old is an open-label, dose escalation trial. RGX-121 is administered directly to the cerebrospinal fluid (CSF) and the study is designed to evaluate three dose levels, ranging from $1.3x10^{10}$ GC/g of brain mass to $2.0x10^{11}$ GC/g of brain mass. The primary endpoint of the study is safety and tolerability of RGX-121. Additional endpoints include the effect of RGX-121 on biomarkers of I2S enzyme activity in the CSF, serum and urine; neurocognitive development; and other outcome measures. The trial is being conducted at four leading clinical centers in the United States and Brazil.

About RGX-121

RGX-121 is a product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome. RGX-121 is designed to use the AAV9 vector to deliver the human iduronate-2-sulfatase gene (IDS) which encodes the iduronate-2-sulfatase (I2S) enzyme to the central nervous system (CNS). Delivery of the IDS gene within cells in the CNS could provide a permanent source of secreted I2S beyond the blood-brain barrier, allowing for long-term cross correction of cells throughout the CNS. RGX-121 has received orphan drug product, rare pediatric disease and Fast Track designations from the U.S. Food and Drug Administration.

About Mucopolysaccharidosis Type II (MPS II)

MPS II, or Hunter Syndrome, is a rare, X-linked recessive disease caused by a deficiency in the lysosomal enzyme iduronate-2-sulfatase (I2S) leading to an accumulation of glycosaminoglycans (GAG), including heparan sulfate (HS) in tissues which ultimately results in cell, tissue, and organ dysfunction. In severe forms of the disease, early developmental milestones may be met, but developmental delay is readily apparent by18 to 24 months. Specific treatment to address the neurological manifestations of MPS II and prevent or stabilize cognitive decline remains a significant unmet medical need. Key biomarkers of I2S enzymatic activity in MPS II patients include its substrate HS, which has been shown to correlate with neurocognitive manifestations of the disorder.

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, 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.

Contacts:
Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

Investors:
Brendan Burns, 212-600-1902
brendan@argotpartners.com

Media:
David Rosen, 212-600-1902
david.rosen@argotpartners.com

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