REGENXBIO Announces Closing of Strategic Partnership with Nippon Shinyaku for MPS Diseases

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ROCKVILLE, Md., March 4, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today announced the closing of its previously announced strategic partnership with Nippon Shinyaku.

Under the terms of the agreement, REGENXBIO and Nippon Shinyaku will develop and commercialize RGX-121 (*clemidsogene lanparvovec)* for Mucopolysaccharidosis II (MPS II), also known as Hunter syndrome and RGX-111 for Mucopolysaccharidosis I (MPS I), also known as Hurler syndrome in the United States and Asia.

"RGX-121 and RGX-111 represent potentially transformative new medicines for patient populations in great need of new options," said Curran M. Simpson, President and CEO of REGENXBIO. "This partnership bolsters our ability to make important progress on these programs, and we look forward to serving the MPS community with Nippon Shinyaku."

RGX-121 is on track to be the first gene therapy for MPS II, with potential FDA approval as early as late 2025.

About RGX-121
RGX-121 is a potential one-time AAV therapeutic for the treatment of boys with MPS II. RGX-121 expressed protein is structurally identical to normal I2S. 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, Fast Track and Regenerative Medicine Advanced Therapy designations from the U.S. Food and Drug Administration and advanced therapy medicinal products (ATMP) classification from the European Medicines Agency.

About RGX-111
RGX-111 is designed to use the AAV9 vector to deliver the α-l-iduronidase (IDUA) gene to the central nervous system (CNS). By providing rapid IDUA delivery to the brain, RGX-111 could potentially help prevent the progression of cognitive deficits that otherwise occurs in MPS I patients. Positive interim data from a Phase I/II trial of RGX-111 were reported in February 2023. RGX-111 has received Orphan Drug Product, Rare Pediatric Disease and Fast Track designations from the U.S. Food and Drug Administration.

ABOUT REGENXBIO Inc.
REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for retinal and rare diseases, including ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, RGX-202 for the treatment of Duchenne and RGX-121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.regenxbio.com.

ABOUT NIPPON SHINYAKU
Based on Nippon Shinyaku's business philosophy, "Helping people lead healthier, happier lives," we aim to be an organization trusted by the community through creating unique medicines that will bring hope to patients and families suffering from illness. Please visit our website (www.nippon-shinyaku.co.jp/english/) for products or detailed information.

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 collaboration with Nippon Shinyaku and REGENXBIO's future operations, clinical trials, and regulatory plans. 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 anticipated completion of REGENXBIO's proposed transaction with Nippon Shinyaku, the outcome of REGENXBIO's proposed collaboration with Nippon Shinyaku, whether the milestones contemplated by the proposed transaction will be achieved, the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, 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, 2023, 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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