



REGENXBIO and Nippon Shinyaku Announce Exclusive Partnership to Develop and Commercialize RGX-121 and RGX-111 for MPS Diseases

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- *REGENXBIO to receive \$110 million upfront, potential milestone payments of up to \$700 million and meaningful double-digit royalties on net sales, and lead manufacturing*
- *Nippon Shinyaku to lead commercialization of first potential gene therapies for Mucopolysaccharidosis II (MPS II) and Mucopolysaccharidosis I (MPS I) in U.S. and Asia*
- *REGENXBIO retains rights to RGX-121 Priority Review Voucher (PRV) with potential accelerated approval expected in 2025; rolling BLA submission underway*

ROCKVILLE, Md., Jan. 14, 2025 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) and Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) today announced a strategic partnership for the development and commercialization of RGX-121 for the treatment of Mucopolysaccharidosis II (MPS II), also known as Hunter syndrome, and RGX-111 for Mucopolysaccharidosis I (MPS I), also known as Hurler syndrome.

Under the terms of the agreement, REGENXBIO will receive \$110 million at closing and up to an additional \$700 million if certain milestones are achieved, consisting of \$40 million in potential development and regulatory milestones and \$660 million in potential sales milestones. REGENXBIO will also receive meaningful double-digit royalties on net sales in the U.S. and Asia (collectively, the "Licensed Territory").

"This partnership with Nippon Shinyaku is exciting in that it maximizes our collective strengths and enables access of two potentially transformational medicines to key markets," said Curran M. Simpson, President and Chief Executive Officer, REGENXBIO. "The structure of the agreement allows us to leverage our expertise in gene therapy manufacturing while also capturing milestones and a meaningful share of future product revenues. RGX-121 is poised to be the first gene therapy for MPS II with potential FDA approval as early as late 2025, and RGX-111 has demonstrated very promising results in Phase 1/2 study. With Nippon Shinyaku's expertise in rare disease and strong commercial capabilities, we look forward to working together to get both of these promising candidates across the finish line for patients."

"RGX-121 and RGX-111 represent one-time gene therapies that can potentially change the course of MPS disease, and we are very pleased to be partnering with REGENXBIO, experts in gene therapy development and manufacturing," said Toru Nakai, President and Representative Director of Nippon Shinyaku. "We are confident these therapies can bring tremendous value to those living with MPS II and I."

Per the agreement, Nippon Shinyaku will commercialize both products in the Licensed Territory and future clinical development of RGX-121 and RGX-111 will be led by REGENXBIO. REGENXBIO retains all rights to, and 100 percent of any proceeds related to the sale of, the Priority Review Voucher (PRV) for RGX-121 received upon potential approval.

REGENXBIO will lead the manufacturing of both products for clinical and commercial supply in the Licensed Territory. REGENXBIO reserves the right to develop and commercialize these products in countries outside of the Licensed Territory.

The transaction is expected to close by the end of the first quarter of 2025, subject to the customary conditions, including applicable regulatory approvals.

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 (FDA).

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 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, 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, 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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