NEWS RELEASE

NS Pharma

January 14, 2025

This press release is being provided for a United States audience as a reference. The original news release was issued by Nippon Shinyaku Co., Ltd., the parent company of NS Pharma on January 14, 2025. Please click here to review: Press Releases. The text contains content related to U.S. unapproved drugs and unapproved indications.

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Nippon Shinyaku and REGENXBIO Enter into an Exclusive Partnership to Develop and Commercialize RGX-121 and RGX-111 for Mucopolysaccharidosis Diseases in the U.S. and Asia

KYOTO, Japan, January 14, 2025 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters: Kyoto; President, Toru Nakai) announced that Nippon Shinyaku and REGENXBIO Inc. (REGENXBIO; Headquarters: Rockville, Maryland, USA; CEO: Curran M. Simpson, NASDAQ: RGNX) have entered into an exclusive license agreement for RGX-121 and RGX-111 for the treatment of Mucopolysaccharidosis II and I (MPS II and I), respectively. Under the terms of the licensing agreement, Nippon Shinyaku will receive exclusive commercialization rights in the United States (U.S.) and exclusive development and commercialization rights in Asia including Japan, and REGENXBIO will retain commercial rights in the rest of the world. After approval of the Biologics License Application in the U.S., RGX-121 and RGX-111 will be marketed by NS Pharma, Inc. (New Jersey, USA; President: Yukiteru Sugiyama), a wholly owned subsidiary of Nippon Shinyaku, in the U.S.

Mucopolysaccharidosis (MPS) is a congenital metabolic disorder in which a specific enzyme is defective or inactive due to genetic factors, resulting in the accumulation of specific glycosaminoglycans (“GAGs”), a type of mucopolysaccharide, and is classified into several forms according to the gene responsible for the disease. The accumulation of GAGs causes systemic organ damage, including the central nervous system, in severe cases, and the prognosis is 10 to 15 years of age. Currently, there is no curative treatment for the disease, and the mainstay of treatment is the suppression of progression through enzyme replacement therapy.

RGX-121 and RGX-111 are first-in-class, investigational gene therapies for the treatment of MPS II and MPS I, respectively. For RGX-121, REGENXBIO has received Fast Track Designation, Rare Pediatric Disease Designation, Regenerative Medicine Advanced Therapy Designation, and Orphan Drug Designation from the U.S. Food and Drug Administration (FDA). Submission of a rolling Biologics

Licensing Application (BLA) for RGX-121 is ongoing. For RGX-111, REGENXBIO has received Fast Track Designation, Rare Pediatric Disease Designation, and Orphan Drug Designation from the FDA and has been conducting a Phase I/II trial in the U.S., Brazil, and Israel.

Nippon Shinyaku is focusing on the field of intractable, rare disorders. We expect that RGX-121 and RGX-111 will contribute to the treatment of patients suffering from MPS.

Entry into force of this agreement is subject to completion of review under the Hart-Scott-Rodino (HSR) Antitrust Reform Act in the U.S.

About MPS II

MPS II, also called Hunter syndrome, is a rare, congenital metabolic disorder caused by a deficiency in the iduronate-2-sulfatase, one of the enzymes that degrades glycosaminoglycans (GAGs). The lack of this enzyme causes heparan sulfate and dermatan sulfate to accumulate in all body tissues. When it develops, it causes systemic symptoms such as growth retardation, osteoarticular symptoms, valvular heart disease, and central nervous system disorders. The current treatment for this disorder is palliative care, enzyme replacement therapy (ERT) and bone marrow and stem cell transplantation.

About MPS I

MPS I is a congenital dysmetabolic disease caused by congenital deficiency or reduced activity of alpha-L-isuronidase, one of the enzymes that degrades glycosaminoglycans (GAGs) in steps, resulting in intracellular accumulation of dermatan sulfate and heparan sulfate and damage to multiple organs. MPS I is classified into two types: severe, with intellectual disability, which is also called Hurler's syndrome and mild.

About HSR Antitrust Reform Act of 1976

The HSR Act provides that before certain size of mergers, tender offers or other acquisition transactions (including certain grants of executive compensation) may be completed, both parties must file notification forms with the Antitrust Division of the Department of Justice and the Federal Trade Commission, and a statutory waiting period expires or is terminated. The HSR Act has been expanded for pharmaceutical licensing agreements in 2013.

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 (https://www.nippon-shinyaku.co.jp/english/) for products or detailed information.

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. For more information, please visit www.regenxbio.com.

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