NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

May 14, 2025

RGX-121 (clemidsogene lanparvovec) :
The U.S. FDA has accepted the Biologics License Application by REGENXBIO for Mucopolysaccharidosis II Disease

KYOTO, Japan, May 14, 2025 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan, President: Toru Nakai) announced that REGENXBIO Inc. (REGENXBIO; Headquarters: Rockville, Maryland, USA; CEO: Curran M. Simpson, NASDAQ: RGNX) has received the acceptance letter from the U.S. Food and Drug Administration (FDA) for its Biologics License Application (BLA) filing for RGX-121 (clemidsogene lanparvovec) for the expected indication of Mucopolysaccharidosis II (MPS II). Additionally, the FDA granted the BLA Priority Review with a Prescription Drug User Fee Act ("PDUFA") target action date of November 9th, 2025 (U.S. time).

For more details, please see the press release from REGENXBIO.
https://ir.regenxbio.com/news-releases/news-release-details/regenxbio-announces-fda-acceptance-and-priority-review-bla-rgx

Nippon Shinyaku and REGENXBIO have entered into a strategic partnership for exclusive commercialization rights in the U.S. and exclusive development and commercialization rights in Asia including Japan.
If REGENXBIO obtains the BLA approval in the U.S., NS Pharma, Inc. (New Jersey, USA, President: Yukiteru Sugiyama), a wholly owned subsidiary of Nippon Shinyaku, will market RGX-121 in the U.S.

MPS II, also called Hunter syndrome, is a rare, congenital metabolic disorder caused by genetic alterations in the IDS gene which encodes iduronate 2-sulfatase, an enzyme that degrades glycosaminoglycans (GAGs). The genetic alterations in IDS gene causes the accumulation of GAGs, including heparan sulfate in all body tissues, leading to multi-organ damage. 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 and enzyme replacement therapy.

RGX-121 is a potential first-in-class, investigational gene therapy for the treatment of MPS II. It is expected to inhibit disease progression long-term by introducing the IDS gene. The filing of BLA for RGX-121 for MPS II in the U.S. is based on the results of its Phase I/II/III CAMPSIITE trial®.

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

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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