NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

August 19 2025

RGX-121 (clemidsogene lanparvovec) :
Regarding the extension of the U.S. FDA review period

KYOTO, Japan, August 19, 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) announced that the U.S. Food and Drug Administration (FDA) extended its review timeline of the Biologics License Application (BLA) for RGX-121 (clemidsogene lanparvovec) for the treatment of Mucopolysaccharidosis II (MPS II). The Prescription Drug User Fee Act (PDUFA) goal date has been extended from November 9, 2025 to February 8, 2026.

The extension follows the REGENXBIO's submission of consistent, positive 12-month clinical data for patients in the pivotal study of RGX-121 in response to an FDA information request. For more details, please see the press release from REGENXBIO.
https://ir.regenxbio.com/news-releases/news-release-details/regenxbio-announces-fda-review-extension-bla-rgx-121-treat

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.

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 which encodes iduronate 2-sulfatase. 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 RGX121 will contribute to the treatment of patients suffering from MPS II.

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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