NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

May 15, 2026

Regulatory Update on RGX-121

KYOTO, Japan, May 15, 2026 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan; President: Toru Nakai) announced that the latest regulatory developments regarding RGX-121, a gene therapy product for Mucopolysaccharidosis Type II (MPS II, Hunter syndrome) being developed by REGENXBIO Inc. (Headquarters: Rockville, Maryland, USA; President and Chief Executive Officer: Curran M. Simpson; hereinafter "REGENXBIO").

- The U.S. Food and Drug Administration (FDA) has lifted the clinical hold on RGX-121.
- REGENXBIO recently filed an appeal of the RGX-121 Complete Response Letter (CRL) and is continuing to engage the agency regarding a path forward for the program.

RGX-121 is a gene therapy under development by REGENXBIO for the treatment of Mucopolysaccharidosis Type II (MPS II, Hunter syndrome). In January 2025, Nippon Shinyaku and REGENXBIO entered into a strategic partnership for RGX-121, under which Nippon Shinyaku acquired exclusive commercialization rights in the U.S., as well as exclusive development and commercialization rights in Asia including Japan.
Following regulatory approval in the U.S., NS Pharma, Inc. (Paramus, New Jersey; President: Yukiteru Sugiyama), a U.S. subsidiary of Nippon Shinyaku, plans to commercialize the product.

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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