NEWS RELEASE

NS Pharma

NS Pharma, Inc.
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FDA Accepts Biologics License Application for Mucopolysaccharidosis II Treatment

PARAMUS, NJ: May 14, 2025 – NS Pharma, Inc. (NS Pharma), a subsidiary of Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) announced today that the U.S. Food and Drug Administration has accepted for review the Biologics License Application (BLA) submission by REGENXBIO Inc. (REGENXBIO; Headquarters: Rockville, Maryland, USA; CEO: Curran M. Simpson, NASDAQ: RGNX) for RGX-121 (*clemidsogene lanparvovec)*, a potential first-in-class, investigational gene therapy for the treatment of Mucopolysaccharidosis II (MPS II). The FDA granted REGENXBIO's BLA Priority Review with a Prescription Drug User Fee Act ("PDUFA") target action date of November 9, 2025.

In January 2025, Nippon Shinyaku and REGENXBIO entered into a strategic partnership for the development and commercialization of RGX-121, as well as RGX-111, which is for the treatment of MPS I. Upon potential approval of RGX-121, NS Pharma will be exclusively responsible for commercializing RGX-121 in the U.S.

"This FDA decision represents a significant milestone in bringing a new, potentially life-changing treatment option to patients in the MPS community," said NS Pharma President, Yukiteru Sugiyama, Ph.D. "We are excited about our partnership with REGENXBIO and the value of our combined expertise in generating renewed hope for MPS families."

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

About RGX-121 (clemidsogene lanparvovec)
RGX-121 is a potential one-time AAV therapeutic for the treatment of boys with MPS II, designed to deliver the iduronate-2-sulfatase (IDS) gene to the central nervous system (CNS). Delivery of the IDS gene within cells in the CNS could provide a permanent source of secreted iduronate-2-sulfatase (I2S) protein beyond the blood-brain barrier, allowing for long-term cross correction of cells throughout the CNS. RGX-121 expressed protein is structurally identical to normal I2S.

RGX-121 has received Orphan Drug Product, Rare Pediatric Disease, Fast Track and Regenerative Medicine Advanced Therapy designations from the FDA.

About MPS II
MPS II, or Hunter Syndrome, is a rare, X-linked recessive disease caused by a deficiency in the lysosomal enzyme I2S leading to an accumulation of glycosaminoglycans (GAGs), including heparan sulfate (HS) in tissues which ultimately results in cell, tissue, and organ dysfunction, including in the CNS. In severe forms of the disease, early developmental milestones may be met, but developmental delay is readily apparent by 18 to 24 months. Specific treatment to address the neurological manifestations of MPS II remains a significant unmet medical need. Key biomarkers of I2S enzymatic activity in MPS II patients include its substrate heparan sulfate (HS) D2S6, which has been shown to correlate with neurocognitive manifestations of the disorder.

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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.

About NS Pharma, Inc.
NS Pharma, Inc., is a wholly owned subsidiary of Nippon Shinyaku Co., Ltd. NS Pharma is a registered trademark of the Nippon Shinyaku Co., Ltd. For more information, please visit nspharma.com.

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