May 14, 2025

Dear MPS Community,

We are excited to share an important update regarding clemidsogene lanparvovec (RGX-121), REGENXBIO's investigational gene therapy for the treatment of MPS II, also known as Hunter syndrome.

The U.S. Food and Drug Administration (FDA) has accepted the clemidsogene lanparvovec Biologics License Application (BLA) for priority review. We are seeking approval of clemidsogene lanparvovec using the accelerated approval pathway, and the FDA plans to complete its review of our application by November 9, 2025 (known as the PDUFA date). This news is another milestone in our mission to bring a potential one-time treatment to individuals living with MPS II.

In January, we announced a partnership with Nippon Shinyaku to commercialize our MPS II and MPS I investigational gene therapies. NS Pharma, the U.S. division of Nippon Shinyaku, will be responsible for the commercialization of clemidsogene lanparvovec once it is approved in the U.S.

Thank you to the MPS II patients, families and physicians who have supported this program. We are grateful to be part of the MPS community and to have the opportunity to work toward what could be the first FDA-approved gene therapy for Hunter syndrome.

Definitions

U.S. FDA's Biologics License Application (BLA): Biologics License Application (BLA) is submitted to the FDA for approval to sell and market an investigational treatment in the U.S. based on the results of preclinical studies, clinical studies, and validated manufacturing process.

A **priority review** designation means FDA's goal is to take action on an application within six months of the acceptance date, compared to ten months under standard review.

The FDA instituted its **Accelerated Approval** Program to allow for earlier approval of drugs that treat serious conditions, and fill an unmet medical need based on a surrogate endpoint that is reasonably likely to predict clinical benefit.

A **Prescription Drug User Fee Act (PDUFA)** date is part of the regulatory process that allows the FDA to set goal dates for reviewing new drug applications. While the assignment of the PDUFA date does not indicate whether a therapy will be approved by the FDA, it does represent a step forward in the regulatory review process.

If the FDA **approves** a treatment, it then becomes available for doctors to prescribe to their patients under the FDA-approved label.

Commercialization refers to the process of bringing a drug to market after the FDA has determined it to be safe and effective for patient use.

We will continue to keep the community informed as we move forward with these developments. If you have questions, please reach out to MPSII@regenxbio.com.

Warm regards from the team at REGENXBIO,

Steve Pakola, MD Chief Medical Officer	Vivian Fernandez Executive Director, Patient Advocacy	Jennifer VanHoutan Patient Advocacy

**RGX-121 is an investigational therapy and has not been approved by regulatory authorities. No claims regarding safety and efficacy can be made.*