

January 14, 2025

Dear MPS Community,

We are writing to share an important and exciting update on REGENXBIO’s two investigational gene therapies, RGX-111 and RGX-121, for the treatment of Mucopolysaccharidosis Type I (MPS I) and MPS II, respectively.

REGENXBIO has partnered with Nippon Shinyaku, a company with a strong track record in rare disease and commercial expertise, to commercialize RGX-121 and RGX-111. Maximizing our collective strengths, REGENXBIO will continue to lead clinical development and manufacturing, and Nippon Shinyaku will bring these medicines to market in the US and Asia, following regulatory approvals.

Today, RGX-121 (MPS II) is in the final stages of the rolling Biologics License Application (BLA)\* submission to the U.S. Food and Drug Administration; the BLA is expected to be completed in the next couple of months.

In November 2023, we communicated our decision to pursue a strategic alternative (partner) to advance RGX-111 for MPS I. We are pleased that this partnership with Nippon Shinyaku allows us to resume the clinical development of RGX-111 (MPS I). We will provide updates as information becomes available.

You can learn more about this announcement in our press release [Exclusive Partnership to Develop and Commercialize RGX-121 and RGX-111 for MPS Diseases\_January 14, 2025](https://regenxbio.gcs-web.com/news-releases/news-release-details/regenxbio-and-nippon-shinyaku-announce-exclusive-partnership). To learn more about Nippon Shinyaku, please visit their company’s website at [www.nippon-shinyaku.co.jp/english/](http://www.nippon-shinyaku.co.jp/english/).

Should you wish to contact REGENXBIO, please email the Patient Advocacy team at MPSI@regenxbio.com or MPSII@regenxbio.com.

On behalf of our entire team, thank you again for your ongoing support. With this partnership, we believe the road ahead will be one of continued innovation, progress, and most importantly, hope for MPS I and MPS II.

Warm regards from the team at REGENXBIO,

Steve Pakola, MD Paulo Falabella, MD, PhD

Chief Medical Officer Vice President, Clinical Development and Operations

Vivian Fernandez Jennifer VanHoutan

Executive Director, Patient Advocacy Patient Advocacy

**\* About the 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 processes.