



M6P Therapeutics Supports Rare Disease Day 2021 and Joins Global Movement to Increase Awareness of Rare Diseases

ST. LOUIS, Mo., – Feb. 25, 2021 – [M6P Therapeutics](#), a privately held life sciences company developing next-generation recombinant enzyme and gene therapies for lysosomal storage disorders (LSDs), today announced its support for Rare Disease Day 2021 and commitment to the rare disease community. Rare Disease Day is an annual awareness day dedicated to elevating the public understanding of rare diseases and their impact on patients’ lives.

According to the National Institutes of Health, there are approximately 7,000 rare diseases affecting between 25 and 30 million Americans. In the U.S., a disease is defined as rare if it affects fewer than 200,000 people. Only 5% of the over 7,000 identified rare diseases have an FDA approved treatment.¹

In honor of Rare Disease Day 2021, M6P Therapeutics today hosted an all-company town hall meeting to highlight the importance of research in rare diseases, share patient stories and testimonials, and motivate and inspire the team as the company works to develop therapies to treat patients with LSDs. In recognition of the vital role of patient organizations in educating communities, empowering patients, and advancing research, M6P Therapeutics has given donations to several patient organizations within the LSD community.

“On Rare Disease Day, we celebrate the strength and perseverance of patients, families, and advocates living with rare diseases who seek to improve awareness, understanding, diagnosis, and treatment of these serious conditions,” said Pawel Krysiak, president and chief executive officer of M6P Therapeutics. “The goal of serving patients with lysosomal storage disorders guides our every decision at M6P Therapeutics, and we are committed to working in partnership with patient communities to develop next-generation therapies with the potential to improve lives.”

About Rare Disease Day

Rare Disease Day takes place every year on the last day of February—the rarest date on the calendar—to underscore the nature of rare diseases and what patients face. It was established in Europe in 2008 by EURORDIS, the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the US by the National Organization for Rare Disorders (NORD®), the leading independent, nonprofit organization committed to the identification, treatment, and cure of rare diseases. The core message of NORD’s “Show Your Stripes” campaign is to wear stripes on Rare Disease Day to raise awareness and show support for those living with rare diseases.

For more information about Rare Disease Day in the US or “Show Your Stripes®,” go to rarediseaseday.us. For information about global activities, go to rarediseaseday.org. To search for information about rare diseases, visit NORD’s website, rarediseases.org.

About M6P Therapeutics

M6P Therapeutics is a privately held, venture-backed biotechnology company developing the next-generation targeted recombinant enzyme and gene therapies for lysosomal storage

disorders (LSDs). M6P Therapeutics' proprietary bicistronic-S1S3 platform has the unique ability to enhance phosphorylation of lysosomal enzymes for both recombinant enzyme and gene therapies, leading to improved biodistribution and cellular uptake of recombinant proteins and efficient cross-correction of gene therapy product. This can potentially lead to more efficacious treatments with lower therapy burden, as well as new therapies for currently untreated diseases. M6P Therapeutics' team, proven in rare diseases drug development and commercialization, is dedicated to fulfilling the promise of recombinant enzyme and gene therapies by harnessing the power of protein phosphorylation using its bicistronic-S1S3 platform. M6P Therapeutics' mission is to translate advanced science into best-in-class therapies that address unmet needs within the LSD community. For more information, please visit: www.m6ptherapeutics.com.

Reference

1. National Organization for Rare Disorders. Rare Disease Day: Frequently Asked Questions. Available at: <https://rarediseases.org/wp-content/uploads/2019/01/RDD-FAQ-2019.pdf>
Accessed Feb. 24, 2021.

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