M6P Therapeutics Receives Six Rare Pediatric Disease Designations from the U.S. FDA for Company's Deep Pipeline of Programs for Lysosomal Storage Disorders

– U.S. FDA Also Grants Two Orphan Drug Designations for the Company's Gene Therapy Programs for Gaucher Disease and Mucolipidosis –

ST. LOUIS, Mo., – Jan. 28, 2021 – M6P Therapeutics, a privately held life sciences company developing next-generation recombinant enzyme and gene therapies for lysosomal storage disorders (LSDs), today announced that the U.S. Food and Drug Administration (FDA) granted six rare pediatric disease designations (RPDDs) for various programs within its development pipeline for LSDs, including four recombinant enzyme and two gene therapy programs. In addition, the FDA granted two orphan drug designations (ODDs) for its gene therapy programs for Gaucher disease and mucolipidosis.

M6P Therapeutics is developing an innovative technology that regulates the natural mechanism for trafficking enzymes to lysosomes. The company's bicistronic-S1S3 platform enables improved biodistribution of recombinant enzymes to target tissues and efficient cross-correction for gene therapies. LSDs, which can present in infancy, childhood or adulthood, are a group of approximately 50 rare genetic disorders that are associated with high morbidity and mortality. Currently, there are no cures for any of these disorders.

"LSDs are progressive and represent significant burden for patients, their families, and the healthcare systems," said Pawel Krysiak, president and chief executive officer of M6P Therapeutics. "Our innovative platform enables efficient trafficking of either a recombinant enzyme or gene therapy product to the affected cells and tissues and has the potential to generate best-in-class treatments for LSDs. We are grateful to the FDA for these multiple orphan and rare pediatric disease designations that highlight the need for new and improved therapies to address unmet needs."

Under the RPDD program, the FDA grants a Priority Review Voucher (PRV) to the sponsor who receives a product approval for a "rare pediatric disease," defined as a serious or life-threatening condition of fewer than 200,000 Americans that primarily affects individuals from birth to 18 years. M6P Therapeutics may be eligible to receive a PRV for each RPDD that gains marketing approval. The vouchers may be sold or transferred or redeemed for subsequent marketing applications.

Designation as an orphan drug serves to advance drug development for rare diseases. The FDA grants the designation to drugs or biologics that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions that affect fewer than 200,000 Americans. Orphan designation provides development and commercial incentives including exemption from FDA user fees and eligibility for a seven-year period of market exclusivity upon approval in the U.S.

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 enzyme replacement 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.

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