M6P Therapeutics Presented Data on M041, A Recombinant Enzyme Therapy, for the Treatment of Sanfilippo B Syndrome at MPS 2021

– *Preclinical data provide proof of concept for recombinant human alpha-N-acetylglucosaminidase (rhNAGLU) demonstrating improved mannose 6-phosphorylation* –

– *Leveraging the Company's proprietary S1S3 bicistronic expression platform, M041 is co-expressed human NAGLU and S1S3, resulting in NAGLU^{M6P}* –

– *With increased efficiency to phosphorylate lysosomal enzymes, M041 is a potential novel recombinant enzyme therapy for Sanfilippo B syndrome* –

ST. LOUIS, Mo., – July 28, 2021 – M6P Therapeutics ("M6PT" or "the Company"), a privately held life sciences company developing next-generation recombinant enzyme and gene therapies for lysosomal storage disorders (LSDs), today announced pre-clinical proof-of-concept data on M041 in a poster presentation by Patricia Dickson, M.D., Centennial Professor of Pediatrics and Chief, Division of Genetics and Genomic Medicine, Washington University of St. Louis, titled "*Recombinant human NAGLU with improved mannose 6-phosphorylation for Sanfilippo B syndrome*" at the 16th Annual International Symposium of MPS and Related Diseases ("MPS 2021"). These data illustrate M041's potential as a recombinant enzyme therapy utilizing a recombinant human alpha-N-acetylglucosaminidase (rhNAGLU) with improved mannose 6-phosphorylation.

"There are currently no approved therapies for Sanfilippo B syndrome, also known as MPS IIIB. As the study suggests, M6PT's M041 recombinant enzyme therapy (NAGLU^{M6P}) employs an efficient and well-trafficked lysosomal enzyme as the key to developing a potentially effective therapy to treat this rare pediatric disorder," said Patricia Dickson, M.D., Centennial Professor of Pediatrics and Chief, Division of Genetics and Genomic Medicine, Washington University of St. Louis. "I anticipate that M6PT's platform technology will lead to advancements in therapies for other LSDs."

Sanfilippo B syndrome is characterized by a defect in the *NAGLU* gene providing instructions for producing the enzyme alpha-N-acetylglucosaminidase. This enzyme is located in lysosomes, which are compartments within cells that process and recycle different types of molecules. Alpha-N-acetylglucosaminidase is involved in the stepwise breakdown of large molecules called glycosaminoglycans (GAGs).

"These preclinical data further demonstrate the utility of M6PT's proprietary S1S3 bicistronic expression system as a platform technology with potential to address multiple LSDs. As such, we remain committed to the research and development of best-in-class recombinant enzyme and gene therapies for LSDs," said Pawel Krysiak, president and chief executive officer of M6P Therapeutics.

Presentation highlights from the poster include:

- M6PT's ability to express NAGLU with enhanced phosphorylation levels.
- Evidence that M041, phosphorylated alpha-N-acetylglucosaminidase, has improved trafficking and binding to M6P receptors.
- M041 has markedly improved cellular uptake in patient fibroblasts when compared to wild type alpha-N-acetylglucosaminidase.

The study's conclusion: co-expression of S1S3 phosphotransferase (Ptase) is an effective way to improve the mannose 6-phosphate content of rhNAGLU, making it more suitable for use as a potential recombinant enzyme therapy.

About Sanfilippo B Syndrome
Sanfilippo B syndrome, also known as mucopolysaccharidosis type IIIB (MPS IIIB), is an inherited lysosomal storage disease (LSD) characterized by progressive degeneration of the central nervous system due to accumulation of glucosaminoglycans (GAGs) in multiple tissues and organs. The disease is characterized by severe intellectual disability, developmental regression, and other neurologic signs and symptoms; musculoskeletal complications; hearing loss; respiratory infections; and heart disease. Onset of the disease is usually age <10 years; and can be come on rapidly or slowly progressive. The disease shows progressive universal neurologic decline. MPS IIIB is an ultrarare, autosomal recessive disorder occurring in less than 1 in 50,000. MPS IIIB results from the deficiency of one of four lysosomal enzymes due to mutations in the genes encoding those enzymes; disease severity correlates with specific enzyme deficiency.

About M6P Therapeutics
M6P Therapeutics is a privately held, venture-backed biotechnology company developing the next-generation of targeted recombinant enzyme and gene therapies for lysosomal storage disorders (LSDs). M6P Therapeutics' proprietary S1S3 bicistronic 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 S1S3 bicistronic 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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