M6P Therapeutics Announces Formation of Distinguished, Experienced Scientific Advisory Board

ST. LOUIS, Mo., – Feb. 3, 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 scientific advisory board (SAB) that will support the Company's mission of translating its innovative bicistronic-S1S3 technology platform into best-in-class therapies that address unmet needs within the LSD community. The Company's platform enables improved biodistribution of recombinant enzymes to target tissues and efficient cross-correction for gene therapies.

"As we work to advance our robust pipeline, we seek the input and support of a world-class team of scientific advisors with deep expertise in genetics, rare diseases, and lysosomal storage and metabolic disorders in particular," said Pawel Krysiak, president and chief executive officer of M6P Therapeutics. "The collective insights, knowledge, commitment, and expertise of our scientific advisory board will help us translate this high science into potential medical benefit for the individuals affected by these serious conditions."

By combining the substantial expertise of the SAB with the expertise of the Company's internal R&D team in recombinant enzyme and gene therapies, M6P Therapeutics is well positioned to rapidly advance its deep pipeline of LSD programs. The members of the SAB are:

- **Stuart Kornfeld, MD,** is a co-founder of M6P Therapeutics, the chairman of its SAB, and a professor in the Department of Medicine and the Department of Biochemistry and Molecular Biophysics at Washington University Medical School. Dr. Kornfeld has devoted decades to understanding mannose 6-phosphate (M6P) and its importance in lysosomal protein trafficking, and he is considered the leading expert on this subject matter. He has advised multiple companies working on LSDs.
- **Nancy Dahms, PhD**, is a Professor of Biochemistry at the Medical College of Wisconsin. She received her doctorate degree from Johns Hopkins University School of Medicine where she studied site-specific N-glycosylation. As a postdoctoral fellow at Washington University School of Medicine, she isolated and characterized cDNA clones for the M6P receptors involved in targeting lysosomal enzymes to the lysosome. A glycobiologist and biochemist, Dr. Dahms investigates glycan-binding proteins in the secretory pathway. Her research has defined the molecular basis for the recognition of lysosomal enzymes by the M6P receptors. Dr. Dahms has developed a rat model for Fabry disease to aid in revealing pathogenic mechanisms of this common LSD.
- **Gregory Enns, MD**, is a Professor of Pediatrics and Genetics at the Lucile Salter Packard Children's Hospital Stanford School of Medicine. Dr. Enns' research interests include novel means of diagnosing and treating mitochondrial disorders, with an emphasis on antioxidant therapy, lysosomal disorders, and newborn screening by tandem mass spectrometry. His current pursuits include the analysis of glutathione and antioxidant status in patients who have mitochondrial disorders and the development of new techniques for diagnosing these conditions.
- **Mark S. Sands, PhD**, is a Professor in the Departments of Medicine and Genetics at Washington University School of Medicine in St. Louis. Dr. Sands received his PhD in Molecular Pharmacology

from the State University of New York at Stony Brook. He was a postdoctoral fellow at The Jackson Laboratory (Bar Harbor, ME) and at the University of Pennsylvania School of Veterinary Medicine before joining the faculty at Washington University School of Medicine. The goals of Dr. Sands laboratory are to better understand the underlying pathogenesis and developing effective therapies for inherited childhood diseases, specifically LSDs. A major focus of his group is to determine the safety and efficacy of adeno-associated viral gene transfer vectors for the treatment of both the CNS and systemic manifestations of these diseases. In addition, his group has developed lentiviral-mediated hematopoietic stem cell-directed gene therapy approaches, as well as small molecule drugs, and more recently rational combinations of these approaches. The primary diseases that Dr. Sands studies are mucopolysaccharidosis type VII (MPS VII), Krabbe disease, and Infantile Neuronal Ceroid Lipofuscinosis.

- **Anna Tylki-Szymanska, MD**, is a Professor, Department of Pediatrics and Pediatric Metabolic and Genetic Medicine at the Children's Memorial Health Institute, Department of Metabolic Diseases in Warsaw, Poland. In the field of metabolic diseases, Prof. Tylki-Szymańska's interests are focused mainly on lysosomal diseases. She has specialized for 30 years at the Department of Metabolic Diseases, which is the only referral center in Poland for inborn errors of metabolism. Prof. Tylki-Szymańska's research interests are directed particularly on neurometabolic and neurodevelopmental diseases. She is especially interested in developing diagnostic assays and new approaches for the treatment of lysosomal diseases. She was a principal investigator for the recombinant enzyme replacement clinical trials for Gaucher, Fabry, MPS II and substrate inhibition therapy trials for MPS III, LAL deficiency and Gaucher disease. Prof. Tylki-Szymańska is a winner of the Order of the Smile. Order of the Smile is an international award given by children, to adults distinguished in their love, care, and aid for children.
- **Raymond Wang, MD**, is the Director of the Multidisciplinary Lysosomal Storage Disorder Program at CHOC Children's, Associate Professor, Pediatrics, UC, Irvine School of Medicine. Dr. Wang is also a board-certified clinical geneticist and biochemical genetics specialist. He is investigating the role of inflammation and the innate immune system, triggered by arterial lysosomal storage, in promoting MPS cardiovascular disease, and identifying novel therapeutic methods to address treatment-refractory cardiovascular disease in MPS. He also actively works on other research projects related to Niemann-Pick C Disease, Pompe, Hunter, and Sanfillipo Syndrome.

"M6P Therapeutics' bicistronic-S1S3 technology platform enhances mannose 6-phosphate content on lysosomal enzymes for both recombinant enzyme and gene therapies, which improves enzyme uptake across target tissues," said Stuart Kornfeld, MD, M6P Therapeutics' co-founder and chairman of its SAB. "With promising pre-clinical data across numerous LSD programs, this innovation can potentially translate into new and more efficacious treatments, reduced immunogenicity, and more efficient dosing regimens."

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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