Dr. Stuart Kornfeld, M6P Therapeutics' Co-Founder, to Receive the Roscoe O. Brady Award For Innovation and Accomplishment at WORLD*Symposium*™ 2022

- The Roscoe O. Brady Award is granted to individuals for their substantial body of innovation and achievement in the field of lysosomal storage disorder (LSDs) research and therapy -

ST. LOUIS, Mo., – September 30, 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 that WORLD*Symposium*™ has named its co-founder, Stuart Kornfeld, MD, as the recipient of the 2022 Roscoe O. Brady Innovation and Accomplishment award. Each year, WORLD*Symposium™* recognizes one individual for innovation and accomplishment in the field of lysosomal disease research and therapy. This award has been renamed the Roscoe O. Brady Award for Innovation and Accomplishment to honor one of the early pioneers in lysosomal research, Roscoe O. Brady, MD, a beloved mentor to many currently working in the field.

Stuart Kornfeld, MD, is co-founder of M6P Therapeutics, chairman of its scientific advisory board, and a professor in the Department of Medicine and the Department of Biochemistry and Molecular Biophysics at Washington University Medical School. He is also a member of the National Academy of Sciences. His lab discovered the mechanism whereby lysosomal enzymes acquire mannose 6-phosphate (M6P) in order to enter the lysosome. Dr. Kornfeld is considered the leading expert on M6P, having devoted decades to understanding the implications of M6P on lysosomal targeting and biodistribution of enzymes that are either exogenously made through recombinant or endogenously through gene addition technologies. Most recently, Dr. Kornfeld, along with his post-doctoral fellow Dr. Lin Liu, made a breakthrough discovery of S1S3, a special truncated variant of GlcNAc-1-Phosphotransferase, that improves the phosphorylation of lysosomal enzymes. This important innovation formed the basis of M6P Therapeutics and allowed for creation of S1S3 bicistronic platform technology that has potential to fulfill the original promise of recombinant enzyme therapies and to develop novel gene therapies with ensured cross-correction across tissues and lysosomes. The S1S3 platform can be utilized for potentially improving current LSD treatments and to develop new therapies for many LSDs that presently do not have any therapeutics options.

"I am honored to receive this recognition and follow in the footsteps of pioneering researchers who have worked tirelessly to understand and develop treatments across the lysosomal storage disorders landscape," noted Dr. Kornfeld. "These concerted efforts have brought us to a critical stage in the understanding of LSDs, and I believe our efforts and discoveries will continue to uncover new treatment options to patients with unmet needs."

Pawel Krysiak, President and CEO, commented on the occasion, "Dr. Kornfeld is by far one of the most accomplished physician scientists in the field of lysosomal storage disorders and has made numerous invaluable contributions that have helped advance the understanding of fundamental mechanisms of lysosomal proteins trafficking. His innovations, patents, manuscripts, and organizational accomplishments are second to none, and he truly deserves to be recognized for his ingenuity, hard work, and dedication to the field. Most importantly, his work has made a tremendous positive impact on trainees and the patient and medical community."

Dr. Kornfeld is an author of more than 270 publications and has trained several leaders in the lysosomal field. He and his lab have been at the forefront of many innovations that have led to critical breakthroughs in the understanding and potential treatment of LSDs including:

1. Helping to establish glycobiology as a new scientific field of study
2. Explaining the role of carbohydrates for sorting proteins for their transport to intracellular organelles within cells and secretion out of cells
3. Discovering Phosphotransferase as the key Golgi enzyme that adds M6P to lysosomal protein
4. Showing that the IGF2 receptor and the cation-independent M6P receptor were in fact the same receptor that can bind different ligands and henceforth known as the IGF2/CI-MPR
5. Showing that the IGF2/CI-MPR cycles to cell surface and is functionally active for binding exogenous phosphorylated lysosomal enzymes for cellular uptake while the cation-dependent M6P receptor (CD-MPR) cannot
6. Empirically determining the binding affinities of different N-glycan structures and IGF2 peptide to IGF2/CI-MPR
7. Elucidating the minimal recognition motif on lysosomal proteins that is utilized by
8. Phosphotransferase for binding and subsequent phosphorylation of lysosomal proteins
9. Developing many critical biochemical and analytical assays for characterizing N-glycans and receptor binding
10. Discovering the uncovering enzyme as the key Golgi enzyme that is required to remove GlcNAc to reveal M6P
11. Discovering that uncovering enzyme cycles to cell surface and can remove GlcNAc-covered phosphorylated N-glycans to enable cellular uptake of exogenous enzymes
12. Identifying two separate M6P receptors [cation-dependent M6P receptor (CD-MPR) and cation independent M6P receptor (CI-MPR)] with differential cellular biodistribution and binding properties for lysosomal enzymes
13. Cloning of CD-MPR and CI-MPR
14. Helping to define the required protein elements for packaging glycoproteins for organellular transport and described how sugars are built on the lipid dolichol prior to transfer onto proteins
15. Identifying and characterizing the different enzymes required for processing of N-glycans
16. Describing how Phosphotransferase deficiency leads to I-cell and pseudo-Hurler diseases
17. Discovering and engineering S1S3, a special truncated variant of GlcNAc-1-Phosphotransferase, with breakthrough properties suitable for therapeutic use 1) much smaller size applicable for gene therapy cassettes, 2) monomer without the need of stabilization by gamma subunit, 3) higher specific activity and capable of phosphorylating a broader set of lysosomal proteins than WT Phosphotransferase
18. Being elected to the National Academy of Science (1982) and won the E.B. Wilson Medal, American Society for Cell Biology (2010), along with James Rothman and Randy Schekman

The award will be presented at the WORLD*Symposium™* 2022 opening session on Monday, Feb. 7, 2022 at 7:30 a.m. Pacific Standard Time (PST).

About the WORLD*Symposium™* Roscoe O. Brady Award For Innovation and Accomplishment
Each year, WORLD*Symposium™* recognizes one individual for innovation and accomplishment in the field of lysosomal disease research and therapy. The award is presented annually to recognize substantial contributions to lysosomal disease research and therapy.

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.

Contact us to learn about partnering opportunities with M6P Therapeutics:

M6P Therapeutics:
314-236-9694
info@m6ptherapeutics.com

Media:
Alex Van Rees, SmithSolve
973-442-1555 x111
alex.vanrees@smithsolve.com