



## M6P Therapeutics to Participate in Key Event Highlights at **WORLDSymposium™ 2022** and Present Preclinical Data Across Multiple Lysosomal Diseases

- Co-founder of M6PT, Dr. Stuart Kornfeld, to receive the 2022 Roscoe O. Brady Award for Innovation and Accomplishment -
- Preclinical data across three lysosomal disorders demonstrate the broad potential of the Company's therapies developed under its novel S1S3 co-expression technology platform -

**ST. LOUIS, Mo., – February 2, 2022** – [M6P Therapeutics](#) (“M6PT” or “the Company”), a privately held life sciences company developing next-generation recombinant enzyme and gene therapies for lysosomal disorders, today announced its participation at **WORLDSymposium™ 2022** conference on lysosomal disorders and research. Presentations involving preclinical research on Sanfilippo syndrome type B, Gaucher disease, and Pompe disease, as well as presentations on the underlying proprietary technology, highlight the Company's broad S1S3 co-expression platform that has the potential to improve LSD treatment modalities through development of best-in-class, next-generation enzyme replacement and gene therapies.

Dr. Stuart Kornfeld, M6PT's co-founder and chairman of the Company's scientific advisory board, kicks off the scientific presentations of the conference with the traditional recipient's address following his acceptance of the *2022 Roscoe O. Brady Award for Innovation and Accomplishment*. Both the award and the recipient's address spotlight Dr. Kornfeld's decades of research on the role of mannose-6 phosphate (M6P) in lysosomal targeting and lysosomal enzyme trafficking.

M6PT will also contribute to the conference's event schedule throughout the week. The 2022 e-Poster abstracts will be available beginning on Monday, Feb. 7, 2022, at 3:00pm PT (6:00pm ET).

### **M6PT CONFERENCE PARTICIPATION DETAILS**

#### **Monday, Feb. 7**

1. **7:30am PT** – M6PT co-founder, Dr. Stuart Kornfeld, will be honored with the presentation of the [Roscoe O. Brady Award](#) and then will present/provide a scientific review, “*Dissecting the Mannose 6-Phosphate Pathway – A Key to Understanding Lysosomal Enzyme Trafficking*”
2. **3:00pm PT – 5:00pm PT** – Poster Session Presentations, (**Poster #70**), Dr. Steven Q. Le, “*Co-expression of S1S3 phosphotransferase in production cell line improves mannose 6-phosphorylation and cellular uptake of alpha-N-acetylglucosaminidase (Sanfilippo syndrome type B)*”

#### **Thursday, Feb. 10**

3. **10:30am PT** – Contemporary Forum Talk, Russell Gotschall, “*M011: A novel highly phosphorylated  $\beta$ -glucocerebrosidase enzyme with broader tissue biodistribution for the treatment of Gaucher disease*”
4. **1:00pm PT** – Contemporary Forum Talk, Dr. Andrew Hedman, “*A novel S1S3 phosphotransferase co-expression gene therapy platform for lysosomal disorders*”
5. **3:00pm PT – 5:00pm PT** – Poster Session Presentations, (**Poster #120**), Dr. Andrew Hedman, “*A novel S1S3 phosphotransferase co-expression gene therapy platform for lysosomal disorders*”
6. **3:00pm PT – 5:00pm PT** – Poster Session Presentations, (**Poster #105**), Russell Gotschall, “*M011: A novel highly phosphorylated  $\beta$ -glucocerebrosidase enzyme with broader tissue biodistribution for the treatment of Gaucher disease*”



7. **3:00pm PT – 5:00pm PT** – Poster Session Presentations, (**Presentation #106**), Russell Gotschall, “*M021: A uniquely glycosylated, highly phosphorylated acid-alpha glucosidase enzyme replacement therapy for the treatment of Pompe disease*”

#### **Friday, Feb. 11**

8. **6.45am PT** – Company Sponsored Satellite Symposium, Dr. Hung Do, M6PT’s Chief Scientific Officer, “*Importance of M6P: Scientific Breakthrough Enables Development of More Potent Treatments for Lysosomal Disorders*”

For more information and participant registration details, including access to live streamed events, presentations, and Q&A sessions, please go to [WORLDSymposium™](#). The abstracts will also be made available on the [Publications](#) section of M6PT’s [corporate website](#) following the presentations.

#### **About Sanfilippo Syndrome Type B**

Sanfilippo syndrome type B, also known as mucopolysaccharidosis type III (MPS III), is an inherited lysosomal storage disease (LSD) characterized by progressive degeneration of the central nervous system due to accumulation of glucosaminoglycans (GAG) 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 III is an ultrarare, autosomal recessive disorder occurring in less than 1 in 50,000. MPS III 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 Gaucher Disease**

Gaucher disease is an ultra-rare, inherited metabolic disorder in which deficiency of the enzyme glucocerebrosidase results in the accumulation of harmful quantities of certain fats (lipids), specifically the glycolipid glucocerebroside, throughout the body especially within the bone marrow, spleen, and liver. The symptoms and physical findings associated with Gaucher disease vary greatly from patient to patient. Some individuals develop few or no symptoms (asymptomatic); others may have serious complications.

#### **About Pompe Disease**

Pompe disease is an ultra-rare inherited metabolic disorder with variable rates of disease progression and different ages of onset. First symptoms can occur at any age from birth to late adulthood. Earlier onset compared to later onset is usually associated with faster progression and greater disease severity. At all ages, skeletal muscle weakness characterizes the disease-causing mobility problems and affecting the respiratory system.

#### **About M6P Therapeutics**

M6P Therapeutics (or “M6PT”) is a privately held, venture-backed biotechnology company developing the next-generation of targeted recombinant enzyme and gene therapies for lysosomal storage disorders (LSDs). M6PT’s proprietary S1S3 co-expression technology 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. M6PT’s 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 co-expression platform. M6PT’s 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](http://www.m6ptherapeutics.com).



Contact us to learn about partnering opportunities with M6PT:

**M6P Therapeutics:**

314-236-9694

[info@m6ptherapeutics.com](mailto:info@m6ptherapeutics.com)

**Media:**

**Alex Van Rees, SmithSolve**

973-442-1555 x111

[alex.vanrees@smithsolve.com](mailto:alex.vanrees@smithsolve.com)