



M6P Therapeutics Presents Promising Preclinical Data In Lysosomal Storage Disorders At The 19th Annual WORLDSymposium™ 2023

Results Highlight Potential Utility of Targeting Mannose 6-Phosphate Pathway to Treat Multiple Lysosomal Storage Disorders

ST. LOUIS, Mo., – February 20, 2023 – [M6P Therapeutics](#) (“M6PT” or “the Company”), a privately held life sciences company developing next-generation enzyme replacement and gene therapies for lysosomal storage disorders (LSDs), today announced the presentation of promising preclinical data at the 19th Annual WORLDSymposium™, a research conference dedicated to lysosomal diseases being held in Orlando, FL, and virtually Feb. 22-26, 2023. In five poster presentations, including two Contemporary Forum presentations, M6P Therapeutics’ researchers report preclinical efficacy results for LSDs, including Gaucher disease and Pompe disease.

“We are thrilled with the continued development of our S1S3 phosphotransferase platform for producing lysosomal enzymes with high levels of mannose 6-phosphate to enable much better drug targeting and delivery of therapeutic lysosomal enzymes to all affected cells and tissues,” said Cuong Do, Chairman of M6P Therapeutics. “We believe that this approach has the potential for creating more potent and effective enzyme replacement therapies and therapeutic proteins from gene therapies for the treatment of lysosomal storage disorders.”

The following forum presentations and posters will be presented at the WORLDSymposium:

Poster #160: S1S3 Gene Therapy for the Potential Treatment of Lysosomal Disorders

Title: *Novel dual promoter AAV gene therapy platform ensures production of therapeutic soluble lysosomal enzymes with high M6P content to enable broad cellular uptake and cross-correction in vivo*

Date and Time:

Contemporary Forum Talk: Saturday, February 25, from 6:45 – 9:00 AM ET
followed by:

Poster Session: Saturday, February 25, 3:00 PM - 4:00 PM ET

Session: Contemporary Forum Poster Presentations

Presenter (In-person): Andrew Hedman, M6PT Scientist

Poster #142: M021 for the Potential Treatment of Pompe Disease

Title: *M021: rhGAA with optimal glycosylation profile containing very high levels of bis-phosphorylated N-glycans clears accumulated glycogen and rapidly normalizes muscle strength in treated Pompe mice*

Date and Time:

Contemporary Forum Talk: Saturday, February 25, from 10:30 – 11:30 AM ET
followed by:

Poster Session: Saturday, February 25, 3:00 PM - 4:00 PM ET

Session: Contemporary Forum Poster Presentations

Presenter (In-person): Russell Gotschall, Vice-President, Research & Development, M6PT



Poster #227: Novel Dual-Promoter Expression Vector for Gaucher Gene Therapy

Title: *Novel AAV gene therapy produces β -glucocerebrosidase with high levels of M6P to enable cellular uptake and cross-correction in the CNS as a potential treatment for Type 2/3 Gaucher disease*

Date and Time: Saturday, February 25, 3:00 PM - 4:00 PM ET

Session: Contemporary Forum

Presenter (In-person): Lin Liu, Director, Research & Development, M6PT

Poster #96: Intra-Nasal Delivery of M011 for Gaucher Type 2/3

Title: *Highly phosphorylated β -glucocerebrosidase (M011) that targets central nervous system neurons as a potential treatment for neuronopathic Gaucher disease type 2 and 3*

Date and Time: Saturday, February 25, 3:00 PM - 4:00 PM ET

Session: Contemporary Forum

Presenter (In-person): Michael Digruccio, M6PT Scientist

Poster #99: M011 May Enable Alternative, Less-frequent ERT Dosing Strategies for Gaucher Disease Type 1

Title: *Highly phosphorylated β -glucocerebrosidase (M011) has much broader tissue distribution and superior substrate reduction with potential for alternative dosing strategies for the treatment of Gaucher type 1*

Date and Time: Saturday, February 25, 3:00 PM - 4:00 PM ET

Session: Contemporary Forum

Presenter (In-person): Hung Do, CSO, M6PT

For more information, registration details and to access the 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 the Annual WORLDSymposium™

The WORLDSymposium™ is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians, and all others who are interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. For additional information on the 18th Annual WORLD Symposium™, please visit <https://worldsymposia.org/>.

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 bicistronic-S1S3 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 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.

Contact us to learn about partnering opportunities with M6P Therapeutics:

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