

Critical Path Institute Awards \$456,000 Grant to Advance Next-Generation Therapy for Rare Glycolipid Storage Disorders

TUCSON, Ariz., April 28, 2026 — Critical Path Institute® (C-Path) today announced a \$456,000 grant to James Shayman, M.D., at the University of Michigan, to support development of a brain-penetrant therapy for glycosphingolipid storage disorders. The award is part of C-Path's Bridging Research and Innovation in Drug Development Grants (BRIDGe) program, offered through its Translational Therapeutics Accelerator.

The funded project, titled "*CNS penetrant inhibition of glucosylceramide synthase for the treatment of Gaucher disease and GM1 gangliosidosis*," focuses on developing a therapy to address neurological symptoms that remain untreated by current options. If successful, the therapy could help address a major gap in treatment for those living with neuronopathic lysosomal storage disorders, where disease progression in the brain remains largely untreatable.



James Shayman, M.D.

Dr. Shayman previously invented eliglustat tartrate, the first and only oral therapy approved globally for the treatment of Gaucher disease type 1. The drug reduces the buildup of glucosylceramide, a harmful lipid that accumulates in affected individuals. However, because eliglustat does not cross the blood-brain barrier, it is not effective for neuronopathic glycolipid storage disorders — including Gaucher disease type 3, GM1 gangliosidosis, Tay-Sachs disease, and Sandhoff disease — where central nervous system involvement leads to severe neurological symptoms.

Building on decades of research in glucosylceramide biology, Dr. Shayman collaborated with medicinal chemist Scott Larsen, Ph.D., a former colleague at the University of Michigan, to design CNS-penetrant compounds targeting glucosylceramide synthase, leading to the identification of BPN-25271, a candidate that retains the proven mechanism of eliglustat.

The C-Path grant will support preclinical studies, including safety, dosing, and manufacturing assessments, with the goal of enabling a future Investigational New Drug application and subsequent clinical testing. "This funding allows us to take the next critical steps toward a therapy that could target the brain and address neurological disease in patients with lysosomal storage disorders who currently have very limited options," said Dr. Shayman. "It builds on decades of work in glucosylceramide biology by our group and others and represents a real opportunity to help patients with these devastating disorders."

"Through the BRIDGe program, C-Path supports innovative research that can accelerate promising discoveries toward clinical development," said Maaïke Everts, Ph.D., Executive Director of C-Path's Translational Therapeutics Accelerator. "This project represents exactly the type of translational effort we aim to advance — building on strong scientific foundations to move a potential therapy closer to patients who urgently need new treatment options."

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the FDA's Critical Path Initiative. C-Path's mission is to lead collaborations that advance better treatments for people worldwide. Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path's global headquarters is located in Tucson, Arizona and C-Path's Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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