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FDA Grants Breakthrough Therapy Designation to Glycomine's GLM101 for the Treatment of PMM2-CDG

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Designation is based on clinical evidence from Phase 2a, including improvements in ataxia and other clinical measures after 24 weeks of GLM101 treatment

Topline data from the randomized, placebo-controlled portion of the global Phase 2b POLAR study expected in the fourth quarter of 2026

SAN CARLOS, Calif.--(BUSINESS WIRE)--Glycomine, Inc. announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to GLM101 for the treatment of phosphomannomutase 2 congenital disorder of glycosylation (PMM2-CDG), a serious, multisystem disorder with no approved treatments. GLM101 is an investigational liposomal mannose-1-phosphate substrate replacement therapy designed to address the underlying deficiency in mannose-1-phosphate and resulting disruption of glycosylation in PMM2-CDG. The designation is based on clinical evidence from Glycomine's open-label Phase 2a study, including improvements in ataxia and other clinical measures after 24 weeks of GLM101 treatment.

> *"This designation reflects the compelling clinical evidence generated to date and the urgent need for a treatment for people living with PMM2-CDG," said Steven Axon, Chief Executive Officer of Glycomine.*

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"This designation reflects the compelling clinical evidence generated to date and the urgent need for a treatment for people living with PMM2-CDG," said Steven Axon, Chief Executive Officer of Glycomine. "It also gives us the opportunity for more frequent discussions with FDA as we analyze the POLAR data and determine the next steps for GLM101. We are grateful to the patients, families, caregivers, and investigators who have made this work possible and look forward to sharing the results later this year."

FDA grants Breakthrough Therapy designation to investigational drugs for serious conditions when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint. The designation is intended to support more efficient development and review through closer engagement with FDA.

GLM101 is currently being evaluated in POLAR, a global, randomized, double-blind, placebo-controlled Phase 2b study that enrolled 43 pediatric and adult patients with PMM2-CDG across 15 sites in the United States, United Kingdom, and Europe (ClinicalTrials.gov Identifier: NCT06892288). Topline data from the randomized portion of POLAR are expected in the fourth quarter of 2026. Following the randomized portion, all patients may receive GLM101 through Week 48, providing additional information on longer-term safety, durability of response, and the experience of patients who cross from placebo to GLM101.

About PMM2-CDG

Phosphomannomutase 2-congenital disorder of glycosylation (PMM2-CDG), previously known as CDG-1a, is the most prevalent congenital disease of glycosylation. PMM2-CDG is caused by genetic mutations in phosphomannomutase 2 (PMM2), which results in the protein having reduced activity. PMM2 is an enzyme that converts mannose-6-phosphate to mannose-1-phosphate, which is required to insert the mannose sugar building block into developing glycans that are crucial for proper protein structure and function. The deficiency of mannose-1-phosphate disrupts the process of N-glycosylation and causes a wide array of clinical symptoms and, in many cases, can be life-threatening. PMM2-CDG has an estimated birth incidence of 1 in 30,000 to 1 in 40,000 in Europe and the United States.

About Glycomine, Inc.

Glycomine is a clinical-stage biotechnology company that is advancing treatments for serious rare diseases with no other therapeutic options. The Company's lead investigational drug candidate GLM101 is a mannose-1-phosphate replacement therapy in development to treat PMM2-CDG. GLM101 is designed to deliver mannose-1-phosphate into cells and thereby bypass disease-causing PMM2 mutations to restore pathway function. GLM101 has received Orphan Drug Designation in the U.S. and E.U., as well as Rare Pediatric Disease Designation, Fast Track Designation, and Breakthrough Therapy Designation in the U.S. The company is based in San Carlos, California, and supported by leading international life sciences investors. For more information, visit www.glycomine.com.

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GLYCOMINE, INC.

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