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Glycomine Announces Encouraging Efficacy Data from Ongoing Phase 2 Clinical Study in PMM2-CDG

- *Initial data from adult patients with PMM2-CDG showed promising evidence of clinical benefit with GLM101*
- *Notable improvements were seen in ataxia, one of the most frequent disease-associated clinical symptoms*
- *Data from this study provides key insights into future planned clinical trials*

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SAN CARLOS, Calif.--(BUSINESS WIRE)--Glycomine, Inc., a biotechnology company focused on developing new therapies for orphan diseases, announced the presentation of summary findings from its ongoing Phase 2 clinical study (GLM101-002) in adult patients with PMM2-CDG at the Rare Disease Day 2024 Symposium, CDG Scientific and Family Conference, that took place March 1-3 in San Diego.

The findings highlight the potential for clinically meaningful benefit with a notable improvement in the International Co-operative Ataxia Rating Scale (ICARS) score in four adult patients in the 30 mg/kg cohort, with an average improvement of 12 points after 12 weeks (n=4) of treatment. This compares favorably to prior results reported with acetazolamide which demonstrated an average improvement of 6 points after 25 weeks in adult and pediatric patients (n=25).

“We are thrilled to present data for the first time showing the potential for GLM101 to have a meaningful impact on ataxia, one of the most frequent and challenging clinical manifestations of this debilitating disease,” said Rose Marino, M.D., CMO of Glycomine. “These data provide invaluable input for the design of our future planned studies in adult and pediatric PMM2-CDG patients.”

“Reducing ataxia has the potential to significantly impact the quality of life for PMM2-CDG patients and their caregivers,” added Mercedes Serrano, M.D., Ph.D., Child Neurologist and Clinical Researcher, U-703 Centre for Biomedical Network Research on Rare Diseases (CIBERER), Sant Joan de Déu Hospital in Barcelona, Spain. “We are very encouraged by the initial results we have seen and delighted to be part of this important study.”

The GLM101-002 Phase 2 clinical study has enrolled 10 adult patients in the U.S. and Spain (ClinicalTrials.gov Identifier: NCT05549219). Study participants have received GLM101 at either 10 mg/kg (n=3), 20 mg/kg (n=3), or 30 mg/kg (n=4) for up to 24 weeks. Over 200 doses of GLM101 have been administered to patients with PMM2-CDG. The drug appears to be

safe and well tolerated with no serious adverse events and only mild to moderate adverse events reported to date. Four adolescent patients have initiated treatment, and the study is planned to be extended into younger PMM2-CDG patients in the coming months.

About GLM101

GLM101 is a mannose-1-phosphate replacement therapy in development to treat phosphomannomutase 2-congenital disorder of glycosylation (PMM2-CDG), previously known as CDG Type Ia. GLM101 has received Orphan Drug Designation in the U.S. and Europe and Rare Pediatric Disease Designation in the U.S. PMM2-CDG is caused by genetic mutations that lead to a deficiency of the enzyme phosphomannomutase 2 (encoded by the *PMM2* gene). GLM101 is designed to deliver mannose-1-phosphate directly into cells and thereby bypass the PMM2 enzyme deficiency and address disease-causing *PMM2* mutations to restore pathway function.

About Glycomine, Inc.

Glycomine is a clinical-stage biotechnology company developing novel drugs for serious rare disorders of metabolism and protein misfolding for which no other therapeutic options exist. The company's approach is to use replacement therapies – substrates, enzymes, or proteins – and to target those molecules to clinically relevant cellular compartments. The company is based in San Carlos, California and supported by leading international life sciences investors. For more info visit www.glycomine.com.

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