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GlycoMimetics Announces Publication of Phase 2 Data for Rivipansel in Sickle Cell Disease

- Data underscores potential to provide meaningful clinical benefits and pharmacoeconomic impacts for patients in vaso-occlusive crisis -

GAITHERSBURG, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ: GLYC) announced today the publication of results from a randomized, placebo-controlled Phase 2 investigational study evaluating the efficacy, safety and pharmacokinetics of rivipansel (GMI-1070) in patients with sickle cell disease hospitalized for a vaso-occlusive crisis (VOC). Study results were pre-published online by *Blood*,[1] the journal of the American Society of Hematology (ASH).

The article, entitled "Randomized Phase 2 Study of GMI-1070 in SCD: Reduction In Time To Resolution Of Vaso-Occlusive Crisis and Decreased Opioid Use," highlights rivipansel's potential to improve clinical outcomes in sickle cell patients experiencing VOC. Results from the study were previously highlighted in two oral presentations and one poster presentation at the December 2013 ASH Annual Meeting and Exposition. The two oral presentations were selected as "Best of ASH."

"We are encouraged by the results obtained in the Phase 2 rivipansel study and by the opportunity we've been afforded to publish the promising clinical and research findings in *Blood,* an esteemed peer-reviewed journal," said Helen Thackray, M.D., Vice President of Clinical Development and Chief Medical Officer at GlycoMimetics. "The study results support a role for selectins in vaso-occlusion and justify moving rivipansel into a pivotal Phase 3 study, a significant step forward in the effort to potentially address the unmet needs of individuals with sickle cell disease."

Rivipansel has previously received both Orphan Drug and Fast Track status for the treatment of VOC from the U.S. Food & Drug Administration (FDA), and Orphan Product status in the European Union. GlycoMimetics entered into a collaboration and exclusive license agreement with Pfizer, Inc. for rivipansel in October 2011.

"If rivipansel continues to demonstrate efficacy in ongoing studies, it would be the first drug to interrupt the mechanism of VOC in sickle cell disease," said corresponding study author Marilyn J. Telen, M.D., of Duke University Medical Center. "Even though we have increased our understanding of how these debilitating crises develop, we have not been able to intervene once they happen. This potential new drug could change that."

About the Study

This prospective multi-national, multi-center, randomized double-blind, placebo-control study enrolled 76 patients 12 to 60 years in age with sickle cell (types SS or S-beta-zero-thalassemia) presenting with VOC to evaluate the efficacy, safety and pharmacokinetics in patients receiving rivipansel. People who took part in the study were evaluated and then randomly assigned to receive either rivipansel or a placebo by IV, in addition to all other usual treatments for their pain crisis.

The primary efficacy endpoint was reduction in time to resolution of VOC, defined as 1.5 cm sustained decrease in visual analog scale (VAS) pain score from baseline and transition to oral pain medications; or feeling ready to leave the hospital; or actual time of discharge from the hospital. In the primary efficacy analysis, the median time to resolution of VOC was reduced by greater than 2.5 days following treatment with rivipansel (time to resolution was 69.6 hours for the rivipansel group compared to 132.9 hours for the placebo group; p=0.187.) Of note, treatment with rivipansel improved clinical outcomes on a number of key secondary endpoints including an 83 percent reduction in mean cumulative IV opioid analgesic use, relative to placebo (p=0.010).

Adverse events (AEs) were evaluated as a secondary endpoint of the phase 2 study. The total AE rates, serious AEs, and AEs considered related to treatment were comparable between patients treated with rivipansel and placebo. The most common treatment emergent AEs (TEAEs) were nausea, constipation, headache and acute chest syndrome. The serious AE rate was 30 percent in both placebo and rivipansel groups, with the most common serious AE being re-hospitalization for VOC. Eighteen participants discontinued the drug, nine in each treatment group, for AEs, no improvement at day 5, or other reasons. All participants were followed until meeting a study endpoint.

About Rivipansel (GMI-1070)

Rivipansel is an investigational selectin inhibitor, inhibiting E-selectin in particular. Selectins are a family of molecules which are believed to play a key role in regulating cellular interactions within the vasculature (blood vessels) and thereby mediating

intravascular blood flow. The intense pain associated with VOC is believed to be caused primarily by microvascular blockage in the post-capillary venules, from adhesion of leukocytes as well as clogging of sickle-shaped red blood cells (which become rigid and inflexible when sickled), both of which restrict blood flow causing local tissue ischemia and pain. GlycoMimetics, the developer of rivipansel, selected VOC of sickle cell disease as the first area of investigation for rivipansel.

About GlycoMimetics, Inc.

GlycoMimetics is a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. GlycoMimetics entered into an exclusive license agreement with Pfizer for rivipansel in October 2011. Under the license agreement, Pfizer is responsible for the clinical development, regulatory approval and potential commercialization of rivipansel.
A GlycoMimetics wholly-owned candidate therapy (GMI-1271) for acute myeloid leukemia (AML) and other blood disorders is also in clinical trials. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using its expertise in carbohydrate chemistry and knowledge of carbohydrate biology, GlycoMimetics is developing a pipeline of glycomimetic drug candidates that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. Learn more at www.glycomimetics.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements regarding the clinical development of rivipansel. Actual results may differ materially from those in these forward-looking statements. For a further description of the risks associated with these statements, as well as other risks facing GlycoMimetics, please see the risk factors described in the Company's quarterly report on Form 10-Q that was filed with the U.S. Securities and Exchange Commission on October 31, 2014, and other filings the Company makes with the SEC from time to time. Forward-looking statements speak only as of the date of this release, and GlycoMimetics undertakes no obligation to update or revise these statements, except as may be required by law.

[1] M.J. Telen, T. Wun, T.L. McCavit, L.M. De Castro, L. Krishnamurti, S. Lanzkron, L.L. Hsu, W.R. Smith, S. Rhee, J.L. Magnani, H.Thackray. Randomized Phase 2 Study of GMI-1070 in SCD: Reduction In Time To Resolution Of Vaso-Occlusive Crisis and Decreased Opioid Use. *Blood: First Edition.* 2015; http://www.bloodjournal.org/content/early/2015/03/02/blood-2014-06-583351

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