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# GlycoMimetics Announces Presentation of Data on Rivipansel (GMI-1070) in Combination with Current FDA-Approved Treatment for Sickle Cell Disease

*- Data on lead drug candidate, rivipansel (GMI-1070), highlighted via oral presentation and poster at 8th Annual Sickle Cell Disease Research and Educational Symposium and 37th National Sickle Cell Disease Scientific Meeting -*

GAITHERSBURG, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ:GLYC) announced today that data for its lead clinical drug candidate, rivipansel (GMI-1070), was highlighted via one oral presentation and one poster at the 8th Annual Sickle Cell Disease Research and Educational Symposium and 37th National Sickle Cell Disease Scientific Meeting, held April 11-14, 2014, at the InterContinental Miami.

Rivipansel is in clinical trials as a potential therapy for the treatment of vaso-occlusive crisis (VOC) in people with sickle cell disease. It 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 is developing rivipansel in collaboration with Pfizer, Inc.

"As we look to see rivipansel enter Phase 3 studies later this year, we are pleased with the opportunity to share data for the program at the 8th Annual Sickle Cell Disease Research and Educational Symposium and the 37th National Sickle Cell Disease Scientific Meeting," said Helen Thackray, M.D., Vice President of Clinical Development and Chief Medical Officer at GlycoMimetics.

The oral presentation by Laura De Castro, M.D., of the University of Pittsburgh was part of an Investigational Drugs and Therapeutics Symposium held Sunday, April 13. Entitled "Hydroxyurea use and effects on outcomes in a Phase 2 Study of GMI-1070, a novel agent for vaso-occlusive crisis," the oral presentation reported results from a randomized, multi-center, double-blind, adaptive Phase 2 study of sickle cell disease patients with VOC. Hydroxyurea is the only FDA-approved disease modifying therapy for sickle cell disease. Given that investigators recently evaluated rivipansel in sickle cell disease patients hospitalized for VOC with promising results, investigators studied what type of effect could be found through use of rivipansel with hydroxyurea. Forty-five of the total 76 subjects enrolled were on hydroxyurea during the trial. Rivipansel improved efficacy outcomes independent of hydroxyurea use. Patients on hydroxyurea appeared to benefit similarly from addition of rivipansel to their standard treatment regimen versus those not on hydroxyurea. This was true for multiple measures of clinical efficacy, including time to resolution of VOC, time to hospital discharge, and improvement in pain scores. These findings are of clinical interest regarding the study target population, especially since hydroxyurea is associated with lower white blood cell counts, activation, and adhesion, and rivipansel likely acts primarily by blocking selectin-mediated leukocyte adhesion.

GlycoMimetics also presented a poster at the Miami meeting entitled "Completion of a Phase 2 study in SCD VOC: Challenges in Enrollment."

**About Sickle Cell Disease**

There are more than 90,000 people in the U.S. with sickle cell disease, and many of them have multiple, acute VOCs annually. These painful crises result in more than 73,000 hospitalizations per year in the U.S., with an average stay of approximately six days.

**About Rivipansel**

Rivipansel is being developed in partnership with Pfizer to treat vaso-occlusive crisis of sickle cell disease (VOC). The compound is a synthetic glycomimetic molecule, which was rationally designed to inhibit all three selectin types (a pan-selectin inhibitor). Selectins are glycoprotein cell adhesion molecules implicated in inflammatory processes. To achieve adequate therapeutic activity in certain inflammatory disorders, inhibition of all three selectin types (E-selectin, L-selectin and P-selectin) may be required. We therefore believe that rivipansel's ability to inhibit all selectins will provide distinct advantages over other approaches that target only one selectin, or which are so broadly active as to be non-specific. GlycoMimetics has conducted a Phase 2 randomized, double-blinded study examining the efficacy, safety and pharmacokinetics of rivipansel in hospitalized sickle cell disease patients experiencing VOC. GlycoMimetics enrolled 76 patients ages 12 to 60 at 22 trial sites in the United States and Canada. The company reported topline data from the trial in April 2013 and presented full data from the clinical trial in two oral presentations and one poster presentation at the December 2013 meeting of the American Society of Hematology (ASH.) One of the oral presentations was selected to be among "Best of ASH." In the Phase 2 trial, patients treated with GMI-1070 experienced reductions in time to reach resolution of VOC, length of hospital stay and use of opioid analgesics for pain

management, in each case as compared to patients receiving placebo.

**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 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.

This press release contains forward-looking statements regarding GlycoMimetics' planned activities with respect to the clinical development of GMI-1070 and other matters. 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 annual report on form 10-K that was filed with the U.S. Securities and Exchange Commission on March 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.

**GlycoMimetics, Inc.**
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