New Efficacy and Biomarker Data From Rivipansel Phase 3 RESET Trial to Be Presented at Sickle Cell Meeting

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- Abstracts for two of GlycoMimetics' wholly-owned E-selectin inhibitors, rivipansel and GMI-1687, to be published at September meeting of the Foundation for Sickle Cell Disease Research

ROCKVILLE, Md.--(BUSINESS WIRE)--Jun. 12, 2020-- GlycoMimetics, Inc. (Nasdaq: GLYC) today announced that a *post hoc* analysis of the Phase 3 RESET study evaluating the efficacy of rivipansel , its wholly-owned development candidate, in acute vaso-occlusive crisis (VOC) shows that patients treated with rivipansel within approximately 26 hours of the onset of pain in their crisis experienced statistically significant improvements in the primary efficacy endpoint of time to readiness for discharge compared to placebo. This analysis and new biomarker data will be presented at the September meeting of the Foundation for Sickle Cell Disease Research (FSCDR). In addition to the rivipansel poster, an abstract containing data on GlycoMimetics' more selective and highly potent E-selectin antagonist, GMI-1687, has been accepted for an oral presentation. The GMI-1687 abstract includes data from a preclinical model showing the drug candidate's potential as a subcutaneously administered treatment for VOC. FSCDR posted the abstracts online today for the meeting now scheduled for September 23-25, 2020, in Ft. Lauderdale, FL.

The rivipansel abstract includes data from a supportive analysis of the Phase 3 RESET trial of 345 patients (ranging in age from six years to adults, with a mean age of 22 years) who were experiencing acute VOC requiring hospitalization for treatment. The analysis shows that patients treated with rivipansel early in their acute episode experienced a statistically significant improvement on the primary efficacy endpoint, time to readiness for discharge (p=0.03, median improvement was 58 hours). This endpoint reflects achievement of multiple clinical criteria assessing healthcare utilization and a patient's medical improvement prior to leaving the hospital. Furthermore, patients treated with rivipansel showed a statistically significant reduction in soluble E-selectin, a biomarker indicating that the drug had the intended biological effect. The effect observed on soluble E-selectin in this trial provides valuable insight into the mechanism for the improvement in the clinical criteria for discharge from the hospital observed in those patients treated early in their acute VOC. Data from the RESET trial additionally demonstrate a safety profile for rivipansel comparable to the placebo.

"The important data disclosed today demonstrate that patients treated within approximately 26 hours of the start of a VOC benefited from receiving rivipansel. In addition, the biomarker data showing reductions in soluble E-selectin indicates that rivipansel is hitting its intended biological target. These two findings confirm the critical role of E-selectin in acute vaso-occlusion, as well as the importance of treating individuals early in the course of their acute painful crisis," said Helen Thackray, GlycoMimetics' Chief Medical Officer.

"The favorable safety profile of rivipansel observed in this trial, as evaluated in a population with pediatric, adolescent, and adult patients, is highly encouraging to us. We are actively considering options for rivipansel in this acute treatment setting, for which there are no approved drugs and, to our knowledge, no drugs currently in late-stage development. Now that Pfizer's development and commercialization rights, including the investigational new drug (IND) application for rivipansel, have been transferred back to us, we intend to discuss these data with the U.S. Food and Drug Administration (FDA) to determine what, if any, next steps could be taken to carry this program forward in acute VOC, either in pediatrics or in the overall population," she added.

The second abstract, accepted for oral presentation, discloses data from a preclinical model of GlycoMimetics E-selectin antagonist, GMI-1687, which is even more potent than rivipansel and is formulated for subcutaneous dosing.

"The data disclosed in this second abstract support development of GMI-1687 as a possible follow-on to rivipansel, which has the potential for subcutaneous self-administration as would be used in an outpatient setting," continued Dr. Thackray. "Taken together, these abstracts support use of rivipansel in early treatment of acute VOC and the potential use of GlycoMimetics' drug candidates to address a very significant unmet medical need."

About Sickle Cell Disease (SCD) and VOC

SCD is the most common inherited blood disorder in the United States, impacting approximately 100,000 people. Worldwide, approximately 100 million people carry the SCD trait and an estimated five million live with the disease. While the majority of people with SCD are of African descent, the disease can affect all ethnic groups, especially those from areas where malaria is or was endemic, such as the Middle East, India and the Southern Mediterranean. Acute pain crises or VOCs are the most common clinical manifestation of SCD. A VOC occurs when hypoxia and inflammation lead to vascular occlusion, tissue ischemia and pain.

About Rivipansel

Rivipansel, a glycomimetic drug candidate that binds to all three members of the selectin family (E-, P- and L-selectin), was GlycoMimetics' first drug candidate to enter clinical development. After the Phase 3 RESET trial conducted by Pfizer, GlycoMimetics' former collaborator, produced disappointing results in 2019, new efficacy data from a *post hoc* analysis of rivipansel were published in June 2020 in advance of a presentation to occur at the Foundation for Sickle Cell Disease Research Meeting in September 2020. GlycoMimetics is committed to exploring a path forward for the use of rivipansel in treating acute VOC in SCD.

About GMI-1687

Discovered and developed by GlycoMimetics, GMI-1687 is a highly targeted highly potent E-selectin antagonist. It has been shown in preclinical studies to be bioavailable via subcutaneous administration. At the 2018 Annual Meeting of the American Society of Hematology, data presented in a poster about GMI-1687 pointed to the potential for a life-cycle extension for GlycoMimetics' uproleselan. The investigational drug is also thought to

represent a more highly-potent and subcutaneously bioavailable potential life-cycle extension for rivipansel, the company's drug candidate being explored for the treatment of acute VOC in SCD.

About GlycoMimetics, Inc.

GlycoMimetics is a biotechnology company with two late-stage clinical development programs and a pipeline of novel glycomimetic drugs, all designed to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. GlycoMimetics' drug candidate, uproleselan, an E-selectin antagonist, was evaluated in a Phase 1/2 clinical trial as a potential treatment for AML and is being evaluated across a range of patient populations including a Company-sponsored Phase 3 trial in relapsed/refractory AML under breakthrough therapy designation. Rivipansel, a pan-selectin antagonist, is being explored for use in treatment of acute VOC in SCD. GlycoMimetics has also completed a Phase 1 clinical trial with another wholly-owned drug candidate, GMI-1359, a combined CXCR4 and E-selectin antagonist. GlycoMimetics is located in Rockville, MD in the BioHealth Capital Region. Learn more at www.glycomimetics.com.

Forward-Looking Statements

This press release contains forward-looking statements regarding the clinical development and potential benefits and impact of the Company's drug candidates. These forward-looking statements include those relating to the planned clinical development of the Company's product candidates, including the presentation of data from preclinical studies and clinical trials. Actual results may differ materially from those described 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 filed with the U.S. Securities and Exchange Commission (SEC) on February 28, 2020, and other filings GlycoMimetics 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.

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