New Pediatric and Secondary Endpoint Data from Rivipansel Phase 3 RESET Trial Presented at The Annual Scientific Conference on Sickle Cell and Thalassemia (ASCAT) Meeting

October 26, 2020

- Data highlight the importance of early intervention with fast-acting E-selectin inhibitors to disrupt the underlying inflammatory mechanisms driving acute vaso-occlusive crisis (VOC)

- Abstracts for two of GlycoMimetics' wholly-owned product candidates, rivipansel and GMI-1687, accepted for poster and oral presentations

ROCKVILLE, Md.--(BUSINESS WIRE)--Oct. 26, 2020-- GlycoMimetics, Inc. (Nasdaq: GLYC) today announced that new *post hoc* analyses of the Phase 3 RESET study evaluating the efficacy of rivipansel, its wholly-owned product candidate, in VOC show that pediatric patients treated with rivipansel within approximately 30 hours of the onset of acute crisis pain experienced statistically significant improvements in the primary efficacy endpoint of time to readiness for discharge (TTRFD) compared to placebo as well as in two key secondary endpoints, namely time-to-discharge (TTD) and time-to-discontinuation-of-IV opioids (TTDIVO). The analyses also show that subjects treated within 26.4 hours of the onset of acute crisis pain achieved statistically significant improvements in the same endpoints. An abstract highlighting these analyses was selected for a poster presentation at the ASCAT meeting, which starts today. In addition to the rivipansel presentation, the meeting organizers also accepted for oral presentation an abstract containing data on GlycoMimetics' more selective and highly potent E-selectin antagonist, GMI-1687. The GMI-1687 abstract includes data from a preclinical model showing the drug candidate's potential as a subcutaneously, self-administered treatment for VOC. The British Society of Haematology (BSH) and the European Hematology Association co-sponsored virtual ASCAT meeting that runs through October 31, 2020.

"The important data published today provide further support for the potential benefits of treatment with rivipansel early in the course of VOC, as observed in sickle cell patients in the RESET trial – both in the total patient population as well as in the pediatric subgroup. In addition to highlighting the importance of treating individuals early in the course of their acute painful crisis, these new findings confirm the critical role of E-selectin in acute vaso-occlusion and the opportunity to resolve that occlusion and pain with effective intervention," said Helen Thackray, MD, FAAP, GlycoMimetics' Chief Medical Officer.

"The favorable safety profile of rivipansel observed in the Phase 3 RESET trial, as evaluated in a population with pediatric, adolescent, and adult patients, is highly encouraging to us. We are engaging in discussions with the 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. This treatment setting remains an area of unmet medical need, as there are no drugs approved nor currently in late-stage development for acute intervention," she added.

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 new data demonstrate that early rivipansel treatment conferred clinically meaningful improvements for two key secondary endpoints not previously reported: shortening IV opioid use and decreasing the hospital stay. Specifically, for the total treated patient population (n=320), rivipansel treatment within 26.4 hours of pain onset (earliest quartile of duration of VOC until treatment) reduced:

- median TTD by 41.5 hours (from 112.8 to 71.3 hours; p=0.02), and
- median TTDIVO by 50.5 hours (from 104.0 to 53.5 hours; p=0.03) vs. placebo.

In addition, newly scheduled for presentation at this meeting are data for the pediatric subgroup, who make up a large proportion (41%) of patients treated in the RESET trial. Namely, data from children 6-17 years old in the study who were treated within 30 hours of VOC onset show:

- a reduction in median TTRFD by 29.3 hours (p=0.02),
- a reduction in median TTD by 23.2 hours (p=0.02),
- a reduction in median TTDIVO by 15.4 hours (p=0.045), and
- more children ready for discharge by 24, 48 and 72 hours, compared to placebo.

The abstract also features key findings previously reported at the Foundation for Sickle Cell Research (FSCDR) meeting in September, 2020. Specifically, the analysis shows that patients treated with rivipansel early in their acute VOC pain episode experienced a statistically significant reduced median on the primary efficacy endpoint, TTRFD (p=0.03, median improvement was 56.3 hours). This endpoint reflects achievement of multiple clinical criteria assessing healthcare utilization and a patient's medical improvement prior to leaving the hospital. Specifically, rivipansel treatment within 26.4 hours of pain onset (earliest quartile of duration of VOC until treatment) reduced median TTRFD by 56.3 hours (from 122.0 to 65.7 hours), reduced median TTD by 41.5 hours (from 112.8 to 71.3 hours), and reduced median TTDIVO by 50.5 hours (from 104.0 to 53.5 hours), compared to placebo. Furthermore, patients treated with rivipansel showed a statistically significant reduction of 59% from baseline after a loading dose 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 that is comparable to the placebo.

The second abstract, accepted for oral presentation, discloses data from two different preclinical models of VOC using GlycoMimetics' highly potent and specific E-selectin antagonist, GMI-1687. These data show the drug candidate's efficacy as a subcutaneously administered treatment for VOC. Using both a human sickle cell transplantation model and the Townes mouse model, GMI-1687 was shown to prevent sickle red blood cell adherence to inflamed vasculature, inhibit vessel occlusion and restore normal blood flow. Preliminary data showing the activity of GMI-1687 for the treatment of

VOC were disclosed in an oral presentation late September at the FSCDR virtual meeting.

Details on GlycoMimetics presentations at the ASCAT Meeting are as follows:

Title: Early Initiation of Treatment with Rivipansel for Acute Vaso-Occlusive Crisis in Sickle Cell Disease (SCD) Achieves Earlier Discontinuation of IV Opioids and Shorter Hospital Stay: RESET Clinical Trial Analysis.
Presenter: Jay Lozier, MD, PhD, FACP, Senior Medical Director, Clinical Development
Date: October 26, 2020

Title: Treatment of Acute Vaso-occlusion in Mouse Models of Sickle Cell Disease Following Intravenous or Subcutaneous Administration of a Highly Potent E-selectin Specific Inhibitor
Presenter: John Magnani, PhD, GlycoMimetics Senior Vice President, Research & Chief Scientific Officer
Session: Sickle Cell Disease – Basic Science: Emerging Therapies
Date and Time: October 26, 2020. 1:10 – 3:30 p.m. BST

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, the company's wholly-owned glycomimetic drug candidate that binds to all three members of the selectin family (E-, P- and L-selectin), was GlycoMimetics' first 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 and subsequently presented at the Foundation for Sickle Cell Disease Research meeting in September 2020. GlycoMimetics is engaging with the FDA in discussions to identify what, if any, next steps to take, with a focus on determining if there is a potential streamlined path forward for this product candidate 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 has been shown to represent a more highly-potent and subcutaneously bioavailable potential life-cycle extension for rivipansel, the company's wholly-owned 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 acute myeloid leukemia (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 preclinical and clinical data, clinical development and potential benefits and impact of the Company's drug candidates. These forward-looking statements include those relating to the planned or potential clinical development of the Company's product candidates, including the Company's engagement with regulatory authorities and 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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