New Supportive Efficacy Data for GlycoMimetics' Rivipansel in Sickle Cell Acute Vaso-Occlusive Crisis Presented at 62nd ASH Annual Meeting and Exposition

December 7, 2020

Open label study demonstrated statistically significant effects of early treatment with rivipansel on time to discharge and time to discontinuation of intravenous opioids

Benefit of early intervention with E-selectin antagonist in acute VOC now reproduced in independent, contemporaneous dataset that includes both the all-ages and pediatric populations

Company expects to initiate Investigational New Drug-enabling work with GMI-1687 during 2021

ROCKVILLE, Md.--(BUSINESS WIRE)--Dec. 7, 2020-- GlycoMimetics (Nasdaq: GLYC) today in an oral presentation at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition disclosed new data reinforcing a now comprehensive set of findings that underscore the improvement of rivipansel when administered early during an acute vaso-occlusive crisis (VOC) in individuals with sickle cell disease (SCD). The new data were part of an analysis of an Open Label Extension (OLE) trial of rivipansel, designed to evaluate real-world data on safety and efficacy in patients treated first in the RESET Phase 3 trial conducted by the company's former collaborator Pfizer. The OLE trial ran in parallel to the RESET trial and provides an independent, contemporaneous dataset for comparison of clinically meaningful efficacy outcomes with rivipansel. The data from the OLE efficacy analysis, together with more extensive data from the RESET trial post-hoc analysis previously reported, were presented at ASH by University of California Davis' Theodore Wun, M.D., Interim Vice Dean for Research, Associate Dean for Research, School of Medicine; Director, Clinical and Translational Science Center; and Chief, Division of Hematology and Oncology.

In his oral presentation, Dr. Wun reviewed key findings from several *post hoc* analyses of the Phase 3 RESET trial and a subsequent pre-planned analysis of the OLE trial. Specifically, these included the achievement of statistically significant improvements in primary and key secondary endpoints, with early treatment in the pediatric subgroup as well as the all-ages treatment group and importantly, on several key secondary endpoints, including time to discharge (TTD) and time to discontinuation of IV opioids (TTDIVO).

Dr. Wun's presentation highlighted data from the OLE trial reproducing the RESET efficacy outcomes for patients treated early with rivipansel. For the all-ages study population:

- On duration of hospital stay, the median TTD with early rivipansel treatment in the OLE study was 80.89 hours compared to 103.97 hours with early placebo in the RESET study, a difference of nearly one day (23.08 hours). This difference achieved significance at the pre-specified 90% confidence level, with a P-value of 0.0617; and
- On duration of IV opioid use, the median TTDIVO for early rivipansel treatment in the OLE study was 63.87 hours compared to 93.99 hours for the early placebo in the RESET study, a difference of more than one day (30.12 hours). This difference achieved significance at the pre-specified 90% confidence level, with a P-value of 0.0867.

Dr. Wun also disclosed data for the pediatric population in the RESET trial, representing 41% of the total data set, including that:

- Children treated with rivipansel within 30 hours of onset of VOC experienced a reduction in the primary endpoint median TTRFD by 29.3 hours (from 94.1 to 64.8 hours); and
- Early treatment with rivipansel led to more children ready for discharge from the hospital by 24, 48, and 72 hours.

Rachel King, GlycoMimetics' Chief Executive Officer, noted, "The OLE analysis of efficacy outcomes for early treatment with rivipansel confirms the observations made in post-hoc assessment of the RESET trial, and does so for two important, clinically meaningful endpoints. The consistency of these findings clearly supports the validity of E-selectin as a target, our product candidate's ability to hit the target, and the use of an E-selectin antagonist for treating patients early in VOC. While we fully expect that additional clinical data will be required to achieve registration for rivipansel, we plan to continue interactions with the FDA to determine the clinical and regulatory path forward prior to making any decision as to how or whether we will proceed in this acute setting. As Dr. Wun presented at ASH, there remains a need for therapy that will abrogate VOC and reduce the duration of hospitalization and opioid use. We are unaware of any late-stage clinical programs targeting the resolution of acute vaso-occlusive episodes. In sum, our data point to the potential for early administration of rivipansel to change the treatment paradigm from delayed palliation to early intervention to reduce length of hospitalization and IV opioid requirement."

The OLE study results, presented for the first time at ASH, began in December of 2015 and treated 81 patients ages six years or older who had completed the double-blind Phase 3 RESET study. The study was designed to evaluate the safety of rivipansel as a treatment for one or more VOC events in hospitalized subjects with SCD. In this context, the OLE protocol originally included collection of data for TTD and TTDIVO analyses as part of the safety assessment. Upon completion of the *post hoc* analysis of the RESET trial dataset, GlycoMimetics recognized the opportunity to leverage the OLE as a contemporaneous clinical trial to confirm the RESET study early treatment efficacy findings for TTD and TTDIVO. GlycoMimetics developed and submitted a Statistical Analysis Addendum (SAA) to FDA to compare the single-arm OLE data to the RESET trial data to assess if the RESET observations were reproducible. The SAA was implemented prior to final reporting of the OLE study datasets and outcomes by Pfizer, allowing the OLE dataset to be evaluated for efficacy outcomes in a pre-planned analysis. In effect, a prospectively planned and executed OLE safety study

was converted prior to final analysis by GlycoMimetics into a study including efficacy outcomes. This allowed independent confirmation of the improvements in TTD and TTDIVO observed with early rivipansel treatment in acute VOC, using a contemporaneous single-arm dataset.

In addition to the rivipansel data, GlycoMimetics also presented preclinical data in another oral presentation at the ASH meeting showing that GMI-1687, the Company's highly potent E-selectin antagonist, was active in restoring blood flow in a mouse model of VOC when administered subcutaneously. "With data from the rivipansel program supporting targeting of E-selectin early in acute vaso-occlusive crisis, a subcutaneously administered E-selectin antagonist such as GMI-1687 could be ideally suited for self-administration by patients," Rachel King added.

GlycoMimetics expects to initiate Investigational New Drug enabling work with GMI-1687 during 2021.

The presentation will be available on the Company's website at www.glycomimetics.com under Scientific Publications, and Rivipansel. Details of the presentation 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 (abstract #678)
Presenter: Theodore Wun, M.D., University of California Davis, Interim Vice Dean for Research, Associate Dean for Research, School of Medicine; Director, Clinical and Translational Science Center; and Chief, Division of Hematology and Oncology.
Session Name: 114 Hemoglobinopathies, Excluding Thalassemia—Clinical: Novel Treatments for Sickle Cell Disease
Date and Time: Monday, December 7, 2020**.** 4:30 – 6:00 p.m. ET
Presentation Time: 4:45 p.m. ET

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 drug candidate to enter clinical development. After the Phase 3 RESET trial conducted by Pfizer, GlycoMimetics' former collaborator, did not meet its primary or key secondary efficacy endpoints 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. Additional new efficacy data from an Open Label Extension trial of rivipansel were presented at the 62nd American Society of Hematology Annual Meeting and Exposition in December 2020. GlycoMimetics is engaging with the FDA to identify what, if any, next steps to take in connection with the development of rivipansel as a treatment for vaso-occlusive crisis of sickle cell disease.

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. During 2020, data from oral presentations at major scientific conferences pointed to the potential for a self-administered drug to treat vaso-occlusive crisis of sickle cell disease. The investigational drug represents a potential life cycle extension opportunity for GlycoMimetics' drug candidates rivipansel and uproleselan.

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 sickle cell disease. 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. 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, as well as the presentation of data from preclinical studies and clinical trials and the potential benefits and impact of the Company's drug candidates. 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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