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GlycoMimetics Completes Enrollment of Relapsed/Refractory AML Patient Cohort in Phase 2 Portion of Clinical Trial of GMI-1271

- | *Clinical trial now fully enrolled with 91 patients: 25 in newly diagnosed arm and 66 in relapsed refractory arm*
- | *Interim data to be presented at ASCO and EHA Meetings showing high remission rates with acceptable tolerability*
- | *Patients with higher levels of novel biomarker were more likely to achieve response when GMI-1271 was added to standard therapy*

ROCKVILLE, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ:GLYC) today announced that the second of two patient cohorts in the Phase 2 portion of its ongoing Phase 1/2 clinical trial of GMI-1271 in patients with acute myeloid leukemia (AML) has completed enrollment. This second cohort is comprised of 66 participants with relapsed/refractory AML. The study is designed to evaluate the potential of GMI-1271, an E-selectin antagonist drug candidate, in combination with chemotherapy, as a treatment for individuals with either newly diagnosed or relapsed/refractory AML. Enrollment in the study's first arm in newly diagnosed elderly AML patients was completed in the first quarter of this year. GlycoMimetics expects to submit interim study data for presentation at the American Society of Hematology (ASH) Annual Meeting in December 2017.

"Across the two cohorts of this Phase 1/2 clinical trial, we have a strong sample size of 91 patients and experienced brisk enrollment, which we feel is indicative of the strong interest that our clinical investigators have for this novel agent," said [Helen Thackray](#), M.D., Chief Medical Officer of GlycoMimetics. "We are further encouraged by GMI-1271's achievement of European Orphan Designation as well as the recent granting of Breakthrough Therapy designation by the U.S. Food & Drug Administration (FDA) for the treatment of adults with relapsed/refractory AML. We believe GMI-1271, when combined with chemotherapy, has the potential to address an unmet therapeutic need for individuals living with AML, and we are encouraged by both our clinical results to date and the acknowledgement of the U.S. and European regulatory agencies."

Interim clinical data from the ongoing trial of GMI-1271 will be presented at the 2017 meetings of the American Society of Clinical Oncology (ASCO) and the European Hematology Association (EHA). To date, GMI-1271 has been consistently well tolerated, with no obvious incremental toxicity when added to chemotherapy. In addition, patients with AML treated with GMI-1271 have experienced higher than expected remission rates and lower than expected 30- and 60-day mortality rates.

"The consistency of our data readouts is great news in and of itself, and the emerging biomarker data is especially encouraging," said [Rachel King](#), CEO of GlycoMimetics. "AML has long been a difficult indication for the developers of new therapeutics, and we continue to feel more confident that our investigational drug, GMI-1271, may play a role in addressing key unmet needs in this deadly cancer."

In addition to the ongoing Phase 1/2 trial, clinical investigators are currently evaluating GMI-1271 in an ongoing Phase 1 clinical trial in multiple myeloma. Preclinical data supporting the multiple myeloma study was recently published in an online preview of the journal *Leukemia* on April 25, 2017. Specifically, the newly published preclinical results indicate that myeloma with higher levels of E-selectin ligands is a more aggressive disease that is more likely to be resistant to bortezomib, which is currently the front-line standard of care. The publication reported that in preclinical studies, the combination treatment of GMI-1271 to bortezomib was able to break this chemoresistance and restore sensitivity to bortezomib which led to significant improvement in survival. This preclinical multiple myeloma conclusion supports the clinical findings on the potential biomarker that the Company is presenting at ASCO and EHA next month.

About AML

AML is a cancer of the blood and bone marrow. AML is the most common type of acute leukemia in adults. The National Cancer Institute estimates that there will be over 21,000 new cases of AML diagnosed in 2017 in the United States, and over 10,000 people will die from all forms of the disease in 2017. AML is more commonly present in elderly patients. Unlike other cancers that start in an organ and spread to the bone marrow, AML is known for rapid growth of abnormal white blood cells that gather in the bone marrow, getting in the way of normal blood cell production. The lack of normal blood cells can cause some of the symptoms of AML, including anemia (shortage of red blood cells resulting in tiredness and weakness), neutropenia (shortage of white blood cells that may lead to increased infections), and thrombocytopenia (shortage of platelets in the blood that may lead to excessive bleeding). Current treatment options for AML consist of reducing and eliminating cancer cells mainly through chemotherapy, radiation therapy, and stem cell transplantation.

About GlycoMimetics, Inc.

GlycoMimetics is a clinical-stage biotechnology company focused on cancer and sickle cell disease. GlycoMimetics' most advanced drug candidate, rivipansel, a pan-selectin antagonist, is being developed for the treatment of vaso-occlusive crisis in sickle cell disease and is being evaluated in a Phase 3 clinical trial being conducted by its strategic collaborator, Pfizer. GlycoMimetics' wholly-owned drug candidate, GMI-1271, a specific E-selectin inhibitor, is being evaluated in an ongoing Phase 1/2 clinical trial as a potential treatment for AML and in a Phase 1 clinical trial for the treatment of multiple myeloma. GlycoMimetics has also recently initiated a clinical trial with a third 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 GlycoMimetics' planned activities with respect to the clinical development of its drug candidate, GMI-1271. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including the availability and timing of data from ongoing clinical trials, the uncertainties inherent in the completion of ongoing clinical trials and the initiation of future clinical trials, whether interim results from a clinical trial will be predictive of the final results of the trial or results of early clinical trials will be indicative of the results of future trials, expectations for regulatory approvals, availability of funding sufficient for GlycoMimetics' foreseeable and unforeseeable operating expenses and capital expenditure requirements, other matters that could affect the availability or commercial potential of GlycoMimetics' drug candidates and other factors discussed in the "Risk Factors" section of GlycoMimetics' Annual Report on Form 10-K that was filed with the U.S. Securities and Exchange Commission on March 1, 2017, and other filings GlycoMimetics makes with the Securities and Exchange Commission from time to time. In addition, the forward-looking statements included in this press release represent GlycoMimetics' views as of the date hereof. GlycoMimetics anticipates that subsequent events and developments may cause its views to change. However, while GlycoMimetics may elect to update these forward-looking statements at some point in the future, GlycoMimetics specifically disclaims any obligation to do so, except as may be required by law. These forward-looking statements should not be relied upon as representing GlycoMimetics' views as of any date subsequent to the date hereof.

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