



May 25, 2017

## **GlycoMimetics' GMI-1271 Receives EU Orphan Drug Designation for Acute Myeloid Leukemia**

ROCKVILLE, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ: GLYC) today announced that the European Commission, based on a favorable recommendation from the European Medicines Agency (EMA) Committee for Orphan Medicinal Products, has granted orphan designation for the company's drug candidate GMI-1271 for the treatment of acute myeloid leukemia (AML). [The U.S. Food and Drug Administration \(FDA\) previously granted orphan drug designation for GMI-1271 for the treatment of AML in May of 2015.](#)

GMI-1271, a specific E-selectin inhibitor is being evaluated in the company's ongoing Phase 1/2 clinical trial, in which clinicians are evaluating the use of GMI-1271 along with chemotherapy in patients with relapsed or refractory AML as well as those with newly diagnosed AML. [Earlier this month, the company announced that GMI-1271 had been granted Breakthrough Therapy designation by the FDA. The company also announced that abstracts had been published by both the American Society of Clinical Oncology \(ASCO\) and the European Hematology Association \(EHA\) highlighting new data from the Phase 2 portion of the company's ongoing Phase 1/2 trial that will be presented at their upcoming annual meetings in June.](#)

"The European orphan designation will provide incentives for the commercialization and development of GMI-1271 in AML, where there are limited therapies available to patients," said [Helen Thackray](#), M.D., FAAP, Senior Vice President, Clinical Development and Chief Medical Officer of GlycoMimetics. "We believe that GMI-1271, when combined with standard 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 achieving this designation from the European Commission."

The European Commission grants orphan designation to drugs intended to treat, prevent or diagnose life-threatening or chronically debilitating rare disorders, defined as diseases with prevalence of no more than 5 in 10,000 in the EU, for which no satisfactory method of diagnosis, prevention or treatment yet exists. Orphan designation provides benefits including commercialization incentives, protection of intellectual property, including 10 years of market exclusivity and protocol assistance through the EMA's Scientific Advice program.

### **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](http://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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Source: GlycoMimetics, Inc.

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