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FDA Grants Orphan Drug Designation To GlycoMimetics' GMI-1271 For Treatment of Acute Myleogenous Leukemia (AML)

GAITHERSBURG, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ: GLYC) announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to GMI-1271, a novel and proprietary E-selectin antagonist in the company's pipeline for treatment of patients with acute myeloid leukemia (AML). GlycoMimetics is currently recruiting patients in a Phase 1/2, open-label multicenter study designed to evaluate the safety, pharmacokinetics and efficacy of GMI-1271 in combination with chemotherapy in adult patients with AML; a Phase 1 study in healthy volunteers was completed last year.

"Having the FDA designate GMI-1271 as an orphan drug for the treatment of AML is an important accomplishment for GlycoMimetics. This is a significant regulatory milestone for our program," said Helen Thackray, M.D., FAAP, Vice President of Clinical Development and Chief Medical Officer, GlycoMimetics. "We look forward to advancing GMI-1271 through clinical trials targeting E-selectin for multiple blood, or hematologic, cancers."

The FDA's Orphan Drug designation program is designed to promote the development of promising therapeutics for the treatment of rare diseases affecting fewer than 200,000 people in the United States. Orphan Drug designation includes benefits such as a potential seven-year period of U.S. marketing exclusivity after approval. Other potential advantages include protocol assistance, the ability to apply for research funding, tax credits for certain research expenses, and regulatory fee waivers.

About AML

Acute myeloid leukemia (AML) is a cancer of the blood and bone marrow. AML is the most common type of acute leukemia in adults, and it is estimated that there were over 18,000 new cases and over 10,000 deaths from the disease in 2014. Unlike other cancers that start in an organ and spread to the bone marrow, AML is associated with rapid growth of abnormal white blood cells that gather in the bone marrow and, as a result, impede normal blood cell production. While leukemic cells move into the blood, the lack of normal blood cells can lead to a number of symptoms, 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 include chemotherapy and stem cell transplantation, both of which can destroy cancer cells but do not reduce the related side effects.

About GlycoMimetics, Inc.

GlycoMimetics is a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Pfizer is the company's development partner for rivipansel, a GlycoMimetics-discovered investigational therapy for pain crisis associated with sickle cell disease, and is preparing to conduct a Phase 3 clinical study. A GlycoMimetics wholly-owned drug candidate (GMI-1271) for acute myeloid leukemia (AML) and other blood disorders is also in clinical trials. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using its expertise in carbohydrate chemistry and knowledge of carbohydrate biology, GlycoMimetics is developing a pipeline of glycomimetic drug candidates that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. Learn more at www.glycomimetics.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements regarding the clinical development of GMI-1271. Actual results may differ materially from those 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 that was filed with the U.S. Securities and Exchange Commission on March 16, 2015, and other filings the Company 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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