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GlycoMimetics Announces Initial Clinical Data from Phase 1 Portion of Clinical Trial of GMI-1271 in AML Patients

- Encouraging Anti-Leukemic Activity Observed in Patients with Advanced AML; Overall Response Rate of 62% in Initial Cohort of 13 Patients

ROCKVILLE, Md.--(BUSINESS WIRE)-- GlycoMimetics, Inc. (NASDAQ: GLYC) today announced that of the first 13 evaluable patients in its clinical trial of GMI-1271 in combination with chemotherapy in patients with relapsed/refractory acute myeloid leukemia (AML), investigators have observed clinical responses in eight patients, for an overall response rate of 62%. Of the eight objective responses, seven patients achieved a complete response (CR), with the eighth patient achieving complete response but with an incomplete blood count recovery (CRi). GMI-1271 was also well tolerated in this group of 13 patients. The detailed findings have been submitted to a major scientific meeting.

"We are encouraged by the responses seen so far in patients in this trial," said Daniel J. DeAngelo, M.D., Ph.D., Director of Clinical and Translational Research of the Adult Leukemia Program at the Dana-Farber Cancer Institute and the Principal Investigator on the trial. "Patients with AML have such unsatisfactory treatment options that it is important to continue enrolling patients into this trial to characterize the full potential of GMI-1271."

"I am pleased to see the favorable safety profile and early clinical activity of GMI-1271 in these patients," said Helen Thackray, M.D., Chief Medical Officer of GlycoMimetics. "We believe that by selectively disrupting cell-adhesion-mediated drug resistance mechanisms within the bone marrow, GMI-1271 may significantly enhance the efficacy of chemotherapy, without adding incremental toxicity. We look forward to presenting the complete safety, pharmacokinetics, pharmacodynamics and anti-tumor activity, including durability of CRs, at an upcoming scientific meeting."

About the Phase 1/2 Clinical Trial

Following the completion of a Phase 1 trial in 2014, GlycoMimetics has initiated a multinational, Phase 1/2, open-label trial in which researchers are evaluating the safety, pharmacokinetics (PK) and efficacy of GMI-1271 as an adjunct to standard chemotherapy in patients with advanced AML. This trial is being conducted at a number of academic institutions in the United States, Ireland and Australia. While the primary objective is to assess safety, additional endpoints include overall response rate, PK, biomarkers of activity, time to response, durability of response and overall survival at six and 12 months.

The trial consists of two parts. In the Phase 1 portion, dose escalation testing is being performed to determine a recommended GMI-1271 dose in combination with standard chemotherapy. The Phase 1 dose-escalation portion of the trial is continuing. In the Phase 2 portion, dose-expansion testing will be conducted to obtain additional safety and efficacy data in defined sub-populations of AML. GlycoMimetics plans to enroll a total of approximately 75 patients in the trial.

About GMI-1271

GlycoMimetics is evaluating GMI-1271, a selective, potent, inhibitor of E-selectin, as an adjunct to standard-of-care chemotherapy, such as anthracycline and cytarabine-based combinations, in adults with AML as a targeted approach to disrupting well-established mechanisms of leukemic cell resistance within the bone marrow microenvironment.

In preclinical studies using animal models of AML, the results of which were presented at meetings of the American Society of Hematology (ASH), administration of GMI-1271 in combination with chemotherapy resulted in improved survival when compared to treatment with chemotherapy alone. In additional preclinical studies also presented at meetings of ASH, GMI-1271 was also associated with a reduction of chemotherapy-induced neutropenia and chemotherapy induced mucositis.

In other preclinical studies from which data was presented at the 2015 ASH annual meeting, GMI-1271 was observed to reverse molecular mechanisms of chemotherapy resistance seen in multiple myeloma. GlycoMimetics has initiated preparations for a Phase 1 multiple dose-escalation clinical trial in defined populations of patients with multiple myeloma and plans to initiate the trial in the second half of 2016.

GlycoMimetics owns all of the development and commercialization rights to GMI-1271.

About AML

AML is a cancer of the blood and bone marrow. AML is the most common type of acute leukemia in adults and the five-year survival rate is approximately 24 percent. According to the Surveillance, Epidemiology, and End Results Program managed by the National Cancer Institute, there were an estimated 20,830 new cases of AML diagnosed in 2015 in the United States and AML caused an estimated 10,460 deaths in 2015 in the United States. 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 and as a result, impede normal blood cell production. While leukemic cells move into the blood, 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 include chemotherapy and stem cell transplantation, both of which can destroy cancer cells, but remission rates remain low and relapsed disease remains a significant problem.

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. 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 and the presentation of clinical data. 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 February 29, 2016, 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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