GlycoMimetics Enrolls First Patient in Global Phase 3 Clinical Trial of Uproleselan in Relapsed/Refractory Acute Myeloid Leukemia

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- *Pivotal Trial to Evaluate Efficacy and Safety of Uproleselan in Patients with Advanced Acute Myeloid Leukemia (AML); Trial to Enroll 380 Patients at US and International Sites*
- *Broad Late-stage Development Program in Place to Evaluate Uproleselan Across the Continuum of Care in AML*

ROCKVILLE, Md.--(BUSINESS WIRE)--Nov. 19, 2018-- GlycoMimetics, Inc. (NASDAQ: GLYC) announced today dosing of the first patient in the company's Phase 3 clinical trial of uproleselan (GMI-1271) in relapsed/refractory AML. The trial's protocol provides for administration of the investigational drug in combination with MEC (mitoxantrone, etoposide and ara-C) or in combination with FAI (fludarabine, ara-C and idarubicin), both of which are standard of care for this indication in the United States. In 2017, the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation to GlycoMimetics for uproleselan in this patient population. The company anticipates the initiation of two complementary Phase 2/3 trials from two leading clinical consortia in early 2019.

"The dosing of the first patient in our pivotal Phase 3 trial for uproleselan is an important milestone for GlycoMimetics," said Helen Thackray, M.D., FAAP, Senior Vice President, Clinical Development, and Chief Medical Officer of GlycoMimetics. "This is a rigorously designed Phase 3 trial that has the potential to bring us one step closer to meeting the significant unmet needs of individuals living with relapsed/refractory AML. The trial is an important component of our comprehensive late-stage development program for uproleselan that positions us to evaluate the use of our product candidate across the spectrum of AML. It is the first of three randomized, controlled trials for uproleselan in AML, which we believe should provide clear efficacy and safety outcome measures in each of the settings being assessed."

"Our clinical development pipeline sets us up for multiple, value-creating clinical data readouts," added Rachel K. King, GlycoMimetics Chief Executive Officer. "During 2019, we anticipate topline data from the Phase 3 study of rivipansel being conducted by Pfizer in patients with sickle cell disease. Then, beginning at the end of 2020, we expect to generate topline data from the several trials that we will have underway in AML."

GlycoMimetics plans to enroll approximately 380 adult patients worldwide in the single pivotal randomized, double-blind, placebo-controlled Phase 3 AML trial, which is designed to align with guidance received from regulatory agencies. The primary endpoint is overall survival, and censoring for transplant in the primary efficacy analysis will not be required. Key secondary endpoints include incidence of severe mucositis and remission rate, which will be assessed in a hierarchical fashion for potential inclusion in the product labeling. The majority of the study sites will be in the United States, with meaningful participation from leading clinical centers in other strategic countries to support regulatory filings in major markets.

More information on the clinical trial can be found at www.clinicaltrials.gov.

About Uproleselan (GMI-1271)

Uproleselan (yoo' pro le'sel an) is designed to block E-selectin (an adhesion molecule on cells in the bone marrow) from binding with blood cancer cells as a targeted approach to disrupting well-established mechanisms of leukemic cell resistance within the bone marrow microenvironment. In a Phase 1/2 clinical trial, uproleselan was evaluated in both newly diagnosed elderly and relapsed/refractory patients with AML. In both populations, patients treated with uproleselan together with standard chemotherapy achieved better than expected remission rates and overall survival, as well as lower than expected induction-related mortality rates, as compared to historical controls which have been derived from results from third party clinical trials evaluating standard chemotherapy The U.S. Food and Drug Administration (FDA) has granted uproleselan Breakthrough Therapy Designation for the treatment of adult AML patients with relapsed/refractory (R/R) disease. GlycoMimetics is currently implementing a comprehensive development program across the clinical spectrum of AML. This includes a company sponsored Phase 3 trial in R/R AML and two consortia-sponsored trials in newly diagnosed patients. One consortium trial is being sponsored by the NCI and will enroll newly diagnosed patients fit for intensive chemotherapy. The other trial is sponsored by the HOVON group in Europe and will enroll newly diagnosed patients unfit for intensive chemotherapy.

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' 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, uproleselan, an E-selectin antagonist, was evaluated in a Phase 1/2 clinical trial as a potential treatment for AML and is currently being evaluated in a company sponsored Phase 3 trial in relapsed/refractory AML. The FDA granted uproleselan Breakthrough Therapy Designation for the treatment of adult acute myeloid leukemia (AML) patients with relapsed/refractory disease. GlycoMimetics has also completed a Phase 1 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.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements regarding the clinical development of the company's drug candidates, including the expected enrollment in and conduct of clinical trials, the presentation of clinical data, and expiration of issued patents. 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 filed with the U.S. Securities and Exchange

Commission (SEC) on March 6, 2018, 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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