



**GlycoMimetics, Inc.**

**Innovation Today, Healing Tomorrow.**

## **GlycoMimetics Announces Design of Phase 3 Clinical Trial for GMI-1271 in Relapsed/Refractory AML**

March 5, 2018

- *Primary endpoint for single pivotal clinical trial will be overall survival*
- *Top-line data expected Q4 2020*
- *Multiple clinical readouts planned starting year-end 2018 and through 2019 and 2020*
- *Details on Phase 3 development plan tomorrow as part of YE financial results conference call: scheduled for 8:30 a.m., March 6, dial-in and webcast details below*

ROCKVILLE, Md.--(BUSINESS WIRE)--Mar. 5, 2018-- GlycoMimetics, Inc. (NASDAQ: GLYC) announced today its design for a randomized, double-blind, placebo-controlled Phase 3 clinical trial to evaluate GMI-1271 in combination with MEC (Mitoxantrone, etoposide and Ara-C) or in combination with FAI (fludarabine, cytosine arabinoside and idarubicin) in individuals with relapsed/refractory acute myeloid leukemia (AML). The design is aligned with guidance received from the U.S. Food and Drug Administration (FDA). The single pivotal trial is planned to enroll 380 adult patients worldwide and is expected to begin in the third quarter of 2018. The primary endpoint will be overall survival, and censoring for transplant in the primary efficacy analysis will not be required. Key secondary endpoints will include incidence of severe mucositis and remission rate, which will be assessed in a hierarchical fashion for potential inclusion in the product labeling, if GMI-1271 is approved by the FDA. In 2017, GMI-1271 received Breakthrough Therapy Designation.

"Reaching alignment with the FDA on overall survival as the primary endpoint for the trial, without statistical censoring for transplant, positions GMI-1271 well for a potential successful outcome," said Rachel King, Chief Executive Officer of GlycoMimetics. "Getting more patients to transplant following treatment with GMI-1271 is one of our goals for this therapy. If we accomplish this, we hope GMI-1271 will contribute to prolonged overall survival for relapsed/refractory AML patients. We believe 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 this patient population. In addition, we believe that our trial design should streamline the path to data on overall survival, considered the 'gold standard' of clinical benefit, and that if this primary endpoint is achieved, it should position GMI-1271 optimally with U.S. and European regulatory agencies, as well as in the marketplace."

"Our development strategy now sets us up for multiple, value-creating clinical data readouts, the first of which is topline data from the ongoing Phase 3 trial of rivipansel in sickle cell disease in the second half of 2018," Ms. King added. "In early 2019, we anticipate topline data from our proof-of-concept trial of GMI-1271 in multiple myeloma, and now, by the end of 2020, we expect to have topline data from our pivotal trial of GMI-1271 in patients with relapsed/refractory AML."

Additional details regarding the Phase 3 trial will be provided in the company's fourth quarter and fiscal year 2017 financial results teleconference on Tuesday, March 6, 2018, at 8:30 a.m. ET. The dial-in number for the conference call is (844) 413-7154 for domestic participants and (216) 562-0466 for international participants, with participant code 1453008. A webcast replay will be available via the "Investors" tab on the GlycoMimetics website for 30 days following the call. A dial-in phone replay will be available for 24 hours after the close of the call by dialing (855) 859-2056 for domestic participants and (404) 537-3406 for international participants, participant code 1453008.

### **About GMI-1271**

GMI-1271 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, GMI-1271 was evaluated in both newly diagnosed elderly and relapsed/refractory patients with acute myeloid leukemia (AML). In both populations, patients treated with GMI-1271 together with standard chemotherapy achieved better than expected remission rates and overall survival compared to historical controls, as well as lower than expected induction-related mortality rates. Treatment in this patient population was well tolerated, with minimal adverse 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. 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, 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 Phase 1 clinical trial for the treatment of multiple myeloma. In 2017, the FDA granted GMI-1271 Breakthrough Therapy designation for the treatment of adult AML patients with relapsed/refractory disease. GMI-1359, a combined CXCR4 and E-selectin antagonist in the GlycoMimetics pipeline, is currently in Phase 1 testing in healthy volunteers. GlycoMimetics is located in Rockville, MD in the BioHealth Capital Region. Learn more at [www.glycomimetics.com](http://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 timing of completion of clinical trials 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 to be filed with the U.S. Securities and Exchange Commission (SEC) on or about 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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