



GlycoMimetics, Inc.

Innovation Today, Healing Tomorrow.

GlycoMimetics to Present Analyses from Phase 1/2 AML Trial of Uproleselan at 60th ASH Meeting

November 1, 2018

- *New and updated clinical outcomes data and subgroup analyses continue to demonstrate potential benefit of treatment with uproleselan when added to chemotherapy*
- *69 percent (11 out of 16) of evaluable patients with relapsed/refractory (R/R) acute myeloid leukemia (AML) and 56 percent (5 out of 9) of evaluable patients with newly diagnosed AML achieved MRD-negative remission in clinical trial*
- *Correlation of E-selectin ligand expression on leukemic blasts and leukemic stem cells (LSCs), as well as clinical data showing that E-selectin ligand expression on leukemic blasts is associated with poor outcomes in patients with AML, also support clinical trial program*

ROCKVILLE, Md.--(BUSINESS WIRE)--Nov. 1, 2018-- GlycoMimetics (NASDAQ: GLYC) announced today that six abstracts covering data from the company's research and clinical portfolio have been accepted for presentation at the 60th American Society of Hematology (ASH) Annual Meeting and Exposition to be held December 1-4, 2018 in San Diego.

Of particular note, the final analysis from the recently completed Phase 1/2 AML trial of uproleselan, an E-selectin antagonist and the company's lead wholly owned clinical candidate, has been selected for an oral presentation on Sunday, December 2 during a platform session on novel therapies in AML. Clinical data to be presented include analyses of measurable residual disease (MRD), analyses of response in high-risk subgroups, and updated event-free and overall survival data, which continue to compare favorably with matched historical controls. New data will also be presented showing a correlation between E-selectin ligand expression on leukemic blasts and leukemic stem cells (LSCs), supporting the premise that binding to E-selectin is a mechanism of resistance in AML.

In addition, a poster presentation of complementary work by investigators at the Fred Hutchinson Cancer Research Center will highlight new clinical data showing E-selectin ligand expression on leukemic blasts is associated with poor outcomes in patients at their center with AML. The combined clinical dataset from these two studies provides further scientific rationale for the potential value of uproleselan as a selective, bone-marrow microenvironment-disrupting agent in patients with AML.

"Our oral presentation at ASH is noteworthy in that we show for the first time that the majority of evaluable patients (11 out of 16) in the R/R cohort and more than half of evaluable patients (5 out of 9) in the newly diagnosed cohort achieved measurable residual disease negativity as assessed by either flow and/or RT-PCR, when uproleselan is added to a standard chemotherapy regimen," noted Helen Thackray, M.D., FAAP, GlycoMimetics Senior Vice President, Clinical Development and Chief Medical Officer. "Furthermore, we now have an updated final analysis of survival that reflects longer term follow up and fewer patients' data censored, and these data also continue to compare very favorably to historical studies of matched patient groups. As we initiate sites for our Phase 3 study, the ASH data on the addition of uproleselan to chemotherapy, namely improved remission rates, low mucositis rates, high MRD and transplant rates – as well as promising survival – give us greater confidence in the opportunity to provide a potentially transformative regimen for not only patients with R/R AML but also for the newly diagnosed patients."

According to the Phase 1/2 trial's lead investigator Daniel J. DeAngelo, M.D., Ph.D., Director of Clinical and Translational Research, Adult Leukemia Program, at the Dana-Farber Cancer Institute and Brigham and Women's Hospital, "Two of the most striking findings from this study are the number of patients negative for measurable residual disease and the low rate of mucositis. These two potential benefits of the candidate drug, combined with the strong survival signals, distinguish uproleselan from other agents in development. These potential benefits clearly underscore the mechanism of action of uproleselan and, importantly, point to the opportunity we have to help change the face of this disease."

Featured GlycoMimetics' ASH data include the following:

- **R/R AML Cohort:** At the recommended Phase 2 dose (RP2D), CR (complete response)/CRi (complete remission with incomplete blood count recovery) rate was 41%, median overall survival was 8.8 months (95% CI 5.7-11.4) and 69% of evaluable patients (11/16) achieved measurable residual disease negativity as assessed by either flow and/or RT-PCR. Overall survival (OS) will be the primary outcome measure in the company's Phase 3 trial in R/R AML patients, and the data reported today compares to 5.2-5.4 months OS in comparable historical controls. ^{1,2} (Abstract #331)
- **Newly Diagnosed (ND) AML Cohort:** At the RP2D, CR/CRi rate was 72%, median overall survival was 12.6 months (95% CI 9.9-not reached), Event Free Survival (EFS) was 9.2 months (3.0-12.6) and 56% of evaluable patients (5 out of 9) achieved measurable residual disease negativity as assessed by either flow and/or RT-PCR. EFS will be the primary outcome measure for the interim analysis in the National Cancer Institute clinical trial in newly diagnosed patients, and the data presented today compare to 2-6.5 months for EFS in historical controls which represent lower risk patient populations than those treated in our study. ^{3,4} (Abstract #331)
- Based on data from 89 serially acquired AML patient samples at the Fred Hutchinson Cancer Research Center, mean fluorescence intensity of E-selectin-Fc binding is four-fold higher for R/R patients than for newly diagnosed patients (p=0.0026), suggesting that sequestration in the vascular niche of the bone marrow is associated with chemotherapy resistance. (Abstract #1513)

Other GlycoMimetics ASH presentations will showcase the potential use of uproleselan to mobilize hematopoietic stem cells with increased reconstitution potential, as well as highlight two new novel compounds discovered by GlycoMimetics that are for the first time being presented to the hematology community. Specifically, Abstract 4678 demonstrates that a highly-potent antagonist of E-selectin, GMI-1687, shows equivalent activity to uproleselan, but at 250-fold lower dose and via subcutaneous administration. In addition, GlycoMimetics researchers will also present preclinical data for a novel dual-function galectin-3 / E-selectin inhibitor, GMI-1757, in a well-established model of venous thrombosis.

Presentation details:

Publication Number: 331

TITLE: Uproleselan (GMI-1271), an E-Selectin Antagonist, Improves the Efficacy and Safety of Chemotherapy in Relapsed/Refractory (R/R) and Newly Diagnosed Older Patients with Acute Myeloid Leukemia: Final, Correlative and Subgroup Analyses

Session Name: 616. Acute Myeloid Leukemia: Novel Therapy, excluding Transplantation: Combination Therapy

Session Date: Sunday, December 2, 2018

Session Time: 9:30 AM - 11:00 AM PST

Presentation Time: 9:30 AM

Room: Manchester Grand Hyatt San Diego, Grand Hall B

Publication Number: 1513

TITLE: E-Selectin Ligand Expression by Leukemic Blasts Is Associated with Prognosis in Patients with AML

Session Name: 617. Acute Myeloid Leukemia: Biology, Cytogenetics, and Molecular Markers in Diagnosis and Prognosis: Poster I

Session Date: Saturday, December 1, 2018

Presentation Time: 6:15 PM - 8:15 PM PST

Location: San Diego Convention Center, Hall GH

Publication Number: 2211

TITLE: A Novel Glycomimetic Compound (GMI-1757) with Dual Functional Antagonism to E-Selectin and Galectin-3 Demonstrates Inhibition of Thrombus Formation in an Inferior Vena Cava Model

Session Name: 802. Chemical Biology and Experimental Therapeutics: Poster I

Session Date: Saturday, December 1, 2018

Presentation Time: 6:15 PM - 8:15 PM PST

Location: San Diego Convention Center, Hall GH

Publication Number: 3846

TITLE: The Vascular Bone Marrow Niche Influences Outcome in Chronic Myeloid Leukemia

Session Name: 506. Hematopoiesis and Stem Cells: Microenvironment, Cell Adhesion, and Stromal Stem Cells: Poster III

Session Date: Monday, December 3, 2018

Presentation Time: 6:00 PM - 8:00 PM PST

Location: San Diego Convention Center, Hall GH

Publication Number: 4552

TITLE: Vascular E-Selectin Acts as a Gatekeeper Inducing Commitment and Loss of Self-Renewal in HSC Transmigrating through the Marrow Vasculature

Session Name: 711. Cell Collection and Processing: Poster III

Session Date: Monday, December 3, 2018

Presentation Time: 6:00 PM - 8:00 PM PST

Location: San Diego Convention Center, Hall GH

Publication Number: 4678

TITLE: A Novel and Potent Inhibitor of E-Selectin, GMI-1687, Attenuates Thrombus Formation and Augments Chemotherapeutic Intervention of AML in Preclinical Models Following Subcutaneous Administration

Session Name: 802. Chemical Biology and Experimental Therapeutics: Poster III

Session Date: Monday, December 3, 2018

Presentation Time: 6:00 PM - 8:00 PM PST

Location: San Diego Convention Center, Hall G

Meeting abstracts are available on [ASH's website](#).

¹ Feldman et al, *J Clin Oncol*. 2005 Jun 20;23(18):4110-6.

² Greenberg et al, *J Clin Oncol*. 2004 Mar 15;22(6):1078-86.

³ Lowenberg et al, *N Engl J Med*. 2009 Sep 24;361(13).

⁴ Lancet et al, *Blood*. 2014 May 22;123(21):3239-46.

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 compared to historical controls, which have been derived from results from third party clinical trials evaluating standard chemotherapy, as well as lower than expected induction-related mortality rates. Treatment in these patient populations was generally well tolerated, with fewer than expected adverse effects. 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, as well in two consortia sponsored trials in newly diagnosed 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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Source: GlycoMimetics, Inc.

GlycoMimetics, Inc.

Investor Contact:

Shari Annes, 650-888-0902

sannes@annesassociates.com

or

Media Contact:

Jamie Lacey-Moreira, 410-299-3310

jamielacey@presscommpr.com