GlycoMimetics Presents New AML Data with Uproleselan at 60th ASH Annual Meeting

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- *Data in relapsed/refractory and newly diagnosed acute myeloid leukemia (AML) patients underscore clinical opportunities across multiple outcomes measures and subgroups*
- *Selective disruption of bone marrow microenvironment with uproleselan resulting in high remission rates; majority of evaluable patients achieving stringent level of measurable residual disease (MRD) negativity; promising survival outcomes across all AML subgroups assessed*
- *Correlative studies further strengthen scientific rationale for inhibiting E-selectin in patients with AML, particularly those individuals with high-risk disease*

ROCKVILLE, Md.--(BUSINESS WIRE)--Dec. 3, 2018-- GlycoMimetics, Inc. (NASDAQ:GLYC) announced today that new data on uproleselan-treated high risk patients with both relapsed/refractory and newly diagnosed AML were presented at an oral session during the 60th American Society of Hematology (ASH) Annual Meeting and Exposition. An analysis of clinical outcomes from the Phase 1/2 clinical study showed that uproleselan (GMI-1271) resulted in the majority of evaluable patients achieving a stringent level of measurable residual disease (MRD) negativity, an effect which translated into extended overall survival relative to matched, historical controls.[1-4]

Additionally, an analysis of E-selectin ligand expression on leukemic cells demonstrated that detectable levels are present in every patient tested, providing strong clinical evidence of biological relevance in this disease setting. In bone marrow blasts, leukemic stem cell expression of E-selectin ligand correlated with leukemic blast E-selectin ligand expression ($p<0.0001$), consistent with the hypothesis that E-selectin-mediated interactions are a mechanism of chemoresistance. Additionally, investigators assessed the association between baseline E-selectin ligand expression and clinical outcomes using a log-rank test. In the R/R cohort of patients (n=22), this analysis demonstrated that ≥10% E-selectin ligand expression at baseline is correlated with prolonged survival ($p<0.01$) for patients treated with uproleselan. This observation is important since separately Chien et al. (Abstract 1513) report that high levels of E-selectin ligand in patients not treated with uproleselan correlate with worse clinical prognosis.

“The new MRD and correlative efficacy data in difficult-to-treat patients, when combined with the already encouraging response rate and survival results from this trial, further demonstrate the potential of uproleselan to be an important new treatment option in AML,” said Daniel J. DeAngelo, M.D., Ph.D., Chief of the Division of Leukemia at the Dana-Farber Cancer Institute and Brigham and Women’s Hospital, and the trial’s lead investigator. “The fact that more than half of the evaluable patients achieved a stringent level of MRD negativity is particularly noteworthy as uproleselan’s mechanism of action is to selectively disrupt the relationship between leukemic cells and the bone marrow microenvironment.”

“It is now clearly established that patients with AML who express E-selectin ligands on their leukemic cells have more infiltrative, aggressive disease and significantly worse clinical outcomes when not treated with uproleselan,” said Helen Thackray, M.D., FAAP, GlycoMimetics Senior Vice President, Clinical Development and Chief Medical Officer. “While we would expect patients with R/R AML and >10% E-selectin ligand expression on their leukemic blasts to do very poorly, it is extremely exciting to see that the addition of uproleselan is resulting in statistically significant improvements in clinical outcomes in these high-risk patients. This is completely counterintuitive to what you would expect and provides robust scientific evidence suggesting that uproleselan is exerting biologic activity.”

The ASH presentations referenced above include:

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

Publication Number: 1513
TITLE: E-Selectin Ligand Expression by Leukemic Blasts Is Associated with Prognosis in Patients with AML

References

[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. The U.S. Food and Drug Administration granted uproleselan Breakthrough Therapy designation for the treatment of adult 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.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the clinical development and potential utility of the company's drug candidates. 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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