



GlycoMimetics Reports Improvements in Median Overall Survival Versus Historical Matched Controls for Two AML Patient Subgroups in Phase 1/2 Trial for GMI-1271

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- *Clinical outcomes, including durability of remission and median overall survival, in high-risk patients plus safety and tolerability data presented in oral presentation at American Society of Hematology Annual Meeting and Expo*
- *Second oral presentation highlights underlying, differentiated mechanism of action for company's lead clinical candidate*

ROCKVILLE, Md.--(BUSINESS WIRE)--Dec. 12, 2017-- GlycoMimetics, Inc. (NASDAQ: GLYC) today announced updated data from the Phase 1/2 trial evaluating the safety, tolerability and efficacy of GMI-1271 in patients with relapsed/refractory (R/R) acute myeloid leukemia (AML) and in older adults with newly diagnosed AML, including the following conclusions:

For patients with R/R AML treated at the Phase 2 dose (n = 54) and for whom median follow up was 6.6 months:

- Clinical remission (CR+CRi) was 43%.
- Median overall survival was 9.4 months (95% CI: 5.7 – 15.1 months; calculated by Kaplan Meier method). This compares favorably to a median overall survival of up to 5.4 months reported for historical, matched controls treated with mitoxantrone, etoposide and cytarabine (MEC) alone.^{1,2}
- Median duration of remission was 11.1 months (95% CI: 5.8-NA; calculated by Kaplan Meier method).

For older patients with newly diagnosed disease (n=25) and for whom median follow up was 10.5 months:

- Clinical remission rate was 68%.
- Median overall survival was 15.8 months (95% CI: 10.3 – NA; calculated by Kaplan Meier method). This compares favorably to a historical median overall survival of approximately 12 months in matched controls treated with 7+3 chemotherapy alone.^{3,4}
- Median duration of remission was 14.8 months (95% CI: 8.3 – NA; calculated by Kaplan Meier method).
- Median event free survival was 11.3 months.

The data were presented yesterday during an oral scientific session at the 59th American Society of Hematology (ASH) Annual Meeting and Expo in Atlanta.

Across both populations, GMI-1271 was well tolerated with no obvious incremental toxicity observed and lower than expected rates of severe, debilitating, grade 3-4 mucositis reported (e.g., 3% incidence reported vs. historical 20-25% incidence with MEC alone).

"These new data from our Phase 1/2 clinical trial demonstrate that encouraging clinical outcomes are possible for both duration of remission and survival endpoints when GMI-1271 is added to chemotherapy in two distinct AML patient populations," noted [Helen Thackray](#), M.D., FAAP, GlycoMimetics Senior Vice-President, Clinical Development and Chief Medical Officer. "Beyond the high response rates previously reported with GMI-1271, we can now point to additional long-term endpoints that further support our plan to move the drug candidate into a Phase 3 clinical trial scheduled to begin in mid-2018. Importantly, with respect to safety, the low mucositis rate in relapsed and refractory patients receiving MEC induction chemotherapy -- where you would expect around 25% severe mucositis -- is quite striking. This was predicted and explained by preclinical models in which GMI-1271 blocked inflammatory macrophages trafficking to the gut and thus prevented mucosal injury."

"These results continue to show that AML patients treated with GMI-1271 consistently perform better than expected," said Daniel J. DeAngelo, M.D., Ph.D., the trial's lead investigator and Director of Clinical and Translational Research, Adult Leukemia Program, at the Dana-Farber Cancer Institute and Brigham and Women's Hospital, who presented the data at the ASH Annual Meeting. "Our Phase 2 population consists of very high-risk patients based on age, disease status, and cytogenetic risk factors. The updated data continue to support the concept that disrupting the relationship between leukemic cells and the protective bone marrow microenvironment, when combined with chemotherapy, could improve the outlook and prognosis for these patients."

The second oral presentation at the ASH meeting highlighted a preclinical study in murine models of AML in which E-selectin was shown to be upregulated, and AML cells binding to E-selectin increased chemo-resistance by activating specific tumor cell survival signaling pathways. This effect within the bone marrow microenvironment is unique to E-selectin as compared to other vascular adhesion molecules and can be blocked by GMI-1271. This translational research provides important evidence that elucidates how treatment with GMI-1271 appears to be improving sensitivity to chemotherapy.

"Given response rates we've observed to date that suggest clinical benefit in combination with chemotherapy in two AML populations, this preclinical work provides important further support for the mechanism of action of GMI-1271," noted Dr. Thackray. "Together, the clinical and preclinical data we have shared at the ASH Annual Meeting demonstrate that GMI-1271 could represent a novel and truly differentiated approach to treatment of AML," Dr. Thackray concluded.

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

GlycoMimetics to Hold Post-ASH Meeting Briefing in Boston on December 19

GlycoMimetics will hold a briefing for investors/analysts, which will also be available via webcast, to review the GMI-1271 program with a focus on the AML clinical data presented at the ASH Annual Meeting, at the Langham Hotel in Boston, December 19, at 7:30 a.m. EST. Dr. DeAngelo will present the clinical data from the ASH oral presentation and respond to questions from on-site participants.

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' first 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, a specific E-selectin antagonist, has been evaluated in a Phase 1/2 clinical trial as a potential treatment for AML and is currently being evaluated in an ongoing Phase 1 clinical trial for the treatment of multiple myeloma. The U.S. Food and Drug Administration has granted GMI-1271 Breakthrough Therapy designation for the treatment of adult AML patients with relapsed/refractory disease. GlycoMimetics has also conducted 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 GMI-1271, including the expected timing 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 that was filed with the U.S. Securities and Exchange Commission (SEC) on March 1, 2017, 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.

References

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