GlycoMimetics Announces U.S. Food and Drug Administration Clears Addition of Time-based Final Analysis to Pivotal Phase 3 Study of Uproleselan in Relapsed/Refractory Acute Myeloid Leukemia

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- Final analysis to be conducted after the earlier of 295 survival events or the FDA-aligned cutoff date; topline results expected by the end of Q2 2024
- Prolonged duration of blinded pooled survival in relapsed/refractory Acute Myeloid Leukemia expected to yield clinically mature dataset in Q2 2024, supporting addition of time-based analysis of overall survival primary endpoint
- Dataset is expected to reflect greater than three years of median follow-up, including at least two years of post-transplant data for a large majority of patients who received stem cell transplantation

ROCKVILLE, Md.--(BUSINESS WIRE)--Jun. 15, 2023-- GlycoMimetics, Inc. (Nasdaq: GLYC) a late clinical-stage biotechnology company discovering and developing glycobiology-based therapies for cancers and inflammatory diseases, today announced the U.S. Food and Drug Administration (FDA) cleared the addition of a protocol amendment to the company's pivotal Phase 3 study of uproleselan for relapsed/refractory (R/R) Acute Myeloid Leukemia (AML). The amendment will allow a time-based analysis of the primary endpoint of overall survival to be conducted following a defined cutoff date if the 295 survival events originally planned for an event driven analysis have not been observed. With the addition of a time-based analysis, topline results are expected to be reported by the end Q2 2024.

"We are pleased the FDA cleared the addition of a time-based pathway to final analysis as it provides the opportunity to evaluate the effect of uproleselan on R/R AML based on a clinically mature database with more than three years median follow-up," said Harout Semerjian, Chief Executive Officer of GlycoMimetics. "This approach is consistent with regulatory precedent adopted for a prior pivotal AML study for an approved drug and reflects our commitment to science-driven analysis as we seek to deliver uproleselan to R/R AML patients in need of new treatment options as soon as possible."

The Phase 3 trial completed enrollment of 388 patients across 70 sites in nine countries in November 2021. An event based final analysis of overall survival was previously expected after year end 2022, but the number of events has slowed, resulting in the projected timeline being extended.

Based on blinded pooled data observed to date, a time-based final analysis in Q2 2024 is expected to yield a clinically mature dataset to evaluate uproleselan in R/R AML. This dataset is expected to reflect a median follow-up of greater than three years, including at least two years of post-transplant data. The majority of surviving patients in the study received hematopoietic stem cell transplantation (HSCT). As a result, the company believes the capture rate of survival events for this study beyond Q2 2024 would provide limited additional value for the primary analysis.

Two years post-transplant is generally considered an important milestone in AML because most patients who survive at least two years post-transplant without experiencing disease relapse are typically deemed to be long-term survivors. For patients over two years post-transplant, disease relapse is less likely.[1]

As part of the protocol amendment, the FDA also cleared the addition of landmark EFS and overall survival analyses as secondary endpoints.

About Uproleselan

Discovered and developed by GlycoMimetics, uproleselan is an investigational first-in-class, E-selectin antagonist. Uproleselan (yoo' pro le'se lan), currently in a comprehensive Phase 3 development program in acute myeloid leukemia (AML), has received Breakthrough Therapy and Fast Track designations from the U.S. FDA and Breakthrough Therapy designation from the Chinese National Medical Products Administration for the treatment of adult AML patients with relapsed or refractory disease. Uproleselan is designed to block E-selectin binding and stimulation of myeloid cells. E-selectin is expressed on the surface of blood vessels, and its binding to myeloid cells confers a pro-survival effect. Uproleselan intends to provide a novel approach to disrupting established mechanisms of leukemic cell resistance.

About GlycoMimetics, Inc.

GlycoMimetics is a late clinical-stage biotechnology company discovering and developing glycobiology-based therapies for cancers, including AML, and for inflammatory diseases with high unmet needs. The company's science is based on an understanding of the role that carbohydrates play in cell recognition. Its specialized chemistry platform is deployed to discover small molecule drugs, known as glycomimetics, which alter carbohydrate-mediated recognition in diverse disease states, including cancer and inflammation. As a leader in this science, GlycoMimetics leverages this unique approach to advance its pipeline of wholly-owned drug candidates, with the goal of developing transformative therapies for diseases with high unmet need. GlycoMimetics is headquartered in Rockville, MD in the BioHealth Capital Region. Learn more at www.glycomimetics.com.

Forward-Looking Statements

This press release contains forward-looking statements. These forward-looking statements may include, but are not limited to, statements regarding the conduct of and data from clinical trials, planned or potential clinical development, regulatory interactions and submissions, and the potential benefits and impact of the company's drug candidate, uproleselan. Actual results may differ materially from those described 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 29, 2023, 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.

[1] Bolon YT, Atshan R, Allbee-Johnson M, Estrada-Merly N, Lee SJ. Current use and outcome of hematopoietic stem cell transplantation: CIBMTR summary slides (slide 79), 2022.

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