GlycoMimetics Announces Comprehensive Results from Pivotal Phase 3 Study of Uproleselan in Relapsed/Refractory (R/R) Acute Myeloid Leukemia (AML)

June 4, 2024 at 7:00 AM EDT

- Company exploring path forward for uproleselan in multiple AML settings based on observed efficacy results, including clinically meaningful results in primary refractory AML, and significant unmet patient need
- Uproleselan demonstrated a clinically meaningful improvement in median overall survival (mOS) for patients with primary refractory AML; mOS was 31.2 months for the uproleselan arm compared to 10.1 months for the placebo arm in this subgroup
- Adverse events for uproleselan were consistent with known side effect profiles of chemotherapy used in the study
- Advancing discussions with the National Cancer Institute (NCI) and the Alliance for Clinical Trials in Oncology for Phase 2/3 study of uproleselan with chemotherapy in older adults with frontline AML
- Conference call and webcast to be hosted today, June 4, 2024, at 8:30 am E.T.

ROCKVILLE, Md.--(BUSINESS WIRE)--Jun. 4, 2024-- GlycoMimetics, Inc. (Nasdaq: GLYC), a late clinical-stage biotechnology company discovering and developing glycobiology-based therapies for cancers and inflammatory diseases, today announced comprehensive results from the company's pivotal Phase 3 study of uproleselan in R/R AML.

“There is a wealth of data across large subsets of this pivotal Phase 3 study that help us understand how prespecified stratification factors such as backbone chemotherapy, disease status, and age impacted survival outcomes for patients,” said Daniel DeAngelo, M.D., Ph.D., Professor of Medicine, Harvard Medical School, Chief, Division of Leukemia, Dana-Farber Cancer Institute, and Principal Investigator of the pivotal Phase 3 study. “In the primary refractory setting, uproleselan’s improvement of mOS and greater duration of remission were particularly compelling, as there is a significant unmet need for new treatment options in this setting that can extend and improve the lives of patients. These results demonstrate uproleselan has the potential to address this unmet need in primary refractory AML.”

“As we have analyzed data from this large, well-balanced, and well-executed study alongside medical, statistical, and regulatory experts, it has become clear that uproleselan may offer clinically meaningful patient benefit in multiple settings, including primary refractory AML,” said Harout Semerjian, Chief Executive Officer of GlycoMimetics. “We are committed to addressing unmet needs of AML patients and plan to engage with regulators and NCI to discuss potential paths forward for uproleselan.”

Results of Pivotal Phase 3 Study of Uproleselan in R/R AML

The randomized, double-blind, placebo-controlled Phase 3 clinical study evaluated uproleselan in combination with MEC (mitoxantrone, etoposide and cytarabine) or FAI (fludarabine, cytarabine and idarubicin) in patients with R/R AML. Patients received either uproleselan or placebo for 8 days over 1 cycle of induction and, if applicable, up to 3 cycles of consolidation. The primary endpoint was overall survival (OS), which was not censored for transplant. Secondary endpoints included incidence of severe oral mucositis, complete remission (CR) rate and CR with partial hematologic recovery (CRh). A total of 388 patients in nine countries were randomized 1:1 between treatment and placebo arms. There were 59 sites that enrolled at least one patient. Median follow up was over three years at the time of primary analysis.

Overall Survival

- Primary Endpoint: mOS in the intent-to-treat (ITT) population (n=388) was 13.0 months for the uproleselan arm, compared to 12.3 months for the placebo arm (hazard ratio [HR] 0.89; 95% confidence interval [CI] 0.69-1.15); this difference is not statistically significant.
- Disease Status
    - Primary Refractory: mOS for primary refractory patients in the uproleselan arm (n=62) was 31.2 months, compared to 10.1 months (HR 0.58; 95% CI 0.37-0.91) for the placebo arm (n=66). This benefit was irrespective of backbone chemotherapy.
        - Median duration of response (DoR) for complete remission (CR) was not reached for primary refractory patients in the uproleselan arm compared to a median DoR of 12.7 months for the placebo arm.
    - Early Relapse: mOS for early relapse patients in the uproleselan arm (n=28) was 3.7 months, compared to 6.4 months (HR 1.50; 95% CI 0.69-3.27) for the placebo arm (n=22).
    - Late Relapse: mOS for late relapse patients in the uproleselan arm (n=104) was 15.4 months, compared to 18.2 months (HR 1.10; 95% CI 0.77-1.57) for the placebo arm (n=106).
- Backbone Chemotherapy:
    - FAI: mOS for patients treated with uproleselan plus FAI (n=98) was 30.2 months compared to 12.8 months (HR 0.73; 95% CI 0.50-1.06) for patients treated with FAI alone (n=96) in the ITT population.
    - MEC: mOS for patients treated with uproleselan plus MEC (n=96) was 8.7 months compared to 12.3 months (HR 1.06; 95% CI 0.75-1.51) for patients treated with MEC alone (n=98) in the ITT population.

- Transplantation Status:
    - For patients who received hematopoietic stem cell transplantation (HSCT) after study treatment, mOS was not reached for patients in the uproleselan arm (n=101). In contrast, for HSCT patients in the placebo arm, mOS for patients receiving FAI (n=53) was 26.3 months and for patients receiving MEC (n=46) was 24.4 months.

Secondary Endpoints

- 7.2% of patients in each arm (n=388) experienced induction emergent severe oral mucositis.
- 36.1% of patients in the uproleselan arm (n=194) experienced CR at the end of induction (EOI) as determined by an independent endpoint review committee (IERC), compared to 33.5% of patients in the placebo arm (n=194).
- 46.4% of patients in the uproleselan arm experienced CR/CRh at EOI as determined by IERC, compared to 41.2% of patients in the placebo arm.
- Post-treatment HSCT rate was 52.1% in the uproleselan arm and 51.0% in the placebo arm.
- Subsequent AML therapy in non-responders was 40.0% in the uproleselan arm (n=80) and 46.2% in the placebo arm (n=78).

Safety

- Adverse events were consistent with the known safety profile for backbone chemotherapy regimens.
- 35.9% of patients in the uproleselan arm experienced serious treatment-emergent adverse events (TEAEs) compared to 34.2% in the placebo arm.
- 85.9% of patients in the uproleselan arm experienced grade 3 or higher TEAEs compared to 87.6% in the placebo arm.

NCI Phase 2/3 Study of Uproleselan in Frontline AML

In addition to the company's pivotal Phase 3 trial of uproleselan, the National Cancer Institute (NCI) and the Alliance for Clinical Trials in Oncology are conducting an adaptive Phase 2/3 study of uproleselan in adults with newly diagnosed AML who are 60 years or older and fit for intensive chemotherapy. Their randomized, controlled study is evaluating the addition of uproleselan to a standard cytarabine / daunorubicin regimen (7+3) versus chemotherapy alone. The Phase 2 portion of the study completed enrollment of 267 patients in December 2021. The Company is advancing discussions with the NCI and the Alliance for Clinical Trials in Oncology based on the results of the pivotal Phase 3 study of uproleselan in R/R AML.

Conference Call Details

To access the call by phone, please go to this registration link and you will be provided with dial in details. Participants are encouraged to connect 15 minutes in advance of the scheduled start time.

A live webcast of the call and the corresponding slides will be available on the "Investors" tab on the GlycoMimetics website. A webcast replay will be available for 30 days following the call.

About AML

AML is the most common acute leukemia in adults. A cancer of the bone marrow, nearly 21,000 people in the United States are diagnosed with AML each year. Despite the availability of multiple treatments, disease prognosis is poor, and new treatment options are needed to improve outcomes. Newly diagnosed AML has the lowest 5-year survival rate of all leukemias at 31.7%. The five-year survival rate for people with relapsed/refractory disease is only 10%.

About Uproleselan

Discovered and developed by GlycoMimetics, uproleselan (yoo' pro le'se lan) is an investigational, first-in-class E-selectin antagonist. GlycoMimetics has received Breakthrough Therapy and Fast Track designations from the U.S. Food and Drug Administration (FDA) and Breakthrough Therapy designation from the Chinese National Medical Products Administration for uproleselan as a potential treatment for adult AML patients with relapsed or refractory disease. E-selectin is a leukocyte adhesion molecule constitutively expressed on endothelial cells of the vasculature and bone marrow. In AML, there is evidence that E-selectin–ligand interaction between endothelial cells in the protective niche of the Bone Marrow microEnvironment (BME) and leukemic stem cells and blasts promotes leukemic cell survival and hides them from AML therapies. Uproleselan is designed to disrupt E-selectin binding and prevent leukemic myeloid cells using the protective niche of the BME.

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. The company's scientific approach is based on an understanding of the role that carbohydrates play in cell recognition. Its specialized chemistry platform is being deployed to discover small molecule drugs, known as glycomimetics, that alter carbohydrate-mediated recognition in diverse disease states, including cancers and inflammation. GlycoMimetics is leveraging its differentiated expertise with this scientific approach in order to advance its pipeline of wholly owned drug candidates. The company's goal is to develop transformative therapies for diseases with high unmet medical 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 timing for analysis and presentation of data from, clinical trials; potential development and regulatory activities; and the potential benefits and impact of 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 27, 2024, the company’s Quarterly Report on Form 10-Q filed with the SEC on May 9, 2024, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20240604811237/en/

Investor Contact:
Argot Partners
Leo Vartorella
212-600-1902
Glycomimetics@argotpartners.com

Source: GlycoMimetics, Inc.