Efficacy and Safety Data for GlycoMimetics' Lead Investigational Drug Uproleselan Published in BLOOD

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Manuscript highlights MRD negative rate of 69% in the relapsed/refractory patient population

ROCKVILLE, Md.--(BUSINESS WIRE)--Sep. 23, 2021-- Efficacy and safety data from a Phase 1/2 clinical study of uproleselan, GlycoMimetics' lead investigational drug, were published online September 16, 2021 in the journal *BLOOD*. In the manuscript, scientists highlight an analysis of minimal residual disease (MRD) and report an MRD negative rate of 69 percent in trial participants with relapsed/refractory acute myeloid leukemia (AML), indicating an enhanced depth of response following addition of uproleselan to salvage therapy. The paper also confirms that uproleselan can be safely combined with an intensive salvage chemotherapy regimen without adding toxicity in both relapsed/refractory and in newly diagnosed older AML patients and builds upon results first reported at the 2018 ASH Meeting. The paper's lead author, Daniel J. DeAngelo, M.D., Ph.D., of the Dana Farber Cancer Institute in Boston, noted that "the combination of uproleselan with a standard salvage regimen of mitoxantrone, etoposide and cytarabine (MEC) demonstrated a substantial improvement in both response rate and survival in relapsed/refractory AML patients compared to previously reported results with chemotherapy alone."

"The results of this Phase 1/2 study demonstrate the safety and tolerability of uproleselan in combination with an intensive salvage chemotherapeutic regimen. The reported response rates and survival outcomes are superior to what has been seen with chemotherapy alone in similar relapsed/refractory AML patient populations," noted Eric J. Feldman, M.D., GlycoMimetics' Chief Medical Officer. "In particular, the MRD negative rate of 69 percent in the relapsed/refractory cohort is unprecedented in producing a high proportion of patients who underwent a successful transplant. We are confident that our ongoing Phase 3 randomized trial will confirm the efficacy seen in the Phase 1/2 study and will help to establish uproleselan in combination with intensive chemotherapy as the new standard of care in relapsed/refractory AML."

According to Dr. DeAngelo, principal investigator of the company's ongoing Phase 3 registrational trial, "We believe that uproleselan is clearly a novel and potent inhibitor of E-selectin that has been shown in the clinic to overcome chemotherapy resistance. Should the ongoing registrational trial prove positive, we will have created a foundational paradigm shift that has the potential to significantly impact outcomes for AML patients worldwide."

Key Data

- MRD was assessed by multi-parametric flow cytometry in the relapsed/refractory cohort. Of the 16 evaluable patients, 11 patients (69%) were MRD negative at the end of induction. Following treatment with MEC plus the recommended Phase 2 dose (RP2D) of uproleselan, 31% of patients (17/54) underwent allogeneic hematopoietic stem cell transplant. Of the 22 patients achieving CR/CRi, 11 (50%) underwent transplant.
- Median overall survival at the RP2D of 10 mg/kg in relapsed/refractory and newly diagnosed AML patients was 8.8 and 12.6 months, respectively.
- The addition of uproleselan was associated with low rates of oral mucositis.
- E-selectin-mediated drug resistance contributes to poor outcomes in patients with AML.
- E-selectin ligand expression on leukemic blasts was higher in patients with relapsed versus primary refractory AML, and with high-risk cytogenetics and secondary AML in newly diagnosed older patients. In the Relapsed/Refractory cohort, E-selectin expression above 10% was associated with a higher response rate and improved survival.

About Uproleselan

Discovered and developed by GlycoMimetics, uproleselan is an investigational, first-in-class, targeted inhibitor of E-selectin. Uproleselan (yoo' pro le' sel an), currently in a comprehensive Phase 3 development program in AML, has received Breakthrough Therapy Designation from the U.S. FDA and 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 (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.

About GlycoMimetics, Inc.

GlycoMimetics is a clinical-stage biotechnology company discovering and developing glycobiology-based therapies for cancers, including acute myeloid leukemia (AML), and for inflammatory diseases with high unmet need. The Company's science is based on an understanding of the role that carbohydrates play on the surface of every living cell and applying its specialized chemistry platform to discover small molecule drugs, known as glycomimetics, which alter these carbohydrate-mediated pathways in a variety of disease states, including signaling in cancer and inflammation. As a leader in this space, GlycoMimetics is leveraging this unique targeted approach to advance its pipeline of wholly owned drug candidates, with the goal of developing transformative therapies for serious diseases. 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. These forward-looking statements include those relating to the planned or potential clinical development of the Company's product candidate, uproleselan, as well as the presentation of data from clinical trials 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 2, 2021, 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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