GlycoMimetics Advances GMI-1359 Program With Patent Issuance and FDA Designations

February 4, 2020

- US Composition of Matter Patent issued

- Orphan Drug and Rare Pediatric Disease Designations granted by FDA for treatment of osteosarcoma

ROCKVILLE, Md.--(BUSINESS WIRE)--Feb. 4, 2020-- GlycoMimetics, Inc. (Nasdaq: GLYC) today announced several achievements for its GMI-1359 development program, including the issuance of a new patent and key designations granted by the U.S. Food and Drug Administration (FDA) that may provide future development support and marketing protections. GMI-1359 is the Company's novel drug candidate designed to simultaneously inhibit both E-selectin and CXCR4, two adhesion molecules involved in tumor trafficking and metastatic spread. Duke University investigators recently dosed the first patient in a proof-of-concept Phase 1b study to evaluate GMI-1359 drug candidate in patients with advanced breast cancer with bone metastases.

New Patent

GlycoMimetics today said that the United States Patent and Trademark Office has issued a patent for GMI-1359, covering composition of matter as well as pharmaceutical formulations, and will provide protection through 2035, excluding any patent term adjustments or extensions.

"The new patent for GMI-1359 will help protect the composition of matter of this innovative approach in oncology. GMI-1359 may have a role in treating rare pediatric cancers, such as osteosarcoma, as well as breast cancer and other solid tumors that metastasize to bone," stated Rachel King, Chief Executive Officer of GlycoMimetics. "This intellectual property, as well as that previously granted in Europe, will play a key role as the company advances the drug candidate, especially with the new orphan and rare pediatric disease designations granted by the FDA."

New FDA Designations

In parallel, GlycoMimetics today announced that the FDA has granted Orphan Drug Designation and Rare Pediatric Disease Designation to GMI-1359 for the treatment of osteosarcoma, a rare cancer affecting about 900 adolescents a year in the United States. These designations will aid in the development of this drug candidate, including making it eligible for the FDA's Pediatric Priority Review Voucher.

"It's encouraging for us as well as for patients and providers that the FDA recognizes the urgent need for new, more effective treatments for this devastating pediatric disease," stated Ms. King.

In addition to its clinical work in breast cancer, GlycoMimetics has conducted preclinical studies that have demonstrated strong support for the potential use of GMI-1359 in osteosarcoma. At the 2018 American Association of Cancer Research Annual Meeting, GlycoMimetics presented data establishing the biologic rationale for the use of a dual e-selectin/CXCR-4 inhibitor in pediatric and young adult patients with osteosarcoma. In that study, GMI-1359 was shown to inhibit tumor progression in an orthopedic model of osteosarcoma as well as inhibit the development of pulmonary metastases from primary osteosarcoma lesions. (https://glycomimetics.com/wp-content/uploads/2018/11/Osteosarcoma-GMI1359-AACR-2018-V4.pdf)

About Orphan Drug Designation

The FDA Orphan Drug Designation program provides orphan status to drugs and biologics that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the U.S. Among the benefits of orphan designation in the U.S. are seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received.

About Rare Pediatric Disease Designation

The FDA defines a "rare pediatric disease" as a serious or life-threatening rare disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an initial approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

About Osteosarcoma

Osteosarcoma is a rare cancer of the bone that usually affects the large bones of the arm or leg, often growing quickly and spreading to other parts of the body. It occurs most often in children and young adults between the ages of 10 and 30. Each year, about 800 to 900 new cases of osteosarcoma are diagnosed in the United States. For more information, please see the osteosarcoma fact sheets at the National Cancer Institute and the American Cancer Society.

About GMI-1359

GMI-1359 is designed to simultaneously inhibit both E-selectin and CXCR4. E-selectin and CXCR4 are both adhesion molecules involved in tumor trafficking and metastatic spread. Preclinical studies indicate that targeting both E-selectin and CXCR4 with a single compound could improve efficacy in the treatment of cancers that involve the bone marrow such as acute myeloid leukemia and multiple myeloma or in solid tumors that metastasize to the bone, such as prostate cancer and breast cancer, as well as in osteosarcoma, a rare pediatric tumor. GMI-1359 has completed a Phase 1 clinical

trial in healthy volunteers. The newly initiated Phase 1b clinical study in breast cancer patients is designed to enable investigators to identify an effective dose of the drug candidate and to generate initial biomarker data around the drug's activity.

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' wholly owned drug candidate, uproleselan, an E-selectin antagonist, was evaluated in a Phase 1/2 clinical trial as a potential treatment for acute myeloid leukemia (AML). It has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration and is being evaluated across a range of patient populations including a company-sponsored Phase 3 trial in relapsed/refractory AML. GlycoMimetics has also completed a Phase 1 clinical trial with 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 regarding the clinical development and potential benefits and impact of the Company's drug candidates. These forward-looking statements include those relating to the planned clinical development of the Company's wholly-owned product 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, 2019, 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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