



## **GlycoMimetics Announces EMA's Granting of Orphan Drug Status in European Union for GMI-1070 in Sickle Cell Disease**

**GAITHERSBURG, MD, September 13, 2013** – [GlycoMimetics, Inc.](#) announced today that the the European Medicines Agency (EMA) has granted orphan drug designation for [GMI-1070](#) (rivipansel sodium) for the treatment of vaso-occlusive crisis (VOC) in patients with sickle cell disease. Orphan drug designation in the European Union (EU) is given to products that are designed for the diagnosis, prevention or treatment of rare diseases that are life-threatening or very serious. A disease is defined as rare in the EU if it affects fewer than five in 10,000 people. Granting of orphan drug designation in the EU provides companies with development and commercial incentives, including a 10-year period of market exclusivity, access to a centralized review process, protocol assistance and scientific advice during product development, waiving or reduction of certain fees, and eligibility for grants and R&D support initiatives.

"We are extremely pleased that the EMA has granted orphan drug status to GMI-1070 for patients with sickle cell disease experiencing VOC," said [Helen Thackray, M.D., Vice President of Clinical Development and Chief Medical Officer](#) at GlycoMimetics. "Given that people living with sickle cell disease currently do not have enough treatment options, we appreciate all regulatory efforts to help bring the drug to market quickly, assuming that additional studies will continue to demonstrate the potential for GMI-1070 to benefit those living with the disease."

GMI-1070, which has previously received both Orphan Drug and Fast Track status for VOC from the U.S. Food & Drug Administration (FDA), is being developed in partnership with Pfizer (NYSE: PFE). Pfizer is responsible for the next steps of clinical development for GMI-1070.

### **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 are molecules that mimic the structure of carbohydrates involved in important biological processes. Using its expertise in carbohydrate chemistry and knowledge of carbohydrate biology, the company is developing a pipeline of glycomimetic drug candidates that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. For additional information, please visit the company's web site at <http://www.glycomimetics.com>.

### **About Sickle Cell Disease and VOC**

Sickle cell disease is a prevalent genetic disorder worldwide. Due to increasing global migration, the number of sickle cell carriers in European countries is increasing steadily. It is a chronic condition causing substantial illness and death. The main clinical feature of sickle cell disease is periodic painful vaso-occlusive crisis episodes, known as VOC or pain crises, which result in clinical complications, interruptions in patients' lives, and cumulative irreversible damage that impacts the morbidity and mortality of patients. Treatment for VOC today consists primarily of supportive therapy, in the form of hydration and pain control, typically requiring extended hospitalization.

GMI-1070 is intended to treat VOC by inhibiting the cell activation and enhanced cell adhesion that causes the ischemia and pain. GlycoMimetics selected VOC of sickle cell disease as the first potential indication for GMI-1070.

Contact: Brian Hahn

Email: [bhahn@glycomimetics.com](mailto:bhahn@glycomimetics.com)

Phone: 240-243-1207