



GlycoMimetics Announces Top Line Results from Phase 2 Trial with GMI-1070 in Sickle Cell Disease

GAITHERSBURG, MD, April 16, 2013 – [GlycoMimetics, Inc.](#) (GMI) today announced top line clinical results from a Phase 2 study of GMI-1070 in patients experiencing vaso-occlusive crisis (VOC) of sickle cell disease. This randomized, double-blind, placebo-controlled trial examined the efficacy, safety and pharmacokinetics of GMI-1070, GlycoMimetics' lead clinical-stage drug, in hospitalized patients with sickle cell disease experiencing VOC. GlycoMimetics enrolled 76 patients 12 to 60 years of age at 22 trial sites in the United States and Canada.

Patients treated with GMI-1070 experienced reduction in duration of vaso-occlusive crisis, reduction in length of hospital stay, and reduction in the use of narcotic pain relief. Both adult and pediatric patients demonstrated improvement, and adverse event rates were comparable between GMI-1070 and placebo. The company plans to submit detailed study results for presentation at a scientific meeting and publication in a peer-reviewed scientific journal.

"We are very pleased with the results of this Phase 2 study, and particularly want to acknowledge the patients and medical centers whose participation made this clinical trial possible," said [Helen Thackray, M.D., Vice President of Clinical Development and Chief Medical Officer](#) at GlycoMimetics, Inc. "There is major unmet clinical need in sickle cell disease, and we hope that additional studies will continue to demonstrate the potential for GMI-1070 to benefit people living with the disease."

"This clinical data provides an important validation of GlycoMimetics' innovative platform technology and of our approach to targeting the selectin family of adhesion molecules," added [John Magnani, Ph.D., the company's Vice President of Research and Chief Scientific Officer](#). "It is also supportive of the potential of other programs in the GlycoMimetics pipeline."

In 2011, [GlycoMimetics entered into a worldwide license agreement with Pfizer Inc.](#) (NYSE: PFE) to develop and, if approved by applicable regulatory authorities, to commercialize GMI-1070 for all indications. Pfizer will be responsible for the next steps of clinical development for GMI-1070.

About GlycoMimetics, Inc.

GlycoMimetics is a privately held biotechnology company that capitalizes on advances in the field of glycobiology to treat inflammation, cancer, and infectious diseases. The company uses rational design of small molecule drugs that mimic the functions of bioactive carbohydrates to develop new drug candidates. For additional information, please visit the company's web site: <http://www.glycomimetics.com>.

About Sickle Cell Disease and VOC

Sickle cell disease is one of the most prevalent genetic disorders in the U.S., affecting over 80,000 people. 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. VOC is responsible for more than 75,000 hospitalizations per year in the U.S. with an average stay of approximately six days. 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 vaso-occlusive crisis of sickle cell disease as the first potential indication for GMI-1070.