



GlycoMimetics, Inc.

## **FOR IMMEDIATE RELEASE**

### **New Drug to Treat Sickle Cell Crisis Begins Pilot Study in Patients**

*GlycoMimetics' GMI-1070 targets vaso-occlusive crisis*

GAITHERSBURG, Md. - September 2, 2009 -- GlycoMimetics, Inc. (GMI), a clinical-stage biotechnology company that is developing a new class of glycobiology-based therapies for a broad range of indications, today announced that a pilot study of the company's lead candidate, GMI-1070, is underway in sickle cell patients. The first patients in this pilot study were treated at Children's Hospital & Research Center Oakland in California.

GMI-1070 is a first-in-class pan-selectin inhibitor intended to treat vaso-occlusive crisis by inhibiting the inflammatory processes underlying cell adhesion. The pilot study will evaluate the safety and pharmacokinetics of GMI-1070, and is also designed to measure the effect of GMI-1070 on blood flow and biomarkers of inflammation in patients with sickle cell disease. The trial will enroll up to twenty sickle cell disease patients not experiencing vaso-occlusive crisis.

"The dosing of patients with this novel agent marks an important milestone in the advancement of an exciting and much-needed approach to addressing sickle cell crisis," said Helen Thackray, M.D., GlycoMimetics' Vice President of Drug Development. "The data we obtain from this pilot study will enable additional studies in sickle cell patients in the midst of vaso-occlusive crisis." "GMI-1070 appears to have interesting potential as a new treatment for vaso-occlusive crisis in sickle cell patients. Vaso-occlusive crisis is a painful and life-threatening complication of sickle cell disease that truly lacks effective treatment options," said Lori Styles, M.D., Hematologist/Oncologist and the lead clinical investigator at Children's for this program. "We are pleased to have the opportunity to be a leading clinical investigation site in the evaluation of GMI-1070."

GlycoMimetics recently announced the completion of two Phase 1 clinical studies of GMI-1070's safety in healthy volunteers. Clinical investigators reported no serious adverse events in either of the two studies.

#### **About GMI-1070**

GlycoMimetics' lead compound, GMI-1070, is a rationally designed glycomimetic inhibitor of E-, P- and L-selectins, and inhibits a key early step in the inflammatory process leading to leukocyte adhesion and recruitment to inflamed tissue. GMI-1070 has been shown to be active in several models of diseases in which leukocyte adhesion and activation play a key role, including vaso-occlusive crisis of sickle cell disease. By inhibiting selectin interactions, GMI-1070 may be able to decrease the enhanced cell adhesion that results in vaso-occlusive crisis. In pre-clinical studies,

GMI-1070 restored blood flow to affected vessels of sickle cell animals experiencing vaso-occlusive crisis. GMI-1070 is also being evaluated in preclinical studies for the treatment of certain hematologic cancers, where selectin-mediated cell adhesion and migration is known to play a key role in the disease process.

### **About Sickle Cell Disease and Vaso-Occlusive Crisis**

Vaso-occlusive crisis is the main clinical feature of sickle cell disease, often resulting in significant clinical complications, and sometimes death. Currently, there are no mechanism-based therapies for treatment of vaso-occlusive crisis. Treatment consists primarily of supportive therapy in the form of hydration and pain control, typically requiring hospitalization for five to six days. There are over 75,000 hospitalizations per year associated with vaso-occlusive crisis in the US.

### **About Children's Hospital & Research Center Oakland**

Children's Hospital & Research Center Oakland is Northern California's only freestanding and independent children's hospital. Children's is a leader in many pediatric specialties including neonatology, cardiology, neurosurgery and intensive care. The hospital is a designated Level 1 pediatric trauma center and has the largest pediatric critical care facility in the region. Children's Hospital has 190 licensed beds, 201 hospital-based physicians in 30 specialties, more than 2,600 employees and an annual operating budget of \$312 million. Children's research arm, Children's Hospital Oakland Research Institute (CHORI), is internationally renowned for taking state-of-the-art basic and clinical research to the bedside with interventions for treating and preventing human disease. CHORI has 300 staffers, a budget of about \$50 million, and is ranked among the nation's top 10 research centers in National Institutes of Health funding to children's hospitals. CHORI is a leader in translational research, developing new vaccines for infectious diseases, and discovering new treatment protocols for previously fatal or debilitating conditions including cancer, sickle cell anemia, thalassemia, diabetes, asthma, HIV/AIDS, pediatric obesity, nutritional deficiencies, birth defects, hemophilia and cystic fibrosis. For more information, go to [www.childrenshospitaloakland.org](http://www.childrenshospitaloakland.org).

### **About GlycoMimetics, Inc.**

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