



NEWS RELEASE

Mast Therapeutics Completes Patient Enrollment In Pivotal Phase 3 "EPIC" Study For The Treatment Of Sickle Cell Crisis

2016-02-22

Company anticipates top-line data in Q2 2016

SAN DIEGO, Feb. 22, 2016 /PRNewswire/ -- **Mast Therapeutics, Inc.** (NYSE MKT: MSTX), a biopharmaceutical company developing novel, clinical-stage therapies for sickle cell disease and heart failure today announced that it has completed patient enrollment in its Phase 3 clinical study of vepoloxamer for the treatment of patients with sickle cell disease experiencing vaso-occlusive crisis, known as the **EPIC** study. Consistent with prior guidance, the Company expects to report top-line results in the second quarter of 2016.

The EPIC study was conducted under the direction of Principal Investigator James F. Casella, M.D., Rainey Professor and Chief, Division of Pediatric Hematology, Vice Chair for Research, Pediatrics and Director of the Basic and Translational Research Program in Sickle Cell Disease, Comprehensive Sickle Cell Center at Johns Hopkins University School of Medicine.

"The full enrollment of EPIC is an important achievement in the history of sickle cell disease clinical development, as it represents the largest placebo-controlled clinical trial conducted to date for our patients with this disease," stated Mark T. Gladwin, M.D., Jack D. Myers Professor and Chair, Chairman of the Department of Medicine, Director, Pittsburgh Heart, Lung, Blood and Vascular Medicine Institute, UPMC and the University of Pittsburgh School of Medicine, and member of the Executive Steering Committee for EPIC. "Interventional treatment of acute painful crisis represents an important unmet medical need, and has remained elusive to large, multicenter randomized trials necessary to support registration of a new drug. EPIC has set a new standard for the field, and the learnings from this study will have an important impact on the design of future studies. The patients and their families, our committed clinical investigators, and Mast Therapeutics should be applauded for this important achievement."

"As physicians, we are frustrated by our inability to offer patients a disease-modifying treatment for an ongoing crisis," stated Gregory J. Kato, M.D., Professor of Medicine, Division of Hematology/Oncology, Director, Sickle Cell Center of Excellence, UPMC and the University of Pittsburgh School of Medicine, and member of the Executive Steering Committee for EPIC. "This is a challenging condition for which multiple underlying pathologies may need to be addressed simultaneously to achieve clinical outcomes. In non-clinical studies, vepoloxamer has demonstrated multiple activities that may improve the complex pathologies occurring during sickle cell crisis. We are anxious to review the data from the trial, once unblinded, to determine how vepoloxamer's pharmacodynamic activity translates to clinical benefit for sickle cell patients."

"We are proud to have completed enrollment in the EPIC study," stated Brian M. Culley, Chief Executive Officer. "We wish to express our deep gratitude to the courageous patients and their family members and the committed investigators and study coordinators around the globe who have helped us achieve this goal."

"Following the last patient's discharge from the hospital and 30-day safety observation period, there will be an extensive and rigorous review of the blinded data for quality control, ultimately leading to database lock. Study unblinding then will be performed by the study biostatistician. After the biostatistician's work is complete, Mast will learn the top-line outcome of the study from the biostatistician and will be able to report top-line results to the public," continued Mr. Culley.

About the EPIC Study

The EPIC study is a randomized, double-blind, two-arm, placebo-controlled, Phase 3 clinical trial of vepoloxamer in patients with sickle cell disease hospitalized for treatment of vaso-occlusive crisis. The primary objective of the study is to demonstrate that vepoloxamer reduces the duration of vaso-occlusive crisis, with the duration of crisis measured from the time a patient is randomized to the time at which the patient receives the last dose of parenteral opioid analgesic for the treatment of vaso-occlusive crisis prior to hospital discharge. A total of 388 patients, ages four to 46 were enrolled in EPIC. Secondary efficacy endpoints are to compare the rates of re-hospitalization for vaso-occlusive crisis within 14 days of initial hospital discharge and the occurrence of acute chest syndrome within 120 hours of randomization between the treatment and control groups.

About Sickle Cell Disease and Vaso-Occlusive Crisis

Sickle cell disease is a chronic, genetic blood disorder that affects millions worldwide and an estimated 90,000 to 100,000 people in the United States, where it is classified as a rare, or orphan, disease. The hallmark of sickle cell disease is recurring episodes of severe, debilitating pain commonly known as sickle cell crisis or vaso-occlusive crisis. The intense pain experienced by patients is the result of obstruction of blood vessels by "sickled" red blood cells, which are rigid and highly adherent to the vessel walls and to each other. This obstruction leads to reduced blood flow to organs, including the bone marrow, not only causing severe pain, but also cumulative tissue damage

and, ultimately, loss of vital organ function and significantly reduced lifespan. There are between 80,000 to 100,000 hospitalizations annually in the U.S. related to vaso-occlusive crisis and no approved treatment option to shorten the duration or reduce the severity of a crisis once underway.

About Mast Therapeutics

Mast Therapeutics, Inc. is a publicly traded biopharmaceutical company headquartered in San Diego, California. The Company is leveraging its **MAST (Molecular Adhesion and Sealant Technology) platform**, derived from over two decades of clinical, nonclinical and manufacturing experience with purified and non-purified poloxamers, to develop vepoloxamer (also known as MST-188), its lead product candidate, for serious or life-threatening diseases and conditions typically characterized by impaired microvascular blood flow and damaged cell membranes. The Company is also developing AIR001, a sodium nitrite solution for inhalation via nebulization, for the treatment of heart failure with preserved ejection fraction (HFpEF).

Vepoloxamer is an investigational new drug being evaluated in a pivotal Phase 3 study called **EPIC** for the treatment of vaso-occlusive crisis in patients with sickle cell disease and in a Phase 2 study for the treatment of patients with chronic heart failure. AIR001 is in Phase 2 clinical development for the treatment of patients with HFpEF. More information can be found on the Company's web site at www.masttherapeutics.com. (Twitter: [@MastThera](https://twitter.com/MastThera))

Mast Therapeutics™ and the corporate logo are trademarks of Mast Therapeutics, Inc.

Forward Looking Statements

Mast Therapeutics cautions you that statements included in this press release that are not a description of historical facts are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that are based on the Company's current expectations and assumptions. Such forward-looking statements may be identified by the use of forward-looking words such as "intend," "plan," "anticipate," "believe," "expect," among others, and include, but are not limited to, statements relating to prospects for successful development and commercialization of the Company's product candidates, including vepoloxamer for the treatment of vaso-occlusive crisis of sickle cell disease, and anticipated timing of achievement of development milestones, such as completion of clinical studies and announcement of study data. There are a number of factors that could cause or contribute to material differences between actual events or results and the expectations indicated by the forward-looking statements. These factors include, but are not limited to: the inherent uncertainty of outcomes in ongoing and future studies of the Company's product candidates and the risk that its product candidates may not demonstrate adequate safety, efficacy or tolerability in one or more such studies, including vepoloxamer in EPIC; delays in the commencement or completion of clinical studies, including as a result of difficulties in obtaining regulatory agency agreement on clinical development plans or clinical study design, opening trial sites, enrolling study subjects, manufacturing sufficient quantities of clinical trial material, being subject to a "clinical hold," and/or suspension or

termination of a clinical study, including due to patient safety concerns or lack of funding; delays in clinical study closeouts, including blinded data review and quality control and assurance procedures; the risk that, even if current and planned clinical studies are successful, the FDA or other regulatory agencies may determine they are not sufficient to support a new drug application; the potential that, even if clinical studies of a product candidate in one indication are successful, clinical studies in another indication may not be successful; the Company's dependence on third parties to assist with important aspects of development of its product candidates, including conduct of its clinical studies and supply and manufacture of clinical trial material, and, if approved, commercial product, and the risk that such third parties may fail to perform as expected, leading to delays in product candidate development or approval or inability to meet market demand for approved products, if any; the risk that the Company may be required to repay its outstanding debt obligations on an accelerated basis and/or at a time that could be detrimental to its financial condition, operations and/or business strategy; risks associated with the Company's ability to manage operating expenses and/or obtain additional funding to support its operations on a timely basis or on acceptable terms, or at all; the potential for the Company to delay, reduce or discontinue current and/or planned development activities, including clinical studies, or partner its product candidates at inopportune times if it is unable to raise sufficient additional capital as needed; the risk that, even if the Company successfully develops a product candidate in one or more indications, it may not realize commercial success and may never achieve profitability; the risk that the Company is not able to obtain and maintain effective patent coverage or other market exclusivity protections for its products, if approved, without infringing the proprietary rights of others; and other risks and uncertainties more fully described in the Company's press releases and periodic filings with the Securities and Exchange Commission. The Company's public filings with the Securities and Exchange Commission are available at www.sec.gov.

You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date when made. Mast Therapeutics does not intend to revise or update any forward-looking statement set forth in this press release to reflect events or circumstances arising after the date hereof, except as may be required by law.

Logo - <http://photos.prnewswire.com/prnh/20120612/LA22456LOGO-a>

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/mast-therapeutics-completes-patient-enrollment-in-pivotal-phase-3-epic-study-for-the-treatment-of-sickle-cell-crisis-300223537.html>

SOURCE Mast Therapeutics, Inc.

Mast Therapeutics, Ioana C. Hone (ir@mastthera.com), 858-552-0866 Ext. 303