



NEWS RELEASE

Mast Therapeutics Reports Top-Line Results From Phase 3 Study In Sickle Cell Disease

2016-09-20

Conference call scheduled for September 21, 2016 at 8:00am ET

SAN DIEGO, Sept. 20, 2016 /PRNewswire/ -- **Mast Therapeutics, Inc.** (NYSE MKT: MSTX), a biopharmaceutical company developing novel, clinical-stage therapies for sickle cell disease and heart failure, today reported top-line results from EPIC, a Phase 3 clinical study of its investigational new drug vepoloxamer (also known as MST-188) for the treatment of individuals with sickle cell disease experiencing vaso-occlusive crisis (VOC). The study did not meet its primary efficacy endpoint of demonstrating a statistically significant reduction in the mean duration of VOC (82 hours in the vepoloxamer group compared to 78 hours in the placebo group in the intent-to-treat population ($p=0.09$)). There were no statistically significant differences between treatment groups in the intent-to-treat population across the two secondary efficacy endpoints, rate of re-hospitalization for VOC and the occurrence of acute chest syndrome. Consistent with previously conducted studies, vepoloxamer was generally well tolerated with no statistically significant differences in treatment-related serious adverse events in the vepoloxamer group compared to the placebo group. No deaths occurred on the study.

"We are exceedingly disappointed with these top-line results. While clearly not the outcome we wanted, we believe the insights and data from the largest placebo-controlled clinical trial ever completed in sickle cell disease will substantially advance the understanding of vaso-occlusive crisis and the still maturing clinical science necessary to support the development of new therapeutics for this debilitating disease," stated Brian M. Culley, the Company's Chief Executive Officer. "We wish to reiterate our sincere appreciation for all of the patients, caregivers, and others who aided us in conducting this informative study."

"These analyses are limited to just top-line data, so in the coming weeks the Company intends to review the full

data set from EPIC. In addition, we plan to perform an interim analysis of the ongoing heart failure trial of vepoloxamer. However, based on the data we've seen to date, we expect we will terminate all clinical development of vepoloxamer. Consequently, while we evaluate our options, we intend to significantly and immediately reduce our operating expenses and continue our efforts with AIR001, our lead asset in heart failure with preserved ejection fraction, which currently is the subject of a 100-patient phase 2 study expected to complete enrollment by the end of 2017," continued Mr. Culley.

Investor Conference Call

The Company will hold a conference call tomorrow, September 21, 2016, at 8:00 a.m. ET / 5:00 a.m. PT to discuss the Phase 3 results. Interested parties may access the conference call by dialing (855) 239-3120 from the U.S., (855) 669-9657 from Canada, and (412) 542-4127 from outside the U.S. and should request the Mast Therapeutics, Inc. Call. A live webcast of the conference call will be available online from the Investors section of Mast's website at <http://www.masttherapeutics.com/investors/events/>. Replays of the webcast will be available on the Company's website for 30 days and a telephone replay will be available through September 28, 2016 by dialing (877) 344-7529 from the U.S., (855) 669-9658 from Canada, and (412) 317-0088 from elsewhere outside the U.S. and entering replay access code 10093117.

About the EPIC Study

The EPIC study was a randomized, double-blind, two-arm, placebo-controlled, Phase 3 clinical trial of vepoloxamer in individuals with sickle cell disease hospitalized for acute pain typical of vaso-occlusive crisis who required treatment with parenteral opioid analgesia. The primary objective of the study was to evaluate the efficacy of vepoloxamer in reducing the duration of vaso-occlusive crisis, with the duration of crisis measured from the time of randomization to the time at which the patient received the last dose of parenteral opioid analgesia for the treatment of vaso-occlusive crisis prior to hospital discharge. Vepoloxamer or placebo (0.45% saline) was administered intravenously as a 1 hour loading dose infusion (100 mg/kg), immediately followed by a continuous maintenance infusion (30 mg/kg/hr) for at least 12 hours and up to 48 hours. Randomization was stratified by age (≥ 4 to < 16 years or ≥ 16 to ≤ 65 years), use of hydroxyurea (yes or no), and pain score (measured using the Wong-Baker FACES® Pain Rating Scale at time of randomization: < 8 or ≥ 8). The study was 90% powered to detect a 17% (16-hour) difference in treatment arms, with a statistical significance level of $p=0.05$ (assuming an average crisis duration of 96 hours in the control arm and a coefficient of variation $> 50\%$). Secondary efficacy endpoints were 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.

A total of 388 patients, ages four to 46, were randomized in EPIC. More than 75 study sites in 14 countries participated. The average age was 15 years. Patients under age 18 accounted for approximately 71% of total subjects. Approximately 61% of patients were concurrently on hydroxyurea therapy.

About Sickle Cell Disease and Vaso-Occlusive Crisis

Sickle cell disease is a chronic, genetic blood disorder that affects millions worldwide and an estimated 100,000 people in the United States, where it is classified as a rare, or orphan, disease. The hallmark of sickle cell disease is vaso-occlusive crisis, which results from obstruction of blood vessels by sickled red blood cells causing tissue ischemia and injury. Vaso-occlusions can occur everywhere blood flows and lead to an accumulating disease burden in every organ system with the ultimate loss of vital organ function and significantly reduced lifespan. Vaso-occlusive crisis is also characterized by intense and debilitating pain which can last for days and even weeks. There are between 80,000 to 100,000 hospitalizations annually in the U.S. related to vaso-occlusive crisis and no FDA approved treatment to shorten its duration or reduce the risk of ischemic injury related to crisis.

About Mast Therapeutics

Mast Therapeutics, Inc. is a publicly traded biopharmaceutical company headquartered in San Diego, California. The Company is developing two clinical-stage investigational new drugs for serious or life-threatening diseases and conditions. Vepoloxamer, the Company's lead product candidate, is in Phase 3 clinical development for the treatment of vaso-occlusive crisis in patients with sickle cell disease and in Phase 2 clinical development for the treatment of patients with heart failure. Enrollment in the Company's Phase 2 study of vepoloxamer in patients with chronic heart failure is ongoing. AIR001, the Company's second product candidate, is in Phase 2 clinical development for the treatment of patients with heart failure with preserved ejection fraction (HFpEF). Enrollment in Phase 2 studies of AIR001 in patients with HFpEF are ongoing, including a 100-patient, multicenter, randomized, double-blind, placebo-controlled, Phase 2 study in patients with HFpEF being conducted by the Heart Failure Clinical Research Network. More information can be found on the Company's web site at www.masttherapeutics.com.

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Forward Looking Statements

Mast Therapeutics cautions you that statements in this press release and statements made during the conference call to be held on September 21, 2016 that are not a description of historical fact are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements may be identified by the use of words referencing future events or circumstances such as "expect," "intend," "plan," "anticipate," "believe," and "will," among others. Examples of forward-looking statements in this press release include statements relating to the Company's development plans for its product candidates, the Company's business plans and objectives, and its anticipated results of operations, financial condition and funding needs. Forward-looking statements should not be read as guarantees of future performance or results because they involve the Company's beliefs and assumptions based on currently available information and are subject to significant known and unknown risks and uncertainties that may cause actual performance and results to differ materially from expectations indicated by the forward-looking statements. Some of the factors that could cause

actual performance or results to differ include, without limitation: the Company's need for additional funding to continue to operate as a going concern; risks associated with the Company's ability to manage operating expenses and obtain additional capital as needed; uncertainty related to the Company's ability to remain in compliance with the terms and conditions under its debt facility and risk that, in addition to the \$10 million prepayment required within three days of announcement of negative EPIC results, the Company may be required to repay its remaining outstanding debt obligations on an accelerated basis and/or at a time that could be detrimental to the Company's financial condition, operations and/or business strategy; the impact of significant reductions in the Company's operations on its ability to develop its product candidates or maintain compliance with laws and regulations relating to public companies; completion of a more detailed analysis of EPIC data and announcement of additional data from the study; uncertainties inherent in the conduct of clinical studies and the risk that the Company's product candidates may not demonstrate adequate safety, efficacy or tolerability in one or more clinical studies; the potential for the Company to significantly delay, reduce or discontinue current and/or planned development activities or sell or license its assets at inopportune times if it is unable to raise sufficient additional capital as needed; that the Company is not the sponsor of the ongoing Phase 2 clinical studies of AIR001 and has no control over the conduct of those studies, including whether they will be completed on anticipated timelines, or at all; the Company's dependence on third parties to assist with important aspects of development of the Company's product candidates, including the conduct of its clinical studies, the manufacture and supply of its clinical trial material and, if approved, commercial product, and the conduct of regulatory activities, including preparation of new drug applications, and the risk that such third parties may fail to perform as expected leading to delays in product candidate development, regulatory approval, commercial launch and/or inability to meet future market demand for any approved products; the risk that vepoloxamer may not realize commercial success even if it receives regulatory approval, and that the Company 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, or that the use or manufacture of the Company's products may infringe 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 or made during its September 21, 2016 conference call to reflect events or circumstances arising after the date hereof, except as may be required by law.

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To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/mast-therapeutics-reports-top-line-results-from-phase-3-study-in-sickle-cell-disease-300331289.html>

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