NEWS RELEASE

Mast Therapeutics Provides Business Update

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- Enrollment in largest-ever interventional Phase 3 trial in sickle cell disease has surpassed 90%; top-line data anticipated Q2 2016
- Enrollment in Phase 2a study of AIR001 in HFpEF is complete; top-line data expected this month
- Company ended 2015 with approximately $41M in cash, cash equivalents and investment securities

SAN DIEGO, Jan. 7, 2016 /PRNewswire/ -- **Mast Therapeutics, Inc.** (NYSE MKT: MSTX), a biopharmaceutical company developing novel, clinical-stage therapies for sickle cell disease and heart failure today provided a business update.

- The Company reported that patient enrollment in the pivotal Phase 3 "**EPIC**" study of its lead product candidate, vepoloxamer (MST-188), in sickle cell disease recently surpassed the 90% mark. The Company expects to complete patient enrollment in February. Consistent with prior guidance, the Company expects to report top-line results in the second quarter of 2016.
- The Company also reported that dosing in its special population pharmacokinetics study of vepoloxamer in subjects with varying degrees of renal insufficiency is scheduled to begin today. This study will support the Company's New Drug Application submission to the U.S. Food and Drug Administration and provide guidance for proper dosing of vepoloxamer in sickle cell disease.
- The Company also announced that patient enrollment is complete in a placebo-controlled Phase 2a study of AIR001 designed to measure its effect on cardiovascular hemodynamics in patients suffering from heart failure with preserved ejection fraction (HFpEF). Top-line results from this 30-patient, investigator-sponsored study are expected to be available this month. Preliminary results from an initial cohort of patients enrolled in a second investigator-sponsored Phase 2a study of AIR001 in HFpEF patients have been submitted for presentation at a scientific conference in May.
- The Company also is progressing with its Phase 2 study of vepoloxamer for the treatment of chronic heart

failure, which is testing a new formulation of vepoloxamer designed to be more suitable for heart failure patients. The Company currently has 5 study sites open in the United States and Australia and plans to open additional sites in the first quarter of 2016.

- As of December 31, 2015, the Company had cash, cash equivalents and investment securities of approximately $41 million.

"We are pleased to report that more than 90% of the planned 388 patients have been enrolled in EPIC, the largest sickle cell crisis intervention study ever conducted," stated Brian M. Culley, Chief Executive Officer. "Participation in EPIC has been outstanding with more than 75 sites in 14 countries and more than two-thirds of those sites located in the U.S. We expect to complete enrollment next month and after the final enrolled patient's 30-day observation period, the process of blinded data review and quality control will begin, leading thereafter to database lock and unblinding of the study data."

"In addition, we have made important progress with the development of AIR001 for HFpEF and look forward to receiving results from two ongoing Phase 2a studies, the first of which is expected to occur this month," continued Mr. Culley.

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 an investigational new drug being evaluated in two institution-sponsored Phase 2a studies in patients with HFpEF. More information can be found on the Company's web site at **www.masttherapeutics.com**. (Twitter: **@MastThera**)

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 that are based on the Company's current expectations and assumptions. Such forward-looking statements may include, but are not limited to, statements relating to prospects for successful development and commercialization of the Company's investigational drugs, including vepoloxamer and AIR001, and anticipated timing of achievement of development milestones, such as commencement and completion of clinical studies or regulatory activities, and of announcement of study data. Among the factors that could cause or contribute to material differences between the Company's actual results and the expectations indicated by the forward-looking statements are risks and uncertainties that include, but are not limited to: the uncertainty of outcomes in ongoing and future studies of the Company's product candidates and the risk that its product candidates, including vepoloxamer, may not demonstrate adequate safety, efficacy or tolerability in one or more such studies, including 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 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 reliance on contract research organizations (CROs), contract manufacturing organizations (CMOs), and other third parties to assist in the conduct of important aspects of development of its product candidates, including clinical studies, manufacturing, and regulatory activities for its product candidates, and that such third parties may fail to perform as expected; 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; the Company's ability to obtain additional funding 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, partner its product candidates at inopportune times or pursue less expensive but higher-risk and/or lower return development paths 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 adequately protect its intellectual property rights, through patents or otherwise, and prevent competitors from duplicating or developing equivalent versions of its product candidates or that the use or manufacture of its products or product candidates 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 to reflect events or circumstances arising after the date hereof, except as may be required by law.

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