



NEWS RELEASE

Mast Therapeutics Provides Business Update

2016-09-26

SAN DIEGO, Sept. 26, 2016 /PRNewswire/ -- **Mast Therapeutics, Inc.** (NYSE MKT: MSTX) today provided an update related to its business strategy and the clinical development of its product candidates.

- The Company's cash, cash equivalents, and investment securities were \$30.3 million at August 31, 2016.
- The Company will focus on clinical development of AIR001 (sodium nitrite solution for intermittent inhalation) for the treatment of heart failure with preserved ejection fraction (HFpEF). Specifically, during 2016 and 2017, the Company will continue to support three ongoing investigator-sponsored Phase 2 clinical studies of AIR001 being conducted at prestigious research institutions.
- The Company has begun to wind down its vepoloxamer programs in sickle cell disease and heart failure and expects those activities will be completed in the fourth quarter of 2016.
- While furthering the development of AIR001 through the ongoing Phase 2 clinical studies, the Company is planning to initiate a process to evaluate partnership opportunities for its assets.
- As a result of cost savings anticipated by the termination of its vepoloxamer clinical programs and related operations and the prioritization of its AIR001 program, the Company estimates that its operating expenses for 2017 will be in the range of \$9 to \$10 million, excluding share-based compensation expense. This anticipated level of spend reflects an approximate 70% reduction from estimated operating expenses for 2016 of approximately \$32 to \$34 million, excluding share-based compensation expense.
- The Company will make the \$10 million prepayment on its debt facility triggered by the recently announced results of its Phase 3 study of vepoloxamer in early October 2016. After that prepayment, the principal amount of the Company's debt will be approximately \$3.5 million, which is scheduled to be repaid in equal monthly installments of principal and interest through January 1, 2019.

"Following a critical review of our pipeline and opportunities we have determined that a focus on AIR001 will provide for a strong foundation from which we will seek to return value to our stockholders," stated Brian M. Culley,

the Company's Chief Executive Officer. "However, these decisions likely will have a significant impact on our talented team members and I want to personally thank those who may be affected for their hard work, dedication and contributions to the vepoloxamer program."

AIR001 Program Update

The Company will prioritize its AIR001 program with continued support for three separate, ongoing, investigator-sponsored Phase 2 clinical studies of AIR001. Earlier this year, the Company reported positive top-line results from a blinded and randomized Phase 2a clinical study of AIR001 in HFpEF patients and detailed results were published on September 16, 2016 in *Circulation Research*, Volume 119, Issue 7 (available [here](#)).

- The Company will continue to support an ongoing, randomized, double-blind, placebo-controlled crossover Phase 2 study of AIR001 in patients with HFpEF being conducted by the Heart Failure Clinical Research Network (HFN) with support from a grant awarded by the National Heart, Lung, and Blood Institute, part of the National Institutes of Health. (ClinicalTrials.gov Identifier: NCT02742129) Patient enrollment in this study is expected to complete before the end of 2017. The majority of expenses for this 100-patient study are being funded by the HFN.
- The Company will continue to support an ongoing, investigator-sponsored Phase 2 clinical study of AIR001 in patients with pulmonary hypertension associated with HFpEF. (ClinicalTrials.gov Identifier: NCT01431313) The Company previously reported positive interim results from the study, which results were presented at the American Thoracic Society International Conference in May 2016 (poster available [here](#)). Additional interim results from this Phase 2 study have been accepted for publication and are expected to be available in the coming weeks.
- The Company will continue to support an ongoing, randomized, placebo-controlled Phase 2 study of AIR001 in patients with HFpEF undergoing cardiac rehabilitation for exercise training to evaluate whether blinded treatment with AIR001 improves exercise capacity and hemodynamic reserve compared to placebo. (ClinicalTrials.gov Identifier: NCT02713126)

Vepoloxamer Program Update

Concurrent with the wind-down of its vepoloxamer programs, which includes the termination and closure of all active clinical study sites in the EPIC extension study known as EPIC-E and the clinical pharmacokinetics study in individuals with varying degrees of renal insufficiency, the Company is in the process of analyzing additional data from the Phase 3 clinical study of vepoloxamer in sickle cell disease known as EPIC and interim data from a Phase 2 study of vepoloxamer in chronic heart failure and will assess opportunities to create value from this asset.

The Company recently announced that it received a Small Business Innovation Research grant from the National Institute Of Neurological Disorders And Stroke of the National Institutes of Health to support investigation of vepoloxamer in combination with tissue plasminogen activator (tPA) in experimental models of embolic stroke and

the Company currently intends that work will continue as planned. The Company is collaborating with leading stroke researchers at the Neuroscience Institute at Henry Ford Health System for the conduct of this preclinical study. In addition, the Company expects that preclinical work being conducted by the U.S. military under a Cooperative Research and Development Agreement (CRADA) to evaluate vepoloxamer's potential as a resuscitation fluid following major trauma will continue.

About AIR001

AIR001 is a sodium nitrite solution for intermittent inhalation via nebulization. Nitrite is a direct vasodilator and can be recycled in vivo to form nitric oxide (NO) independent of the classical NO synthase (NOS) pathway. Nitrite mediated NO formation has several beneficial effects, including dilation of blood vessels and reduction of inflammation and undesirable cell growth. Generation of NO from sodium nitrite is not dependent upon endothelial function and is enhanced in the setting of tissue hypoxia and acidosis, conditions in which NOS activity typically is depressed. In early clinical studies, AIR001 demonstrated positive hemodynamic effects with reductions observed in right atrial pressure and pulmonary capillary wedge pressure, as well as improvements in mean pulmonary artery pressures, cardiac output, and exercise tolerance as measured by six-minute walk distance. In a randomized, double-blind, placebo-controlled Phase 2a study of AIR001 in patients with HFpEF (n=26), the AIR001 treatment group showed a statistically significant decrease in pulmonary capillary wedge pressure during exercise compared to the control group and AIR001 was generally well-tolerated.

About Mast Therapeutics

Mast Therapeutics, Inc. is a publicly traded biopharmaceutical company headquartered in San Diego, California. The Company has two clinical-stage investigational new drugs, AIR001 and vepoloxamer. AIR001, a sodium nitrite solution for intermittent inhalation via nebulizer, is in Phase 2 clinical development for the treatment of heart failure with preserved ejection fraction (HFpEF). More information can be found on the Company's web site at www.masttherapeutics.com. Mast Therapeutics™ and the corporate logo are trademarks of Mast Therapeutics, Inc.

Forward Looking Statements

Mast Therapeutics cautions you that statements in this press release 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 operating expenses, results of operations and financial condition. 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 to be made in early October 2016, 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; the Company's ability to maintain compliance with NYSE MKT continued listing standards and policies and to maintain the listing and trading of its common stock on that exchange; completion of a more detailed analysis of EPIC data and reporting 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 for approval by regulatory authorities; the potential for the Company to sell or license part or all of its assets; the potential for significant delays, reductions, or discontinuation of current and/or planned development activities if the Company is unable to raise sufficient additional capital as needed; the Company's lack of control over the ongoing, investigator-sponsored Phase 2 clinical studies of AIR001, including whether any of the studies 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 the conduct of regulatory activities, and the risk that such third parties may fail to perform as expected leading to delays in product candidate development and additional costs; 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 its reports filed 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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