



NEWS RELEASE

Mast Therapeutics Joins New Sickle Cell Disease Coalition, Supports Campaign To Improve Treatment And Care

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Sickle Cell Disease Coalition launches a call to action on neglected disease

SAN DIEGO, Sept. 6, 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 is joining other industry leaders, patient advocacy groups, researchers, clinicians, policymakers, and foundations in a collective effort to improve sickle cell disease (SCD) care, early diagnosis, treatment, and research both in the United States and globally. The newly formed Sickle Cell Disease Coalition is spearheaded by the American Society of Hematology (ASH) and aspires to change the status quo of SCD treatment and awareness.

SCD is an inherited, lifelong disorder characterized by red blood cells that become rigid and sickle-shaped, which causes them to stick together and block the flow of oxygen to the body, leading to intense pain and other serious issues such as stroke, organ failure, and death. SCD affects approximately 100,000 Americans and millions worldwide. It is a growing health problem and it is estimated that by 2050 the number of people with SCD will increase by 30 percent globally. Over the last century, several important discoveries have led to improved diagnosis and treatments, but people with the disease still have severe complications and shorter life expectancy. Many are unable to access the care they need and live with pain and disability due to a lack of effective treatment options.

Today, at a press conference and formal launch of the Coalition at the Knight Conference Center of the Newseum in Washington, D.C., the Coalition will announce a call to action on SCD and the American Society of Hematology and several members of the Coalition will issue a new report, *State of Sickle Cell Disease: 2016*, which outlines unmet needs in four priority areas: Access to care, training and professional education, research and clinical trials, and



global health. The report shows that significant improvements are needed across all areas and highlights strategies for change.

"Around the world, people with sickle cell disease face unique challenges – from early death and childhood stroke in countries without early screening programs to lifetimes of mental and physical disability due to lack of access to appropriate care in the United States. We can no longer accept that this is the status quo," said Charles S. Abrams, M.D., President of the American Society of Hematology, the organization spearheading the Coalition. "The Sickle Cell Disease Coalition is bringing together a number of groups that care about this disease to take advantage of the opportunities we have for change."

"We are proud to join ASH and other organizations to support the Coalition's ultimate objective of improving outcomes for individuals with sickle cell disease," stated Brian M. Culley, Chief Executive Officer of Mast Therapeutics. "At Mast, we are working to improve outcomes for sickle cell patients through the development of our investigational new drug vepoloxamer. We are hopeful Mast will be able to provide the first approved medication for intervention in vaso-occlusive crisis, a debilitating and recurrent condition for sickle cell patients that can lead to organ damage and early death," continued Mr. Culley. "We believe that the Coalition will be able to increase awareness of this devastating disease and create more opportunity for changes that can improve care and treatment of individuals living with sickle cell disease."

The Coalition will provide a platform to encourage stakeholders to work together to develop and implement important projects and activities that will ultimately help the SCD community and improve outcomes for individuals with the disease. For more information on the Coalition and its mission, visit www.scdcoalition.org.

About the Sickle Cell Disease Coalition

The mission of the Sickle Cell Disease Coalition (SCDC) (www.scdcoalition.org) is to help amplify the voice of the SCD community, promote awareness, and improve outcomes for individuals with SCD. The Coalition is focused on promoting research, clinical care, education, training, and advocacy as well as providing a platform to encourage stakeholders to work together to develop and implement important projects and activities that will ultimately help the SCD community and improve outcomes for individuals with the disease. The Coalition is comprised of leading patient advocacy groups, people with SCD and their families, researchers, clinicians, policymakers, industry, and foundations with an interest in SCD.

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 388-patient Phase 3 study of vepoloxamer in patients with sickle cell disease, known as the EPIC study, was completed earlier this year. 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. (Twitter: @MastThera)

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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 "expect," "intend," "plan," "anticipate," "believe," 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. 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 the Phase 3 "EPIC" study, and may not receive FDA or other regulatory authority approval; risks associated with the Company's ability to manage operating expenses and obtain additional capital as needed; the Company's potential inability to continue as a going concern if it does not raise sufficient additional capital as needed; 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, including the prepayment of \$10 million of the principal balance of its debt facility if results from the EPIC study are not positive and/or not available on or before October 14, 2016; the potential for the Company to significantly delay, reduce or discontinue current and/or planned development and commercial-readiness activities or sell or license its assets at inopportune times if it is unable to raise sufficient additional capital as needed; the risk that, even if EPIC study results are positive, the FDA may require a second Phase 3 study or other clinical or nonclinical studies to demonstrate substantial evidence of vepoloxamer's effectiveness for sickle cell patients or to provide additional safety and tolerability data or that the FDA may require changes to manufacturing controls or processes that could delay filing of a new drug application; 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; 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, 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.

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To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/mast-therapeutics-joins-new-sickle-cell-disease-coalition-supports-campaign-to-improve-treatment-and-care-300322612.html>

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