MyoKardia Announces Receipt of Milestone Payment from Sanofi for DCM Candidate MYK-491

$25 Million Milestone Reflects Continued Momentum in Ongoing Collaboration

Phase 1 Clinical Trial Planned for First Half of 2017

SOUTH SAN FRANCISCO, Calif., Dec. 02, 2016 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq:MYOK), a clinical stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, announced today that the Company has received a $25 million milestone payment in accordance with the research agreement between MyoKardia and Sanofi established in August 2014 for the filing of an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) for MYK-491 in dilated cardiomyopathy (DCM).

“We are pleased to be moving into the clinic in the first half of 2017 with the second product candidate from MyoKardia’s precision medicine platform,” said Robert McDowell, Ph.D., senior vice president, drug discovery. “Both the Company’s initial product candidate for HCM, now in Phase 2, and our investigational therapy for DCM may correct the inappropriate power output caused by sarcomere mutations that is an underlying driver of disease progression.”

“Our therapeutic hypothesis is that MYK-491 may increase muscle contraction to restore cardiac output,” added Dr. McDowell. “Further, based on preclinical research across multiple animal models, we believe it holds potential for controlled increases in the heart’s contractility with minimal impact on diastole or relaxation.”

Topline results from the planned Phase 1 single ascending dose study of MYK-491 in healthy volunteers are expected in the third quarter of 2017.

About MyoKardia

MyoKardia is a clinical stage biopharmaceutical company pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and rare cardiovascular diseases. MyoKardia’s initial focus is on the treatment of heritable cardiomyopathies, a group of rare, genetically-driven forms of heart failure that result from biomechanical defects in cardiac muscle contraction. MyoKardia has used its precision medicine platform to generate a pipeline of therapeutic programs for the chronic treatment of the two most prevalent forms of heritable cardiomyopathy—hypertrophic cardiomyopathy, or HCM, and dilated cardiomyopathy, or DCM. MyoKardia’s most advanced product candidate, MYK-461, is an orally-administered small molecule designed to reduce excessive cardiac muscle contractility leading to HCM and has been evaluated in three Phase 1 clinical trials. MyoKardia is now studying MYK-461 in the Phase 2 PIONEER-HCM trial in symptomatic, obstructive HCM (oHCM), a subset of HCM. In April 2016, the U.S. Food

and Drug Administration (FDA) granted MYK-461 Orphan Drug Designation for the treatment of symptomatic oHCM. MYK-491, the second clinical candidate generated by MyoKardia's product engine, is designed to increase the overall force of the heart's contraction in DCM patients by increasing cardiac contractility. MyoKardia intends to initiate a Phase 1 study of MYK-491 in healthy volunteers in the first half of 2017. A cornerstone of the MyoKardia platform is the Sarcomeric Human Cardiomyopathy Registry, or SHaRe, a multi-center, international repository of clinical and laboratory data on individuals and families with genetic heart disease, which MyoKardia helped form in 2014. MyoKardia believes that SHaRe, currently consisting of data from approximately 10,000 individuals, is the world's largest registry of patients with heritable cardiomyopathies. MyoKardia's mission is to change the world for patients with serious cardiovascular disease through bold and innovative science. For more information, please visit www.myokardia.com.

Forward-Looking Statements

Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements regarding the clinical and therapeutic potential of MYK-491, the Company's ability to initiate Phase 1 clinical development of MYK-491 and generate topline data from its planned Phase 1 single ascending dose study of MYK-491 in healthy volunteers, and the timing of these events, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, risks associated with the development and regulation of our product candidates, as well as those set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, and our other filings with the SEC. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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