First Subjects Dosed in Phase 1 Study of Dilated Cardiomyopathy Candidate MYK-491

Second Clinical Candidate Generated by MyoKardia's Product Engine

Topline Results Expected in the Third Quarter of 2017

SOUTH SAN FRANCISCO, Calif. – Feb. 2, 2017 – MyoKardia, Inc. (Nasdaq: MYOK), a clinical stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, today announced dose administration for the first cohort of healthy subjects in its Phase 1 single ascending dose study of MYK-491.

MYK-491 is the Company's candidate for the treatment of dilated cardiomyopathy (DCM), and is being developed in an ongoing collaboration between MyoKardia and Sanofi. MYK-491 is designed to increase cardiac muscle contractility in the diseased DCM heart. Reduced cardiac contractility is believed to be the cause of several types of DCM.

This Phase 1 trial, a randomized, placebo-controlled study to assess safety, tolerability, preliminary pharmacokinetics and pharmacodynamics of single ascending oral doses of MYK-491 in healthy volunteers, includes measurements of potential changes in systolic ejection time and other established echocardiographic measures of cardiac contractility.

"There are currently no approved therapies that address the underlying biomechanical causes of DCM, which can lead to chronic, progressive, debilitating heart failure that can shorten life," said Marc Semigran, M.D., chief medical officer of MyoKardia. "Preclinical research across many animal models has demonstrated that MYK-491 may increase the heart's contractility with minimal adverse effects on myocardial diastolic function, which is also abnormal in DCM patients."

Similar to MYK-461, MyoKardia's candidate for hypertrophic cardiomyopathy currently in Phase 2 clinical development, MYK-491 targets the underlying biomechanical defects of the heart muscle. MyoKardia believes that by addressing the underlying cause, molecules like MYK-491 have the potential to change the course of disease.

"The first dosing of MYK-491 into healthy volunteers represents a significant milestone in the development of a potential new therapeutic agent for the treatment of dilated cardiomyopathy," said Tony Muslin, M.D., head of cardiovascular and metabolism unit, Sanofi Global Research & Development. "Sanofi remains excited about our productive collaboration with MyoKardia to develop new treatment options for patients with heritable cardiomyopathies."

Topline results of the study are expected in the third quarter of 2017.

About MYK-491

The oral small molecule MYK-491 is an allosteric modulator of myosin designed to increase cardiac contractility in a DCM heart. Like MYK-461, the Company's candidate for hypertrophic cardiomyopathy, MYK-491 targets the underlying biomechanical defects of the heart muscle. Based on preclinical research across multiple animal models, MYK-491 may hold potential for controlled increases in the heart's contractility with minimal impact on diastole or relaxation. MyoKardia initiated a Phase 1 study of MYK-491 in healthy volunteers in early 2017, with topline results expected in the third quarter of 2017.

About DCM

DCM is a disease that affects about one million people in the United States. DCM can progress to heart failure and severe complications such as stroke, arrhythmias, and sudden cardiac death. In DCM, the walls of the left ventricle are thin and over-expanded, leading to diminished contraction and insufficient blood being pumped by the heart. Typical symptoms include shortness of breath, fatigue, swelling in the extremities, or an irregular heartbeat. As the disease progresses, patients become increasingly debilitated, experience persistent shortness of breath, even at rest, and are at elevated risk for fatal arrhythmias. Diastolic function, or the heart's ability to relax and fill with blood, is also impaired. There is currently no approved medical therapy that addresses the underlying biomechanical causes of DCM

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 currently studying MYK-461 in the Phase 2 PIONEER-HCM trial in symptomatic, obstructive HCM (oHCM), a subset of HCM for which the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation. 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 is currently evaluating MYK-491 in a Phase 1 study in healthy volunteers; topline data is expected in the third quarter 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-461 and MYK-491, the Company's ability to advance MYK-491 in its Phase 1 study in healthy volunteers and to generate data from the study, 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.

Investor Contact:
Beth DelGiacco
Stern Investor Relations, Inc.
212-362-1200
beth@sternir.com

Media Contact:
Steven Cooper
Edelman
415-486-3264
steven.cooper@edelman.com