News Release

MyoKardia Receives Orphan Drug Designation for MYK-461 for Treatment of Symptomatic Obstructive Hypertrophic Cardiomyopathy (oHCM)

SOUTH SAN FRANCISCO, Calif., May 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, today announced that the U.S. Food and Drug Administration (FDA) has granted the company Orphan Drug Designation for MYK-461 for treatment of symptomatic obstructive hypertrophic cardiomyopathy (oHCM), a subset of hypertrophic cardiomyopathy (HCM).

MYK-461 is the Company's lead product candidate and the first therapy designed to target the underlying cause of HCM. MyoKardia's proposed initial indication for MYK-461 is oHCM.

"We greatly appreciate the FDA's Orphan Drug Designation for MYK-461 for treatment of symptomatic obstructive hypertrophic cardiomyopathy," said Tassos Gianakakos, chief executive officer. "This represents another step toward filling a critical need for patients and families who struggle with a chronic and debilitating illness for which there are no approved therapies."

The FDA Office of Orphan Products Development grants orphan status to support development of medicines for underserved patient populations, or rare disorders that affect fewer than 200,000 people in the United States. Orphan drug designation provides MyoKardia with certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

About MYK-461

MYK-461, is an orally administered small molecule designed to reduce excessive cardiac muscle contractility leading to HCM. Three Phase 1 clinical trials have been initiated to assess MYK-461's ability to modulate cardiac myosin by measuring reduction in cardiac muscle contractility via echocardiography. Cardiac muscle contractility is an important biomarker of HCM. Data from two of the Phase 1 trials demonstrate clinical proof of mechanism in both HCM patients and healthy volunteers, and indicate that MYK-461 is well tolerated with dose-proportional pharmacokinetics.

About Symptomatic Obstructive Hypertrophic Cardiomyopathy (oHCM)

Symptomatic obstructive hypertrophic cardiomyopathy (oHCM) is a subset of a group of genetically-driven forms of heart failure that result from biomechanical defects in cardiac muscle contraction. It is estimated that as many as 630,000 people in the United States have a form of HCM.

HCM is defined as an otherwise unexplained thickening of the walls of the heart, known as hypertrophy. The consequences include reduced blood volumes and cardiac output, reduced ability of the left ventricle to expand, and high filling pressures. These can all contribute to reduced effort tolerance and symptoms that include shortness of breath and chest pain. HCM is a chronic disease, and for the majority of patients, the disease progresses slowly and can be extremely disabling. HCM can also cause stroke or sudden cardiac death, and is the most common cause of sudden cardiac death in young people.

There are currently no approved therapeutic products indicated for the treatment of HCM. Patients are typically prescribed one or more drugs (including beta blockers, non-dihydropyridine calcium channel blockers and disopyramide) indicated for the treatment of hypertension, heart failure or other cardiovascular disorders more generally. For a subset of HCM patients with more advanced disease progression or more pronounced symptoms, surgical or other invasive interventions may be appropriate.

About MyoKardia

MyoKardia (Nasdaq:MYOK) is a clinical stage biopharmaceutical company pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and neglected rare cardiovascular diseases. MyoKardia's initial focus is 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 is currently being evaluated in three Phase 1 clinical trials. 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 purpose is to improve the lives of patients and families suffering from cardiovascular disease by creating targeted therapies that can change the course of their condition. 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 and Section 21E of the Securities Exchange Act, 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, the Company’s ability to generate data from its Phase 1 clinical trials of MYK-461 and to initiate Phase 2 clinical development for MYK-461 and Phase 1 clinical development for its DCM product candidate, as well as the timing of such 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 the prospectus for our recent initial public offering of common stock and our other filings with the Securities and Exchange Commission. 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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