News Release

MyoKardia Publishes Article in Science Demonstrating That MYK-461 Prevents and Reverses Disease in Genetic Mouse Models of Hypertrophic Cardiomyopathy

Supports Therapeutic Hypothesis Linking Reduction in Sarcomere Power Output to Improvements in Structural Pathology of HCM

SOUTH SAN FRANCISCO, Calif., Feb. 04, 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 the publication of an article in the leading medical journal *Science.* The article demonstrates the ability of MYK-461, the company's lead drug candidate, to prevent and reverse development of disease in multiple genetic mouse models of hypertrophic cardiomyopathy (HCM). The published research represents the product of collaboration among scientists from MyoKardia, Harvard Medical School, the University of Colorado and Stanford University. These data add to a growing body of laboratory and clinical research demonstrating the potential of MYK-461 as an important and novel approach to treating HCM.

The study, titled "A Small-Molecule Inhibitor of Sarcomere Contractility Suppresses Hypertrophic Cardiomyopathy in Mice," will be published in the Feb. 5 issue of the journal *Science.*

"I am encouraged by these data that illustrate MYK-461's ability to effectively reduce the consequences of HCM mutations at the biochemical, cellular and whole animal levels," said Jonathan Fox, M.D., Ph.D., chief medical officer of MyoKardia. "Translation of these findings from mouse to human could offer great potential to improve the lives of patients living with this devastating disease."

To study the role of sarcomere mutations in the development of HCM, MyoKardia used previously generated mouse models of HCM, which recapitulate key morphologic and functional features of human HCM. To quantify the level of left ventricular hypertrophy, the cardinal manifestation of HCM, the researchers noninvasively measured left ventricular wall thickness (LVWT).

MyoKardia researchers and their collaborators demonstrated that early and chronic administration of MYK-461 could prevent the development of disease. Compared to the characteristic increase in LVWT observed in untreated mutant mice, no increase in LVWT was observed in mutant mice treated with MYK-461. The research also showed that MYK-461 promoted partial reversal of disease, as shown by a measurable decline, upon administration of MYK-461, of LVWT in HCM mice that had already developed hypertrophy. Furthermore, the research showed that MYK-461 could prevent the development of left ventricular fibrosis, which is an important histopathological feature of HCM and causally implicated in other potentially dangerous heart conditions.

"We are encouraged by these findings, which support our therapeutic hypothesis that reduction in contractility can prevent or reverse the abnormalities in structure and function leading to symptoms that afflict patients living with HCM," said Tassos Gianakakos, chief executive officer of MyoKardia. "We believe that MYK-461 is the first drug in clinical development to achieve either of these effects. This approach could represent a more general paradigm for the treatment of heritable cardiomyopathies and may bring us closer to our ultimate goal, to improve the lives of patients and their families with this debilitating disease."

MyoKardia is currently evaluating MYK-461 in three Phase 1 clinical trials, which are primarily designed to evaluate the safety and tolerability of MYK-461 and are expected to provide data on its pharmacokinetic and pharmacodynamic profile.

The paper will be published in the Feb. 5 issue of *Science* and can be viewed online

at http://www.sciencemag.org/magazine .

About Hypertrophic Cardiomyopathy (HCM)
HCM is the most common form of heritable cardiomyopathy. 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. HCM 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 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 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 .

About *Science* Magazine
Science has been at the center of important scientific discovery since its founding in 1880—with seed money from Thomas Edison. Today, *Science* continues to publish the very best in research across the sciences, with articles that consistently rank among the most cited in the world. The *Science* family of journals is published by the American Association for the Advancement of Science (AAAS), the world's oldest and largest general science organization. The nonprofit AAAS serves 10 million people through primary memberships and affiliations with some 262 scientific societies and academies.

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 and the Company's ability to generate data from its Phase 1 clinical trials of MYK-461 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 most recent Quarterly Report on Form 10-Q. 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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