News Release

MyoKardia Doses First Patient in MAVA Long-Term Extension Study of Mavacamten for Hypertrophic Cardiomyopathy

Key Remaining Study in Registration Program for oHCM

Enables Assessment of Mavacamten's Potential for Disease Modification

SOUTH SAN FRANCISCO, Calif., Oct. 24, 2018 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq: MYOK), a clinical-stage biopharmaceutical company pioneering precision medicine for the treatment of cardiovascular diseases, today announced that patient dosing has begun in the MAVA Long-Term Extension (MAVA-LTE) clinical trial. The MAVA-LTE study will assess long-term safety of mavacamten, as well as its effects on symptoms and echocardiographic measures of systolic and diastolic cardiac function in patients with hypertrophic cardiomyopathy (HCM). MAVA-LTE will serve as the key remaining study in the registration program for mavacamten in obstructive HCM (oHCM).

"The MAVA-LTE study is expected to be a critical component of mavacamten's anticipated registration package, providing important information regarding the long-term safety and efficacy of mavacamten in HCM," said June Lee, M.D., MyoKardia's Chief Operating Officer and Chief Development Officer. "Several adverse clinical outcomes in HCM are associated with structural changes in the heart, including elevated risk of heart failure, cardiac arrest and atrial fibrillation. By reducing the excessive contractility that is the key driver of HCM, we believe mavacamten treatment may remodel the heart over time. The duration of MAVA-LTE and the incorporation of a cardiac magnetic resonance imaging substudy into its protocol will allow us to investigate potential structural improvements to the diseased HCM heart that occur with daily mavacamten treatment."

Up to 280 patients who successfully complete either MyoKardia's MAVERICK-HCM or EXPLORER-HCM clinical trials of mavacamten will be eligible for enrollment into MAVA-LTE. Approximately 100 patients are expected to participate in a cardiac magnetic resonance imaging substudy to assess the potential effects of mavacamten treatment on cardiac mass and structure. The treatment period for patients in MAVA-LTE will be up to two years from the time of their enrollment and through the planned registration filing with the U.S. Food and Drug Administration (FDA) for the potential regulatory approval of mavacamten. Data from the MAVA-LTE clinical trial, along with results of the EXPLORER-HCM trial, are intended to support the registration submission of mavacamten for the treatment of oHCM.

All patients in MAVA-LTE will receive mavacamten in line with the active cohort protocol from their respective study. Study participants' treatment status (active or placebo) from MAVERICK-HCM or EXPLORER-HCM will remain blinded. Patients in MAVA-LTE may maintain pre-existing background medications for HCM, such as beta blockers or calcium channel blockers throughout the course of the trial.

About Mavacamten (MYK-461)

Mavacamten is a novel, oral, allosteric modulator of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). MyoKardia has advanced mavacamten into a pivotal Phase 3 clinical trial, known as the EXPLORER-HCM study, in patients with symptomatic, obstructive HCM and a Phase 2 clinical trial, the MAVERICK-HCM study, in patients with symptomatic non-obstructive HCM. Mavacamten is intended to reduce cardiac muscle contractility by inhibiting the excessive myosin-actin cross-bridge formation that underlies the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. In April 2016, the U.S. FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic oHCM, a subset of HCM. Mavacamten is being developed in an ongoing collaboration between MyoKardia and Sanofi.

About Hypertrophic Cardiomyopathy
Hypertrophic cardiomyopathy (HCM) is the most common genetic cause of heart disease, in which the walls of the heart thicken and prevent the left ventricle from expanding, resulting in a reduced pumping capacity. HCM is a chronic, progressive disease that can be extremely disabling. According to recent research published in the journal *Circulation*[(1)], HCM patients are at substantially elevated risks of long-term complications and comorbidities, such as atrial fibrillation and heart failure. HCM patients also have significantly higher mortality rates compared to that of the general U.S. population.

In approximately two-thirds of HCM patients, or an estimated 410,000 people in the U.S., the path followed by blood exiting the heart, known as the left ventricular outflow tract (LVOT), becomes obstructed by the enlarged and diseased muscle, restricting the flow of blood from the heart to the rest of the body. Mild exertion can quickly result in fatigue or shortness of breath, and a patient's ability to participate in normal work, family or recreational activities can be substantially curtailed. Approximately one-third of patients, or 220,000 people in the U.S., have non-obstructive HCM. As nHCM progresses, symptoms begin to resemble those of a congestive heart failure patient and heart transplantation may become the only viable treatment option.

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 two of the most prevalent forms of heritable cardiomyopathy – hypertrophic cardiomyopathy (HCM), and dilated cardiomyopathy (DCM). MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461), a novel, oral, allosteric modulator of cardiac myosin intended to reduce hypercontractility. Mavacamten has advanced into a pivotal Phase 3 clinical trial, known as EXPLORER-HCM in patients with symptomatic, obstructive HCM. MyoKardia is also developing mavacamten in a second indication, non-obstructive HCM, in the Phase 2 MAVERICK clinical trial. MYK-491, MyoKardia's second product candidate, is designed to increase cardiac output in DCM patients by increasing the overall extent of the heart's cardiac contractility. MyoKardia is currently evaluating MYK-491 in a Phase 1b study in DCM patients. A cornerstone of the MyoKardia platform is the Sarcomeric Human Cardiomyopathy Registry

(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's mission is to change the world for patients with serious cardiovascular disease through bold and innovative science.

[(1)] Ho, et al, *Circulation* 2018

Forward-Looking Statement
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 mavacamten, the initiation of patient dosing in the MAVA-LTE trial, mavacamten's ability to achieve applicable endpoints in the MAVA-LTE trial, the availability of data from the MAVA-LTE trial, the potential for data from the Company's clinical trials of mavacamten to support a marketing application, as well as 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, 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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