News Release

MyoKardia Doses First Patient in PIONEER Open-Label Extension Study of Mavacamten for Symptomatic, Obstructive Hypertrophic Cardiomyopathy

SOUTH SAN FRANCISCO, Calif., May 10, 2018 (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 start of the PIONEER open-label extension (OLE) study of its investigational drug, mavacamten. MyoKardia recently completed the Phase 2 PIONEER-HCM clinical study of mavacamten in patients with symptomatic, obstructive hypertrophic cardiomyopathy (oHCM). The open-label extension study will enroll patients who previously completed the Phase 2 PIONEER-HCM trial.

“Following the encouraging results observed in the Phase 2 PIONEER-HCM study of mavacamten, many of the patients who participated in the study expressed a strong interest in continuing treatment,” said Dr. Stephen Heitner, cardiologist at the Oregon Health & Science University Knight Cardiovascular Institute and clinical investigator for the PIONEER-HCM and PIONEER-OLE studies. “Symptomatic oHCM patients are frequently limited by their disease from participating fully in daily activities, so the prospect of a treatment such as mavacamten that appears to address the biomechanical underpinnings of disease and provide an improvement in how patients feel and function represents an important potential breakthrough in HCM treatment.”

The PIONEER-OLE study is intended to provide data on longer-term exposure to mavacamten. Patients will receive an individualized daily dose of 5mg, 10mg, or 15 mg of mavacamten, with a dose adjustment at week 6 based on pharmacodynamic and pharmacokinetic criteria, as well as the patient’s past experience with mavacamten. MyoKardia anticipates reporting the first interim data update from the PIONEER-OLE study in the first quarter of 2019.

“We are excited to offer this extension study for the patients who participated in PIONEER and we are very appreciative of their commitment and willingness to continue on this journey with us to test mavacamten in oHCM,” said Marc Semigran, M.D., Chief Medical Officer. “This open label extension study will provide us with additional data on mavacamten’s longer-term safety, tolerability and efficacy. As we advance mavacamten into our planned Phase 3 EXPLORER-HCM pivotal trial this quarter, the

ability to learn from the experience of our PIONEER patients in this open-label extension study will provide valuable insights on mavacamten's continued use over time."

In MyoKardia's Phase 2 PIONEER-HCM clinical trial, twenty patients with symptomatic oHCM were treated with mavacamten for twelve weeks followed by a four-week washout period. Primary and secondary endpoints were achieved across key signs and symptoms of disease as measured from baseline to week twelve, such as reduction in left ventricular outflow tract (LVOT) gradient post-exercise and at rest, increased exercise capacity as measured by peak VO_2, improved New York Heart Association (NYHA) classification and reduced dyspnea over time. Mavacamten has been generally well tolerated in multiple clinical trials.

About Obstructive HCM
Hypertrophic cardiomyopathy 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 disease and for the majority of patients, the disease progresses slowly and can be extremely disabling. 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. Patients with oHCM are at an increased risk of severe heart failure and death. HCM can also cause stroke or sudden cardiac death.

About Mavacamten (MYK-461)
Mavacamten is a novel, oral, allosteric modulator of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). MyoKardia is currently advancing 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 crossbridge 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 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 is advancing 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 contraction 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.

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 mavacamten, the outcome of the PIONEER-OLE trial, and the initiation of patient dosing in the EXPLORER-HCM trial, 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 March 31, 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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