MyoKardia Announces Exploratory Digital Health Substudy as Part of PIONEER-HCM Trial of MYK-461 in Symptomatic, Obstructive Hypertrophic Cardiomyopathy Patients

Trial to Assess Potential Effects of MYK-461 Treatment on Arterial Pulse Wave Morphology as Measured by Investigational Optical Biosensor Wristband

SOUTH SAN FRANCISCO, Calif., Nov. 11, 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 an exploratory digital health substudy that is under way as part of the Company's PIONEER-HCM trial of MYK-461 in symptomatic, obstructive hypertrophic cardiomyopathy (oHCM) patients.

The digital health exploratory objective of the trial assesses the potential effects of MYK-461 on the arterial pulse wave using an investigational photoplethysmography (PPG) biosensor. Data collected with the biosensor will be compared with traditional measures of disease. Abnormal arterial flow patterns have been previously observed in HCM patients using conventional tools such as echocardiography. This substudy will investigate whether a non-invasive investigational PPG wristband, similar to commercially available digital health wearables, could provide information to supplement standard measures and support development of a digital biomarker for treating oHCM patients.

Use of the investigational PPG wristband is part of a partnership between MyoKardia and Wavelet Health, a wearable technology platform for health research. The wristband shines beams of light onto the skin to measure patterns of blood flow in the wrist with each heartbeat. Data from the PPG signal will be analyzed with Wavelet's proprietary algorithms to characterize the blood flow patterns of the oHCM patients in the study.

"Wearable devices will likely play a transformative role in how we gather, analyze and interpret medical information," said Steven Steinhubl, M.D., director of digital medicine for Scripps Translational Science Institute. "Early investigational research in this field is an important first step in seeking validation of their considerable potential."

MyoKardia anticipates that the technology in this study, while investigational, could be applied in longitudinal studies to provide insight into patient physiology during daily activities as well as potential response to treatment.

"One of MyoKardia's core values is Imagine and Innovate, which extends beyond the discovery of much needed therapies and includes novel applications of digital health to our target disease areas," said Tassos Gianakakos, chief executive officer. "The digital health substudy is aligned with our precision medicine approach and may provide

important insights for clinicians and researchers by deepening our understanding of the HCM patient's journey."

PIONEER-HCM is a Phase 2 study assessing the effect of multiple doses of MYK-461 in symptomatic oHCM patients. The primary endpoint is the level of reduction in post-exercise left ventricular outflow tract (LVOT) gradient over 12 weeks of drug treatment. PIONEER-HCM will also explore the relationship between reduction in contractility and LVOT gradient, endpoints measuring functional capacity (i.e., exercise) and clinical symptoms in addition to gathering safety and tolerability data on MYK-461 in an outpatient setting. Topline data from PIONEER-HCM are expected in the second half of 2017.

About MYK-461 and PIONEER-HCM

MYK-461 is an orally administered small molecule designed to reduce left ventricular contractility by allosterically modulating the function of cardiac myosin, the motor protein that drives heart muscle contraction. MyoKardia has evaluated MYK-461 in three Phase 1 clinical trials, primarily designed to evaluate safety and tolerability of oral doses of MYK-461, as well as provide pharmacokinetic and pharmacodynamic data. In April 2016, the U.S. FDA granted Orphan Drug Designation for MYK-461 for the treatment of symptomatic oHCM, a subset of HCM.

MyoKardia is currently studying MYK-461 in PIONEER-HCM, a Phase 2 open-label single-arm study to evaluate safety, tolerability and efficacy of MYK-461 in patients with symptomatic oHCM. The primary endpoint of PIONEER-HCM is the level of reduction in post-exercise left ventricular outflow tract (LVOT) gradient over 12 weeks of drug treatment. PIONEER-HCM will also explore the relationship between reduction in contractility and LVOT gradient, endpoints measuring functional capacity (i.e., exercise) and clinical symptoms in addition to gathering safety and tolerability data on MYK-461 in an outpatient setting.

About HCM and oHCM

It is estimated that one in every 500 people in the United States has HCM, the most prevalent form of heritable cardiomyopathy. HCM is defined as an otherwise unexplained thickening of the walls of the heart, known as hypertrophy. The consequences include reduced left ventricular volumes and cardiac output, reduced ability of the left ventricle to expand, and elevated 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 substantially increases the risk of developing atrial fibrillation that can lead to stroke or malignant ventricular arrhythmias that can cause sudden cardiac death. There are currently no approved drug 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.

oHCM is a physiological complication of HCM in which the thickened heart muscle obstructs the LVOT. Approximately two thirds of all HCM patients have obstruction, either at rest or with provocation like exercise. Measured most commonly by non-invasive imaging (echocardiography), oHCM is defined as ≥30 mm Hg pressure gradient across the LVOT. Symptoms of oHCM can include shortness of breath, chest pain, dizziness, fainting, and palpitations. The presence of obstruction in an HCM patient further increases risk of progression to severe symptoms, and risk of death from heart failure or stroke.

The degree of LVOT obstruction in oHCM patients is a primary criterion for surgical and other invasive interventions (recommended for symptomatic patients with LVOT gradients measured at ≥50 mmHg). Relief of obstruction has been associated with improved symptoms, function and clinical outcomes. Surgical or other invasive interventions, including septal myectomy, an open heart procedure, may be appropriate. There are no approved drug products indicated for this condition. The primary endpoint of the Phase 2 PIONEER-HCM study is to assess level of reduction in LVOT gradient over 12 weeks of drug treatment. The trial is also exploring relationships among reductions in contractility, LVOT gradient and endpoints that include safety, tolerability, functional capacity and clinical symptoms.

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 now studying MYK-461 in a Phase 2 PIONEER-HCM trial in symptomatic oHCM, for which the FDA has granted MYK-461 Orphan Drug Designation. 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, the Company’s ability to generate topline data from its Phase 2 PIONEER-HCM study and the timing of receipt of such data, the anticipated clinical endpoints and pathway to approval for MYK-461 and the scope and significance of the data expected to be gathered from the exploratory digital health substudy, 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.

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