MyoKardia Presents Data from Study of Machine Learning Algorithm Intended to Identify Obstructive Hypertrophic Cardiomyopathy Using a Wearable Biosensor

Digital Health Data from Substudy of PIONEER-HCM Presented in Late-Breaker at American Heart Association Scientific Sessions

SOUTH SAN FRANCISCO, Calif., Nov. 13, 2017 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq:MYOK), a clinical-stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, presented results from testing a novel machine learning algorithm intended to identify patients with obstructive hypertrophic cardiomyopathy (oHCM) during a late-breaker session at the American Heart Association (AHA) Scientific Sessions.

MyoKardia designed an exploratory digital health substudy as part of the Phase 2 PIONEER-HCM trial of mavacamten (formerly MYK-461) to determine if an optical biosensor, similar to those that monitor heart rate on commercially available fitness trackers, could identify patients with oHCM. Using an investigational wrist-worn photoplethysmography (PPG) digital health device, arterial pulse wave patterns of oHCM patients were shown to be distinct from those of individuals without oHCM. After training and cross-validation, MyoKardia's proprietary machine learning algorithm identified individuals with oHCM with 95 percent accuracy, with a sensitivity of 0.95 and a specificity of 0.95.

"Hypertrophic cardiomyopathy affects approximately 630,000 people in the U.S., but an estimated eighty-five percent of patients go undiagnosed. Use of a wrist-worn digital health device to identify irregular blood flow patterns and screen patients for disease as they go about their daily activities could have a remarkable impact on the diagnosis and treatment of HCM, particularly given the potential health risks for patients unaware of their condition," said Marc Semigran, M.D., Chief Medical Officer of MyoKardia.

"We believe the data obtained in this study provide proof-of-principle for the possible use of wearable PPG technology to screen for obstructive hypertrophic cardiomyopathy," said Charles Wolfus, Executive Director, Digital Health, Technology and Business Operations at MyoKardia. "These results lend themselves to further testing of our machine learning algorithms, with the potential to expand its application to different wrist-worn wearable platforms, to monitor for treatment effects in oHCM patients, and to screen for other types of cardiomyopathy."

About MyoKardia's Digital Health Substudy
Using an investigational wearable PPG device developed by Wavelet Health, arterial pulse wave data were collected at baseline from 19 oHCM patients enrolled in MyoKardia's PIONEER-HCM clinical trial. A beat-by-beat machine learning model was developed to identify digital signatures of oHCM. The pulse wave patterns of oHCM patients were then analyzed against comparable patterns from a database of 86

volunteers. The pulse wave patterns of patients with oHCM were consistent with the turbulent blood flow and heart rate variability associated with obstruction of the left ventricular outflow tract (LVOT) characteristic of oHCM.

These data were the subject of today's late-breaking basic science oral session in a presentation titled "*Machine Learning Detection of Obstructive Hypertrophic Cardiomyopathy Using a Wearable Biosensor*" (Abstract #24031).

About Hypertrophic Cardiomyopathy
Hypertrophic cardiomyopathy (HCM) is a genetic condition in which the walls of the heart thicken and prevent the left ventricle from expanding, resulting in a reduced pumping capacity. HCM is a progressive disease that can result in shortness of breath, chest pain, inability to participate in normal activities, disabling heart failure and even stroke. HCM is the leading cause of sudden cardiac death in young people. Obstructive HCM (oHCM) is a form of HCM characterized by a dynamic obstruction of the LVOT that results in abnormalities in arterial blood flow. HCM is typically diagnosed by echocardiogram to visually identify the thickening of the heart wall, anatomical blood flow obstruction and heart valve movement.

About Mavacamten (MYK-461)
Mavacamten is a novel, oral, allosteric modulator of cardiac myosin that reduced hypercontractility in a Phase 1 clinical study of hypertrophic cardiomyopathy (HCM) patients. MyoKardia has evaluated mavacamten in multiple Phase 1 clinical studies, primarily designed to evaluate safety and tolerability of oral doses of mavacamten, and provide pharmacokinetic and pharmacodynamic data. In April 2016, the U.S. FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic oHCM, a subset of HCM. MyoKardia is currently studying mavacamten in the Phase 2 PIONEER-HCM study.

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 (HCM), and dilated cardiomyopathy (DCM). MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461), a novel, oral, allosteric modulator of cardiac myosin that has been shown to reduce hypercontractility in early clinical studies and is currently being studied in the Phase 2 PIONEER-HCM clinical trial. MYK-491, MyoKardia's second product candidate, is designed to increase the overall extent of the heart's contraction in DCM patients by increasing cardiac contractility. MyoKardia is currently evaluating MYK-491 in a Phase 1 study in healthy volunteers. 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.

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