MyoKardia Provides Update on Two Phase 1 Trials of MYK-461 for the Treatment of Hypertrophic Cardiomyopathy

Clinical Proof of Mechanism Demonstrated in Both Healthy Volunteers and Patients

SOUTH SAN FRANCISCO, Calif. – October 6, 2015 – MyoKardia, Inc., a clinical stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, today announced initial clinical data from two Phase 1 trials of MYK-461, the Company's lead product candidate, which targets the underlying cause of hypertrophic cardiomyopathy (HCM). In both trials, MYK-461 was well tolerated with dose-proportional pharmacokinetics. MYK-461 has demonstrated clinical proof of mechanism in reducing cardiac muscle contractility, an important biomarker of disease.

In the first-in-human, double-blind, placebo-controlled trial evaluating single oral doses in 48 healthy adult volunteers (36 active, 12 placebo), MyoKardia has completed dosing in six cohorts at doses of up to 48 mg of MYK-461 or matching placebo. Clinical proof of mechanism was demonstrated at doses of 12 mg and above by dose-dependent pharmacodynamic activity, as assessed by three different echocardiographic biomarkers of contractility. MYK-461 was well tolerated at all dose levels and there were no serious adverse events or clinically meaningful findings in vital signs, electrocardiogram recordings or safety laboratory tests. MYK-461 demonstrated a dose-linear and dose-proportional pharmacokinetic profile, with low inter-subject variability.

Additionally, MyoKardia initiated an open label, single ascending dose trial of MYK-461 in adult patients with HCM. Observations from the first two patients dosed at 48 mg were similar to those from healthy volunteers treated at the same dose.

"I am encouraged by the data disclosed today on the demonstration of proof of mechanism for MYK-461 across a range of doses with a favorable safety profile. The approach of addressing the underlying genetic cause of the disease has great potential to make a meaningful difference for patients suffering from this condition," said Sharlene M. Day, M.D., associate professor and director, Program for Inherited Cardiomyopathies at the University of Michigan and a participant in the MYK-461 investigational program.

About MYK-461

MYK-461 is an orally administered small molecule that reduces left ventricular contractility. MyoKardia is currently evaluating MYK-461 in three Phase 1 clinical trials, which are primarily designed to evaluate safety and tolerability of oral doses of MYK-461 and are expected to provide data on its pharmacokinetic and pharmacodynamics profile. These studies assess MYK-461's engagement of cardiac myosin by measuring reduction in cardiac muscle contractility via echocardiography.

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, and 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 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.

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