

MyoKardia Provides Update on MYK-491 Clinical Progress

Single Ascending Dose Trial in Healthy Volunteers Continues Enrollment in Order to Refine Exposure-Activity-Relationship; Topline Data Expected by Early 2018

Next Trial of MYK-491 in DCM Patients Expected to Initiate by Year-End

SOUTH SAN FRANCISCO, Calif., Sept. 27, 2017 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq:MYOK) ("MyoKardia"), a clinical stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, today provided a clinical update on its MYK-491 program for dilated cardiomyopathy (DCM).

"We are encouraged by the favorable safety and tolerability profile observed thus far in our Phase 1 trial of MYK-491 and look forward to sharing topline data in the coming months," said Marc Semigran, M.D., chief medical officer of MyoKardia. "These additional dose cohorts in the 001 study may allow us to gain further insights into relationships among MYK-491 dosing, exposure and activity seen to date. Data from this healthy volunteer trial, along with anticipated data from our upcoming patient trial, will best position us to advance this program into Phase 2 and later-stage trials."

Single Ascending Dose Trial in Healthy Volunteers (001 Trial)

Earlier this year, MyoKardia initiated a Phase 1 single ascending dose trial of MYK-491 in healthy volunteers. The objectives of this randomized, placebo-controlled trial are to assess safety, tolerability, preliminary pharmacokinetics and pharmacodynamics of MYK-491.

Preliminary safety data available from this ongoing trial show that MYK-491 has been generally safe and well-tolerated in all dose cohorts tested thus far. Based on results observed to date, MyoKardia has elected to include additional dose cohorts in the trial in order to further refine its understanding of MYK-491 pharmacokinetics and pharmacodynamics. Topline data from the 001 trial are now expected to be released by early 2018.

Single Ascending Dose Trial in Symptomatic DCM Patients (003 Trial)

MyoKardia intends to initiate a single ascending dose trial of MYK-491 in symptomatic DCM patients before year-end. The objectives of this randomized, double-blind, placebo-controlled trial are to assess safety, tolerability, preliminary pharmacokinetics and pharmacodynamics of MYK-491 in patients.

About MYK-491

MYK-491 is an oral, small molecule, allosteric activator of myosin designed to increase cardiac contractility in a DCM heart. Reduced cardiac contractility, or hypocontractility, is believed to be the underlying cause of DCM and MYK-491 is designed to increase cardiac muscle contractility. Based on preclinical research across multiple animal models, MYK-491 may hold potential for controlled increases in the heart's contractility with minimal impact on diastole or relaxation. MYK-491 is being studied in a Phase 1 single ascending dose trial in healthy volunteers.

About DCM

DCM is a disease that affects about one million people in the United States. DCM can progress to heart failure and severe complications such as stroke, arrhythmias, and sudden cardiac death. In DCM, the walls of the left ventricle are thin and over-expanded, leading to diminished contraction and insufficient blood being pumped by the heart. Typical symptoms include shortness of breath, fatigue, swelling in the extremities, or an irregular heartbeat. As the disease progresses, patients become increasingly debilitated, experience persistent shortness of breath, even at rest, and are at elevated risk for fatal arrhythmias. Diastolic function, or the heart's ability to relax and fill with blood, is also impaired. There is currently no approved medical therapy that addresses the underlying biomechanical causes of DCM.

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). Mavacamten is a novel, oral, allosteric modulator of cardiac myosin that reduced hypercontractility in Phase 1 clinical trials of HCM patients. In April 2016, the FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic oHCM, a subset of HCM. MyoKardia is currently studying mavacamten in PIONEER-HCM, a Phase 2 open-label trial of mavacamten in patients with symptomatic oHCM. 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 trial 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.

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-491, the Company's ability to expand its Phase 1 single ascending dose trial of MYK-491 in healthy volunteers into additional dose cohorts and to generate topline data from this trial, its ability to initiate its planned Phase 1 single ascending dose trial of MYK-491 in symptomatic DCM patients, and 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, 2017, 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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