MyoKardia Provides Clinical Trial Update in the Context of the COVID-19 Pandemic

March 26, 2020

Reaffirms Second Quarter 2020 Expected Timing for Topline Data from Phase 3 EXPLORER-HCM Clinical Trial of Mavacamten and Phase 2a Danicamtiv Study

BRISBANE, Calif., March 26, 2020 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq: MYOK) today provided an update to the status of its clinical programs in the context of the COVID-19 (coronavirus) pandemic given the impact of the pandemic on the global healthcare system's present ability to support the conduct of clinical trials.

At this time, the company does not foresee changes to key clinical and regulatory milestones associated with the development of mavacamten for obstructive hypertrophic cardiomyopathy (HCM), including reporting topline data from the Phase 3 EXPLORER-HCM study in the second quarter of 2020. Enrollment in EXPLORER-HCM concluded in August 2019 and as of this month, all patient visits have been conducted through the 30-week treatment period. Similarly, enrollment and patient dosing in the Phase 2a multiple-ascending dose trial of danicamtiv in patients with stable heart failure is complete, and MyoKardia still expects to report results from this study in the second quarter of 2020. MyoKardia has temporarily suspended enrollment in two ongoing studies and new clinical trials planned to commence in the second quarter of 2020 will be delayed.

"The COVID-19 pandemic reminds us of the vital importance of our clinical communities, our commitment to our patients and our collective responsibility to support each other. Our primary focus remains on the safety and well-being of our study participants, clinical investigators and their site staffs, as well as enabling the healthcare professionals in our network to focus critical resources on those affected by COVID-19," said Tassos Gianakakos, MyoKardia's Chief Executive Officer. "We are learning that people with HCM and DCM are among the most at risk from COVID-19 infections and have put in place several measures intended to protect our study participants, as well as the investigators, site coordinators and support staff who care for them. We are suspending enrollment in certain ongoing clinical trials and will not initiate new studies until we can proceed safely, without unnecessarily adding risk to study participants or site staff, as part of our efforts to lighten the incredible burden facing the healthcare system."

The company, together with its steering committees, patient advocacy and other expert clinical advisors, assessed each of its clinical programs with the goals of protecting the safety of study participants, investigators and staff, and ensuring consistent and appropriate clinical trial conduct. All efforts will be made to allow patients to continue in ongoing mavacamten studies, including the MAVA-LTE long-term extension and the PIONEER open-label extension (OLE) studies. MyoKardia has temporarily suspended the rollover of patients from EXPLORER into the MAVA-LTE and plans to resume enrollment when conditions permit. The company has also paused the enrollment of healthy volunteers in the Phase 1 clinical trial evaluating MYK-224.

Studies that were planned to initiate in the second quarter of 2020 will be delayed until conditions change, including the VALOR-HCM Phase 3 clinical study of mavacamten as an alternative to septal reduction therapy (SRT) procedures, the Phase 2 proof-of-concept study for mavacamten in subgroups of patients with heart failure with preserved ejection fraction (HFpEF), and the Phase 2 study of danicamtiv in patients with genetic dilated cardiomyopathy (DCM).

MyoKardia will continue to closely monitor the evolving situation and expects to resume patient enrollment and to initiate delayed studies as soon as conditions safely permit.

About MyoKardia

MyoKardia is a clinical-stage biopharmaceutical company discovering and developing targeted therapies for the treatment of serious cardiovascular diseases. The company is pioneering a precision medicine approach to its discovery and development efforts by 1) understanding the biomechanical underpinnings of disease; 2) targeting the proteins that modulate a given condition; 3) identifying patient populations with shared disease characteristics; and 4) applying learnings from research and clinical studies to inform and guide pipeline growth and product advancement. MyoKardia's initial focus is on small molecule therapeutics aimed at the proteins of the heart that modulate cardiac muscle contraction to address diseases driven by excessive contraction, impaired relaxation, or insufficient contraction. Among its discoveries are three clinical-stage therapeutics: mavacamten (formerly MYK-461); danicamtiv (formerly MYK-491) and MYK-224.

MyoKardia's mission is to change the world for people 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 our product candidates, the conduct of, and our anticipated announcement of topline data and complete results from, our Phase 3 EXPLORER-HCM trial of mavacamten for the treatment of obstructive HCM; our plans to present data from our Phase 2 MAVERICK-HCM study of mavacamten for the treatment of non-obstructive HCM; the conduct of our PIONEER-OLE trial of mavacamten; the conduct of, and our plans to resume enrollment in, our MAVA-LTE study; the initiation of our planned VALOR-HCM Phase 3 clinical study of mavacamten as an alternative to septal reduction therapy (SRT) procedures and our Phase 2 proof-of-concept study for mavacamten in subgroups of patients with heart failure with preserved ejection fraction (HFpEF); the conduct of, and our planned announcement of complete results from our Phase 2a multiple-ascending dose trial of danicamtiv in patients with stable heart failure; our plans to resume enrollment in our Phase 1 clinical trial of MYK-224 in healthy volunteers and to initiate a Phase 2 study of MYK-224 in HCM patients; and our plans to consult with the FDA and to provide a regulatory update, 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 Annual Report on Form 10-K for the year ended December 31, 2019, 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.

Contacts

Michelle Corral
Executive Director, Corporate Communications and Investor Relations
MyoKardia, Inc.
650-351-4690
ir@myokardia.com

Hannah Deresiewicz (Investors)
Stern Investor Relations, Inc.
212-362-1200
hannah.dereseiwicz@sternir.com

Julie Normart (Media)
W2O
628-213-3754
jnormart@w2ogroup.com

Source: MyoKardia, Inc.