MYOKARDIA

News Release

MyoKardia Announces HCM Program Updates: Accelerates Timing for Mavacamten Topline Phase 3 Data; Re-acquires U.S. Royalty Rights to HCM Programs from Sanofi

EXPLORER-HCM Patient Screening Closes Ahead of Schedule – Initial Results Now Anticipated in the Second Quarter of 2020

Royalty Purchase Creates Additional Economic Value for MyoKardia's Emerging HCM Treatment Portfolio

SOUTH SAN FRANCISCO, Calif., July 18, 2019 -- MyoKardia, Inc. (Nasdaq: MYOK) today announced updates related to its hypertrophic cardiomyopathy (HCM) treatment portfolio, including lead therapeutic candidate, mavacamten.

Patient screening has closed for the Phase 3 EXPLORER-HCM registrational clinical study to assess the effect of mavacamten in treating patients with obstructive hypertrophic cardiomyopathy (oHCM). Clinical site engagement remains high, and enrollment into the pivotal trial is expected to be completed by mid-August. MyoKardia now anticipates reporting topline data from the EXPLORER-HCM trial in the second quarter of 2020, ahead of previous guidance of the second half of 2020.

"The enthusiasm for mavacamten, along with the hard work and partnership of the EXPLORER-HCM clinical sites and our MyoKardia team, enabled this first-of-its-kind study to enroll ahead of expectations," said Tassos Gianakakos, MyoKardia's Chief Executive Officer. "We are greatly appreciative of the investigators, support staff and the oHCM patients and their families who have participated in this pioneering study. The data generated from our mavacamten program to date indicate that this first-in-class medicine has the potential to provide substantial benefit to individuals with symptomatic, obstructive HCM."

MyoKardia also announced the re-acquisition of U.S. royalty rights to mavacamten and MYK-224 from Sanofi S.A. (Sanofi). As consideration for the buyback of the U.S. royalty rights to these programs, MyoKardia is paying Sanofi $50 million upfront, with an additional $30 million payable by June 30, 2020. Under the terms of the former license and collaboration agreement with Sanofi, Sanofi was eligible for tiered royalties, ranging from 5 percent to 10 percent, on U.S. sales of mavacamten and MYK-224 in HCM or any additional indications.

"Regaining U.S. royalty rights for mavacamten and MYK-224 allows us to capture the economic value of our HCM therapies on a global basis and increases our strategic flexibility and control over their development and commercialization," Mr. Gianakakos said. "This was an important strategic step for us as we look ahead to mavacamten's potential registration and commercial launch in the U.S. and the imminent advancement of MYK-224 into clinical studies."

MyoKardia's cash guidance remains unchanged. The company anticipates that current cash, cash equivalents and investments will be sufficient to fund planned operations into the second half of 2021. The company will announce second quarter financial results on August 7, 2019.

About EXPLORER-HCM Trial

Mavacamten is currently being tested in the pivotal, Phase 3 EXPLORER-HCM clinical trial for the treatment of symptomatic, obstructive HCM. HCM is the most common form of genetic heart disease. It is a chronic, progressive condition estimated to affect one in every 500 people worldwide and is characterized by the thickening of the heart walls due to excessive contraction. EXPLORER-HCM is a multi-national, randomized, double-blind study that will enroll more than 220 patients. Patients will be randomized 1:1 to

receive mavacamten or placebo for 30 weeks with a primary endpoint of clinical response designed to assess improvements in patient symptoms and function. Topline data from the Phase 3 EXPLORER-HCM trial is anticipated in the second quarter of 2020.

About the MyoKardia-Sanofi Collaboration
MyoKardia and Sanofi entered into a multi-product collaboration in 2014 focused on developing novel treatments for cardiomyopathies, including HCM. The companies discontinued that agreement in conjunction with the end of the Research term of the collaboration in December 2018 and the collaboration agreement with Sanofi concluded in its entirety on April 1, 2019. Over the course of the collaboration, MyoKardia received approximately $230 million in funding from Sanofi, advanced mavacamten from preclinical development into a late-stage pivotal study for the treatment of HCM, and MYK-491 from discovery to a Phase 2 proof-of-concept study in patients with diastolic cardiomyopathy (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 cardiovascular diseases. MyoKardia's initial focus is on the development of small molecule therapeutics aimed at the cardiac muscle proteins that modulate cardiac muscle contraction and underlie diseases of systolic and diastolic dysfunction. Based on an in-depth understanding of disease biology, MyoKardia applies a precision medicine approach to develop its therapeutic candidates for patient populations with shared characteristics, such as causal genetic mutations or disease subtypes. MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461), a novel, oral, allosteric modulator of cardiac myosin intended to reduce hypercontractility. Mavacamten has advanced into a pivotal Phase 3 clinical trial, known as EXPLORER-HCM in patients with symptomatic, obstructive hypertrophic cardiomyopathy (HCM). MyoKardia is also developing mavacamten in a second indication, non-obstructive HCM, in the Phase 2 MAVERICK-HCM clinical trial. MYK-491, MyoKardia's second product candidate, is designed to increase cardiac output among patients with systolic heart dysfunction by increasing the overall extent of the heart's cardiac contractility. MyoKardia is currently evaluating MYK-491 in a Phase 1b/2a study in stable heart failure patients.

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 Company's ability to enroll patients in its Phase 3 EXPLORER-HCM study of mavacamten in symptomatic oHCM and the timing of the completion of enrollment, the availability of data from EXPLORER-HCM, the Company's expectation with respect to release of data from these studies, the Company's ability to advance MYK-224 into clinical development, and the timing of these events, as well as the Company's projected cash runway, 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 March 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.

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