UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): March 30, 2020

MYOKARDIA, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-37609**	**44-5500552**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

1000 Sierra Point Parkway
Brisbane, CA 94005
(Address of principal executive offices, including zip code)

(650) 741-0900
(Registrant's telephone number, including area code)

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001	**MYOK**	**Nasdaq Global Select Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 8.01 Other Items.

On March 30, 2020, MyoKardia, Inc. issued a press release titled "MyoKardia Announces Mavacamten Treatment Well Tolerated and Significantly Reduced Biomarkers of Cardiac Injury and Wall Stress in Non-Obstructive Hypertrophic Cardiomyopathy Patients." A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits.

Exhibit No.	Description
99.1	Press Release, dated March 30, 2020.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

MyoKardia, Inc.

Date: March 30, 2020

By: /s/ Taylor Harris
Taylor Harris
Chief Financial Officer
(principal financial officer)

News Release

MyoKardia Announces Mavacamten Treatment Well Tolerated and Significantly Reduced Biomarkers of Cardiac Injury and Wall Stress in Non-Obstructive Hypertrophic Cardiomyopathy Patients

MAVERICK-HCM Phase 2 Clinical Trial Results Consistent with Tolerability Observations from Prior Studies of Mavacamten

Improvement in NT-proBNP and Troponin Levels Support Future Development in Non-Obstructive HCM and Heart Failure with Preserved Ejection Fraction (HFpEF)

MyoKardia to Host Webcast Conference Call at 4:30 p.m. EDT

Brisbane, Calif., March 30, 2020 — MyoKardia, Inc. (Nasdaq: MYOK) today announced results from the dose-ranging MAVERICK-HCM Phase 2 clinical trial of mavacamten for the treatment of non-obstructive hypertrophic cardiomyopathy (HCM). Data were presented during a late-breaker session at the American College of Cardiology's 69th Annual Scientific Session together with the World Congress of Cardiology (ACC.20/WCC Virtual) In the MAVERICK-HCM study, mavacamten was generally well tolerated, and statistically significant improvements in key biomarkers of cardiac injury and wall stress were observed. Further, subgroup analyses of study participants with indicators of more advanced disease demonstrated clinical responses across multiple parameters among patients on active treatment versus placebo.

"Non-obstructive HCM is especially challenging to treat as there are no proven or approved pharmacological therapies. Thus, for patients who develop symptoms refractory to medications, cardiac transplantation may be the only option," said Carolyn Ho, M.D., Medical Director of the Cardiovascular Genetics Center at Brigham and Women's Hospital and lead author on behalf of the MAVERICK-HCM study investigators. "Although the primary objective of MAVERICK was to assess the safety and tolerability of mavacamten in non-obstructive HCM, in exploratory analyses we observed an encouraging result with reductions in serum levels of NT-proBNP, a biomarker of hemodynamic stress, and also cardiac troponin I, a biomarker of myocardial injury. We believe MAVERICK is the first study to show an improvement in important serum biomarkers in this patient population and suggests that there is potential physiological benefit from the drug. We were also intrigued by findings that patients with more severe disease expression, those with elevated serum troponin levels or evidence of diastolic dysfunction by echo, may have achieved functional benefit. These findings, combined with mavacamten's tolerability profile, are encouraging, and they provide direction for further evaluation of mavacamten for patients with non-obstructive HCM."

"MAVERICK has succeeded in providing us with the important data we needed to proceed in our planned clinical trials in non-obstructive HCM, as well as a targeted subset of patients with heart failure with preserved ejection fraction, or HFpEF. We gained unique insights into dosing strategies using markers linked to clinical benefit, as well as how to identify patients who may be most likely to benefit from mavacamten," said Jay Edelberg, M.D. Ph.D., MyoKardia's Senior Vice President of Development. "The MAVERICK results also further our confidence in mavacamten's development in obstructive HCM, as we approach our Phase 3 EXPLORER-HCM readout, which is expected in the second quarter."

MAVERICK-HCM Results

Safety and Tolerability Observations

Mavacamten was generally well tolerated, consistent with prior clinical studies.

- Adverse events (AEs) reported during the treatment period were predominantly mild or moderate. The most commonly reported AEs were palpitations, dizziness and fatigue. Adverse events were reported by 90% of study participants in the mavacamten treatment arm and 68% of those on placebo. Of those reported in the active treatment group, 76% of AEs were mild, and 21% were moderate.

- The percentage of patients experiencing serious adverse events (SAEs) was 21.1% in the placebo arm and 10.3% in the active treatment arm. Most SAEs reported were cardiovascular in nature, the most frequent of which was atrial fibrillation/flutter. Thirty-nine percent of study participants entered the MAVERICK study with a prior diagnosis of atrial fibrillation, and all of the atrial fibrillation/flutter SAEs that occurred were in patients with a history of atrial fibrillation.
- As previously disclosed, five of the 39 patients on active treatment in this dose-ranging study reached a pre-specified stopping criterion of left ventricular ejection fraction (LVEF) measurements of £45%. The majority of these patients were asymptomatic for symptoms of heart failure, and LVEF recovered or was recovering within 4-12 weeks. The mean (SD) reduction in LVEF from baseline to week 16 was 4.1% (8.0) on active treatment when compared to a mean reduction of 2.3% (4.9) in the placebo arm.

Effect on Exploratory Endpoints of Efficacy: Biomarkers of Cardiac Wall Stress and Injury

Among several pre-specified endpoints analyzed, treatment with mavacamten resulted in significant changes in circulating biomarkers associated with heightened risks of cardiac complications.

- Mavacamten treatment resulted in statistically significant reductions in serum NT-proBNP ($p=0.0005$[1]) in the intent-to-treat population. At the conclusion of the 16-week treatment period, the reduction in the geometric mean of NT-proBNP among those receiving mavacamten was 53% vs. 1% in the placebo group. A significant drop in NT-proBNP occurred within the first four weeks of treatment and was maintained throughout the treatment period. NT-proBNP is a well-established biomarker of cardiac wall stress that has been associated with increased mortality in HCM patients.[2]
- Mavacamten treatment also resulted in a 34% decrease in the geometric mean of cardiac troponin I levels from baseline to Week 16. Cardiac troponin I levels increased by 4% in the placebo group ($p=0.009$). Cardiac troponin is closely associated with increased incidence of heart failure, atrial fibrillation and death in patients with HCM.[3]

For the intent-to-treat population, no difference was observed between active and placebo groups in the other exploratory endpoints.

“The effect of mavacamten on NT-proBNP and cardiac troponin levels in non-obstructive HCM patients is a first-of-its-kind finding for a product candidate in this patient population,” said Michael R. Zile, M.D., Director of Cardiology, Ralph H. Johnson VA Medical Center. “NT-proBNP is a measure of cardiac wall stress, and elevated troponins signal heart muscle injury, both of which have been established in the literature as prognosticators of dire complications in both HCM and HFpEF patients, including the need for hospitalizations, surgical intervention and death. The reductions in biomarkers associated with poor outcomes are encouraging, and I look forward to seeing the potential for mavacamten to impact outcomes in HCM, as well as certain targeted HFpEF populations, over time.”

Patient Subgroups with Advanced Diastolic Disease

MyoKardia also shared its analyses of the effect of mavacamten treatment on two subgroups of patients with advanced disease: one comprising 19 of the 59 enrolled patients (32%) with elevated cardiac troponin levels of >0.03ng/mL and another including 25 patients (42%) who had elevated filling pressures, defined by E/e’ >14[4]. HCM patients with higher cardiac troponin levels are known to be at greater risk for serious complications, and elevated filling pressures are indicative of impaired diastolic compliance, or the ability of the left ventricle to relax and fill with oxygenated blood.

A trend toward potential benefit was observed across numerous clinical measurements in patients with elevated cardiac troponin I levels and those with higher diastolic filling pressures versus placebo:

- Reductions in the plasma biomarkers NT-proBNP and cardiac troponin I were consistent with or greater than those observed across the intent-to-treat population.
- Changes in echocardiographic imaging measures including E/e’, and left ventricular end diastolic volume, indicated improvements in diastolic relaxation.

- Mean average E/e' improved on a placebo-adjusted basis by 4.8 and 3.6 in the elevated troponin and filling pressure subgroups, respectively.

- Improvements in measures of symptoms and function:
  - Mean peak VO_2 improved by 2.7 and 1.9 mL/kg/min on a placebo-adjusted basis in the subgroups with elevated troponin and filling pressure, respectively.
  - In the troponin subgroup, New York Heart Association classification improved in six of the 13 participants in the mavacamten cohorts versus only one of six individuals on placebo. No differences were observed in the elevated filling pressure subgroup.
  - The Clinical Kansas City Cardiomyopathy Questionnaire (KCCQ) Summary Score improved by 8.8 in the elevated troponin subgroup and 4.1 in the elevated filling pressure subgroups on a placebo-adjusted basis. KCCQ is designed to measure patients' perception of their heart failure health status and its impact on activities of daily living. A clinically significant change in KCCQ is considered to be greater than or equal to 6.

Composite Functional Endpoint Analysis

A composite functional endpoint(4) analysis was utilized to compare responses among the intent-to-treat population and the subgroups of patients with more advanced disease to those within the placebo population.

- Among the intent-to-treat study population (n=59), 23% of participants in the active arm vs. 21% of participants in the placebo arm achieved the composite functional definition of response. (p=0.92)
- For the combined subgroups (n=33, patients with either elevated average E/e' or elevated troponin levels), 33% of patients on mavacamten achieved the composite functional endpoint, compared to none of the subgroup patients in the placebo group. (p=0.03)

"Based on our observations that multiple markers responded to mavacamten, we believe we may be able to utilize markers of likely clinical benefit to guide dosing in the non-obstructive HCM patient population moving forward, similar to the way we are utilizing LVOT gradient to guide dosing in obstructive HCM," said Jay Edelberg, M.D., Senior Vice President, Development at MyoKardia. "Additionally, we observed in MAVERICK that patients who were most impaired showed the most meaningful trends toward benefit with mavacamten treatment within the 16-week treatment period. We look forward to leveraging these learnings, as well as knowledge gained from the longer exposures to treatment provided by our long-term extension study, as we look to advance mavacamten for the treatment of non-obstructive HCM patients and adjacent HFpEF populations."

Data from the MAVERICK-HCM Phase 2 clinical trial were presented by Carolyn Ho, M.D., Medical Director of the Cardiovascular Genetics Center at Brigham and Women's Hospital and Associate Professor of Medicine at Harvard Medical School, during the American College of Cardiology's Annual Scientific Session together with World Congress of Cardiology virtual meeting this morning during the "Featured Clinical Research III Session" in a presentation titled "Mavacamten Improves Biomarkers Of Myocardial Wall Stress And Injury In Patients With Symptomatic Non-Obstructive Hypertrophic Cardiomyopathy (nHCM): Results From The Phase 2 MAVERICK-HCM Study" (#412-16).

About the Phase 2 MAVERICK-HCM Clinical Trial

The Phase 2 MAVERICK-HCM trial assessed the safety and tolerability of a range of exposures over 16 weeks of treatment in patients with symptomatic, non-obstructive HCM. All study participants were required to be diagnosed with non-obstructive HCM, with left ventricular wall thickness either ³15mm or ³13mm with a family history of HCM, New York Heart Association (NYHA) classifications of Class II or III, and NT-proBNP levels of greater than 300 pg/mL at rest. Baseline characteristics, such as age, weight, gender, pathogenic mutation status, background beta blocker use, NYHA classification and exercise capacity were evenly distributed between active and placebo arms.

A total of 59 participants were enrolled in the study and randomized into one of three groups to receive once-daily doses of mavacamten or placebo. The active mavacamten treatment arms were designed to assess a range of drug concentrations around target levels of 200 ng/mL and 500 ng/mL. All participants in the active treatment arms began the study receiving 5mg doses of mavacamten. At Week 4,

pharmacokinetic (PK) assessments were conducted and doses were adjusted in a blinded fashion per the protocol based on the participant's assigned cohort. Following the 16-week treatment period, participants were monitored for an additional 8 weeks and became eligible to participate in MyoKardia's MAVA Long-Term Extension (LTE) study.

Conference Call and Webcast

MyoKardia management will also host a virtual event for investors and analyst today to review the data from MAVERICK-HCM and discuss future development plans for mavacamten in targeted groups of patients with diastolic disease. This live webcast event will begin at 4:30 p.m. EDT / 1:30 p.m. PDT and include remarks by Dr. Anjali Owens, Medical Director, Center for Inherited Cardiac Disease at the University of Pennsylvania, and Dr. Michael Zile, Professor of Medicine at the Medical University of South Carolina.

To access the call, please dial (844) 494-0193 (U.S.) or (508) 637-5584 (international), and reference the conference ID 2982709. A live webcast of the event will be available on the Investors section of MyoKardia's website at http://investors.myokardia.com. A replay of the webcast, and accompanying slides, will be available on the MyoKardia website for 90 days following the call.

About Non-obstructive HCM and Heart Failure with preserved Ejection Fraction

Hypertrophic cardiomyopathy is the most common genetic form of heart disease, affecting an estimated one in every 500 people worldwide. There are two main forms of HCM, obstructive HCM and non-obstructive HCM, which often share the same underlying genetic defects in the sarcomere that result in hypercontractility. In non-obstructive HCM, the heart contracts excessively and the left ventricle becomes abnormally thick, restricting the ability of the heart to relax and fill or pump to meet the body's needs, but no physical obstruction is present in the outflow tract of the left ventricle. Non-obstructive HCM affects an estimated one-third of all HCM patients and presents unique treatment challenges. Patients may progress to a more advanced state of disease than those with obstructive disease before being diagnosed, and there are no approved pharmacological treatment options available. As non-obstructive HCM progresses, symptoms begin to resemble those of a congestive heart failure patient and heart transplantation may become the only viable treatment option.

Heart failure with preserved ejection fraction (HFpEF) is a heterogeneous clinical syndrome, which in many patients is characterized by impairment of the left ventricle's ability to relax and fill during diastole, resulting in insufficient blood flow to meet the body's needs. HFpEF is estimated to affect approximately three million people in the U.S. and is associated with significant morbidity and mortality. There are currently no approved therapies for HFpEF.

About Mavacamten (MYK-461)

Mavacamten is a novel, oral, allosteric inhibitor of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). Mavacamten is intended to reduce cardiac muscle contractility by inhibiting the excessive myosin-actin cross-bridge formation that underlies the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. MyoKardia is currently evaluating mavacamten in multiple clinical trials for the treatment of obstructive and non-obstructive HCM. The pivotal Phase 3 clinical trial, known as EXPLORER-HCM, is being conducted in patients with symptomatic, obstructive HCM and MyoKardia anticipates data from this program in the second quarter of 2020. Two long-term follow-up studies are also ongoing, the PIONEER open-label extension study of obstructive HCM patients from MyoKardia's Phase 2 PIONEER trial and the MAVA-LTE, an extension study for patients who have completed either EXPLORER-HCM or MAVERICK-HCM, the company's Phase 2 clinical trial of symptomatic non-obstructive HCM patients. In April 2016, the U.S. FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic obstructive HCM.

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.

(1) Based on non-parametric Wilcoxon test

(2) Geske JB, et al. J Am Coll Cardiol 2013; 61:2456-2460

(3) Kubo T et al. J Am Coll Cardiol 2013; 62:1252-9

(4) Eleven individuals met the criteria of having both elevated cardiac troponin I levels and average E/e' of >14.

The composite function endpoint is defined as either (a) an improvement of at least 1.5 mL/kg/min in peak VO2 and a reduction of 1 or more NYHA Class, or (b) an improvement of 3.0 mL/kg/min or more in peak VO2 with no worsening in NYHA Class. This endpoint is being utilized as the primary endpoint in MyoKardia's pivotal Phase 3 EXPLORER-HCM clinical trial as a means of capturing symptomatic and functional improvements in HCM patients.

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 mavacamten, our plans to advance the clinical development of mavacamten in non-obstructive HCM patients and a targeted population of HFpEF patients, the anticipated data readout from our Phase 3 EXPLORER trial of mavacamten, our plans to consult with the FDA on potential pathways to registration and to provide a regulatory update, the initiation, progress and availability of data from our ongoing and planned clinical trials, 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.

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