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): September 10, 2019

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.)

333 Allerton Ave.
South San Francisco, CA 94080
(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	**MYOK**	**The 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 Events.

On September 10, 2019 (the "Effective Date"), MyoKardia, Inc. issued a press release titled "MyoKardia to Evaluate Mavacamten as an Alternative to Septal Reduction Therapy in 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 September 10, 2019
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.

Date: September 10, 2019

MyoKardia, Inc.

By: /s/ Cynthia Ladd
Cynthia Ladd
General Counsel

Exhibit 99.1

News Release

MyoKardia to Evaluate Mavacamten as an Alternative to Septal Reduction Therapy in Obstructive Hypertrophic Cardiomyopathy Patients

Planned Study Will Assess Effect of Mavacamten on the Need for SRT Among HCM Patients Referred for Invasive Procedures

SOUTH SAN FRANCISCO, Calif., September 10, 2019 – MyoKardia, Inc. (Nasdaq: MYOK), today announced that it will study mavacamten as a therapeutic alternative to septal reduction therapy (SRT). The study will be conducted at leading HCM centers that regularly perform surgical myectomy or alcohol septal ablation procedures, with support from the established referral networks at those centers. The randomized, double-blind study will enroll symptomatic, obstructive HCM patients referred for SRT, and MyoKardia anticipates that enrollment will begin in the first half of 2020.

The study's Executive Committee is being chaired by Steve Nissen, M.D., Chief Academic Officer at the Cleveland Clinic Heart and Vascular Institute. Milind Desai, M.D., Director, Clinical Operations, Cleveland Clinic Heart and Vascular Institute, is serving as Principal Investigator. The study is being run by Cleveland Clinic C5 Research

"This study is designed to address the question of whether mavacamten, which addresses the underlying cause of HCM, can be an effective alternative to surgical procedures which can only address removal of the physical obstruction of oHCM," said Jay Edelberg, Senior Vice President of Clinical Development at MyoKardia. "This septal reduction therapy trial will allow us to focus on obstructive HCM patients who have been referred for SRT and to expand our knowledge for the broader HCM community. The data from this blinded, controlled study will supplement the clinical data from our ongoing Phase 3 EXPLORER pivotal trial in understanding the potential of mavacamten in treatment of these patients."

HCM is a chronic, progressive condition in which the heart muscle thickens due to excess contraction. In the obstructive form of HCM, the wall of the septum thickens and may block the flow of blood from the left ventricle to the aorta. Each year, approximately 1,500 patients with obstructive HCM undergo septal reduction therapy in the United States. SRT is performed as either an open-heart surgical procedure, known as a myectomy, or injection of alcohol into the heart muscle, which causes the heart muscle cells in the thickened area to die. Both procedures are intended to reduce the thickness of the septal wall and alleviate obstruction.

"HCM can be a debilitating disease, interfering with the ability of patients to go about the activities of daily living and putting them at long-term risk of atrial fibrillation, stroke and heart failure," said Dr. Desai.

MyoKardia is developing mavacamten for the treatment of HCM. Mavacamten was specifically developed to correct the abnormal mechanics of the HCM heart by normalizing the number of myosin-actin cross-bridges that drive the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. In the company's Phase 2 PIONEER-HCM clinical trial of patients with obstructive HCM, mavacamten reduced or eliminated the obstruction of the left ventricular outflow tract, resulting in improvements in how patients feel (as measured by New York Heart Association classification), and how their hearts are functioning (based on peak VO2 measured by cardiopulmonary exercise testing).

"MyoKardia is committed to leadership within the broader HCM community, including the development of robust evidence regarding treatment alternatives," commented Tassos Gianakakos, president and CEO at MyoKardia. "We look forward to results from this and other potential studies outside of our registration program to further inform referral and treatment decisions for patients across the full spectrum of this potentially debilitating disease."

Driven by the **Heart**
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MyoKardia is currently conducting the pivotal Phase 3 EXPLORER-HCM clinical trial of mavacamten in patients with symptomatic (NYHA Class II or III) obstructive HCM. Enrollment in the EXPLORER study completed in August 2019 at clinical sites in the U.S., Europe, and Israel. Topline data from the EXPLORER-HCM trial are anticipated in the second quarter of 2020. The EXPLORER trial, along with data from the MAVA long-term extension study, as well as MyoKardia's ongoing and completed clinical trials of mavacamten, are expected to form the basis for a registrational submission to the U.S. Food and Drug Administration.

About Mavacamten (MYK-461)

Mavacamten is a novel, oral, allosteric modulator 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 advancing mavacamten in a pivotal Phase 3 clinical trial, known as the EXPLORER-HCM study, in patients with symptomatic, obstructive HCM and a Phase 2 clinical trial, the MAVERICK-HCM study, in patients with symptomatic non-obstructive HCM. 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-HCM trial, and the MAVA-LTE, an extension study for patients who have completed either the EXPLORER-HCM or MAVERICK-HCM trials. 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 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 muscle proteins of the heart that modulate cardiac muscle contraction and underlying diseases of systolic and diastolic dysfunction. 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 has discovered a pipeline of product candidates directed at diseases driven by excessive contraction, impaired relaxation, or insufficient contraction. Among its discoveries are three clinical-stage therapeutics: mavacamten (formerly 461) in Phase 3 and Phase 2 clinical trials for hypertrophic cardiomyopathies (HCM); MYK-491 in Phase 2 for patients with stable heart failure; and MYK-224, in Phase 1 development for HCM.

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 timing of the commencement of the SRT study and the availability of data from our Phase 3 EXPLORER-HCM study in patients with obstructive HCM as well as our expectation with respect to release of data from the EXPLORER-HCM study, 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, 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
Corporate Communications & Investor Relations
MyoKardia, Inc.
650-351-4690
mcorral@myokardia.com

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

Steven Cooper (media)
Edelman
415-486-3264
steven.cooper@edelman.com