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): May 9, 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))

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. ☐

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

Item 2.02 Results of Operations and Financial Condition.

On May 9, 2019, MyoKardia, Inc. announced its financial results for the first quarter ended March 31, 2019. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in Item 2.02 of this Form 8-K (including Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits.

Exhibit No.	Description
99.1	Press Release issued by MyoKardia, Inc. on May 9, 2019, furnished herewith

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: May 9, 2019

MyoKardia, Inc.

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

EXHIBIT 99.1

MyoKardia Reports First Quarter 2019 Financial Results

Announces Enrollment Completing in the Phase 2 MAVERICK-HCM Trial

Company to Host Conference Call and Webcast Today at 4:30 p.m. ET (1:30 p.m. PT)

SOUTH SAN FRANCISCO, Calif., May 9, 2019 – MyoKardia, Inc. (Nasdaq: MYOK), a clinical-stage biopharmaceutical company pioneering a precision medicine approach for the treatment of serious cardiovascular diseases, today reported financial results for the quarter ended March 31, 2019.

"With a strong first quarter behind us, MyoKardia continues to make progress toward our aim of becoming a world-leader in developing new treatments for cardiovascular diseases. As we head into the second half of 2019, our focus remains on realizing the full potential of mavacamten and unlocking the full potential of our growing pipeline, including MYK-491 and MYK-224. Underpinning our strategy is continued HCM disease-area leadership, including initiatives to engage with the broader HCM and cardiology community," said Tassos Gianakakos, Chief Executive Officer. "The MAVERICK study exemplifies this focus and the completion of enrollment is a significant milestone. In MAVERICK, we're exploring mavacamten's potential to help individuals with non-obstructive HCM – a population for which no targeted therapies exist. We expect findings from this trial to enable us to move into a registration program for nHCM - extending mavacamten's franchise into an additional important indication and providing critically valuable insights to fuel our pipeline of earlier-stage therapeutics aimed at addressing additional diseases of diastolic dysfunction and cardiac contractility."

Recent Clinical Program Highlights

Mavacamten for Hypertrophic Cardiomyopathy (HCM)

- **Presented 12- and 24-week data from PIONEER Open-Label Extension (PIONEER-OLE) Study of Mavacamten in Obstructive HCM (oHCM):** At the American College of Cardiology (ACC) 68th Annual Scientific Session in March 2019, MyoKardia presented positive twenty-four week safety and efficacy data from the PIONEER-OLE study. Mavacamten treatment improved patient symptoms as measured by NYHA classification and reduced or eliminated obstruction of the left ventricular outflow tract (LVOT) to levels below the guideline-based thresholds for diagnosis (30 mmHg) and for invasive intervention (50 mmHg) in all patients, without reducing left ventricular ejection fraction below normal. Mavacamten was well tolerated with no cardiac-related adverse events. These data reflect the longest duration of mavacamten treatment reported to date.
    - **Thirty-six-week data from PIONEER-OLE has been accepted for presentation at ESC Congress 2019**: PIONEER-OLE investigators will present safety, efficacy and biomarker data for twelve patients at the upcoming European Society of Cardiology (ESC) Congress being held August 31-September 4, 2019 in Paris.
- **Enrollment Completing in Phase 2 MAVERICK-HCM Clinical Trial of Mavacamten in Non-Obstructive HCM (nHCM):** MyoKardia announced that screening of patients for the Phase 2 MAVERICK clinical trial was closed on schedule on April 19, 2019 and that the last patient will be enrolled by May 17, 2019. The randomized, double-blind, placebo-controlled MAVERICK-HCM Phase 2 clinical trial is designed to assess the safety and tolerability of a 16-week treatment course of mavacamten in patients with nHCM. Patients are being randomized evenly into three groups to receive a once-daily dose of mavacamten targeting one of two plasma concentration levels (200 ng/mL, 500 ng/mL) or placebo. MyoKardia plans to report topline data from the MAVERICK-HCM study in the fourth quarter of 2019.

- **Pivotal Phase 3 EXPLORER-HCM Clinical Trial Enrollment Remains on Track:** MyoKardia expects to complete enrollment in the 220-patient study in the second half of 2019, with topline data available in the second half of 2020.

- **Published Results from the Phase 2 PIONEER-HCM Clinical Trial in the *Annals of Internal Medicine***: Data from Cohorts A and B of the Phase 2 PIONEER study of mavacamten for the treatment of oHCM were published in the April 29, 2019 edition of the *Annals of Internal Medicine*. As previously reported, mavacamten was shown to be well tolerated and demonstrated the ability to eliminate LVOT gradient in patients with obstructive HCM. Improvements were observed in how patients felt and how their hearts functioned as assessed by NYHA functional class, dyspnea scores, peak VO2 testing and relevant biomarkers. Results of the PIONEER study informed the design of MyoKardia's ongoing EXPLORER and MAVERICK clinical trials of mavacamten.

MYK-491 for Dilated Cardiomyopathy (DCM)
- **Abstract Detailing MYK-491 Phase 1a Data Accepted for Presentation at The European Society of Cardiology, Heart Failure Meeting**: Data from the Phase 1a study of MYK-491 in healthy volunteers will be presented during a poster session at the 6th World Congress on Acute Heart Failure in Athens, Greece at 8:30 a.m. EEST (1:30 a.m. ET) on Monday, May 27, 2019. Topline results, which were shared during MyoKardia's October 2018 R&D Day, show an increase in cardiac contractility following treatment with MYK-491. Data from the ongoing multiple-ascending dose study of MYK-491 are expected in the second half of 2019.

HCM Leadership Initiatives
- **Awarded Inaugural MyoSeeds™ Research Grants:** MyoKardia granted three awards to researchers, of $250,000 each, under its MyoSeeds Research Grants Program to support original, independent research in the biology and underlying mechanisms of cardiomyopathies and precision treatment for heart disease.

- **In Collaboration with 23andMe, Launched HCM Patient Community Page and Initiated Joint Research Project:** MyoKardia and 23andMe have begun collecting deidentified data on current symptoms and therapies utilized in self-identified HCM customers in order to gain new insights into how HCM manifests across diverse patient groups. 23andMe customers now have access to an education page that provides information on the condition and new research taking place, as well as links to other resources. 23andMe customers will also have the opportunity to participate in joint research studies with the aim of gathering more information about patient experiences in coping with HCM.

Recent Corporate Highlights
- **Completed Successful Follow-on Offering:** MyoKardia completed a follow-on offering of 5,663,750 shares of common stock, including the full exercise of the underwriters' over-allotment option, at a public offering price of $51.00 per share, raising approximately $271.2 million in net proceeds. MyoKardia anticipates using net proceeds from the offering to support the ongoing registration studies, regulatory approval process, and commercial preparations for mavacamten for the potential treatment of oHCM; to fund ongoing and potential later-stage studies of mavacamten in nHCM and MYK-491 in targeted segments of systolic heart failure; to fund ongoing preclinical, discovery and research programs; and for working capital, business development, and other general corporate purposes.

- **Regained Worldwide Rights to Mavacamten and MYK-491, as well as Certain Preclinical Programs:** MyoKardia's collaboration with Sanofi concluded in its entirety effective April 1, 2019. As a result, MyoKardia now has global commercial rights to all programs in its portfolio.

First Quarter 2019 Financial Results
- **Cash Position:** Cash, cash equivalents and investments (short-term and long-term) as of March 31, 2019 were $628.3 million, compared to $394.8 million as of December 31, 2018. The increase in the company's cash position is primarily attributable to net proceeds of approximately $271.2 million from MyoKardia's follow-on offering of common stock, which priced in March 2019.

- **Revenue**: In the first quarter of 2019, MyoKardia recorded no revenue from collaborations, compared with $5.3 million during the same period in 2018. As of December 31, 2018, MyoKardia had fulfilled all of its performance obligations to Sanofi, and no further revenue will be recorded from the Sanofi collaboration agreement.

- **R&D Expenses:** Research and development expenses were $26.2 million in the first quarter 2019, net of reimbursement credits from Sanofi of $9.9 million, up from $16.6 million (net of Sanofi credits of $2.8 million) for the same period in 2018. The increase in R&D expenses for the first quarter of 2019 was primarily driven by an increase in clinical expenses related to the advancement of mavacamten and MYK-491 into later-stage trials, increases in personnel expenses and stock compensation due to higher headcount, and increases in research and preclinical activities.

- **G&A Expenses:** General and administrative expenses were $13.6 million for the three months ended March 31, 2019, compared to $7.3 million for the same period in 2018. The change in G&A expense was primarily driven by an increase in personnel expenses and stock compensation due to higher headcount and an increase in accounting and consulting fees.

- **Net Loss**: Net loss was $37.5 million ($0.93 loss per share) for the first quarter of 2019, compared to a net loss of $17.8 million ($0.50 loss per share) for the first quarter of 2019.

Financial Guidance
Based on its current operating plans, MyoKardia anticipates that current cash, cash equivalents and investments are sufficient to fund operations into the second half of 2021.

Conference Call and Webcast
MyoKardia management will host a conference call and live audio webcast on Thursday, May 9, 2019, at 4:30 p.m. ET / 1:30 p.m. PT to discuss current operations and first quarter 2019 financial results. The call may be accessed by phone by calling 844-494-0193 from the U.S. and Canada or 508-637-5584 internationally and using the conference ID 9189629. The webcast may be accessed live on the Investor Relations section of the Company's website at http://investors.myokardia.com. A replay of the webcast will be available on the MyoKardia website for approximately 90 days following the call.

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 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 mavacamten and MYK-491, the Company's ability to enroll patients in its Phase 3 EXPLORER-HCM study of mavacamten in symptomatic oHCM, the Company's ability to enroll patients in its Phase 2 MAVERICK-HCM study of mavacamten in nHCM, and the availability of data from EXPLORER-HCM, MAVERICK-HCM, the MYK-491 Phase 2a multiple ascending dose trial and PIONEER-OLE, the Company's expectation with respect to release of data from these studies, the Company's ability to advance additional research programs 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 Annual Report on Form 10-K for the year ended December 31, 2018, 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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MYOKARDIA, INC.

Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)
(Unaudited)

	March 31, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 466,254	$ 246,122
Short-term investments	92,540	68,564
Prepaid expenses and other current assets	4,312	4,760
Total current assets	563,106	319,446
Property and equipment, net	5,592	5,138
Operating lease right-of-use assets	2,473	—
Long-term investments	69,524	80,148
Restricted cash and other	2,611	2,521
Total assets	$ 643,306	$ 407,253
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	5,194	$ 2,946
Accrued liabilities	20,469	20,758
Prepayment from collaboration partner	3,099	12,973
Operating lease liabilities - current	2,529	—
Total current liabilities	31,291	36,677
Other long-term liabilities	73	9
Total liabilities	31,364	36,686
Commitments and contingencies		
Stockholders' equity		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 150,000,000 shares authorized at March 31, 2019 and December 31, 2018; 46,001,775 and 40,288,949 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	5	4
Additional paid-in capital	851,664	573,183
Accumulated other comprehensive income (loss)	296	(67)
Accumulated deficit	(240,023)	(202,553)
Total stockholders' equity	611,942	370,567
Total liabilities and stockholders' equity	$ 643,306	$ 407,253

Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended March 31,	
	2019	2018
Collaboration and license revenue	$ —	$ 5,331
Operating expenses:		
Research and development	26,190	16,618
General and administrative	13,551	7,313
Total operating expenses	39,741	23,931
Loss from operations	(39,741)	(18,600)
Interest and other income, net	2,271	780
Net loss	(37,470)	(17,820)
Other comprehensive gain (loss)	363	(137)
Comprehensive loss	$ (37,107)	$ (17,957)
Net loss per share, basic and diluted	$ (0.93)	$ (0.50)
Weighted average number of shares used to compute net loss per share, basic and diluted	40,506,313	35,827,235