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): November 2, 2017

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. ☒

Item 2.02 Results of Operations and Financial Condition.

On November 2, 2017, MyoKardia, Inc. announced its financial results for the three and nine months ended September 30, 2017. 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 November 2, 2017, furnished herewith

EXHIBIT INDEX

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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: November 2, 2017

MyoKardia, Inc.

By: /s/ Jacob Bauer
Jacob Bauer
Senior Vice President, Finance and Corporate Development
(principal financial officer)

Exhibit 99.1

MyoKardia Reports Third Quarter 2017 Financial Results and Operational Progress

Received FDA Feedback for Mavacamten Registration Program in Symptomatic, Obstructive Hypertrophic Cardiomyopathy

MyoKardia to Initiate EXPLORER-HCM as Pivotal Trial in Second Quarter 2018

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

SOUTH SAN FRANCISCO, Calif., November 2, 2017 – MyoKardia, Inc. (Nasdaq: MYOK), a clinical-stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, today reported financial results for the third quarter ended September 30, 2017.

MyoKardia also provided an update on the company's End-of-Phase 2 meeting with the U.S. Food and Drug Administration's (FDA) Division of Cardiovascular and Renal Products. The meeting focused on MyoKardia's proposed registration program for mavacamten (formerly MYK-461) in symptomatic, obstructive hypertrophic cardiomyopathy (oHCM). MyoKardia is incorporating guidance from the FDA on study design elements and plans to proceed with the pivotal Phase 3 trial, EXPLORER-HCM, and a long-term extension study, as the key remaining studies in the mavacamten registration program. The company anticipates dosing the first patient in EXPLORER-HCM in the second quarter of 2018. Between now and study start, the company plans to review the final trial design with the FDA.

"It has been a remarkable quarter for MyoKardia, highlighted by the PIONEER-HCM Phase 2 results and by our meeting with the FDA further clarifying our path to registration for mavacamten," said Tassos Gianakakos, Chief Executive Officer. "The progress we've made across our clinical pipeline has generated important disease insights that we believe strengthen our research platform. In the months to come, we expect to report important progress in our MYK-491 clinical program for dilated cardiomyopathy, present mavacamten data at the 2017 American Heart Association annual meeting and initiate a Phase 2 study of mavacamten in its second indication, non-obstructed HCM."

Clinical Updates and Highlights

Mavacamten for Hypertrophic Cardiomyopathy (HCM)

- **End-of-Phase 2 Meeting with FDA for oHCM.** In consultation with the FDA, MyoKardia has established the key elements of the registration program for mavacamten in symptomatic oHCM, including the Phase 3 EXPLORER-HCM clinical trial and a planned long-term extension study. The primary endpoint of the EXPLORER-HCM trial will be an improvement in exercise capacity as measured by the change in peak oxygen consumption (peak VO_2) from baseline. The Phase 3 clinical study is designed to enroll between 200-250 patients with symptomatic oHCM. MyoKardia expects to begin patient dosing of the Phase 3 trial in the second quarter of 2018.
- **Lower-Dose Cohort of PIONEER-HCM Trial Enrollment Complete.** In August 2017, MyoKardia announced that enrollment had completed in the second, lower-dose cohort of PIONEER-HCM. Ten patients with symptomatic, obstructive HCM who were not required to discontinue their background beta blocker therapy were enrolled. MyoKardia anticipates reporting topline data from this dosing cohort in the first quarter of 2018.
- **Mavacamten Phase 2 Trial in Non-Obstructive HCM to Begin in First Quarter 2018.** MyoKardia is incorporating input received from the FDA in the End-of-Phase 2 meeting into its planned Phase 2 trial of mavacamten in non-obstructive HCM (nHCM), the MAVERICK-HCM study. MyoKardia anticipates commencing patient dosing in MAVERICK-HCM in the first quarter of 2018. The purpose of this Phase 2 study is to assess efficacy and determine optimal dosing of mavacamten in patients without left ventricular outflow tract obstruction.

- **Data Reported from Phase 2 PIONEER-HCM study.** Data from the first cohort of the PIONEER-HCM study of mavacamten in symptomatic, oHCM patients were reported in August 2017 by the company and presented at the Heart Failure Society of America's Annual Scientific Meeting in September 2017.
    - A statistically significant improvement in the primary endpoint of a change in post-exercise peak LVOT gradient from baseline to week 12 (p=0.002) was observed in all ten patients treated with mavacamten. Clinically meaningful improvements were also observed across most secondary endpoints, including peak VO_2, the expected primary endpoint for registration in this patient population. Mavacamten was generally well-tolerated.
- **New Data to be Presented at AHA**. MyoKardia researchers will present new clinical and *in vivo* preclinical data related to its mavacamten program and HCM at the upcoming American Heart Association Scientific Sessions being held on November 11-15, 2017, in Anaheim, CA.

MYK-491 for Dilated Cardiomyopathy (DCM)

- **MYK-491 Phase 1 Trial Enrollment Ongoing.** In September 2017, MyoKardia announced plans to enroll additional dose cohorts in its Phase 1 single-ascending dose trial of MYK-491 in healthy volunteers to refine exposure-activity relationship. Topline data from this Phase 1 single-ascending dose study are expected to be announced in early 2018.
- **MYK-491 Phase 1b Study in DCM Patients to Begin by Year-End.** A single-ascending dose study of MYK-491 in symptomatic DCM patients is expected to initiate before the end of 2017. The objectives of this randomized, double-blind, placebo-controlled Phase 1b trial are to assess safety, tolerability, preliminary pharmacokinetics and pharmacodynamics of MYK-491 in patients.

Corporate Updates and Highlights

- **Public Offering Raised $133.8 Million.** In August 2017, MyoKardia announced the closing of a follow-on public offering of 4,025,000 shares of common stock at the public offering price of $35.50 per share, raising approximately $133.8 million in net proceeds.
- **Roles Expanded for Two Executive Committee Members**. Robert McDowell, Ph.D. has been promoted to the role of Chief Scientific Officer reflecting his leadership of the company's integrated research organization. June Lee, M.D., who serves as MyoKardia's Chief Operating Officer, has also been appointed Chief Development Officer. In this new role, she will be growing the company's development organization to support the expansion of MyoKardia's clinical pipeline, including advancement of mavacamten into late-stage development and additional indications.

Third Quarter and Year-to-Date 2017 Financial Results

- **Cash Position:** Cash and cash equivalents as of September 30, 2017 were $227.2 million, compared to $135.8 million as of December 31, 2016. Investments (short-term and long-term) as of September 30, 2017 were $56.0 million, compared to $16.1 million as December 31, 2016. The increase in the company's cash position is attributable to proceeds received from the offering of common stock.
- **Revenues:** Collaboration and license revenue was $5.6 million during the three months ended September 30, 2017, compared with $3.6 million during the same period in 2016. Collaboration and license revenue was $16.9 million for the nine months ended September 30, 2017, compared to $10.7 million for the same period in 2016. The increase in collaboration revenue for the first three quarters of 2017 was attributable to recognition of the $45.0 million Sanofi Continuation Payment.
- **R&D Expenses:** Research and development expenses for the three months ended September 30, 2017 were $14.4 million, up from $8.8 million for the same period in 2016. Research and development expenses were $40.0 million for the nine months ended September 30, 2017, compared to $26.2 million for the same period in 2016. The increase in R&D expenses was primarily driven by the company's ongoing clinical studies, including the Phase 2 PIONEER-HCM clinical study for mavacamten and the Phase 1 study for MYK-491, expansion of

R&D staff and increased costs for contract research, chemistry and biology expenses on discovery and preclinical programs.

- **G&A Expenses:** General and administrative expenses were $5.9 million for the three months ended September 30, 2017, compared to $4.0 million for the same period in 2016. For the nine months ended September 30, 2017, G&A expenses were $16.5 million, compared to $12.0 million for the same period in 2016. The change in G&A expenses was primarily attributable to an increase in employee headcount, marketing and recruiting expenses and stock compensation expenses.

- **Net Loss:** Net loss was $14.2 million ($0.42 per share) for the third quarter of 2017, compared to a net loss of $9.2 million ($0.35 per share) for the third quarter of 2016. Net loss was $38.6 million ($1.21 per share) for the nine months ended September 30, 2017, compared to $27.4 million ($1.04 per share) for the same period in 2016. The increase in net loss was primarily attributable to the increase in operating expenses noted above.

Based on its current operating plans, the Company expects that its cash, cash equivalents and investments as of September 30, 2017, together with anticipated payments from Sanofi under its collaboration agreement, will enable the Company to fund its anticipated operating expenses and capital expenditure requirements at least into 2020.

Conference Call and Webcast

MyoKardia will host a conference call and live audio webcast on Thursday, November 2, 2017 at 4:30 p.m. ET / 1:30 p.m. PT. 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 7295509. 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 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 and rare cardiovascular diseases. MyoKardia's initial focus is on the treatment of heritable cardiomyopathies, a group of rare, genetically-driven forms of heart failure that result from biomechanical defects in cardiac muscle contraction. MyoKardia has used its precision medicine platform to generate a pipeline of therapeutic programs for the chronic treatment of the two most prevalent forms of heritable cardiomyopathy—hypertrophic cardiomyopathy (HCM), and dilated cardiomyopathy (DCM). MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461), a novel, oral, allosteric modulator of cardiac myosin that has been shown to reduce hypercontractility in early clinical studies and is currently being studied in the Phase 2 PIONEER-HCM clinical trial. MYK-491, MyoKardia's second product candidate, is designed to increase the overall extent of the heart's contraction in DCM patients by increasing cardiac contractility. MyoKardia is currently evaluating MYK-491 in a Phase 1 study in healthy volunteers. A cornerstone of the MyoKardia platform is the Sarcomeric Human Cardiomyopathy Registry (SHaRe), a multi-center, international repository of clinical and laboratory data on individuals and families with genetic heart disease, which MyoKardia helped form in 2014. 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 commencement of enrollment and patient dosing in the EXPLORER-HCM trial,

the trial design for EXPLORER-HCM, the release of topline data from the Company's lower-dose cohort in the PIONEER-HCM trial, the commencement of a Phase 2 trial of mavacamten in nHCM patients, the release of topline data from the Phase 1 single ascending dose trial of MYK-491 in healthy volunteers and the initiation of the Company's planned Phase 1b single ascending dose trial of MYK-491 in DCM patients, as well as the timing of these events, and the Company's expected cash runway and ability to receive additional payments from its collaboration agreement with Sanofi, 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, 2017, 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.

MYOKARDIA, INC.

Condensed Consolidated Balance Sheets

(In thousands, except share and per share amounts)

(Unaudited)

	September 30, 2017	December 31, 2016
Assets		
Current assets		
Cash and cash equivalents	$ 227,184	$ 135,797
Short-term investments	19,993	4,072
Receivable from collaboration partner	—	45,000
Prepaid expenses and other current assets	1,545	1,394
Total current assets	248,722	186,263
Property and equipment, net	2,633	2,758
Long-term investments	36,003	12,002
Other long-term assets	432	283
Total assets	$ 287,790	$ 201,306
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 2,030	$ 1,798
Accrued liabilities	10,634	8,690
Deferred revenue - current	22,500	22,500
Total current liabilities	35,164	32,988
Other long-term liabilities	246	436
Deferred revenue - noncurrent	5,625	22,500
Total liabilities	41,035	55,924
Commitments and contingencies (Note 6)		
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 and 150,000,000 shares authorized at September 30, 2017 and December 31, 2016, respectively; 35,733,002 and 31,428,998 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	4	3
Additional paid-in capital	363,205	223,208
Accumulated other comprehensive (loss) income	(52)	8
Accumulated deficit	(116,402)	(77,837)
Total stockholders' equity	246,755	145,382
Total liabilities and stockholders' equity	$ 287,790	$ 201,306

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

MYOKARDIA, INC.

Condensed Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Collaboration and license revenue	$ 5,625	$ 3,550	$ 16,875	$ 10,649
Operating expenses:				
Research and development	14,361	8,783	39,967	26,192
General and administrative	5,884	4,031	16,442	11,947
Total operating expenses	20,245	12,814	56,409	38,139
Loss from operations	(14,620)	(9,264)	(39,534)	(27,490)
Interest and other income, net	447	33	977	79
Net loss	(14,173)	(9,231)	(38,557)	(27,411)
Other comprehensive loss	2	—	60	—
Comprehensive loss	(14,171)	(9,231)	(38,497)	(27,411)
Net loss attributable to common stockholders	$ (14,173)	$ (9,231)	$ (38,557)	$ (27,411)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.42)	$ (0.35)	$ (1.21)	$ (1.04)
Weighted average number of shares used to compute net loss per share attributable to common stockholders, basic and diluted	33,525,567	26,470,298	31,951,631	26,331,852

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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