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 4, 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, 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 2.02 Results of Operations and Financial Condition.

On November 4, 2019, MyoKardia, Inc. announced its financial results for the third quarter ended September 30, 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 November 4, 2019, furnished herewith
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: November 4, 2019

MyoKardia, Inc.

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

Exhibit 99.1
News Release

MYOKARDIA

MyoKardia Reports Third Quarter 2019 Financial Results

SOUTH SAN FRANCISCO, Calif., Nov. 04, 2019 -- MyoKardia, Inc. (Nasdaq: MYOK), today reported financial results for the quarter ended September 30, 2019.

“As we look across the milestones recently achieved and those just ahead, we see steady progress on our near- and long-term corporate objectives. We are forging a position of disease-area leadership in the improved care of hypertrophic cardiomyopathy, while applying all that we are learning from those efforts to tackle additional targeted populations with diseases caused by diastolic or systolic dysfunction,” said Tassos Gianakakos, MyoKardia’s Chief Executive Officer. “Before year-end, we will be reporting 48-week data from our PIONEER-OLE study in obstructive HCM, as well as data from our Phase 2 MAVERICK-HCM study in patients with non-obstructive hypertrophic cardiomyopathy. Our momentum will continue into next year, with additional data and program progress planned across our portfolio for the first half of 2020, including the topline readout from our pivotal Phase 3 EXPLORER-HCM trial of mavacamten in obstructive HCM and the advancement of MYK-491 into a targeted genetic cardiomyopathy population.”

Recent Clinical Program Highlights

Mavacamten for Obstructive Hypertrophic Cardiomyopathy

- **Completed Enrollment of 251 Patients in the Phase 3 EXPLORER-HCM Trial:** Enrollment in the pivotal Phase 3 EXPLORER-HCM registrational clinical study of mavacamten for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (HCM) completed in July of 2019. MyoKardia anticipates reporting topline data from the EXPLORER-HCM trial in the second quarter of 2020.

- **Presented 36-week Data from PIONEER-OLE Study of Mavacamten:** At the European Society of Cardiology Congress 2019, Dr. Andrew Wang of Duke University presented 36-week data from the PIONEER open-label extension study of twelve patients with obstructive HCM. Mavacamten treatment has resulted in durable reductions in obstruction of the left ventricular outflow tract (LVOT) while maintaining left ventricular ejection fraction (LVEF) at or above normal levels. Sustained improvements in multiple biomarkers of cardiac stress and diastolic filling pressures were also observed.

- **Formed Partnership with the Cleveland Clinic for Septal Reduction Therapy (SRT) Study:** MyoKardia and the Cleveland Clinic will begin a randomized, double-blind study of symptomatic, obstructive HCM patients referred for septal reduction therapy (SRT) starting in the first half of 2020. 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.

MYK-491 for Dilated Cardiomyopathy

- **Reported Positive Data from Phase 2a Study of MYK-491:** An interim analysis of 26 patients with stable heart failure due to left ventricular systolic dysfunction in the ongoing Phase 2a clinical trial showed that MYK-491 demonstrated improvement in systolic function, including stroke volume increases of greater than 10 percent relative to baseline on a placebo-adjusted basis, without impacting diastolic function. Given the favorable safety profile observed to date, the Phase 2a study has been extended to evaluate higher exposures of MYK-491.

- **Announced Plans to Advance MYK-491 into a Phase 2 Study in Genetic Dilated Cardiomyopathy (DCM) Population:** MyoKardia plans to advance MYK-491 into a Phase 2 clinical trial in DCM patients with certain genetic mutations of the cardiac sarcomere in the first half of 2020. Genetic mutations of the sarcomere impair the ability of the heart muscle proteins to function effectively, leading to progressive worsening of function. Genetic abnormalities contributing to DCM are estimated to be present in about 30-40% of DCM patients, corresponding to an estimated prevalence of 250,000 to 500,000 p eople in the U.S.

MYK-224 for HCM

- **Began Dosing Healthy Subjects in a Phase 1 Clinical Trial of MYK-224:** The Phase 1 clinical trial of MYK-224 is intended to evaluate safety, tolerability and pharmacokinetics. Pharmacodynamic effects on cardiac function and dimensions will also be assessed. MYK-224 joins MyoKardia's growing portfolio of therapeutic candidates targeting proteins of the heart muscle to address cardiovascular diseases of excessive contraction, inadequate contraction, or impaired relaxation. Topline results are expected in mid-2020.

Recent Corporate Highlights

- **Launched 2nd Annual MyoSeeds™ Research Grant Program Funding Cycle:** MyoKardia received more than 60 proposals for its MyoSeeds initiative to support original, independent research in the biology and underlying mechanisms of cardiomyopathies and precision treatment for heart disease. Through this program, MyoKardia will fund up to four awards, with a total investment of up to $1 million in funding over 2019-2020. Award recipients will be announced in the first quarter of 2020.

Third Quarter 2019 Financial Results

- **Cash Position:** Cash, cash equivalents and investments (short-term and long-term) as of September 30, 2019 were $480.4 million, compared to $394.8 million as of December 31, 2018. The change in MyoKardia's cash position was primarily attributable to the net proceeds from our March 2019 offering of $271.2 million, offset by our year-to-date losses of $217.4 million, which includes a $80.0 million payment to Sanofi for the purchase of mavacamten and MYK-224 royalty rights.

- **Revenues:** MyoKardia did not record revenue from the Sanofi collaboration during the third quarter and nine months ended September 30, 2019. For the three and nine months ended September 30, 2018, collaboration and license revenue totaled $9.2 million and $21.2 million, respectively. MyoKardia fulfilled its research and development obligations under the collaboration agreement during the year ended December 31, 2018, and no further revenue will be recorded under the agreement.

- **R&D Expenses:** Research and development expenses were $47.4 million for the third quarter of 2019, up from $15.9 million for the same period in 2018. R&D expenses in the first nine months of 2019 were $101.3 million, up from $49.7 million for the same period in 2018. The increase in R&D expenses was primarily driven by clinical trial activity for mavacamten, MYK-491, and MYK-224, a decrease in Sanofi reimbursement credits, increases in contract research, chemistry and biology expenses related to discovery and pre-clinical programs, increases in contract manufacturing expenses, and increases in headcount and stock-based compensation expense.

- **SG&A Expenses:** Selling, general and administrative expenses were $17.7 million for the three months ended September 30, 2019, compared to $11.0 million for the same period in 2018. For the first nine months of 2019, SG&A expenses were $45.2 million compared to $27.2 million in the same period in 2018. The increase in SG&A expenses was primarily attributable to an

increase in headcount, stock-based compensation expense, professional service and consulting expenses, and office and facilities-related expenses.

- **Net Loss:** Net loss for the third quarter of 2019 was $141.8 million ($3.07 per share), of which $80.0 million ($1.73 per share) was related to the non-recurring repurchase from Sanofi of royalty rights to mavacamten and MYK-224, and $61.8 million ($1.34 per share) was related to loss from operations, compared to a net loss of $15.8 million ($0.39 per share) for the third quarter of 2018. For the nine months ended September 30, 2019, net loss was $217.4 million ($4.91 per share), of which $80.0 million ($1.81 per share) was related to the repurchase of royalty rights and $137.4 million ($3.10 per share) was related to loss from operations, compared to $52.0 million ($1.38 per share) during the same period of 2018.

Based on the company's current balance of cash and investments, MyoKardia estimates having sufficient funds to execute on current operating plans into the second half of 2021.

In lieu of conducting a third quarter financial results call, MyoKardia management will host a conference call on Monday, November 11, 2019 at 8:30 a.m. ET/5:30 a.m. PT to discuss PIONEER-OLE 48-week data being presented at the upcoming American Heart Association Scientific Sessions 2019. Investors and analysts are invited to participate by phone by calling 844-494-0913 in the U.S. and Canada or 508-637-5584 internationally and using the conference ID 3177984 or by webcast. The webcast can be accessed from the investor section of the MyoKardia website at www.myokardia.com.

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 clinical and therapeutic potential of mavacamten, MYK-491 and MYK-224, the availability of the 48-week data from PIONEER-OLE and data from EXPLORER-HCM, as well as from the Phase 2 MAVERICK-HCM study in patients with non-obstructive HCM, the Company's expectation with respect to release of data from these studies, the Company's ability to advance MYK-491 into a Phase 2 study in patients with genetic DCM and the commencement of a study of mavacamten in symptomatic, obstructive HCM patients referred for SRT, and the timing of these events and the availability of data from the Phase 1 study of MYK-224 in healthy subjects, 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 ad 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 September 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.

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MYOKARDIA, INC.

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

	September 30, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 235,159	$ 246,122
Short-term investments	181,932	68,564
Prepaid expenses and other current assets	7,598	4,760
Total current assets	424,689	319,446
Property and equipment, net	7,148	5,138
Operating lease right-of-use assets	1,028	—
Long-term investments	63,328	80,148
Restricted cash and other	2,404	2,521
Total assets	$ 498,597	$ 407,253
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	6,528	$ 2,946
Accrued liabilities	36,507	20,758
Prepayment from collaboration partner	—	12,973
Operating lease liabilities - current	1,050	—
Total current liabilities	44,085	36,677
Other long-term liabilities	2,159	9
Total liabilities	46,244	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 September 30, 2019 and December 31, 2018; 46,218,925 and 40,288,949 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	5	4
Additional paid-in capital	871,894	573,183
Accumulated other comprehensive income (loss)	453	(67)
Accumulated deficit	(419,999)	(202,553)
Total stockholders' equity	452,353	370,567
Total liabilities and stockholders' equity	$ 498,597	$ 407,253

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,	
	2019	2018	2019	2018
Collaboration and license revenue	$ —	$ 9,188	$ —	$ 21,158
Operating expenses:				
Repurchase of royalty rights	80,000	—	80,000	—
Research and development	47,372	15,910	101,270	49,746
Selling, general and administrative	17,746	10,957	45,153	27,182
Total operating expenses	145,118	26,867	226,423	76,928
Loss from operations	(145,118)	(17,679)	(226,423)	(55,770)
Interest and other income, net	3,332	1,890	8,775	3,748
Loss before income taxes	(141,786)	(15,789)	(217,648)	(52,022)
Income tax expense (benefit)	16	—	(202)	—
Net loss	(141,802)	(15,789)	(217,446)	(52,022)
Other comprehensive (loss) income, net of tax effect of $17, $0, $(202) and $0, respectively	(44)	37	520	(30)
Comprehensive loss	$ (141,846)	$ (15,752)	$ (216,926)	$ (52,052)
Net loss per share, basic and diluted	$ (3.07)	$ (0.39)	$ (4.91)	$ (1.38)
Weighted average number of shares used to compute net loss per share, basic and diluted	46,133,068	40,116,644	44,255,657	37,765,631