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): February 28, 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. ☐

Item 2.02 Results of Operations and Financial Condition.

On February 28, 2019, MyoKardia, Inc. announced its financial results for the fourth quarter and year ended December 31, 2018. 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 February 28, 2019, 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: February 28, 2019

MyoKardia, Inc.

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

Exhibit 99.1

MyoKardia Reports Fourth Quarter and Full Year 2018 Financial Results

Company to Host Conference Call and Webcast on March 4, 2019 at 8:30 a.m. ET (5:30 a.m. PT)

SOUTH SAN FRANCISCO, Calif., February 28, 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 fourth quarter and full year ended December 31, 2018.

"2018 was MyoKardia's most significant year of progress towards building a leading cardiovascular company addressing the number one global health burden with novel precision medicines," said Tassos Gianakakos, Chief Executive Officer. "Our commitment to a mission-driven, values-based culture continues to be a key differentiator as we build a growing community of employees motivated to change the world for people with serious cardiovascular diseases. We've added incredible people at all levels of the company and strengthened our HCM disease-area leadership. In 2018, we initiated six clinical trials for mavacamten and MYK-491, including the pivotal Phase 3 EXPLORER-HCM trial, shared important clinical data from both, and unveiled three new therapeutic programs, including MYK-224 which will enter the clinic this year."

Gianakakos continued, "As we move into 2019, our pipeline is set up to generate meaningful clinical data across multiple programs, including six-month mavacamten data from our open-label PIONEER-OLE study to be presented at the upcoming American College of Cardiology annual meeting. Over the course of the year, we will report additional 12-month clinical data from PIONEER-OLE and complete enrollment for our Phase 3 EXPLORER-HCM trial, bringing us closer to delivering much-needed new therapies to people with HCM. Phase 2 data from our MAVERICK study of mavacamten in non-obstructed HCM is expected later this year and will generate important insights towards addressing diastolic heart disease. We also expect to share Phase 2 data from our MYK-491 program designed to improve systolic function in people with impaired cardiac contractility."

MyoKardia's management will conduct a conference call on Monday, March 4 at 8:30 a.m. ET/5:30 a.m. PT to discuss fourth quarter and year-end 2018 financial results. The call and webcast will also review six-month safety, efficacy and biomarker data from the PIONEER open-label extension (OLE) trial of mavacamten in patients with obstructive HCM. These data will become available electronically on March 4th and will be presented at the American College of Cardiology's 68th Annual Scientific Session being held March 16-18 in New Orleans.

Therapeutic Program Highlights

Mavacamten for Hypertrophic Cardiomyopathy (HCM)

- **In 2018, MyoKardia initiated four new clinical studies of mavacamten for the treatment of hypertrophic cardiomyopathy**, including**:** the pivotal Phase 3 EXPLORER-HCM trial in patients with obstructive hypertrophic cardiomyopathy (oHCM); the MAVERICK-HCM Phase 2 trial in non-obstructive HCM (nHCM); and two long-term safety studies, PIONEER-OLE and MAVA-LTE. Data from the MAVA-LTE clinical trial, along with results of the pivotal Phase 3 EXPLORER-HCM trial, are intended to support the registration submission for mavacamten for the treatment of obstructive HCM (oHCM).
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- **In October 2018, MyoKardia reported 12-week data from the PIONEER-OLE study.** Interim data for seven patients demonstrated statistically significant reductions in left ventricular outflow tract (LVOT) obstruction compared to baseline following treatment with mavacamten. Each patient's rate of ejection fraction remained well in the normal range of greater than 50 percent, and there were no significant adverse events reported.

- **In November 2018, MyoKardia presented preclinical and clinical evidence of mavacamten's potential to improve diastolic compliance.** The ability for the heart to fill with blood in between beats, or diastolic relaxation, is typically impaired in HCM. In a genetic mini-pig model of non-obstructive HCM, mavacamten was shown to decrease filling pressure, while increasing the end diastolic volume of the left ventricle. An analysis of echocardiographic measures of left ventricular relaxation, pressure and filling from MyoKardia's Phase 2 PIONEER-HCM clinical trial in oHCM patients also demonstrated a decrease in filling pressure and an

increase in the volume of blood that enters the left ventricle between heart beats. MyoKardia plans to report data from the ongoing Phase 2 MAVERICK-HCM clinical trial of mavacamten in nHCM in the second half of 2019.

- **Enrollment in the pivotal Phase 3 EXPLORER-HCM clinical trial remains on track with MyoKardia's expectations.** The 220-patient study is expected to complete enrollment in the second half of 2019, with topline data available from the pivotal Phase 3 in the second half of 2020.

MYK-491

- **This past December, MyoKardia reported positive topline results from its Phase 1b clinical study of MYK-491 in patients with stable heart failure.** MYK-491 was generally well-tolerated, and improvements in cardiac contractility of approximately 10 percent from baseline were observed across multiple echocardiographic measures, including stroke volume, left ventricular ejection fraction and fractional shortening. In increasing the heart's contractility, MYK-491 did not appear to meaningfully change duration of contraction or the heart's ability to relax and fill with oxygenated blood. Results from an ongoing multiple-ascending dose study of MYK-491 are anticipated in the second half of 2019.

Research

- **MyoKardia unveiled three new research programs at the company's fourth quarter 2018 R&D Day.** MYK-224, the company's second candidate addressing HCM, is expected to enter a Phase 1 clinical study in mid-2019. ACT-1, a proprietary cardiac muscle activator, is being developed for the treatment of genetic dilated cardiomyopathy (DCM). LUS-1, the first known compound to specifically target impaired cardiac relaxation, is being developed for homogenous subgroups of patients with diseases of diastolic dysfunction.

- **Strengthened HCM disease area leadership with two new initiatives focused on increasing understanding of HCM.** MyoKardia initiated the inaugural MyoSeeds™ Research Grant Program to encourage early-stage research in HCM and DCM. MyoKardia also formed a partnership with 23andMe to advance research for patients with HCM.

Recent Corporate Highlights

- **Expanded senior leadership team.** Following robust organizational development in 2018, including the appointments of Cynthia Ladd as General Counsel and Taylor Harris as Chief Financial Officer, MyoKardia recently added William Fairey and Jay Edelberg, M.D., Ph.D, to the company's senior leadership team.
    - Fairey joined MyoKardia in the role of Chief Commercial Officer and will have global responsibility for oversight of all aspects of commercial operations. Most recently, Mr. Fairey served as Chief Commercial Officer of ChemoCentryx, Inc. and President of Actelion Pharmaceuticals, US, overseeing over 400 employees and $1.5 billion in net sales in 2017 before its acquisition by Johnson and Johnson for $30 billion. At Actelion US, he helped the company gain FDA approvals and launch three important therapies, OPSUMIT® and UPTRAVI® for pulmonary arterial hypertension and VALCHLOR® for cutaneous T-cell lymphoma.
    - Dr. Edelberg a clinical cardiologist and vascular biologist, having served in leadership roles for two decades in both academic medicine and the biopharmaceutical industry has recently been appointed Senior Vice President of Clinical Development. Most recently, Dr. Edelberg led cardiovascular drug development at Sanofi, where he spearheaded the development of Praluent® and oversaw the clinical development and global project teams involved in the MyoKardia partnership. Previously, Dr. Edelberg directed cardiovascular biomarker research at Bristol-Myers Squibb, where he served as the U.S. medical lead for Eliquis®, and at Glaxo SmithKline. Prior to his time in industry, he served as an Associate Professor of Medicine (Cardiology) at Weill Medical College of Cornell University.

- **In January, MyoKardia announced that the collaboration with Sanofi was ending.** MyoKardia regained worldwide rights to clinical-stage candidates mavacamten and MYK-491, as well as certain preclinical programs that had been the subject of the company's collaboration with Sanofi. The collaboration will conclude in its entirety effective April 1, 2019. As a result, MyoKardia now has global commercial rights to all programs in its portfolio. In February 2019, Sanofi sold its equity holdings in MyoKardia, consistent with its investment policy, and no longer holds any shares in the company.

Fourth Quarter and Full Year 2018 Financial Results

- **Cash Position:** Cash, cash equivalents and investments (short-term and long-term) as of December 30, 2018 were $394.8 million, compared to $276.4 million as of December 31, 2017. The increase in the company's cash position is primarily attributable to proceeds received from a follow-on offering of common stock in May 2018.

- **Revenues**: Collaboration and license revenue was $12.4 million during the three months ended December 31, 2018, compared with $3.0 million during the same period in 2017. Full-year collaboration and license revenue was $33.6 million compared to $11.4 million for the full year 2017. The increases in 2018 reflect the higher level of research and development efforts incurred in 2018 as compared to the same periods in 2017. Collaboration and license revenue reflects adoption of Accounting Standards Codification Topic 606, Revenue from Contracts with Customers ("ASC 606"). Under ASC 606, the $45 million payment received from Sanofi S.A. (Sanofi) in the first quarter of 2017 is being recognized based on research and development costs incurred in each period as a percent of estimated total program costs to be incurred in the two years ended December 31, 2018.
- **R&D Expenses:** Research and development expenses were $19.0 million in the fourth quarter 2018, net of reimbursement credits from Sanofi of $8.3 million, up from $8.2 million (net of Sanofi credits of $7.3 million) for the same period in 2017. Research and development expenses totaled $68.8 million for the full year 2018, compared to $48.1 million for the same period in 2017. These amounts are net of reimbursement credits from Sanofi of $23.1 million and $7.3 million, respectively. Cash received during 2018 from Sanofi totaled $12.9 million and $32.7 million for the three- and twelve-month periods, respectively. The increase in R&D expenses over the course of 2018 was primarily driven by the clinical trials for mavacamten and MYK-491, research activity for multiple preclinical programs, an increase in headcount, and stock-based compensation expense.

- **G&A Expenses:** General and administrative expenses were $11.3 million for the three months ended December 31, 2018, compared to $5.5 million for the same period in 2017. For the twelve months ended December 31, 2018, G&A expenses were $38.4 million, compared to $22.0 million for the prior year. The change in G&A expenses was primarily attributable to an increase in employee headcount, stock-based compensation expense, facilities-related and other administrative expenses.

- **Net Loss**: Net loss was $15.7 million ($0.39 loss per share) for the fourth quarter of 2018, compared to a net loss of $10.1 million ($0.28 loss per share) for the fourth quarter of 2017. For full year 2018, net loss was $67.7 million ($1.76 loss per share), compared to $57.0 million ($1.74 loss per share) for the same period in 2017.

Financial Guidance
Based on its current operating plans, MyoKardia anticipates that current cash, cash equivalents and investments are sufficient to fund operations into late 2020.

Conference Call and Webcast
MyoKardia management will host a conference call and live audio webcast on Monday, March 4, 2019, at 8:30 a.m. ET / 5:30 a.m. PT to discuss current operations and fourth quarter and year end 2018 financial results and review new data from the PIONEER-OLE study. 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 7688075. 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 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, the Company's ability to enroll patients in its long-term safety studies, PIONEER-OLE and MAVA-LTE, and the availability of data from EXPLORER-HCM, MAVERICK-HCM, the MYK-491 Phase 2a multiple ascending dose trial and PIONEER-OLE and MAVA-LTE, 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.

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

	As of December 31,	
	2018	**2017**
		As Revised
Assets		
Current assets		
Cash and cash equivalents	$ 246,122	$ 224,571
Short-term investments	68,564	31,933
Receivable from collaboration partner	—	1,013
Prepaid expenses and other current assets	4,760	1,876
Total current assets	319,446	259,393
Property and equipment, net	5,138	3,147
Long-term investments	80,148	19,900
Restricted cash and other	2,521	368
Total assets	$ 407,253	$ 282,808
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 2,946	$ 2,301
Accrued liabilities	20,758	11,639
Prepayment from collaboration partner	12,973	4,432
Deferred revenue	—	33,558
Total current liabilities	36,677	51,930
Other long-term liabilities	9	202
Total liabilities	36,686	52,132
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 December 31, 2018 and 2017; 40,288,949 and 35,812,791 shares issued and outstanding at December 31, 2018 and 2017, respectively	4	4
Additional paid-in capital	573,183	365,719
Accumulated other comprehensive loss	(67)	(192)
Accumulated deficit	(202,553)	(134,855)
Total stockholders' equity	370,567	230,676
Total liabilities and stockholders' equity	$ 407,253	$ 282,808

MYOKARDIA, INC.

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

	Year Ended December 31,		
	2018	2017	2016
		As Revised	
Collaboration and license revenue	$ 33,558	$ 11,442	$ 41,971
Operating expenses:			
Research and development, net	68,774	48,136	36,215
General and administrative	38,435	21,973	16,289
Total operating expenses	107,209	70,109	52,504
Loss from operations	(73,651)	(58,667)	(10,533)
Interest and other income, net	5,953	1,657	153
Net loss	(67,698)	(57,010)	(10,380)
Other comprehensive income (loss)	125	(200)	8
Comprehensive loss	$ (67,573)	$ (57,210)	$ (10,372)
Net loss per share, basic and diluted	$ (1.76)	$ (1.74)	$ (0.38)
Weighted average number of shares used to compute net loss per share, basic and diluted	38,386,906	32,832,514	27,475,792

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