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): August 4, 2020

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.)**

1000 Sierra Point Parkway
Brisbane, CA 94005
(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 August 4, 2020, MyoKardia, Inc. announced its financial results for the second quarter ended June 30, 2020. 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 August 4, 2020, 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: August 4, 2020

MyoKardia, Inc.

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

Exhibit 99.1

News Release

MYOKARDIA

MyoKardia Reports Second Quarter 2020 Financial Results

Brisbane, Calif., August 4, 2020 -- MyoKardia, Inc. (Nasdaq: MYOK), today reported financial results for the second quarter ended June 30, 2020.

“The second quarter of 2020 was one of incredible progress across our R&D pipeline, including the exciting new data from our danicamtiv program highlighting its unique ability to directly activate both the left atrium and the left ventricle, and capped off by the positive results from our pivotal Phase 3 EXPLORER trial. These data, together with a successful financing, position us well in the pursuit of our mission to change the world for the tens of millions of people with serious cardiovascular disease,” said Tassos Gianakakos, MyoKardia’s Chief Executive Officer. “We are highly focused on the U.S. regulatory approval and successful launch of mavacamten and continue to develop evidence supporting its potential as a backbone therapy for people with HCM and possibly beyond. Beyond mavacamten and danicamtiv, the application of our R&D platform continues to be fruitful and is generating proprietary scientific insights that could lead to several important new therapies.”

Clinical Program Highlights

Mavacamten for Hypertrophic Cardiomyopathy

- **Positive Phase 3 Topline Data from EXPLORER-HCM:** MyoKardia announced positive topline data from its Phase 3 pivotal EXPLORER-HCM clinical trial of mavacamten for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (HCM). Mavacamten demonstrated a robust treatment effect: the primary and all secondary endpoints were met with statistical significance ($p \leq 0.0006$). Mavacamten was well tolerated, and meaningful improvements in symptoms, functional status and quality of life, as well as reduction or elimination in obstruction of the left ventricle, were observed among patients on treatment versus placebo.

- **EXPLORER Data Accepted for Late-Breaker Presentation at ESC 2020:** The Phase 3 data from the EXPLORER-HCM clinical trial have been accepted as a late-breaker presentation at the upcoming virtual European Society of Cardiology (ESC) meeting. Dr. Iacopo Olivotto, from the Careggi University Hospital, Florence, Italy, will present the data on Saturday, August 29, 2020 at 6:30 CET.

- **Breakthrough Therapy Designation Granted by FDA:** MyoKardia announced that the U.S. Food and Drug Administration granted Breakthrough Therapy Designation to mavacamten for the treatment of symptomatic, obstructive HCM. Breakthrough Therapy Designation is intended to expedite the development and review of a drug candidate that is planned for use to treat a serious or life-threatening disease or condition when clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. MyoKardia plans to submit a New Drug Application to the FDA in the first quarter of 2021.

- **Initiated VALOR-HCM Phase 3 Clinical Trial:** The Phase 3 VALOR-HCM trial is a randomized, double-blind, placebo-controlled, multicenter study designed to provide direct clinical evidence of mavacamten’s ability to improve outcomes for individuals with HCM by reducing the need for invasive septal reduction therapy (SRT) procedures. VALOR-HCM is being conducted in partnership with Cleveland Clinic C5Research, an academic research organization, with participation from approximately 15 HCM specialty centers with well-recognized expertise in SRT procedures in the U.S.

Danicamtiv for Dilated Cardiomyopathy

- **Presented and Published Encouraging Danicamtiv Clinical and Non-clinical Data:** In a Phase 2a clinical trial, danicamtiv was generally well-tolerated in patients with stable chronic heart failure and was associated with clinically meaningful improvements in left ventricular (LV) contractility, including statistically significant increases in LV stroke volume, without impairing the heart's ability to relax and fill. Treatment with danicamtiv also improved left atrial (LA) volume and function, a new and potentially important finding given LA size is a well-established prognostic factor for atrial fibrillation. Data from nonclinical studies indicate that danicamtiv directly activates the LV and LA, providing mechanistic rationale for the robust changes observed clinically. These data were presented at the European Society of Cardiology's Heart Failure Association (HFA) Discoveries online event, and published in the *European Journal of Heart Failure*.

  Pending circumstances related to the coronavirus pandemic, MyoKardia expects to initiate a Phase 2 study in patients with genetic dilated cardiomyopathy (DCM) in the second half of 2020 and a Phase 2 study in patients with systolic heart failure and paroxysmal or persistent atrial fibrillation in the first half of 2021.

Research

- **Entered into Collaboration with Fulcrum Therapeutics:** MyoKardia announced a strategic collaboration and license agreement with Fulcrum Therapeutics. Under the agreement, MyoKardia will access Fulcrum's proprietary target discovery engine to identify therapeutics that control the expression of genes known to be underlying drivers of genetic cardiomyopathies. MyoKardia will be responsible for all development and commercialization activities for, and will have global rights to, any potential therapeutics identified through this collaboration.

In March 2020, in response to the coronavirus pandemic, MyoKardia paused new patient enrollment in the MAVA-LTE long-term extension study of mavacamten and in the Phase 1 healthy volunteer study of MYK-224. The company has subsequently resumed enrollment in both studies.

Recent Corporate Highlights

- **Announced New General Counsel**: MyoKardia appointed Denelle J. Waynick as General Counsel and Corporate Secretary. Cynthia Ladd, MyoKardia's former General Counsel, will be retiring following a transition period in the second half of 2020.

- **Completed Successful Follow-On Offering:** MyoKardia completed a follow-on offering of 6,037,500 shares of its common stock at a public offering price of $105.00 per share, raising approximately $605.0 million in net proceeds. MyoKardia anticipates using net proceeds from the offering to support the regulatory approval process and potential commercial launch of mavacamten for the treatment of obstructive HCM; to fund ongoing and potential later-stage clinical trials of mavacamten in non-obstructive HCM and HFpEF, danicamtiv in targeted segments of systolic heart failure and MYK-224; to advance ACT-1 and LUS-1 into clinical development; to fund ongoing preclinical, discovery and research programs, and for working capital, business development and other general corporate purposes.

Second Quarter 2020 Financial Results

Cash Position: Cash, cash equivalents and investments as of June 30, 2020 were $918.1 million, compared to $430.3 million as of December 31, 2019. The increase in MyoKardia's cash position is primarily attributable to net proceeds of approximately $605.0 million from MyoKardia's follow-on offering of common stock, which priced in May 2020.

R&D Expenses: Research and development expenses were $44.3 million for the second quarter of 2020, compared to $27.7 million for the same period in 2019. The increase in R&D expenses for the

second quarter of 2020 was primarily driven by the absence of R&D reimbursements from Sanofi, our previous collaboration partner, increased personnel costs including stock-based compensation, as well as increases in R&D infrastructure, consulting, and contract manufacturing expenses.

G&A Expenses: General and administrative expenses were $20.3 million for the three months ended June 30, 2020, compared to $13.9 million for the same period in 2019. The increase in G&A expenses for the second quarter of 2020 was primarily driven by an increase in personnel costs including stock-based compensation, as well as increases in professional fees and marketing expenses.

Net Loss: Net loss for the second quarter of 2020 was $63.6 million ($1.27 per share), compared to a net loss of $38.2 million ($0.83 loss per share) for the second quarter of 2019.

Conference Call and Webcast
MyoKardia management will host a conference call and live audio webcast on Tuesday, August 4, 2020, at 4:30 p.m. ET / 1:30 p.m. PT to discuss current operations and second quarter 2020 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 4340819. 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 discovering and developing targeted therapies for the treatment of serious cardiovascular diseases. The company is pioneering a precision medicine approach to its discovery and development efforts by 1) understanding the biomechanical underpinnings of disease; 2) targeting the proteins that modulate a given condition; 3) identifying patient populations with shared disease characteristics; and 4) applying learnings from research and clinical studies to inform and guide pipeline growth and product advancement. MyoKardia's initial focus is on small molecule therapeutics aimed at the proteins of the heart that modulate cardiac muscle contraction to address diseases driven by excessive contraction, impaired relaxation, or insufficient contraction. Among its discoveries are three clinical-stage therapeutics: mavacamten (formerly MYK-461); danicamtiv (formerly MYK-491) and MYK-224.

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 timing with respect to submission of a New Drug Application to the FDA for mavacamten, expectation and timing of approval and launch of mavacamten in the US, the likelihood of developing evidence supporting mavacamten's use as a backbone therapy for individuals with HCM and other populations, the ongoing enrollment of patients in the Phase 3 VALOR-HCM clinical trial as well as our expectation that the clinical trial design will result in direct clinical evidence supporting mavacamten's ability to improve outcomes for individuals with HCM by reducing the need for invasive SRT procedures, the clinical and therapeutic potential of danicamtiv, the ability to continue to discover and develop our pipeline of targeted cardiovascular medicines, as well as the potential for identifying therapeutics controlling the expression of genes known to be underlying drivers of genetic cardiomyopathies and the subsequent potential development and commercialization as a result of the collaboration with Fulcrum Therapeutics, 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 March 31, 2020, 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)

	June 30, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 659,107	$ 101,436
Short-term investments	258,946	314,691
Prepaid expenses and other current assets	7,075	7,709
Total current assets	925,128	423,836
Property and equipment, net	19,703	15,743
Operating lease right-of-use assets	51,189	417
Long-term investments	—	14,153
Restricted cash and other	2,706	1,945
Total assets	$ 998,726	$ 456,094
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 3,349	$ 6,237
Accrued liabilities	32,600	41,292
Operating lease liabilities - current	8,042	383
Total current liabilities	43,991	47,912
Operating lease liability	44,188	—
Other long-term liabilities	1,908	1,908
Total liabilities	90,087	49,820
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 June 30, 2020 and December 31, 2019; 52,965,033 and 46,379,073 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	5	5
Additional paid-in capital	1,520,101	884,486
Accumulated other comprehensive income	752	549
Accumulated deficit	(612,219)	(478,766)
Total stockholders' equity	908,639	406,274
Total liabilities and stockholders' equity	$ 998,726	$ 456,094

MYOKARDIA, INC.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 44,334	$ 27,708	$ 96,212	$ 53,898
Selling, general and administrative	20,251	13,856	40,153	27,407
Total operating expenses	64,585	41,564	136,365	81,305
Loss from operations	(64,585)	(41,564)	(136,365)	(81,305)
Interest and other income, net	1,012	3,172	2,924	5,443
Loss before income taxes	(63,573)	(38,392)	(133,441)	(75,862)
Income tax expense (benefit)	12	(218)	12	(218)
Net loss	(63,585)	(38,174)	(133,453)	(75,644)
Other comprehensive income	81	201	203	564
Comprehensive loss	$ (63,504)	$ (37,973)	$ (133,250)	$ (75,080)
Net loss per share, basic and diluted	$ (1.27)	$ (0.83)	$ (2.77)	$ (1.75)
Weighted average number of shares used to compute net loss per share, basic and diluted	49,878,578	46,065,901	48,222,787	43,301,417