



## MyoKardia Reports Fourth Quarter and Full Year 2019 Financial Results

February 27, 2020

BRISBANE, Calif., Feb. 27, 2020 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq: MYOK), today reported financial results for the fourth quarter and full year ended December 31, 2019.

"MyoKardia achieved significant progress in 2019, advancing our pipeline and regaining global rights to our entire portfolio. There were important data readouts across our clinical programs, including one-year safety and efficacy data from our PIONEER-OLE study of mavacamten in obstructive HCM; encouraging results from our Phase 2 MAVERICK study in non-obstructive HCM pointing to mavacamten's ability to favorably impact diastolic relaxation; positive interim data from our Phase 2a study of danicamtiv in patients enabling us to move forward in a genetic DCM population; and the initiation of our Phase 1 study of MYK-224 in HCM," said Tassos Gianakakos, MyoKardia's Chief Executive Officer. "These accomplishments position us for a transformational year in 2020, which will include results from our pivotal Phase 3 EXPLORER study of mavacamten and multiple additional milestones across our pipeline, further demonstrating the value of our precision medicine approach to cardiovascular therapeutic development."

### **2020 Anticipated Milestones**

#### *Mavacamten*

- Present complete results from the Phase 2 MAVERICK-HCM clinical trial of mavacamten in patients with non-obstructive hypertrophic cardiomyopathy (HCM) at the upcoming American College of Cardiology/World Cardiology Congress joint meeting in March 2020
- Announce topline data from the Phase 3 EXPLORER-HCM clinical trial of mavacamten for the treatment of obstructive HCM in the second quarter of 2020
  - Present complete results from the EXPLORER-HCM trial before year-end
- Initiate a Phase 2 clinical trial of mavacamten in a targeted population of patients with heart failure with preserved ejection fraction (HFpEF) in the second quarter of 2020
- Initiate VALOR-HCM, the Phase 3 study of mavacamten as a possible alternative to septal reduction therapy (SRT) in mid-2020
- Initiate a late-stage study of mavacamten in non-obstructive HCM before year-end
  - Provide update from regulatory interactions in the first half of 2020

#### *Danicamtiv (formerly MYK-491)*

- Present complete results from the Phase 2a study of danicamtiv in patients with stable heart failure in the first half of 2020
- Initiate a Phase 2 study of danicamtiv in patients with genetic dilated cardiomyopathy (DCM) in the second quarter of 2020

#### *MYK-224*

- Share results from the Phase 1 clinical trial of MYK-224 in healthy volunteers in the third quarter of 2020
- Initiate a Phase 2 study of MYK-224 by year end

### **Recent Clinical Program Highlights**

#### *Mavacamten for Hypertrophic Cardiomyopathy*

- **Presented 48-week Data from the PIONEER-OLE Study of Mavacamten:** At the American Heart Association Scientific Sessions, 48-week data from the PIONEER open-label extension study of 12 patients with obstructive HCM were presented. Consistent with data reported at 12, 24 and 36 weeks, treatment with mavacamten was generally well-tolerated, and patients continue to experience sustained clinical benefit, including reductions in left ventricular outflow tract (LVOT) gradient, improvements in NYHA functional class and improvement of multiple biomarkers toward normal ranges. A reduction in septal wall thickness, a defining characteristic of HCM, as well as an improvement in patient reported quality of life, as measured by the Kansas City Cardiomyopathy Questionnaire (KCCQ), were also reported.
- **Reported Positive Topline Data from the Phase 2 MAVERICK-HCM Study:** Mavacamten was generally well-tolerated in patients with non-obstructive HCM in the 16-week dose-ranging study. Meaningful reductions in biomarkers of cardiac stress were observed in patients treated across both mavacamten drug concentration cohorts, and clear signals of clinical benefit were seen in a subgroup of patients with elevated cardiac filling pressure at baseline and in a pre-specified group of patients at higher risk for morbidity and mortality.

#### *Danicamtiv for Dilated Cardiomyopathy*

- **Reported Positive Data from Phase 2a Study of Danicamtiv:** 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 danicamtiv 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 was extended to evaluate higher exposures of danicamtiv.

#### Fourth Quarter and Full Year 2019 Financial Results

**Cash Position:** Cash, cash equivalents and investments (short-term and long-term) as of December 31, 2019 were \$430.3 million, compared to \$394.8 million as of December 31, 2018. The change in MyoKardia's cash position was primarily attributable to net proceeds from the March 2019 equity offering of \$271.2 million, offset by expenditures related to operating activities of \$241.8 million, which included an \$80.0 million payment to Sanofi for the purchase of mavacamten and MYK-224 royalty rights.

**Revenues:** MyoKardia did not record revenue during the fourth quarter and year ended December 31, 2019. For the three and twelve months ended December 31, 2018, collaboration and license revenue from the company's collaboration with Sanofi totaled \$12.4 million and \$33.6 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 \$44.9 million for the fourth quarter of 2019, up from \$19.0 million for the same period in 2018. R&D expenses totaled \$146.2 million for the full year 2019, up from \$68.8 million for the same period in 2018. The increase in R&D expenses was primarily driven by trial expenditures related to mavacamten, danicamtiv and MYK-224 clinical programs, a decrease in Sanofi reimbursement credits, increases in expenses related to discovery and preclinical programs, and increases in headcount and stock-based compensation expense.

**G&A Expenses:** General and administrative expenses were \$16.5 million for the three months ended December 31, 2019, compared to \$11.3 million for the same period in 2018. For the year ended December 31, 2019, G&A expenses were \$61.7 million compared to \$38.4 million in the same period in 2018. The increase in G&A expenses was primarily due to increases in headcount and related costs to support the growth of our research and development activities and to prepare for the potential commercialization of mavacamten.

**Net Loss:** Net loss for the fourth quarter of 2019 was \$58.8 million (\$1.27 per share), compared to a net loss of \$15.7 million (\$0.39 per share) for the fourth quarter of 2018. For full year 2019, net loss was \$276.2 million (\$6.17 per share), of which \$80.0 million (\$1.79 per share) was related to the repurchase of royalty rights and \$196.2 million (\$4.38 per share) was related to loss from operations, compared to \$67.7 million (\$1.76 per share) during the same period of 2018.

Based on the company's current cash position, MyoKardia estimates having sufficient funds to execute on current operating plans into mid-2021.

#### Conference Call and Webcast

MyoKardia management will host a conference call and live audio webcast on Thursday, February 27, 2020, at 4:30 p.m. ET / 1:30 p.m. PT to discuss current operations and fourth quarter and year end 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 8698045. 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 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 the clinical and therapeutic potential of mavacamten, danicamtiv and MYK-224, the availability of data from the Phase 3 EXPLORER-HCM clinical trial of mavacamten for the treatment of obstructive HCM, the Phase 2 MAVERICK-HCM study in patients with non-obstructive HCM and the Phase 2a study of danicamtiv in patients with stable heart failure and the Company's expectation with respect to release of data from these studies, the Company's ability to advance danicamtiv into a Phase 2 study in patients with genetic DCM, the commencement of a Phase 2 clinical trial of mavacamten in a targeted population of patients with HFpEF, the commencement of VALOR-HCM, the Phase 3 study of mavacamten as a possible alternative to SRT, the commencement of the late-stage study of mavacamten in non-obstructive HCM, the availability of data from the Phase 1 study of MYK-224 in healthy subjects and the commencement of a Phase 2 study of MYK-224, 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, 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.**

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

	December 31,	
	2019	2018
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 101,436	\$ 246,122
Short-term investments	314,691	68,564
Prepaid expenses and other current assets	7,709	4,760
Total current assets	423,836	319,446
Property and equipment, net	15,743	5,138
Operating lease right-of-use assets	417	—
Long-term investments	14,153	80,148
Restricted cash and other	1,945	2,521
Total assets	<u>\$ 456,094</u>	<u>\$ 407,253</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities		
Accounts payable	\$ 6,237	\$ 2,946
Accrued liabilities	41,292	20,758
Prepayment from collaboration partner	—	12,973
Operating lease liabilities - current	383	—
Total current liabilities	47,912	36,677
Other long-term liabilities	1,908	9
Total liabilities	49,820	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 December 31, 2019 and 2018; 46,379,073 and 40,288,949 shares issued and outstanding at December 31, 2019 and 2018, respectively	5	4
Additional paid-in capital	884,486	573,183
Accumulated other comprehensive income (loss)	549	(67)
Accumulated deficit	(478,766)	(202,553)
Total stockholders' equity	406,274	370,567
Total liabilities and stockholders' equity	<u>\$ 456,094</u>	<u>\$ 407,253</u>

MYOKARDIA, INC.

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

	Year Ended December 31,		
	2019	2018	2017
Collaboration and license revenue	\$ —	\$ 33,558	\$ 11,442
Operating expenses:			
Repurchase of royalty rights	80,000	—	—
Research and development	146,171	68,774	48,136
Selling, general and administrative	61,663	38,435	21,973
Total operating expenses	287,834	107,209	70,109
Loss from operations	(287,834)	(73,651)	(58,667)
Interest and other income, net	11,621	5,953	1,657
Net loss	(276,213)	(67,698)	(57,010)
Other comprehensive income (loss)	616	125	(200)
Comprehensive loss	\$ (275,597)	\$ (67,573)	\$ (57,210)
Net loss per share, basic and diluted	\$ (6.17)	\$ (1.76)	\$ (1.74)
Weighted average number of shares used to compute net loss per share, basic and diluted	44,765,496	38,386,906	32,832,514



Source: MyoKardia, Inc.