MyoKardia Reports Fourth Quarter and Full Year 2017 Financial Results

Announced Achievement of Primary Endpoint and Additional Key Data from Low-Dose Mavacamten Cohort of PIONEER-HCM Phase 2 Study in Symptomatic, Obstructive Hypertrophic Cardiomyopathy

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

SOUTH SAN FRANCISCO, Calif., March 08, 2018 (GLOBE NEWSWIRE) -- 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 fourth quarter and full year ended December 31, 2017. In addition, the company also announced positive results from the PIONEER-HCM Phase 2 proof-of-concept trial, including results from the low-dose cohort, in a press release issued this afternoon.

“Following an eventful and important year of progress, we are poised in 2018 to move closer to achieving our mission to change the lives of patients suffering from serious cardiovascular diseases,” said Tassos Gianakakos, Chief Executive Officer. “As we prepare to initiate our Phase 3 EXPLORER-HCM pivotal trial, the additional PIONEER-HCM study data announced today increase our confidence in mavacamten’s potential to help patients with oHCM. This enthusiasm carries over into our growing clinical pipeline, which also includes the Phase 2 study of mavacamten in non-obstructive HCM and the continued clinical study of MYK-491 in dilated cardiomyopathy patients.”

Full Year 2017 and Recent Clinical Program Highlights

Mavacamten for Hypertrophic Cardiomyopathy (HCM)

- **Mavacamten Met Primary Endpoint and Key Secondary Endpoints of PIONEER-HCM Study in Both Study Cohorts:** In the PIONEER-HCM Phase 2 clinical trial of 21 symptomatic obstructive HCM patients, mavacamten achieved the primary endpoint of reduction in post-exercise left ventricular outflow tract (LVOT) gradient from baseline to week 12 with statistical significance across both cohorts tested. Improvements in peak VO_2, New York Heart Association (NYHA) classification and dyspnea rating scores were also observed. Mavacamten was generally well-tolerated.

- **Mavacamten Activity was Persistent and Safety was not Discernably Impacted by Use of Background Beta Blockers:** Benefits were observed across endpoints among those taking once-daily 2mg and 5mg doses of mavacamten who remained on background beta blocker therapy (“Cohort B”), and those who received daily doses ranging from 10mg, 15mg and 20mg who had discontinued background medications (as presented at the Heart Failure Society of America Annual Meeting in September 2017).

- **Target Concentration Range Identified to Inform EXPLORER-HCM Trial:** The PIONEER-HCM study has informed a target concentration

range at which mavacamten is expected to achieve a clinical improvement in oHCM symptoms (e.g., NYHA class) and exercise capacity (peak VO_2) while maintaining normal ejection fraction of greater than or equal to 50 percent. Data from the PIONEER-HCM study will guide the starting dose and inform dose-adjustments for MyoKardia's planned Phase 3 pivotal EXPLORER-HCM clinical trial expected to start in the second quarter of 2018.

- **Mavacamten Registration Program Outlined with FDA in September:** MyoKardia established 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 in consultation with the U.S. Food and Drug Administration's (FDA) Division of Cardiovascular and Renal Products during an end-of-Phase 2 meeting following results from the first cohort of the PIONEER-HCM clinical trial.

- **Presented New Data at AHA**: MyoKardia researchers presented new digital health and *in vivo* preclinical data related to its mavacamten program at the American Heart Association Scientific Sessions in November 2017.

MYK-491 for Dilated Cardiomyopathy (DCM)

- **First-in-Human Phase 1 Clinical Trial of MYK-491 Completed:** In a single-ascending dose study of healthy volunteers, MYK-491 was generally well tolerated across the range of oral doses tested. Adverse events (AE) observed were benign and transient. Evidence of clinical proof-of-mechanism was observed at higher dose levels in the form of increased contractility, as measured by echocardiographic biomarkers.

- **MYK-491 Phase 1b Study in DCM Patients Initiated:** A single-ascending dose study of MYK-491 in symptomatic DCM patients was initiated with first patients dosed in February 2018. 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 DCM patients.

Corporate Updates

- Kevin Starr, Partner at Third Rock Ventures, has resigned from MyoKardia's Board. MyoKardia thanks Mr. Starr for his significant contributions to the company's formation and growth. He was not a member of any board committees.

- Cynthia Ladd joined MyoKardia as General Counsel in January 2018.

2018 Anticipated Milestones

- Present complete results from PIONEER-HCM Phase 2 clinical trial of mavacamten in symptomatic oHCM patients, including data from Cohort B assessing lower doses, during the “Highlighted Original Research: Heart Failure and Cardiomyopathies and the Year in Review” session at the American College of Cardiology (ACC) 67th Annual Scientific Session on March 11, 2018 at 8:00 a.m. ET

- Begin Pivotal Study EXPLORER-HCM for Mavacamten in Symptomatic oHCM
  -- Finalize EXPLORER-HCM Phase 3 protocol to assess mavacamten for the treatment of symptomatic oHCM and provide a regulatory update in the second quarter 2018
  -- Initiate patient dosing in the Phase 3 EXPLORER-HCM clinical trial of mavacamten in second quarter 2018
  -- Initiate patient dosing in the long term EXPLORER-HCM extension study before the end of 2018

- Begin open-label extension study for patients who participated in the Phase 2 PIONEER-HCM clinical trial of mavacamten in second quarter 2018 (PIONEER-OLE clinical trial)

- Initiate Phase 2 MAVERICK-HCM clinical study of mavacamten for the treatment of non-obstructive HCM (nHCM) in first quarter 2018

- Report topline data from Phase 1b clinical trial of MYK-491 in DCM patients in the second half of 2018

- Begin Phase 2 clinical trial of MYK-491 in DCM patients in second half of 2018

Fourth Quarter and Full Year 2017 Financial Results

- **Cash Position:** Cash and cash equivalents as of December 30, 2017 were $224.6 million, compared to $135.8 million as of December 31, 2016. Investments (short-term and long-term) as of December 31, 2017 were $51.8 million, compared to $16.1 million as December 31, 2016. The increase in the company’s cash position is primarily attributable to proceeds received from the follow-on offering of common stock.

- **Revenues:** Collaboration and license revenue was $5.6 million during the three months ended December 31, 2017, compared with $28.6 million during the same period in 2016. Full-year collaboration and license revenue was $22.5 million compared to $39.2 million for the full year 2016. The decrease in collaboration revenue in 2017 was attributable to recognition of a $25.0 million one-time milestone payment in the fourth quarter of 2016 for the submission of an Investigational New Drug

application for MYK-491 with the FDA in November 2016.

- **R&D Expenses:** Research and development expenses, before consideration of $7.3 million in cost sharing for registration program plan (RPP) costs for the fourth quarter 2017 were $15.5 million, up from $10.0 million for the same period in 2016. Research and development expenses were $48.1 million for the full year 2017, compared to $36.2 million for the same period in 2016. The increase in R&D expenses over the course of 2017 were 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 $5.5 million for the three months ended December 31, 2017, compared to $4.3 million for the same period in 2016. For the twelve months ended December 31, 2017, G&A expenses were $22.0 million, compared to $16.3 million for the prior year. 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 $7.4 million ($0.21 loss per share) for the fourth quarter of 2017, compared to a net income of $14.3 million ($0.46 net income per share, basic or $0.44 net income per share, fully diluted) for the fourth quarter of 2016. For full year 2017, net loss was $46.0 million ($1.40 loss per share), compared to $13.1 million($0.48 loss per share) for the same period in 2016. The net loss per share in the fourth quarter of 2017 versus the net income per share in 2016 was due to the $25 million received in the fourth quarter of 2016 as noted above.

2018 Financial Guidance

- Based on its current operating plans, MyoKardia expects that its cash, cash equivalents and investments as of December 31, 2017, together with anticipated payments from Sanofi under its collaboration agreement, will fund anticipated operating expenses and capital expenditure requirements into 2020.

Conference Call and Webcast

MyoKardia management will host a conference call and live audio webcast today, March 8, at 4:30 p.m. ET / 1:30 p.m. PT to review data from the Phase 2 PIONEER-HCM clinical trial, as well as fourth quarter and year end 2017 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 8496928. 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 two of the 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 intended to reduce hypercontractility. Mavacamten is advancing into a pivotal Phase 3 clinical trial, known as EXPLORER-HCM in patients with symptomatic, obstructive HCM and a Phase 2 trial, the MAVERICK-HCM study, in patients with non-obstructive HCM. 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 1b study in DCM patients. 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, our use of data from the PIONEER-HCM trial to guide the starting dose and inform the dose-adjustments for our planned Phase 3 EXPLORER-HCM trial, the initiation of patient dosing in the Phase 3 EXPLORER-HCM trial, the trial design for EXPLORER-HCM, the release of complete results from the Company's Phase 2 PIONEER-HCM trial, the commencement of a Phase 2 trial of mavacamten in nHCM patients, the open-label extension study for patients who participated in the Phase 2 PIONEER-HCM trial, the release of topline data from the Phase 1b clinical trial of MYK-491 in DCM patients and the initiation of the Company's planned Phase 2 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 Annual Report on Form 10-K for the year ended December 31, 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.
Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share amounts)

	Year Ended
	2017
Collaboration and license revenue	$ 22,500
Operating expenses:	
Research and development, net	48,136
General and administrative	21,973
Total operating expenses	70,109
Loss from operations	(47,609
Interest and other income (loss), net	1,657
Change in fair value of redeemable convertible preferred stock call option liability	—
Net loss	(45,952
Other comprehensive (loss) income	(200
Comprehensive loss	(46,152
Cumulative dividend relating to redeemable convertible preferred stock	—
Accretion of redeemable convertible preferred stock to redemption value	—
Net loss attributable to common stockholders	$ (45,952
Net loss per share attributable to common stockholders, basic and diluted	$ (1.40
Weighted average number of shares used to compute net loss per share attributable to common stockholders, basic and diluted	32,832,514

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

Assets
Current assets
Cash and cash equivalents
Short-term investments
Receivable from collaboration partner
Prepaid expenses and other current assets
Total current assets
Property and equipment, net
Long-term investments
Other long-term assets
Total assets
Liabilities and stockholders' equity
Current liabilities
Accounts payable
Accrued liabilities
Prepayment from collaboration partner
Deferred revenue - current
Total current liabilities
Other long-term liabilities
Deferred revenue - noncurrent
Total liabilities
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 and 150,000,000 shares authorized at December 31, 2017 and 2016, respectively; 35,812,791 and 31,428,998 shares, issued and outstanding at December 31, 2017 and 2016, respectively
Additional paid-in capital
Accumulated other comprehensive (loss) income
Accumulated deficit
Total stockholders' equity
Total liabilities and stockholders' equity

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