MyoKardia Reports Second Quarter 2017 Financial Results and Operational Progress

Positive Topline Results in First Patient Cohort of Phase 2 PIONEER-HCM Study

Company to Host Conference Call and Webcast Today at 8:30 a.m. EDT (5:30 a.m. PDT)

SOUTH SAN FRANCISCO, Calif., Aug. 07, 2017 (GLOBE NEWSWIRE) -- MyoKardia, Inc. (Nasdaq:MYOK) ("MyoKardia" or the "Company"), a clinical stage biopharmaceutical company pioneering a precision medicine approach for the treatment of heritable cardiovascular diseases, today reported business highlights and financial results for the second quarter ended June 30, 2017.

In a separate release issued today, MyoKardia announced positive top-line data from the first patient cohort of PIONEER-HCM, the Phase 2 clinical study of mavacamten (formerly MYK-461) in patients with symptomatic, obstructive hypertrophic cardiomyopathy (oHCM). In this study's first patient cohort, statistically significant improvement was observed in the primary endpoint, change in post-exercise peak left ventricular outflow tract (LVOT) gradient from baseline to week 12, and in key secondary endpoints, including peak oxygen consumption (peak VO_2) and New York Heart Association class. MyoKardia also announced today that a second, low-dose patient cohort in PIONEER-HCM has completed enrollment. Based on these results and subject to discussions in the coming months with the U.S. Food and Drug Administration (FDA), MyoKardia is planning for its next study, EXPLORER-HCM, to be a pivotal study. EXPLORER-HCM is expected to initiate by the end of this year. The full topline data release can be found here.

"We are positioned for a successful second half of 2017 with several key clinical and regulatory developments expected on the heels of the positive topline data announced today from PIONEER-HCM," said Tassos Gianakakos, chief executive officer. "We have made great progress across our entire pipeline, both clinical-stage and research-stage programs, that strengthens our confidence in MyoKardia's unique approach to treat heritable cardiomyopathies. We look forward to sharing important details about these programs throughout the remainder of this year."

Development and Product Pipeline Highlights

Mavacamten for Hypertrophic Cardiomyopathy

- Positive topline data announced today from Phase 2 PIONEER-HCM study of mavacamten in symptomatic, obstructive hypertrophic cardiomyopathy patients
  - A statistically significant improvement was observed in the primary endpoint, change in post-exercise peak LVOT gradient from baseline to week 12 (p=0.002)
  - After 12 weeks of treatment, all 10 subjects (100%) achieved a reduction in post-exercise peak LVOT gradient from a baseline mean of 125 mmHg
  - In eight of the 10 subjects, the post-exercise peak LVOT gradient was reduced below the diagnostic threshold for oHCM (≤ 30 mmHg), with the other two patients' measurements below 50 mmHg
  - Clinically meaningful improvements (≤ 30 mmHg) in resting LVOT gradient were observed as early as week 2 in nine out of 10 subjects
  - Clinically and statistically significant improvements were observed in peak VO_2 (p=0.004)

    - Mavacamten was generally well-tolerated in this first cohort
- Results from the first patient cohort of the Phase 2 PIONEER-HCM study of mavacamten in symptomatic oHCM have been accepted for oral presentation at the Heart Failure Society of America's Annual Scientific Meeting in September 2017
- Enrollment complete in the second, low-dose patient cohort of PIONEER-HCM; topline data is expected to be released in the first quarter of 2018
- EXPLORER-HCM, the next stage and potentially pivotal study of mavacamten in patients with symptomatic oHCM, remains on track for initiation before the end of this year
- In the coming months, MyoKardia intends to discuss the mavacamten clinical development plan in an End-of-Phase 2 meeting with the FDA
- Phase 2 study evaluating mavacamten in patients with non-obstructive HCM (nHCM) is expected to commence in the fourth quarter of 2017

MYK-491 for Dilated Cardiomyopathy

- Topline data from the Phase 1 single ascending dose study in healthy volunteers of MYK-491, MyoKardia's product candidate for dilated cardiomyopathy (DCM), is expected to be announced in the third quarter of 2017
- A Phase 1 study of MYK-491 in DCM patients is expected to initiate before the end of this year

Second Quarter and Year-to-Date 2017 Financial Results

- **Cash Position:** Cash and cash equivalents as of June 30, 2017 were $117.3 million, compared to $135.8 million as of December 31, 2016. Investments (short-term and long-term) as of June 30, 2017 were $48.0 million, compared to $16.1 million as December 31, 2016.
- **Revenues:** Collaboration and license revenue was $5.6 million during the three months ended June 30, 2017, compared with $3.5 million during the same period in 2016. Collaboration and license revenue was $11.3 million for the first half of 2017, compared to $7.1 million for the same period in 2016.
- **R&D Expenses:** Research and development expenses for the three months ended June 30, 2017 were $13.7 million, up from $9.3 million for the three months ended June 30, 2016. Research and development expenses were $25.6 million for the first half of 2017, compared to $17.4 million for the same period in 2016. The increase in R&D expense was 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 cost for contract research, chemistry and biology expenses on discovery and pre-clinical programs.
- **G&A Expenses:** General and administrative expenses were $5.1 million for the three months ended June 30, 2017, compared to $4.1 million for the same period in 2016. General and administrative expenses were $10.6 million for the first half of 2017, compared to $7.9 million for the same period in 2016. The increase was primarily attributable to G&A staff expansion and office related costs, as well as other personnel related expenses.

- **Net Loss:** Net loss was $12.8 million for the second quarter of 2017, compared to a net loss of $9.8 million for the second quarter of 2016. Net loss was $24.4 million for the first half of 2017, compared to $18.2 million for the same period in 2016. The increase in net loss was primarily attributable to the increase in operating expenses noted above.

Based on its current operating plans, the Company expects that its cash, cash equivalents and investments as of June 30, 2017, together with anticipated payments from Sanofi under our collaboration agreement, will enable the Company to fund its anticipated operating expenses and capital expenditure requirements at least into 2019.

Conference Call and Webcast

MyoKardia will host a conference call and live audio webcast on Monday, August 7, 2017 at 8:30 a.m. EDT / 5:30 a.m. PDT. 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 62326435. 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 the two most prevalent forms of heritable cardiomyopathy—hypertrophic cardiomyopathy (HCM), and dilated cardiomyopathy (DCM). MyoKardia's most advanced product candidate is mavacamten (formerly MYK-461). Mavacamten is a novel, oral, allosteric modulator of cardiac myosin that reduced hypercontractility in Phase 1 clinical studies of HCM patients. In April 2016, the FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic oHCM, a subset of HCM. MyoKardia is currently studying mavacamten in PIONEER-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 1 study in healthy volunteers. 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 (formerly MYK-461) and MYK-491, the Company's expectations with respect to the timing of the release of topline data from the second patient cohort of PIONEER-HCM, the Company's ability to continue to advance mavacamten in the PIONEER-HCM study and MYK-491 in its Phase 1 study in healthy volunteers and its expectations with respect to the timing of the release of data from this study, the Company's ability to initiate its planned double-blind, placebo-controlled study of mavacamten (EXPLORER-HCM) in symptomatic oHCM and its expectations that it will be considered a pivotal study, the anticipated inclusion and exclusion criteria and number of patients expected to be enrolled in EXPLORER-HCM, the Company's plans to expand the clinical investigation of mavacamten to patients with non-obstructive HCM in a planned Phase 2 study, and the timing of the initiation of these studies, the Company's expectations with regard to its End-of-Phase 2 meeting with the FDA, as well as the requirements for registration of the Company's product candidates, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, which we expect to be filed on or about August 7, 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.

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

	June 30, 2017	**December 31, 2016**
Assets		
Current assets		
Cash and cash equivalents	$117,305	$ 135,797
Short-term investments	16,011	4,072
Receivable from collaboration partner	—	45,000
Prepaid expenses and other current assets	1,242	1,394
Total current assets	134,558	186,263
Property and equipment, net	2,856	2,758
Long-term investments	31,986	12,002

Other long-term assets	363	283
Total assets	$169,763	$ 201,306
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$1,362	$ 1,798
Accrued liabilities	10,153	8,690
Deferred revenue - current	22,500	22,500
Total current liabilities	34,015	32,988
Other long-term liabilities	303	436
Deferred revenue - noncurrent	11,250	22,500
Total liabilities	45,568	55,924
Commitments and contingencies (Note 6)		
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 June 30, 2017 and December 31, 2016, respectively; 31,455,894 and 31,428,998 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	3	3
Additional paid-in capital	226,471	223,208
Accumulated other comprehensive (loss) income	(50)	8
Accumulated deficit	(102,229)	(77,837)
Total stockholders' equity	124,195	145,382
Total liabilities and stockholders' equity	$169,763	$ 201,306

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,**	
	2017	**2016**	**2017**	**2016**
Collaboration and license revenue	$5,625	$3,549	$11,250	$7,099
Operating expenses:				
Research and development	13,689	9,279	25,606	17,409
General and administrative	5,082	4,056	10,558	7,916
Total operating expenses	18,771	13,335	36,164	25,325
Loss from operations	(13,146)	(9,786)	(24,914)	(18,226)
Interest and other income, net	309	26	530	46
Net loss	(12,837)	(9,760)	(24,384)	(18,180)
Other comprehensive loss	(3)	—	(58)	—

Comprehensive loss	(12,840)	(9,760)	(24,442)	(18,180)
Net loss attributable to common stockholders	$(12,837)	$(9,760)	$(24,384)	$(18,180)
Net loss per share attributable to common stockholders, basic and diluted	$(0.41)	$(0.37)	$(0.78)	$(0.69)
Weighted average number of shares used to compute net loss per share attributable to common stockholders, basic and diluted	31,200,773	26,337,184	31,151,216	26,284,630

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