MyoKardia Reports Progress on Clinical Programs and Third Quarter 2016 Financial Results

Dosing in Phase 2 PIONEER-HCM trial of MYK-461 in Symptomatic, Obstructive Hypertrophic Cardiomyopathy Under Way; Pathway to Registration Outlined

MYK-491 Dilated Cardiomyopathy Candidate Set to Enter Clinic in First Half of 2017

SOUTH SAN FRANCISCO, Calif., Nov. 07, 2016 (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 business highlights and financial results for the third quarter ended September 30, 2016.

"MyoKardia continues to make significant progress in our mission to help people with genetic cardiomyopathies," said Tassos Gianakakos, chief executive officer. "In this last quarter, we advanced our pipeline of therapeutic candidates, including MYK-461 and MYK-491, and in October, we completed an equity financing to further support our precision medicine platform and product engine. For MYK-461, our recently initiated Phase 2 PIONEER-HCM study will generate data across a broad range of clinically important measurements in oHCM which we expect to significantly de-risk subsequent clinical studies."

PIONEER-HCM will study relationships among important disease biomarkers such as contractility, left ventricular outflow tract (LVOT) gradient and potential registrable endpoints of functional capacity (i.e., peak VO2) and clinical symptoms. Topline data for PIONEER-HCM are expected in the second half of 2017.

"In addition to MYK-461 in oHCM, our MYK-491 program in dilated cardiomyopathy (DCM) is making great progress and is on track to initiate a Phase 1 study in healthy volunteers in the first half of 2017," said Mr. Gianakakos. "Like our MYK-461 candidate in HCM, MYK-491 is the first potential therapy designed to target the underlying biomechanical defect causing genetic DCM."

Other developments during the quarter include MyoKardia's inaugural Research and Development Day in which the Company outlined the anticipated pathway to registration for MYK-461 in oHCM, as well as further updates on its pipeline strategy and product engine. To advance its research and development pipeline and support its operations, the Company in October completed a follow-on public offering of common stock resulting in net proceeds of approximately $61 million.

Recent Highlights

Development and Product Pipeline Milestones

- MyoKardia's Phase 2 PIONEER-HCM study of MYK-461 in symptomatic oHCM patients has initiated dosing with topline data expected in the second half of 2017.

- A pathway to registration for MYK-461 in symptomatic oHCM has been outlined, with a potential single Phase 3 pivotal study. Improvement in functional capacity and/or clinical symptoms may be suitable endpoints for registration. No mortality-based efficacy endpoints are required for registration.

- In advance of an anticipated Phase 3 pivotal study of MYK-461 in symptomatic oHCM, the Company plans to initiate a Phase 2 trial of MYK-461 in symptomatic oHCM in the second half of 2017.

- MyoKardia plans to initiate a clinical trial of MYK-461 in non-obstructive HCM patients in the second half of 2017.

- MyoKardia intends to initiate a Phase 1 study of MYK-491 in healthy volunteers in the first half of 2017, with topline results expected in the third quarter of 2017.

Corporate

- In early October, MyoKardia secured approximately $61 million in financing in a public offering of 4,370,000 shares of common stock, after deducting underwriting discounts, commissions and estimated offering costs.

- An inaugural MyoKardia Research and Development Day program outlined the anticipated pathway to registration for MYK-461 in symptomatic oHCM, and provided further updates on the Company's pipeline strategy and product engine.

- Research on HCM based on data from the Sarcomeric Human Cardiomyopathy Registry (SHaRe) was presented at the American Society of Human Genetics Annual Meeting. The study, published in *Proceedings of the National Academy of Sciences of the United States of America*, focused on clinical, genetic and structural data to gain insight into HCM.

- MyoKardia continues its outreach to the genetic cardiomyopathy community. The Company has supported and participated in a series of informational sessions for HCM and DCM patients and families. The sessions are conducted by major cardiomyopathy treatment centers in partnership with leading clinicians and patient advocacy groups. To date, more than 400 cardiomyopathy patients and families have participated.

Third Quarter and Year-to-Date 2016 Financial Results

- **Cash Position:** Cash and cash equivalents as of September 30, 2016 were $77.1 million, compared to $112.3 million as of December 31, 2015. The $77.1 million as of September 30, 2016 does not reflect the additional $61

million, net of underwriting discounts, commissions, and estimated offering expenses, received in October from the public financing of 4,370,000 shares of common stock.

- **Revenues:** Collaboration and license revenue was $3.6 million during the three months ended September 30, 2016, unchanged from the $3.6 million during the three months ended September 30, 2015. Collaboration and license revenue was $10.6 million for the first nine months of 2016, unchanged from $10.6 million for the first nine months of 2015. Revenue recognized in both years relate to revenue recognized under the 2014 Sanofi Collaboration Agreement covering MyoKardia's three main research programs.
- **R&D Expenses:** Research and development expenses increased $2.1 million, from $6.7 million for the three months ended September 30, 2015 to $8.8 million for the three months ended September 30, 2016. Research and development expenses increased $6.2 million from $20.0 million for the nine months ended September 30, 2015 to $26.2 million for the nine months ended September 30, 2016. The increase in research and development expenses was primarily due to expansion of R&D staff to support discovery and pipeline development, pre-clinical expenses for MYK-491 and other research programs, and drug manufacturing costs for MYK-461 and MYK-491.
- **G&A Expenses:** General and administrative expenses increased $1.7 million from $2.3 million for the three months ended September 30, 2015 to $4.0 million for the three months ended September 30, 2016. General and administrative expenses increased $5.9 million from $6.0 million for the nine months ended September 30, 2015 to $11.9 million for the nine months ended September 30, 2016. The increase was attributable to continued expansion of G&A activities and staff in key areas required to support a public company infrastructure, as well as increased facilities and office expenses related to our new facility.
- **Net Loss:** Net loss was $9.2 million for the third quarter of 2016, compared to a net loss of $5.5 million for the third quarter of 2015. Year-to-date net loss was $-27.4 million, compared to $15.0 million for the comparable period in 2015. The increase in net loss was primarily attributable to the increase in operating expenses noted above.

MYOKARDIA, INC.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	**2015**	**2016**	**2015**
Collaboration and license revenue	$ 3,550	$ 3,550	$ 10,649	$ 10,649
Operating expenses				
Research and development	8,783	6,672	26,192	20,017
General and administrative	4,031	2,300	11,947	5,961

Total operating expenses	12,814	8,972	38,139	25,978
Loss from operations	(9,264)	(5,422)	(27,490)	(15,329)
Interest and other income, net	33	(40)	79	(22)
Change in fair value of redeemable convertible preferred stock call option liability	—	—	—	314
Net loss and comprehensive loss	(9,231)	(5,462)	(27,411)	(15,037)
Cumulative dividend relating to redeemable convertible preferred stock	—	(1,900)	—	(4,530)
Accretion of redeemable convertible preferred stock to redemption value	—	(26)	—	(88)
Net loss attributable to common stockholders	$ (9,231)	$ (7,388)	$ (27,411)	$ (19,655)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.35)	$ (2.78)	$ (1.04)	$ (7.98)
Weighted-average number of common shares used to compute net loss per share, basic and diluted	26,470,298	2,654,365	26,331,852	2,463,876

MYOKARDIA, INC.

Condensed Consolidated Balance Sheets

(In thousands, except share and per share amounts)

(Unaudited)

	September 30, 2016	December 31, 2015
Assets		
Current assets		
Cash and cash equivalents	$ 77,120	$ 112,265
Prepaid expenses and other current assets	762	1,282
Total current assets	77,882	113,547
Property and equipment, net	2,729	2,744
Other long term assets	750	289
Total assets	$ 81,361	$ 116,580
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 1,731	$ 2,143
Accrued liabilities	6,930	5,633
Deferred revenue	3,550	14,199
Total current liabilities	12,211	21,975
Other long-term liabilities	503	732
Total liabilities	12,714	22,707
Common stock	3	3
Additional paid-in-capital	160,740	158,555
Accumulated deficit	(92,096)	(64,685)
Total stockholders' equity	68,647	93,873
Total liabilities and stockholders' equity	$ 81,361	$ 116,580

About MYK-461 and PIONEER-HCM

MYK-461 is an orally administered small molecule designed to reduce left ventricular contractility by allosterically modulating the function of cardiac myosin, the motor protein that drives heart muscle contraction. MyoKardia has evaluated MYK-461 in three Phase 1 clinical trials, primarily designed to evaluate safety and tolerability of oral doses of MYK-461, as well as provide pharmacokinetic and pharmacodynamic data. In April 2016, the U.S. FDA granted Orphan Drug Designation for MYK-461 for the treatment of symptomatic oHCM, a subset of HCM.

MyoKardia is currently studying MYK-461 in PIONEER-HCM, a Phase 2 open-label single-arm study to evaluate safety, tolerability and efficacy of MYK-461 in patients with symptomatic oHCM. The primary endpoint of PIONEER-HCM is the level of reduction in post-exercise left ventricular outflow tract (LVOT) gradient over 12 weeks of drug treatment. PIONEER-HCM will also explore the relationship between reduction in contractility and LVOT gradient, endpoints measuring functional capacity (i.e., exercise) and clinical symptoms in addition to gathering safety and tolerability data on MYK-461 in an outpatient setting.

About HCM and oHCM

It is estimated that one in every 500 people in the United States has HCM, the most prevalent form of heritable cardiomyopathy. HCM is defined as an otherwise unexplained thickening of the walls of the heart, known as hypertrophy. The consequences include reduced left ventricular volumes and cardiac output, reduced ability of the left ventricle to expand, and elevated filling pressures. These can all contribute to reduced effort tolerance and symptoms that include shortness of breath and chest pain. HCM is a chronic disease and for the majority of patients, the disease progresses slowly and can be extremely disabling. HCM substantially increases the risk of developing atrial fibrillation that can lead to stroke or malignant ventricular arrhythmias that can cause sudden cardiac death. There are currently no approved drug products indicated for the treatment of HCM. Patients are typically prescribed one or more drugs (including beta blockers, non-dihydropyridine calcium channel blockers and disopyramide) indicated for the treatment of hypertension, heart failure or other cardiovascular disorders more generally.

oHCM is a physiological complication of HCM in which the thickened heart muscle obstructs the LVOT. Approximately two thirds of all HCM patients have obstruction, either at rest or with provocation like exercise. Measured most commonly by non-invasive imaging (echocardiography), oHCM is defined as ≥30 mm Hg pressure gradient across the LVOT. Symptoms of oHCM can include shortness of breath, chest pain, dizziness, fainting, and palpitations. The presence of obstruction in an HCM patient further increases risk of progression to severe symptoms, and risk of death from heart failure or stroke.

The degree of LVOT obstruction in oHCM patients is a primary criterion for surgical and

other invasive interventions (recommended for symptomatic patients with LVOT gradients measured at ≥50 mmHg). Relief of obstruction has been associated with improved symptoms, function and clinical outcomes. Surgical or other invasive interventions, including septal myectomy, an open heart procedure, may be appropriate. There are no approved drug products indicated for this condition. The primary endpoint of the Phase 2 PIONEER-HCM study is to assess level of reduction in LVOT gradient over 12 weeks of drug treatment. The trial is also exploring relationships among reductions in contractility, LVOT gradient and endpoints that include safety, tolerability, functional capacity and clinical symptoms.

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, or HCM, and dilated cardiomyopathy, or DCM. MyoKardia's most advanced product candidate, MYK-461, is an orally-administered small molecule designed to reduce excessive cardiac muscle contractility leading to HCM and has been evaluated in three Phase 1 clinical trials. MyoKardia is now studying MYK-461 in a Phase 2 PIONEER-HCM study in symptomatic oHCM, for which the FDA has granted MYK-461 Orphan Drug Designation. A cornerstone of the MyoKardia platform is the Sarcomeric Human Cardiomyopathy Registry, or 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 believes that SHaRe, currently consisting of data from approximately 10,000 individuals, is the world's largest registry of patients with heritable cardiomyopathies. MyoKardia's mission is to change the world for patients with serious cardiovascular disease through bold and innovative science. For more information, please visit www.myokardia.com.

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 MYK-461 and

MYK-491, the Company's ability to generate topline data from its Phase 2 PIONEER-HCM study, the Company's ability to advance MYK-491 into a Phase 1 clinical trial for DCM and generate topline data from this trial, the timing of these events, the anticipated clinical endpoints and pathway to approval for MYK-461 and the timing of the Company's initiation of additional Phase 2 clinical trials for MYK-461, 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 fiscal year ended December 31, 2015, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, our Registration Statement on Form S-1 (File No. 333-213680) filed with the Securities and Exchange Commission (SEC) on September 16, 2016, as amended, 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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