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): November 11, 2019

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.)**

333 Allerton Ave.
South San Francisco, CA 94080
(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	**MYOK**	**The 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 8.01 Other Events.

On November 11, 2019, MyoKardia, Inc. issued a press release titled "MyoKardia Announces Positive Topline Data from its Phase 2MAVERICK-HCM Clinical Trial of Mavacamten." In addition, on November 11, 2019, MyoKardia, Inc. issued a press release titled "MyoKardia Announces 48-week Data from PIONEER-OLE Study of Mavacamten." Copies of the press releases are attached as Exhibit 99.1 and Exhibit 99.2, respectively, to this Current Report on Form 8-K and are incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated November 11, 2019
99.2	Press Release, dated November 11, 2019
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: November 12, 2019

MyoKardia, Inc.

By: /s/ Cynthia Ladd
Cynthia Ladd
General Counsel

Exhibit 99.1

MyoKardia Announces Positive Topline Data from its Phase 2 MAVERICK-HCM Clinical Trial of Mavacamten

November 11, 2019

Achieved Primary Study Objective of Safety and Tolerability in Patients with Non-obstructive HCM

Significant Reductions in Biomarkers of Cardiac Stress Observed in Patients on Treatment vs. Placebo

Data Support Advancement of Mavacamten in Non-obstructive HCM; Regulatory Update Anticipated in the First Half of 2020

Phase 2 Study of Mavacamten Targeting Subgroup of Patients with Diastolic Heart Failure (HFpEF) Anticipated to Begin in the Second Quarter of 2020

Conference Call Today at 8:30 a.m. ET (5:30 a.m. PT)

SOUTH SAN FRANCISCO, Calif., Nov. 11, 2019 (GLOBE NEWSWIRE) — MyoKardia, Inc. (Nasdaq: MYOK), today announced topline data from MAVERICK-HCM, the company's Phase 2 clinical trial of mavacamten in patients with non-obstructive hypertrophic cardiomyopathy (HCM). The study achieved its primary objective of establishing safety and tolerability of mavacamten in non-obstructive HCM over a treatment period of 16 weeks. Meaningful reductions in biomarkers of cardiac stress were observed across both mavacamten drug concentration cohorts and clear signals of clinical benefit were noted in a subgroup with elevated cardiac filling pressures and in a pre-specified group of patients at higher risk for morbidity and mortality.

"We are encouraged by the evidence from MAVERICK of improved diastolic function in non-obstructive HCM patients with guideline-based measures of diastolic impairment, and by mavacamten's robust effect in reducing cardiac wall stress in patients across all our HCM studies," said Jay Edelberg, Senior Vice President of Clinical Development at MyoKardia. "Consistent with our precision medicine approach, the results of MAVERICK have provided us with important insights, including how to identify groups of patients with diseases of diastolic dysfunction and how to best measure clinical benefit. These data will inform enrollment criteria, dosing, duration and potential endpoints for our planned future clinical trials of mavacamten in non-obstructive HCM and targeted HFpEF patients."

Based on the safety and pharmacologic benefits observed in MAVERICK, MyoKardia plans to advance mavacamten into additional studies in defined groups of patients with non-obstructive HCM and heart failure with preserved ejection fraction (HFpEF). For the non-obstructive indication, the company will seek to consult with the U.S. Food and Drug Administration (FDA) on potential pathways to registration and expects to provide a regulatory update in the first half of 2020. For the targeted HFpEF population, MyoKardia plans to initiate a Phase 2 study in the second quarter of 2020 in a subgroup of patients sharing many characteristics with the subgroups identified in MAVERICK. Further analysis of the findings from MAVERICK is ongoing, and these data will be submitted for presentation at an upcoming scientific conference.

"Of all our prior attempts to address the needs of our patients with non-obstructive HCM, for whom there are no effective medical therapies, the MAVERICK-HCM trial provides us with the most encouraging data to date," said Stephen Heitner, M.D., Director of the Hypertrophic Cardiomyopathy Clinic at Oregon Health & Science University and a principal investigator for the MAVERICK-HCM trial. "The data reported today are especially promising in that they provide a glimpse into how we may better phenotype this group of patients, and may begin to understand the unpredictability of symptoms. I am hopeful that this study will prove to be the springboard for the thoughtful development of the next phase of studies aimed at bringing mavacamten, a unique and precise therapy, to our non-obstructive HCM patients and potentially other similar individuals suffering from HFpEF."

Heart failure with preserved ejection fraction is a heterogeneous clinical syndrome, which in many patients is characterized by impairment of the left ventricle's ability to relax and fill during diastole, resulting in insufficient blood flow to meet the body's needs. HFpEF is estimated to affect approximately three million people in the U.S. and is associated with significant morbidity and mortality. There are currently no approved therapies for HFpEF. The subgroup identified for future evaluation of mavacamten is estimated to include approximately 10-20 percent of the broader HFpEF population.

Phase 2 MAVERICK-HCM Trial - Topline Results

Mavacamten was well tolerated and the observed safety data were consistent with prior studies. The rate of adverse events (AEs) was greater in the mavacamten groups than the placebo group. The majority of AEs reported were mild or moderate in severity and reversible or self-resolving. Serious adverse events (SAEs) occurred twice as frequently in the placebo arm as compared to patients receiving mavacamten. Transient ejection fraction reductions below the protocol-defined threshold of 45% occurred in five participants in the active drug arms.

For the intent-to-treat population, there were no statistically significant differences at 16 weeks between active and placebo groups in exploratory endpoints, with the exception of levels of the biomarker NT-proBNP, which were markedly reduced in patients receiving mavacamten (p=0.004) across both treatment cohorts, as compared to the placebo group. NT-proBNP is a well-established biomarker of cardiac wall stress, and elevated NT-proBNP levels are associated with reductions in the rate of heart failure-related death or hospitalization, progression to end-stage disease and stroke.

In a pre-specified subgroup representing patients believed to be at higher risk of morbidity and mortality, meaningful trends suggesting clinical benefit were observed for patients on treatment versus placebo across multiple endpoints of symptoms, function, biomarkers of cardiac stress and diastolic compliance.

Additionally, similar trends were observed in a subgroup of patients with elevated cardiac filling pressures (measured by E/e'), suggesting improvement driven by reduced left ventricular pressure, consistent with mavacamten's targeted mechanism.

"The topline data reported today are important in the advancement of mavacamten across multiple indications. The safety and tolerability data, evidence of mavacamten's beneficial impact on parameters of diastolic function, and placebo response observations confirm our assumptions and increase our confidence in the EXPLORER-HCM Phase 3 clinical study of mavacamten in obstructive HCM," said Tassos Gianakakos, Chief Executive Officer of MyoKardia.

About MAVERICK-HCM

The Phase 2 MAVERICK-HCM trial was designed to assess the safety and tolerability of a range of exposures over 16 weeks of treatment in patients with symptomatic, non-obstructive HCM. All study participants were required to be diagnosed withnon-obstructive HCM, with left ventricular wall thickness either >15mm or >13mm with a family history of HCM, New York Heart Association (NYHA) classifications of Class II or III, and NT-proBNP levels of greater than 300 pg/mL at rest. Baseline characteristics, such as age, weight, gender, pathogenic mutation status, background beta blocker use, NYHA classification and exercise capacity were evenly distributed between active and placebo arms.

A total of 59 participants were enrolled in the study and randomized into one of three groups to receive once-daily doses of mavacamten or placebo. The active mavacamten treatment arms were designed to assess a range of drug concentrations around target levels of 200ng/mL and 500ng/mL. All participants in the active treatment arms began the study receiving 5mg doses of mavacamten. At Week 4, pharmacokinetic (PK) assessments were conducted and doses were adjusted in a blinded fashion per the protocol based on the participant's assigned cohort. Following the 16-week treatment period, participants were monitored for an additional eight weeks and became eligible to participate in MyoKardia's MAVA Long-Term Extension (LTE) study.

Conference Call and Webcast

MyoKardia management will host a conference call and live audio webcast this morning at 8:30 a.m. ET / 5:30 a.m. PT to review the topline data reported today from the MAVERICK Phase 2 clinical trial and new data from the PIONEER-OLE study. Investors and analysts are invited to participate in the call by dialing 844-494-0913 (U.S.) or 508-637-5584 using the conference ID 3177984. The webcast may be accessed live on the Investor Relations section of the MyoKardia website. A replay of the webcast will be available on MyoKardia's website for 90 days following the call.

About Non-obstructive HCM

Hypertrophic cardiomyopathy is the most common genetic form of heart disease, affecting an estimated one in every 500 people worldwide. There are two main forms of HCM, obstructive HCM and non-obstructive HCM, which often share the same underlying genetic defects in the sarcomere that results in hypercontractility. In non-obstructive HCM, the heart contracts excessively and the left ventricle becomes abnormally thick, restricting the ability of the heart to relax and fill or pump to meet the body's needs, but no physical obstruction is present in the outflow tract of the left ventricle. Non-obstructive HCM affects an estimated one-third of all HCM patients and presents unique treatment challenges. Patients may progress to a more advanced state of disease than those with obstructive disease before being diagnosed, and there are no approved treatment options available. As non-obstructive HCM progresses, symptoms begin to resemble those of a congestive heart failure patient and heart transplantation may become the only viable treatment option.

About Mavacamten (MYK-461)

Mavacamten is a novel, oral, allosteric inhibitor of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). Mavacamten is intended to reduce cardiac muscle contractility by inhibiting the excessive myosin-actin cross-bridge formation that underlies the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. MyoKardia is currently evaluating mavacamten in multiple clinical trials for the treatment of obstructive and non-obstructive HCM. The pivotal Phase 3 clinical trial, known asEXPLORER-HCM, is being conducted in patients with symptomatic, obstructive HCM and MyoKardia anticipates data from this program in Q2'2020. Two long-term follow-up studies are also ongoing, the PIONEER open-label extension study of obstructive HCM patients from MyoKardia's Phase 2 PIONEER trial and the MAVA-LTE, an extension study for patients who have completed either EXPLORER-HCM or MAVERICK-HCM, the company's Phase 2 clinical trial of symptomaticnon-obstructive HCM patients. In April 2016, the U.S. FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic obstructive HCM.

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 muscle 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) in Phase 3 and Phase 2 clinical trials for hypertrophic cardiomyopathy (HCM); MYK-491 in Phase 2 for patients with stable heart failure; andMYK-224 in Phase 1 development for HCM.

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, our plans to consult with the FDA on potential pathways to registration and to provide a regulatory update, the initiation, progress and availability of data from our ongoing and planned clinical trials, and the timing of these events, 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 September 30, 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

Exhibit 99.2

MyoKardia Announces 48-week Data from PIONEER-OLE Study of Mavacamten

November 11, 2019

Mavacamten's Safety and Efficacy Profile in the PIONEER Study Maintained Through One Year in Open-Label Extension Study of 12 Patients with Symptomatic, Obstructive HCM

Evidence Suggests Mavacamten's Favorable Impact on Cardiac Structure

Conference Call Today at 8:30 a.m. ET (5:30 a.m. PT);
Data Presentation at American Heart Association Scientific Sessions on Monday, November 18, 2019

SOUTH SAN FRANCISCO, Calif., Nov. 11, 2019 (GLOBE NEWSWIRE) — MyoKardia, Inc. (Nasdaq: MYOK) today announced new data from the company's PIONEER open-label extension (OLE) study of mavacamten for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (HCM).

Data for twelve patients at 48 weeks of treatment with mavacamten were consistent with prior safety and efficacy observations at the 12, 24, and 36-week readouts. Highlights of the data include continued safety and tolerability and sustained clinical benefits, 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.

"Mavacamten's continued favorable safety profile and the apparent consistency and durability across so many parameters through one year of treatment are incredibly encouraging and enhance our confidence that mavacamten has the potential to make a lasting impact on the lives of people with HCM. We look forward to building on these data with the results from our pivotal EXPLORER-HCM Phase 3 study in the first half of next year," said Jay Edelberg, M.D., Ph.D., Senior Vice President of Clinical Development at MyoKardia.

"The hearts of people with HCM beat as if they are in fight-or-flight mode all the time, and as disease progresses, that excessive contraction can result in a cascade of damaging, and ultimately fatal, consequences for HCM patients. Mavacamten acts at a molecular level on specific heart muscle proteins to reduce the excessive contractility driving disease," said Daniel Jacoby, M.D. Director, Comprehensive Heart Failure Program at the Yale School of Medicine and an investigator in the PIONEER-OLE clinical trial. "Over time, and across multiple measures, we are seeing evidence that mavacamten treatment is bringing the HCM heart toward a more normal state. The new data being reported during the AHA sessions, including the improvement of patient-reported outcomes and heart structure, add further weight to the sustained safety and efficacy observed to date in this study population."

PIONEER-OLE 48-week Results

Twelve patients with symptomatic (NYHA Class II-III), obstructive HCM are currently enrolled in the PIONEER-OLE study and receive individualized doses of mavacamten aimed at reducing or eliminating their LVOT obstruction. All twelve patients were evaluable at 48 weeks.

- Mavacamten was well tolerated throughout the one-year treatment period. Consistent with data reported at Week 36, there were no cardiac-related adverse events (AEs) attributed to study drug throughout the 48-week period. To date, all AEs attributed to treatment have been mild or moderate and transient.
- LVOT gradient, a measure of obstruction of the left ventricle, was decreased from baseline with statistical significance among the twelve patients under multiple conditions of testing: i.e. at rest, post-exercise and upon provocation with a Valsalva maneuver. At week 48, resting LVOT gradient for all patients was below 50mmHg, the guideline-based threshold for an invasive intervention, and 11 of 12 patients were below the 30mmHg threshold at which obstructive HCM is diagnosed. Provoked gradient measurements, taken using a Valsalva maneuver and post-exercise, were also below 50mmHg in all but two patients at Week 48.
- Left ventricular ejection fraction (LVEF) remained above normal (50%) for all 12 patients at all times of assessment.

PIONEER-OLE: Observed Mean Values (SD)
LVOT Gradient and LVEF

	Baseline (N=13)	Week 12 (N=13)	Week 24 (N=13)	Week 36 (N=12)	Week 48 (N=12)
Resting LVOT gradient (mmHg)	67.3 (42.80)	11.8 (5.20)	11.6 (6.01)	21.1 (20.89)	14.0 (9.70)
Valsalva LVOT gradient (mmHg)	89.9 (30.72)	22.5 (19.60)	20.5 (10.59)	31.5 (23.25)	22.4 (18.96)
Post-exercise gradient (mmHg)	127.5 (33.38)	—	—	—	39.8 (31.13)†
Resting LVEF (%)	72.0 (4.90)	67.5 (6.93)	69.0 (6.11)	69.4 (6.04)	70.6 (9.10)

† *n=11*

Note: Post-exercise gradient was collected at Baseline and Week 48.

- NT-proBNP, an established circulating blood marker of cardiac wall stress, significantly decreased to ranges closer to normal (considered less than 125 pg/mL). NT-proBNP levels in HCM patients of <310 pg/mL have been associated with a 75 percent reduction in the rate of heart failure-related death or hospitalization, progression to end-stage disease, and stroke, as compared with patients with levels >310 pg/mL.(1)
- E/e', an echocardiographic measure of left ventricular filling pressure, decreased from a mean baseline measure of 12.8 to 9.1.
- Left atrial volume index decreased to normal levels from a baseline mean of 41 mL/m^2 to a mean of 32 mL/m^2. Left atrial volumes are a measure of the filling pressure of the left ventricle, and increased volumes are potentially associated with an increased risk of atrial fibrillation in HCM patients.(2)
- Reductions in interventricular septal (IVS) thickness as measured by echocardiography were observed inPIONEER-OLE patients. Overall, PIONEER-OLE patients began the study with a mean IVS of 17mm at baseline, and progressively decreased to 15mm after 48 weeks of mavacamten treatment. Studies of HCM patients following septal reduction interventions have shown that IVS reductions in HCM patients are associated with improvements in LVOT gradient, functional capacity and symptoms. The risk of sudden cardiac death in HCM patients has been observed to increase progressively as wall thickness increases above 15mm.(3)

PIONEER-OLE: Biomarker Measurements, Mean (SD)
Cardiac Wall Stress, Diastolic Filling Pressure and Structural Changes

	Normal ranges	Baseline (N=13)	Week 12 (N=13)	Week 24 (N=13)	Week 36 (N=12)	Week 48 (N=12)	Change from Baseline to Week 48
NT-proBNP (pg/mL), median (IQR)	<125	594	99	93	168	137	-472(-2467, -157)**
E/e' lateral	<13	12.8 (2.9)	9.8 (2.5)	10.2 (2.7)	8.5 (2.3)	9.1 (2.0)†	-3.4 (3.0)**
LA volume index (mL/m^2)	16-34	40.9 (16.4)	31.8 (8.4)	30.8 (8.0)	30.4 (8.7)	31.5 (6.9)	-9.8 (13.5)*
IVS (mm)	6-10mm	16.7 (2.8)	16.0 (2.7)	15.8 (2.7)	15.4 (2.7)	15.3 (2.2)	-1.5 (2.6)

† *n=11*
** *p<0.01*
* *p<0.05*

Improvements in both symptom burden and quality of life has been observed among thePIONEER-OLE patients.

- At baseline, patients enrolled in PIONEER-OLE were symptomatic with a NYHA classification of Class II or III. NYHA classifications were measured at Week 24 and Week 48 and have consistently demonstrated improvements, with nine out of twelve patients achieving asymptomatic (Class I) status.
- Positive results from the KCCQ, designed to measure patients' perception of their heart failure health status and its impact on the activities of daily living, were also reported. In PIONEER-OLE, KCCQ mean scores went from 74.1 at baseline to 87.3 at Week 48 (scores range from 0-100, and higher scores reflect better status). A clinically significant change in KCCQ is defined as greater than or equal to 6.

These data will be presented at the 2019 American Heart Association's Annual Scientific Sessions by Stephen Heitner, M.D., Director of the Hypertrophic Cardiomyopathy Clinic at Oregon Health and Science University, on Monday, November 18, 2019 at 2:05 p.m. during the session titled: *Pharmacological Therapy in HF/Cardiomyopathy: The Next Important Indication or Agent?*

Conference Call and Webcast

MyoKardia management will host a conference call and live audio webcast this morning at 8:30 a.m. ET / 5:30 a.m. PT to review new data from the PIONEER-OLE study and the topline data reported today from the MAVERICK Phase 2 clinical trial. Investors and analysts are invited to participate in the call by dialing 844-494-0913 (U.S.) or 508-637-5584 using the conference ID 3177984. The webcast may be accessed live on the investor section of the MyoKardia website. A replay of the webcast will be available on MyoKardia's website for 90 days following the call.

About HCM

Hypertrophic cardiomyopathy (HCM) is a chronic, progressive disease in which excessive contraction of the heart muscle and reduced ability of the left ventricle to fill can lead to the development of debilitating symptoms and cardiac dysfunction. HCM is estimated to affect one in every 500 people.

The most frequent cause of HCM is mutations in the heart muscle proteins of the sarcomere. In approximatelytwo-thirds of HCM patients, the path followed by blood exiting the heart, known as the left ventricular outflow tract (LVOT), becomes obstructed by the enlarged and diseased muscle, restricting the flow of blood from the heart to the rest of the body (obstructive HCM). In other patients, the thickened heart muscle does not block the LVOT, and their disease is driven by diastolic impairment due to the enlarged and stiffened heart muscle (non-obstructive HCM). In either obstructive or non-obstructive HCM patients, exertion can result in fatigue or shortness of breath, interfering with a patient's ability to participate in activities of daily living. HCM has also been associated with increased risks of atrial fibrillation, stroke, heart failure and sudden cardiac death.

About Mavacamten (MYK-461)

Mavacamten is a novel, oral, allosteric modulator of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). Mavacamten is intended to reduce cardiac muscle contractility by inhibiting the excessive myosin-actin cross-bridge formation that underlies the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. MyoKardia is currently evaluating mavacamten in multiple clinical trials for the treatment of obstructive and non-obstructive HCM. The pivotal Phase 3 clinical trial, known asEXPLORER-HCM, is being conducted in patients with symptomatic, obstructive HCM and MyoKardia anticipates data from this program in Q2'2020. Two long-term follow-up studies are also ongoing, the PIONEER open-label extension study of obstructive HCM patients from MyoKardia's Phase 2 PIONEER trial and the MAVA-LTE, an extension study for patients who have completed either EXPLORER-HCM or MAVERICK-HCM, the company's Phase 2 clinical trial of symptomatic non-obstructive HCM patients. In April 2016, the U.S. FDA granted Orphan Drug Designation for mavacamten for the treatment of symptomatic obstructive HCM.

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MyoKardia's mission is to change the world for people with serious cardiovascular disease through bold and innovative science.

(1) Amato, et al, *American Journal of Cardiology*, 2013
(2) Debonnaire, et al, *Circulation: Arrhythmia and Electrophysiology,* 2017
(3) Spirito P, et al, *New England Journal of Medicine*, 2000

Forward-looking Statement

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 the availability of data from EXPLORER-HCM, the Company's expectation with respect to release of data fromEXPLORER-HCM, as well as the Company's expectations for the potential for success of EXPLORER-HCM, 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 September 30, 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.

Contacts

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