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): August 7, 2017

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))

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 August 7, 2017, MyoKardia, Inc. (the "Company") announced positive topline data from the first patient cohort of its Phase 2 PIONEER-HCM study of mavacamten in symptomatic, obstructive hypertrophic cardiomyopathy ("oHCM") patients. This cohort met the primary endpoint of change in post-exercise peak left ventricular outflow tract ("LVOT") gradient from baseline to week 12 as well as key secondary endpoints, including peak oxygen consumption (peak VO_2). Based on these results and subject to discussions in the coming months with the U.S. Food and Drug Administration ("FDA"), the Company 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.

In this first patient cohort of PIONEER-HCM, 11 patients enrolled and 10 completed the study. 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, providing the rationale for the addition of a second, low-dose cohort to the PIONEER study. Additionally, clinically and statistically significant improvements were observed in peak VO_2 (p=0.004).

The following table summarizes the results observed in post-exercise peak LVOT gradient and peak VO_2:

	Baseline, mean (SD) n=11	**Week 12,** mean (SD) n=10	**Change from Baseline to Week 12,** mean (SD) n=10	***p*-value**
Post-Exercise Peak LVOT Gradient, mmHg	125 (60.0)	19 (12.9)	-112 (63.8)	0.002
Peak VO_2, mL/kg/min	20.7 (7.44)	24.6 (8.78)	+3.5 (3.25)	0.004

With respect to New York Heart Association Functional Classification, an exploratory endpoint of PIONEER-HCM, improvements from baseline were observed at week 12 (p=0.016), by at least one class in seven patients, with two of these patients improving by two classes.

Mavacamten was generally well-tolerated. One patient with a history of paroxysmal atrial fibrillation experienced a serious adverse event. In order to participate in the study, this patient had discontinued background beta blocker and disopyramide therapy, both of which are indicated for the management of atrial fibrillation. During the study, the patient experienced a recurrent episode of atrial fibrillation and was cardioverted. The patient had another episode of atrial fibrillation and was hospitalized and successfully treated with anti-arrhythmic therapy. The patient elected to stop study drug at week 4. All other adverse events ("AEs") were mild to moderate, and a majority of the AEs were deemed to be unrelated to study drug.

After reviewing safety data from PIONEER-HCM, the Independent Data Monitoring Committee recommended continuation of the study.

In the coming months, the Company intends to discuss the mavacamten clinical development plan in an End-of-Phase 2 meeting with the FDA and seek feedback on the potential for EXPLORER-HCM, its next study of mavacamten in symptomatic oHCM, to be a pivotal study with peak VO_2 as the primary endpoint. The key inclusion and exclusion criteria for EXPLORER-HCM are anticipated to be similar to those for PIONEER-HCM, and the Company expects to enroll between 200 and 250 patients in EXPLORER-HCM. The Company expects to initiate EXPLORER-HCM before the end of this year.

The Company also announced in August 2017 that the second, low-dose patient cohort in PIONEER-HCM has completed enrollment. Given the marked improvement observed in patients in the first cohort within the first two weeks of dosing, the Company added this patient cohort to explore lower daily doses of mavacamten. This second cohort did not require discontinuation of beta blocker therapy prior to enrollment. The Company expects to release topline data from this second patient cohort in the first quarter of 2018.

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: August 7, 2017

MyoKardia, Inc.

By: /s/ Jacob Bauer
Jacob Bauer
Senior Vice President, Finance and Corporate Development
(principal financial officer)