# 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): March 13, 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))

**Item 2.02 Results of Operations and Financial Condition.**

On March 13, 2017, MyoKardia, Inc. announced its financial results for the three and twelve months ended December 31, 2016. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in Item 2.02 of this Form 8-K (including Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits.

| Exhibit No. | Description |
|---|---|
| 99.1 | Press Release issued by MyoKardia, Inc. on March 13, 2017, furnished herewith |

**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: March 13, 2017

**MyoKardia, Inc.**

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

**EXHIBIT INDEX**

| Exhibit No. | Description |
| --- | --- |
| 99.1 | Press Release issued by MyoKardia, Inc. on March 13, 2017, furnished herewith |

**Exhibit 99.1**

**MyoKardia Reports Fourth Quarter and Full Year 2016 Financial Results and Operational Progress**

***Topline Data from Phase 2 PIONEER-HCM Trial of MYK-461 in Symptomatic Obstructive Hypertrophic Cardiomyopathy Expected in Third Quarter of 2017***

***MYK-491 Dilated Cardiomyopathy Candidate Phase 1 Study of Healthy Volunteers Under Way; Topline Data Expected in Third Quarter of 2017***

***Sanofi Payment Received for Global Cardiomyopathy Research Collaboration***

***Strong Cash Position to Further Advance Programs***

SOUTH SAN FRANCISCO, Calif., March 13, 2017 – 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 fourth quarter and year ended December 31, 2016.

"The year 2016 saw MyoKardia deepen our leadership, expand our scientific platform and advance our growing clinical pipeline," said Tassos Gianakakos, chief executive officer. "We enter 2017 with two programs in the clinic aimed at the two most common heritable cardiomyopathies, HCM and DCM, and sufficient cash to fund operations into 2019. Both programs have data reporting out in the third-quarter of this year that we hope will bring us one step closer to helping HCM and DCM patients."

During 2016, the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for MYK-461 for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (oHCM). Topline data from the PIONEER-HCM trial of MYK-461, and the Company's Phase 1 study of dilated cardiomyopathy (DCM) candidate MYK-491, are expected in the third quarter of 2017.

"We are proud of the progress MyoKardia made in 2016 in reaching clinical milestones and broadening our pipeline of novel therapies that target the underlying biomechanical defects of the heart muscle," said Marc Semigran, M.D., chief medical officer. "As these programs advance in the clinic, we continue to execute on our mission to change the world for patients with serious cardiovascular disease through bold and innovative science."

Upcoming Clinical Milestones

- Topline data from the Phase 2 PIONEER trial of MYK-461 in symptomatic oHCM are expected in the third quarter of 2017.
- Topline results from the Phase 1 single ascending dose (SAD) study of DCM candidate MYK-491, currently being studied in healthy volunteers, are expected in the third quarter of 2017.
- A double-blind, placebo-controlled Phase 2 trial of MYK-461 (named EXPLORER-HCM) in symptomatic oHCM, based on PIONEER data, is planned for the second half of 2017.
- The Company plans to expand study of MYK-461 to patients with non-obstructive HCM in a Phase 2 trial planned to start in the second half of 2017.

***Development and Product Pipeline***

*HCM Program*

- MyoKardia's PIONEER study of MYK-461 in symptomatic oHCM patients continues, with topline data expected in the third quarter of 2017. Based on FDA feedback received in a Type C meeting held during 2016, the Company has outlined a path to registration for MYK-461, potentially involving a single Phase 3 pivotal study. The Company confirms that mortality-based efficacy endpoints will not be required for registration, and that improvement in functional capacity and/or clinical symptoms may be suitable endpoints for registration.

- PIONEER is providing additional safety data in oHCM patients in a 12-week, outpatient setting. The trial is also studying relationships among 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. Reduction of the LVOT gradient has been shown in published studies to improve symptoms, increase functional capacity and reduce the incidence of heart failure and stroke related death in oHCM patients.

- The year 2016 saw the completion of three phase 1 programs, studying 86 healthy volunteers and 15 HCM patients, including two with a history of obstruction. The studies together demonstrated the ability of MYK-461 to reduce the excessive cardiac contractility associated with HCM in a dose dependent fashion within a well tolerated dose range.

- The Company plans to initiate the Phase 2 EXPLORER trial of MYK-461 in symptomatic oHCM, based on PIONEER data, in the second half of 2017. Additionally, MyoKardia plans to initiate a clinical trial of MYK-461 in non-obstructive HCM patients in the second half of 2017.

- MyoKardia announced an exploratory digital health substudy in PIONEER to investigate the potential for a non-invasive investigational wristband to provide information on blood flow abnormalities in oHCM patients.

*DCM Program*

- MyoKardia initiated a Phase 1 SAD study of MYK-491 in healthy volunteers in January 2017, with topline results expected in the third quarter of 2017. MYK-491 may hold potential for controlled increases in the heart's contractility with minimal impact on diastole or relaxation.

***Leadership and Business Momentum***

- In December 2016, Sanofi elected to continue the global cardiomyopathy research collaboration formed with MyoKardia in August 2014. In connection with this decision, MyoKardia received an associated $45 million payment.

- Also in the fourth quarter of 2016, MyoKardia received an additional $25 million milestone payment from Sanofi for the filing of an Investigational New Drug application with the FDA for the MYK-491 program in DCM.

- MyoKardia secured net proceeds of 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.

- The Company appointed two additional independent directors to the Board of Directors: Audit Committee chair Mary Cranston, and Sunil Agarwal, M.D.

- The Company named Marc Semigran, M.D., as the Company's chief medical officer.

- In early 2017, MyoKardia appointed June Lee, M.D., as chief operating officer, and Radhika Tripuraneni, M.D., as vice president, medical affairs.

***Research***

- Data demonstrating the ability of MYK-461 to prevent and reverse development of disease in multiple genetic mouse models of HCM were published in *Science*.

- Presentations at American Heart Association Scientific Sessions 2016 highlighted the Company's clinical and animal model research in HCM, as well as novel insights from its Sarcomeric Human Cardiomyopathy Registry (SHaRe) and precision cardiovascular approach.

- SHaRe data, which revealed that certain variants of myosin protein can lead to earlier onset of HCM, were published in *Proceedings of the National Academy of Sciences of the United States of America*.

***Cardiomyopathy Community and Patient Outreach***

MyoKardia continued 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. More than 400 cardiomyopathy patients and families have participated.

**Fourth Quarter and 2016 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities as of December 31, 2016 were $139.9 million, compared to $112.3 million as of December 31, 2015, an increase of $27.6 million. Based on its current operating plans, the Company expects that its existing cash, cash equivalents and marketable securities as of December 31, 2016, together with anticipated interest income, and 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.

- **Revenues:** Collaboration and license revenue was $28.6 million for the fourth quarter of 2016, compared to $3.6 million for the fourth quarter of 2015. Collaboration and license revenue was $39.2 million for 2016 compared to $14.2 million for 2015, an increase of $25 million. The increase from year to year was the result of ongoing revenue from the Company's collaboration contract with Sanofi S.A., signed in 2014, covering three of MyoKardia's programs.

- **R&D Expenses:** Research and development expenses were $10.0 million for the fourth quarter of 2016, compared to $8.4 million for the fourth quarter of 2015, an increase of $1.6 million. R&D expenses were $36.2 million for 2016, compared to $28.4 million for 2015, an increase of $7.8 million. The year-over-year growth in R&D expense was primarily driven by our ongoing clinical trials, including initiation of our Phase 2 PIONEER clinical trial for MYK-461, the expansion of R&D staff, and increased manufacturing activities for all of MyoKardia's ongoing clinical stage programs.

- **G&A Expenses:** General and administrative expenses were $4.3 million for the fourth quarter of 2016, compared to $3.1 million for the year-earlier period, an increase of $1.2 million. G&A expenses were $16.3 million for 2016, compared to $9.0 million for 2015, an increase of $7.3 million. The increase was primarily attributable to G&A staff expansion and professional fees required to support a public company infrastructure, as well as other personnel related expenses.

- **Net Income/Loss:** Net income was $14.3 million for the fourth quarter of 2016, compared to a net loss of $7.9 million for the fourth quarter of 2015, an increase of $22.2 million. Net loss was $13.2 million for 2016, compared to a net loss of $22.9 million for 2015, a decrease of $9.7 million.

**About MYK-461**

MYK-461 is an oral 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 MYK-491**

The oral small molecule MYK-491 is an allosteric modulator of myosin designed to increase cardiac contractility in a DCM heart. Like MYK-461, the Company's candidate for hypertrophic cardiomyopathy, MYK-491 targets the underlying biomechanical defects of the heart muscle. Based on preclinical research across multiple animal models, MYK-491 may hold potential for controlled increases in the heart's contractility with minimal impact on diastole or relaxation. MyoKardia initiated a Phase 1 study of MYK-491 in healthy volunteers in early 2017, with topline results expected in the third quarter of 2017.

**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 oral small molecule designed to reduce excessive cardiac muscle contractility leading to HCM and has been evaluated in three Phase 1 clinical trials. MyoKardia is currently studying MYK-461 in the Phase 2 PIONEER-HCM trial in symptomatic, obstructive HCM (oHCM), a subset of HCM for which the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation. MYK-491, the second clinical candidate generated by MyoKardia's product engine, is designed to increase the overall force 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; topline data is expected in the third quarter of 2017. 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 advance MYK-461 in the PIONEER-HCM trial and MYK-491 in its Phase 1 trial in healthy volunteers and to generate data from these trials, the Company's ability to initiate additional clinical trials of MYK-461 in symptomatic oHCM and non-obstructive HCM and the timing of these events, as well as the requirements for registration of the Company's product candidates, the Company's ability to successfully maintain its collaboration with Sanofi and the Company's anticipated expenditures and cash runway, 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, 2016, 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 Income (Loss)**
(In thousands, except share and per share amounts)
(Unaudited)

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2016 | 2015 | 2016 | 2015 |
| Collaboration and license revenue | $ 28,550 | $ 3,550 | $ 39,199 | $ 14,199 |
| Operating expenses: | | | | |
| Research and development | 10,023 | 8,376 | 36,215 | 28,393 |
| General and administrative | 4,342 | 3,058 | 16,289 | 9,019 |
| Total operating expenses | 14,365 | 11,434 | 52,504 | 37,412 |
| Loss from operations | 14,185 | (7,884) | (13,305) | (23,213) |
| Interest and other income, net | 74 | (25) | 153 | (47) |
| Change in fair value of redeemable convertible preferred stock call option liability | — | — | — | 314 |
| Net loss | 14,259 | (7,909) | (13,152) | (22,946) |
| Other comprehensive income | 8 | — | 8 | — |
| Comprehensive loss | 14,267 | (7,909) | (13,144) | (22,946) |
| Cumulative dividend relating to redeemable convertible preferred stock | — | (621) | — | (5,151) |
| Accretion of redeemable convertible preferred stock to redemption value | — | (10) | — | (98) |
| Net loss attributable to common stockholders | $ 14,259 | $ (8,540) | $ (13,152) | $ (28,195) |
| Net loss per share attributable to common stockholders, basic | $ 0.46 | $ (0.48) | $ (0.48) | $ (4.48) |
| Weighted average number of shares used to compute net loss per share attributable to common stockholders, basic | 30,878,973 | 17,702,564 | 27,475,792 | 6,292,800 |
| Net loss per share attributable to common stockholders, diluted | $ 0.44 | $ (0.48) | $ (0.48) | $ (4.48) |
| Weighted average number of shares used to compute net loss per share attributable to common stockholders, diluted | 32,228,172 | 17,702,564 | 27,475,792 | 6,292,800 |

**MYOKARDIA, INC.**

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

| | As of December 31, | |
|---|---|---|
| | **2016** | **2015** |
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 135,797 | $ 112,265 |
| Short-term investments | 4,072 | — |
| Receivable from collaboration partner | 45,000 | — |
| Prepaid expenses and other current assets | 1,394 | 1,282 |
| Total current assets | 186,263 | 113,547 |
| Property and equipment, net | 2,758 | 2,744 |
| Long-term investments | 12,002 | — |
| Other long term assets | 283 | 289 |
| Total assets | $ 201,306 | $ 116,580 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities | | |
| Accounts payable | $ 1,798 | $ 2,143 |
| Accrued liabilities | 8,690 | 5,633 |
| Deferred revenue - current | 22,500 | 14,199 |
| Total current liabilities | 32,988 | 21,975 |
| Other long-term liabilities | 436 | 732 |
| Deferred revenue - noncurrent | 22,500 | — |
| Total liabilities | 55,924 | 22,707 |
| 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 December 31, 2016 and 2015, respectively; 31,428,998 and 27,053,156 shares, issued and outstanding at December 31, 2016 and 2015, respectively | 3 | 3 |
| Additional paid-in capital | 223,208 | 158,555 |
| Accumulated other comprehensive income | 8 | — |
| Accumulated deficit | (77,837) | (64,685) |
| Total stockholders' equity | 145,382 | 93,873 |
| Total liabilities and stockholders' equity | $ 201,306 | $ 116,580 |

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