UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2017

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission File Number: 001-37609

MYOKARDIA, INC.
(Exact name of registrant as specified in its charter)

Delaware	**44-5500552**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

333 Allerton Ave.
South San Francisco, CA
(Address of principal executive offices)

94080
(Zip Code)

(650) 741-0900
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☒
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐
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. ☒

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The number of outstanding shares of the registrant's common stock on October 31, 2017 was 35,741,036 shares.

MYOKARDIA, INC.

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PART I—FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements

MYOKARDIA, INC.

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

	September 30, 2017	December 31, 2016
Assets		
Current assets		
Cash and cash equivalents	$ 227,184	$ 135,797
Short-term investments	19,993	4,072
Receivable from collaboration partner	—	45,000
Prepaid expenses and other current assets	1,545	1,394
Total current assets	248,722	186,263
Property and equipment, net	2,633	2,758
Long-term investments	36,003	12,002
Other long-term assets	432	283
Total assets	$ 287,790	$ 201,306
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 2,030	$ 1,798
Accrued liabilities	10,634	8,690
Deferred revenue - current	22,500	22,500
Total current liabilities	35,164	32,988
Other long-term liabilities	246	436
Deferred revenue - noncurrent	5,625	22,500
Total liabilities	41,035	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 September 30, 2017 and December 31, 2016, respectively; 35,733,002 and 31,428,998 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	4	3
Additional paid-in capital	363,205	223,208
Accumulated other comprehensive (loss) income	(52)	8
Accumulated deficit	(116,402)	(77,837)
Total stockholders' equity	246,755	145,382
Total liabilities and stockholders' equity	$ 287,790	$ 201,306

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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,	
	2017	2016	2017	2016
Collaboration and license revenue	$ 5,625	$ 3,550	$ 16,875	$ 10,649
Operating expenses:				
Research and development	14,361	8,783	39,967	26,192
General and administrative	5,884	4,031	16,442	11,947
Total operating expenses	20,245	12,814	56,409	38,139
Loss from operations	(14,620)	(9,264)	(39,534)	(27,490)
Interest and other income, net	447	33	977	79
Net loss	(14,173)	(9,231)	(38,557)	(27,411)
Other comprehensive loss	2	—	60	—
Comprehensive loss	(14,171)	(9,231)	(38,497)	(27,411)
Net loss attributable to common stockholders	$ (14,173)	$ (9,231)	$ (38,557)	$ (27,411)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.42)	$ (0.35)	$ (1.21)	$ (1.04)
Weighted average number of shares used to compute net loss per share attributable to common stockholders, basic and diluted	33,525,567	26,470,298	31,951,631	26,331,852

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

MYOKARDIA, INC.

Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2017	**2016**
Cash flows from operating activities:		
Net loss	$ (38,557)	$ (27,411)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	4,347	1,781
Depreciation	977	815
Accretion of discounts and amortization of premiums on investments	62	—
Change in operating assets and liabilities:		
Receivable from collaboration partner	45,000	—
Prepaid expenses and other current assets	(151)	520
Other long-term assets	(149)	—
Accounts payable	228	(372)
Accrued liabilities	2,029	1,027
Other long-term liabilities	(118)	(75)
Deferred revenue	(16,875)	(10,649)
Net cash used in operating activities	(3,207)	(34,364)
Cash flows from investing activities:		
Purchases of investments	(44,044)	—
Sale of investments	4,000	—
Purchases of property and equipment	(917)	(854)
Net cash used in investing activities	(40,961)	(854)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of offering costs	133,925	—
Proceeds from exercise of stock options and employee stock purchase plan	1,668	226
Payments of prior period offering costs	(38)	(153)
Net cash provided by financing activities	135,555	73
Net increase (decrease) in cash and cash equivalents	91,387	(35,145)
Cash and cash equivalents, beginning of period	135,797	112,265
Cash and cash equivalents, end of period	$ 227,184	$ 77,120
Supplemental disclosures of noncash investing and financing activities		
Unpaid offering costs included in period-end accounts payable and accrued liabilities	$ 63	$ 461
Vesting of early exercised options and restricted stock	$ 156	$ 226
Unpaid portion of property and equipment purchases included in period-end accounts payable and accrued liabilities	$ 38	$ 7

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

1. Formation and Business of the Company

MyoKardia, Inc. (the "Company") is a clinical stage biopharmaceutical company pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and neglected rare cardiovascular diseases. Our 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. We have used our precision medicine platform to generate a robust pipeline of therapeutic programs for the chronic treatment of the two most common forms of heritable cardiomyopathy—hypertrophic cardiomyopathy ("HCM"), and dilated cardiomyopathy ("DCM").

The Company has completed enrollment in a Phase 2 clinical trial of mavacamten (formerly known as MYK-461), our product candidate for the treatment of HCM, and is currently enrolling subjects in a Phase 1 clinical trial of MYK-491, our product candidate for the treatment of DCM. Using our precision medicine development strategy, we believe we have efficiently generated clinical proof of mechanism for mavacamten in both healthy volunteers and in HCM patients, and we intend to pursue a similar path for MYK-491. In 2016, mavacamten was granted Orphan Drug Designation by the U.S. Food and Drug Administration ("FDA"), for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy ("oHCM"), a subset of HCM.

Through September 30, 2017, the Company has financed its operations through an initial public offering ("IPO"), two follow-on public offerings, private placements of redeemable convertible preferred stock and funds received in connection with a license and collaboration agreement with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A., entered into in August 2014 (the "Collaboration Agreement") (See Note 4). The Company received net proceeds of $93.9 million from the sale of shares of its Series A, A-1 and B redeemable convertible preferred stock. On November 3, 2015, the Company completed its IPO of 6,253,125 shares of common stock at an offering price of $10.00 per share, resulting in net proceeds of approximately $55.6 million, after deducting underwriting discounts, commissions and offering costs. On October 3, 2016, the Company completed a follow-on public offering of 4,370,000 shares of common stock at an offering price of $15.00 per share, resulting in net proceeds of approximately $61.1 million, after deducting underwriting discounts, commissions and estimated offering costs. On August 14, 2017, the Company completed another follow-on public offering of 4,025,000 shares of common stock at an offering price of $35.50 per share, resulting in net proceeds of approximately $133.8 million, after deducting underwriting discounts, commissions and estimated offering costs. In connection with the Collaboration Agreement, the Company has received $105.0 million from Sanofi S.A., consisting of a $35.0 million upfront payment, a $25.0 million milestone payment for the submission of an investigational new drug application ("IND") for MYK-491 with the FDA in November 2016, and a $45.0 million continuation payment from Sanofi in January 2017. As of September 30, 2017, the Company had an accumulated deficit of $116.4 million and cash and cash equivalents of $227.2 million, short-term investments of $20.0 million and long-term investments of $36.0 million.

The accompanying unaudited Condensed Consolidated Financial Statements, in the opinion of management, include all adjustments which the Company considers necessary for the fair statement of the Condensed Consolidated Results of Operations and Comprehensive Loss and Cash Flows for the interim periods covered and the Condensed Consolidated Financial Position of the Company at the date of the balance sheets. The consolidated financial statements of the Company as at December 31, 2016 included the Company's accounts and have been prepared in conformity with accounting principles generally accepted in the United States of America ("US GAAP"). The interim results presented herein are not necessarily indicative of the results of operations that may be expected for the full fiscal year ending December 31, 2017, or any other future period.

The accompanying unaudited Condensed Consolidated Financial Statements and related financial information should be read in conjunction with the Company's audited consolidated financial statements and the related notes thereto for the year ended December 31, 2016 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 filed with the U.S. Securities and Exchange Commission (the "SEC") on March 13, 2017 (the "Annual Report").

2. Summary of Significant Accounting Policies

Significant accounting policies are described in Note 2 to the consolidated financial statements for the year ended December 31, 2016 included in the Annual Report. There have been no changes to the Company's significant accounting policies during the nine months ended September 30, 2017, except as described below.

Adopted Accounting Pronouncements

Beginning in fiscal year 2017, the Company adopted *Accounting Standard Update ("ASU") No. 2016-09, Improvements to employee share-based payment accounting,* which simplifies the accounting for employee share-based transactions. The amendments change, among other things, the recognition of excess tax benefits and deficiencies, the classification of those excess tax benefits on the statement of cash flows, an accounting policy election for forfeitures, the amount an employer can withhold to cover income taxes and still qualify for equity classification, and the classification of those taxes paid on the statement of cash flows. The Company adopted ASU 2016-09 in the first quarter of 2017. As a result of adopting this standard, we have made an accounting policy election to account for forfeitures as they occur. This change has been applied on a modified retrospective basis, resulting in an immaterial cumulative effect adjustment to the opening accumulated deficit on January 1, 2017. Upon adoption, the previously unrecognized excess tax benefits were recorded as a deferred tax asset, which was fully offset by a valuation allowance resulting in no impact to the accumulated deficit.

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB"), or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the impact of recently issued standards that are not yet effective will not have a material impact on the Company's consolidated financial statements upon adoption. Under the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"), the Company meets the definition of an emerging growth company, and has irrevocably elected to opt out of the extended transition period for complying with new or revised accounting standards pursuant to Section 107(b) of the JOBS Act.

In May 2017, the FASB issued *ASU 2017-09—Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting.* The amendments in this ASU provide guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718 to address diversity in practice. An entity should account for the effects of a modification unless all the three specified conditions are met. The current disclosure requirements in Topic 718 apply regardless of whether an entity is required to apply modification accounting under the amendments in this ASU. The amendments in this ASU are effective for all entities for annual periods, and interim periods within those annual periods, beginning after December 15, 2017, with early adoption permitted. The amendments in this ASU should be applied prospectively to an award modified on or after the adoption date. The Company has not determined the potential effects of this ASU on its consolidated financial statements.

In November 2016, the FASB issued *ASU No. 2016-18 (Topic 230), Restricted Cash, Statement of Cash Flows*. This ASU 2016-18 requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents, and amounts generally described as restricted cash or restricted cash equivalents. Therefore, amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU 2016-18 is effective for public business entities for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years, with early adoption permitted. The amendments in this ASU should be applied using a retrospective transition method to each period presented. The adoption of this standard is not expected to have a material impact on the Company's consolidated financial statements.

In February 2016, the FASB issued *ASU No. 2016-02 (Topic 842), Leases.* This ASU requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company has not determined the potential effects of this ASU on its consolidated financial statements.

In May 2014, the FASB issued *ASU No. 2014-09 (Topic 606), Revenue from Contracts with Customers*, which supersedes the revenue recognition requirements in *ASC 605, Revenue Recognition.* This ASU is based on the principle that revenue is recognized to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. In March, April, and May 2016, the FASB issued amended guidance including clarifying guidance on principal versus agent considerations, identification of performance obligations, collectability and noncash considerations. ASU 2014-09 and its amendments are effective for public entities for annual and interim periods beginning after December 15, 2017; therefore, the Company will adopt the new revenue standards in the first quarter of 2018. Since its formation, the Company has only had transactions which are recognized in revenue which relate to its Collaboration Agreement with Sanofi S.A. The Company is evaluating whether or not the identification of performance obligations and determination of their stand-alone values would remain unchanged under the new revenue standards. The Company plans to adopt the standard in the first quarter of fiscal year 2018 by applying the full retrospective method. The Company's ability to adopt using the full retrospective method is dependent on the

completion of its analysis of information necessary to restate prior period financial statements. The Company is continuing to evaluate the accounting, transition and disclosure requirements of the standard and is in process of assessing the financial statement impact of adoption.

3. Fair Value Measurements

Financial assets and liabilities are recorded at fair value. The accounting guidance for fair value provides a framework for measuring fair value, clarifies the definition of fair value and expands disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability (an exit price) in an orderly transaction between market participants at the reporting date. The accounting guidance establishes a three-tiered hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2—Inputs other than quoted market prices included in Level 1 are either directly or indirectly observable for the asset or liability through correlation with market data at the measurement date and for the duration of the instrument's anticipated life.

Level 3—Inputs reflect management's best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

The following table sets forth the Company's financial instruments that were measured at fair value on a recurring basis by level within the fair value hierarchy (in thousands):

	Fair Value Measurements at September 30, 2017			
	Total	Level 1	Level 2	Level 3
Assets				
Money market funds	$ 227,466	$ 227,466	$ —	$ —
U.S. government agency obligations	31,963	—	31,963	—
Corporate securities	24,033	—	24,033	—
Total	$ 283,462	$ 227,466	$ 55,996	$ —

	Fair Value Measurements at December 31, 2016			
	Total	Level 1	Level 2	Level 3
Assets				
Money market funds	$ 136,481	$ 136,481	$ —	$ —
U.S. government agency obligations	12,075	—	12,075	—
Corporate securities	3,999	—	3,999	—
Total	$ 152,555	$ 136,481	$ 16,074	$ —

The following table is a summary of amortized cost, unrealized gain and loss, and fair value (in thousands) of the Company's marketable securities by contractual maturities:

	Fair Value Measurements at September 30, 2017			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash equivalents (due within 90 days)	$ 227,466	$ —	$ —	$ 227,466
Short-term investments (due within one year)	20,009	—	(16)	$ 19,993
Long-term investments (due between one and two years)	36,033	3	(33)	$ 36,003
Total	$ 283,508	$ 3	$ (49)	$ 283,462

	Fair Value Measurements at December 31, 2016			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
Cash equivalents (due within 90 days)	$ 136,481	$ —	$ —	$ 136,481
Short-term investments (due within one year)	4,072	—	—	4,072
Long-term investments (due between one and two years)	11,988	14	—	12,002
Total	$ 152,541	$ 14	$ —	$ 152,555

4. Collaboration and License Agreement

Sanofi (Aventis Inc.)

In August 2014, the Company entered into the Collaboration Agreement with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A., for the research, development and potential commercialization of pharmaceutical products for the treatment, prevention and diagnosis of hypertrophic and dilated cardiomyopathy, as well as potential additional indications.

Pursuant to the Collaboration Agreement, in addition to potential future royalty payments, Sanofi agreed to provide up to $200.0 million in financial consideration to the Company consisting of the following components:

1. a $35.0 million upfront cash payment
2. a $10.0 million initial equity investment
3. a $25.0 million milestone-based contingent payment
4. up to an $85.0 million project continuation payment if Sanofi elects to extend the term of the research collaboration beyond December 31, 2016, as described below
5. up to $45.0 million in funding from Sanofi of approved in-kind research and clinical activities over a four-year period.

The Company is also entitled to receive tiered royalties beginning in the mid-single digits to the mid-teens on net sales of certain hypertrophic cardiomyopathy ("HCM") and dilated cardiomyopathy ("DCM") finished products outside the United States and on net sales of certain DCM finished products in the United States. Sanofi is eligible to receive tiered royalties beginning in the mid-single digits to the low teens on the Company's net sales of certain HCM finished products in the United States. In addition, under the terms of the Collaboration Agreement, Sanofi may reimburse the Company for a portion of the registration program costs for mavacamten. These registration costs will be reimbursed under the Registration Program Plan approved in October 2017.

The Collaboration Agreement covers three main research programs, "HCM1" (or HCM-1 or mavacamten, formerly known as MYK-461), "HCM2" (or HCM-2) and "DCM1" (or DCM-1 or MYK-491). The Company is solely responsible for conducting research and development activities through early human efficacy studies, except for specified research activities to be conducted by Sanofi. The estimated completion of proof-of-concept phases are staggered, depending on the program. Thereafter, the Company will lead worldwide development and United States commercial activities for the mavacamten and HCM-2 programs, Sanofi will lead global development and commercial activities for DCM-1 and Sanofi will lead ex-United States development and commercial activities for the mavacamten and HCM-2 programs where it has ex-United States commercialization rights. Sanofi also has the option to co-promote in the U.S. for potential expanded cardiovascular diseases outside of the genetically targeted indications for the mavacamten and HCM-2 programs, with the Company having the option to co-promote the DCM-1 program in the United States.

The Company accounted for the Collaboration Agreement by evaluating each of the financial components discussed above:

1. **$35.0 million upfront payment.** The Company received a non-refundable upfront payment and identified the following performance obligations at the inception of the Collaboration Agreement: (i) the transfer of intellectual property rights and know-how (license), (ii) the obligation to provide certain limited research and development services during the term of the license agreement and (iii) the obligation to participate on the development and commercialization committees. The Company applied the guidance under ASC 605-25, *Multiple Element Arrangements,* to account for this upfront payment. The Company evaluated the underlying goods and services delivered under the Collaboration Agreement and concluded that the performance obligations do not have standalone value, and accordingly accounted for the deliverables as one unit of accounting. The $35.0 million payment was recorded by the Company as deferred revenue on its consolidated balance sheet upon receipt, which the Company was recognizing as revenue on a straight-line basis over the expected term of research and development services through December 31, 2016 because there was not a more discernible pattern of performance in which the research and development services occurred. During the three months ended September 30, 2017 and 2016, the Company recognized zero and $3.6 million, respectively, and during the nine months ended September 30, 2017 and 2016, the Company recognized zero and $10.6 million of revenue, respectively, related to the $35.0 million upfront payment under the Collaboration Agreement. As of September 30, 2017 and December 31, 2016, the Company did not have any deferred revenue on its consolidated balance sheet related to this upfront payment.

2. **$10.0 million upfront investment in Series A-1 redeemable convertible preferred stock.** In August 2014, the Company entered into a Series A-1 redeemable convertible preferred stock purchase agreement with Sanofi. The Agreement was signed as a separate transaction from the Collaboration Agreement. Pursuant to the stock purchase agreement, the Company sold 6,666,667 shares of Series A-1 redeemable convertible preferred stock to Sanofi at $1.50 per share. The Company concluded that the $1.50 per share price represented the fair value of the redeemable convertible preferred stock issued. As of September 30, 2017, Sanofi owned 11.2% of the Company's common stock.

3. **$25.0 million milestone-based payment.** The Company was eligible to receive a one-time, non-refundable, non-creditable payment of $25.0 million upon the submission of an investigational new drug application for any DCM-1 development candidate to the FDA or a comparable regulatory authority in Europe or another major market country for any DCM-1 product. The Company accounted for this milestone payment separately from the rest of the agreement. The Company has determined that the milestone was substantive as it was achieved based upon the Company's past performance. The Company achieved this milestone in October 2016 and as a result, recognized the $25.0 million milestone payment from Sanofi as revenue during the year ended December 31, 2016.

4. **Up to $85.0 million continuation payments.** Under the Collaboration Agreement, Sanofi needed to determine by December 31, 2016 whether or not to continue the Collaboration Agreement. Under the terms of the Collaboration Agreement, if Sanofi so elected to continue the Collaboration Agreement, it would be obligated to pay:

   - a one-time, non-refundable, non-creditable cash payment of $45.0 million; and
   - an additional $40.0 million reduced by $5.0 million in connection with the purchase of the Company's preferred stock, assuming the Company has not previously closed (i) either a Qualified IPO (at which time this obligation will terminate) or a private financing prior to a Qualified IPO and (ii) Sanofi has not previously purchased shares of the Company's stock pursuant to such rights to purchase the Company's capital stock in accordance with the terms of the Collaboration Agreement. The $40.0 million payment was reduced by $5.0 million to $35.0 million in connection with Sanofi's subsequent purchase of shares of the Company's Series B redeemable convertible preferred stock in April 2015, and the remaining obligation terminated in connection with the Company's IPO in October 2015.

   Sanofi elected to continue the Collaboration Agreement in December 2016. The Company recorded a receivable and deferred revenue as of December 31, 2016 for the $45.0 million continuation payment, upon receipt of the election to continue, which the Company is recognizing on a straight-line basis over the expected term of research and development services through December 31, 2018 because there is no more discernable pattern of performance for which the R&D services occur. The payment was subsequently received in January 2017. In relation to this continuation payment, the Company recognized $5.6 million and $16.9 million as revenue during the three and nine months ended September 30, 2017, respectively, and had deferred revenue on its consolidated balance sheet of $28.1 million as of September 30, 2017.

   Sanofi also had a time-restricted right to purchase $40.0 million in shares of the Company's redeemable convertible preferred stock at the discounted price, which would have satisfied the $40.0 million obligation to purchase shares of the Company's capital stock in connection with the continuation decision. Sanofi's option to purchase $40.0 million of additional shares of the Company's redeemable convertible preferred stock at the discounted price expired upon the closing of the Series B redeemable convertible preferred stock financing in April 2015.

   The Company had determined that Sanofi's right to purchase the redeemable convertible preferred stock at the discounted price, and the Company's corresponding obligation to issue this additional redeemable convertible preferred stock, represented a freestanding financial instrument. The freestanding convertible preferred stock call option liability was initially recorded at its fair value of $0.7 million in 2014. The Company did not have a liability related to the redeemable convertible preferred stock call option on its consolidated balance sheet as of September 30, 2017 and December 31, 2016.

5. **Up to $45.0 million in-kind research and collaboration activities.** Sanofi can fund up to $45.0 million of pre-approved funding of research and collaboration activities. Since Sanofi will pay its vendors and personnel directly as per the Collaboration Agreement, the Company will not receive cash from Sanofi and therefore will not account for the funding of the in-kind services.

5. Balance Sheet Components

Property and Equipment

Property and equipment consist of the following (in thousands):

	September 30, 2017	December 31, 2016
Scientific equipment	$ 5,531	$ 4,858
Furniture and equipment	682	546
Capitalized software	280	237
Leasehold improvements	308	308
Total	6,801	5,949
Less: Accumulated depreciation	(4,168)	(3,191)
Property and equipment, net	$ 2,633	$ 2,758

Depreciation expense was $0.4 million and $0.3 million, for the three months ended September 30, 2017 and 2016, respectively, and $1.0 million and $0.8 million, for the nine months ended September 30, 2017 and 2016, respectively.

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	September 30, 2017	December 31, 2016
Clinical research and development	$ 5,318	$ 3,981
Payroll and related expenses	3,919	3,717
Other	1,397	992
Total accrued liabilities	$ 10,634	$ 8,690

6. Commitments and Contingencies

Purchase Commitments

The Company conducts product research and development programs through a combination of internal and collaborative programs that include, among others, arrangements with universities, contract research organizations and clinical research sites. The Company has contractual arrangements with these organizations; however, these contracts are generally cancelable on 30 days' notice and the obligations under these contracts are largely based on services performed.

Facility Leases

On June 29, 2012, the Company entered into a 66-month lease for approximately 12,000 square feet of office and laboratory space in South San Francisco with annual payments of approximately $0.5 million. In connection with this lease agreement, the Company also entered into a shared facilities and services agreement with Global Blood Therapeutics, Inc. ("GBT"), a co-tenant in the office building. In October 2014, the Company entered into a lease assignment agreement with the owner of the building and GBT to allow GBT to sublease the Company's portion of the building beginning in March 2015. For the three and nine months ended September 30, 2017, the Company recorded approximately $0.1 million and $0.3 million, respectively, of sublease income and $0.1 million and $0.3 million, respectively, of sublease expense, which is recorded in interest and other income, net in the consolidated statements of operations and comprehensive loss. For the three and nine months ended September 30, 2016, the Company recorded approximately $0.1 million and $0.3 million, respectively, of sublease income and $0.1 million and $0.3 million, respectively, of sublease expense, which is recorded in interest and other income, net in the consolidated statements of operations and comprehensive loss.

On September 15, 2014, the Company entered into a five-year lease for approximately 34,400 square feet of office and laboratory space in South San Francisco. The Company may extend the lease for an additional three year term. The initial annual lease payments are $1.3 million, increasing to $1.6 million in the final year of the agreement. The lease period commenced in January 2015. The Company received a lease abatement for the first three months of the lease term, which is recorded as deferred rent and recognized over the lease term.

The Company has provided deposits for letters of credit totaling $0.3 million to secure its obligations under its leases, which have been classified as long-term assets on the Company's consolidated balance sheet as of September 30, 2017.

Rent expense, net, was $0.3 million and $1.0 million, for each of the three and nine months ended September 30, 2017 and 2016, respectively.

Contingencies

From time to time, the Company may have contingent liabilities that arise in the ordinary course of business activities. The Company accrues for such a liability when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. There were no contingent liabilities requiring accrual or disclosure as of September 30, 2017, or December 31, 2016.

Guarantees and Indemnifications

The Company enters into standard indemnification arrangements in the ordinary course of business.

Pursuant to certain of these arrangements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified parties for losses suffered or incurred by the indemnified party, in connection with any trade secret, copyright, patent or other intellectual property infringement claim by any third-party with respect to the Company's technology. The term of these indemnification arrangements is generally perpetual. The maximum potential amount of future payments the Company could be required to make under these agreements is not determinable because it involves claims that may be made against the Company in the future, but have not yet been made.

The Company indemnifies its officers and directors for certain events or occurrences, subject to certain limits, while the officer or director is or was serving at the Company's request in such capacity, as permitted under Delaware law and in accordance with its certificate of incorporation and bylaws, and agreements providing for indemnification entered into with its officers and directors. The term of the indemnification period lasts as long as an officer or director may be subject to any proceeding arising out of acts or omissions of such officer or director in such capacity.

The maximum amount of potential future indemnification of directors and officers is unlimited; however, the Company currently holds director and officer liability insurance. This insurance allows the transfer of risk associated with its exposure and may enable it to recover a portion of any future amounts paid.

The Company believes that the fair value of these indemnification obligations is minimal. Accordingly, the Company has not recognized any liabilities relating to these obligations for any period presented.

7. Stockholders' Equity

Common Stock Reserved for Issuance

The Company has reserved shares of common stock for issuance as follows:

	September 30, 2017	December 31, 2016
Options issued and outstanding	3,289,677	2,141,868
Shares available for issuance under 2015 Stock Option and Incentive Plan	587,696	720,921
Shares available for issuance under 2015 Employee Stock Purchase Plan	479,947	202,087
Total	4,357,320	3,064,876

8. Stock-Based Compensation

The Company classifies stock-based compensation expense in the accompanying condensed consolidated statements of operations and comprehensive loss based on the department to which a recipient belongs. The following table sets forth stock-based compensation expense related to options granted to employees and consultants for all periods presented (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Research and development	$ 713	$ 269	$ 1,913	$ 690
General and administrative	888	494	2,434	1,091
Total	$ 1,601	$ 763	$ 4,347	$ 1,781

The following summarizes option activity under the 2012 Equity Incentive Plan and 2015 Stock Option and Incentive Plan:

	Shares Subject to Outstanding Options	Weighted Average Exercise Price Per Share
Balance at December 31, 2016	2,141,868	$ 6.42
Options granted	1,685,575	14.69
Options exercised	(282,978)	4.46
Options canceled	(254,788)	9.13
Balance at September 30, 2017	3,289,677	$ 10.62

In relation to stock options to purchase common stock that vest upon the achievement of performance criteria, the Company recorded zero and $174,000 in stock-based compensation expense for the three and nine months ended September 30, 2017, respectively, and $74,000 and $147,000 for the three and nine months ended September 30, 2016, respectively. The Company begins to recognize expenses related to these options during the period upon concluding that certain performance criteria are considered probable.

9. Net Loss per Share Attributable to Common Stockholders

The following table sets forth the computation of basic and diluted net loss per share attributable to common stockholders (in thousands, except share and per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Numerator				
Net loss	$ (14,173)	$ (9,231)	$ (38,557)	$ (27,411)
Net loss attributable to common stockholders, basic and diluted	$ (14,173)	$ (9,231)	$ (38,557)	$ (27,411)
Denominator				
Weighted average shares outstanding	33,686,799	27,023,650	32,195,471	27,023,951
Less: weighted average shares subject to repurchase	(161,232)	(553,352)	(243,840)	(692,099)
Weighted average shares used to compute basic and diluted net loss per share	33,525,567	26,470,298	31,951,631	26,331,852
Net loss per share attributable to common stockholders, basic and diluted	$ (0.42)	$ (0.35)	$ (1.21)	$ (1.04)

The following outstanding shares of potentially dilutive securities were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been antidilutive:

	As of September 30,	
	2017	2016
Common stock subject to repurchase	127,524	517,219
Stock options to purchase common stock	3,289,677	2,034,777

As of September 30, 2017, the Company has contributions from plan participants of $297,000 under the 2015 ESPP, which if converted, would be equivalent to 25,668 shares based on 85% of the stock price at the beginning of the offering period.

As of September 30, 2016, the Company had contributions from plan participants of $235,060 under the 2015 ESPP, which if converted, would be equivalent to 24,148 shares based on 85% of the stock price at the beginning of the offering period.

10. Related Party Transactions

In September 2012, the Company began receiving consulting and management services pursuant to an unwritten agreement with Third Rock Ventures, which is one of the Company's largest shareholders. Kevin Starr, a director of the Company, is a partner of Third Rock Ventures. Charles Homcy, a former director who resigned from the Board of Directors in March 2017, is a venture partner of Third Rock Ventures. The consulting fees paid to Third Rock Ventures were incurred by the Company in the ordinary course of business, and were $12,000 and $45,000 for the three and nine months ended September 30, 2017, respectively, and $7,000 and $31,000 for the three and nine months ended September 30, 2016, respectively. As of September 30, 2017 and December 31, 2016, the Company had outstanding obligations to Third Rock Ventures of $13,000 and $9,000, respectively.

11. Subsequent Events

The Company currently leases approximately 34,400 square feet of laboratory and office space in South San Francisco, California under a lease that expires on January 19, 2020. On October 1, 2017, the Company entered into an additional 25-month sublease agreement for approximately 8,000 square feet of office space in South San Francisco with annual payments of approximately $0.3 million. The lease period commenced on October 1, 2017.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operation

You should read the following management's discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed consolidated financial statements and notes thereto included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited consolidated financial statements and notes thereto for the year ended December 31, 2016, included in our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the U.S. Securities and Exchange Commission (SEC) on March 13, 2017 (the "Annual Report").

Special note regarding forward-looking statements

This report contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed in the forward-looking statements. The statements contained in this report that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the " Exchange Act"). Forward-looking statements are often identified by the use of words such as, but not limited to, "anticipate," "believe," "can," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "seek," "should," "strategy," "target," "will," "would" and similar expressions or variations intended to identify forward-looking statements. These statements are based on the beliefs and assumptions of our management based on information currently available to management. Such forward-looking statements are subject to risks, uncertainties and other important factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section titled "Risk Factors" included under Part II, Item 1A below. Furthermore, such forward-looking statements speak only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Overview

We are a clinical stage biopharmaceutical company pioneering a precision medicine approach to discover, develop and commercialize targeted therapies for the treatment of serious and neglected rare cardiovascular diseases. Our 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. We have used our precision medicine platform to generate a robust pipeline of therapeutic programs for the chronic treatment of the two most common forms of heritable cardiomyopathy—hypertrophic cardiomyopathy, or HCM, and dilated cardiomyopathy, or DCM.

We have completed enrollment in a Phase 2 clinical trial of mavacamten (formerly known as MYK-461), our product candidate for the treatment of HCM, and are currently enrolling subjects in a Phase 1 clinical trial of MYK-491, our product candidate for the treatment of DCM. Using our precision medicine development strategy, we believe we have efficiently generated clinical proof of mechanism for mavacamten in both healthy volunteers and in HCM patients, and we intend to pursue a similar path for MYK-491. In 2016, mavacamten was granted Orphan Drug Designation by the U.S. Food and Drug Administration, or the FDA, for the treatment of symptomatic, obstructive hypertrophic cardiomyopathy (oHCM), a subset of HCM.

Financial Overview

We have not generated net income from operations, and, as of September 30, 2017, we had an accumulated deficit of $116.4 million, primarily as a result of research and development and general and administrative expenses.

To date, all of our revenue has been derived from non-refundable payments under the license and collaboration agreement we entered into with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A., in August 2014, which we refer to as the Collaboration Agreement, and we have not yet generated any revenue from product sales. We have never been profitable and have incurred net losses in each year since commencement of our operations. We expect to incur significant and increasing losses from operations for the foreseeable future, and we can provide no assurance that we will ever generate significant revenue or profits.

Through September 30, 2017, we have financed our operations through an IPO, two follow-on public offerings, private placements of redeemable convertible preferred stock and funds received in connection with the Collaboration Agreement with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A., entered into in August 2014. Prior to our IPO, we received net proceeds of $93.9 million from the sale of shares of our Series A, A-1 and B redeemable convertible preferred stock. On November 3, 2015, we completed our IPO of 6,253,125 shares of common stock at an offering price of $10.00 per share, resulting in net proceeds of approximately $55.6 million, after deducting underwriting discounts, commissions and offering costs. On October 3, 2016, we completed a follow-on public offering of 4,370,000 shares of common stock at an offering price of $15.00 per share, resulting in net proceeds of approximately $61.1 million, after deducting underwriting discounts, commissions and estimated offering costs. On August 14, 2017, we completed another follow-on

public offering of 4,025,000 shares of common stock at an offering price of $35.50 per share, resulting in net proceeds of approximately $133.8 million, after deducting underwriting discounts, commissions and estimated offering costs. In connection with the Collaboration Agreement, we have received $105.0 million from Sanofi S.A., consisting of a $35.0 million upfront payment, a $25.0 million milestone payment for the submission of an investigational new drug application ("IND"), for MYK-491 with the FDA in November 2016, and a $45.0 million continuation payment from Sanofi in January 2017. As of September 30, 2017, we had cash and cash equivalents of $227.2 million, short-term investments of $20.0 million and long-term investments of $36.0 million, which we believe will be sufficient to fund our planned operations through at least the next twelve months.

We have no manufacturing facilities, and all of our manufacturing activities are contracted out to a third party. Additionally, we currently utilize third-party clinical research organizations ("CROs") to carry out our clinical development and trials. We do not yet have a sales organization.

We expect to incur substantial expenditures in the foreseeable future for the advancement of our precision medicine platform, the development and potential commercialization of mavacamten and MYK-491, and the discovery, development and potential commercialization of any additional product candidates we may pursue. Specifically, we expect to continue to incur substantial expenses in connection with our ongoing PIONEER-HCM Phase 2 clinical trial of mavacamten and any additional Phase 2 and Phase 3 clinical trials that we may conduct for mavacamten, as well as our ongoing and planned clinical development activities for MYK-491. We will need substantial additional funding to support our operating activities as we advance mavacamten, MYK-491, and other potential product candidates through clinical development, seek regulatory approval and prepare for, and if approved, proceed to commercialization. Adequate funding may not be available to us on acceptable terms, or at all.

The research and development expenses incurred in the development and potential commercialization of mavacamten, MYK-491 and other product candidates are (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Mavacamten	$ 7,920	$ 4,668	$ 20,344	$ 13,703
MYK-491	2,778	2,026	8,174	5,612
Other	3,663	2,089	11,449	6,877
Total research and development expenses:	$ 14,361	$ 8,783	$ 39,967	$ 26,192

License and Collaboration Agreement with Sanofi

In August 2014, we entered into the Collaboration Agreement with Aventis, Inc., a wholly-owned subsidiary of Sanofi S.A., that covers three main research programs: our first program in HCM (referred to as mavacamten or HCM-1), a second program in HCM (referred to as HCM-2) and our first program in DCM (referred to as MYK-491 or DCM-1). For purposes of this filing, we refer to Sanofi as our co-party to the Collaboration Agreement.

Under the Collaboration Agreement, we are responsible for conducting research and development activities through early human efficacy studies, except for specified research activities to be conducted by Sanofi. Thereafter, we will lead worldwide development and U.S. commercial activities for the mavacamten and HCM-2 programs, Sanofi will lead global development and commercial activities for MYK-491 and Sanofi will lead commercial activities for the mavacamten and HCM-2 programs where it has ex-U.S. commercialization rights. Sanofi also has the option to co-promote the mavacamten and HCM-2 programs in the United States only in the event of a potential expanded cardiovascular disease indication outside of the genetically targeted indications for mavacamten and HCM-2. We have co-commercialization rights to MYK-491 in the United States, at our option.

We are entitled to receive tiered royalties ranging from the mid-single digits to the mid-teens on net sales of certain HCM and DCM finished products outside the United States and on net sales of certain DCM finished products in the United States. Sanofi is eligible to receive tiered royalties ranging from the mid-single digits to the low teens on our net sales of certain HCM finished products in the United States.

Under the Collaboration Agreement, Sanofi also agreed to provide up to $200.0 million in upfront and milestone payments, equity investments and research and development support. As of September 30, 2017, of such amount, we have received from Sanofi an initial non-refundable upfront cash payment of $35.0 million and equity investments of $10.0 million in exchange for Series A-1 redeemable convertible preferred stock, and a $25.0 million milestone-based payment. In addition, we have received equity funding outside of the original agreement of $5.0 million in exchange for Series B redeemable convertible preferred stock and $9.0 million in exchange for shares of our common stock in our IPO. In January 2017, we also received a $45.0 million continuation payment. The total payments we were originally eligible to receive also included an obligation from Sanofi to purchase an additional $40.0 million of our capital stock if Sanofi provided notice of its intent to continue the collaboration prior to December 31, 2016. Under the Collaboration Agreement, Sanofi's obligation to purchase the additional $40.0 million of our capital stock (which was reduced by $5.0 million for its purchase of the Series B redeemable convertible preferred stock) terminated in connection with the closing of our IPO in November 2015. Additionally, we are eligible to receive up to $45.0 million of approved in-kind research and clinical activities.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are fully described in Note 2 of our Annual Report. We believe that the accounting policies discussed in our Annual Report are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates. There have been no changes to our significant accounting policies during the nine months ended September 30, 2017, except with respect to changes in our policy on Stock-Based Compensation. As permitted under ASU 2016-09, we have elected to recognize forfeitures as they occur, and no longer estimate a forfeiture rate when calculating the stock-based compensation for our equity awards.

Components of Operating Results

Collaboration and License Revenue

We generate revenue from the Collaboration Agreement with Sanofi for the development and commercialization of products under the collaboration.

Operating Expense

Research and Development Expenses

Research and development expenses consist of salaries and benefits, including stock-based compensation, lab supplies and facility costs, as well as fees paid to CROs to conduct certain research and development activities on our behalf. Amounts incurred in connection with collaboration and license agreements are also included in research and development expense. Payments made to third parties in advance of the performance of the related services by the third parties are recorded as prepaid expenses until the services are rendered.

General and Administrative Expenses

General and administrative expenses consist principally of personnel-related costs, professional fees for legal, consulting, audit and tax services, market research, rent and other general operating expenses not otherwise classified as research and development expenses.

Interest and Other Income, Net

Interest and other income, net consists primarily of interest income earned on our cash and cash equivalents, short-term investments and long-term investments.

Results of Operations

Comparison of the Three-Month Periods Ended September 30, 2017 and 2016

	Three Months Ended September 30,		Increase (Decrease)
	2017	2016	
	(in thousands)		
Collaboration and license revenue	$ 5,625	$ 3,550	$ 2,075
Operating expenses:			
Research and development	14,361	8,783	5,578
General and administrative	5,884	4,031	1,853
Total operating expenses	20,245	12,814	7,431
Loss from operations	(14,620)	(9,264)	5,356
Interest and other income, net	447	33	414
Net loss and comprehensive loss	$ (14,173)	$ (9,231)	$ 4,942

Collaboration and License Revenue

Collaboration and license revenue increased $2.1 million, or 58%, from the $3.6 million during the three months ended September 30, 2016 to $5.6 million for the three months ended September 30, 2017. The amount for the period ended September 30, 2016 relates to revenue we recognized from the initial upfront payment of $35.0 million under the Collaboration Agreement with Sanofi. The amount for the period ended September 30, 2017 relates to revenue we recognized from the continuation payment of $45.0 million under the Collaboration Agreement.

Research and Development Expenses

Research and development expenses increased $5.6 million, or 64%, from $8.8 million for the three months ended September 30, 2016 to $14.4 million for the three months ended September 30, 2017. The increase in research and development expenses was primarily due to a $1.3 million increase in personnel expenses as a result of an increase in employee headcount, a $1.1 million increase in contract research, chemistry and biology expenses on discovery and pre-clinical programs including HCM-2 and a back-up program for DCM-1, a $3.2 million increase in clinical expenses for mavacamten and MYK-491 clinical trials, and a $0.4 million increase in stock compensation expense.

We expect research and development expenses to increase in future periods as we continue the development of our lead product candidate, mavacamten, in clinical trials as well as preclinical and subsequent clinical activities for our MYK-491, HCM-2, additional mechanisms within DCM, and other back-up programs. As product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials, we expect that our research and development expenses will increase substantially in the future.

General and Administrative Expenses

General and administrative expenses increased $1.9 million, or 46%, from $4.0 million for the three months ended September 30, 2016 to $5.9 million for the three months ended September 30, 2017. The increase in general and administrative expenses was primarily due to a $0.2 million increase in personnel expenses as a result of an increase in employee headcount, increases of $0.2 million in office and related expenses, $0.4 million in marketing expense, $0.5 million in professional and consulting expense, $0.4 million in recruiting expense, and a $0.4 million increase in stock compensation expense as we expanded our operations.

We expect general and administrative expenses to continue to increase in future periods, reflecting both the increased costs in connection with the continued clinical development and potential future commercialization of mavacamten, the ongoing and planned clinical development of MYK-491, as well as an expanded infrastructure and increased professional fees.

Interest and Other Income, Net

Interest and other income increased $414,000 or 1255%, from $33,000 for the three months ended September 30, 2016 to $447,000 for the period ending September 30, 2017. The increase in interest income was primarily due to investments.

Comparison of the Nine-Month Periods Ended September 30, 2017 and 2016

	Nine Months Ended September 30,		Increase
	2017	2016	(Decrease)
	(in thousands)		
Collaboration and license revenue	$ 16,875	$ 10,649	$ 6,226
Operating expenses:			
Research and development	39,967	26,192	13,775
General and administrative	16,442	11,947	4,495
Total operating expenses	56,409	38,139	18,270
Loss from operations	(39,534)	(27,490)	12,044
Interest and other income, net	977	79	898
Net loss and comprehensive loss	$ (38,557)	$ (27,411)	$ 11,146

Collaboration and License Revenue

Collaboration and license revenue increased $6.2 million, or 58%, from the $10.7 million during the nine months ended September 30, 2016 to $16.9 million for the nine months ended September 30, 2017. The amount for the period ended September 30, 2016 relates to revenue we recognized from the initial upfront payment of $35.0 million under the Collaboration Agreement with Sanofi. The amount for the period ended September 30, 2017 relates to revenue we recognized from the continuation payment of $45.0 million under the Collaboration Agreement.

Research and Development Expenses

Research and development expenses increased $13.8 million, or 53%, from $26.2 million for the nine months ended September 30, 2016 to $40.0 million for the nine months ended September 30, 2017. The increase in research and development expenses was primarily due to a $3.9 million increase in personnel expenses as a result of an increase in employee headcount, a $3.6 million increase in contract research, chemistry and biology expenses on discovery and pre-clinical programs including HCM-2 and a back-up program for DCM-1, $5.9 million increase in clinical expenses for mavacamten and MYK-491 clinical trials, and a $1.2 million increase in stock compensation expense.

We expect research and development expenses to increase in future periods as we continue the development of our lead product candidate, mavacamten, in clinical trials as well as preclinical and subsequent clinical activities for our MYK-491, HCM-2, additional mechanisms within DCM, and other back-up programs. As product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials, we expect that our research and development expenses will increase substantially in the future.

General and Administrative Expenses

General and administrative expenses increased $4.5 million, or 38%, from $12.0 million for the nine months ended September 30, 2016 to $16.5 million for the nine months ended September 30, 2017. The increase in general and administrative expenses was primarily due to a $0.7 million increase in personnel expenses as a result of an increase in employee headcount, increase of $0.6 million in office and related expenses, $1.0 million in marketing, $0.7 million in professional and consulting expense, and a $1.3 million increase in stock-compensation expense as we expanded our operations.

We expect general and administrative expenses to continue to increase in future periods, reflecting both the increased costs in connection with the continued clinical development and potential future commercialization of mavacamten, the ongoing and planned clinical development of MYK-491, as well as an expanded infrastructure and increased professional fees.

Interest and Other Income, Net

Interest and other income increased $898,000 or 1137%, from $79,000 for the nine months ended September 30, 2016 to $977,000 for the nine months ending September 30, 2017. The increase in interest income was primarily due to investments.

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through private placements of our equity securities, payments received in connection with the Collaboration Agreement, and our public offerings of common stock. During 2014, we received net proceeds of $28.9 million from the sale of Series A and Series A-1 redeemable convertible preferred stock and, in August 2014, we received an upfront payment of $35.0 million from Sanofi in connection with the entry into the Collaboration Agreement. In April 2015, we received net proceeds of $45.8 million from the sale of Series B redeemable convertible preferred stock. Pursuant to the IPO in November 2015, we received net proceeds of $55.6 million, net of underwriting discounts, commissions and offering costs. On October 3, 2016, we completed a follow-on public offering of 4,370,000 shares of common stock at an offering price of $15.00 per share, resulting in net proceeds of approximately $61.1 million, after deducting underwriting discounts, commissions and estimated offering costs. On August 14, 2017, the Company completed another follow-on public offering of 4,025,000 shares of common stock at an offering price of $35.50 per share, resulting in net proceeds of approximately $133.8 million, after deducting underwriting discounts, commissions and estimated offering costs. In connection with the Collaboration Agreement, we have received $60.0 million from Sanofi S.A., consisting of a $35.0 million upfront payment, and a $25.0 million payment for the submission of an IND for MYK-491 with the FDA in November 2016. In addition, we received a $45.0 million continuation payment from Sanofi in January 2017 in connection with Sanofi's decision in December 2016 to extend the collaboration term. As of September 30, 2017, we had cash and cash equivalents of $227.2 million, short-term investments of $20.0 million and long-term investments of $36.0 million, which we believe will be sufficient to fund our planned operations through at least the next twelve months.

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales and do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize mavacamten, MYK-491 or other product candidates.

We expect that our existing cash and cash equivalents will provide sufficient funds to sustain operations through at least the next 12 months based on our existing business plan. However, we expect to incur substantial expenditures in the foreseeable future for the advancement of our precision medicine platform, the development and potential commercialization of mavacamten and MYK-491, and the discovery, development and potential commercialization of any additional product candidates we may pursue. Specifically, we have incurred substantial expenses in connection with our Phase 1 clinical trials of mavacamten and expect to continue to incur substantial expenses in connection with our ongoing PIONEER-HCM Phase 2 clinical trial of mavacamten and any additional Phase 2 and Phase 3 clinical trials that we may conduct for mavacamten, as well as our ongoing and planned clinical development activities for MYK-491. Furthermore, if our planned Phase 2 and potential Phase 3 clinical trials for mavacamten are successful, or our other product candidates, including MYK-491, enter into later stage clinical trials or more advanced discovery and development stages, we will need to raise additional capital in order to further advance our product candidates towards regulatory approval.

We will continue to require additional financing to develop our product candidates and fund operations for the foreseeable future through equity or debt financings, collaborative or other arrangements with corporate sources, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms or at all. Our failure to raise capital as and when needed could have a negative impact on our financial condition and our ability to pursue our business strategies. We anticipate that we will need to raise substantial additional capital, the requirements of which will depend on many factors, including:

- the rate of progress and the costs of our ongoing and planned clinical trials of mavacamten and MYK-491;
- our ability to receive additional payments from Sanofi pursuant to the Collaboration Agreement and the timing thereof;
- the number of product candidates that we intend to develop using our precision medicine platform;
- the costs of research and preclinical studies to support the advancement of other product candidates into clinical development;
- the timing of, and costs involved in, seeking and obtaining approvals from the FDA and comparable foreign regulatory authorities, including the potential by the FDA or comparable regulatory authorities to require that we perform more studies than those that we currently expect;
- the costs of preparing to manufacture mavacamten on a larger scale, and to manufacture MYK-491 for clinical development;
- the costs of commercialization activities if mavacamten or any future product candidate is approved, including the formation of a sales force;
- the degree and rate of market acceptance of any products launched by us or our partners;
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- our need and ability to hire additional personnel;

- our ability to enter into additional collaboration, licensing, commercialization or other arrangements and the terms and timing of such arrangements; and
- the emergence of competing technologies or other adverse market developments.

We may seek funds through borrowings or additional rounds of financing, including private or public equity or debt offerings and collaborative arrangements with corporate partners. If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Any future debt financing into which we enter may impose upon us additional covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, repurchase our common stock, make certain investments and engage in certain merger, consolidation or asset sale transactions. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. If we are unable to raise additional funds when needed, we may be required to delay, reduce, or terminate some or all of our development programs and clinical trials. We may also be required to sell or license to others technologies, product candidates or programs that we would prefer to develop and commercialize ourselves.

Cash Flows

The following table sets forth the primary sources and uses of cash for each of the periods presented below (in thousands):

	Nine Months Ended September 30,	
	2017	2016
	(in thousands)	
Cash Flows from Continuing Operations:		
Net cash used in operating activities	$ (3,207)	$ (34,364)
Net cash used in investing activities	(40,961)	(854)
Net cash provided by financing activities	135,555	73
Increase (decrease) in cash and cash equivalents	$ 91,387	$ (35,145)

Cash Used in Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2017 was $3.2 million, and was primarily due to the $45.0 million continuation payment received from Sanofi, partially offset by the net loss for the period of $38.6 million and a decrease in deferred revenue of $16.9 million, stock-based compensation expense of $4.3 million, and changes in operating assets and liabilities, including, an increase in accrued liabilities of $2.0 million.

Net cash used in operating activities for the nine months ended September 30, 2016 was $34.4 million, and was primarily due to the net loss for the period partially offset by stock-based compensation expense of $1.8 million and depreciation expense of $0.8 million, and was also affected by changes in operating assets and liabilities, including a decrease in deferred revenue of $10.6 million, and an increase in accrued liabilities of $1.0 million.

Cash Used in Investing Activities

Cash used in investing activities for the nine months ended September 30, 2017 consisted primarily of purchases of investments of $44.0 million, offset by sale of investments of $4.0 million. During the nine months ended September 30, 2017 and 2016, we also had investments in equipment of $0.9 million.

Cash Provided by Financing Activities

Cash provided by financing activities for the nine months ended September 30, 2017 consisted of net proceeds from the issuance of common stock in connection with a follow-on offering of $133.9 million and funds received as a result of common stock option exercise of $1.7 million.

Cash provided by financing activities in the nine months ended September 30, 2016 consisted of proceeds from the issuance of common stock in connection with purchases pursuant to the 2015 ESPP of $0.2 million, offset by payments of $0.2 million in deferred offering costs.

Contractual Obligations and Other Commitments

There have been no material changes outside the ordinary course of our business to our contractual obligations during the nine months ended September 30, 2017, as compared to those disclosed in our Annual Report.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements as defined in the rules and regulations of the SEC.

Jumpstart Our Business Startups Act of 2012

The Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"), permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We have elected to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable. See Note 2, Summary of Significant Accounting Policies, to our consolidated financial statements appearing in our Annual Report regarding recent accounting pronouncements.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

The market risk inherent in our financial instruments represents the potential loss arising from adverse changes in interest rates or exchange rates. As of September 30, 2017, we had cash and cash equivalents of $227.2 million, consisting of interest-bearing money market accounts, short-term investments of $20.0 million, consisting of corporate securities, and long-term investments of $36.0 million, consisting of United States government agency obligations, which would be affected by changes in the general level of United States interest rates. However, due to the low- risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair value of our cash and cash equivalents.

In addition, we are also exposed to foreign currency exchange rate risk inherent in our contracts with research institutions and contract research organizations as certain services are performed by them outside the United States. We have payments due to one Australian vendor in foreign currency. A significant movement in the Australian dollar may have a material impact on our financial position in the future.

We do not believe that inflation, interest rate changes or exchange rate fluctuations had a significant impact on our results of operations for any periods presented.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our Chief Executive Officer and Senior Vice President, Finance and Corporate Development, has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2017, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and Senior Vice President, Finance and Corporate Development have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any material litigation or other material legal proceedings.

Item 1A. RISK FACTORS

You should consider carefully the following risk factors, together with all the other information in this report, including our consolidated financial statements and notes thereto, and in our other public filings with the SEC. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to Our Limited Operating History, Financial Condition and Capital Requirements

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are an early-stage company. We were incorporated and commenced operations in June 2012. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, developing our technology, creating and expanding on our precision medicine platform, identifying potential product candidates, undertaking preclinical studies for our programs, conducting Phase 1 clinical trials for our most advanced product candidate, mavacamten (formerly known as MYK-461) and commencing and conducting Phase 2 clinical development of mavacamten and Phase 1 clinical development of our second product candidate, MYK-491. We have not yet demonstrated our ability to successfully complete the clinical development of a product candidate, including the completion of any clinical trials designed to show the efficacy of a product candidate, obtain marketing approvals, manufacture a commercial scale medicine or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes many years to develop a new medicine from the time it is discovered to when it is available for treating patients. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting larger scale clinical development and commercial activities. If we are not successful in such a transition, our business, results and financial condition will be harmed.

We have a history of significant losses and may not achieve or sustain profitability and, as a result, you may lose all or part of your investment.

Our initial product candidates, mavacamten and MYK-491, are in the early stages of clinical testing and we must conduct significant additional clinical trials before we can seek the regulatory approvals necessary to begin commercial sales of these or any other product candidates we may develop. We have incurred operating losses in each year since our inception due to costs incurred in connection with our research and development activities and general and administrative costs associated with our operations. Our net loss for the three and nine months ended September 30, 2017 was $14.2 million and $38.6 million, respectively. As of September 30, 2017, we had an accumulated deficit of $116.4 million. We expect to incur increasing losses for several years as we continue our research activities and conduct development of, and seek regulatory approvals for, our initial product candidates, and commercialize any approved drugs. If our product candidates fail in clinical trials or do not gain regulatory approval, or if our product candidates do not achieve market acceptance, we will not be profitable. If we fail to become and remain profitable, or if we are unable to fund our continuing losses, you could lose all or part of your investment.

We have never generated any revenue from product sales and may never be profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaborators, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory approvals to market product candidates for which we complete clinical trials;

- developing a sustainable, scalable, reproducible and transferable manufacturing process for our product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand, if any, for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory approval, either through a collaboration or, if launched independently, by establishing a sales force, marketing and distribution infrastructure;
- obtaining market acceptance of our product candidates and the use of precision medicine as a viable treatment option for cardiovascular diseases;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new product candidates from our platform;
- maintaining our existing collaboration agreement with Sanofi and negotiating favorable terms in any new collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel who are suitable to our culture and mission.

Even if one or more of the product candidates that we are developing is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond our expectations if we are required by the U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (the "EMA") or other regulatory agencies, domestic or foreign, to perform clinical trials and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We are currently advancing mavacamten and MYK-491, our initial product candidates, through clinical development, and conducting preclinical discovery and development activities in our other programs. Drug development is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in clinical trials.

As of September 30, 2017, our cash and cash equivalents were $227.2 million. We intend to use our cash and cash equivalents to fund the advancement of our mavacamten clinical development program, including our ongoing PIONEER-HCM Phase 2 study in symptomatic oHCM patients and our planned additional clinical trials of mavacamten, the progression of MYK-491 through clinical proof-of-concept, our ongoing preclinical, discovery and research programs and the expansion of our platform, as well as for working capital and general corporate purposes. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, mavacamten, MYK-491 or any other product candidates we may identify and develop. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

Our funding requirements and the timing of our need for additional capital are subject to change based on a number of factors, including:

- the rate of progress and the cost of our ongoing and planned clinical trials of mavacamten and MYK-491;
- our ability to successfully maintain our collaboration with Sanofi, and the amount and the timing of any subsequent payments we may receive pursuant to the collaboration;
- the number of product candidates that we intend to develop using our precision medicine platform;

- the costs of research and preclinical studies to support the advancement of other product candidates into clinical development;
- the timing of, and costs involved in, seeking and obtaining approvals from the FDA and comparable foreign regulatory authorities, including the potential by the FDA or comparable regulatory authorities to require that we perform more studies than those that we currently expect;
- the costs of preparing to manufacture mavacamten on a larger scale, and to manufacture MYK-491 for further clinical development;
- the costs of commercialization activities if mavacamten or any future product candidate is approved, including the formation of a sales force;
- the degree and rate of market acceptance of any products launched by us or our partners;
- the costs of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- our need and ability to hire additional personnel;
- our ability to enter into additional collaboration, licensing, commercialization or other arrangements and the terms and timing of such arrangements; and
- the emergence of competing technologies or other adverse market developments.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at a different stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially and adversely affect our business, financial condition and results of operations.

Our reported financial results may be adversely affected by changes in accounting principles generally accepted in the U.S.

We prepare our financial statements in conformity with accounting principles generally accepted in the U.S. These accounting principles are subject to interpretation by the Financial Accounting Standards Board ("FASB") and the Securities and Exchange Commission. A change in these policies or interpretations could have a significant effect on our reported financial results, may retroactively affect previously reported results, could cause unexpected financial reporting fluctuations, and may require us to make costly changes to our operational processes and accounting systems. In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers, which supersedes nearly all existing U.S. GAAP revenue recognition guidance. The new standard and its amendments will be effective for our fiscal year 2018 with early adoption permitted for our fiscal year 2017. Although we are currently in the process of evaluating the impact of ASU 2014-09 on our consolidated financial statements, it could change the way we account for certain of our revenue transactions. Thus, adoption of the standard could have a significant impact on our financial statements and may retroactively affect the accounting treatment of transactions completed before adoption. See "Note 2 – Summary of Significant Accounting Policies" for additional discussion of the accounting changes.

Risks Related to Our Precision Medicine Platform and the Discovery and Development of Our Product Candidates

The precision medicine approach we are taking to discover and develop drugs for heritable cardiovascular diseases is novel and may never lead to marketable products.

We have concentrated our therapeutic product research and development efforts on the application of precision medicine to the treatment of heritable cardiovascular diseases, and our future success depends on the successful development of products based on our precision medicine platform and the continued development of this platform. We believe we are the first company to apply precision medicine to the treatment of cardiovascular disease, and neither we nor any other company has received regulatory approval to market therapeutics specifically targeting any form of heritable cardiomyopathy. The scientific discoveries that form the basis for our efforts to discover and develop product candidates are novel, and the scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. If we do not successfully develop and commercialize product candidates based upon our technological approach, we will not become profitable and the value of our common stock may decline.

Further, our focus solely on precision medicine for the development of drugs for heritable cardiomyopathies as opposed to multiple, more proven technologies for drug development increases the risks associated with the ownership of our common stock. If we are not successful in developing any product candidates using our precision medicine platform, we may be required to change the scope and direction of our product development activities. In that case, we may not be able to identify and implement successfully an alternative product development strategy, which would materially and adversely affect our business, financial condition and results of operations.

We depend heavily on the success of mavacamten (formerly known as MYK-461) and MYK-491, our initial product candidates. Other than mavacamten and MYK-491, all of our other programs are in discovery or preclinical development. Preclinical testing and clinical trials of our product candidates may not be successful. If we are unable to commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the identification of our initial product candidates, mavacamten for the treatment of hypertrophic cardiomyopathy ("HCM") and MYK-491 for the treatment of dilated cardiomyopathy ("DCM"). We are currently evaluating mavacamten and MYK-491 in early-stage clinical trials, and, if these product candidates fail to demonstrate safety or efficacy in their respective target indications to the satisfaction of the FDA or other comparable regulatory authorities, we will need to identify and rely on other product candidates or target indications, or both, for clinical development. All of our other programs are still in discovery or preclinical development. Our ability to generate revenue from product sales, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of mavacamten, MYK-491 or other product candidates that we may identify from our precision medicine platform.

The success of mavacamten, MYK-491 and any other product candidates that we discover and develop will depend on many factors, including the following:

- timely and successful initiation, enrollment in, and completion of, clinical trials, including our ongoing PIONEER-HCM Phase 2 clinical trial of mavacamten in HCM, our ongoing Phase 1 clinical trial of MYK-491 in DCM and our planned additional clinical trials of these product candidates;
- our ability to receive, and the timing of receipt of, any marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity for our product candidates;
- launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- a continued acceptable safety profile of our products following approval;
- enforcing and defending intellectual property rights and claims; and
- achieving desirable medicinal properties for the intended indications.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

Preclinical and clinical drug development involves a lengthy and expensive process with an uncertain outcome, and observations and results from earlier studies and trials may not be applicable or predictive in future clinical trials.

Preclinical and clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the preclinical development or clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. For example, although our preclinical observations and data generated to date from our Phase 1 clinical trials of mavacamten support our hypothesis that mavacamten has the potential to reduce cardiac muscle contractility and our belief that such data have demonstrated clinical proof of mechanism in both HCM patients and healthy volunteers, we have not completed clinical trials of mavacamten in larger populations. In addition, our precision medicine platform is based on a translational medicine approach. Translational medicine, or the application of basic scientific findings to develop therapeutics that promote human health, is subject to a number of inherent risks. In particular, scientific hypotheses formed from preclinical or early clinical observations may prove to be incorrect, and the data generated in animal models or observed in limited patient populations may be of limited value, and may not be applicable in clinical trials conducted under the controlled conditions required by applicable regulatory requirements and our protocols. For example, although mavacamten has been observed to reduce cardiac contractility as measured by certain established biomarkers in our first Phase 1 clinical trial in healthy volunteers, the predictive value of these biomarkers in HCM patients may prove to be less than anticipated in subsequent, larger clinical trials. The initial clinical data from our Phase 1 clinical trials of mavacamten are preliminary in nature, based on limited doses and a small sample size, and the clinical development of mavacamten is not complete. Early positive data may not be repeated or observed in ongoing or future trials involving our product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. There is a high failure rate for drugs and biologics proceeding through clinical trials, particularly in the field of cardiovascular medicine. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies, and any such setbacks in our clinical development could have a material adverse effect on our business and operating results.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. We may experience delays in our ongoing clinical trials and we do not know whether planned clinical trials will begin on time, need to be redesigned, enroll patients on time or be completed on schedule, if at all. Additionally, although we believe that our precision medicine approach should eliminate the need for mavacamten to undergo the large outcomes-based studies that are often required for cardiovascular drugs as a condition to regulatory approval by the FDA or other regulatory authorities, regulatory authorities may nevertheless require us to conduct additional trials or generate additional data, including potential trials studying the interaction of our product candidates with other therapeutics commonly administered in the patient populations we are seeking to treat, which would increase the time and cost of our clinical development process. Furthermore, we will need to conduct larger clinical trials, and the FDA may subsequently require us to evaluate a larger number of patients than we presently anticipate, or to assess other endpoints besides those presently contemplated, in order to support regulatory approval.

Clinical trials can be delayed for a variety of reasons, including:

- delays in reaching a consensus with regulatory agencies on trial design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in obtaining required Institutional Review Board ("IRB") approval at each clinical trial site;
- delays in recruiting suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory agencies, including after an inspection of our clinical trial operations or trial sites;
- failure by our CROs, other third parties or us to adhere to clinical trial requirements;
- failure by us or our CROs or other third-party contractors to perform clinical trials in accordance with the FDA's good clinical practice ("GCP") requirements or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;

- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical trial sites deviating from a trial protocol or dropping out of a trial;
- clinical trial subjects failing to comply with the trial regimen or dropping out of a trial;
- adding new clinical trial sites;
- failure to manufacture or supply sufficient quantities of product candidates for use in clinical trials;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, or suspension or termination is recommended by the Data Safety Monitoring Board ("DSMB") for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or other regulatory authorities. The FDA or other regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or other regulatory authorities may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authorities, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

If the results of our clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy ("REMS");
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing to commence and complete our clinical trials depends on the speed at which we can recruit patients to participate in testing our product candidates. If patients are unwilling to participate in our clinical trials because of a lack of familiarity with our approach to the treatment of cardiovascular diseases, negative publicity from adverse events in biotechnology or the fields of precision medicine or cardiovascular disease or for other reasons, including competitive clinical trials for similar patient populations, our timelines for recruiting patients, conducting clinical trials and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of our clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical trials in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the clinical trial protocol;
- size and nature of the patient population;
- eligibility criteria for the clinical trial in question;
- perceived risks and benefits of the product candidate under study in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions in which we plan to evaluate our current product candidates is a rare genetic disorder with limited patient pools from which to draw for clinical trials. To date, the HCM and DCM patient populations have not been extensively evaluated in clinical trials. As a result, enrollment in our ongoing and planned clinical trials is difficult to predict and may take longer or cost more than we anticipate.

We plan to seek initial marketing approval in the United States. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with CROs and physicians;
- different standards for the conduct of clinical trials;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

We may not be successful in our efforts to identify or discover potential product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize therapeutics for the treatment of genetic cardiovascular diseases based on our precision medicine approach. A key element of our strategy is to use our precision medicine platform to identify and study compounds that can be used to correct or offset the abnormal contraction caused by HCM and DCM. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying appropriate biomarkers or potential product candidates;
- our initial hypotheses based on our preclinical or early clinical observations may not be supported by later clinical results;
- potential product candidates may, on further study, be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval; or
- research programs to identify new product candidates require substantial technical, financial and human resources. We may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful.

If we are unable to identify suitable compounds for preclinical and clinical development, we may be forced to abandon our development efforts for a research program or programs and we will not be able to obtain product revenues in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

We may not be able to successfully use the Sarcomeric Human Cardiomyopathy Registry, or SHaRe, to identify or recruit patients for our clinical trials or to develop targeted precision therapeutics for the treatment of heritable cardiomyopathies.

We rely, and expect to continue to rely, on genetic and clinical data gathered through SHaRe to provide us with insight into risk profiles and disease progression in heritable cardiomyopathies. Although the body of information in SHaRe is growing, we may face challenges collecting additional data through SHaRe in the future for a variety of reasons, including:

- insufficient funding to support the research necessary to generate patient data for SHaRe;
- our failure to maintain existing relationships and establish new relationships with clinical investigators and research institutions whose activities support SHaRe and provide us with access to patient data;
- our failure to maintain or increase interest in SHaRe within our target patient communities; and
- third parties may generate competing databases to which we do not have access.

Additionally, the predictive value of the information generated through SHaRe to date may be limited. Although we expect to use these data to define and identify patient subgroups most likely to respond to our product candidates, our initial hypotheses regarding these data may prove to be incorrect, or our patient selection strategies based on our analysis of these data may fail to yield suitable patients for evaluation in our clinical trials or suitable indications and product candidates for clinical development.

Any of our product candidates may cause adverse effects or have other properties that could delay or prevent their regulatory approval, limit the scope of any approved label or market acceptance or result in other significant negative consequences following marketing approval, if any.

Adverse events or other unintended side effects or safety signals caused by our product candidates could cause us, IRBs or ethics committees, clinical trial sites or regulatory authorities to interrupt, delay or halt clinical trials and could result in the denial of regulatory approval. For example, through additional studies, we may determine that although mavacamten has been shown to be specific to striated muscle, which includes both skeletal and cardiac muscle, and selective for cardiac muscle, it may target myosin in skeletal muscle, which could result in unintended adverse effects. We have observed a number of adverse events in our clinical trials of mavacamten. In particular, we have observed at least one serious adverse event to date that occurred in the highest dose cohort of our single ascending dose Phase 1 clinical trial of mavacamten in HCM patients, which was described as a transient episode of hypotension and asystole, due to a vasovagal reaction, or low blood pressure and a temporary loss of heartbeat due to a nervous system reflex. Results of our ongoing and planned trials could reveal a high and unacceptable severity and prevalence of these or other adverse events in subjects treated with our product candidates. Additionally, if the adverse events we have observed are deemed to be unacceptable or other unacceptable side effects or safety signals are observed in any ongoing or subsequent preclinical studies or clinical trials of our product candidates, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. Any adverse effects encountered in our preclinical studies or clinical trials, whether or not drug-related, could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Additionally,

adverse effects may represent safety signals that could influence the benefit-risk assessment for further development or commercialization of a product candidate and may warrant further clinical or nonclinical investigation, consultation with health authorities, changes to product labeling or guidelines for its safe use, or other scientific or regulatory actions. Any of these occurrences may harm our business, financial condition and prospects significantly.

Further, if any of our future products, if and when approved for commercial sale, cause serious or unexpected adverse events, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw their approval of the product or impose restrictions on its distribution in the form of a REMS or provide a medication guide outlining the risks of such side effects for distribution to patients;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications;
- we may be required to change the way the product is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; or
- our reputation may suffer.

Any of these events could prevent us or our partners from achieving or maintaining market acceptance of the affected product and could substantially increase the costs of commercializing our future products and impair our ability to generate revenues from the commercialization of these products.

Risks Related to Government Regulation

We currently do not have regulatory approval to market any of our product candidates. The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA, EMA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a New Drug Application (" NDA") or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; or
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market mavacamten, MYK-491 or any other product candidate we may develop, which would significantly harm our business, results of operations and prospects.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate or the approval may be for a more limited indication than we expect.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. If we are unable to obtain regulatory approval for our product candidates for use in the treatment of heritable cardiomyopathies, our business may suffer.

Failure to obtain marketing approval in international jurisdictions would prevent our products from being marketed in such jurisdictions.

In order to market and sell our products in the European Union and many other jurisdictions, we or our third-party collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval in other jurisdictions. We may not be able to file for marketing approvals, and even if we do, we may not obtain necessary approvals to commercialize our medicines in any market.

Even if we receive regulatory approval for any of our product candidates, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to extensive and ongoing regulatory requirements and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, current Good Manufacturing Practice ("cGMP") requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. For example, the holder of an approved NDA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the NDA. The holder of an approved NDA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP and adherence to commitments made in the NDA and other marketing authorizations.

Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

The FDA closely regulates the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our medicines for their approved indications, we may be subject to enforcement action for off-label marketing.

In addition, later discovery of previously unknown problems with our medicines, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in various negative consequences, including:

- restrictions on the labeling, marketing or manufacturing of the product;
- restrictions on distribution or use of the product;
- requirements to conduct post-marketing clinical trials or holds on ongoing or planned clinical trials;
- warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal by the FDA or comparable foreign regulatory authorities to approve pending applications or supplements to approved applications that we submit;
- mandatory or voluntary recalls;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our medicines;
- product seizure or detention; and
- injunctions or the imposition of civil or criminal penalties.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

We may seek one or more special designations from regulatory authorities for our product candidates, including Breakthrough Therapy Designation, Fast Track Designation or Orphan Drug Designation. These designations may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek one or more special designations from regulatory authorities for our product candidates, including Breakthrough Therapy Designation, Fast Track Designation or Orphan Drug Designation.

A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically important endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Products designated as breakthrough therapies by the FDA can also be eligible for accelerated approval. If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this condition, the product sponsor may apply for Fast Track Designation.

The FDA has broad discretion whether or not to grant these designations, so even if we believe a particular product candidate is eligible for a particular designation, we cannot assure you that the FDA would decide to grant it. Accordingly, even if we believe one of our product candidates meets the criteria for a designation, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a particular designation for a product candidate may not result in a faster development

process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification and rescind the breakthrough designation. Further, the FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from a clinical development program.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, Orphan Drug Designation entitles a party to incentives such as tax advantages and user-fee waivers. In April 2016, the FDA granted Orphan Drug Designation for mavacamten for use in the treatment of symptomatic obstructive HCM.

In addition, if a product that has Orphan Drug Designation subsequently receives the first approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which in the United States means that the FDA may not approve any other applications to market the same drug for the same indication for seven years, except in limited circumstances. The exclusivity granted under any Orphan Drug Designations that we have received or may receive may not effectively protect the product candidate from competition. Although we have received Orphan Drug Designation from the FDA for mavacamten for use in the treatment of symptomatic obstructive HCM, we may not be the first to obtain marketing approval of this drug for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we obtain orphan drug exclusivity, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior, in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. Any inability to secure or maintain Orphan Drug Designation or the exclusivity benefits of this designation would have an adverse impact on our ability to develop and commercialize our product candidates.

Risks Related to Our Reliance on Third Parties

We are substantially dependent upon our collaboration agreement with Sanofi for the development and eventual commercialization of mavacamten, MYK-491 and any product candidates from our HCM-2 program. If this collaboration is unsuccessful or is terminated, we may be unable to commercialize certain product candidates and we will not receive additional funding from this relationship.

We depend upon our license and collaboration agreement with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A., which we refer to as the Collaboration Agreement, for financial and scientific resources related to the clinical development and commercialization of product candidates under our mavacamten, MYK-491 and HCM-2 programs. While Sanofi has obligations to fund various research and development activities under the collaboration and with respect to the commercialization of product candidates in selected territories under certain programs under the Collaboration Agreement, our ability to receive funding from this relationship will depend upon the ability and willingness of Sanofi to successfully meet its responsibilities under our Collaboration Agreement and continue the collaboration. We may not receive some or all of the financial and scientific resources that we currently expect to receive under our Collaboration Agreement.

Our ability to generate additional funding from our Collaboration Agreement may be impaired by several factors including:

- Sanofi may shift its priorities and resources away from our programs due to a change in business strategies, or a merger, acquisition, sale or downsizing of its company or business unit;
- Sanofi may cease development and commercialization activities in therapeutic areas which are the subject of our collaboration;
- Sanofi may change the success criteria for a particular program or potential product candidate, thereby delaying or ceasing development of such program or candidate;

- Sanofi may exercise its rights to terminate the collaboration; or
- a dispute may arise between us and Sanofi concerning financial obligations or the research, development or commercialization of a program or product candidate, resulting in a delay in payments or termination of a program and possibly resulting in costly litigation or arbitration which may divert management attention and resources.

Specifically, with respect to termination, the initial term of the research program under our Collaboration Agreement with Sanofi is set to end on December 31, 2018. At any time after December 31, 2018, Sanofi may, upon prior written notice to us, terminate the Collaboration Agreement for convenience in its entirety or on a region-by-region or program-by-program basis with respect to selected regions or programs. The Collaboration Agreement is also subject to termination by either party upon a material breach by the other party, subject to a notice and cure period, or upon a bankruptcy, insolvency or similar event affecting the other party.

If our Collaboration Agreement with Sanofi is terminated, then, depending on the event:

- the development of our product candidates subject to the Collaboration Agreement may be terminated or significantly delayed;
- our cash expenditures could increase significantly if it is necessary for us to hire additional employees and allocate internal resources to the development and commercialization of product candidates that were previously funded, or expected to be funded, by Sanofi;
- we would bear all of the risks and costs related to the further development and commercialization of product candidates that were previously the subject of the Collaboration Agreement;
- in order to fund further development and commercialization, we may need to seek out and establish alternative strategic collaborations with third-party partners, which may not be possible; or
- we may not be able to do so on terms which are acceptable to us, in which case it may be necessary for us to limit the size or scope of one or more of our programs or increase our expenditures and seek additional funding by other means.

Any of these events would have a material adverse effect on our results of operations and financial condition.

We expect to rely on third parties to conduct some or all aspects of our protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our protocol development, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our preclinical studies and clinical trials are conducted in accordance with the study plan and protocols. We and our third-party contractors and CROs are required to comply with GCP regulations, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area ("EEA"), and comparable foreign regulatory authorities for all products in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our third-party contractors or CROs fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will be delayed in completing, or may not be able to complete, the preclinical and clinical studies required to support future IND submissions and approval of our product candidates. Any of these events could lead to clinical study delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, request for voluntary recall, seizure or total or partial suspension of production.

We contract with third parties for the manufacture of our product candidates for preclinical and clinical testing and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or medicines or that such supply will not be available to us at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities. We currently rely, and expect to continue to rely, on third-party manufacturers for the manufacture of our product candidates for preclinical and clinical testing and for the commercial supply of any of these product candidates for which we or our collaborators obtain marketing approval. To date, we have obtained materials for mavacamten for our Phase 1 clinical trials and our ongoing PIONEER-HCM Phase 2 clinical trial from third-party manufacturers, and we intend to rely on third-party manufacturers for our ongoing and planned clinical development activities for mavacamten and MYK-491. We do not have a long term supply agreement with the third-party manufacturers, and we purchase our required drug supply on a purchase order basis. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- reliance on the third party for regulatory compliance, quality assurance, and safety and pharmacovigilance reporting;
- the possible breach of the manufacturing agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

The facilities used by our contract manufacturers to manufacture any of our future products must be evaluated by the FDA pursuant to inspections that will be conducted after we submit an NDA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the cGMP regulation for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority finds deficiencies with or does not approve these facilities for the manufacture of our product candidates or if it finds deficiencies or withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or voluntary recalls of product candidates or medicines, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our medicines and harm our business and results of operations.

Any products that we may develop may compete with our other product candidates and products and the products of third parties for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for a redundant supply of bulk drug substances. If any one of our current contract manufacturers cannot perform as agreed, we may be required to replace that manufacturer. Although we believe that there are several potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of our product candidates or medicines may adversely affect our future profit margins and our ability to commercialize any medicines that receive marketing approval on a timely and competitive basis.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our medicines and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize medicines and technology similar or identical to ours, and our ability to successfully commercialize our medicines and technology may be adversely affected.

Our commercial success will depend, in part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary products and technology. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and medicines that are important to our business. To date, we own two issued patents that cover our proprietary technology or product candidates. We cannot be certain that we will secure any additional rights to any issued patents with claims that cover any of our proprietary technology or product candidates.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and medicines, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. With respect to our proprietary molecularly targeted small molecule drugs, we consider trade secrets and know-how to be our primary intellectual property. Trade secrets and know-how can be difficult to protect. In particular, we anticipate that with respect to our precision medicine platform, these trade secrets and know-how will over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology, and the movement of personnel skilled in the art from academic to industry scientific positions.

We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be harmed.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the U.S. Patent and Trademark Office, or the U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. In the future, we may have ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition

proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Risks Related to Commercialization and the Market for Our Product Candidates

If the market opportunities for our product candidates are smaller than we believe they are or if we are unable to market our products to expanded patient populations, our revenues may be adversely affected and our business may suffer.

We focus our research and product development efforts on treatments for heritable cardiomyopathies, and our targeted indications are rare genetic diseases. In particular, we estimate that approximately 630,000 people in the United States have a form of HCM, and that approximately 360,000 people in the United States have a form of genetic DCM. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates derived from primary research with physicians and payors, analysis of medical journals and peer-reviewed literature, the work of third-party consultants and other publicly- or non-publicly-available data sources. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of our targeted disease indications. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products, and new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Additionally, because the target patient populations of our product candidates are small, we must be able to successfully identify patients and achieve a significant market share to achieve or maintain profitability and growth. Although we plan to use SHaRe to identify and select patients who are suitable for treatment with our products, if approved, we may not be able to identify or target a sufficient number of patients through SHaRe or our sales and marketing efforts to achieve the necessary market share.

Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. For example, current cardiovascular disease treatments such as beta blockers, non-dihydropyridine calcium channel blockers and disopyramide are well-established in the medical community, and doctors may continue to rely on these treatments. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- the ability to offer our medicines for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;

- sufficient third-party coverage or reimbursement; and
- the prevalence and severity of any side effects.

Any failure to achieve or maintain sufficient market acceptance of mavacamten, MYK-491 or any of our other product candidates, if approved, could significantly harm our business, prospects, financial condition and results of operations.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenues.

We have no experience marketing or selling our product candidates. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. We may enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be unable to enter into marketing agreements on favorable terms, if at all. If our future collaborations do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

The insurance coverage and reimbursement status of newly-approved products targeting small patient populations is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford expensive treatments, such as endothelin receptor antagonists used in the treatment of certain cardiovascular diseases. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. Additionally, therapies directed at small patient populations, such as our product candidates, may be more expensive, and reimbursement options for these therapies may be more limited. If reimbursement or coverage is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products and for products whose targeted patient populations are small. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services (“CMS”), an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. It is difficult to predict what CMS or third-party payors will decide with respect to reimbursement and coverage for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries may put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Risk Related to Our Business and Industry

We may be subject to healthcare laws, regulation and enforcement, and our failure to comply with these laws could harm our results of operations and financial conditions.

Although we do not currently have any products on the market, if we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our customers and third-party payors, subject to various U.S. federal and state fraud and abuse, patient privacy laws and other healthcare regulatory laws, and to additional healthcare, statutory and regulatory requirements and enforcement by foreign regulatory authorities in jurisdictions in which we conduct our business. Healthcare providers, physicians and others play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. These laws may impact, among other things, our proposed sales, marketing and education programs and constrain the business of financial arrangements and relationships with healthcare providers, physicians and other parties through which we market, sell and distribute our products for which we obtain marketing approval. The laws that may affect our ability to operate include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or paying any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal false claims laws, which imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the U.S. federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") which imposes criminal and civil liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services; similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH") and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to the HIPAA Rules, published in January 2013, which imposes certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization on covered entities subject to the rule, such as health plans, healthcare clearinghouses and healthcare providers as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information;
- the U. S. Federal Food, Drug, and Cosmetic Act ("FDCA") which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U. S. legislation commonly referred to as the Physician Payments Sunshine Act, enacted as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, "ACA") and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program to report annually to CMS, information related to payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

- analogous state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U. S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; and state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and
- European and other foreign law equivalents of each of the laws, including reporting requirements detailing interactions with and payments to healthcare providers.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

Our competitors may develop drugs that are less expensive, safer, or more effective, which may diminish or eliminate the commercial success of any drugs that we may commercialize.

Our competitors may:

- develop drug candidates and market drugs that are less expensive or more effective than our future drugs;
- commercialize competing drugs before we or our partners can launch any drugs developed from our drug candidates;
- initiate or withstand substantial price competition more successfully than we can;
- have greater success in recruiting skilled scientific workers from the limited pool of available talent;
- more effectively negotiate third-party licenses and strategic collaborations; and
- take advantage of acquisition or other opportunities more readily than we can.

We will compete for market share against large pharmaceutical and biotechnology companies and smaller companies that are collaborating with larger pharmaceutical companies, new companies, academic institutions, government agencies and other public and private research organizations. Many of these competitors, either alone or together with their partners, may develop new drug candidates that will compete with ours, as these competitors may, and in certain cases do, operate larger research and development programs or have substantially greater financial resources than we do. Our competitors may also have significantly greater experience in:

- developing product candidates;
- undertaking preclinical testing and clinical trials;
- building relationships with key customers and opinion-leading physicians;
- obtaining and maintaining FDA and other regulatory approvals of product candidates;
- formulating and manufacturing drugs; and
- launching, marketing and selling drugs.

If our competitors market drugs that are less expensive, safer or more effective than our potential drugs, or that reach the market sooner than our potential drugs, we may not achieve commercial success. In addition, the life sciences industry is characterized by rapid technological change. Because our research approach integrates many technologies, it may be difficult for us to stay abreast of the rapid changes in each technology. If we fail to stay at the forefront of technological change, we may be unable to compete effectively. Our competitors may render our technologies obsolete by advances in existing technological approaches or the development of new or different approaches, potentially eliminating the advantages in our drug discovery process that we believe we derive from our research approach and proprietary technologies.

Public opinion and heightened regulatory scrutiny of precision medicine for the treatment of cardiovascular disease may impact public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Precision medicine remains a novel technology, particularly in the field of cardiovascular disease, with no products approved to date in the United States that are specifically targeted at correcting the underlying biomechanical defects in cardiac contractility associated with HCM and DCM. Public perception may be influenced by claims that these therapies are unproven or unsafe, and our product candidates may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians, who specialize in the treatment of those diseases that our product candidates target, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion could have an adverse effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity, could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States, the EU, and other foreign jurisdictions, there have been a number of legislative and regulatory changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, ACA changes the way healthcare is financed by both governmental and private insurers and significantly impacts the U.S. pharmaceutical and biotechnology industries. ACA, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs and biologic products, and a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D. At this time, the full effect that ACA or any successor laws and regulations would have on our business remains unclear.

In addition, other legislative changes have been proposed and adopted in the United States since ACA was enacted. On August 2, 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2025 unless additional Congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other health care funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. The U.S. federal government has set a goal of moving 50% of Medicare payments into these "Alternative Payment Models" by the end of 2018. In addition, recently there has been heightened governmental scrutiny over the manner in which drug manufacturers set prices for their commercial products. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Our future success depends on our ability to retain key employees and consultants, including our scientific advisors and founders, and to attract, retain and motivate qualified personnel.

We are highly dependent on our scientific advisors and founders and the principal members of our executive team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives and scientific experts in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies, as well as from academic and research institutions, for individuals with similar skill sets. In addition, any failure of our programs to succeed in preclinical studies or clinical trials may make it more challenging to recruit and retain qualified personnel. The inability to recruit or the loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2017, we had 98 full-time employees. As we mature, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial medicines or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Our anticipated international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement and economic risks associated with doing business outside of the United States.

We have a wholly-owned Australian subsidiary through which we conduct clinical trials in Australia. Our business strategy also contemplates potential additional international operations as we seek to continue the development of mavacamten, MYK-491 and other product candidates that we have or may identify, seek regulatory approval for our product candidates, and commercialize any product candidates that are approved outside the United States. If any product candidates for which we have retained worldwide commercial rights are approved, we may hire sales representatives and conduct physician and patient group outreach activities outside of the United States. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- complexities and difficulties in obtaining protection for and enforcing our intellectual property rights;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as exposure to foreign currency exchange rate fluctuations and their impact on payments required in local currency;
- natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our future international expansion and operations and, consequently, our results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Our employees, independent contractors, principal investigators, CROs, consultants, vendors and collaboration partners may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.

We are exposed to the risk that our employees, independent contractors, principal investigators, CROs, consultants, vendors and collaboration partners may engage in fraudulent conduct or other illegal activities. Misconduct by these parties could include intentional, reckless and/or negligent conduct or unauthorized activities that violate: (i) the regulations of the FDA, EMA and other regulatory authorities, including those laws that require the reporting of true, complete and accurate information to such authorities; (ii) manufacturing standards; (iii) federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the United States and abroad; or (iv) laws that require the reporting of true, complete and accurate financial information and data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws could also involve the improper use of information obtained in the course of clinical trials or creating fraudulent data in our preclinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent misconduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other U.S. federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Unfavorable global economic and political conditions could adversely affect our business, financial condition or results of operations.

Our ability to invest in and expand our business and meet our financial obligations, to attract and retain third-party contractors and collaboration partners and to raise additional capital depends on our operating and financial performance, which, in turn, is subject to numerous factors, including the prevailing economic and political conditions and financial, business and other factors beyond our control, such as the rate of unemployment, the number of uninsured persons in the United States, political influences and inflationary pressures. For example, an overall decrease in or loss of insurance coverage among individuals in the United States as a result of unemployment, underemployment or the potential repeal of certain provisions of the ACA, may decrease the demand for healthcare services and pharmaceuticals. If fewer patients are seeking medical care because they do not have insurance coverage, we may experience difficulties in any eventual commercialization of our product candidates and our business, results of operations, financial condition and cash flows could be adversely affected.

In addition, our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets upon which biopharmaceutical companies such as us are dependent for sources of capital. In the past, global financial crises have caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including a reduced ability to raise additional capital when needed on acceptable terms, if at all, and weakened demand for our product candidates. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any medicines that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any medicines that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or medicines caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or medicines that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize mavacamten, MYK-491 or any other product candidates that we may develop.

Although we maintain product liability insurance, including coverage for clinical trials that we sponsor, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage as we commence additional clinical trials and if we successfully commercialize any product candidates. The market for insurance coverage is increasingly expensive, and the costs of insurance coverage will increase as our clinical programs increase in size. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Our internal computer systems, or those of our third-party CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our operations.

Despite the implementation of security measures, our internal computer systems and those of our third-party CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data for our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and experience delays or disruptions to various aspects of our operations, including our financial reporting and the development of our product candidates.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code") if a corporation undergoes an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a rolling three-year period, the corporation's ability to use its pre-change net operating loss carryforwards ("NOLs") and other pre-change tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. While we have determined that an ownership change occurred in April 2015 in connection with our Series B redeemable convertible preferred stock financing, we do not believe that this ownership change will result in the expiration of any of our existing NOLs prior to utilization. We may experience subsequent shifts in our stock ownership, some of which are outside our control. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Risks Related to Our Common Stock

The market price of our common stock has been and may continue to be highly volatile.

The market price of our common stock has experienced volatility since our IPO in October 2015 and is likely to continue to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- adverse results or delays in preclinical studies or clinical trials;
- reports of adverse events in clinical trials of our product candidates or in other products for the treatment of cardiovascular diseases or clinical trials of such products;
- inability to obtain additional funding;
- any delay in filing an IND or NDA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or NDA;
- failure to develop successfully and commercialize our product candidates;
- any adverse developments relating to our Collaboration Agreement with Sanofi, or any failure to maintain our existing strategic collaborations or enter into new collaborations;
- failure by us or our licensors and strategic collaborators to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate product supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions affecting our product candidates or development programs;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public or to the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic collaboration partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

In addition, companies trading in the stock market in general, and The NASDAQ Global Select Market ("NASDAQ") in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business, financial condition, results of operations and growth prospects.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

In addition, sales of a substantial number of shares of our outstanding common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. Persons who were our stockholders prior to our IPO continue to hold a substantial number of shares of our common stock that many of them are now able to sell in the public market. Significant portions of these shares are held by a relatively small number of stockholders. Sales by our stockholders of a substantial number of shares, or the expectation that such sales may occur, could significantly reduce the market price of our common stock.

We have also registered all shares of our common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. As a result, these shares will be eligible for sale in the public market to the extent permitted by any applicable vesting requirements and the exercise of options, and restrictions under applicable securities laws. In addition, our directors, executive officers and certain affiliates have established or may in the future establish programmed selling plans under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, for the purpose of effecting sales of our common stock. If any of these events cause a large number of our shares to be sold in the public market, the sales could reduce the trading price of our common stock and impede our ability to raise future capital. Moreover, certain holders of our common stock have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. In particular, we have filed a registration statement on Form S-3 registering for resale 9,184,352 shares of common stock held by entities affiliated with our major shareholder, Third Rock Ventures, which was declared effective by the SEC on July 21, 2017. These shares, together with any additional shares that we may register for resale, can be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Pursuant to our 2015 Stock Option and Incentive Plan (the "2015 Plan"), we are authorized to grant stock options and other equity-based awards to our employees, directors and consultants. Beginning on January 1, 2017, the number of shares available for future grant under the 2015 Plan will automatically increase each year by up to 4% of all shares of our capital stock outstanding as of December 31 of the prior calendar year, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. In addition, pursuant to our 2015 Employee Stock Purchase Plan (the "2015 ESPP"), we initially reserved 255,000 shares for purchase by eligible employees. Beginning on January 1, 2017 and ending on January 1, 2025, the number of shares available for future issuance under the 2015 ESPP will automatically increase each year by up to the lesser of 3,000,000 shares of common stock or 1% of all shares of our capital stock outstanding as of December 31 of the prior calendar year, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. Currently, we plan to register the increased number of shares available for issuance under the 2015 Plan and the 2015 ESPP each year. If our board of directors elects to increase the number of shares available for future grant under these plans by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of October 31, 2017, our executive officers, directors, five percent or greater stockholders and their affiliates beneficially own approximately 47.9% of our outstanding voting stock. These stockholders will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

We have broad discretion in the use of our cash and cash equivalents and may not use them effectively.

Our management has broad discretion in the application of our existing cash and cash equivalents, and you will not have the opportunity to assess whether our existing cash and cash equivalents are being used appropriately. Because of the number and variability of factors that will determine our use of our existing cash and cash equivalents, their ultimate use may vary substantially from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest our cash and cash equivalents in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend, in part, on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts may not publish an adequate amount of research on our company, which may negatively impact the trading price for our stock. In addition, if one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. Further, if our operating results fail to meet the forecasts of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

We are an "emerging growth company," and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We will remain an emerging growth company until the earliest of (i) December 31, 2020, (ii) the last day of the fiscal year in which we have total annual gross revenue of $1.07 billion or more, (iii) the last day of the fiscal year in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th and (iv) the date on which we have issued more than $1.07 billion in non-convertible debt during any three-year period before that time. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act ("Section 404"), and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, and particularly after we are no longer an emerging growth company, we incur and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and NASDAQ have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting starting with our Annual Report on Form 10-K for the fiscal year ending December 31, 2016. However, while we remain an emerging growth company, we will not be required to obtain an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting.

Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could impair our ability to produce timely and accurate consolidated financial statements and result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our consolidated financial statements.

Our operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult to predict our future operating results. Our net loss and other operating results will be affected by numerous factors, many of which are outside of our control and may be difficult to predict, including:

- variations in the level of expenses related to our precision medicine platform, our product candidates or our research and development programs;
- the timing and success or failure of preclinical studies and clinical trials for our product candidates or competing product candidates;
- our ability to obtain regulatory approval for our product candidates, and the timing and scope of any such approvals we may receive;
- if any of our product candidates receives regulatory approval, the level of underlying demand for these product candidates;
- addition or termination of clinical trials or funding support;
- our execution of any new collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements.
- any intellectual property infringement or other lawsuits in which we may become involved; and
- regulatory developments affecting our product candidates or those of our competitors.

If our operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. Additionally, due to the unpredictability of our quarterly and annual operating results, we believe that period-to-period comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and amended and restated bylaws include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors, the chairperson of our board of directors, our chief executive officer or our president;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that our directors may be removed only for cause and with the vote of the holders of 75% or more of our outstanding capital stock then entitled to vote at an election of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even if less than a quorum;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated bylaws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated bylaws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us.

Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

(a) Recent Sales of Unregistered Equity Securities

None.

(b) Use of Proceeds

Not applicable.

(c) Issuer Repurchases of Company Equity Securities.

Period	(a) Total Number of Shares Purchased (1)	(b) Average Price Paid per Share	(c) Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	(d) Maximum Number (or Approximate Dollar Value) of Shares that May Yet Be Purchased Under the Plans or Programs
		(in thousands, except per share amounts)		
July 1, 2017 through July 31, 2017	—	$ —	—	—
August 1, 2017 through August 31, 2017	15,589	1.51	—	—
September 1, 2017 through September 30, 2017	—	—	—	—
Total	15,589	$ 1.51	—	—

(1) Under certain stock purchase agreements with employees, we have the right to repurchase common stock at the lower of fair value and the stockholders' original purchase price, which right lapses according to individual vesting schedules.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

The following exhibits are filed or furnished as part of this Quarterly Report on Form 10-Q:

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference From	Date	Number	Filed Herewith
3.1	Restated Certificate of Incorporation.	10-Q	11/18/2015	3.1	
3.2	Amended and Restated Bylaws.	S-1/A	10/13/2015	3.4	
4.1	Specimen Common Stock Certificate.	S-1/A	10/19/2015	4.1	
31.1	Certification of Principal Executive Officer Required Under Rule 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as amended.				X
31.2	Certification of Principal Financial Officer Required Under Rule 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as amended.				X
32.1*	Certification of Principal Executive Officer and Principal Financial Officer Required Under Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. §1350.				X
101.INS	XBRL Instance Document.				X
101.SCH	XBRL Taxonomy Extension Schema Document.				X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.				X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.				X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.				X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.				X

* The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of MyoKardia, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

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, thereunto duly authorized.

Date: November 3, 2017

MYOKARDIA, INC.

By: /s/ Tassos Gianakakos
Tassos Gianakakos
President, Chief Executive Officer
(Principal Executive Officer)

Date: November 3, 2017

By: /s/ Jake Bauer
Jake Bauer
Senior Vice President, Finance and Corporate Development
(Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER

PURSUANT TO

SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Tassos Gianakakos, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of MyoKardia, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 3, 2017

/s/ Tassos Gianakakos
Tassos Gianakakos
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER

PURSUANT TO

SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Jake Bauer, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of MyoKardia, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 3, 2017

/s/ Jake Bauer
Jake Bauer
Senior Vice President, Finance and Corporate Development
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of MyoKardia, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended September 30, 2017, as filed with the Securities and Exchange Commission (the "Report"), Tassos Gianakakos, Chief Executive Officer of the Company, and Jake Bauer, Senior Vice President, Finance and Corporate Development (Principal Financial and Accounting Officer) of the Company, respectively, do each hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of his knowledge:

- The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- The information in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 3, 2017

/s/ Tassos Gianakakos
Tassos Gianakakos
Chief Executive Officer
(Principal Executive Officer)

/s/ Jacob Bauer
Jacob Bauer
Senior Vice President, Finance and Corporate Development
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. Section 1350), has been provided to MyoKardia, Inc. and will be retained by MyoKardia, Inc. and furnished to the Securities and Exchange Commission or its staff upon request. This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of MyoKardia, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.