**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 March 31, 2020**

**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.)** |

**1000 Sierra Point Parkway**
**Brisbane, CA**
**(Address of principal executive offices)**

**94005**
**(Zip Code)**

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

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common Stock | MYOK | NASDAQ Global Select Market |

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 every Interactive Data File required to be submitted 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 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 | ☐ | 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 May 1, 2020 was 46,680,808 shares.

**MYOKARDIA, INC.**

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## PART I—FINANCIAL INFORMATION

**Item 1. Unaudited Condensed Consolidated Financial Statements**

**MYOKARDIA, INC.**

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

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| **Assets** | | |
| Current assets | | |
| Cash and cash equivalents | $ 131,204 | $ 101,436 |
| Short-term investments | 218,809 | 314,691 |
| Prepaid expenses and other current assets | 7,486 | 7,709 |
| Total current assets | 357,499 | 423,836 |
| Property and equipment, net | 19,801 | 15,743 |
| Operating lease right-of-use assets | 51,981 | 417 |
| Long-term investments | 10,077 | 14,153 |
| Restricted cash and other | 1,968 | 1,945 |
| Total assets | $ 441,326 | $ 456,094 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities | | |
| Accounts payable | 5,573 | $ 6,237 |
| Accrued liabilities | 32,236 | 41,292 |
| Operating lease liabilities - current | 7,851 | 383 |
| Total current liabilities | 45,660 | 47,912 |
| Operating lease liability | 44,658 | — |
| Other long-term liabilities | 1,908 | 1,908 |
| Total liabilities | 92,226 | 49,820 |
| Commitments and contingencies (Note 7) | | |
| 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 shares authorized at March 31, 2020 and December 31, 2019; 46,612,186 and 46,379,073 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively | 5 | 5 |
| Additional paid-in capital | 897,058 | 884,486 |
| Accumulated other comprehensive income | 671 | 549 |
| Accumulated deficit | (548,634) | (478,766) |
| Total stockholders' equity | 349,100 | 406,274 |
| Total liabilities and stockholders' equity | $ 441,326 | $ 456,094 |

*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 March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Operating expenses: | | |
| Research and development | $ 51,878 | $ 26,190 |
| Selling, general and administrative | 19,902 | 13,551 |
| Total operating expenses | 71,780 | 39,741 |
| Loss from operations | (71,780) | (39,741) |
| Interest and other income, net | 1,912 | 2,271 |
| Net loss | (69,868) | (37,470) |
| Other comprehensive income | 122 | 363 |
| Comprehensive loss | $ (69,746) | $ (37,107) |
| Net loss per share, basic and diluted | $ (1.50) | $ (0.93) |
| Weighted average number of shares used to compute net loss per share, basic and diluted | 46,566,995 | 40,506,313 |

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

**MYOKARDIA, INC.**

## Condensed Consolidated Statements of Stockholders' Equity
## (In thousands, except share and per share amounts)
## (Unaudited)

**For the three months ended March 31, 2020**

| | Common Stock | | Additional Paid-In Capital | Accumulated other comprehensive income | Accumulated Deficit | Total Stockholders' Equity |
|---|---|---|---|---|---|---|
| | Shares | Amount | | | | |
| **BALANCE—December 31, 2019** | 46,379,073 | $ 5 | $ 884,486 | $ 549 | $ (478,766) | $ 406,274 |
| Issuance of common stock upon the exercise of options and release of stock awards | 233,113 | — | 1,820 | — | — | 1,820 |
| Stock-based compensation | — | — | 10,752 | — | — | 10,752 |
| Unrealized gains | — | — | — | 122 | — | 122 |
| Net loss | — | — | — | — | (69,868) | (69,868) |
| **BALANCE—March 31, 2020** | 46,612,186 | $ 5 | $ 897,058 | $ 671 | $ (548,634) | $ 349,100 |

**For the three months ended March 31, 2019**

| | Common Stock | | Additional Paid-In Capital | Accumulated other comprehensive income/ (loss) | Accumulated Deficit | Total Stockholders' Equity |
|---|---|---|---|---|---|---|
| | Shares | Amount | | | | |
| **BALANCE—December 31, 2018** | 40,288,949 | $ 4 | $ 573,183 | $ (67) | $ (202,553) | $ 370,567 |
| Issuance of common stock in connection with the 2019 follow-on offering, net of issuance costs of $17,638 | 5,663,750 | 1 | 271,212 | — | — | 271,213 |
| Issuance of common stock upon the exercise of options and release of stock awards | 49,076 | — | 280 | — | — | 280 |
| Vesting of early exercised stock options | — | — | 8 | — | — | 8 |
| Stock-based compensation | — | — | 6,981 | — | — | 6,981 |
| Unrealized gains | — | — | — | 363 | — | 363 |
| Net loss | — | — | — | — | (37,470) | (37,470) |
| **BALANCE—March 31, 2019** | 46,001,775 | $ 5 | $ 851,664 | $ 296 | $ (240,023) | $ 611,942 |

*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)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2020** | **2019** |
| **Cash flow from operating activities:** | | |
| Net loss | $ (69,868) | $ (37,470) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Stock-based compensation expense | 10,752 | 6,981 |
| Depreciation | 779 | 461 |
| Amortization of discount on investments | (418) | (292) |
| Loss on disposal of equipment | 49 | — |
| Change in operating assets and liabilities: | | |
| Prepaid expenses and other current assets | 226 | 448 |
| Operating lease right-of-use assets | 1,066 | 635 |
| Other long-term assets | — | (13) |
| Accounts payable | (467) | 1,950 |
| Accrued liabilities | (1,512) | (180) |
| Prepayment from collaboration partner | — | (9,874) |
| Operating lease liabilities | (504) | — |
| Other long-term liabilities | — | (692) |
| **Net cash used in operating activities** | (59,897) | (38,046) |
| **Cash flow from investing activities:** | | |
| Purchases of investments | — | (32,697) |
| Sales of investments | 4,000 | 4,000 |
| Maturities of investments | 96,475 | 16,000 |
| Purchases of property and equipment | (12,627) | (813) |
| **Net cash provided by (used in) investing activities** | 87,848 | (13,510) |
| **Cash flow from financing activities:** | | |
| Proceeds from issuance of common stock in follow-on offerings, net of issuance and financing costs | — | 271,485 |
| Proceeds from exercise of stock options and employee stock purchase plan | 1,817 | 280 |
| **Net cash provided by financing activities** | 1,817 | 271,765 |
| **Net increase in cash, cash equivalents and restricted cash** | 29,768 | 220,209 |
| Cash, cash equivalents and restricted cash, beginning of period | 103,630 | 248,265 |
| Cash, cash equivalents and restricted cash, end of period | $ 133,398 | $ 468,474 |
| **Non-cash investing and financing activities:** | | |
| Unpaid portion of property and equipment purchases included in period-end accounts payable and accrued liabilities | $ 2,015 | $ 570 |
| Vesting of early exercised options and restricted stock | $ — | $ 8 |
| Unpaid financing-related costs | $ — | $ 262 |

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

## 1. Organization

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. The Company's initial focus is on the treatment of cardiomyopathies, a group of diseases of the heart muscle. MyoKardia's pipeline includes: mavacamten and MYK-224, which are being studied for the treatment of hypertrophic cardiomyopathy; LUS-1, being studied for the treatment of diseases of diastolic dysfunction; and danicamtiv (formerly MYK-491) and ACT-1, being studied for the treatment of diseases of systolic dysfunction.

MyoKardia's most advanced programs are: mavacamten, which is in four clinical trials including a Phase 3 study in patients with hypertrophic cardiomyopathy (HCM); danicamtiv, which recently completed a Phase 2a multiple-ascending dose study in patients with stable systolic heart failure and is being advanced to a Phase 2 study in patients with genetic dilated cardiomyopathy; and MYK-224, which is in a Phase 1 randomized, placebo-controlled study in healthy volunteers.

The Company was incorporated on June 8, 2012 in Delaware and its corporate headquarters and operations are in Brisbane, California.

### *Liquidity*

The Company has incurred significant operating losses since inception and has an accumulated deficit of $548.6 million as of March 31, 2020. The Company has relied on its ability to fund its operations through private and public equity financings and to a lesser extent, through a license and collaboration arrangement with a collaboration partner, Sanofi S.A. (Sanofi) via its subsidiary, Aventis Inc. The collaboration agreement ended on December 31, 2018 and the Company had no revenues relating to its Sanofi collaboration after December 31, 2018, nor has it received reimbursements of research and development expenses after June 30, 2019. The Company has not yet received regulatory approval to commercialize or sell any product and does not have customers. Management expects operating losses and negative operating cash flows to continue for the foreseeable future. As the Company continues to incur losses, a transition to profitability is dependent upon the successful development, approval, and commercialization of the Company's products and product candidates and the achievement of a level of revenues adequate to support its cost structure. The Company's ultimate success depends on the outcome of its research and development activities and anticipates the need to raise additional capital to fully implement its business plan. The Company intends to raise such capital through the issuance of additional equity, debt and/or strategic alliances with partner companies. There is no assurance that such financing will be available or that such strategic alliances will be executed on terms acceptable to the Company, or at all.

As of March 31, 2020, the Company had $360.1 million of cash, cash equivalents and short and long-term investments, which management believes will be sufficient to meet the Company's anticipated operating and capital expenditure requirements for the twelve months following the date of issuance of these financial statements. Management's belief with respect to its ability to fund operations is based on estimates that are subject to risks and uncertainties. If actual results are different from management's estimates, the Company may need to seek additional funding. In addition, the Company is closely monitoring ongoing developments in connection with the COVID-19 pandemic, which may negatively impact its financial and operating results.

The Company will continue to assess its operating expenses and cash and cash equivalents and, if circumstances warrant, the Company will make appropriate adjustments to its operating plan.

## 2. Basis of Presentation and Summary of Significant Accounting Policies

*Basis of Presentation*

The accompanying condensed consolidated financial statements are unaudited, include the Company's accounts and those of its wholly-owned subsidiaries MyoKardia Australia Pty Ltd and MyoKardia Netherlands B.V., and have been prepared in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP").

The condensed consolidated balance sheet at December 31, 2019, has been derived from the audited consolidated financial statements as of that date but does not include all of the information and footnotes required by U.S. GAAP for complete financial statements. The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full fiscal year or any interim period and should be read in conjunction with the audited financial statements for the year ended

December 31, 2019 and the notes thereto, which are included in the Company's Annual Report on Form 10-K as of and for the year ended December 31, 2019. The significant accounting policies used in preparation of these condensed consolidated financial statements for the periods shown are consistent with those discussed in notes to the consolidated financial statements in the Company's 2019 Annual Report on Form 10-K and are updated below as necessary.

The Company currently operates in one business segment, which is the identification, development and commercialization of therapies for the treatment of serious and neglected rare cardiovascular diseases and has a single reporting unit and operating segment. These interim statements, in the opinion of management, reflect all normal recurring adjustments necessary for the fair statement of the Company's financial position and results of operations for the interim periods ended March 31, 2020 and 2019, respectively.

*Reconciliation of Cash, Cash Equivalents, and Restricted Cash as Reported in Consolidated Statements of Cash Flows*

Cash as reported in the consolidated statements of cash flows includes the aggregate amounts of cash, cash equivalents and restricted cash as presented on the consolidated balance sheets. Restricted cash at March 31, 2020 and December 31, 2019 represents cash balances held as security in connection with the Company's facility lease agreements. The following table provides a reconciliation of cash, cash equivalents and restricted cash within the consolidated balance sheets to the total shown in the consolidated statements of cash flows (in thousands):

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Cash and cash equivalents | $ 131,204 | $ 101,436 |
| Restricted cash included in prepaid expenses and other current assets | 337 | 337 |
| Restricted cash included in restricted cash and other | 1,857 | 1,857 |
| Total cash, cash equivalents and restricted cash shown in the consolidated statements of cash flows | $ 133,398 | $ 103,630 |

*Use of Estimates*

The preparation of consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities and the reported amounts of revenues and expenses in the consolidated financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to clinical trials accrued liabilities, income tax valuation allowance and stock-based compensation. Management bases its estimates on historical experience and on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

***Recently Adopted Accounting Pronouncements***

In November 2018, the Financial Accounting Standards Board (FASB) issued ASU 2018-18 (Topic 808), *Clarifying the Interaction Between Topic 808 and Topic 606*, which provides guidance on how to assess whether certain transactions between collaborative arrangement participants should be accounted for within the revenue recognition standard. The ASU also provides more comparability in the presentation of revenue for certain transactions between collaborative arrangement participants. It accomplishes this by allowing organizations to only present units of account in collaborative arrangements that are within the scope of the revenue recognition standard together with revenue accounted for under the revenue recognition standard. The parts of the collaborative arrangement that are not in the scope of the revenue recognition standard should be presented separately from revenue accounted for under the revenue recognition standard. The Company adopted this amendment in the first quarter of 2020 and the adoption did not have a material impact to the Company's financial statements.

In August 2018, the FASB issued ASU 2018-13 (Topic 820), *Fair Value Measurement*, which modifies the disclosure requirements in Topic 820 by removing requirements for disclosing (i) amounts of and reasons for transfers between the Level 1 and Level 2 hierarchies, (ii) the policy for timing of transfers between levels and (iii) the valuation processes for Level 3 fair value measurements. The ASU 2018-13 amendment also adds requirements for disclosure of changes in unrealized gains and losses for the period relating to Level 3 fair value measurements and other factors considered in the valuation of Level 3 investments. The Company adopted this amendment in the first quarter of 2020 and the adoption did not have a material impact to the Company's financial statements.

In June 2016, the FASB issued ASU No. 2016-13 (Topic 326), *Financial Instruments –Measurement of Credit Losses on Financial Instruments*, which requires measurement and recognition of expected credit losses for financial assets by requiring an allowance to be recorded as an offset to the amortized cost of such assets. For available-for-sale debt securities, expected credit losses

should be estimated when the fair value of the debt securities is below their associated amortized costs. The Company adopted this amendment in the first quarter of 2020 and the adoption did not have a material impact to the Company's financial statements.

**3. Sanofi License and Collaboration Agreement**

***Sanofi (Aventis Inc.)***

*Agreement Overview, Termination and Repurchase of Royalty Rights*

Until December 31, 2018 the Company had an exclusive license and collaboration agreement (Collaboration Agreement) with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A. (Sanofi). On December 31, 2018, Sanofi notified the Company of its intent to terminate the collaboration, specifically, Sanofi elected not to continue with the mavacamten, MYK-224 and danicamtiv programs. As a result, cost sharing and Sanofi's reimbursement of our research and development costs for mavacamten and MYK-224 ended in the first half of 2019. At that time Sanofi had continuing rights to royalties in the event of commercialization of the mavacamten and MYK-224 programs. In July 2019, the Company repurchased those rights from Sanofi for $80.0 million. Neither the Company nor Sanofi have any material continuing rights or obligations under the Collaboration Agreement.

**4. Fair Value Measurements**

Fair value accounting is applied for all financial assets and liabilities, including short-term and long-term investments and non-financial assets and liabilities that are recognized or disclosed at fair value in the consolidated financial statements on a recurring basis (at least annually). The carrying amount of the Company's financial instruments, including accounts payable and accrued liabilities and other current liabilities approximate fair value due to their short-term maturities.

Marketable securities are stated at their estimated fair values. The counterparties to the agreements relating to the Company's investment securities consist of the U.S. Treasury, governmental agencies, various major corporations and financial institutions with high credit standing. The carrying amounts for financial instruments consisting of cash and cash equivalents, receivable from collaboration partner, accounts payable and accrued liabilities approximate fair value due to their short maturities.

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 March 31, 2020 | | | |
|---|---|---|---|---|
| | Total | Level 1 | Level 2 | Level 3 |
| **Assets** | | | | |
| Money market funds | $ 130,176 | $ 130,176 | $ — | $ — |
| U.S. government agency obligations | 107,657 | — | 107,657 | — |
| Corporate securities | 121,229 | — | 121,229 | — |
| Total | $ 359,062 | $ 130,176 | $ 228,886 | $ — |

| | Fair Value Measurements at December 31, 2019 | | | |
|---|---|---|---|---|
| | Total | Level 1 | Level 2 | Level 3 |
| **Assets** | | | | |
| Money market funds | $ 100,441 | $ 100,441 | $ — | $ — |
| U.S. government agency obligations | 134,055 | — | 134,055 | — |
| Corporate securities | 194,789 | — | 194,789 | — |
| Total | $ 429,285 | $ 100,441 | $ 328,844 | $ — |

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

| | Fair Value Measurements at March 31, 2020 | | | |
|---|---|---|---|---|
| | Amortized Cost | Unrealized Gain | Unrealized Loss | Fair Value |
| Cash equivalents (due within 90 days) | $ 130,153 | $ 23 | $ — | $ 130,176 |
| Short-term investments (due within one year) | 218,129 | 827 | (147) | 218,809 |
| Long-term investments (due between one and two years) | 10,104 | 27 | (54) | 10,077 |
| Total | $ 358,386 | $ 877 | $ (201) | $ 359,062 |

| | Fair Value Measurements at December 31, 2019 | | | |
|---|---|---|---|---|
| | Amortized Cost | Unrealized Gain | Unrealized Loss | Fair Value |
| Cash equivalents (due within 90 days) | $ 100,440 | $ 1 | $ — | $ 100,441 |
| Short-term investments (due within one year) | 314,181 | 523 | (13) | 314,691 |
| Long-term investments (due between one and two years) | 14,110 | 47 | (4) | 14,153 |
| Total | $ 428,731 | $ 571 | $ (17) | $ 429,285 |

**5. Leases**

The Company determines if an arrangement is or contains a lease at inception. Operating lease right-of-use (ROU) assets and liabilities are presented separately on our consolidated balance sheets. The Company does not have any finance leases.

Operating lease ROU assets and operating lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term beginning at the commencement date. As the Company's leases do not provide enough information to determine an implicit interest rate, the Company determines its incremental borrowing rate based on the rate of interest that the Company would have to pay to borrow on a collateralized basis over a similar term in a similar economic environment. The operating lease ROU assets also include any lease payments made and excludes lease incentives and initial direct costs incurred. Lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term.

In September 2018, the Company entered into a lease agreement (the "Lease") for approximately 129,800 square feet of office and laboratory space in Brisbane, California, which is now the Company's corporate headquarters. The Company performed an evaluation of the Lease and determined it is an operating lease. The lease commencement date was in January 2020 and on the commencement date, the Company recognized $52.6 million of ROU asset and lease liability on its consolidated balance sheet in accordance with ASC 842. The Lease grants the Company an option to extend the Lease for an additional 10-year period. This optional period was not included in the measurement of the ROU asset or lease liability as it was not reasonably certain that the Company would exercise the option. The Lease requires the Company to share in prorated operating expenses and property taxes based upon actual amounts incurred. These expenses were classified as variable lease costs due to the Company's election to account for each separate lease component and the non-lease components associated with that lease component as a single lease component.

The Lease provides for annual base rent of approximately $5.5 million in the first year of the lease term. The annual base rent for the second twelve months will be approximately $8.5 million, which will increase on an annual basis beginning from the 25th month to approximately $11.1 million for the tenth year of the lease.

As of March 31, 2020, the Company has capitalized $10.6 million of leasehold improvements within property and equipment and amortizes it over the lease term.

Supplemental balance sheet information related to operating leases was as follows (in thousands, except lease term and discount rate).

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| **Assets** | | |
| Operating lease right-of-use assets | $ 51,981 | $ 417 |
| | | |
| **Liabilities** | | |
| Operating lease liabilities - current | $ 7,851 | $ 383 |
| Operating lease liabilities - noncurrent | 44,658 | — |
| | $ 52,509 | $ 383 |
| | | |
| Weighted average remaining lease term (years) | 9.8 | 0.3 |
| Weighted average discount rate | 13.5% | 6.0% |

Information related to operating lease activity during the three months ended March 31, 2020 and 2019 was as follows (in thousands):

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Operating lease cost | $ 2,738 | $ 669 |
| Variable lease cost | 532 | — |
| Total lease cost | $ 3,270 | $ 669 |
| | | |
| Operating lease right-of-use assets obtained in exchange for lease obligations | $ 52,630 | $ 1,095 |
| | | |
| Operating lease payments | $ 2,174 | $ 727 |

Future annual payments of operating lease liabilities as of March 31, 2020 are as follows (in thousands):

| Year ending December 31: | Amount |
|---|---|
| 2020 (nine months remaining) | $ 6,185 |
| 2021 | 8,449 |
| 2022 | 8,745 |
| 2023 | 9,051 |
| 2024 | 9,368 |
| Thereafter | 52,442 |
| Total future lease payments | 94,240 |
| Less: imputed interest | (41,731) |
| Total operating lease liabilities | $ 52,509 |

The operating leases require the Company to share in prorated operating expenses and property taxes based upon actual amounts incurred; those amounts are not fixed for future periods and, therefore, are not included in the future commitments listed above.

## 6. Balance Sheet Components

### *Property and Equipment*

Property and equipment are stated at cost and depreciated using the straight-line method over the estimated useful lives of the assets, ranging from two to five years. Leasehold improvements are amortized over the shorter of their estimated useful lives or the related lease term. Upon retirement or sale, the cost and related accumulated depreciation are removed from the consolidated balance sheet and the resulting gain or loss is reflected in the consolidated statement of operations and comprehensive loss.

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

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Scientific equipment | $ 12,430 | $ 10,642 |
| Furniture and equipment | 3,185 | 2,572 |
| Capitalized software | 389 | 389 |
| Leasehold improvements | 12,272 | 509 |
| Construction in progress | — | 9,568 |
| Total | 28,276 | 23,680 |
| Less: Accumulated depreciation | (8,475) | (7,937) |
| Property and equipment, net | $ 19,801 | $ 15,743 |

Depreciation expense was $0.8 million and $0.5 million for the three months ended March 31, 2020 and 2019, respectively.

### *Accrued Liabilities*

Accrued liabilities consist of the following (in thousands):

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Clinical research and development | $ 12,946 | $ 11,494 |
| Outside services | 8,164 | 6,592 |
| Payroll-related liabilities | 6,166 | 11,724 |
| Construction in progress | — | 9,139 |
| Other | 4,960 | 2,343 |
| Total accrued liabilities | $ 32,236 | $ 41,292 |

## 7. 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.

### *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 liabilities 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 March 31, 2020 or December 31, 2019.

***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.

**8. Stockholders' Equity**

On January 3, 2020, the Company entered into a sales agreement with a sales agent to establish an at-the-market (ATM) offering program, under which the Company is permitted to offer and sell, from time to time, shares of common stock having a maximum aggregate offering price of up to $200 million. As of March 31, 2020, no securities have been issued pursuant to the ATM agreement.

In March 2019, the Company completed a follow-on offering and issued 5,663,750 shares of common stock at a price of $51.00 per share, which included 738,750 shares sold directly to the underwriters upon exercise of their over-allotment option. During the three months ended March 31, 2019, the Company received proceeds totaling approximately $271.2 million from the offering, net of underwriting discounts and commissions and offering expenses.

***Common Stock Reserved for Issuance***

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

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Options and awards issued and outstanding | 6,181,912 | 5,315,254 |
| Shares available for issuance under 2015 Stock Option and Incentive Plan | 1,440,826 | 685,435 |
| Shares available for issuance under 2015 Employee Stock Purchase Plan | 1,604,331 | 1,140,541 |
| Total | 9,227,069 | 7,141,230 |

**9. 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 equity awards granted to employees and consultants for all periods presented (in thousands):

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Research and development | $ 5,192 | $ 2,963 |
| Selling, general and administrative | 5,560 | 4,018 |
| Total | $ 10,752 | $ 6,981 |

The following summarizes option and other equity award 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, 2019 | 4,574,158 | $ 31.81 |
| Options granted | 801,937 | 70.08 |
| Options exercised | (107,870) | 16.88 |
| Options canceled/forfeited | (27,486) | 44.50 |
| Balance at March 31, 2020 | 5,240,739 | 37.91 |

| | Shares Subject to Outstanding Awards | Weighted Average Grant Date Fair Value |
|---|---|---|
| Balance at December 31, 2019 | 741,096 | $ 46.59 |
| RSUs awarded | 333,324 | 70.09 |
| RSUs released | (125,243) | 43.19 |
| RSUs forfeited | (8,004) | 47.68 |
| Balance at March 31, 2020 | 941,173 | 55.36 |

Restricted stock units ("RSUs") settle into shares of common stock upon vesting and the fair value is the market price on the date of grant.

In relation to stock options and awards that vest upon the achievement of performance criteria, there was $0.3 million and zero in stock-based compensation expense recorded for the three months ended March 31, 2020 and 2019, respectively. The Company begins to recognize expenses related to these stock options and awards during the period upon concluding that certain performance criteria are considered probable.

**10. Net Loss per Share**

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 March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Numerator | | |
| Net loss | $ (69,868) | $ (37,470) |
| Denominator | | |
| Weighted average shares outstanding | 46,566,995 | 40,512,983 |
| Less: weighted average shares subject to repurchase | — | (6,670) |
| Weighted average shares used to compute basic and diluted net loss per share | 46,566,995 | 40,506,313 |
| Net loss per share, basic and diluted | $ (1.50) | $ (0.93) |

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 March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Common stock subject to repurchase | — | 5,003 |
| Options and awards issued and outstanding | 6,181,912 | 5,257,140 |

As of March 31, 2020, the Company has contributions from plan participants of $1.0 million under the 2015 ESPP, which if converted, would be equivalent to approximately 20,000 shares based on 85% of the stock price at the beginning of the offering period. As of March 31, 2019, the Company had contributions from plan participants of $0.7 million under the 2015 ESPP, which if converted, would have been equivalent to approximately 15,000 shares based on 85% of the stock price at the beginning of the offering period.

**11. Income Taxes**

Deferred tax assets and deferred tax liabilities are determined based on temporary differences between the financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some portion or all of the deferred tax assets will not be realized.

The Company does not recognize a tax benefit for uncertain tax positions unless it is more likely than not that the position will be sustained upon examination by tax authorities, including resolution of any related appeals or litigation processes, based on the technical merits of the position. The tax benefit potentially recorded for these positions is measured at the largest amount of cumulative benefit that has greater than a 50 percent likelihood of being realized upon ultimate settlement. Deferred tax assets that do not meet these recognition criteria are not recorded and the Company recognizes a liability for uncertain tax positions that may result in tax payments. If such unrecognized tax benefits were realized and not subject to valuation allowances, the entire amount would impact the tax provision.

The Coronavirus Aid, Relief, and Economic Security Act (the “CARES Act”) was enacted by the United States Congress on March 27, 2020. The Company has not participated in any provision of the CARES Act to date and has decided not to apply for a loan under the Paycheck Protection Program (“PPP”) component of this legislation. The CARES Act did not impact the Company’s provision for income taxes or consolidated financial statements for the three months ended March 31, 2020.

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

*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, 2019, included in our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the U.S. Securities and Exchange Commission (SEC) on February 27, 2020 (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 these 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 cardiovascular diseases. Our goal is to be the world's leading precision cardiovascular medicine company. Precision medicine involves discovering and developing therapies that integrate clinical and molecular information based on the biological basis of disease. Our strategy is to identify homogenous subgroups of patients with a given cardiovascular disease, understand the causal factors underlying that subgroup's condition and develop targeted therapies designed to correct the common underlying defect leading to abnormal cardiac contraction or relaxation within each subgroup.

Our lead clinical-stage candidate is mavacamten, a novel, oral, allosteric modulator of cardiac myosin being developed for the treatment of hypertrophic cardiomyopathy (HCM). Mavacamten is intended to reduce cardiac muscle contractility by inhibiting the excessive myosin-actin cross-bridge formation that underlies the excessive contractility, left ventricular hypertrophy and reduced compliance characteristic of HCM. In 2016, mavacamten was granted Orphan Drug Designation by the United States Food and Drug Administration (FDA) for the treatment of symptomatic obstructive HCM. We are currently conducting a Phase 3 pivotal study of mavacamten, known as EXPLORER-HCM in patients with symptomatic obstructive (New York Heart Association Class II or III) HCM. Enrollment in the EXPLORER-HCM study completed in August 2019 at clinical sites in the United States, Europe, and Israel. Topline data from the EXPLORER-HCM study are anticipated in the second quarter of 2020. Pending the outcome of this study, we plan to file a New Drug Application seeking regulatory approval of mavacamten for the treatment of obstructive HCM.

In addition to the EXPLORER-HCM study of mavacamten, we are conducting a MAVA long-term extension (LTE) study of mavacamten in patients who have completed our Phase 3 EXPLORER-HCM study or the Phase 2 MAVERICK-HCM in non-obstructive HCM. To protect the safety of study participants, investigators and staff, and to ensure consistent and appropriate clinical trial conduct in light of the COVID-19 pandemic, we have temporarily suspended the rollover of patients from EXPLORER into the MAVA-LTE study and plan to resume enrollment when conditions permit.

MAVERICK-HCM is a Phase 2 clinical trial initiated in 2018 for the potential treatment of symptomatic non-obstructive HCM. In November 2019, we reported positive top-line results and established safety and tolerability of mavacamten in non-obstructive HCM over a treatment period of 16 weeks. Additional data were reported in March 2020. Meaningful reductions in biomarkers of cardiac stress were observed in patients receiving mavacamten versus those in the placebo cohort and clear signals of clinical benefit were noted in a subgroup with elevated cardiac filling pressures and in a pre-specified group of patients at higher risk for morbidity and mortality. Based on the safety and pharmacologic benefits observed in the MAVERICK-HCM study, we plan to advance mavacamten into additional studies in defined groups of patients with non-obstructive HCM and heart failure with preserved ejection fraction (HFpEF).

Patient enrollment in the VALOR-HCM study was previously planned to begin in the second quarter of 2020. In March of 2020 we announced that patient enrollment in the VALOR-HCM study would be delayed due to the global outbreak of COVID-19. The VALOR-HCM study is designed to evaluate mavacamten as a therapeutic alternative to septal reduction therapy (SRT) in obstructive HCM patients. SRT is a highly invasive procedure consisting of a surgical or catheter-based therapy that eliminates or reduces the left ventricular outflow tract obstruction. We will begin enrollment of the study when conditions safely permit.

We are also conducting PIONEER-OLE, an open label extension study of obstructive HCM patients from our Phase 2 PIONEER study. Data for twelve patients at 48 weeks of treatment with mavacamten were consistent with prior safety and efficacy observations at the 12, 24, and 36-week readouts. Highlights of the data included continued safety and tolerability and sustained clinical benefits, including reductions in left ventricular outflow tract (LVOT) gradient, improvements in New York Heart Association functional class and improvement of multiple biomarkers toward normal ranges. A reduction in septal wall thickness, a defining characteristic of HCM, as well as an improvement in patient reported quality of life, as measured by the Kansas City Cardiomyopathy Questionnaire were also reported.

Our second clinical-stage candidate is danicamtiv, designed to increase the contractility of the heart (systolic function) with minimal or no effect on myocardial relaxation and compliance (diastolic function) by acting directly on the proteins in the heart muscle responsible for contraction.

We recently announced interim results from a Phase 2a multiple-ascending dose clinical trial of danicamtiv, in patients with stable heart failure. This trial follows the completion of two single-ascending dose Phase 1 studies, in healthy volunteers and in patients with dilated cardiomyopathy, a disease of systolic dysfunction that can result in heart failure. After seven days of treatment with danicamtiv, the average increase in stroke volume, a measure of the amount of blood pumped from the left ventricle, was greater than 10% relative to baseline, on a placebo-adjusted basis. No impact on measures of diastolic function, or the heart's ability to relax and fill, was observed. Patient enrollment into the Phase 2 study of danicamtiv in patients with genetic dilated cardiomyopathy was previously scheduled to begin in the second quarter of 2020. In March of 2020 we announced that patient enrollment into that study would be delayed due to the impact of COVID-19. We will begin enrollment when conditions safely permit.

In August 2019, we commenced dosing healthy volunteers in a Phase 1 study of small molecule MYK-224. The randomized, placebo-controlled Phase 1 study is designed to evaluate the safety, tolerability and pharmacokinetics of MYK-224. In March of 2020 we announced that due to the impact of COVID-19 we have temporarily suspended enrollment of healthy volunteers in the study. We plan to resume enrollment as soon as conditions safely permit.

The ultimate impacts of COVID-19 on our business are currently unknown. We will continue to actively monitor the situation and may take further precautionary and preemptive actions as may be required by federal, state or local authorities or that we determine are in the best interests of public health and safety and that of our patient community, employees, partners, suppliers and stockholders. We cannot predict the effects that such actions, or the impact of COVID-19 on global business operations and economic conditions may have on our business or strategy, including the effects on our ongoing and planned clinical development activities and prospects, or on our financial and operating results.

**Financial Overview**

We have not generated net income from operations and, as of March 31, 2020, had an accumulated deficit of $548.6 million, primarily as a result of research and development and selling, general and administrative expenses. We have not generated any revenue from product sales since our inception and have funded our operations primarily through the issuance of equity securities and payments from Sanofi pursuant to our Collaboration Agreement with Sanofi that was terminated in December 2018. 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. As of March 31, 2020, we have cash and cash equivalents of $131.2 million, short-term investments of $218.8 million and long-term investments of $10.1 million.

On January 3, 2020, we entered into a sales agreement with a sales agent to establish an at-the-market (ATM) offering program, under which we are permitted to offer and sell, from time to time, shares of common stock having a maximum aggregate offering price of up to $200 million. As of March 31, 2020, no securities have been issued pursuant to the ATM agreement.

*Termination of Sanofi Collaboration*

Until December 31, 2018 we had an exclusive Collaboration Agreement with Sanofi. On December 31, 2018, Sanofi notified us of its intent to terminate our collaboration, specifically, Sanofi elected not to continue with the mavacamten, MYK-224 and danicamtiv programs. As a result, cost sharing and Sanofi's reimbursement of our research and development costs for mavacamten and MYK-224 ended in the first half of 2019. At that time Sanofi had continuing rights to royalties in the event of commercialization

of the mavacamten and MYK-224 programs. In July 2019, we repurchased those rights from Sanofi for $80.0 million. Neither we nor Sanofi have any material continuing rights or obligations under the Collaboration Agreement.

**Components of Operating Results**

***Operating Expense***

*Research and Development Expenses*

Research and development expenses consist of salaries and benefits, including stock-based compensation, lab supplies and facility costs and fees paid to contract manufacturing organizations (CMOs) and clinical research organizations (CROs) to conduct certain research and development activities on our behalf. Research and development expenses are shown net of amounts that Sanofi agreed to reimburse us under the cost sharing program for research and development activities. Payments made prior to the receipt of goods or services are capitalized until the goods or services are received.

Research and development expenses incurred in the development and potential commercialization of mavacamten, danicamtiv and other product candidates are shown net of zero and $9.9 million in reductions in expense due to Sanofi research and development reimbursements during the three months ended March 31, 2020 and 2019, respectively as follows (in thousands):

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Mavacamten | $ 28,913 | $ 12,623 |
| Danicamtiv | 4,378 | 4,829 |
| Other | 18,587 | 8,738 |
| Total research and development expenses | $ 51,878 | $ 26,190 |

*Selling, General and Administrative Expenses*

Selling, general and administrative expenses consist principally of salaries and benefits, including stock-based compensation, 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.

***Critical Accounting Estimates***

See Part I, Item 7, “Critical Accounting Estimates” in our Annual Report. There have been no material changes to our critical accounting estimates disclosed in our Annual Report.

**Results of Operations**

***Comparison of the Three Months Ended March 31, 2020 and 2019***

The following table compares the operating results (in thousands):

| | Three Months Ended March 31, | | Change | |
|---|---|---|---|---|
| | 2020 | 2019 | $ | % |
| Operating expenses: | | | | |
| Research and development | 51,878 | 26,190 | 25,688 | 98% |
| Selling, general and administrative | 19,902 | 13,551 | 6,351 | 47% |
| Total operating expenses | 71,780 | 39,741 | 32,039 | 81% |
| Loss from operations | (71,780) | (39,741) | (32,039) | 81% |
| Interest and other income, net | 1,912 | 2,271 | (359) | -16% |
| Net loss | $ (69,868) | $ (37,470) | $ (32,398) | 86% |

* Not meaningful

*Research and Development Expenses*

Research and development expenses increased $25.7 million, or 98%, from $26.2 million for the three months ended March 31, 2019 to $51.9 million for the three months ended March 31, 2020. The increase in research and development expenses was primarily due to the following:

- a $9.9 million decrease in research and development reimbursements from Sanofi;
- a $4.0 million increase in personnel expenses due to a higher employee headcount;
- a $3.7 million increase in clinical expenses related to our mavacamten and danicamtiv clinical trials;
- a $3.0 million increase in facility and information technology expenses;
- a $2.4 million increase in consultant fees;
- a $2.2 million increase in stock compensation expense;
- a $0.9 million increase in contract manufacturing; and
- a $0.8 million increase in medical affairs expense and other;
- offset by a $1.2 million decrease in contract research, lab supplies and software.

*Selling, General and Administrative Expenses*

Selling, general and administrative expenses increased $6.4 million, or 47%, from $13.6 million for the three months ended March 31, 2019 to $19.9 million for the three months ended March 31, 2020. The increase in selling, general and administrative expenses was primarily due to the following:

- a $1.9 million increase in personnel expenses due to a higher employee headcount;
- a $1.5 million increase in stock compensation expense;
- a $1.2 million increase in marketing expenses;
- a $1.0 million increase in software, facilities and other; and
- a $0.8 million increase in professional fees.

*Interest and Other Income, Net*

Interest and other income decreased $0.4 million, or 16%, from $2.3 million for the three months ended March 31, 2019 to $1.9 million for the three months ended March 31, 2020. The decrease in interest income was primarily due to interest earned on lower invested balances and lower interest rates on investments.

**Liquidity and Capital Resources**

We consider the following when assessing our liquidity and capital resources (in thousands):

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Cash and cash equivalents | $ 131,204 | $ 101,436 |
| Short-term investments | $ 218,809 | $ 314,691 |
| Long-term investments | $ 10,077 | $ 14,153 |

Since inception, we have funded our operations primarily through the issuance of our equity securities and payments from Sanofi pursuant to the Collaboration Agreement, which was terminated in December 2018. All our investments are in investment-grade securities.

On January 3, 2020, we entered into a sales agreement with a sales agent to establish an at-the-market (ATM) offering program, under which we are permitted to offer and sell, from time to time, shares of common stock having a maximum aggregate offering price of up to $200 million. As of March 31, 2020, no securities have been issued pursuant to the ATM agreement.

In March 2019, we completed a follow-on offering in which we issued 5,663,750 shares of our common stock at a price of $51.00 per share, including 738,750 shares sold directly to the underwriters upon exercise of their option to purchase up to 738,750 shares of our common stock within 30 days of the offering. We received proceeds totaling approximately $271.2 million from the offering, net of underwriting discounts, commissions and offering expenses.

We believe we have sufficient financial resources to meet our business requirements for the 12 months following the date of this Quarterly Report on Form 10-Q. We expect to incur substantial expenditures in the foreseeable future for the advancement of our precision medicine platform, the development and potential commercialization of our product candidates and the discovery, development and potential commercialization of any additional product candidates we may pursue. Furthermore, if our clinical trials for mavacamten are successful, or our other product candidates, including danicamtiv, enter into late-stage clinical trials or more advanced discovery and development stages, we may need to raise additional capital in order to further advance our product candidates towards regulatory approval. We will continue to seek 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. If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Any 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 other 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):

| | Three Months Ended March 31, | |
|---|---|---|
| | 2020 | 2019 |
| Net cash (used in) provided by: | | |
| Operating activities | $ (59,897) | $ (38,046) |
| Investing activities | 87,848 | (13,510) |
| Financing activities | 1,817 | 271,765 |
| Net (decrease) increase in cash, cash equivalents and restricted cash | $ 29,768 | $ 220,209 |

*Operating Activities*

Net cash used in operating activities for the three months ended March 31, 2020 was $59.9 million and was primarily due to the net loss for the period of $69.9 million, adjusted for non-cash stock-based compensation expense of $10.8 million and depreciation of $0.8 million, offset by amortization of discount on investment of $0.4 million. Changes in working capital primarily consisted of decreases of $1.5 million in accrued liabilities and $0.5 million in accounts payable, offset by a decrease of $0.2 million in prepaid expenses.

Net cash used in operating activities for the three months ended March 31, 2019 was $38.0 million and was primarily due to the net loss for the period of $37.5 million, adjusted for non-cash stock-based compensation expense of $7.0 million and depreciation of $0.5 million, offset by amortization of discount on investment of $0.3 million. Changes in working capital primarily consisted of a $9.9 million decrease in prepayments from our collaboration partner, a $2.0 million increase in accounts payable and a $0.7 million decrease in other long-term liabilities.

*Investing Activities*

Cash provided by investing activities for the three months ended March 31, 2020 was $87.9 million and consisted primarily of sales and maturities of investments of $4.0 million and $96.5 million, respectively, offset by cash outflows of $12.6 million for leasehold improvements and purchases of equipment related to the occupancy of our new corporate headquarters in Brisbane, California in January 2020.

Cash used in investing activities for the three months ended March 31, 2019 was $13.5 million and consisted primarily of purchases of investments of $32.7 million and purchases of equipment of $0.8 million, offset by sales and maturities of investments of $4.0 million and $16.0 million, respectively.

*Financing Activities*

Cash provided by financing activities for the three months ended March 31, 2020 was $1.8 million and consisted of proceeds from common stock option exercises.

Cash provided by financing activities for the three months ended March 31, 2019 was $271.8 million and consisted of proceeds from the issuance of common stock in connection with a follow-on offering of $271.5 million, net of underwriter's discount and funds received as a result of common stock option exercises of $0.3 million.

**Contractual Obligations and Commitments**

There have been no material changes to our contractual obligations during the three months ended March 31, 2020, as compared to those disclosed in our Annual Report.

**Off-Balance Sheet Arrangements**

Since our inception, we have not engaged in any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

## 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 March 31, 2020, we had cash, cash equivalents and investments (short-term and long-term) of $360.1 million, consisting of interest-bearing money market accounts and money market funds, which would be affected by changes in the general level of United States interest rates. However, due to the short-term maturities of our cash and cash equivalents and 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, cash equivalents or investments.

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 Securities Exchange Act of 1934, as amended (the Exchange Act), refers to controls and procedures that are designed to provide reasonable assurance 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 provide reasonable assurance 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 Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2020, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and Principal Financial and Accounting Officer 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 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 serious diseases of systolic or diastolic dysfunction 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 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 the underlying cause of 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, which has been solely on precision medicine for the development of drugs for diseases of cardiac muscle contraction 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, danicamtiv and MYK-224, our initial product candidates. Other than mavacamten, danicamtiv and MYK-224, 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 and MYK-224 for the treatment of hypertrophic cardiomyopathy (HCM) and danicamtiv for the treatment of dilated cardiomyopathy (DCM). We are currently evaluating mavacamten, danicamtiv and MYK-224 in 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 years, if ever, will depend heavily on the successful development and eventual commercialization of mavacamten, danicamtiv, MYK-224 or other product candidates that we may identify from our precision medicine platform.

The success of mavacamten, danicamtiv, MYK-224 and any other product candidates that we discover and develop will depend on many factors, including the following:

- timely and successful initiation of, enrollment in, and completion of, clinical trials, including our Phase 3 clinical trial of mavacamten in HCM, our Phase 2 clinical trial of danicamtiv in genetic DCM, our Phase 1 clinical trial of MYK-224 and any additional clinical trials of these product candidates;
- achieving positive safety and efficacy data and desirable medicinal properties for our product candidates for the intended indications;
- our ability to receive, and the timing of our receipt of, any marketing approvals from applicable regulatory authorities;
- establishing and maintaining manufacturing capabilities or making arrangements with third-party manufacturers for the manufacture of our product candidates for clinical trials and, if approved, for commercialization;
- 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; and
- enforcing and defending intellectual property rights and claims.

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 from our Phase 1 and Phase 2 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 placebo controlled clinical trials of mavacamten in larger populations using the current dosing strategy, inclusion/exclusion criteria, and endpoints of EXPLORER-HCM. 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. The initial clinical data from our Phase 1 and Phase 2 clinical trials of mavacamten, as well as our Phase 1 and 2 clinical trials of danicamtiv, are preliminary in nature, and the clinical development of mavacamten and danicamtiv 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, 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 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, and, if applicable under any collaboration or similar agreement, 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 restrictions on use or distribution of the drug;
- require safety warnings in the label and/or require risk management plan post-approval;
- 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 product candidates 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 are evaluating or plan to evaluate our product candidates are rare genetic disorders or involve segmented patient populations 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 cardiovascular diseases based on our precision medicine approach. A key element of our initial 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.

***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 adverse events in our clinical trials of mavacamten. 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 find deficiencies with or 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, danicamtiv 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.

***Although the FDA provided feedback on the Phase 3 clinical trial for mavacamten, the FDA may raise questions regarding mavacamten's safety data, which may delay or prevent the approval of mavacamten or adversely affect our ability to commercialize mavacamten on the timelines we have announced.***

The size of a safety database when submitting an NDA is usually between 3,000 to 5,000 patients. When we sought feedback on EXPLORER-HCM, our Phase 3 clinical trial for mavacamten, we estimated that at the time of submitting our NDA, our safety database would have 250 patient-years of exposure, as we are treating an orphan disease. When we submit our NDA, we anticipate that we will have approximately 200 patient-years in the safety database and at the time of submission of the 120-day safety update, it will have increased to 300-350 patient-years. While we plan to conduct a prospective registry study to demonstrate our commitment to post-approval product characterization, and the FDA has accepted a lesser number of patients for a safety database in the past, the FDA may request additional clinical data, which may delay or prevent the approval and commercialization of mavacamten and could have a negative impact on our business.

***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 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 any 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 to expedite the review and approval process for our product candidates, including Breakthrough Therapy Designation or Fast Track 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 to expedite the review and approval process for our product candidates, including Breakthrough Therapy Designation or Fast Track 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.

If we elect to pursue Breakthrough Therapy Designation or Fast Track Designation, any inability to secure or maintain the applicable designation would have an adverse impact on our development timelines and our ability to obtain approval for and commercialize our product candidates.

**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, completing our ongoing clinical trials for our most advanced product candidate, mavacamten, planning further clinical development of mavacamten and completing our ongoing clinical development of our second product candidate, danicamtiv, and beginning clinical development of our third product candidate, MYK-224. 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 support the registration 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 and development 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 anticipate that we will continue to incur losses for the near future 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, danicamtiv and MYK-224, are in various stages of clinical testing and we must successfully complete our ongoing clinical trials of mavacamten and conduct significant additional clinical trials for danicamtiv and MYK-224 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 selling, general and administrative costs associated with our operations. Our net loss for the quarter ended March 31, 2020 was $69.9 million and as of March 31, 2020, we had an accumulated deficit of $548.6 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 in the near 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, 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 (FDA), the European Medicines Agency (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, danicamtiv and MYK-224, 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 continue to advance our product candidates in clinical trials and identify additional product candidates from our pipeline for clinical development.

As of March 31, 2020, our cash, cash equivalents and investments (short-term and long-term) totaled $360.1 million. We intend to use our cash, cash equivalents and investments to fund the advancement of our mavacamten clinical development program, including our ongoing Phase 3 clinical trial in symptomatic obstructive HCM patients, and our planned additional clinical trials of mavacamten and danicamtiv, our ongoing Phase 1 trial of MYK-224, 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, including the effects of the COVID-19 pandemic on our research and development activities, 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, danicamtiv, MYK-224 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, danicamtiv and MYK-224;
- 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 commercial scale, and to manufacture danicamtiv and MYK-224 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, if any;
- 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 and maintain 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 United States.***

We prepare our financial statements in conformity with accounting principles generally accepted in the United States. These accounting principles are subject to interpretation by the Financial Accounting Standards Board and the Securities and Exchange Commission (SEC). 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.

***Comprehensive tax reform bills could increase the tax burden on our orphan drug programs and adversely affect our business and financial condition.***

In December 2017, the U.S. government enacted comprehensive tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, (i) a permanent reduction to the corporate income tax rate, (ii) a partial limitation on the deductibility of business interest expense, (iii) a shift of the U.S. taxation of multinational corporations from a tax on worldwide income to a generally more territorial focused system (along with certain rules designed to prevent erosion of the U.S. income tax base) and (iv) a one-time tax on accumulated offshore earnings held in cash and illiquid assets, with the latter taxed at a lower rate.

Further, the recently enacted comprehensive tax legislation, among other things, reduces the orphan drug tax credit from 50% to 25% of qualifying expenditures. When and if we become profitable, this reduction in tax credits may result in an increased federal income tax burden on our orphan drug programs as it may cause us to pay federal income taxes earlier under the revised tax law than under the prior law and, despite being partially off-set by a reduction in the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, may increase our total federal tax liability attributable to such programs.

Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to this federal tax law.

***We may choose not to develop a potential drug candidate, or we may suspend, deprioritize or terminate one or more discovery programs or pre-clinical drug candidates or programs.***

At any time and for any reason, we may determine that one or more of our discovery programs or preclinical drug candidates or programs does not have sufficient potential to warrant the allocation of resources toward such program or drug candidate. Accordingly, we may choose not to develop a potential drug candidate or elect to suspend, deprioritize or terminate one or more of our discovery programs or preclinical drug candidates or programs. If we suspend, deprioritize or terminate a program or drug candidate in which we have invested significant resources, we will have expended resources on a program that will not provide a full return on our investment and may have missed the opportunity to have allocated those resources to potentially more productive uses, including existing or future programs or drug candidates.

**Risks Related to Our Reliance on Third Parties**

***From the inception of our collaboration arrangement with Sanofi in August of 2014 and through December 31, 2018 we were substantially dependent upon Sanofi for the development and eventual commercialization of mavacamten, danicamtiv, and MYK-224. As a result of the termination of the arrangement, we may be unable to commercialize certain product candidates.***

We have previously depended upon our license and collaboration agreement with Aventis Inc., a wholly-owned subsidiary of Sanofi S.A. (Sanofi), 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-224, danicamtiv and HCM-2 programs and for the manufacturing of danicamtiv. On December 31, 2018, Sanofi notified us of their intent to terminate the collaboration and as a result, reimbursement for our research and development collaboration on mavacamten and MYK-224 ended in the first half of 2019. In addition, Sanofi did not elect to continue with the danicamtiv and HCM-2 programs, and the collaboration with respect to such programs was deemed terminated as of December 31, 2018. As a result of the termination, the development of our product candidates previously subject to the Collaboration Agreement could be significantly delayed and may have a material adverse effect on our results of operations and financial condition.

***We may enter into collaborations that place the development and commercialization of our product candidates outside our control, require us to relinquish important rights or may otherwise be on terms unfavorable to us, and if our collaborations are not successful, our product candidates may not reach their full market potential.***

Our drug development programs and the potential commercialization of our drug candidates will require substantial additional cash to fund expenses. For some of our drug candidates, we may decide to collaborate with additional pharmaceutical and biotechnology companies for the development and potential commercialization of those drug candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject drug candidate, the costs and complexities of manufacturing and delivering such drug candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative drug candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our drug candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms or at all. If we are unable to do so, we may have to curtail the development of the drug candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our drug candidates or bring them to market and generate drug revenue. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable drug candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party.

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

We currently rely on third parties to conduct our clinical trials and expect to continue to rely on third parties with respect to some or all aspects of our clinical trials, protocol development and research and development activities.

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 Investigational New Drug Application (IND) submissions and approval of our product candidates. Any of these events could lead to clinical trial 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, nor do we plan to acquire, 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 may obtain marketing approval. To date, we have obtained materials for mavacamten for our clinical trials from third-party manufacturers, and we intend to rely on third-party manufacturers for our planned Phase 2 and Phase 3 clinical development activities for mavacamten and for our Phase 2 and any subsequent clinical trials of danicamtiv. Due to the Sanofi Collaboration Agreement termination on December 31, 2018, we no longer rely on Sanofi for our danicamtiv supplies and have executed service agreements with other third-party manufacturers for the manufacturing of danicamtiv.

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 or maintain 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 or our marketing applications will not be approved. 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, by pursuing the grant of patents from those applications around the world, and by taking steps to defend those patents if challenged by third parties. It is not uncommon in the pharmaceutical industry for patents covering successful drugs to be challenged for invalidity by third parties before or after the grant of such patents by a patent office (e.g., by a pre- or post-grant proceeding in a patent office or a court action). Currently we own five issued U.S. patents, several foreign patents and multiple pending applications worldwide that relate to 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 on or due to the public disclosures of others or ourselves. 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 scientific insights, screening assays and manufacturing processes, 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 acquired within the industry through independent development, the publication of journal articles describing methodologies and insights, and the movement of personnel skilled in the art into the pharmaceutical and biotechnology industry.

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, and discovery processes to aid in proving trade secret misappropriation may be limited in many foreign countries. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we may 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 could 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 court actions for patent infringement or nullification, pre- and post-grant proceedings before the U.S. Patent and Trademark Office (USPTO), and corresponding proceedings in 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 or delay our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Parties making claims against us may also obtain injunctive or other equitable relief, which could effectively block or delay our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, could involve substantial litigation expense and could be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may be required to take a number of steps, including but not limited to, paying substantial damages, including treble damages and attorneys' fees for willful infringement, paying lost profits or royalties, redesigning our infringing products or manufacturing process, obtaining one or more licenses from third parties for activities going forward, 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 for many reasons, including but not limited to, a determination 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 or collaborators. An adverse result in any litigation or patent office proceeding could put one or more of our patents at risk, for example, of being invalidated, deemed unenforceable or interpreted narrowly or could put our patent applications at risk of not issuing.

An unfavorable outcome could require us to cease using a 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 our patents and patent applications 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, these perceptions 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 third parties, including but not limited to, former employees and collaborators, 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 the exclusive ownership of, or the 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 or arbitration 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 or the use or manufacture thereof, 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 utility, written description, novelty, non-obviousness or enablement. Additionally, in the United States, a patent can be deemed unenforceable if someone connected with the prosecution of a patent application intentionally withheld materially relevant information from the USPTO, or intentionally mislead the USPTO during prosecution. Third-party challenges to the validity and/or enforceability of a patent can occur in courts in the United States or abroad, or in pre- or post-grant proceedings in some foreign patent offices (e.g., but not limited to re-examination, post grant review, inter parties review, or opposition proceedings). Such proceedings could result in the revocation of, 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 of one of our patents for a product candidate, this could substantially affect our ability to protect that product candidate in the country in which the patent was issued. Such a loss of patent protection could 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 and suffering reputational harm, 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.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with any products that we may develop and commercialize, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology and pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from these intellectual property rights.

**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 cardiac muscle contraction and our targeted indications affect relatively small populations. 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.

***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, danicamtiv or any of our other product candidates, if approved, could significantly harm our business, prospects, financial condition and results of operations.

***We are currently establishing a sales and marketing organization; however, if we are unable to enter into agreements with third parties to sell and market our drug candidates, we may not be successful in commercializing our drug candidates if and when they are approved, and we may not be able to generate any revenue.***

We have limited experience in the sale, marketing or distribution of drugs. To achieve commercial success for any approved drug candidate for which we retain sales and marketing responsibilities, we must build our sales, marketing, managerial, and other non-technical capabilities or make arrangements with third parties to perform these services. In the future, we may choose to build a focused sales and marketing infrastructure to sell, or participate in sales activities with our collaborators for some of our drug candidates if and when they are approved.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time-consuming and could delay any drug launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost.

Factors that may inhibit our efforts to commercialize our drug candidates on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;

- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our drug revenues or the profitability of these drug revenues to us are likely to be lower than if we were to market and sell any drug candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our drug candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drug candidates effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug candidates. Further, our business, results of operations, financial condition and prospects will be materially adversely affected.

***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.

***While we have received orphan drug designation for our most advanced drug candidate, mavacamten, for the treatment of symptomatic obstructive HCM, we may seek orphan drug designation for some of our other drug candidates. However, we may be***

***unsuccessful in obtaining or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.***

The FDA has granted orphan drug designation to mavacamten for the treatment of symptomatic obstructive HCM. As part of our business strategy, we may seek orphan drug designation for some of our other drug candidates, and we may be unsuccessful. Regulatory authorities in some jurisdictions, including the United States, 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 financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers.

Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. The applicable period is seven years in the United States. Even if we obtain orphan drug exclusivity for a drug, that exclusivity may not effectively protect the designated drug from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug 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. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States 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 quantity of the drug to meet the needs of patients with the rare disease or condition. 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. While we might seek orphan drug designation for our other drug candidates in addition to mavacamten for the treatment of symptomatic oHCM, we may never receive such designations. Even if we receive orphan drug designation for any of our drug candidates, there is no guarantee that we will enjoy the benefits of those designations.

***If we participate in and then fail to comply with our reporting and payment obligations under governmental pricing programs in the United States, we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business financial condition, results of operations and growth prospects.***

With the approval of any product candidate, we anticipate that we may participate in a number of federal and state government pricing programs in the United States in order to obtain coverage for the product by certain government healthcare programs. These programs would generally require us to pay rebates or provide discounts to certain private purchasers or government payers in connection with our products when dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. We may also have reimbursement obligations or be subject to penalties if we fail to provide timely and accurate information to the government, pay the correct rebates or offer the correct discounted pricing. Changes to the price reporting or rebate requirements of these programs would affect our obligations to pay rebates or offer discounts. Responding to current and future changes may increase our costs and the complexity of compliance, will be time-consuming, and could have a material adverse effect on our results of operations.

**Risks Related to Our Business and Industry**

***The outbreak of the novel strain of coronavirus, SARS-CoV-2, which causes COVID-19, could adversely impact our business.***

The outbreak of the novel strain of coronavirus, SARS-CoV-2, which causes coronavirus disease 2019 (COVID-19) has evolved into a global pandemic. In response to the spread of COVID-19, we have closed our executive offices with our administrative employees continuing their work outside of our offices, and restricted on-site staff to only those required to maintain the facilities and equipment.

As a result of the COVID-19 outbreak, or similar pandemics, we have and may in the future experience disruptions that could severely impact our business, research and clinical development activities, including:

- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;

- delays or disruptions in non-clinical studies due to the inability of our research and development personnel to perform their regular duties or unforeseen circumstances at contract research organizations and vendors along their supply chain;

- increased rates of patients withdrawing from our clinical trials following enrollment as a result of contracting COVID-19, being forced to quarantine, or not accepting home health visits;

- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;

- interruption of key clinical trial activities, such as clinical trial site data monitoring and site inspections, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures (particularly any procedures that may be deemed non-essential), which may impact the integrity of subject data and clinical trial endpoints;

- interruption or delays in the operations of the U.S. Food and Drug Administration and comparable foreign regulatory agencies, which may impact review, inspection and approval timelines;

- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems; and

- limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, including because of sickness of employees or their families, the desire of employees to avoid contact with large groups of people, an increased reliance on working from home or mass transit disruptions.

In addition, the trading prices for our common stock and other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through equity or debt financings, or such financing transactions may be on unfavorable terms.

The COVID-19 outbreak continues to rapidly evolve, and it is unknown how long disruptions to our research, clinical development and other business operations resulting from the COVID-19 pandemic, including any disruptions relating to the ultimate geographic spread of the disease, the duration of the outbreak, travel restrictions and actions by businesses and governmental authorities to contain the outbreak, such as quarantines or “stay at home” orders and business closures, will continue. However, any prolonged disruption could have a material adverse impact our business, financial condition and results of operations, and we will continue to monitor the situation closely.

To the extent the COVID-19 pandemic adversely affects our business and financial results, it may also have the effect of heightening many of the other risks described in this ‘‘Risk Factors’’ section, such as those relating to our clinical development operations, the supply chain for our ongoing and planned clinical trials, and our need to raise additional capital to support our operations.

***We may be subject to healthcare, health information privacy and security 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 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 other healthcare market participants play a primary role in the recommendation and prescription of any product 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. There are ambiguities as to what is required to comply with these requirements, and if we fail to comply with any applicable federal, state or foreign legal requirement, we could be subject to penalties.

Regulators globally are also imposing greater monetary fines for privacy violations. For example, in 2016, the European Union adopted a new regulation governing data practices and privacy called the General Data Protection Regulation (GDPR) which became effective on May 25, 2018. The GDPR applies to any company established in the European Union as well as to those outside the European Union if they collect and use personal data in connection with the offering goods or services to individuals in the European Union or the monitoring of their behavior. The GDPR enhances data protection obligations for processors and controllers of personal data, including, for example, special protections for “sensitive information” such as health and genetic information, expanded disclosures about how personal information is to be used, limitations on retention of information, mandatory data breach notification

requirements and onerous new obligations on service providers. Non-compliance with the GDPR may result in monetary penalties of up to €20 million or 4% of worldwide revenue, whichever is higher. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of personal data, such as healthcare data or other sensitive information, could greatly increase our cost of developing or commercializing our product candidates or impair our ability to collect data from patients resident in the European Union, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, including as implemented by individual countries.

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, if any, 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.

In the field of heart failure drug development, our principal competitors include Amgen Inc., AstraZeneca plc, Bayer AG, Bristol-Myers Squibb Company, C.H. Boehringer Sohn AG & Co. KG, Eli Lilly and Company, Novartis AG and Takeda Pharmaceutical Company Limited. Specific to our initial drug discovery and development focus areas, we believe that Cytokinetics, Inc., Takeda Pharmaceutical Company Limited and Novartis AG have ongoing programs in HCM and that Novartis AG, Pfizer Inc., Tenaya Therapeutics, Berlin Cures, and Zensun (Shanghai) Sci. & Tech. Co., Ltd. have ongoing programs in DCM. Additionally, there may be other companies pursuing therapeutic candidates from which we face current or future competition.

***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 changes may have a material adverse effect on our business and results of operations.***

In the United States, the European Union 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, the Patient Protection and Affordable Care Act (ACA) changed the way healthcare is financed by both governmental and private insurers and significantly impacted the U.S. pharmaceutical and biotechnology industries. Since its enactment, there have been many judicial, Presidential, and Congressional challenges to numerous aspects of the ACA, and the long ranging effects of these challenges on reimbursement by third-party payors, the viability of the ACA marketplace, providers, and potentially, our business are unknown at this time. In addition, the full impact of the ACA, any law repealing and/or replacing elements of it, and the political uncertainty surrounding any repeal or replacement legislation on our business remains unclear.

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.

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 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.

***Our future success depends on our ability to retain our key executives, employees and consultants and to attract, retain and motivate qualified personnel.***

We are highly dependent on our scientific advisors 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 March 31, 2020, we had 246 full-time employees. As we mature, we expect to expand our full-time employee base and to hire more consultants and contractors. Over the next several years, we expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations. 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.

***Inadequate funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including most recently from December 22, 2018 to January 25, 2019, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

***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 two wholly-owned subsidiaries: an Australian subsidiary through which we conduct clinical trials in Australia and a Dutch subsidiary through which we intend to determine the feasibility of commercialization in the EU. Our business strategy also contemplates potential additional international operations as we seek to continue the development of mavacamten, danicamtiv, MYK-224 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 (such as the outbreak of the novel strain of coronavirus in December 2019);
- 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 due to high levels of unemployment (particularly as a result of the COVID-19 pandemic), underemployment or the potential repeal of certain provisions of the ACA, may decrease the demand for healthcare services and pharmaceuticals. Additionally, the availability of healthcare services and resources is currently constrained due to the COVID-19 pandemic. If fewer patients are seeking medical care because they do not have insurance coverage or are unable to obtain medical care for their conditions due to resource constraints on the healthcare system, 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, including as a result of the COVID-19 pandemic, 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 COVID-19 pandemic, 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, outbreak of disease or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Earthquakes, outbreak of disease, 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. For example, our corporate headquarters is located in the San Francisco Bay Area, which in the past has experienced severe earthquakes. If a natural disaster, power outage, outbreak of disease, 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. Due to the effects of the COVID-19 pandemic, we have temporarily suspended the enrollment of new patients into our Phase 1 study of MYK-224 and the enrollment of existing patients from EXPLORER-HCM rolling over into the MAVA-LTE study. In addition, we have delayed the initiation of our Phase 3 VALOR-HCM study, Phase 2 HFpEF proof-of-concept study, and our Phase 2 study of danicamtiv in patients with genetic DCM. We will continue to closely monitor the evolving situation and expect to resume patient enrollment and to initiate delayed studies as soon as conditions safely permit. In addition, we may experience delays in the supply of drug product for our clinical trials as a result of disruptions to the operations of manufacturing facilities of some of our third-party contract manufacturers due to the COVID-19 pandemic. Any continued or subsequent measures taken by governmental authorities or businesses to contain the spread of COVID-19, or the perception that such measures may be required in the future should another outbreak occur, could adversely affect our business, financial condition or results of operations by limiting our contract manufacturers' ability to manufacture product and forcing temporary closure of facilities that we rely upon. The extent to which COVID-19 impacts our results will depend on future developments, which are highly uncertain and cannot be accurately predicted, including new information which may emerge concerning the severity of COVID-19 and the actions to contain COVID-19 or treat its impact, among others. 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 study participants or patients;
- loss of revenue; and
- the inability to commercialize mavacamten, danicamtiv, MYK-224 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% 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 and in August 14, 2017 due to a subsequent stock offering, we do not believe that these ownership changes 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.

In addition, under the Tax Act, the amount of post-2017 NOLs that we are permitted to deduct in any taxable year is limited to 80% of our taxable income in such year, where taxable income is determined without regard to the NOL deduction itself. The Tax Act generally eliminates the ability to carry back any NOL to prior taxable years, while allowing post-2017 unused NOLs to be carried forward indefinitely. There is a risk that due to changes under the Tax Act, regulatory changes or other unforeseen reasons, our existing NOLs could expire or otherwise be unavailable to offset future income tax liabilities.

***The United Kingdom's withdrawal from the EU may have a negative effect on our business, global economic conditions, and financial markets.***

As a result of the United Kingdom's vote to leave the EU in March 2019 (known as Brexit), the EMA relocated its headquarters from London to Amsterdam. Since a significant proportion of the regulatory framework in the United Kingdom is derived from EU directives and regulations, Brexit could materially impact the regulatory regime with respect to the approval of product candidates, disrupt the manufacture of our products and product candidates in the United Kingdom or the EU, disrupt the import and export of active substances and other components of drug formulations, and disrupt the supply chain for clinical trial product and final authorized formulations. While negotiations continue regarding the terms of the United Kingdom's withdrawal from the EU, the specific impact to the supervision, regulation and supply of medicines in the United Kingdom and Europe remain unclear. The cumulative effect of disruptions to the regulatory framework or supply chains may add considerably to the development lead time to, and expense of, marketing authorization and commercialization of products in the EU and/or the United Kingdom. In view of the uncertainty surrounding the Brexit implementation, we are unable to predict the effects of such disruption to the regulatory framework and supply chain in Europe.

**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;
- failure to develop successfully and commercialize our product candidates;
- 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 by us or our licensors and strategic collaborators, if any, 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, including the effects of the COVID-19 pandemic on the global economy, 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. A substantial number of our outstanding shares of common stock are held by a relatively small number of stockholders who are not subject to restrictions on trading. 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. 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, beginning on January 1, 2017 and ending on January 1, 2025, the number of shares available for future issuance under our 2015 Employee Stock Purchase Plan (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 April 22, 2020, our executive officers, directors, 5% or greater stockholders and their affiliates beneficially own approximately 50.8% 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 will continue to incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to existing and new public company compliance and reporting regulations.***

As a public company, we incur and will continue to incur significant legal, accounting and other expenses. 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 have devoted and will continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased and will continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

Pursuant to Section 404 of the Sarbanes-Oxley Act, we are required to furnish a report by our management on our internal control over financial reporting with our Annual Report on Form 10-K for each fiscal year and 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 are and will continue to 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 adhere to 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.

In addition, as a public company we are required to file accurate and timely quarterly, annual and current reports with the SEC under the Exchange Act. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from The Nasdaq Global Select Market or other adverse consequences that would materially harm our business.

***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 clinical development programs, our precision medicine platform or our preclinical 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 and our ability to successfully commercialize any approved product;
- 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 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 | |
| 10.1 | Common Stock Sales Agreement, by and between the Registrant and Cowen and Company, LLC dated January 3, 2020. | 8-K | 1/03/2020 | 1.1 | |
| 10.2# | Amended and Restated 2015 Employee Stock Purchase Plan. | | | | X |
| 10.3# | Amended and Restated Non-Employee Director Compensation Policy. | | | | X |
| 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.SCH | Inline XBRL Taxonomy Extension Schema Document. | | | | X |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Linkbase Document. | | | | X |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document. | | | | X |
| 101.LAB | Inline XBRL Taxonomy Extension Label Linkbase Document. | | | | X |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Linkbase Document. | | | | X |
| 104 | Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101.) | | | | 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.

\# Represents management compensation plan, contract or arrangement.

**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: May 6, 2020

**MYOKARDIA, INC.**

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

Date May 6, 2020

By: /s/ Taylor Harris
Taylor Harris
*Chief Financial Officer*
*(Principal Financial and Accounting Officer)*

# MYOKARDIA, INC.

# AMENDED AND RESTATED 2015 EMPLOYEE STOCK PURCHASE PLAN

The purpose of the MyoKardia, Inc. Amended and Restated 2015 Employee Stock Purchase Plan ("the Plan") is to provide eligible employees of MyoKardia, Inc. (the "Company") and each Designated Subsidiary (as defined in Section 11) with opportunities to purchase shares of the Company's common stock, par value $0.0001 per share (the "Common Stock"). 255,000 shares of Common Stock in the aggregate have been approved and reserved for this purpose, plus on January 1, 2017, and each January 1 thereafter through January 1, 2025, the number of shares of Common Stock reserved and available for issuance under the Plan shall be cumulatively increased by the lesser of (i) 3,000,000 shares of Common Stock, (ii) one percent (1%) of the number of shares of Common Stock issued and outstanding on the immediately preceding December 31st, or (iii) such lesser number of shares of Common Stock as determined by the Administrator. The Company intends for the Plan to have two components: a Code Section 423 component (the "423 Component") and a non-Code Section 423 component (the "Non-423 Component"). The Company's intention is to have the 423 Component of the Plan qualify as an "employee stock purchase plan" within the meaning of Section 423(b) of the Internal Revenue Code of 1986, as amended (the "Code"), and shall be interpreted in accordance with that intent. In addition, this Plan authorizes the grant of an Option (as defined in Section 8) to purchase shares of Common Stock under the Non-423 Component that does not qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code; such an Option will be granted pursuant to the rules, procedures or subplans adopted by the Administrator (as defined in Section 1) designed to achieve tax, securities laws or other objectives for eligible employees and

the Company. Except as otherwise provided herein, the Non-423 Component will operate and be administered in the same manner as the 423 Component.

1. Administration. The Plan will be administered by the person or persons (the "Administrator") appointed by the Company's Board of Directors (the "Board") for such purpose. The Administrator has authority at any time to: (i) adopt, alter and repeal such rules, subplans, guidelines and practices for the administration and operation of the Plan and for its own acts and proceedings as it shall deem advisable, including to accommodate the specific requirements of local laws, regulations and procedures for jurisdictions outside of the United States; (ii) interpret the terms and provisions of the Plan; (iii) make all determinations it deems advisable for the administration of the Plan; (iv) decide all disputes arising in connection with the Plan; (v) designate Subsidiaries (as defined in Section 11) as participating in the 423 Component or Non-423 Component; and (vi) otherwise supervise the administration of the Plan. Unless otherwise determined by the Administrator, the employees eligible to participate in each subplan will participate in a separate Offering (as defined in Section 2) or in the Non-423 Component. Without limiting the generality of the foregoing, the Administrator is specifically authorized to adopt rules and procedures regarding eligibility to participate, the definition of Compensation (as defined in Section 11), handling of contributions, making of contributions to the Plan (including, without limitation, in forms other than payroll deductions), establishment of bank or trust accounts to hold contributions, payment of interest, conversion of local currency, obligations to pay payroll tax, determination of beneficiary designation requirements, withholding procedures and handling of stock certificates that vary with applicable local requirements. All interpretations and decisions of the Administrator shall be binding on all persons, including the Company and the Participants (as defined in Section 11). No member of

the Board or individual exercising administrative authority with respect to the Plan shall be liable for any action or determination made in good faith with respect to the Plan or any option granted hereunder.

2. Offerings. The Company will make one or more offerings to eligible employees to purchase Common Stock under the Plan ("Offerings") consisting of one or more Purchase Periods (as defined in Section 11) . Unless otherwise determined by the Administrator, an Offering will be 12 months long and will begin on the first business day occurring on or after each May 1 and November 1 and will end on the last business day occurring on or before the immediately following April 30 and October 31, respectively. The Administrator may, in its discretion, designate a different period for any Offering, provided that no Offering shall exceed 27 months in duration. Unless the Administrator determines otherwise, each Offering will be divided into two equal six-month Purchase Periods. Furthermore, unless as otherwise determined by the Administrator, Participants will only be permitted to participate in one Offering at a time.

3. Eligibility. All individuals classified as employees on the payroll records of the Company and each Designated Subsidiary are eligible to participate in any one or more of the Offerings under the Plan (provided, that the Participant is not permitted to participate in multiple Offerings at the same time, unless as otherwise determined by the Administrator), provided that as of the first day of the applicable Offering (the "Offering Date") they are customarily employed by the Company or a Designated Subsidiary for more than 20 hours a week and have completed at least 30 days of employment, or such other period as determined by the Administrator, unless, with respect to the Non-423 Component, the exclusion of employees who do not meet this requirement is not permissible under applicable law. Notwithstanding any other

provision herein, individuals who are not contemporaneously classified as employees of the Company or a Designated Subsidiary for purposes of the Company's or applicable Designated Subsidiary's payroll system are not considered to be eligible employees of the Company or any Designated Subsidiary and shall not be eligible to participate in the Plan. In the event any such individuals are reclassified as employees of the Company or a Designated Subsidiary for any purpose, including, without limitation, common law or statutory employees, by any action of any third party, including, without limitation, any government agency, or as a result of any private lawsuit, action or administrative proceeding, such individuals shall, notwithstanding such reclassification, remain ineligible for participation. Notwithstanding the foregoing, the exclusive means for individuals who are not contemporaneously classified as employees of the Company or a Designated Subsidiary on the Company's or Designated Subsidiary's payroll system to become eligible to participate in this Plan is through the adoption of subplan to this Plan, which specifically renders such individuals eligible to participate herein.

4. Participation.

(a) Participants in Offerings. An eligible employee who is not a Participant in any prior Offering may participate in a subsequent Offering by submitting an enrollment form to his or her appropriate payroll location at least 15 business days before the Offering Date (or by such other deadline as shall be established by the Administrator for the Offering).

(b) Enrollment. The enrollment form will (i) state a whole percentage or the amount to be deducted from an eligible employee's Compensation per pay period, (ii) authorize the purchase of Common Stock in each Offering in accordance with the terms of the Plan and (iii) specify the exact name or names in which shares of Common Stock purchased for such individual are to be issued pursuant to Section 10. An employee who does not enroll in

accordance with these procedures will be deemed to have waived the right to participate. Unless a Participant submits a new enrollment form or withdraws from the Plan, such Participant's deductions and purchases will continue at the same percentage or amount of Compensation for future Offerings, provided he or she remains eligible. Notwithstanding the foregoing and with respect to the 423 Component, participation in the Plan will neither be permitted nor be denied contrary to the requirements of the Code.

5. Employee Contributions. Each eligible employee may authorize payroll deductions at a minimum of 1 percent up to a maximum of 10 percent of such employee's Compensation for each pay period. The Company will maintain book accounts showing the amount of payroll deductions made by each Participant for each Purchase Period within an Offering. No interest will accrue or be paid on payroll deductions, except as may be required by applicable law. If payroll deductions for purposes of the Plan are prohibited or otherwise problematic under applicable law (as determined by the Administrator in its discretion), the Administrator may permit the participants to contribute to the Plan by such other means as determined by the Administrator. Any reference to "payroll deductions" in this section (or in any other section of the Plan) shall similarly cover contributions by other means made pursuant to this Section 5.

6. Deduction Changes. Except as may be determined by the Administrator in advance of an Offering, a Participant may not increase or decrease his or her payroll deduction during any Offering, but may increase or decrease his or her payroll deduction with respect to the next Offering (subject to the limitations of Section 5) by submitting a new enrollment form at least 15 business days before the next Offering Date (or by such other deadline as shall be established by the Administrator for the Offering). The Administrator may, in advance of any

Offering, establish rules permitting a Participant to increase, decrease or terminate his or her payroll deduction during an Offering.

7. Withdrawal. A Participant may withdraw from participation in the Plan by submitting a written notice of withdrawal to his or her appropriate payroll location. The Participant's withdrawal will be effective as of the next business day. Following a Participant's withdrawal, the Company will promptly refund such individual's entire account balance under the Plan to him or her (after payment for any Common Stock purchased before the effective date of withdrawal). Partial withdrawals are not permitted. Such an employee may not begin participation again during the remainder of the Offering, but may enroll in a subsequent Offering in accordance with Section 4, unless otherwise determined by the Administrator.

8. Grant of Options. On each Offering Date, the Company will grant to each eligible employee who is then a Participant in the Plan an option ("Option") to purchase on the last day of a Purchase Period (an "Exercise Date"), at the Option Price hereinafter provided for, the lowest of (a) a number of shares of Common Stock determined by dividing such Participant's accumulated payroll deductions on such Exercise Date by the Option Price (as defined herein), (b) two thousand five hundred (2,500) shares; or (c) such other lesser maximum number of shares as shall have been established by the Administrator in advance of the Offering; provided, however, that such Option shall be subject to the limitations set forth below. Each Participant's Option shall be exercisable only to the extent of such Participant's accumulated payroll deductions on the Exercise Date. The purchase price for each share purchased under each Option (the "Option Price") will be 85 percent of the Fair Market Value (as defined in Section 11) of the Common Stock on the Offering Date or the Exercise Date, whichever is less.

Notwithstanding the foregoing, no Participant may be granted an option hereunder if such Participant, immediately after the option was granted, would be treated as owning stock possessing 5 percent or more of the total combined voting power or value of all classes of stock of the Company or any Parent (as defined in Section 11) or Subsidiary. For purposes of the preceding sentence, the attribution rules of Section 424(d) of the Code shall apply in determining the stock ownership of a Participant, and all stock which the Participant has a contractual right to purchase shall be treated as stock owned by the Participant. In addition, no Participant may be granted an Option which permits his or her rights to purchase stock under the Plan, and any other employee stock purchase plan of the Company and its Parents and Subsidiaries, to accrue at a rate which exceeds $25,000 of the Fair Market Value of such stock (determined on the option grant date or dates) for each calendar year in which the Option is outstanding at any time. The purpose of the limitation in the preceding sentence is to comply with Section 423(b)(8) of the Code and shall be applied taking Options into account in the order in which they were granted.

9. Exercise of Option and Purchase of Shares. Each employee who continues to be a Participant in the Plan on an Exercise Date shall be deemed to have exercised his or her Option on such date and shall acquire from the Company such number of whole shares of Common Stock reserved for the purpose of the Plan as his or her accumulated payroll deductions on such date will purchase at the Option Price, subject to any other limitations contained in the Plan. Any amount remaining in a Participant's account after the purchase of shares on an Exercise Date of an Offering solely by reason of the inability to purchase a fractional share will be carried forward to the next Purchase Period; provided, that if such Exercise Date is the final Exercise Date of an Offering, such amount will be carried forward to the next Offering and any other

balance remaining in a Participant's account at the end of an Offering will be refunded to the Participant promptly.

To the extent permitted by applicable laws, if the Fair Market Value of the Common Stock on any Exercise Date in an Offering is lower than the Fair Market Value of the Common Stock on the Offering Date of such Offering, then all Participants in such Offering will be automatically withdrawn from such Offering immediately after the exercise of their Option on such Exercise Date and will be automatically re-enrolled in the immediately following Offering as of the first day thereof.

10. Issuance of Certificates. Certificates, or book entries for uncertificated shares, representing shares of Common Stock purchased under the Plan may be issued only in the name of the employee, in the name of the employee and another person of legal age as joint tenants with rights of survivorship, or in the name of a broker authorized by the employee to be his, her or their, nominee for such purpose.

11. Definitions.

The term "Compensation" means the amount of base pay, prior to salary reduction pursuant to Sections 125, 132(f) or 401(k) of the Code or comparable reductions under laws outside of the United States, but excluding overtime, commissions, incentive or bonus awards, allowances and reimbursements for expenses such as relocation allowances or travel expenses, income or gains on the exercise, vesting or settlement of Company equity incentive awards, and similar items.

The term "Designated Subsidiary" means any Subsidiary that has been designated by the Board to participate in the Plan. For purposes of the Section 423 Component, only the Company and its Subsidiaries may be Designated Subsidiaries; provided, however, that at any given time, a

Subsidiary that is a Designated Subsidiary under the 423 Component will not be a Designated Subsidiary under the Non-423 Component. The Board may so designate any Subsidiary, or revoke any such designation, at any time and from time to time, either before or after the Plan is approved by the stockholders. The current list of Designated Subsidiaries is attached hereto as Appendix A.

The term "Fair Market Value of the Common Stock" on any given date means the fair market value of the Common Stock determined in good faith by the Administrator; provided, however, that if the Common Stock is admitted to quotation on the National Association of Securities Dealers Automated Quotation System ("NASDAQ"), NASDAQ Global Market or another national securities exchange, the determination shall be made by reference to the closing price on such date. If there is no closing price for such date, the determination shall be made by reference to the last date preceding such date for which there is a closing price.

The term "Parent" means a "parent corporation" with respect to the Company, as defined in Section 424(e) of the Code.

The term "Participant" means an individual who is eligible as determined in Section 3 and who has complied with the provisions of Section 4.

The term "Purchase Period" means a period of time specified within an Offering beginning on the Offering Date or on the next day following an Exercise Date within an Offering and ending on an Exercise Date. An Offering may consist of one or more Purchase Periods.

The term "Subsidiary" means a "subsidiary corporation" with respect to the Company, as defined in Section 424(f) of the Code.

12. Rights on Termination of Employment. If a Participant's employment terminates for any reason before the Exercise Date for any Offering, no payroll deduction will be taken

from any pay due and owing to the Participant and the balance in the Participant's account will be paid to such Participant or, in the case of such Participant's death, to his or her designated beneficiary as if such Participant had withdrawn from the Plan under Section 7. An employee will be deemed to have terminated employment, for this purpose, if the corporation that employs him or her, having been a Designated Subsidiary, ceases to be a Subsidiary, or if the employee is transferred to any corporation other than the Company or a Designated Subsidiary. An employee will not be deemed to have terminated employment for this purpose, if the employee is on an approved leave of absence for military service or sickness or for any other purpose approved by the Company, if the employee's right to reemployment is guaranteed either by a statute or by contract or under the policy pursuant to which the leave of absence was granted or if the Administrator otherwise provides in writing.

13. Special Rules. Notwithstanding anything herein to the contrary, the Administrator may adopt special rules applicable to the employees of a particular Designated Subsidiary, whenever the Administrator determines that such rules are necessary or appropriate for the implementation of the Plan in a jurisdiction where such Designated Subsidiary has employees; provided that with respect to the 423 Component, such rules are consistent with the requirements of Section 423(b) of the Code and if such rules are inconsistent with the requirements of Section 423(b) of the Code, these employees will participate in the Non-423 Component. Any special rules established pursuant to this Section 13 shall, to the extent possible and with respect to the 423 Component, result in the employees subject to such rules having substantially the same rights as other Participants in the Plan.

14. Optionees Not Stockholders. Neither the granting of an Option to a Participant nor the deductions from his or her pay shall constitute such Participant a holder of the shares of

Common Stock covered by an Option under the Plan until such shares have been purchased by and issued to him or her.

15. Rights Not Transferable. Rights under the Plan are not transferable by a Participant other than by will or the laws of descent and distribution, and are exercisable during the Participant's lifetime only by the Participant.

16. Application of Funds. All funds received or held by the Company under the Plan may be combined with other corporate funds and may be used for any corporate purpose, unless otherwise required under applicable law.

17. Adjustment in Case of Changes Affecting Common Stock. In the event of a subdivision of outstanding shares of Common Stock, the payment of a dividend in Common Stock or any other change affecting the Common Stock, the number of shares approved for the Plan and the share limitation set forth in Section 8 shall be equitably or proportionately adjusted to give proper effect to such event.

18. Amendment of the Plan. The Board or, to the extent delegated by the Board, the Administrator, may at any time and from time to time amend the Plan in any respect, except that without the approval within 12 months of such Board action by the stockholders, no amendment shall be made increasing the number of shares approved for the Plan or making any other change that would require stockholder approval in order for the 423 Component of the Plan, as amended, to qualify as an "employee stock purchase plan" under Section 423(b) of the Code.

19. Insufficient Shares. If the total number of shares of Common Stock that would otherwise be purchased on any Exercise Date plus the number of shares purchased under previous Offerings under the Plan exceeds the maximum number of shares issuable under the Plan, the shares then available shall be apportioned among Participants in proportion to the

amount of payroll deductions accumulated on behalf of each Participant that would otherwise be used to purchase Common Stock on such Exercise Date.

20. Termination of the Plan. The Plan may be terminated at any time by the Board. Upon termination of the Plan, all amounts in the accounts of Participants shall be promptly refunded.

21. Governmental Regulations. The Company's obligation to sell and deliver Common Stock under the Plan is subject to obtaining all governmental approvals required in connection with the authorization, issuance, or sale of such stock.

22. Governing Law. This Plan and all Options and actions taken thereunder shall be governed by, and construed in accordance with, the laws of the State of Delaware, applied without regard to conflict of law principles.

23. Issuance of Shares. Shares may be issued upon exercise of an Option from authorized but unissued Common Stock, from shares held in the treasury of the Company, or from any other proper source.

24. Tax Withholding. Participation in the Plan is subject to any minimum required tax withholding on income of the Participant in connection with the Plan. Each Participant agrees, by entering the Plan, that the Company and its Subsidiaries shall have the right to deduct any such taxes from any payment of any kind otherwise due to the Participant, including shares issuable under the Plan.

25. Notification Upon Sale of Shares. Each Participant who is subject to tax in the United States with respect to his or her participation in the Plan agrees, by entering the Plan, to give the Company prompt notice of any disposition of shares purchased under the Plan where

such disposition occurs within two years after the date of grant of the Option pursuant to which such shares were purchased or within one year after the date such shares were purchased.

26. Effective Date. The Amended and Restated 2015 Employee Stock Purchase Plan shall become effective as of May 1, 2020.

**APPENDIX A**

Designated Subsidiaries

MyoKardia Netherlands B.V.

**Exhibit 10.3**

MYOKARDIA, INC.

AMENDED AND RESTATED NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

The purpose of this Amended and Restated Non-Employee Director Compensation Policy (the "Policy") of MyoKardia, Inc., a Delaware corporation (the "Company"), is to provide a total compensation package that enables the Company to attract and retain, on a long-term basis, high-caliber directors who are not employees or officers of the Company. In furtherance of this purpose, effective as of the date of approval by the Company's Board of Directors (the "Board") of this Policy (the "Effective Date"), all non-employee directors shall be paid compensation for services provided to the Company as set forth below:[1]

**Cash Retainers**

Annual Retainer for Board Membership: $45,000 for general availability and participation in meetings and conference calls of the Board. Additional $32,500 for service as lead independent director or non-executive Chairperson of the Board. No additional compensation for attending individual Board meetings.

Additional Annual Retainers for Committee Membership and Service as Chairperson:

Audit Committee Chairperson: $20,000

Audit Committee member: $10,000

Compensation Committee Chairperson: $15,000

Compensation Committee member: $7,500

Science and Technology Committee Chairperson: $15,000

Science and Technology Committee member: $7,500

Nominating and Corporate Governance Committee Chairperson: $10,000

Nominating and Corporate Governance Committee member: $5,000

No additional compensation for attending individual committee meetings.

All cash retainers will be paid quarterly, in arrears, or upon the earlier resignation or removal of the non-employee director. Cash retainers owing to non-employee directors shall be annualized, meaning that with respect to non-employee directors who join the Board during the calendar year, such amounts shall be pro-rated based on the number of calendar days served by such director.

1 Upon effectiveness, this policy shall supersede any prior arrangements between the Company and the directors.

**Equity Retainers**

Initial Equity Grant: One-time equity grants to each new non-employee director upon his/her election to the Board after the Effective Date of (a) an option to purchase 7,400 shares of the Company's common stock, par value $0.0001 per share ("Common Stock") and (b) a grant of restricted stock units for 4,800 shares of Common Stock. Such initial option grant shall vest in equal monthly installments during the 48 months following the date upon which the director is first elected to the Board and such initial restricted stock unit grant shall vest in equal annual installments during the four years following the date upon which the director is first elected to the Board, in each case subject to the director's continued service on the Board.

On the date of each Annual Meeting of Stockholders: Annual equity grants to each non-employee director who (a) is serving on the Board as of immediately prior the Company's annual meeting of stockholders and (b) continues to serve on the Board immediately after such annual meeting consisting of (i) an option to purchase 3,700 shares of Common Stock and (ii) restricted stock units for 2,400 shares of Common Stock; provided, that for any non-employee director who joins the Board within 12 months preceding the date of grant, such annual option and restricted stock unit grants shall be pro-rated based on the number of calendar days served by such director through the date immediately preceding the grant date. Such annual option grant shall vest in equal monthly installments during the 12 months following the date of grant, and such annual restricted stock unit grant shall vest in a single installment on the first anniversary of the date of grant, in each case subject to the director's continued service on the Board as of such date.

Additional Equity Grants: In addition to the foregoing, non-employee directors may also be granted such additional equity awards in such amounts and on such dates as the Board may recommend.

Upon the consummation of a Sale Event (as defined in the Company's 2015 Stock Option and Incentive Plan, as may be amended, restated or otherwise modified from time to time), the vesting of all outstanding unvested equity awards, including stock options and restricted stock units, granted to each non-employee director under this policy shall accelerate in full.

The form of option agreement will give directors up to one year following cessation of service as a director to exercise the options (to the extent vested at the date of such cessation), provided that the director has not been removed for cause.

All of the foregoing option grants will have an exercise price equal to the fair market value of a share of Common Stock on the date of grant.

**Expenses**

The Company shall reimburse all reasonable out-of-pocket expenses incurred by non-employee directors in attending Board and committee meetings.

*Amended and Restated Non-Employee Director Compensation Policy approved by the Board of Directors on March 9, 2016.*

*Amended and Restated Non-Employee Director Compensation Policy approved by the Board of Directors on December 6, 2018, effective retroactively to January 1, 2018.*

*Amended and Restated Non-Employee Director Compensation Policy approved by the Board of Directors on January 23, 2019.*

*Amended and Restated Non-Employee Director Compensation Policy approved by the Board of Directors on April 2, 2020.*

**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: May 6, 2020

/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, Taylor Harris, 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: May 6, 2020

/s/ Taylor Harris

Taylor Harris
Chief Financial Officer
(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 March 31, 2020, as filed with the Securities and Exchange Commission (the "Report"), Tassos Gianakakos, Chief Executive Officer of the Company, and Taylor Harris, Chief Financial Officer (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: May 6, 2020

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

/s/ Taylor Harris
Taylor Harris
Chief Financial Officer
(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.