UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 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: 001-38978

FULCRUM THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-4839948**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
26 Landsdowne Street	
Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 651-8851

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, par value $0.001 per share	FULC	Nasdaq Global 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 ☒

As of November 4, 2020, the registrant had 27,462,565 shares of common stock, $0.001 par value per share, outstanding.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements, which reflect our current views with respect to, among other things, our operations and financial performance. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth are forward-looking statements. The words "anticipate," "believe," "continue" "could," "estimate," "expect," "intend," "may," "might," “outlook,” "plan," "potential," "predict," "project," "should," "target," "would," and the negative version of these words and other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such forward-looking statements are subject to various risks and uncertainties. Accordingly, there are or will be important factors that could cause actual outcomes or results to differ materially from those indicated in these statements. We believe these factors include but are not limited to those described under the “Risk Factors” section and include, among other things:

- our ongoing clinical trials of losmapimod, including our ongoing Phase 2b and Phase 2 open label clinical trials for the treatment of facioscapulohumeral muscular dystrophy, and our ongoing Phase 3 clinical trial for the treatment of the novel coronavirus, or COVID-19;
- our ongoing Phase 1 clinical trial of FTX-6058;
- the impact of the COVID-19 pandemic on our business and operations and our future financial results;
- the initiation, timing, progress and results of our drug target discovery screening programs;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and our research and development programs;
- our plans to develop and, if approved, subsequently commercialize losmapimod and any other product candidates, including in combination with other drugs and therapies;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for losmapimod and other product candidates;
- our expectations regarding our ability to fund our operating expenses and capital expenditure requirements with our cash, cash equivalents, and marketable securities;
- the potential advantages of our product candidates;
- the rate and degree of market acceptance and clinical utility of our products;
- our estimates regarding the potential market opportunity for our product candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position;
- the progress and results of our collaborations with Acceleron Pharma Inc. and MyoKardia, Inc.;

- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- our estimates regarding expenses, future revenue, timing of any future revenue, capital requirements and needs for additional financing;
- the impact of government laws and regulations;
- our competitive position;
- developments relating to our competitors and our industry;
- our ability to maintain and establish collaborations or obtain additional funding; and
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the "Risk Factors" section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments we may make or enter into.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report on Form 10-Q are made as of the date of this Quarterly Report on Form 10-Q, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Fulcrum Therapeutics, Inc.

Consolidated Balance Sheets

(In thousands, except share and per share amounts)

(Unaudited)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 85,221	$ 96,713
Marketable securities	41,804	—
Unbilled accounts receivable	592	—
Prepaid expenses and other current assets	4,495	3,370
Total current assets	132,112	100,083
Property and equipment, net	8,395	9,205
Restricted cash	1,092	1,092
Other assets	616	59
Total assets	$ 142,215	$ 110,439
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,634	$ 2,186
Accrued expenses and other current liabilities	8,743	5,496
Deferred lease incentive, current portion	469	469
Deferred revenue, current portion	13,609	3,989
Total current liabilities	26,455	12,140
Deferred rent, excluding current portion	1,634	1,559
Deferred lease incentive, excluding current portion	3,169	3,521
Deferred revenue, excluding current portion	5,965	6,011
Other liabilities, excluding current portion	7	55
Total liabilities	37,230	23,286
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 5,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value; 200,000,000 shares authorized; 27,459,196 and 23,335,514 shares issued as of September 30, 2020 and December 31, 2019, respectively; 27,277,983 and 22,654,444 shares outstanding as of September 30, 2020 and December 31, 2019, respectively	27	23
Treasury stock, at cost; no shares as of September 30, 2020 and December 31, 2019	—	—
Additional paid-in capital	308,827	237,931
Accumulated other comprehensive income	31	—
Accumulated deficit	(203,900)	(150,801)
Total stockholders' equity	104,985	87,153
Total liabilities and stockholders' equity	$ 142,215	$ 110,439

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

Fulcrum Therapeutics, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except per share data)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Collaboration revenue	$ 1,848	$ —	$ 4,598	$ —
Operating expenses:				
Research and development	15,640	13,496	42,897	58,985
General and administrative	5,312	3,510	15,525	8,742
Total operating expenses	20,952	17,006	58,422	67,727
Loss from operations	(19,104)	(17,006)	(53,824)	(67,727)
Other income, net	142	464	725	1,173
Net loss	$ (18,962)	$ (16,542)	$ (53,099)	$ (66,554)
Cumulative convertible preferred stock dividends	—	(796)	—	(7,128)
Net loss attributable to common stockholders	$ (18,962)	$ (17,338)	$ (53,099)	$ (73,682)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.70)	$ (0.97)	$ (2.16)	$ (10.33)
Weighted average number of common shares used in net loss per share attributable to common stockholders, basic and diluted	27,261	17,785	24,621	7,133
Comprehensive loss:				
Net loss	$ (18,962)	$ (16,542)	$ (53,099)	$ (66,554)
Other comprehensive (loss) income:				
Unrealized (loss) gain on marketable securities	(50)	—	31	—
Total other comprehensive (loss) income	(50)	—	31	—
Comprehensive loss	$ (19,012)	$ (16,542)	$ (53,068)	$ (66,554)

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

Fulcrum Therapeutics, Inc.

Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)

(In thousands, except share amounts)

(Unaudited)

	Series A Convertible Preferred Stock		Series B Convertible Preferred Stock		Common Stock		Treasury Stock		Additional Paid-In	Accumulated other comprehensive	Accumulated	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital	income (loss)	Deficit	(Deficit)
Balance at December 31, 2018	60,000,000	$ 59,909	40,000,000	$ 79,761	1,587,953	$ 2	67,024	$ —	$ 4,452	$ —	$ (68,124)	$ (63,670)
Issuance of Series B convertible preferred stock in connection with asset acquisition, net of issuance costs	—	—	12,500,000	25,466	—	—	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	134,013	—	—	—	5	—	—	5
Repurchase of unvested restricted stock awards	—	—	—	—	—	—	43,922	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(110,946)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	821	—	—	821
Net loss	—	—	—	—	—	—	—	—	—	—	(36,843)	(36,843)
Balance at March 31, 2019	60,000,000	$ 59,909	52,500,000	$ 105,227	1,721,966	$ 2	—	$ —	$ 5,278	$ —	$ (104,967)	$ (99,687)
Issuance of common stock	—	—	—	—	148,320	—	—	—	225	—	—	225
Repurchase of unvested restricted stock awards	—	—	—	—	—	—	7,451	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(6,019)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	950	—	—	950
Net loss	—	—	—	—	—	—	—	—	—	—	(13,169)	(13,169)
Balance at June 30, 2019	60,000,000	$ 59,909	52,500,000	$ 105,227	1,870,286	$ 2	1,432	$ —	$ 6,453	$ —	$ (118,136)	$ (111,681)
Issuance of common stock	—	—	—	—	116,167	—	—	—	35	—	—	35
Conversion of convertible preferred stock into common stock	(60,000,000)	(59,909)	(52,500,000)	(105,227)	16,071,418	16			165,120			165,136
Initial public offering, net of underwriting discounts, commissions and offering costs	—	—			4,500,000	5			63,996			64,001
Repurchase of unvested restricted stock awards	—	—	—	—	—	—	4,951	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(6,383)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	1,179	—	—	1,179
Net loss	—	—	—	—	—	—	—	—	—	—	(16,542)	(16,542)
Balance at September 30, 2019	—	$ —	—	$ —	22,557,871	$ 23	—	$ —	$ 236,783	$ —	$ (134,678)	$ 102,128
Balance at December 31, 2019	—	—	—	—	22,654,444	23	—	—	237,931	—	(150,801)	87,153
Issuance of common stock	—	—	—	—	138,693	—	—	—	290	—	—	290
Repurchase of unvested restricted stock awards	—	—	—	—	—	—	8,787	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(8,787)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	1,693	—	—	1,693
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	(53)	—	(53)
Net loss	—	—	—	—	—	—	—	—	—	—	(18,452)	(18,452)
Balance at March 31, 2020	—	$ —	—	$ —	22,793,137	$ 23	—	$ —	$ 239,914	$ (53)	$ (169,253)	$ 70,631
Issuance of common stock	—	—	—	—	401,248	—	—	—	530	—	—	530
Issuance of common stock in connection with private placement, net of placement agent fees and offering costs	—	—	—	—	4,029,411	4	—	—	64,313	—	—	64,317
Repurchase of unvested restricted stock awards					—	—	10,642	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(10,642)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	2,204	—	—	2,204
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	134	—	134
Net loss	—	—	—	—	—	—	—	—	—	—	(15,685)	(15,685)
Balance at June 30, 2020	—	$ —	—	$ —	27,223,796	$ 27	—	$ —	$ 306,961	$ 81	$ (184,938)	$ 122,131
Issuance of common stock	—	—	—	—	54,187	—	—	—	90	—	—	90
Repurchase of unvested restricted stock awards					—	—	4,581	—	—	—	—	—
Retirement of treasury shares	—	—	—	—	—	—	(4,581)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	1,776	—	—	1,776
Unrealized loss on marketable securities	—	—	—	—	—	—	—	—	—	(50)	—	(50)
Net loss	—	—	—	—	—	—	—	—	—	—	(18,962)	(18,962)
Balance at September 30, 2020	—	$ —	—	$ —	27,277,983	$ 27	—	$ —	$ 308,827	$ 31	$ (203,900)	$ 104,985

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

Fulcrum Therapeutics, Inc.

Consolidated Statements of Cash Flows

(In thousands)

(Unaudited)

	Nine Months Ended September 30,	
	2020	**2019**
Operating activities		
Net loss	$ (53,099)	$ (66,554)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	1,646	1,534
Stock-based compensation expense	5,673	2,950
In-process research and development expenses	—	25,591
Net amortization of premiums and discounts on marketable securities	(103)	—
Changes in operating assets and liabilities:		
Unbilled accounts receivable	(592)	—
Prepaid expenses and other current assets	(1,126)	(2,714)
Other assets	(557)	(20)
Accounts payable	1,590	2,849
Accrued expenses and other liabilities	3,442	1,237
Deferred revenue	9,574	—
Deferred rent and deferred lease incentive	(277)	139
Net cash used in operating activities	$ (33,829)	$ (34,988)
Investing activities		
Purchases of marketable securities	(81,270)	—
Maturities of marketable securities	39,600	—
Purchases of property and equipment	(870)	(745)
Transaction costs associated with asset acquisition	—	(91)
Net cash used in investing activities	(42,540)	(836)
Financing activities		
Payment of Series B convertible preferred stock issuance costs	—	(34)
Proceeds from initial public offering of common stock, net of underwriting discounts and commissions	(193)	64,443
Proceeds from issuance of common stock in connection with private placement, net of placement agent fees and offering costs	64,210	—
Principal payments on capital lease obligations	(37)	(34)
Proceeds from issuance of common stock under benefit plans, net	897	249
Net cash provided by financing activities	64,877	64,624
Net (decrease) increase in cash, cash equivalents and restricted cash	(11,492)	28,800
Cash, cash equivalents, and restricted cash, beginning of period	97,805	73,889
Cash, cash equivalents, and restricted cash, end of period	$ 86,313	$ 102,689
Supplemental cash flow information		
Cash paid for interest	$ 3	$ 6
Non-cash investing and financing activities:		
Acquisition of in process research and development through issuance of stock	$ —	$ 25,500
Conversion of convertible preferred stock into common stock	$ —	$ 165,136
Property and equipment purchases unpaid at end of period	$ —	$ —
Public offering costs unpaid at end of period	$ —	$ 442

The following table provides a reconciliation of the cash, cash equivalents, and restricted cash balances as of each of the periods shown above:

	September 30, 2020	**September 30, 2019**
Cash and cash equivalents	$ 85,221	$ 101,597
Restricted cash	1,092	1,092
Total cash, cash equivalents, and restricted cash	$ 86,313	$ 102,689

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

1. Nature of the Business and Basis of Presentation

Fulcrum Therapeutics, Inc. (the "Company" or "Fulcrum") was incorporated in Delaware on August 18, 2015. The Company is focused on improving the lives of patients with genetically defined rare diseases in areas of high unmet medical need.

The Company is subject to a number of risks similar to other companies in the biotechnology industry, including, but not limited to, risks of failure of preclinical studies and clinical trials, dependence on key personnel, protection of proprietary technology, reliance on third party organizations, risks of obtaining regulatory approval for any product candidate that it may develop, development by competitors of technological innovations, compliance with government regulations, and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Basis of Presentation

The accompanying consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

The accompanying consolidated financial statements and footnotes to the financial statements have been prepared on the same basis as the most recently audited annual consolidated financial statements and, in the opinion of management, reflect all normal recurring adjustments necessary for the fair presentation of the Company's financial position as of September 30, 2020 and the results of its operations and its cash flows for the three and nine months ended September 30, 2020 and 2019. The results for the three and nine months ended September 30, 2020 are not necessarily indicative of results to be expected for the year ending December 31, 2020, any other interim periods, or any future year or period. These consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2019 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 5, 2020 (the "Annual Report on Form 10-K").

Sales of Common Stock

On July 22, 2019, the Company completed an initial public offering ("IPO") of its common stock and issued and sold 4,500,000 shares of common stock at a public offering price of $16.00 per share, resulting in net proceeds of $63.9 million after deducting underwriting discounts and commissions and offering expenses. Upon the closing of the IPO, all 112,500,000 shares of outstanding preferred stock automatically converted into 16,071,418 shares of common stock.

On July 5, 2019, in connection with the IPO, the Company effected a one-for-seven reverse stock split of the Company's issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each of the Company's outstanding series of preferred stock. All share and per share amounts in the accompanying consolidated financial statements and notes thereto for periods prior to the reverse stock split have been retroactively adjusted to give effect to this reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital.

On June 9, 2020, the Company issued and sold 4,029,411 shares of common stock to investors in a private placement at a price of $17.00 per share, resulting in gross proceeds of $68.5 million, before deducting offering costs of approximately $4.2 million.

Liquidity

The Company has incurred recurring losses and negative cash flows from operations since inception and has primarily funded its operations with proceeds from a private placement of the Company's common stock, proceeds from the IPO, issuances of convertible notes and convertible preferred stock, an upfront payment received from its collaboration and license agreement (the "Acceleron Collaboration Agreement") with Acceleron Pharma Inc. ("Acceleron"), and an upfront payment received from its collaboration and license agreement (the "MyoKardia Collaboration Agreement") with MyoKardia, Inc. ("MyoKardia"). As of September 30, 2020, the Company had an accumulated deficit of $203.9 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future as it continues to expand its research and development efforts. The Company expects to finance its future cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements.

As of the date of issuance of these financial statements, the Company expects that its cash, cash equivalents, and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these financial statements. However, the Company has based this estimate on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. If the Company is unable to raise additional funds through equity or debt financings when needed, it may be required to delay, limit, reduce or terminate development or future commercialization efforts or grant rights to develop and market product candidates that it would otherwise prefer to develop and market itself.

2. Summary of Significant Accounting Policies

Principles of Consolidation

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiary, Fulcrum Therapeutics Securities Corp., which is a Massachusetts subsidiary created to buy, sell, and hold securities. All intercompany transactions and balances have been eliminated.

Summary of Significant Accounting Policies

The significant accounting policies and estimates used in the preparation of the accompanying consolidated financial statements are described in the Company's audited consolidated financial statements for the year ended December 31, 2019 included in the Company's Annual Report on Form 10-K. There have been no material changes in the Company's significant accounting policies during the nine months ended September 30, 2020, except as noted below with respect to the Company's accounting policies related to marketable securities and except as noted below under "Recently Adopted Accounting Pronouncements".

Use of Estimates

The preparation of financial statements in accordance with GAAP requires management to make certain estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities as of the date of the financial statements, and the reported amount of expenses during the reported periods. Estimates inherent in the preparation of these consolidated financial statements include, but are not limited to, estimates related to revenue recognition, accrued expenses, stock-based compensation expense, the fair value of the Company's common stock and convertible preferred stock prior to the completion of the IPO, and income taxes. The Company bases its estimates on historical experience and other market specific or other relevant assumptions it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates as there are changes in circumstances, facts and experience. Actual results could differ from those estimates or assumptions.

Fair Value of Financial Instruments

The fair value of the Company's financial assets and liabilities reflects the Company's estimate of amounts that it would have received in connection with the sale of the assets or paid in connection with the transfer of the liabilities in an orderly transaction between market participants at the measurement date. In connection with measuring the fair value of its assets and liabilities, the Company seeks to maximize the use of observable inputs (market data obtained from sources independent from the Company) and to minimize the use of unobservable inputs (the Company's assumptions about how market participants would price assets and liabilities). The following fair value hierarchy is used to classify assets and liabilities based on the observable inputs and unobservable inputs used in order to value the assets and liabilities:

Level 1: Quoted prices in active markets for identical assets or liabilities. An active market for an asset or liability is a market in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis.

Level 2: Observable inputs other than Level 1 inputs. Examples of Level 2 inputs include quoted prices in active markets for similar assets or liabilities and quoted prices for identical assets or liabilities in markets that are not active.

Level 3: Unobservable inputs based on the Company's assessment of the assumptions that market participants would use in pricing the asset or liability.

The Company's cash equivalents and marketable securities are carried at fair value and are classified according to the fair value hierarchy described above (Note 3). The cash equivalents and marketable securities are initially valued at the transaction price, and subsequently revalued at the end of each reporting period, utilizing third-party pricing services. The pricing services utilize industry standard valuation models, including both income and market based approaches, to determine fair value.

Marketable Securities

The Company classifies marketable securities with a remaining maturity when purchased of greater than three months as marketable securities. As of September 30, 2020, the Company's marketable securities consisted of investments in U.S. Treasury securities, corporate bonds, and commercial paper. Marketable securities are classified as current assets on the consolidated balance sheets if the marketable securities are available to be converted into cash to fund current operations.

Marketable securities are carried at fair value with the unrealized gains and losses included in accumulated other comprehensive loss, which is a component of stockholders' equity, until such gains and losses are realized. Any premium arising at purchase is amortized to interest expense over the period of the earliest call date, and any discount arising at purchase is accreted to interest income over the life of the instrument. Realized gains and losses are determined using the specific identification method and are included in other income, net.

If any adjustment to fair value reflects a decline in value of the investment, the Company considers all available evidence to evaluate the extent to which the decline is "other-than-temporary" and, if so, marks the investment to market through a charge to the Company's statement of operations and comprehensive loss.

Off-Balance Sheet Risk and Concentrations of Credit Risk

The Company has no significant off-balance sheet risk such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents, marketable securities, and restricted cash. The Company's cash, cash equivalents, and restricted cash are deposited in accounts at large financial institutions. The Company believes it is not exposed to significant credit risk due to the financial strength of the depository institutions in which the cash, cash equivalents and restricted cash are held. The Company maintains its cash equivalents in money market funds that invest in U.S. Treasury securities. The Company's marketable securities primarily consist of U.S. Treasury securities, corporate bonds, and commercial paper, and potentially subject the Company to concentrations of credit risk. The Company has adopted an investment policy that limits the amounts the Company may invest in any one type of investment. The Company has not experienced any credit losses and does not believe it is exposed to any significant credit risk on these funds.

Recently Adopted Accounting Pronouncements

In March 2017, the FASB issued ASU No. 2017-08, *Receivables - Nonrefundable Fees and Other Costs (Subtopic 310-20): Premium Amortization on Purchased Callable Debt Securities* ("ASU 2017-08"). This standard amends the amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period to the earliest call date. The new standard became effective for the Company on January 1, 2020. The adoption of this standard did not have a material impact on the Company's consolidated financial statements or footnote disclosures.

In July 2017, the FASB issued ASU No. 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) I. Accounting for Certain Financial Instruments with Down Round Features II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* ("ASU 2017-11"). Part I of the standard applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II of the standard replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. The new standard became effective for the Company on January 1, 2020 under the extended transition period. The adoption of this standard did not have a material impact on the Company's consolidated financial statements or footnote disclosures.

In November 2018, the FASB issued ASU No. 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606*. The standard clarifies that certain transactions between collaborative arrangement participants should be accounted for as revenue under ASC 606 when the collaborative arrangement participant is a customer in the context of a unit of account. The standard amends ASC 808 to refer to the unit-of-account guidance in ASC 606 and requires it to be used only when assessing whether a transaction is in the scope of ASC 606 when an entity is assessing whether the collaborative arrangement or a part of the arrangement is within the scope of ASC 606. The standard requires that in a transaction with a collaborative arrangement participant that is not directly related to sales to third parties, presenting that transaction together with revenue recognized under ASC 606 is precluded if the collaborative arrangement participant is not a customer. The new standard became effective for the Company on January 1, 2020. The adoption of this standard did not have a material impact on the Company's consolidated financial statements or footnote disclosures.

Recent Accounting Pronouncements—To Be Adopted

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), as amended by various subsequently issued ASUs. The standard requires lessees to recognize an operating lease with a term greater than one year on their balance sheets as a right-of-use asset and corresponding lease liability, measured at the present value of the lease payments. Lessees are required to classify leases as either finance or operating leases. If the lease is effectively a financed-purchase by the lessee, it is classified as a financing lease, otherwise it is classified as an operating lease. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. In July 2018, the FASB also issued ASU 2018-11, *Leases (Topic 842): Targeted Improvements* ("ASU 2018-11"), which permits entities to continue applying legacy guidance in ASC 840, *Leases*, including its disclosure requirements, in the comparative periods presented in the year that the entity adopts the new leasing standard. In November 2019, the FASB deferred the effective date of ASU 2018-11 for private companies to fiscal years beginning after December 15, 2020. In June 2020, the FASB further deferred the effective date of ASU 2018-11 for private companies to fiscal years beginning after December 15, 2021. The new standard will become effective for the Company on January 1, 2022. The Company will apply the transition method permitted by ASU 2018-11. The Company is currently evaluating the effect that adoption of the standard is expected to have on the Company's consolidated financial statements and related disclosures. The Company expects to take advantage of certain available expedients by electing the transition package of practical expedients permitted within ASU 2016-02, which allows the Company to not reassess previous accounting conclusions around whether arrangements are, or contain, leases, the classification of leases, and the treatment of initial direct costs. The Company also expects to make an accounting policy election to exclude leases with an initial term of twelve months or less from the balance sheet.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). The standard requires that credit losses be reported using an expected losses model rather than the incurred losses model that is currently used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, this standard requires allowances to be recorded instead of reducing the amortized cost of the investment. The new standard will be effective for the Company on January 1, 2023. The Company is currently evaluating the potential impact that this standard may have on its consolidated financial position and results of operations.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes-Simplifying the Accounting for Income Taxes* ("ASU 2019-12"). The standard eliminates certain exceptions related to the approach for intraperiod tax allocation, the methodology for calculating income taxes in an interim period, and the recognition of deferred tax liabilities for outside basis differences. The new guidance also simplifies aspects of the accounting for franchise taxes and enacted changes in tax laws or rates and clarifies the accounting for transactions that result in a step-up in the tax basis of goodwill. The new standard will be effective for the Company on January 1, 2023. The Company is currently evaluating the potential impact that this standard may have on its consolidated financial position and results of operations.

3. Fair Value Measurements

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the fair value hierarchy classification of such fair values as of September 30, 2020 and December 31, 2019 (in thousands):

	Fair Value Measurements at September 30, 2020			
	Total	Level 1	Level 2	Level 3
Cash equivalents:				
Money market funds	$ 85,221	$ 85,221	$ —	$ —
Marketable securities:				
U.S. Treasury securities	14,998	—	14,998	—
Corporate bonds	13,780	—	13,780	—
Commercial paper	13,026	—	13,026	—
Total	$ 127,025	$ 85,221	$ 41,804	$ —

	Fair Value Measurements at December 31, 2019			
	Total	Level 1	Level 2	Level 3
Cash equivalents:				
Money market funds	$ 96,713	$ 96,713	$ —	$ —
Total	$ 96,713	$ 96,713	$ —	$ —

There were no transfers between fair value levels during the three and nine months ended September 30, 2020.

4. Marketable Securities

Marketable securities consisted of the following as of September 30, 2020 (in thousands):

	Fair Value Measurements at September 30, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash equivalents:				
Money market funds	$ 85,221	$ —	$ —	$ 85,221
Total cash equivalents	85,221	—	—	85,221
Marketable securities:				
U.S. Treasury securities	14,996	2	—	14,998
Corporate bonds	13,751	29	—	13,780
Commercial paper	13,026	—	—	13,026
Total marketable securities	41,773	31	—	41,804
Total cash equivalents and marketable securities	$ 126,994	$ 31	$ —	$ 127,025

The Company did not hold any marketable securities as of December 31, 2019.

There were no sales of marketable securities during the three and nine months ended September 30, 2020. As of September 30, 2020, no securities were in an unrealized loss position. The Company determined that it did not hold any securities with any other-than-temporary impairment as of September 30, 2020. As of September 30, 2020, the remaining contractual maturity of all of the Company's marketable securities is less than one year.

5. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Lab equipment	$ 6,468	$ 5,710
Furniture and fixtures	594	548
Computer equipment	373	512
Software	199	90
Leasehold improvements	6,210	6,210
Construction in process	—	82
Total property and equipment	13,844	13,152
Less: accumulated depreciation	(5,449)	(3,947)
Property and equipment, net	$ 8,395	$ 9,205

Depreciation expense for each of the three months ended September 30, 2020 and 2019 was $0.5 million. Depreciation expense for the nine months ended September 30, 2020 and 2019 was $1.6 million and $1.5 million, respectively.

6. Additional Balance Sheet Detail

Prepaid expenses and other current assets consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Prepaid expenses	$ 4,286	$ 2,796
Prepaid sign-on bonuses subject to vesting provisions	66	179
Interest income receivable	71	111
Other	72	284
Total prepaid expenses and other current assets	$ 4,495	$ 3,370

Accrued expenses and other current liabilities consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
External research and development	$ 5,954	$ 2,250
Payroll and benefits	2,255	2,239
Professional services	431	891
Capital lease obligation, current portion	30	50
Restricted stock liability, current portion	9	17
Other	64	49
Total accrued expenses and other current liabilities	$ 8,743	$ 5,496

7. Preferred Stock

As of September 30, 2020 and December 31, 2019, 5,000,000 shares of undesignated preferred stock were authorized. No shares of preferred stock were issued or outstanding as of September 30, 2020 and December 31, 2019.

During the nine months ended September 30, 2019, the Company issued 12,500,000 shares of Series B convertible preferred stock (the "Series B Preferred Stock") in connection with the right of reference and license agreement, as amended (the "GSK Agreement"), with subsidiaries of GlaxoSmithKline plc (collectively referred to as "GSK") (Note 11). The rights, privileges, and preferences of the Series B Preferred Stock issued in connection with the GSK Agreement were consistent with the rights, privileges, and preferences of the Series B Preferred Stock issued during prior periods.

On July 5, 2019, the Company eliminated the gross proceeds threshold of $45.0 million for a firm-commitment underwritten public offering that would result in the automatic conversion of all outstanding shares of Series A convertible preferred stock and Series B Preferred Stock (together with the Series A convertible preferred stock, the "Preferred Stock").

Upon the completion of the IPO on July 22, 2019, all 112,500,000 shares of outstanding Preferred Stock automatically converted into 16,071,418 shares of common stock. In addition, upon the completion of the IPO, the Company amended and restated its certificate of incorporation to authorize 5,000,000 shares of preferred stock, which shares of preferred stock are currently undesignated.

No dividends have been declared since inception.

8. Common Stock

As of September 30, 2020 and December 31, 2019, the Company's restated certificate of incorporation authorized the Company to issue 200,000,000 shares of common stock, $0.001 par value per share.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are not entitled to receive dividends, unless declared by the Company's board of directors, subject to the preferential dividend rights of any preferred stock then outstanding. No dividends have been declared or paid by the Company since its inception.

On August 11, 2020, the Company entered into an Equity Distribution Agreement with Piper Sandler & Co ("Piper Sandler"), as sales agent, pursuant to which the Company may offer and sell shares of its common stock with an aggregate offering price of up to $75.0 million under an "at-the-market" offering program (the "ATM Offering"). The Equity Distribution Agreement provides that Piper Sandler will be entitled to a sales commission equal to 3.0% of the gross sales price per share of all shares sold under the ATM Offering. From the initiation of the ATM Offering through September 30, 2020, the Company has sold no shares under the ATM Offering.

As of September 30, 2020 and December 31, 2019, the Company has reserved for future issuance the following number of shares of common stock:

	September 30, 2020	December 31, 2019
Shares reserved for exercises of outstanding stock options	2,868,693	2,023,828
Shares reserved for future issuance under the 2019 Stock Incentive Plan	1,860,978	1,866,694
Shares reserved for future issuance under the 2019 Employee Stock Purchase Plan	485,497	252,142
	5,215,168	4,142,664

9. Stock-based Compensation Expense

2016 Stock Incentive Plan

In July 2016, the Company adopted the 2016 Stock Incentive Plan (the "2016 Plan"), which provided for the grant of restricted stock awards, restricted stock units, incentive stock options, non-statutory stock options, and other stock-based awards to the Company's eligible employees, officers, directors, consultants, and advisors. As of the effective date of the 2019 Stock Incentive Plan (the "2019 Plan"), and as of September 30, 2020, no shares remained available for future issuance under the 2016 Plan. Any options or awards outstanding under the 2016 Plan remain outstanding and effective.

2019 Stock Incentive Plan

On July 2, 2019, the Company's stockholders approved the 2019 Plan, which became effective on July 17, 2019. The 2019 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards to the Company's officers, employees, directors, consultants and advisors. The number of shares initially reserved for issuance under the 2019 Plan is 2,017,142 shares, plus the shares of common stock remaining available for issuance under the 2016 Plan as of July 17, 2019. The number of shares reserved shall be annually increased on January 1, 2020 and each January 1 thereafter through January 1, 2029 by the least of (i) 2,000,000 shares, (ii) 4% of the number of shares of the Company's common stock outstanding on the first day of each such year or (iii) an amount determined by the Company's board of directors. On January 1, 2020, the number of shares reserved for issuance under the 2019 Plan was increased by 933,420 shares. As of September 30, 2020, there were 1,860,978 shares available for future issuance under the 2019 Plan.

The shares of common stock underlying any awards that expire, terminate, or are otherwise surrendered, cancelled, forfeited or repurchased by the Company under the 2016 Plan or the 2019 Plan will be added back to the shares of common stock available for issuance under the 2019 Plan. As of July 17, 2019, no further awards will be made under the 2016 Plan.

For financial reporting purposes, the Company performed common stock valuations with the assistance of a third-party specialist as of May 10, 2019, March 15, 2019, November 30, 2018, August 24, 2018, June 1, 2018, December 31, 2017, and December 31, 2016 to determine stock-based compensation expense for restricted stock awards and stock options. Upon completion of the IPO, the fair value of the common stock on the grant date was based on the closing price of the stock on the Nasdaq Global Market on the date of grant.

The Company may repurchase unvested shares at the original purchase price if employees or non-employees are terminated or cease their employment or service relationship with the Company. Shares of common stock repurchased from employees and non-employees are shares held in the Company's treasury ("Treasury Shares"). The board of directors may, at its discretion, authorize that the Treasury Shares be returned to the pool of authorized but unissued common stock.

The shares of common stock underlying restricted stock awards typically vest over a four-year period. The shares of common stock are recorded in stockholders' equity as they vest.

The following table summarizes the Company's restricted stock activity under the 2019 Plan and 2016 Plan since December 31, 2019:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2019	346,423	$ 3.05
Granted	—	—
Vested	(198,343)	2.99
Repurchased	(24,010)	3.02
Unvested at September 30, 2020	124,070	$ 3.15

Stock options granted by the Company typically vest over a four-year period and have a ten year contractual term. The following table summarizes the Company's stock option activity under the 2019 Plan and 2016 Plan since December 31, 2019:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2019	2,023,828	$ 9.31	8.94	$ 2,730,209
Granted	1,032,506	15.73		
Exercised	(118,281)	7.59		
Cancelled	(69,360)	12.23		
Outstanding at September 30, 2020	2,868,693	$ 11.62	8.73	$ 425,192
Exercisable at September 30, 2020	733,937	$ 10.16	8.41	$ 181,578

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock as of the balance sheet date for those options that had exercise prices lower than the fair value of the Company's common stock.

The weighted average grant date fair value of stock options granted during the three and nine months ended September 30, 2020 was $4.84 per share and $10.41 per share, respectively. The weighted average grant date fair value of stock options granted during the three and nine months ended September 30, 2019 was $10.36 per share and $6.97 per share, respectively. The total intrinsic value of stock options exercised during the three and nine months ended September 30, 2020 was $0.1 million and $1.2 million, respectively. The aggregate intrinsic value of stock options exercised during the three and nine months ended September 30, 2019 was less than $0.1 million and $0.2 million, respectively. The fair value of stock options granted during the three and nine months ended September 30, 2020 and 2019 under the 2019 Plan and the 2016 Plan has been calculated on the date of grant using the following weighted average assumptions:

	Three Months Ended September 30, 2020	Three Months Ended September 30, 2019	Nine Months Ended September 30, 2020	Nine Months Ended September 30, 2019
Risk-free interest rate	0.3%	1.7%	1.4%	2.4%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%
Expected term (years)	6.0	6.0	6.0	6.0
Expected stock price volatility	78.1%	80.1%	75.9%	81.0%

Grants Outside of Stock Incentive Plans

The following table summarizes the Company's restricted stock activity outside of the 2019 Plan and 2016 Plan since December 31, 2019:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2019	334,647	$ 2.94
Granted	—	—
Vested	(277,504)	2.94
Repurchased	—	—
Unvested at September 30, 2020	57,143	$ 2.94

The aggregate intrinsic value of all restricted stock awards that vested during the three months ended September 30, 2020 and 2019 was $0.7 million and $0.6 million, respectively. The aggregate intrinsic value of all restricted stock awards that vested during the nine months ended September 30, 2020 and 2019 was $8.3 million and $3.0 million, respectively.

Stock-based Compensation Expense

The total compensation cost recognized in the statements of operations associated with all stock-based compensation awards granted by the Company is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 779	$ 533	$ 2,723	$ 1,398
General and administrative	997	646	2,950	1,552
Total stock-based compensation expense	$ 1,776	$ 1,179	$ 5,673	$ 2,950

As of September 30, 2020, the Company had an aggregate of $16.7 million of unrecognized stock-based compensation expense, which is expected to be recognized over a weighted average period of 2.62 years.

2019 Employee Stock Purchase Plan

On July 2, 2019, the Company's stockholders approved the 2019 Employee Stock Purchase Plan (the "ESPP"), which became effective on July 17, 2019. A total of 252,142 shares of common stock were initially reserved for issuance under the ESPP. In addition, the number of shares of common stock reserved under the ESPP shall be annually increased on January 1, 2020, and each January 1 thereafter through January 1, 2029, by the least of (i) 428,571 shares of common stock, (ii) 1% of the number of shares of the Company's common stock outstanding on the first day of each such year or (iii) an amount determined by the Company's board of directors. On January 1, 2020, the number of shares reserved for issuance under the 2019 ESPP was increased by 233,355 shares.

10. Collaboration and License Agreements

Acceleron Collaboration Agreement

On December 20, 2019, the Company entered into the Acceleron Collaboration Agreement to identify biological targets to modulate specific pathways associated with a targeted indication within the pulmonary disease space (the "Indication"). Under the terms of the Acceleron Collaboration Agreement, the Company granted Acceleron an exclusive worldwide license under certain intellectual property rights to make, have made, use, sell, have sold, import, export, distribute and have distributed, market, have marketed, promote, have promoted, or otherwise exploit molecules and products directed against or expressing certain biological targets identified by the Company for the treatment, prophylaxis, or diagnosis of the Indication.

Pursuant to a mutually agreed research plan, the Company will perform assay screening and related research activities to identify and validate potential biological targets for further research in order to support the development, manufacture and commercialization of product candidates by Acceleron. Upon completion of the research activities, the Company will deliver a data package to Acceleron with respect to the biological targets identified by the Company in the conduct of the research activities for the treatment, prophylaxis, or diagnosis of the Indication. As provided for under the exclusive worldwide license that was conveyed at the inception of the arrangement, Acceleron has the right to designate a specified number of the biological targets identified by the Company for Acceleron's research, development, manufacture and commercialization of products or molecules directed to such targets for the treatment, prophylaxis, or diagnosis of the Indication (the "Targets"). If Acceleron does not designate any Targets during the designated period, then the Acceleron Collaboration Agreement will automatically terminate. If Acceleron designates one or more Targets, then Acceleron will be obligated to use commercially reasonable efforts to seek regulatory approval for one product directed to a Target in certain specified countries. Upon receipt of regulatory approval for any product directed to a Target, Acceleron must use commercially reasonable efforts to commercialize such product in certain specified countries.

Acceleron may also request that the Company perform medicinal chemistry services related to the generation and optimization of molecules directed against or expressing biological targets for the treatment, prophylaxis, or diagnosis of the Indication beyond the scope of the research plan. If the Company agrees to provide such medicinal chemistry services, the Company and Acceleron will negotiate to determine the scope, timeline and budget for such medicinal chemistry services.

The Company received a non-refundable upfront payment of $10.0 million in December 2019 upon the execution of the Acceleron Collaboration Agreement. The Company will be entitled to research milestone payments of up to $18.5 million in the aggregate upon achievement of specified research milestones, development milestone payments of up to $202.5 million in the aggregate upon achievement of specified clinical and regulatory milestones, and sales milestones payments of up to $217.5 million in the aggregate upon the achievement of certain aggregate annual worldwide net sales milestones for certain products directed to a Target that have achieved such milestones. In addition, the Company will be entitled to tiered royalties ranging from a mid single-digit percentage to a low double-digit percentage on Acceleron's annual worldwide net sales of products directed to any Target, subject to reduction in specified circumstances. The Company is also entitled to receive reimbursement from Acceleron for research costs incurred under the research plan, including internal and external costs.

The Acceleron Collaboration Agreement continues on a country-by-country and Target-by-Target basis until the last to expire royalty term for a product directed to such Target, at which time the Acceleron Collaboration Agreement expires with respect to such Target in such country. Either party has the right to terminate the Acceleron Collaboration Agreement if the other party has materially breached in the performance of its obligations under the contract and such breach has not been cured within the applicable cure period. Acceleron also has the right to terminate the Acceleron Collaboration Agreement for convenience in its entirety or on a Target-by-Target and, if the Company performs medicinal chemistry services, on a molecule-by-molecule basis with respect to any molecule directed against a Target.

While the Company is performing the research activities pursuant to the research plan and for a specified period thereafter, the Company may not research, develop, manufacture, commercialize, use, or otherwise exploit any compound or product for the treatment, prophylaxis, or diagnosis of the Indication other than for Acceleron. While the Company is performing the research activities pursuant to the research plan and for a specified period thereafter, other than for Acceleron, the Company may not research, develop, manufacture, commercialize, use, or otherwise exploit any compound or product for the treatment, prophylaxis, or diagnosis of the Indication that is directed against certain specified biological targets identified by the Company in the performance of the research activities.

Accounting Analysis

Identification of the Contract

The Company assessed the Acceleron Collaboration Agreement and concluded that it represents a contract with a customer within the scope of ASC 606.

Identification of the Promises and Performance Obligations

The Company determined that the Acceleron Collaboration Agreement contains the following promises: (i) an exclusive worldwide license under certain intellectual property rights, including rights to a specified number of biological targets identified by the Company for the treatment, prophylaxis, or diagnosis of a targeted indication within the pulmonary disease space that was conveyed at the inception of the arrangement (the "License"), (ii) research services to identify and validate potential biological targets (the "Research Services"), and (iii) participation in the joint steering committee (the "JSC").

The Company assessed the above promises and concluded that the License is not capable of being distinct from the Research Services given that the License has limited value without the performance of the Research Services and the Research Services can only be performed by the Company due to their specialized nature. Therefore, the Company has concluded that the License and the Research Services represent a single combined performance obligation.

The Company also assessed the participation on the JSC and concluded that the promise is quantitatively and qualitatively immaterial in the context of the Acceleron Collaboration Agreement. Accordingly, the Company has disregarded its participation on the JSC as a performance obligation.

The potential medicinal chemistry services were not identified as a promised good or service because the Company is under no obligation to provide those services.

Determination of the Transaction Price

The Company received a non-refundable upfront payment of $10.0 million upon the execution of the Acceleron Collaboration Agreement, which the Company included in the transaction price. Based on the uncertainty associated with the achievement of any research and development milestone payments that the Company is eligible to receive, the Company has constrained the variable consideration associated with those milestone payments and excluded them from the transaction price. As part of its evaluation of constraining the research and development milestones, the Company considered numerous factors, including the fact that the achievement of the research and development milestones are contingent upon the results of the underlying research and development activities and are thus outside of the control of the Company. The Company also included in the transaction price the expected amount of costs to be reimbursed for the Research Services. The Company reassesses the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, and, if necessary, adjusts its estimate of the transaction price. There was no change in the amount of variable consideration constrained during the three and nine months ended September 30, 2020.

Any consideration related to sales milestone payments (including royalties) will be recognized when the related sales occur as these amounts have been determined to relate predominantly to the license granted to Acceleron and therefore are recognized at the later of when the related sales occur or the performance obligation is satisfied.

Allocation of the Transaction Price to Performance Obligations

As noted above, the Company has identified a single performance obligation associated with the Acceleron Collaboration Agreement. Therefore, the Company will allocate the entire amount of the transaction price to the identified single performance obligation.

Recognition of Revenue

The Company recognizes revenue related to the Acceleron Collaboration Agreement over time as the Research Services are rendered. The Company has concluded that an input method is a representative depiction of the transfer of services under the Acceleron Collaboration Agreement. The method of measuring progress towards the delivery of the services incorporates actual cumulative internal and external costs incurred relative to total internal and external costs expected to be incurred to satisfy the performance obligation. The period over which total costs are estimated reflects the Company's estimate of the period over which it will perform the Research Services. Changes in estimates of total internal and external costs expected to be incurred are recognized in the period of change as a cumulative catch-up adjustment.

During the three and nine months ended September 30, 2020, the Company recognized $1.5 million and $4.3 million, respectively, of collaboration revenue associated with the Acceleron Collaboration Agreement. As of December 31, 2019, no Research Services had been performed. For the three and nine months ended September 30, 2019, the Company did not recognize any revenue under the Acceleron Collaboration Agreement as it had not yet entered into the Acceleron Collaboration Agreement. As of September 30, 2020 and December 31, 2019, the Company recorded deferred revenue of $7.4 million and $10.0 million, respectively, associated with the Acceleron Collaboration Agreement, which is classified as either current or net of current portion in the accompanying consolidated balance sheets based on the period over which the revenue is expected to be recognized. The aggregate deferred revenue balance represents the aggregate amount of the transaction price allocated to the performance obligations that are unsatisfied as of September 30, 2020 and December 31, 2019, respectively. As of September 30, 2020, the Company had received $1.1 million of cost reimbursement payments, and no milestone or royalty payments under the Acceleron Collaboration Agreement. As of December 31, 2019, the Company had not received any milestone, royalty, or cost reimbursement payments under the Acceleron Collaboration Agreement. As of September 30, 2020, the Company recorded unbilled accounts receivable of $0.6 million related to reimbursable Research Services costs under the Acceleron Collaboration Agreement for activities performed during the three months ended September 30, 2020.

MyoKardia Collaboration Agreement

On July 20, 2020, the Company entered into the MyoKardia Collaboration Agreement with MyoKardia, pursuant to which the Company granted to MyoKardia an exclusive worldwide license under certain intellectual property rights to research, develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, export, have exported, distribute, have distributed, market, have marketed, promote, have promoted, or otherwise exploit products directed against certain biological targets identified by the Company that are capable of modulating up to a certain number of genes of interest with relevance to certain genetically defined cardiomyopathies.

Pursuant to a mutually agreed research plan, the Company will perform assay screening and related research activities to identify and validate up to a specified number of potential cardiomyopathy gene targets ("Identified Targets") for further research, development, manufacture and commercialization by MyoKardia. The Company and MyoKardia will work together to determine how best to advance at each stage of the research activities under the research plan and to identify which of the Identified Targets, if any, meet the criteria set forth in the research plan (the "Cardiomyopathy Target Candidates"). Upon completion of the research plan, the parties will work together to prepare a final data package and MyoKardia may designate certain Cardiomyopathy Target Candidates for MyoKardia's further exploitation under the MyoKardia Collaboration Agreement (the "Cardiomyopathy Targets"). If MyoKardia does not designate any Cardiomyopathy Targets during the designated period, then the MyoKardia Collaboration Agreement will automatically terminate. If MyoKardia designates one or more Cardiomyopathy Targets, then MyoKardia will be obligated to use commercially reasonable efforts to seek regulatory approval for and to commercialize one product directed against an Identified Target in certain specified countries.

During the period in which the Company is performing the research activities pursuant to the research plan (the "Research Term") and for a specified period beyond the Research Term if MyoKardia designates a Cardiomyopathy Target, the Company may only use the data generated from such research activities for MyoKardia in accordance with the MyoKardia Collaboration Agreement. During the Research Term and for a specified period thereafter, the Company may not research, develop, manufacture, commercialize, use, or otherwise exploit any compound or product (a) that is a Compound or Product under the MyoKardia Collaboration Agreement that is directed against the Cardiomyopathy Target Candidates for the treatment, prophylaxis, or diagnosis of any indication or (b) for the treatment of any genetically defined cardiomyopathies shown to be related to certain specified genes of interest that are modulated by the Cardiomyopathy Targets.

Under the MyoKardia Collaboration Agreement, MyoKardia made a $10.0 million upfront payment and a $2.5 million payment as prepaid research funding to the Company in July 2020. MyoKardia will also reimburse the Company for the costs of the research activities not covered by the prepaid research funding, up to a maximum amount of total research funding (including the prepaid research funding). Upon the achievement of specified preclinical, development and sales milestones, the Company will be entitled to preclinical milestone payments, development milestone payments and sales milestone payments of up to $298.5 million in the aggregate per target for certain Identified Targets, and of up to $150.0 million in the aggregate per target for certain other Identified Targets. MyoKardia will also pay the Company tiered royalties ranging from a mid single-digit percentage to a low double-digit percentage based on MyoKardia's, and any of its affiliates' and sublicensees', annual worldwide net sales of products under the MyoKardia Collaboration Agreement directed against any Identified Target. The royalties are payable on a product-by-product basis during a specified royalty term, and may be reduced in specified circumstances.

The MyoKardia Collaboration Agreement continues on a country-by-country and product-by-product basis until the last to expire royalty term for a product, at which time the MyoKardia Collaboration Agreement expires with respect to such product in such country. Either party has the right to terminate the MyoKardia Collaboration Agreement if the other party has materially breached in the performance of its obligations under the MyoKardia Collaboration Agreement and such breach has not been cured within the applicable cure period. MyoKardia also has the right to terminate the MyoKardia Collaboration Agreement for convenience in its entirety or on a target-by-target, product-by-product or molecule-by-molecule basis.

Accounting Analysis

Identification of the Contract

The Company assessed the MyoKardia Collaboration Agreement and concluded that it represents a contract with a customer within the scope of ASC 606.

Identification of the Promises and Performance Obligations

The Company determined that the MyoKardia Collaboration Agreement contains the following promises: (i) an exclusive worldwide license under certain intellectual property rights, including rights to a specified number of potential cardiomyopathy gene targets identified by the Company for further research, development, manufacture and commercialization for the treatment, prophylaxis, or diagnosis of certain genetically defined cardiomyopathies that was conveyed at the inception of the arrangement (the "MyoKardia License"), (ii) research services to identify and validate potential biological targets (the "MyoKardia Research Services"), and (iii) participation in the joint steering committee (the "MyoKardia JSC").

The Company assessed the above promises and concluded that the MyoKardia License is not capable of being distinct from the MyoKardia Research Services given that the MyoKardia License has limited value without the performance of the MyoKardia Research Services and the MyoKardia Research Services can only be performed by the Company due to their specialized nature. Therefore, the Company has concluded that the MyoKardia License and the MyoKardia Research Services represent a single combined performance obligation.

The Company also assessed the participation on the MyoKardia JSC and concluded that the promise is quantitatively and qualitatively immaterial in the context of the MyoKardia Collaboration Agreement. Accordingly, the Company has disregarded its participation on the MyoKardia JSC as a performance obligation.

Determination of the Transaction Price

The Company received a non-refundable upfront payment of $10.0 million, which the Company included in the transaction price. Based on the uncertainty associated with the achievement of any preclinical and development milestone payments that the Company is eligible to receive, the Company has constrained the variable consideration associated with those milestone payments and excluded them from the transaction price. As part of its evaluation of constraining the preclinical and development milestones, the Company considered numerous factors, including the fact that the achievement of the preclinical and development milestones are contingent upon the results of the underlying preclinical and development activities and are thus outside of the control of the Company. The Company also included in the transaction price the expected amount of costs to be reimbursed for the MyoKardia Research Services, which includes the $2.5 million prepaid research funding payment that the Company received in the third quarter of 2020. The Company reassesses the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, and, if necessary, adjusts its estimate of the transaction price.

Any consideration related to sales milestone payments (including royalties) will be recognized when the related sales occur as these amounts have been determined to relate predominantly to the license granted to MyoKardia and therefore are recognized at the later of when the related sales occur or the performance obligation is satisfied.

Allocation of the Transaction Price to Performance Obligations

As noted above, the Company has identified a single performance obligation associated with the MyoKardia Collaboration Agreement. Therefore, the Company will allocate the entire amount of the transaction price to the identified single performance obligation.

Recognition of Revenue

The Company recognizes revenue related to the MyoKardia Collaboration Agreement over time as the MyoKardia Research Services are rendered. The Company has concluded that an input method is a representative depiction of the transfer of services under the MyoKardia Collaboration Agreement. The method of measuring progress towards the delivery of the services incorporates actual cumulative internal and external costs incurred relative to total internal and external costs expected to be incurred to satisfy the performance obligation. The period over which total costs are estimated reflects the Company's estimate of the period over which it will perform the MyoKardia Research Services. Changes in estimates of total internal and external costs expected to be incurred are recognized in the period of change as a cumulative catch-up adjustment.

During the three and nine months ended September 30, 2020, the Company recognized $0.3 million of collaboration revenue associated with the MyoKardia Collaboration Agreement. For the three and nine months ended September 30, 2019, the Company did not recognize any revenue under the MyoKardia Collaboration Agreement as it had not yet entered into the MyoKardia Collaboration Agreement. As of September 30, 2020, the Company recorded deferred revenue of $12.2 million associated with the MyoKardia Collaboration Agreement, which is classified as either current or net of current portion in the accompanying consolidated balance

sheets based on the period over which the revenue is expected to be recognized. As of December 31, 2019, the Company recorded no deferred revenue associated with the MyoKardia Collaboration Agreement as it had not yet entered into the MyoKardia Collaboration Agreement. The aggregate deferred revenue balance represents the aggregate amount of the transaction price allocated to the performance obligations that are unsatisfied as of September 30, 2020. As of September 30, 2020, the Company had not received any milestone, royalty, or cost reimbursement payments under the MyoKardia Collaboration Agreement, other than the $2.5 million payment as prepaid research funding in July 2020. As of September 30, 2020, the Company has recorded no accounts receivable related to reimbursable MyoKardia Research Services costs under the MyoKardia Collaboration Agreement.

11. Asset Acquisition

In February 2019, the Company entered into the GSK Agreement, pursuant to which the Company has been granted an exclusive worldwide license to develop and commercialize losmapimod. Under the GSK Agreement, the Company also acquired reference rights to relevant regulatory and manufacturing documents and GSK's existing supply of losmapimod drug substance and product. The Company also has the right to sublicense its rights under the license agreement, subject to certain conditions. The Company is obligated to use commercially reasonable efforts to develop and commercialize losmapimod at its sole cost. The Company is also responsible for costs related to the filing and maintenance of the licensed patent rights.

Under the GSK Agreement, the Company issued 12,500,000 shares of Series B Preferred Stock to GSK with an estimated fair value of $25.5 million, or $2.04 per share, which was determined with the assistance of a third-party specialist contemporaneously with the issuance of the Series B Preferred Stock to GSK. In addition, the Company may owe GSK up to $37.5 million in certain specified clinical and regulatory milestones, including $2.5 million due upon the initiation of a Phase 2 clinical trial, which was achieved and paid during the third quarter of 2019, and up to $60.0 million in certain specified sales milestones. The Company has agreed to pay tiered royalties on annual net sales of losmapimod that range from mid single-digit percentages to a low double-digit, but less than teens, percentage. The royalties are payable on a product-by-product and country-by-country basis, and may be reduced in specified circumstances. The Company also incurred $0.1 million of direct expenses related to the transaction, which the Company included in the total consideration for the transaction.

The GSK Agreement may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the GSK Agreement will continue in effect until the expiration of the Company's royalty obligations, which expire on a country-by-country basis on the later of (i) ten years after the first commercial sale in the country or (ii) approval of a generic version of losmapimod by the applicable regulatory agency.

The Company concluded the arrangement did not result in the acquisition of a business, as substantially all of the fair value of the gross assets acquired was concentrated in a single in-process research and development asset, losmapimod. In addition, the Company did not obtain any substantive processes in connection to the GSK Agreement and losmapimod was not generating revenue at the time the GSK Agreement was executed. Therefore, the Company accounted for the arrangement as an asset acquisition. The Company also concluded that the acquired assets do not have an alternative future use, and therefore the fair value attributable to the GSK Agreement of $25.6 million, inclusive of transaction costs, was recorded as in-process research and development expense (a component of research and development expenses) in the Company's consolidated statement of operations and comprehensive loss during the first quarter of 2019, which is the period in which the Company obtained (i) the license to losmapimod, (ii) the right to reference relevant regulatory and manufacturing documents, and (iii) GSK's existing supply of losmapimod drug substance and product. Additionally, the Company will recognize clinical and regulatory milestone payments when the underlying contingency is resolved and the consideration is paid or becomes payable. The milestone payments will be capitalized or expensed depending on the nature of the associated asset as of the date of recognition. The Company will record sales milestone payments and royalties as additional expense of the related product sales in the period in which the corresponding sales occur.

12. Commitments and Contingencies

Operating Leases

In November 2017, the Company entered into a lease agreement for its current corporate headquarters for approximately 28,731 square feet of office and laboratory space in Cambridge, Massachusetts. The lease has a total commitment of $25.1 million over the ten year term, and includes escalating rent payments. The lease agreement requires the Company to either pay a security deposit or maintain a letter of credit of $1.1 million. The Company maintains a letter of credit for this lease and has recorded the cash held to secure the letter of credit as restricted cash on the consolidated balance sheet as of September 30, 2020 and December 31, 2019. Rent expense associated with this lease for each of the three months ended September 30, 2020 and 2019 was approximately $0.5 million. Rent expense associated with this lease for each of the nine months ended September 30, 2020 and 2019 was approximately $1.4 million.

The future minimum lease payments associated with the lease for the Company's current headquarters as of September 30, 2020, are as follows (in thousands):

2020[1]	$ 579
2021	2,354
2022	2,424
2023	2,497
2024	2,572
Thereafter	9,615
Total minimum lease payments	$ 20,041

(1) Amounts are for the three months ending December 31, 2020.

Other Agreements

The Company has agreements with third parties in the normal course of business under which it can license certain developed technologies. If the Company exercises its rights to license the technologies it may be subject to additional fees and milestone payments. As of September 30, 2020, the Company has not exercised its rights to license such technologies.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters arising out of the relationship between such parties and the Company. In addition, the Company has entered into indemnification agreements with members of its board of directors and senior management that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any claims under indemnification arrangements, and it has not accrued any liabilities related to such obligations as of September 30, 2020 or December 31, 2019.

Legal Proceedings

The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses the costs related to its legal proceedings as they are incurred. No such costs have been incurred during the three and nine months ended September 30, 2020 and 2019.

13. Defined Contribution Plan

The Company has a defined contribution savings plan under Section 401(k) of the Internal Revenue Code (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants the option to elect to defer a portion of their annual compensation on a pretax basis. As currently established, the Company is not required to make and has not made any contributions to the 401(k) Plan.

14. Net Loss per Share

The following common stock equivalents were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Outstanding stock options	2,868,693	1,981,457	2,868,693	1,981,457
Unvested restricted stock awards	181,213	782,548	181,213	782,548
Total	3,049,906	2,764,005	3,049,906	2,764,005

15. Related-Party Transactions

During the three and nine months ended September 30, 2019, the Company paid fees to Third Rock Ventures, LLC ("TRV"), an affiliate of one of the Company's principal stockholders, in exchange for consulting services. The Company recorded expenses related to such fees of less than $0.1 million during the nine months ended September 30, 2019. The Company did not record expenses related to such fees during the three and nine months ended September 30, 2020 or the three months ended September 30, 2019. During the three and nine months ended September 30, 2020, the Company did not pay fees to TRV in exchange for services. As of December 31, 2019, there was less than $0.1 million of amounts due to TRV for such services that were included in accounts payable and accrued expenses. As of September 30, 2020, there were no amounts due to TRV for such services that were included in accounts payable and accrued expenses. Additionally, consultants that provide services to the Company are employees of TRV. The Company has issued an aggregate of 142,284 shares of common stock to these consultants in exchange for their continuing consulting services.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on March 5, 2020. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company focused on improving the lives of patients with genetically defined rare diseases in areas of high unmet medical need. We have developed a proprietary product engine that we employ to systematically identify and validate cellular drug targets that can potentially modulate gene expression to treat the known root cause of genetically defined diseases. We are using our product engine to identify targets that can be drugged by small molecules regardless of the particular underlying mechanism of gene mis-expression. We have identified drug targets to treat the root causes of facioscapulohumeral muscular dystrophy, or FSHD, and certain hemoglobinopathies, namely sickle cell disease, or SCD, and ß-thalassemia. In August 2019, we initiated a Phase 2b clinical trial, known as ReDUX4, and a Phase 2 open label clinical trial of losmapimod, our product candidate for FSHD, to evaluate the efficacy and safety of losmapimod in addressing the underlying cause of FSHD. We submitted an investigational new drug application, or IND, for FTX-6058, our product candidate for certain hemoglobinopathies, in the third quarter of 2020, which came into effect in October 2020. We plan to begin dosing subjects in a Phase 1 clinical trial of FTX-6058, to evaluate safety, tolerability and pharmacokinetics of FTX-6058 in healthy volunteers, in the fourth quarter of 2020. FTX-6058 is a novel upregulator of fetal hemoglobin.

ReDUX4 is a randomized, double-blind, placebo-controlled multicenter international Phase 2b clinical trial in 80 subjects with FSHD to investigate the efficacy and safety of oral administration of losmapimod 15 mg twice per day. The primary endpoint is to evaluate the reduction of DUX4-driven gene expression in affected skeletal muscle biopsies. The original design of ReDUX4 included a muscle biopsy at week 16 during the 24-week treatment period followed by an open label extension. Sixteen of the 80 subjects have completed the 24-week treatment period and rolled over to the open label extension portion of the trial.

As a result of the COVID-19 pandemic, we have extended the ReDUX4 treatment period from 24 to 48 weeks through a protocol amendment to ensure the safety of the subjects and to allow for the opportunity for a biopsy at week 16 as originally intended or at week 36. Approximately 68 subjects who did not complete the original 24-week treatment period remain active in the randomized portion of the trial. The extension from 24 to 48 weeks also allows for a longer assessment in a placebo-controlled design of the skeletal muscle MRI secondary endpoint and the various exploratory clinical endpoints, such as reachable workspace, optimized time up and go test for FSHD, muscle function measures and patient reported outcomes. We expect to present full data from the trial in the second quarter of 2021. We no longer intend to report topline data on the primary endpoint in the first quarter of 2021 because we believe that reporting the full data from the trial, including the primary endpoint, will allow for a more comprehensive assessment of the efficacy and safety of losmapimod for the treatment of FSHD. We believe that the amendment to the trial protocol provides flexibility to address the challenges presented by the COVID-19 pandemic and supports collection of efficacy and safety data to support continued discussions with regulatory agencies regarding potential registration strategies.

In August 2020, we announced results from a pre-specified interim analysis of the primary endpoint of the ReDUX4 trial, which is the reduction from baseline of DUX4-driven gene expression in affected skeletal muscle after subjects have been treated with losmapimod or placebo. Secondary and exploratory endpoints were not assessed as part of this analysis. Results from the interim analysis in the first 29 randomized subjects indicate that DUX4-driven gene expression did not show a separation from placebo at 16 weeks. However, in a pre-specified sensitivity analysis, those with the highest pre-treatment DUX4-driven gene expression in their muscle biopsy sample showed a large reduction in DUX4-driven gene expression following treatment with losmapimod compared to placebo. The highest expressing muscle biopsies represent the top quartile of biopsies assessed based on baseline DUX4-driven gene expression.

The interim results included an analysis of the first 29 subjects who completed their 16-week biopsy out of the 80 subjects enrolled. Pharmacokinetics, demographics and the primary endpoint were assessed. The interim analysis was not powered for statistical significance and did not include individual patient level data. Subjects were randomized to receive an oral dose of losmapimod 15mg (n=15) or placebo (n=14) twice per day. While results showed a significant reduction in DUX4-driven gene expression in the muscle biopsies of subjects whose baseline biopsy showed the highest levels of DUX4 gene expression (38-fold decrease with losmapimod, n=3, and 5.4 fold-decrease with placebo, n=5), the population level data analysis of the reduction in DUX4-driven gene expression from all 29 subjects did not show a separation of losmapimod from placebo (3.7 fold increase with losmapimod, n=15, and 2.8 fold increase with placebo, n=14). Results indicated that muscle biopsies within the higher range of DUX4-driven gene expression at baseline may be needed to observe a reduction.

In June 2020, we announced plans to evaluate losmapimod as a potential treatment for patients with COVID-19. On June 24, 2020, we announced that we received notification from the U.S. Food and Drug Administration, or FDA, that we may proceed with initiating a Phase 3, randomized, double-blind, placebo-controlled trial of losmapimod in higher risk hospitalized adults with COVID-19, or the LOSVID trial. The LOSVID trial is a Phase 3, international, multicenter trial designed to assess the safety and efficacy of a 15 mg twice per day oral dose of losmapimod compared to placebo for 14 days on top of standard of care in approximately 400 patients hospitalized with COVID-19 and at risk of progression to critical illness based on older age and elevated systemic inflammation. We began enrolling patients in the fourth quarter of 2020. The primary endpoint of the trial is the proportion of patients who progress to death or respiratory failure by day 28. The trial's secondary endpoints include clinical status on days seven and 14 as measured on the nine point World Health Organization ordinal scale of COVID-19 severity, total number of study days free of oxygen supplementation, all-cause mortality, length of hospitalization and intensive care unit stay, adverse events and viral clearance. We intend to conduct an interim analysis for futility and sample size re-estimation using an independent data monitoring committee when approximately 50 percent of subjects complete the 28-day visit. We expect to provide an update regarding the timing of the LOSVID trial during the first quarter of 2021.

In October 2020, we announced that our IND came into effect for our Phase 1 clinical trial of FTX-6058 in healthy adult volunteers. In this trial, we are evaluating the safety, tolerability and pharmacokinetics of FTX-6058. The trial is comprised of four parts. Part A will be a randomized, double-blind, placebo-controlled, single ascending dose (SAD) study in up to six cohorts. Part B will be a randomized, double-blind, placebo-controlled, multiple ascending dose (MAD) study in up to four cohorts dosed once daily for 14 days. Part C will be an open label pilot food effect study in subjects randomized to take FTX-6058 with and without a high-fat meal, and Part D will be an open label study to evaluate the potential of FTX-6058 to induce a liver enzyme known as CYP3A, which is involved in drug metabolism (using midazolam).

Since inception, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property, building our discovery platform, including our proprietary compound library and product engine, identifying drug targets and potential product candidates, in-licensing assets, producing drug substance and drug product material for use in clinical trials and conducting preclinical studies and clinical trials. To date, we have primarily funded our operations through the issuance of common stock in a private placement and in our initial public offering, or IPO, the issuance of convertible preferred stock and convertible notes, and upfront payments received under our collaboration and license agreements.

We have incurred significant operating losses since our inception and we expect to continue to incur significant operating losses for the foreseeable future. Our ability to generate product revenue sufficient to achieve profitability, if ever, will depend heavily on the successful development and eventual commercialization of one or more of our product candidates. Our net losses were $19.0 million and $53.1 million for the three and nine months ended September 30, 2020, respectively, and $16.5 million and $66.6 million for the three and nine months ended September 30, 2019, respectively. As of September 30, 2020, we had an accumulated deficit of $203.9 million. We expect our expenses and operating losses will increase substantially over the next several years in connection with our ongoing activities, as we:

- continue our clinical development of losmapimod, including our ongoing Phase 2b and Phase 2 open label clinical trials for the treatment of FSHD, and our ongoing Phase 3 clinical trial for the treatment of COVID-19;
- continue our clinical development of FTX-6058, including our ongoing Phase 1 clinical trial in healthy volunteers;
- advance clinical-stage product candidates into later stage trials;
- pursue the discovery of drug targets for other rare diseases and the subsequent development of any resulting product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- scale up our manufacturing processes and capabilities, or arrange for a third party to do so on our behalf, to support our clinical trials of our product candidates and commercialization of any of our product candidates for which we obtain marketing approval;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval;
- acquire or in-license products, product candidates, technologies and/or data referencing rights;
- make any milestone payments to affiliates of GlaxoSmithKline plc, or GSK, under our right of reference and license agreement with GSK upon the achievement of specified clinical or regulatory milestones;
- maintain, expand, enforce, defend and protect our intellectual property;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts and our operations as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates, or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of increased expenses or the timing of when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of September 30, 2020, we had $127.0 million in cash, cash equivalents, and marketable securities. We believe that our existing cash, cash equivalents, and marketable securities as of September 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "—Liquidity and Capital Resources."

Components of Results of Operations

Revenue

We have not generated any revenue from product sales and do not expect to generate revenue from the sale of products for several years, if at all. If our development efforts for our current or future product candidates are successful and result in marketing approval, we may generate revenue in the future from product sales. We cannot predict if, when or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates.

In December 2019, we entered into a collaboration and license agreement, or the Acceleron Collaboration Agreement, with Acceleron Pharma Inc., or Acceleron, to identify biological targets to modulate specific pathways associated with a targeted indication within the pulmonary disease space. Under the Acceleron Collaboration Agreement, we granted Acceleron an exclusive worldwide license under certain intellectual property rights to make, have made, use, sell, have sold, import, export, distribute and have distributed, market, have marketed, promote, have promoted, or otherwise exploit molecules and products directed against or expressing certain biological targets identified by us for the treatment, prophylaxis, or diagnosis of a targeted indication within the pulmonary disease space. The primary goal of the collaboration is to identify and validate potential biological targets for further research in order to support the development, manufacture and commercialization of product candidates by Acceleron for the targeted indication by leveraging our proprietary product engine.

Under the terms of the Acceleron Collaboration Agreement, we received a $10.0 million upfront payment from Acceleron in December 2019. We are also eligible to receive up to $438.5 million in the aggregate in milestone payments with respect to certain research, developmental, clinical, regulatory and sales-related milestones, and tiered royalty payments based on Acceleron's (and any of its affiliates' and sublicensees') annual worldwide net sales of products directed to any identified targets.

For the three and nine months ended September 30, 2020, we recognized $1.5 million and $4.3 million, respectively, of collaboration revenue under the Acceleron Collaboration Agreement. For the three and nine months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the Acceleron Collaboration Agreement. As of September 30, 2020 and December 31, 2019, we have recorded $7.4 million and $10.0 million, respectively, of deferred revenue associated with the Acceleron Collaboration Agreement, which is classified as either current or net of current portion in our consolidated balance sheets based on the period over which the revenue is expected to be recognized. As of September 30, 2020, we had received $1.1 million of cost reimbursement payments and no milestone or royalty payments under the Acceleron Collaboration Agreement. As of September 30, 2020, we recorded unbilled accounts receivable of $0.6 million related to reimbursable research and development costs under the Acceleron Collaboration Agreement for activities performed during the three months ended September 30, 2020.

In the future, we will recognize additional revenue associated with the $10.0 million upfront payment as we satisfy our performance obligation, and from reimbursement of costs incurred under the Acceleron Collaboration Agreement, and we may generate additional revenue from milestones and royalty payments under the Acceleron Collaboration Agreement. We expect that our revenue will fluctuate from quarter-to-quarter and year-to-year based upon our pattern of performance under the Acceleron Collaboration Agreement and as a result of the timing, amount, and achievement of milestones and reimbursement of costs incurred under the Acceleron Collaboration Agreement.

On July 20, 2020, we entered into a collaboration and license agreement, or the MyoKardia Collaboration Agreement, with MyoKardia, Inc., or MyoKardia, pursuant to which we granted to MyoKardia an exclusive worldwide license under certain intellectual property rights to research, develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, export, have exported, distribute, have distributed, market, have marketed, promote, have promoted, or otherwise exploit products directed against certain biological targets identified by us that are capable of modulating up to a certain number of genes of interest with relevance to certain genetically defined cardiomyopathies. The primary goal of the collaboration is to identify and validate potential biological targets for further research, in order to support the development, manufacture and commercialization of product candidates by MyoKardia for the potential treatment of certain genetically defined cardiomyopathies.

Under the terms of the MyoKardia Collaboration Agreement, we received a $10.0 million upfront payment and a $2.5 million payment as prepaid research funding in July 2020. MyoKardia will also reimburse us for the costs of the research activities not covered by the prepaid research funding, up to a maximum amount of total research funding (including the prepaid research funding). Upon the achievement of specified preclinical, development and sales milestones, we will be entitled to preclinical milestone payments, development milestone payments and sales milestone payments of up to $298.5 million in the aggregate per target for certain potential cardiomyopathy gene targets, and of up to $150.0 million in the aggregate per target for certain other potential cardiomyopathy gene targets. MyoKardia will also pay us tiered royalties ranging from a mid single-digit percentage to a low double-digit percentage based on MyoKardia's, and any of its affiliates' and sublicensees', annual worldwide net sales of products under the MyoKardia Collaboration Agreement directed against any identified target. The royalties are payable on a product-by-product basis during a specified royalty term, and may be reduced in specified circumstances.

For each of the three and nine months ended September 30, 2020, we recognized $0.3 million of collaboration revenue under the MyoKardia Collaboration Agreement. For the three and nine months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the MyoKardia Collaboration Agreement. As of September 30, 2020, we have recorded $12.2 million of deferred revenue associated with the MyoKardia Collaboration Agreement, which is classified as either current or net of current portion in our consolidated balance sheets based on the period over which the revenue is expected to be recognized. We recorded no deferred revenue associated with the MyoKardia Collaboration Agreement as of December 31, 2019 as we had not yet entered into the MyoKardia Collaboration Agreement. As of September 30, 2020, we had received no cost reimbursement payments and no milestone or royalty payments under the MyoKardia Collaboration Agreement, other than the $2.5 million payment as prepaid research funding in July 2020. As of September 30, 2020, we recorded no accounts receivable related to reimbursable research and development costs under the MyoKardia Collaboration Agreement.

In the future, we will recognize additional revenue associated with the $10.0 million upfront payment, and from reimbursement of costs incurred under the MyoKardia Collaboration Agreement, including the $2.5 million prepaid research funding payment, and we may generate additional revenue from milestones and royalty payments under the MyoKardia Collaboration Agreement. We expect that our revenue will fluctuate from quarter-to-quarter and year-to-year based upon our pattern of performance under the MyoKardia Collaboration Agreement and as a result of the timing, amount, and achievement of milestones and reimbursement of costs incurred under the MyoKardia Collaboration Agreement.

We may also in the future enter into additional license or collaboration agreements for our product candidates or intellectual property, and we may generate revenue in the future from payments as a result of such license or collaboration agreements.

Operating Expenses

Research and Development Expenses

Research and development expenses represent costs incurred by us for the discovery, development, and manufacture of our product candidates and include:

- external research and development expenses incurred under agreements with contract research organizations, contract manufacturing organizations, and consultants;
- salaries, payroll taxes, employee benefits and stock-based compensation expenses for individuals involved in research and development efforts;

- laboratory supplies;
- in-process research and development, or IPR&D, expenses, which relate to IPR&D acquired as part of an asset acquisition for which there is no alternative future use;
- costs related to compliance with regulatory requirements; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, maintenance of facilities, insurance and other operating costs.

We expense research and development costs as incurred. We recognize expenses for certain development activities, such as clinical trials and manufacturing, based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment or other information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of expenses incurred. Non-refundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. These amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

External costs represent a significant portion of our research and development expenses, which we track on a program-by-program basis following the nomination of a development candidate. Our internal research and development expenses consist primarily of personnel-related expenses, including stock-based compensation expense. We do not track our internal research and development expenses on a program-by-program basis as the resources are deployed across multiple projects.

The following table summarizes our external research and development expenses by program following nomination as a development candidate for the three and nine months ended September 30, 2020 and 2019. Pre-development candidate expenses, unallocated expenses and internal research and development expenses are classified separately. Losmapimod external expenses include IPR&D expenses. We nominated FTX-6058 as a development candidate in the third quarter of 2019.

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	**2020**	**2019**	**2020**	**2019**
Losmapimod external expenses	$ 7,217	$ 5,492	$ 16,983	$ 36,367
FTX-6058 external expenses	1,381	1,383	3,720	1,383
Pre-development candidate expenses and unallocated expenses	3,382	3,413	10,303	12,113
Internal research and development expenses	3,660	3,208	11,891	9,122
Total research and development expenses	$ 15,640	$ 13,496	$ 42,897	$ 58,985

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing, and estimated costs of the efforts that will be necessary to complete the remainder of the development of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from our product candidates, if approved. This is due to the numerous risks and uncertainties associated with developing our product candidates, including the uncertainty related to:

- the timing and progress of preclinical and clinical development activities, including in light of COVID-19;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to raise additional funds necessary to complete clinical development of and commercialize our product candidates;
- our ability to maintain our current research and development programs and to establish new ones;
- our ability to establish new licensing or collaboration arrangements;
- the progress of the development efforts of parties with whom we may enter into collaboration arrangements;
- the successful initiation and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;

- the receipt and related terms of regulatory approvals from applicable regulatory authorities;
- the availability of raw materials and active pharmaceutical ingredient, or API, for use in production of our product candidates;
- our ability to establish and operate a manufacturing facility, or secure manufacturing supply through relationships with third parties;
- our ability to consistently manufacture our product candidates in quantities sufficient for use in clinical trials;
- our ability to obtain and maintain intellectual property protection and regulatory exclusivity, both in the United States and internationally;
- our ability to maintain, enforce, defend and protect our rights in our intellectual property portfolio;
- the commercialization of our product candidates, if and when approved;
- our ability to obtain and maintain third-party coverage and adequate reimbursement for our product candidates, if approved;
- the acceptance of our product candidates, if approved, by patients, the medical community and third-party payors;
- competition with other products; and
- a continued acceptable safety profile of our products following receipt of any regulatory approvals.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate, and potentially other candidates.

Research and development activities account for a significant portion of our operating expenses. We expect our research and development expenses to increase significantly in future periods as we continue to implement our business strategy, which includes advancing losmapimod, for the treatment of both FSHD and COVID-19, and FTX-6058, expanding our research and development efforts, including hiring additional personnel to support our research and development efforts, and seeking regulatory approvals for our product candidates that successfully complete clinical trials. In addition, product candidates in later stages of clinical development generally incur higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect our research and development expenses to increase as our product candidates advance into later stages of clinical development. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development.

General and Administrative Expenses

General and administrative expenses consist of personnel-related costs, including salaries, benefits and stock-based compensation expense, for our personnel in executive, finance and accounting, human resources, business operations and other administrative functions, legal fees related to patent, intellectual property and corporate matters, fees paid for accounting and tax services, consulting fees and facility-related costs not otherwise included in research and development expenses.

We expect our general and administrative expenses will increase for the foreseeable future to support our expanded infrastructure and increased costs of expanding our operations and operating as a public company. These increases will likely include increased expenses related to accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums, and investor relations costs associated with operating as a public company.

Other Income, Net

Other income, net consists primarily of interest income related to our investments in cash equivalents and marketable securities.

Results of Operations

Comparison of the Three Months Ended September 30, 2020 and 2019

The following summarizes our results of operations for the three months ended September 30, 2020 and 2019, along with the changes in those items in dollars:

	Three Months Ended September 30,		Change
(in thousands)	**2020**	**2019**	**$**
Collaboration revenue	$ 1,848	$ —	$ 1,848
Operating expenses:			
Research and development	15,640	13,496	2,144
General and administrative	5,312	3,510	1,802
Total operating expenses	20,952	17,006	3,946
Loss from operations	(19,104)	(17,006)	(2,098)
Other income, net	142	464	(322)
Net loss	$ (18,962)	$ (16,542)	$ (2,420)

Collaboration Revenue

Collaboration revenue was $1.8 million for the three months ended September 30, 2020. We did not record any collaboration revenue during the three months ended September 30, 2019. We recognize revenue under each of the Acceleron Collaboration Agreement and MyoKardia Collaboration Agreement based on our pattern of performance related to the respective identified performance obligation, which is the period over which we will perform research services under each of the Acceleron Collaboration Agreement and MyoKardia Collaboration Agreement. For the three months ended September 30, 2020, we recognized $1.5 million of collaboration revenue under the Acceleron Collaboration Agreement. For the three months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the Acceleron Collaboration Agreement. For the three months ended September 30, 2020, we recognized $0.3 million of collaboration revenue under the MyoKardia Collaboration Agreement. For the three months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the MyoKardia Collaboration Agreement.

Research and Development Expenses

Research and development expense increased by $2.1 million from $13.5 million for the three months ended September 30, 2019 to $15.6 million for the three months ended September 30, 2020. The increase in research and development expense was primarily attributable to the following:

- $2.2 million in increased manufacturing costs associated with the production of losmapimod and FTX-6058 to support our ongoing and planned clinical trials;
- $1.7 million in increased costs for external clinical activities as we continued our ongoing Phase 2b and Phase 2 open label clinical trials of losmapimod for the treatment of FSHD and Phase 3 clinical trial of losmapimod for the treatment of COVID-19, and as we prepared to advance FTX-6058 into a Phase 1 clinical trial;
- $0.5 million in increased personnel-related costs due to increased headcount, including $0.2 million of increased stock-based compensation expense; and
- $2.5 million in decreased costs associated with the achievement of a milestone due under the right of reference and license agreement with GSK upon the initiation of a Phase 2 clinical trial of losmapimod, which was achieved during the three months ended September 30, 2019.

General and Administrative Expenses

General and administrative expenses increased by $1.8 million from $3.5 million for the three months ended September 30, 2019 to $5.3 million for the three months ended September 30, 2020. The increase in general and administrative expenses was primarily attributable to the following:

- $1.1 million in increased consulting and professional fees, including for legal services, insurance premiums, recruiting, accounting, and tax; and
- $0.7 million in increased personnel-related costs, primarily due to increased general and administrative headcount to support the growth of our research and development organization, including $0.4 million of increased stock-based compensation expense.

Other Income, Net

Other income, net decreased by $0.4 million from $0.5 million for the three months ended September 30, 2019 to $0.1 million for the three months ended September 30, 2020. The decrease in other income, net was primarily attributable to a decrease in investment income on our cash, cash equivalents, and marketable securities as a result of an overall decreased rate of return.

Comparison of the Nine Months Ended September 30, 2020 and 2019

The following summarizes our results of operations for the nine months ended September 30, 2020 and 2019, along with the changes in those items in dollars:

	Nine Months Ended September 30,		Change
(in thousands)	**2020**	**2019**	**$**
Collaboration revenue	$ 4,598	$ —	$ 4,598
Operating expenses:			
Research and development	42,897	58,985	(16,088)
General and administrative	15,525	8,742	6,783
Total operating expenses	58,422	67,727	(9,305)
Loss from operations	(53,824)	(67,727)	13,903
Other income, net	725	1,173	(448)
Net loss	$ (53,099)	$ (66,554)	$ 13,455

Collaboration Revenue

Collaboration revenue was $4.6 million for the nine months ended September 30, 2020. We did not record any collaboration revenue during the nine months ended September 30, 2019. We recognize revenue under each of the Acceleron Collaboration Agreement and MyoKardia Collaboration Agreement based on our pattern of performance related to the respective identified performance obligation, which is the period over which we will perform research services under each of the Acceleron Collaboration Agreement and MyoKardia Collaboration Agreement. For the nine months ended September 30, 2020, we recognized $4.3 million of collaboration revenue under the Acceleron Collaboration Agreement. For the nine months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the Acceleron Collaboration Agreement. For the nine months ended September 30, 2020, we recognized $0.3 million of collaboration revenue under the MyoKardia Collaboration Agreement. For the nine months ended September 30, 2019, we did not recognize any collaboration revenue as we had not yet entered into the MyoKardia Collaboration Agreement.

Research and Development Expenses

Research and development expense decreased by $16.1 million from $59.0 million for the nine months ended September 30, 2019 to $42.9 million for the nine months ended September 30, 2020. The decrease in research and development expense was primarily attributable to a $25.6 million decrease in IPR&D expenses associated with the recognition of the fair value attributable to the right of reference and license agreement with GSK during the nine months ended September 30, 2019 as well as a $2.5 million decrease in milestone expense associated with the milestone payable to GSK upon the initiation of a Phase 2 clinical trial of losmapimod during the nine months ended September 30, 2019, partially offset by increases in research and development expenses as a result of the following:

- $4.7 million in increased manufacturing costs associated with the production of losmapimod and FTX-6058 to support our ongoing and planned clinical trials;
- $3.8 million in increased costs for external clinical activities as we continued our ongoing Phase 2b and Phase 2 open label clinical trials of losmapimod for the treatment of FSHD and Phase 3 clinical trial of losmapimod for the treatment of COVID-19, and as we prepared to advance FTX-6058 into a Phase 1 clinical trial;
- $3.0 million in increased personnel-related costs due to increased headcount, including $1.3 million of increased stock-based compensation expense; and
- $0.8 million in increased costs for IND-enabling studies for FTX-6058.

General and Administrative Expenses

General and administrative expenses increased by $6.8 million from $8.7 million for the nine months ended September 30, 2019 to $15.5 million for the nine months ended September 30, 2020. The increase in general and administrative expenses was primarily attributable to the following:

- $3.6 million in increased consulting and professional fees, including for legal services, insurance premiums, recruiting, accounting, and tax; and
- $3.0 million in increased personnel-related costs, primarily due to increased general and administrative headcount to support the growth of our research and development organization, including $1.4 million of increased stock-based compensation expense.

Other Income, Net

Other income, net decreased by $0.4 million from $1.2 million for the nine months ended September 30, 2019 to $0.7 million for the nine months ended September 30, 2020. The decrease in other income, net was primarily attributable to a decrease in investment income on our cash, cash equivalents, and marketable securities as a result of an overall decreased rate of return.

Liquidity and Capital Resources

Sources of Liquidity

We have incurred significant operating losses since our inception and expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. We have not yet commercialized any of our product candidates, which are in various phases of preclinical and clinical development, and we do not expect to generate revenue from sales of any products for several years, if at all. Through September 30, 2020, we have primarily funded our operations with aggregate gross proceeds of $300.5 million from the issuance of common stock in a private placement and in our IPO, the issuance of convertible preferred stock and convertible notes, an upfront payment received under the Acceleron Collaboration Agreement, and an upfront payment received under the MyoKardia Collaboration Agreement. As of September 30, 2020, we had cash, cash equivalents, and marketable securities of $127.0 million. On July 22, 2019, we completed an IPO of our common stock and issued and sold 4,500,000 shares of common stock at a public offering price of $16.00 per share, resulting in net proceeds of $63.9 million after deducting underwriting discounts and commissions and offering expenses. On June 9, 2020, we issued and sold 4,029,411 shares of common stock in a private placement at a price of $17.00 per share, resulting in gross proceeds of $68.5 million, before deducting offering costs of $4.2 million. In December 2019, we received a $10.0 million upfront payment upon execution of the Acceleron Collaboration Agreement. In July 2020, we received a $10.0 million upfront payment and a $2.5 million payment as prepaid research funding under the MyoKardia Collaboration Agreement.

On August 11, 2020, we filed a shelf registration statement on Form S-3 with the SEC, which was declared effective on August 17, 2020, or the Shelf Registration Statement. Under the Shelf Registration Statement, we may offer and sell up to $250.0 million of a variety of securities including common stock, preferred stock, warrants, depositary shares, debt securities or units during the three-year period the commenced upon the Shelf Registration Statement becoming effective. In connection with the filing of the Shelf Registration Statement, we entered into an Equity Distribution Agreement with Piper Sandler & Co., or Piper Sandler, as sales agent, pursuant to which we may offer and sell shares of our common stock with an aggregate offering price of up to $75.0 million under an "at-the-market" offering program, or the ATM Offering. To date, we have not sold any securities under the Shelf Registration Statement or shares under the ATM Offering.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2020 and 2019:

	Nine Months Ended September 30,	
(in thousands)	**2020**	**2019**
Net cash used in operating activities	$ (33,829)	$ (34,988)
Net cash used in investing activities	(42,540)	(836)
Net cash provided by financing activities	64,877	64,624
Net (decrease) increase in cash, cash equivalents, and restricted cash	$ (11,492)	$ 28,800

Net Cash Used in Operating Activities

Net cash used in operating activities was $33.8 million during the nine months ended September 30, 2020 compared to net cash used in operating activities of $35.0 million during the nine months ended September 30, 2019. The decrease in net cash used in operating activities of $1.2 million was primarily due to the receipt of a $10.0 million upfront payment and a $2.5 million payment as prepaid research funding associated with the MyoKardia Collaboration Agreement during the nine months ended September 30, 2020, partially offset by increased personnel-related costs, increased research and development costs as we continue to advance our lead programs, and increased general and administrative costs associated with operating as a public company.

Net Cash Used in Investing Activities

Net cash used in investing activities was $42.5 million during the nine months ended September 30, 2020 compared to net cash used in investing activities of $0.8 million during the nine months ended September 30, 2019. The increase in net cash used in investing activities of $41.7 million was primarily due to net purchases of marketable securities during the nine months ended September 30, 2020.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $64.9 million during the nine months ended September 30, 2020 compared to net cash provided by financing activities of $64.6 million during the nine months ended September 30, 2019. Net cash provided by financing activities during the nine months ended September 30, 2020 primarily consisted of net proceeds of approximately $64.2 million from the completion of the private placement of the Company's common stock in June 2020. Net cash provided by financing activities during the nine months ended September 30, 2019 primarily consisted of net proceeds of approximately $64.4 million from the completion of our IPO in July 2019.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing research and development activities, particularly as we continue the research and development of, initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, we expect to incur additional costs associated with operating as a public company. As a result, we expect to incur substantial operating losses and negative operating cash flows for the foreseeable future.

Based on our current operating plan, we believe that our existing cash, cash equivalents, and marketable securities as of September 30, 2020, will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. However, we have based this estimate on assumptions that may prove to be wrong and we could exhaust our capital resources sooner than we expect.

Our funding requirements and timing and amount of our operating expenditures will depend largely on:

- the progress, costs and results of our ongoing Phase 2b and Phase 2 open label clinical trials of losmapimod for the treatment of FSHD, our ongoing Phase 3 clinical trial of losmapimod for the treatment of COVID-19, and our ongoing Phase 1 clinical trial of FTX-6058;
- the scope, progress, costs and results of discovery research, preclinical development, laboratory testing and clinical trials for our product candidates;
- the impact of the COVID-19 pandemic on our business and operations;
- the number of and development requirements for other product candidates that we pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to enter into contract manufacturing arrangements for supply of API and manufacture of our product candidates and the terms of such arrangements;
- the success of our collaborations with Acceleron and MyoKardia;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the costs and timing of future commercialization activities, including product manufacturing, sales, marketing and distribution, for any of our product candidates for which we may receive marketing approval;
- the amount and timing of revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other products, product candidates, technologies or data referencing rights.

A change in the outcome of any of these or other variables with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. We will need to continue to rely on additional financing to achieve our business objectives.

In addition to the variables described above, if and when any of our product candidates successfully complete development, we will incur substantial additional costs associated with regulatory filings, marketing approval, post-marketing requirements, maintaining our intellectual property rights, and regulatory protection, in addition to other commercial costs. We cannot reasonably estimate these costs at this time.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaboration arrangements, strategic alliances and marketing, distribution or licensing arrangements. We currently have no credit facility or committed sources of capital. To the extent that we raise additional capital through the future sale of equity securities, the ownership interests of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through the issuance of debt securities, these securities could contain covenants that would restrict our operations. We may require additional capital beyond our currently anticipated amounts, and additional capital may not be available on reasonable terms, or at all. If we raise additional funds through collaboration arrangements, strategic alliances or marketing, distribution or licensing arrangements in the future, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

During the three and nine months ended September 30, 2020, there have been no material changes to our contractual obligations and commitments outside the ordinary course of business from those described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations – Contractual Obligations" in our Annual Report on Form 10-K filed with the SEC on March 5, 2020.

Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting periods. Our estimates are based on our historical experience, known trends and events, and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities and amount of expense recognized that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We evaluate our estimates and assumptions on an ongoing basis. The effects of material revisions in estimates, if any, will be reflected in the consolidated financial statements prospectively from the date of change in estimates. During the three months ended September 30, 2020, there were no material changes to our critical accounting policies from those described in our Annual Report on Form 10-K filed with the SEC on March 5, 2020.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

Emerging Growth Company Status

The Jumpstart Our Business Startups Act of 2012 permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our cash equivalents are in the form of money market funds that are invested in U.S. Treasury securities. As of September 30, 2020, we had cash, cash equivalents, and marketable securities of $127.0 million. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio.

We are also exposed to market risk related to changes in foreign currency exchange rates. We contract with vendors that are located outside of the United States and certain invoices are denominated in foreign currencies. We are subject to fluctuations in foreign currency rates in connection with these arrangements. We do not currently hedge our foreign currency exchange rate risk. As of September 30, 2020, we had minimal or no liabilities denominated in foreign currencies.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the three and nine months ended September 30, 2020 and 2019.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Operating Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2020. 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, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2020, our Chief Executive Officer and Chief Operating Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15(d)-15(f) under the Exchange Act) 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 1A. Risk Factors.

Our future operating results could differ materially from the results described in this Quarterly Report on Form 10-Q due to the risks and uncertainties described below. You should consider carefully the following information about risks below in evaluating our business. If any of the following risks actually occur, our business, financial conditions, results of operations and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline. In addition, we cannot assure investors that our assumptions and expectations will prove to be correct. Important factors could cause our actual results to differ materially from those indicated or implied by forward-looking statements. See page i of this Quarterly Report on Form 10-Q for a discussion of some of the forward-looking statements that are qualified by these risk factors. Factors that could cause or contribute to such differences include those factors discussed below.

Risks Related to our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was $82.7 million for the year ended December 31, 2019 and $53.1 million for the nine months ended September 30, 2020. As of September 30, 2020, we had an accumulated deficit of $203.9 million. To date, we have primarily funded our operations through the issuance of common stock in a private placement and in our initial public offering, or IPO, the issuance of convertible preferred stock and convertible notes, an upfront payment received under the collaboration and license agreement with Acceleron Pharma Inc., or Acceleron, and an upfront payment received under the collaboration and license agreement, or the MyoKardia Collaboration Agreement, with MyoKardia, Inc., or MyoKardia. We have devoted substantially all of our financial resources and efforts to research and development, including clinical trials and preclinical studies. We are still in the early stages of development of our product candidates, and we have not completed development of any product candidates. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue our clinical development of losmapimod, including our ongoing Phase 2b and Phase 2 open label clinical trials for the treatment of facioscapulohumeral muscular dystrophy, or FSHD, and our ongoing Phase 3 clinical trial for the treatment of COVID-19;
- continue our clinical development of FTX-6058, including our ongoing Phase 1 clinical trial in healthy volunteers;
- advance clinical-stage product candidates into later stage trials;
- pursue the discovery of drug targets for other rare diseases and the subsequent development of any resulting product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- scale up our manufacturing processes and capabilities, or arrange for a third party to do so on our behalf, to support our clinical trials of our product candidates and commercialization of any of our product candidates for which we may obtain marketing approval;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval;
- acquire or in-license products, product candidates, technologies and/or data referencing rights;
- make any milestone payments to affiliates of GlaxoSmithKline plc, or GSK, under our right of reference and license agreement with GSK upon the achievement of specified clinical or regulatory milestones;
- maintain, expand, enforce, defend and protect our intellectual property;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts and our operations as a public company.

To become and remain profitable, we must succeed in developing, and eventually commercializing, a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, discovering additional product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling any products for which we may obtain regulatory approval. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Our expenses will increase if, among other things:

- we are required by the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or the EMA, or other regulatory authorities to perform trials or studies in addition to, or different than, those expected;
- there are any delays in completing our clinical trials or the development of any of our product candidates; or
- there are any third-party challenges to our intellectual property or we need to defend against any intellectual property-related claim.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or even continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect to devote substantial financial resources to our ongoing and planned activities, particularly as we continue our Phase 2b and Phase 2 open label clinical trials of losmapimod for the treatment of FSHD, continue our recently initiated Phase 3 clinical trial of losmapimod for the treatment of COVID-19, and continue our recently initiated Phase 1 clinical trial of FTX-6058, and continue research and development and initiate additional clinical trials of, and seek regulatory approval for, these and other product candidates. We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our preclinical activities and clinical trials. In addition, if we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution. Furthermore, we will incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Our future capital requirements will depend on many factors, including:

- the progress, costs and results of our ongoing Phase 2b and Phase 2 open label clinical trials of losmapimod for the treatment of FSHD, our ongoing Phase 3 clinical trial of losmapimod for the treatment of COVID-19, and our ongoing Phase 1 clinical trial of FTX-6058;
- the scope, progress, costs and results of discovery research, preclinical development, laboratory testing and clinical trials for our product candidates;
- the impact of the COVID-19 pandemic on our business and operations;
- the number of and development requirements for other product candidates that we pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to enter into contract manufacturing arrangements for supply of active pharmaceutical ingredient, or API, and manufacture of our product candidates and the terms of such arrangements;
- the success of our collaborations with Acceleron and MyoKardia;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the costs and timing of future commercialization activities, including product manufacturing, sales, marketing and distribution, for any of our product candidates for which we may receive marketing approval;

- the amount and timing of revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other products, product candidates, technologies or data referencing rights.

As of September 30, 2020, we had cash, cash equivalents, and marketable securities of approximately $127.0 million. We believe that our cash, cash equivalents, and marketable securities as of September 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Commercial revenues, if any, will not be derived unless and until we can achieve sales of products, which we do not anticipate for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce or terminate preclinical studies, clinical trials or other development activities for one or more of our product candidates or discovery stage programs or delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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

We commenced activities in 2015 and are an early-stage company. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, establishing our intellectual property, building our discovery platform, identifying drug targets and potential product candidates, in-licensing assets, producing drug substance and drug product material for use in clinical trials and undertaking preclinical studies and conducting clinical trials. We have not yet demonstrated our ability to successfully develop any product candidate, obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions stockholders make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

In addition, as our business grows, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

The ongoing COVID-19 pandemic has and may continue to affect our ability to initiate and complete current or future preclinical studies or clinical trials, disrupt regulatory activities or have other adverse effects on our business and operations. In addition, this pandemic may continue to adversely impact economies worldwide, which could result in adverse effects on our business and operations.

The ongoing COVID-19 pandemic has caused many governments to implement measures to slow the spread of the outbreak through quarantines, travel restrictions, heightened border scrutiny, and other measures. The outbreak and government measures taken in response have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The future progression of the outbreak and its effects on our business and operations are uncertain.

We and our contract manufacturing organizations, or CMOs, and contract research organizations, or CROs, may face disruptions that may affect our ability to initiate and complete preclinical studies or clinical trials, including disruptions in procuring items that are essential for our research and development activities, including, for example, raw materials and API used in the manufacturing of our product candidates, and laboratory supplies for our current and future preclinical studies and clinical trials, in each case, for which there may be shortages because of ongoing efforts to address the outbreak. We and our CMOs and CROs, may face disruptions related to our planned and ongoing clinical trials or future clinical trials arising from delays in IND-enabling studies, manufacturing disruptions, and the ability to obtain necessary institutional review board or other necessary site approvals, as well as enrollment and other delays at clinical trial sites. For example, in the wake of COVID-19, the clinical trial sites for our Phase 2b clinical trial temporarily postponed trial-related activities, impacting our clinical trial execution plans. We may also face difficulties recruiting or retaining patients for our planned and ongoing clinical trials if patients are affected by the virus or are fearful of visiting or traveling to clinical trial sites because of the outbreak. The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

Additionally, the impact of the COVID-19 outbreak in Massachusetts resulted in a temporary reduction in workforce presence at our Cambridge research facility. While we increased workforce presence at our facilities starting in the second quarter of 2020, not all employees have returned to our facility and we cannot be certain that we will not be required to close our facilities in the future as a result of the COVID-19 outbreak. A closure of our facility may substantially impact our discovery and translational activities and may delay the experimentation needed to identify novel drug targets, prosecute such targets, identify development candidates for such targets and identify biomarkers that inform the potential clinical development paths for such targets. Moreover, discovery and implementation of clinical biomarker assays for ongoing clinical trials may be delayed. Furthermore, any negative impact that the outbreak has on the ability of our CROs to deliver data sets and execute on experimentation could cause substantial delays for our discovery activities and materially impact our ability to fuel our pipeline with new product candidates.

The pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to adversely affect our business, financial condition, results of operations and prospects.

In the past, we have identified conditions and events that raise substantial doubt about our ability to continue as a going concern and it is possible that we may identify conditions and events in the future that raise substantial doubt about our ability to continue as a going concern.

Previously, we have identified conditions and events that raised substantial doubt about our ability to continue as a going concern, and our independent registered public accounting firm's report on our consolidated financial statements as of and for the year ended December 31, 2018 that was issued prior to our IPO included a going concern uncertainty paragraph. With the completion of our private placement of our common stock and IPO, we believe that our existing cash, cash equivalents, and marketable securities as of September 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. However, we have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. In the future, if we are unable to obtain sufficient funding to support our operations, we could be forced to delay, reduce or eliminate all of our research and development programs, product portfolio expansion or commercialization efforts, and our financial condition and results of operations will be materially and adversely affected and we may be unable to continue as a going concern. In the future, reports from our independent registered public accounting firm may also include a going concern uncertainty paragraph. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

Recent changes in tax law may adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted the Tax Cuts and Jobs Act, or the TCJA, which significantly reformed the U.S. Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for net operating losses arising in taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of net operating loss carrybacks for losses arising in taxable years ending after December 31, 2017 (though any such net operating losses may be carried forward indefinitely), the imposition of a one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, the elimination of U.S. tax on foreign earnings (subject to certain important exceptions), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

As part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, was enacted on March 27, 2020. Both contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of net operating losses, which was enacted as part of the TCJA. It also provides that net operating losses arising in any taxable year beginning after December 31, 2017, and before January 1, 2021 are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30% to 50% of adjusted taxable income.

Regulatory guidance under the TCJA, the FFCR Act and the CARES Act is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also possible that Congress will enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the TCJA, the FFCR Act or the CARES Act.

Our ability to use our net operating losses and research and development tax credit carryforwards to offset future taxable income may be subject to certain limitations.

As of December 31, 2019, we had federal and state net operating loss carryforwards of $111.6 million and $111.1 million, respectively, which begin to expire in 2035. Approximately $80.6 million of the federal net operating losses can be carried forward indefinitely. As of December 31, 2019, we also had federal and state research and development tax credit carryforwards of $2.8 million and $2.4 million, respectively, which begin to expire in 2035 and 2030, respectively. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities.

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses or research and development tax credit carryforwards.

In general, under Section 382 of the Code and corresponding provisions of state law, a corporation that undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership by certain stockholders over a three-year period, is subject to limitations on its ability to utilize its pre-change net operating losses and research and development tax credit carryforwards to offset future taxable income. We have not conducted a study to assess whether any such ownership changes have occurred. We may have experienced such ownership changes in the past and may experience such ownership changes in the future (which may be outside our control). As a result, if, and to the extent that, we earn net taxable income, our ability to use our pre-change net operating losses and research and development tax credit carryforwards to offset such taxable income may be subject to limitations. Our net operating losses or credits may also be impaired under state law.

There is also a risk that due to regulatory changes, such as suspensions on the use of net operating losses, or other unforeseen reasons, our existing net operating losses could expire or otherwise become unavailable to offset future income tax liabilities. As described above in "Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition," the TCJA, as amended by the CARES Act, includes changes to U.S. federal tax rates and the rules governing net operating loss carryforwards that may significantly impact our ability to utilize our net operating losses to offset taxable income in the future. In addition, state net operating losses generated in one state cannot be used to offset income generated in another state. For these reasons we may be unable to use a material portion of our net operating losses and other tax attributes.

Risks Related to the Discovery and Development of our Product Candidates

We are early in our development efforts, and we only have two product candidates in clinical trials. If we are unable to commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts, and we have advanced only two product candidates into clinical trials, losmapimod for the treatment of FSHD and for the treatment of COVID-19, and FTX-6058 in healthy volunteers. We have invested substantially all of our efforts and financial resources in our proprietary product engine to identify and validate cellular drug targets that can potentially modulate gene expression to address the root cause of rare diseases. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our product candidates will depend on several factors, including the following:

- successfully completing preclinical studies and clinical trials;
- allowance by the FDA or other regulatory agencies of the investigational new drug applications, or INDs, clinical trial applications, or CTAs, or other regulatory filings for losmapimod, FTX-6058 and future product candidates;
- expanding and maintaining a workforce of experienced scientists and others to continue to develop our product candidates;
- applying for and receiving marketing approvals from applicable regulatory authorities;
- obtaining and maintaining intellectual property protection and regulatory exclusivity for our product candidates;
- making arrangements with third-party manufacturers for, or establishing, commercial manufacturing capabilities;
- establishing sales, marketing and distribution capabilities and successfully launching commercial sales of the products, if and when approved, whether alone or in collaboration with others;
- acceptance of the products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining coverage, adequate pricing and adequate reimbursement from third-party payors, including government payors;
- maintaining, enforcing, defending and protecting our rights in our intellectual property portfolio;
- not infringing, misappropriating or otherwise violating others' intellectual property or proprietary rights; and
- maintaining a continued acceptable safety profile of the products following receipt of any regulatory approvals.

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 develop and commercialize our product candidates, which would materially harm our business.

We may not be successful in our efforts to develop losmapimod for the treatment of patients with COVID-19.

In June 2020, we initiated a Phase 3, randomized, double-blind, placebo-controlled trial of losmapimod in higher risk hospitalized adults with COVID-19. This Phase 3 clinical trial is an international, multicenter trial designed to assess the safety and efficacy of a 15 mg twice per day oral dose of losmapimod compared to placebo for 14 days on top of standard of care in approximately 400 patients hospitalized with COVID-19 and at risk of progression to critical illness based on older age and elevated systemic inflammation.

While we have initiated our Phase 3 clinical trial of losmapimod for the treatment of patients with COVID-19, the timing and success of the trial will depend on our ability to reach agreement with clinical trial sites and successfully screen and enroll patients in the trial, which, even if we are able to do, may not be on the timelines we expect. We experienced delays in establishing clinical trials sites and began enrolling patients in the fourth quarter of 2020. Many other companies are pursuing the development of product candidates for the treatment of COVID-19, and availability of clinical trial sites and patient enrollment may be affected by availability of commercially available treatments and other clinical trials of competing product candidates. Patient enrollment may also be affected by other factors, including the incidence of COVID-19 over time and the perceived risks and benefits of the use of losmapimod as a treatment relative to competing treatments. We have not activated clinical trial sites and enrolled patients in the trial on the timelines that we previously expected, which has resulted in delays to the trial, including expected delays to the completion of the interim analysis previously planned for the fourth quarter of 2020 and the expected reporting of topline data previously planned for the first quarter of 2021. We may continue to experience these delays or encounter further delays or may decide to abandon the trial and development of losmapimod for the treatment of COVID-19 altogether.

The COVID-19 pandemic may be effectively contained before we can successfully develop losmapimod as a treatment for COVID-19 and recoup our financial investment in the trial through sales of losmapimod. For example, a vaccine may be developed and available in sufficient quantities to contain the spread of the SARS-CoV-2 virus before we are able to complete our Phase 3 clinical trial and obtain marketing approval. Similarly, other therapeutics may prove efficacious in treating patients with COVID-19 before we are able to complete the development of losmapimod in this indication. Our commitment of financial resources and personnel to the development of losmapimod for the treatment of COVID-19 may cause delays in or otherwise negatively impact our other development programs and research and discovery efforts with our product engine.

Additionally, while losmapimod has been previously tested in more than 3,600 subjects, including in clinical trials evaluating the ability of an oral dose of 15 mg twice per day of losmapimod to restore a normal immune response in older patients challenged with a viral antigen, it has not been evaluated as a treatment in the acute infection setting. It is possible that unexpected safety issues, including issues that have not been observed in prior clinical trials of losmapimod, could occur in patients with COVID-19. Any such safety issues could affect our development program for losmapimod for the treatment of FSHD and our ability to obtain marketing approval for losmapimod, including our ability to apply for accelerated approval.

In this trial, we plan to administer losmapimod as an add-on to the current standard of care. The standard of care for COVID-19 patients may change during our trial or following our trial, and the clinical data that we obtain in our Phase 3 clinical trial may not translate to supporting the use of losmapimod as an add-on to a new standard of care.

Given the rapidity of the onset of the COVID-19 pandemic, scientific and medical research on the SARS-CoV-2 virus is ongoing and evolving. We cannot be certain that the preclinical and clinical evidence that we believe suggests that losmapimod may be beneficial to patients with COVID-19 will be established in a clinical trial. Similarly, we cannot be certain that the Phase 3 clinical trial will be sufficient to enable us to obtain marketing approval of losmapimod for the treatment of COVID-19, and we may need to conduct additional clinical trials before we are able to apply for marketing approval. Furthermore, the failure of losmapimod to demonstrate safety and efficacy in patients with COVID-19 could negatively impact the perception of us and losmapimod by investors.

To date, the evaluation of losmapimod for the treatment of COVID-19 has moved quickly through the FDA regulatory review and approval process. The speed at which all parties are acting to create and test many therapeutics and vaccines for COVID-19 is unusual, and evolving or changing plans or priorities within the FDA, including changes based on new knowledge of COVID-19 and how the disease affects the human body, may significantly affect the regulatory timeline for losmapimod for the treatment of COVID-19.

In the future, we may seek to make losmapimod available for the treatment of COVID-19 through an Emergency Use Authorization Program, or the FDA or other government body may request that losmapimod is made available through an Emergency Use Authorization. The FDA has the authority to grant an Emergency Use Authorization to allow unapproved medical products to be used in an emergency to diagnose, treat or prevent serious or life-threatening diseases or conditions when there are no adequate, approved and available alternatives. If we are granted an Emergency Use Authorization for losmapimod for the treatment of COVID-19, we would be able to commercialize losmapimod for the treatment of COVID-19 prior to FDA approval of a new drug application, or NDA. However, the FDA may revoke an Emergency Use Authorization where it is determined that the underlying health emergency no longer exists or warrants such authorization, and we cannot predict how long, if ever, an Emergency Use Authorization would remain in place. Such revocation could adversely impact our business in a variety of ways, including if losmapimod for the

treatment of COVID-19 is not yet approved by the FDA via an NDA and if we and our manufacturing partners have invested in the supply chain to provide losmapimod for the treatment of COVID-19 under an Emergency Use Authorization.

We may not be successful in our efforts to use our product engine to build a pipeline of product candidates.

A key element of our strategy is to use our proprietary product engine to identify and validate cellular drug targets that can potentially modulate gene expression to address the root cause of rare diseases, with an initial focus on identifying small molecules specific to the identified cellular target. Even if we are successful in identifying drug targets and potential product candidates, such candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance. Identifying, developing, obtaining regulatory approval for and commercializing additional product candidates will require substantial additional funding and is prone to the risks of failure inherent in product development. We cannot provide stockholders any assurance that we will be able to successfully identify additional product candidates with our product engine, including those subject to our collaborations with Acceleron and MyoKardia, advance any of these additional product candidates through the development process or successfully commercialize any such additional product candidates. Regulatory authorities have substantial discretion in the approval process and may cause delays in the approval or rejection of an application. As a result of these factors, it is difficult for us to predict the time and cost of product candidate development. There can be no assurance that any development problems we experience in the future related to our proprietary product engine or any of our research or development programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. If we do not successfully identify, develop, obtain regulatory approval for and commercialize product candidates based upon our technological approach, we will not be able to generate product revenues.

Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. The results of preclinical studies and early clinical trials may not be predictive of future results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

We have two product candidates in clinical development. The risk of failure for each of our product candidates is high. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. We have not yet completed a pivotal clinical trial of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. Even if the clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned INDs and other regulatory filings in the United States and abroad. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory agencies will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our current or future product candidates. As a result, we cannot be sure that we will be able to submit INDs or similar applications for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or other regulatory authorities allowing clinical trials to begin. Furthermore, product candidates are subject to continued preclinical safety studies, which may be conducted concurrent with our clinical testing. The outcomes of these safety studies may delay the launch of or enrollment in future clinical trials and could impact our ability to continue to conduct our clinical trials.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in study design, dose selection issues, placebo effects, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and preliminary or interim results of a clinical trial do not necessarily predict final results. For example, our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Losmapimod may not be effective at reducing DUX4-driven gene expression or, even if losmapimod successfully reduces expression of DUX4-driven genes, such reduction may not result in overall clinical benefit. A lack of clinical benefit may be due to insufficient dosing or for other reasons. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials, and we cannot be certain that we will not face similar setbacks. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Furthermore, the failure of any of our product candidates to demonstrate safety and efficacy in any clinical trial could negatively

impact the perception of our other product candidates and/or cause the FDA or other regulatory authorities to require additional testing before approving any of our product candidates.

In February 2019, we entered into a right of reference and license agreement, or the GSK Agreement, with affiliates of GSK pursuant to which, among other things, GSK granted us a right of reference to certain INDs filed with the FDA and controlled by GSK or its affiliates relating to losmapimod and an exclusive worldwide license to certain of GSK's preclinical and clinical data with respect to losmapimod. Although losmapimod was originally evaluated by GSK in nearly 3,600 subjects, GSK did not evaluate losmapimod in FSHD or in any other muscular dystrophy, or in novel diseases, such as COVID-19, and most of the subjects in these trials were given a dose that was lower than our planned dosage of 15 mg of losmapimod twice per day, so the safety data generated from GSK's clinical trials of losmapimod may not be predictive or indicative of the results of our clinical trials. Similarly, while we believe the safety data from GSK's clinical trials may, in part, enable us to apply for accelerated approval, there can be no assurance that this will happen. Regulatory authorities may also raise questions regarding the transition in the future from GSK-manufactured tablets to tablets manufactured by us or another party, and we may be required to conduct comparability assessments, which could result in delays in development and additional costs.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- regulators or institutional review boards, or IRBs, may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- regulators may decide the design of our clinical trials is flawed, for example if our trial protocol does not evaluate treatment effects in trial subjects for a sufficient length of time;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- we may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, or, if we seek accelerated approval, biomarker efficacy endpoints that applicable regulatory authorities would consider likely to predict clinical benefit;
- preclinical testing may produce results based on which we may decide, or regulators may require us, to conduct additional preclinical studies before we proceed with certain clinical trials, limit the scope of our clinical trials, halt ongoing clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may decide, or regulators or IRBs may require us, to suspend or terminate clinical trials of our product candidates for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- regulators or IRBs may require us to perform additional or unanticipated clinical trials to obtain approval or we may be subject to additional post-marketing testing requirements to maintain regulatory approval;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;

- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials;
- unforeseen global instability, including political instability or instability from an outbreak of pandemic or contagious disease, such as the COVID-19 pandemic, in or around the countries in which we conduct our clinical trials, could delay the commencement or rate of completion of our clinical trials; and
- regulators may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a risk evaluation and mitigation strategy, or REMS.

For example, in the wake of COVID-19, the clinical trial sites for our Phase 2b clinical trial temporarily postponed trial-related activities, impacting our clinical trial execution plans, and we cannot be certain that we will not face other postponements or similar difficulties in the future.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling or a REMS that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or in obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, including to add additional patients or arms, which could result in increased costs and expenses and/or delays. Significant preclinical study or clinical trial delays also could 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 and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

Because we are developing some of our product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to predict or provide clinically meaningful results.

There are currently no therapies approved to treat FSHD, and there may be no therapies approved to treat the underlying causes of diseases that we attempt to address or may address in the future. As a result, the design and conduct of clinical trials of product candidates for the treatment of these diseases may take longer, be more costly or be less effective as part of the novelty of development in these diseases. In some cases, we may use new or novel endpoints or methodologies, such as the optimized time up and go test we intend to use in our losmapimod clinical trials, which we refer to as the FSHD-TUG test, and the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results. Even if applicable regulatory authorities do not object to our proposed endpoints in an earlier stage clinical trial, such regulatory authorities may require evaluation of additional or different clinical endpoints in later-stage clinical trials. Additionally, if we pursue accelerated approval for certain product candidates, the FDA or another regulatory authority may determine that the biomarker efficacy endpoint we select for evaluation is not sufficiently predictive of clinical benefit to support accelerated approval. For example, if we pursue accelerated approval with the FDA for losmapimod for the treatment of FSHD, the FDA may determine that our proposed biomarker efficacy endpoint of measuring DUX4-driven gene expression as a biomarker in muscle biopsies is inadequate to accurately capture treatment effects in muscle over time or is not sufficiently predictive of clinical benefit to support accelerated approval. The FDA may also determine that the measurement interval for our Phase 2b clinical trial is too short to evaluate the potential clinical benefit of losmapimod for FSHD where the progression of symptoms is relatively slow and chronic dosing is required.

Even if the FDA does find our clinical trial success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoint to a degree of statistical significance in any pivotal or other clinical trials we may conduct for our product candidates. Further, even if we do achieve the pre-specified criteria, our trials may produce results that are unpredictable or inconsistent with the results of the more traditional efficacy endpoints in the trial. The FDA also could give overriding weight to other efficacy endpoints over a primary endpoint, even if we achieve statistically significant results on that primary endpoint, if we do not do so on our secondary efficacy endpoints. The FDA also weighs the benefits of a product against its risks and the FDA may view the efficacy results in the context of safety as not being supportive of approval. Other regulatory authorities in Europe and other countries may make similar findings with respect to these endpoints.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Identifying and qualifying patients to participate in clinical trials for our product candidates is critical to our success. Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients who remain in the trial until its conclusion. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside of the United States. Because of our primary focus on rare diseases, we may have difficulty enrolling a sufficient number of eligible patients.

Patient enrollment is affected by a variety of other factors, including:

- the prevalence and severity of the disease under investigation;
- the eligibility criteria for the trial in question;
- the perceived risks and benefits of the product candidate under trial;
- the requirements of the trial protocols, including invasive procedures such as muscle biopsies;
- the availability of existing treatments for the indications for which we are conducting clinical trials;
- the ability to recruit clinical trial investigators with the appropriate competencies and experience;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- the proximity and availability of clinical trial sites for prospective patients;
- the conduct of clinical trials by competitors for product candidates that treat the same indications as our product candidates;
- the ability to identify specific patient populations for biomarker-defined trial cohort(s); and
- the cost to, or lack of adequate compensation for, prospective patients.

Our inability to locate and enroll a sufficient number of patients for our clinical trials would result in significant delays, could require us to abandon one or more clinical trials altogether and could delay or prevent our receipt of necessary regulatory approvals. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

If serious adverse events or unacceptable side effects are identified during the development of our product candidates, we may need to abandon or limit our development of some of our product candidates.

If our product candidates are associated with serious adverse events or undesirable side effects in clinical trials or have characteristics that are unexpected in clinical trials or preclinical testing, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. In pharmaceutical development, many compounds that initially show promise in early-stage or clinical testing are later found to cause side effects that delay or prevent further development of the compound.

Additionally, if results of our clinical trials reveal unacceptable side effects, we, the FDA or the IRBs at the institutions in which our studies are conducted could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete any of our clinical trials. If we elect or are forced to suspend or terminate any clinical trial of our product candidates, the commercial prospects of such product candidate will be harmed, and our ability to generate product revenue from such product candidate will be delayed or eliminated. Any of these occurrences could materially harm our business.

If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability to market the drug could be compromised.

Clinical trials of our product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If one or more of our product candidates receives regulatory approval, and we, or others, later discover that they are less effective than previously believed, or cause undesirable side effects, a number of potentially significant negative consequences could result, including:

- withdrawal or limitation by regulatory authorities of approvals of such product;
- seizure of the product by regulatory authorities;
- recall of the product;
- restrictions on the marketing of the product or the manufacturing process for any component thereof;
- requirement by regulatory authorities of additional warnings on the label, such as a “black box” warning or contraindication;
- requirement that we implement a REMS or create a medication guide outlining the risks of such side effects for distribution to patients;
- commitment to expensive post-marketing studies as a prerequisite of approval by regulatory authorities of such product;
- the product may become less competitive;
- initiation of regulatory investigations and government enforcement actions;
- initiation of legal action against us to hold us liable for harm caused to patients; and
- harm to our reputation and resulting harm to physician or patient acceptance of our products.

Any of these events could prevent us from achieving or maintaining market acceptance of a particular product candidate, if approved, and could significantly harm our business, financial condition, and results of operations.

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 are focusing our research and development efforts on rare neuromuscular disorders, hemoglobinopathies and central nervous system diseases. In light of the recent COVID-19 pandemic, we have also begun to focus some of our research and development efforts to evaluate losmapimod as a potential treatment for COVID-19. 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 products 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 products. 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. Failure to allocate resources or capitalize on strategies in a successful manner will have an adverse impact on our business.

We are conducting Phase 2b and Phase 2 open label clinical trials of losmapimod in patients with FSHD in Europe and currently plan to conduct additional clinical trials for our product candidates at sites outside the United States and the FDA may not accept data from trials conducted in such locations.

We are currently conducting Phase 2b and Phase 2 open label clinical trials of losmapimod in patients with FSHD in Europe, and a Phase 3 clinical trial of losmapimod for the treatment of COVID-19 at sites in Mexico and South America. We may also conduct additional clinical trials outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and be performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials also complied with all applicable U.S. laws and regulations. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates.

Risks Related to the Commercialization of our Product Candidates

Even if any of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, and the market opportunity for any of our product candidates, if approved, may be smaller than we estimate.

If any of our product candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. 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:

- the efficacy and potential advantages of our product candidates compared to the advantages and relative risks of alternative treatments;
- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our products, if approved, for sale at competitive prices;
- the clinical indications for which the product is approved;
- the 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;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement, and patients' willingness to pay out of pocket for required co-payments or in the absence of third-party coverage or adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

Our assessment of the potential market opportunity for our product candidates is based on industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties, one of which we commissioned. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data. We commissioned Clarion Healthcare, LLC to conduct market research with physicians and payors to better understand the commercial landscape and to assist in our commercial planning with respect to losmapimod for the treatment of FSHD. A total of 14 physicians in the United States, the European Union and Asia and nine payors and payor experts in the United States and the European Union were surveyed. As the survey involved a limited number of physicians and payors, the results from such survey may be less reflective of market opportunity than a survey conducted with a larger sample size. Our estimates of the potential market opportunities for our product candidates include several key assumptions based on our industry knowledge, industry publications and third-party research, surveys and studies, which may be based on a small sample size and fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions. If any of our assumptions or estimates, or these publications, research, surveys or studies prove to be inaccurate, then the actual market for any of our product candidates may be smaller than we expect, and as a result our product revenue may be limited and it may be more difficult for us to achieve or maintain profitability. Specifically, the commercial opportunity for losmapimod for the treatment of COVID-19 is uncertain given the novel nature of the disease, the rapidity of the onset of the COVID-19 pandemic, the number of recent entrants in the market and the potential that a vaccine may be developed and available in sufficient quantities to contain the spread of the SARS-CoV-2 virus before we are able to complete our Phase 3 clinical trial and obtain marketing approval. Similarly, other therapeutics may prove efficacious in treating patients with COVID-19 before we are able to complete the development of losmapimod in this indication.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing our product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we expect to build a focused, specialty sales and marketing infrastructure to market some of our product candidates in the United States, if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product 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. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

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

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products 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 are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates or may be unable to do so on terms that are acceptable 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 products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of many of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

For example, we are aware of several product candidates in clinical development that could be competitive with product candidates that we may successfully develop and commercialize. bluebird bio, Inc., Aruvant Sciences, Inc., EpiDestiny, Inc., or EpiDestiny (in collaboration with Novo Nordisk A/S), Imara, Inc., Agios Pharmaceuticals, Inc., Forma Therapeutics Holdings, Inc., Sangamo Therapeutics Inc., or Sangamo (in collaboration with Bioverativ Inc.) and CRISPR Therapeutics AG (in collaboration with Vertex Pharmaceuticals, Inc.) are developing therapeutic approaches for patients with sickle cell disease, or SCD. Acceleron (in collaboration with Celgene Corp.), Bellicum Pharmaceuticals, Inc., Kiadis Pharma, EpiDestiny (in collaboration with Novo Nordisk A/S), Imara Inc., Agios Pharmaceuticals, Inc., Forma Therapeutics Holdings, Inc., Orchard Therapeutics plc, Sangamo (in collaboration with Bioverativ, Inc.) and CRISPR Therapeutics AG (in collaboration with Vertex Pharmaceuticals, Inc.) are developing therapeutic approaches for patients with ß-thalassemia. Eli Lilly and Company, Incyte Corporation, Alexion Pharmaceuticals, Inc., InflaRx N.V., OncoImmune, Inc., NeuroRx, Inc., CytoDyn Inc., Vanda Pharmaceuticals, Inc., CTI Biopharma Corp., AstraZeneca PLC, Biohaven Pharmaceutical Holding Company Ltd., Chimerix, Inc., and Novartis AG, are developing immunomodulating therapeutics for the treatment of COVID-19, and other companies, such as Moderna, Inc., BioNTech SE (in collaboration with Pfizer Inc.), Novavax, Inc., AstraZeneca PLC, Johnson & Johnson and CureVac B.V. have COVID-19 vaccine candidates currently in clinical trials.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. If our product candidates achieve marketing approval, we expect that they will be priced at a significant premium over competitive generic products.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If the market opportunities for our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because certain of the target patient populations of our product candidates are small, and the addressable patient population even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth.

We primarily focus our research and product development on treatments for rare diseases. Given the small number of patients who have the rare diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. 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 our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research that we conducted, and may prove to be incorrect or contain errors. New studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations for many of the indications we are evaluating are very small, we may never achieve profitability despite obtaining such significant market share.

The target patient populations for some of the indications we are evaluating are relatively small, and there is currently no standard of care treatment directed at some of our target indications, such as FSHD. As a result, the pricing and reimbursement of our product candidates, if approved, is uncertain, but must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected.

We rely and expect to continue to rely, on contract manufacturing organizations to manufacture our product candidates. If we are unable to enter into such arrangements as expected or if such organizations do not meet our supply requirements, development and/or commercialization of our product candidates may be delayed.

We rely, and expect to continue to rely, on third parties to manufacture clinical supplies of our product candidates and we expect to rely on third parties to manufacture commercial supplies of our products, if and when approved for marketing by applicable regulatory authorities, as well as for packaging, sterilization, storage, distribution and other production logistics. If we are unable to enter into such arrangements on the terms or timeline we expect, development and/or commercialization of our product candidates may be delayed. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements, if there are disagreements between us and such parties or if such parties are unable to expand capacities to support commercialization of any of our product candidates for which we obtain marketing approval, we may not be able to fulfill, or may be delayed in producing sufficient product candidates to meet, our supply requirements. These facilities may also be affected by natural disasters, such as floods or fire, as well as public health issues (for example, an outbreak of a contagious disease such as COVID-19), or such facilities could face manufacturing issues, such as contamination or regulatory concerns following a regulatory inspection of such facility. In such instances, we may need to locate an appropriate replacement third-party facility and establish a contractual relationship, which may not be readily available or on acceptable terms, which would cause additional delay and increased expense, including as a result of additional required FDA approvals, and may have a material adverse effect on our business.

Our third-party manufacturers will be subject to inspection and approval by the FDA before we can commence the manufacture and sale of any of our product candidates, and thereafter subject to FDA inspection from time to time. Failure by our third-party manufacturers to pass such inspections and otherwise satisfactorily complete the FDA approval regimen with respect to our product candidates may result in regulatory actions such as the issuance of FDA Form 483 notices of observations, warning letters or injunctions or the loss of operating licenses.

We or our third-party manufacturers may also encounter shortages in the raw materials or API necessary to produce our product candidates in the quantities needed for our clinical trials or, if our product candidates are approved, in sufficient quantities for commercialization or to meet an increase in demand, as a result of capacity constraints or delays or disruptions in the market for the raw materials or API, including shortages caused by the purchase of such raw materials or API by our competitors or others. The failure of us or our third-party manufacturers to obtain the raw materials or API necessary to manufacture sufficient quantities of our product candidates, may have a material adverse effect on our business.

Even if we are able to commercialize any product candidates, the products may become subject to unfavorable pricing regulations, third-party coverage or reimbursement practices or healthcare reform initiatives, which could harm our business.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any product candidates successfully also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Coverage and reimbursement may not be available for any product that we commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Reimbursement may affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside of the United States. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

There can be no assurance that our product candidates, even if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payors, or that coverage and an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably.

Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties that, if they materialize, could harm our business.

Our future profitability will depend, in part, on our ability to commercialize our product candidates in markets outside of the United States and the European Union. If we commercialize our product candidates in foreign markets, we will be subject to additional risks and uncertainties, including:

- economic weakness, including inflation, or political instability in particular economies and markets;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements, many of which vary between countries;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;
- tariffs and trade barriers, as well as other governmental controls and trade restrictions;
- other trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or foreign governments;
- longer accounts receivable collection times;
- longer lead times for shipping;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is common;
- language barriers for technical training;
- reduced protection of intellectual property rights in some foreign countries, and related prevalence of generic alternatives to therapeutics;
- foreign currency exchange rate fluctuations and currency controls;
- differing foreign reimbursement landscapes;
- uncertain and potentially inadequate reimbursement of our products; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

If risks related to any of these uncertainties materializes, it could have a material adverse effect on our business.

Clinical trial and product liability lawsuits against us could divert our resources and could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in human clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no products that have been approved for commercial sale, the current and future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

We currently hold $10 million in clinical trial liability insurance coverage in the aggregate, with a per incident limit of $10 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. 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. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Risks Related to our Dependence on Third Parties

We rely, and expect to continue to rely, on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, which may harm our business.

We currently rely on third-party clinical research organizations to conduct our ongoing Phase 2b, Phase 2 open label and Phase 3 clinical trials of losmapimod and our ongoing Phase 1 clinical trial of FTX-6058. We plan to rely on third-party clinical research organizations or third-party research collaboratives to conduct any future clinical trials. We do not plan to independently conduct clinical trials of our other product candidates. We expect to continue to rely on third parties, such as clinical research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials. These agreements might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, our product development activities might be delayed.

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 responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully develop and commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors.

We also rely, and expect to continue to rely, on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We plan to 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 products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities. Although we believe we have obtained sufficient losmapimod tablets from GSK to complete our ongoing Phase 2 and Phase 3 clinical trials and that we have received a sufficient quantity of losmapimod API to complete further clinical trials in FSHD and COVID-19, we cannot be sure we have correctly estimated our drug product and API requirements or that such drug product or API will not expire before we want to use it. We have also engaged CMOs to prepare our own API and to manufacture losmapimod tablets. While we believe that we have all the necessary information from GSK to enable any required technology transfer to a CMO, there can be no assurances that we will be able to effect such transfer in a timely manner.

In addition, although we believe we have obtained sufficient quantities of FTX-6058 for the completion of our ongoing Phase 1 clinical trial, we cannot be sure we have correctly estimated our drug product requirements.

We expect to rely on third parties for the manufacture of FTX-6058 for any future clinical trials and for the manufacture of any future product candidates for preclinical and clinical testing, as well as for commercial manufacture if any of our product candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We also expect to rely on third-party manufacturers or third-party collaborators for the manufacture of commercial supply of any other product candidates for which we or our collaborators obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practices, or cGMP, regulations or similar regulatory requirements outside of 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 clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Our product candidates and any products that we may develop may compete with other product candidates and products 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 redundant supply or a source for bulk drug substance. If any of our future contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers. 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 products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We have entered into, and may in the future enter into, collaborations with third parties for the development or commercialization of our product candidates. If our collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates and our business could be adversely affected.

In December 2019, we entered into a collaboration and license agreement with Acceleron to identify biological targets to modulate specific pathways associated with a targeted indication within the pulmonary disease space. In July 2020, we entered into a collaboration and license agreement with MyoKardia to identify and validate potential biological targets for the potential treatment of certain genetically defined cardiomyopathies. While we have retained all rights to and are developing on our own our current product candidates, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to our other existing or future product candidates. Our likely collaborators for any such sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Collaborations that we enter into, including our collaborations with Acceleron and MyoKardia, may not be successful, and any success will depend heavily on the efforts and activities of such collaborators. Collaborations pose a number of risks, including the following:

- collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development of our product candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may not pursue commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates on a discretionary basis;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all. If any collaborations that we enter into do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed and we may need additional resources to develop our product candidates. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our collaborators.

Additionally, subject to its contractual obligations to us, if a collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

If we are not able to establish or maintain collaborations, we may have to alter our development and commercialization plans and our business could be adversely affected.

For some of our product candidates, we may decide to collaborate with pharmaceutical or biotechnology companies for the development and potential commercialization of those product candidates. For example, in December 2019, we entered into a collaboration and license agreement with Acceleron to identify biological targets to modulate specific pathways associated with a targeted indication within the pulmonary disease space, and in July 2020, we entered into a collaboration and license agreement with MyoKardia to identify and validate potential biological targets for the potential treatment of certain genetically defined cardiomyopathies. We face significant competition in seeking appropriate collaborators, and a number of more established companies may also be pursuing strategies to license or acquire third-party intellectual property rights that we consider attractive. These established companies may have a competitive advantage over us due to their size, financial resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. 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 product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, 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 product 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 product candidate.

We may also be restricted under existing or future license agreements from entering into agreements on certain terms with potential collaborators. For example, we are restricted by GSK's right of first negotiation under our current license agreement with them. We are also restricted under our collaboration with Acceleron from, directly or indirectly, researching, developing, manufacturing, commercializing, using or otherwise exploiting any compound or product for the treatment, prophylaxis, or diagnosis of a targeted indication within the pulmonary disease space, other than for Acceleron, while we are performing the research activities pursuant to the research plan and for a specified period thereafter. Additionally, we are restricted under our collaboration with Acceleron from researching, developing, manufacturing, commercializing, using, or otherwise exploiting any compound or product for the treatment, prophylaxis, or diagnosis of a targeted indication within the pulmonary disease space that is directed against certain specified biological targets identified by us in the performance of the research activities while we are performing the research activities pursuant to the research plan and for a specified period thereafter. Under our collaboration with MyoKardia, we are restricted from researching, developing, manufacturing, commercializing, using, or otherwise exploiting any compound or product (a) that is a compound or product under the agreement that is directed against certain targets identified by us in the performance of the research activities for the treatment, prophylaxis, or diagnosis of any indication or (b) for the treatment of any genetically defined cardiomyopathies shown to be related to certain specified genes of interest that are modulated by the targets chosen by MyoKardia under our collaboration, in the case of both (a) and (b), while we are performing the research activities pursuant to the research plan and for a specified period thereafter.

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 and biotechnology companies that have resulted in a reduced number of potential future collaborators.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, 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 fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our product engine.

Risks Related to our Intellectual Property

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

Our success depends in large part on our ability to obtain and maintain protection of the intellectual property we may own solely and jointly with others or may license from others, particularly patents, in the United States and other countries with respect to any proprietary technology and product candidates we develop. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates that are important to our business and by in-licensing intellectual property related to our technologies and product candidates. If we are unable to obtain or maintain patent protection with respect to any proprietary technology or product candidate, our business, financial condition, results of operations and prospects could be materially harmed.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, defend or license 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. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce and defend the patents, covering technology that we license from third parties. Therefore, these in-licensed patents and applications may not be prepared, filed, prosecuted, maintained, defended and enforced in a manner consistent with the best interests of our business.

The patent position of pharmaceutical and biotechnology companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the scope of patent protection outside of the United States is uncertain and laws of foreign countries may not protect our rights to the same extent as the laws of the United States or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. With respect to both owned and in-licensed patent rights, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. In addition, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not published at all. Therefore, neither we nor our licensors can know with certainty whether either we or our licensors were the first to make the inventions claimed in the patents and patent applications we own or in-license now or in the future, or that either we or our licensors were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our owned and in-licensed patent rights are highly uncertain. Moreover, our owned and in-licensed pending and future patent applications may not result in patents being issued which protect our technology and product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents and our ability to obtain, protect, maintain, defend and enforce our patent rights, narrow the scope of our patent protection and, more generally, could affect the value or narrow the scope of our patent rights.

Currently, our patent portfolio related to FTX-6058 is in early stages and comprises three owned pending applications: one U.S. non-provisional application, one PCT application, and one Taiwanese application. We have no issued patents related to FTX-6058 or our SCD or ß-thalassemia programs. In order to continue to pursue protection based on the PCT application, we will need to nationalize this application into corresponding foreign applications prior to the expiration deadline of the PCT application. Even then, as highlighted above, patents may never issue from our patent applications, or the scope of any patent may not be sufficient to provide a competitive advantage. With respect to losmapimod, the patents to losmapimod licensed from GSK as a composition of matter and pharmaceutical composition are expected to expire on February 10, 2023. We own one patent covering the use of losmapimod for the treatment of patients with FSHD and we own one patent covering the use of other clinical-stage p38 inhibitors for the treatment of patients with FSHD. In addition, our owned patents and patent applications pertaining to losmapimod are not to the composition but, rather, are directed to certain methods of treating FSHD. We cannot be certain that our pending patent applications related to the losmapimod program will be granted. Even if such patent applications issue as patents, they will not prevent third parties from commercializing losmapimod for other indications.

Moreover, we or our licensors may be subject to a third-party preissuance submission of prior art to the United States Patent and Trademark Office, or USPTO, or become involved in opposition, derivation, revocation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. If the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Additionally, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if our owned and in-licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and in-licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Such proceedings also may result in substantial cost and require significant time from our management and employees, even if the eventual outcome is favorable to us. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Furthermore, our competitors may be able to circumvent our owned or in-licensed patents by developing similar or alternative technologies or products in a non-infringing manner. As a result, our owned and in-licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing technology and products similar or identical to any of our technology and product candidates.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. For example, the composition of matter patents covering losmapimod, licensed from GSK, are expected to expire on February 10, 2023. Given the near term expiration date of these patents, and the fact that safe harbor protections in many jurisdictions permit third parties to engage in development, including clinical trials, these patents may not provide us with any meaningful competitive advantage.

If we are unable to obtain licenses from third parties on commercially reasonable terms or fail to comply with our obligations under such agreements, our business could be harmed.

It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. If we are unable to license such technology, or if we are forced to license such technology on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales or an obligation on our part to pay royalties and/or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us.

If we are unable to obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology and product candidates, which could harm our business, financial condition, results of operations and prospects significantly.

Additionally, if we fail to comply with our obligations under license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market, or may be forced to cease developing, manufacturing or marketing, any product that is covered by these agreements or may face other penalties under such agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements, or restrictions on our ability to freely assign or sublicense our rights under such agreements when it is in the interest of our business to do so, may result in our having to negotiate new or reinstated agreements with less favorable terms, cause us to lose our rights under these agreements, including our rights to important intellectual property or technology or impede, or delay or prohibit the further development or commercialization of one or more product candidates that rely on such agreements.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for any product candidates we may develop, our business may be materially harmed.

In the United States, the patent term of a patent that covers an FDA-approved drug may be eligible for limited patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only one patent applicable to an approved drug may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. Similar provisions are available in Europe and certain other non-United States jurisdictions to extend the term of a patent that covers an approved drug. While, in the future, if and when our product candidates receive FDA approval, we expect to apply for patent term extensions on patents covering those product candidates, there is no guarantee that the applicable authorities will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions. We may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request. If we are unable to obtain any patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following the expiration of our patent rights, and our business, financial condition, results of operations and prospects could be materially harmed.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a U.S. patent covering any of our product candidates that we may identify even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the USPTO a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

Also, there are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Approved Drug Products with Therapeutic Equivalence Evaluations, or the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of our product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, a manufacturer of generic drugs would not have to provide advance notice to us of any abbreviated new drug application filed with the FDA to obtain permission to sell a generic version of such product candidate.

Changes to patent laws in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of patent laws in the United States, including patent reform legislation such as the Leahy-Smith America Invents Act, or the Leahy-Smith Act, could increase the uncertainties and costs surrounding the prosecution of our owned and in-licensed patent applications and the maintenance, enforcement or defense of our owned and in-licensed issued patents. The Leahy-Smith Act includes a number of significant changes to United States patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

Although we or our licensors are not currently involved in any litigation, we may become involved in lawsuits to protect or enforce our patent or other intellectual property rights, which could be expensive, time-consuming and unsuccessful.

Competitors and other third parties may infringe, misappropriate or otherwise violate our or our licensor's issued patents or other intellectual property. As a result, we or our licensors may need to file infringement, misappropriation or other intellectual property related claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke such parties to assert counterclaims against us alleging that we infringe, misappropriate or otherwise violate their intellectual property. In addition, in a patent infringement proceeding, such parties could counterclaim that the patents we or our licensors have asserted are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may institute such claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). The outcome following legal assertions of invalidity and unenforceability is unpredictable.

An adverse result in any such proceeding could put one or more of our owned or in-licensed patents at risk of being invalidated or interpreted narrowly, and could put any of our owned or in-licensed patent applications at risk of not yielding an issued patent. A court may also refuse to stop the third party from using the technology at issue in a proceeding on the grounds that our owned or in-licensed patents do not cover such technology. 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 or trade secrets could be compromised by disclosure during this type of litigation. Any of the foregoing could allow such third parties to develop and commercialize competing technologies and products and have a material adverse impact on our business, financial condition, results of operations and prospects.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference or derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

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

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property and proprietary rights of third parties. There is considerable patent and other intellectual property litigation in the pharmaceutical and biotechnology industries. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our technology and product candidates, including interference proceedings, post grant review, *inter partes* review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the European Patent Office. 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 technologies or product candidates that we may identify may be subject to claims of infringement of the patent rights of third parties.

The legal threshold for initiating litigation or contested proceedings is low, so that even lawsuits or proceedings with a low probability of success might be initiated and require significant resources to defend. Litigation and contested proceedings can also be expensive and time-consuming, and our adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we can. The risks of being involved in such litigation and proceedings may increase if and as our product candidates near commercialization and as we gain the greater visibility associated with being a public company. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of merit. We may not be aware of all such intellectual property rights potentially relating to our technology and product candidates and their uses, or we may incorrectly conclude that third party intellectual property is invalid or that our activities and product candidates do not infringe such intellectual property. Thus, we do not know with certainty that our technology and product candidates, or our development and commercialization thereof, do not and will not infringe, misappropriate or otherwise violate any third party's intellectual property.

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 or methods, such as methods of manufacture or methods for treatment, related to the discovery, use or manufacture of the product candidates that we may identify or related to our technologies. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that the product candidates that we may identify may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Moreover, as noted above, there may be existing patents that we are not aware of or that we have incorrectly concluded are invalid or not infringed by our activities. If any third-party patents were held by a court of competent jurisdiction to cover, for example, the manufacturing process of the product candidates that we may identify, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

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

We may choose to take a license or, if we are found to infringe, misappropriate or otherwise violate a third party's intellectual property rights, we could also be required to obtain a license from such third party to continue developing, manufacturing and marketing our technology and product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us and could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right and could be forced to indemnify our customers or collaborators. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business, financial condition, results of operations and prospects.

Intellectual property litigation or other legal proceedings relating to intellectual property could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and may also have an advantage in such proceedings due to their more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other proceedings could compromise our ability to compete in the marketplace.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance, renewal and annuity fees and various other government fees on any issued patent and pending patent application must be paid to the USPTO and foreign patent agencies in several stages or annually over the lifetime of our owned and in-licensed patents and patent applications. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In certain circumstances, we rely on our licensing partners to pay these fees to, or comply with the procedural and documentary rules of, the relevant patent agency. With respect to our patents, we rely on an annuity service, outside firms and outside counsel to remind us of the due dates and to make payment after we instruct them to do so. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, potential competitors might be able to enter the market with similar or identical products or technology. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it would have a material adverse effect on our business, financial condition, results of operations and prospects.

If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, or otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.

We are party to license and funding agreements, such as the GSK Agreement, that impose, and we may enter into additional licensing and funding arrangements with third parties that may impose, diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. Under our existing licensing and funding agreements, we are obligated to pay royalties on net product sales of product candidates or related technologies to the extent they are covered by the agreements. If we fail to comply with such obligations under current or future license and funding agreements, our counterparties may have the right to terminate these agreements or require us to grant them certain rights. Such an occurrence could materially adversely affect the value of any product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology, which would have a material adverse effect on our business, financial condition, results of operations and prospects. We also have licenses and agreements to certain technologies used in our product engine, all of which are non-exclusive. While we still face all of the risks described herein with respect to those agreements, we cannot prevent third parties from also accessing those technologies. In addition, our licenses may place restrictions on our future business opportunities. For example, under our license with GSK, GSK has certain rights of first negotiation if we wish to sublicense any of the patent or data rights licensed by GSK to us to a third party for use outside the United States. This may prevent or delay certain transactions, which could have an adverse effect on the development and commercialization of losmapimod and on our business.

Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation related issues;

- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected technology and product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

Our current or future licensors may have relied on third-party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents and patent applications we in-license. If other third parties have ownership rights to patents or patent applications we in-license, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize product candidates and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products and technologies identical to ours. This could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of 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, and even where such protection is nominally available, judicial and governmental enforcement of such intellectual property rights may be lacking. 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, further, may export otherwise infringing products to territories where we have patent protection or licenses but enforcement is not as strong as that in the United States. These products may compete with our products, 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 products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. In addition, certain jurisdictions do not protect to the same extent or at all inventions that constitute new methods of treatment.

Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions 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, could put 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 and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

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

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, 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 our or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. 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. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims by third parties asserting that our employees, consultants or contractors have wrongfully used or disclosed confidential information of third parties, or we have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees, consultants and contractors were previously employed at universities or other pharmaceutical or biotechnology companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our intellectual property assignment agreements with them may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial conditions, results of operations and prospects.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could have a material adverse effect on our competitive business position and prospects. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products, which license may not be available on commercially reasonable terms, or at all, or such license may be non-exclusive. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our management and employees.

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 our product candidates, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology and other proprietary information, to maintain our competitive position, including certain aspects of our proprietary product engine. We seek to protect our trade secrets and other proprietary technology, 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, CMOs, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants, but we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology. 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. Detecting the disclosure or misappropriation of a trade secret and 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 of the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our competitive position would be materially and adversely harmed.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- portions of our product engine are protected by trade secrets, but much of our product engine is not protected by intellectual property, including patents, trade secrets and know-how, and we may not be able to develop, acquire or in-license any patentable technologies or other intellectual property related to the unprotected portions of our product engine;
- others may be able to make product candidates that are similar to ours but that are not covered by the claims of the patents that we own;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or in-licensed intellectual property rights;
- it is possible that our owned and in-licensed pending patent applications or those we may own or in-license in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates;
- we cannot ensure that any patents issued to us or our licensors will provide a basis for an exclusive market for our commercially viable product candidates or will provide us with any competitive advantages;
- we cannot ensure that our commercial activities or product candidates will not infringe upon the patents of others;
- we cannot ensure that we will be able to successfully commercialize our product candidates on a substantial scale, if approved, before the relevant patents that we own or license expire;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Regulatory Approval of our Product Candidates and Other Legal Compliance Matters

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including design, testing, manufacture, packaging, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, export, import and adverse event reporting, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA and similar regulatory authorities outside of the United States. Marketing approval of drugs in the United States requires the submission of a new drug application, or NDA, to the FDA and we are not permitted to market any drug candidate in the United States until we obtain approval from the FDA of the NDA for that product. An NDA must be supported by extensive clinical and preclinical data, as well as extensive information regarding pharmacology, chemistry, manufacturing and controls. We have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction.

We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party clinical research organizations or other third-party consultants or vendors to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. If any of our product candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the product.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired.

We may not be able to obtain or maintain orphan drug designation or exclusivity for our product candidates and, even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product 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 in the United States. The FDA and EMA have granted orphan drug designation to losmapimod for the treatment of FSHD. We may seek orphan drug designation for our other current and future product candidates.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that time period. The applicable period is seven years in the United States and ten years in Europe. The exclusivity period in Europe can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA 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.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because competing drugs containing a different active ingredient can be approved for the same condition. In addition, 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. Moreover, if we pursue and obtain approval for the same product for another indication for which we are not entitled to or do not have orphan drug exclusivity, our period of orphan exclusivity will not prevent third parties from obtaining approval for a competing drug containing the same active ingredient for use in this other, non-orphan indication. If that were to occur, the protection we derive from orphan exclusivity may be adversely effected.

On August 3, 2017, the U.S. Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

A Fast Track designation by the FDA may not lead to a faster development or regulatory review or approval process.

We may seek Fast Track designation for some of our product candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA Fast Track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure stockholders that the FDA would decide to grant it. Even if we do receive Fast Track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

A Breakthrough Therapy designation by the FDA for our product candidates 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 a Breakthrough Therapy designation for some of our product candidates. A Breakthrough Therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs and biologics 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. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive Breakthrough Therapy designation, the receipt of such designation for a product candidate may not result in a faster development process, review or approval compared to drugs 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 or decide that the time period for FDA review or approval will not be shortened.

Even if the FDA agrees that we may pursue an accelerated approval NDA submission, that does not guarantee that the NDA will receive an accelerated approval, or a complete response letter, nor does submission of an accelerated approval NDA ensure that the product candidate will receive a faster development or regulatory review process.

We may seek approval of our product candidates using the FDA's accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit (i.e., an intermediate clinical endpoint). The FDA or other applicable regulatory agency makes the determination regarding whether a biomarker efficacy endpoint is reasonably likely to predict long-term clinical benefit.

Prior to seeking such accelerated approval, we will seek feedback from the FDA and otherwise evaluate our ability to seek and receive such accelerated approval. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform one or more adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence and we may be required to evaluate different or additional endpoints in these post-marketing confirmatory trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

There can be no assurance that the FDA will agree with our biomarker efficacy endpoints or intermediate clinical endpoints, such as measuring DUX4-driven gene expression in muscle tissue biopsies or measuring the fraction of muscle tissue by replaced by fat, or that we will decide to pursue or submit an NDA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that, after feedback from FDA, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or under another expedited regulatory designation, there can be no assurance that such submission or application will be accepted or that any expedited review or approval will be granted on a timely basis, or at all.

Moreover, as noted above, for drugs granted accelerated approval, the FDA typically requires post-marketing confirmatory trials to evaluate the anticipated effect on IMM or other clinical benefit. These confirmatory trials must be completed with due diligence. We may be required to evaluate additional or different clinical endpoints in these post-marketing confirmatory trials. These confirmatory trials may require enrollment of more patients than we currently anticipate and will result in additional costs, which may be greater than the estimated costs we currently anticipate. The FDA may withdraw approval of a product candidate approved under the accelerated approval pathway if, for example, the trial required to verify the predicted clinical benefit of our product candidate fails to verify such benefit or does not demonstrate sufficient clinical benefit to justify the risks associated with the drug. The FDA may also withdraw approval if other evidence demonstrates that our product candidate is not shown to be safe or effective under the conditions of use, we fail to conduct any required post approval trial of our product candidate with due diligence or we disseminate false or misleading promotional materials relating to our product candidate. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our product candidates, or withdrawal of a product candidate, would result in a longer time period for commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other foreign jurisdictions, we or our potential 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 of the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside of 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 our potential third-party collaborators may not obtain approvals, including conditional authorization, from regulatory authorities outside of 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 of the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in other countries. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical product, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the regulatory regime that applies to products and with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

Any product candidate for which we obtain marketing approval could be subject to post-marketing restrictions or withdrawal from the market and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

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 continual requirements of 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, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. 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, including the requirement to implement a REMS. If any of our product candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the product.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product, including the adoption and implementation of REMS. The FDA and other agencies, including the Department of Justice, or the DOJ, closely regulate and monitor the post-approval marketing and promotion of drugs to ensure they are marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and DOJ impose stringent restrictions on manufacturers' communications regarding off-label use, and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug and Cosmetic Act, or FDCA, and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations and enforcement actions alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have various consequences, including:

- suspension of or restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension of any ongoing clinical trials;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure or detention;
- injunctions or the imposition of civil or criminal penalties; or
- litigation involving patients using our products.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs applicable to drug manufacturers or quality assurance standards applicable to medical device manufacturers, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, any contract manufacturers we may engage in the future, our future collaborators and their contract manufacturers will also be subject to other regulatory requirements, including submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements regarding the distribution of samples to clinicians, recordkeeping, and costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product such as the requirement to implement a REMS.

The efforts of the Trump administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.

The Trump administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. For example, on January 30, 2017, President Trump issued an executive order, applicable to all executive agencies, including the FDA, requiring that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management and on February 2, 2017, the Trump administration indicated that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. More recently, on October 9, 2019, President Trump issued another executive order ("Executive Order on Promoting the Rule of Law Through Improved Agency Guidance Documents"). The order is meant to ensure that agency guidance documents do not establish legally binding requirements and it directs each agency to rescind guidance documents that it determines should no longer be in effect. In response to the COVID-19 pandemic, the Trump administration has indicated that it will take additional actions to provide regulatory relief. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Our relationships with healthcare providers, physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security, and other healthcare laws and regulations, which, in the event of a violation, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

If we obtain regulatory approval and commercialize any products, healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with healthcare providers, physicians and third-party payors may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at a minimum of $11,181 and a maximum of $22,363 per false claim;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and their respective implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the federal Physician Payments Sunshine Act requires applicable manufacturers of covered drugs to report payments and other transfers of value to physicians and teaching hospitals; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws and transparency statutes, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Additionally, some state and local laws require the registration of pharmaceutical sales representatives in the jurisdiction. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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, including anticipated activities that would be conducted by our sales team, are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government funded healthcare programs.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that EU member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act, which went into effect on January 1, 2020, is creating similar risks and obligations as those created by the GDPR, though the California Consumer Privacy Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with these requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations. Similarly, failure to comply with federal and state laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain for any products that are approved in the United States or foreign jurisdictions.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by legislative initiatives. Current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any FDA approved product.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician-administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA. Among the provisions of the ACA of potential importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any of our product candidates that are approved for sale, are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the civil False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- requirements to report certain financial arrangements with physicians and teaching hospitals;
- a requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2029 unless additional congressional action is taken, and the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. Further, there have been several recent U.S. congressional inquiries and proposed state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

Since the enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the TCJA, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, will become effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

The Trump administration has also taken executive actions to undermine or delay implementation of the ACA. For example, President Trump signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One Executive Order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the Trump administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017.

In addition, the Centers for Medicare & Medicaid Services, or CMS, has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. This decision is under review by the U.S. Supreme Court during its current term. The full effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known. On November 30, 2018, CMS announced a proposed rule that would amend the Medicare Advantage and Medicare Part D prescription drug benefit regulations to reduce out of pocket costs for plan enrollees and allow Medicare plans to negotiate lower rates for certain drugs. Among other things, the proposed rule changes would allow Medicare Advantage plans to use pre authorization (PA) and step therapy (ST) for six protected classes of drugs, with certain exceptions; permit plans to implement PA and ST in Medicare Part B drugs; and change the definition of "negotiated prices" as well as add a definition of "price concession" in the regulations. It is unclear whether these proposed changes will be accepted, and if so, what effect such changes will have on our business. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the same judge issued an order staying the judgment pending appeal. The Trump administration thereafter represented to the Court of Appeals considering this judgment that it does not oppose the lower court's ruling. On July 10, 2019, the Court of Appeals for the Fifth Circuit heard oral argument in this case. In those arguments, the Trump administration argued in support of upholding the lower court decision. On December 18, 2019, that court affirmed the lower court's ruling that the individual mandate portion of the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. On January 21, 2020, the U.S. Supreme Court declined to review this decision on an expedited basis. On March 2, 2020, that court agreed to hear this case. On June 25, 2020, the Trump administration and a coalition of 18 states asked the court to strike down the entirety of the ACA. Oral argument in this case is scheduled for November 10, 2020. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States, and members of Congress and the Trump administration have previously stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the Trump administration has pressed for further drug price control measures that could be enacted during the annual budget process or in other future legislation. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Specifically, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, on May 11, 2018, the Trump administration issued a plan to lower drug prices. Under this blueprint for action, the Trump administration indicated that the Department of Health and Human Services, or HHS, will: take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers' advertisements to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare's drug-pricing dashboard to increase transparency; prohibit Part D contracts that include "gag rules" that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending, and drug price increases. Finally, the Trump administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs.

More recently, on July 24, 2020, President Trump issued four executive orders that are intended to lower the costs of prescription drug products. The first order would require all federally qualified health centers, or FQHCs, to pass on to patients the discounts the health centers receive on insulin and epinephrine through Medicare's 340B Drug Discount Program. The second order would establish an international pricing index that would set the price Medicare Part B pays for the costliest medications covered under the program to the lowest price in other economically advanced countries.

The third order is intended to reduce the costs of drugs by supporting the safe importation of prescription drugs. Specifically, the order calls upon HHS to facilitate grants to individuals of waivers of the prohibition of importation of prescription drugs that would allow patients to import FDA approved drug products from abroad, so long as doing so would result in lower costs. In addition, the order would allow wholesalers and pharmacies to re-import both biological drugs and insulin that were originally manufactured in the United States and then exported for international sale. This action preceded the finalization of a rulemaking on September 24, 2020 that allows states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants would be required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, the FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

The fourth order would end drug rebates used by health plan sponsors, pharmacies or pharmacy benefit managers, or PBMs, in operating the Medicare Part D program. Specifically, the order directs HHS to exclude from safe harbor protections under the federal anti-kickback statute retroactive price reductions that are not applied at the point-of-sale. Instead, the order requires HHS to establish new safe harbors that would allow health plan sponsors, pharmacies, and PBMs to pass on those discounts to consumers at point-of-sale in order to lower the patient's out-of-pocket costs and permit the use of certain bona fide PBM service fees. Each of these orders directs the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

Following issuance of these orders, President Trump issued a fifth executive order which instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including especially China. The order is meant reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. Increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Governments outside of the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

If we or any third-party manufacturers we engage now or in the future fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs or liabilities that could harm our business.

We and third-party manufacturers we engage now are, and any third-party manufacturers we may engage in the future will be, 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. Liability under certain environmental laws governing the release and cleanup of hazardous materials is joint and several and could be imposed without regard to fault. We also could incur significant costs associated with civil or criminal fines and penalties or become subject to injunctions limiting or prohibiting our activities for failure to comply with such laws and regulations.

Although we maintain general liability insurance as well as 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, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

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. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Further, with respect to the operations of our current and any future third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products. In addition, our supply chain may be adversely impacted if any of our third-party contract manufacturers become subject to injunctions or other sanctions as a result of their non-compliance with environmental, health and safety laws and regulations.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, be precluded from developing manufacturing and selling certain products outside the United States or be required to develop and implement costly compliance programs, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act, the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. Compliance with the FCPA, in particular, is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted. If we expand our operations outside of the United States, we will need to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control laws by United Kingdom, U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Our employees, independent contractors, consultants and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of fraud or other misconduct by our employees, independent contractors, consultants and vendors. Misconduct by these partners could include intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or

disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of HIPAA, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive. We are also exposed to risks in connection with any insider trading violations by employees or others affiliated with us. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity 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 be in compliance with such laws, standards, regulations, guidance or codes of conduct. 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 fines or other sanctions.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of any collaborators, contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such systems are also vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, third-party vendors and/or business partners, or from cyber-attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, unauthorized access to or deletion of files, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. For example, we make extensive use of cloud-based storage systems, and in October 2018, we experienced a breach of one such system. While this breach did not result in the permanent loss or theft of any of our critical information or any other material consequences, it could have, and while we took steps to remediate this breach, such as establishing multi-factor authentication and implementing improvements to our data securities protocols, we cannot guarantee that the measures we have taken to date, and actions we may take in the future, will be sufficient to remediate any future breaches. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

While we have not experienced any material system failure, accident, cyber-attack 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 development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials 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 were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

Risks Related to Employee Matters and Managing Growth

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

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment offer letters with our executive officers, each of them may terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to our Common Stock

Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to control or significantly influence all matters submitted to stockholders for approval.

As of November 4, 2020, our executive officers and directors and our stockholders who owned more than 5% of our outstanding common stock in the aggregate beneficially owned shares representing approximately 63.4% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management and board of directors; or
- delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;

- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

An active trading market for our common stock may not be sustained.

Our shares of common stock began trading on the Nasdaq Global Market on July 18, 2019. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares, or at all.

If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. There can be no assurance that existing analysts will continue to cover us or that new analysts will begin to cover us. There is also no assurance that any covering analyst will provide favorable coverage. Although we have obtained analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provides more favorable relative recommendations about our competitors, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for our stockholders.

The trading price of our common stock has been, and is likely to continue to be, volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results of or developments in preclinical studies and clinical trials of our product candidates or those of our competitors or potential collaborators;
- our success in commercializing our product candidates, if and when approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or the financial results of companies that are perceived to be similar to us;

- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation has often been instituted against that company. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and divert management’s attention and resources. Furthermore, negative public announcements of the results of hearings, motions or other interim proceedings or developments could have a negative effect on the market price of our common stock.

A significant portion of our total outstanding shares are eligible to be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Persons who were our stockholders prior to our IPO continue to hold a substantial number of shares of our common stock. If such persons sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline.

Moreover, holders of a substantial number of shares of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

In June 2020, we issued and sold 4,029,411 shares of common stock to investors in a private placement. We have filed a registration statement covering the resale of these shares by the purchasers in the private placement and have agreed to keep such registration statement effective until the date the shares covered by the registration statement have been sold or can be resold without restriction under Rule 144 of the Securities Act of 1933, as amended, or the Securities Act.

We currently have on file with the SEC a universal shelf registration statement which allows us to offer and sell registered common stock, preferred stock, debt securities, depositary shares, warrants and/or units from time to time pursuant to one or more offerings up to an aggregate of $250 million, at prices and terms to be determined at the time of sale.

In addition, we have filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates.

We are an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an “emerging growth company,” or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We may remain an EGC until December 31, 2024, although if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have annual gross revenues of $1.07 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than $1 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;

- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We may choose to take advantage of some or all of the available exemptions. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an EGC.

We have incurred and will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company we have incurred, and particularly after we are no longer an EGC, we will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements, and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our Annual Report on Form 10-K for the year ending December 31, 2020. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial and accounting personnel, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders' 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. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future.

Our certificate of incorporation designates the state courts in the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors, officers and employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware or (4) any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine. This exclusive forum provision will not apply to actions arising under the Securities Act or the Securities Exchange Act of 1934, as amended.

This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could materially adversely affect our business, financial condition and operating results.

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

Recent Sales of Unregistered Securities

Other than as reported in a Current Report on Form 8-K, we did not sell any securities that were not registered under the Securities Act during the three months ended September 30, 2020.

Use of Proceeds from Initial Public Offering

On July 22, 2019, we completed our IPO, pursuant to which we issued and sold 4,500,000 shares of our common stock at a public offering price of $16.00 per share. The offer and sale of all of the shares of our common stock in our IPO were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-232260), which was declared effective by the Securities and Exchange Commission, or SEC, on July 17, 2019. Morgan Stanley & Co. LLC, BofA Securities, Inc. and SVB Leerink LLC. acted as joint book-running managers for our IPO. The IPO commenced on July 17, 2019 and terminated without the sale of the 675,000 shares registered for potential issuance upon exercise of the underwriters' option to purchase additional shares in the IPO.

We received aggregate gross proceeds from our IPO of $72.0 million, or aggregate net proceeds of $63.9 million after deducting underwriting discounts and commissions and offering expenses payable by us. None of the underwriting discounts and commissions or offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10% or more of our common stock or to any of our affiliates.

We have used approximately $26.5 million of the net proceeds from the IPO as of September 30, 2020 to fund the clinical development of losmapimod, to advance our pipeline of clinical-stage and preclinical product candidates, and to research and develop additional preclinical product candidates using our platform, and for working capital and other general corporate purposes. We have invested the unused net proceeds from the offering in cash equivalents and marketable securities. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on July 18, 2019.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

	Issuer Purchases of Equity Securities			
Period	**(a) Total Number of Shares Purchased(1)**	**(b) Average Price Paid per Share**	**(c) Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs**	**(d) Approximate Dollar Value of Shares that May Yet Be Purchased Under the Plans or Programs**
July 1, 2020 through July 31, 2020	4,581	$ 0.07	—	$ —
August 1, 2020 through August 31, 2020	—	—	—	—
September 1, 2020 through September 30, 2020	—	—	—	—
Total	4,581	$ 0.07	—	$ —

(1) Represents shares of unvested common stock that were repurchased by us from certain former employees upon termination of employment in accordance with the terms of the applicable employee's restricted stock agreement. We repurchased the shares from the former employees at the original purchase price.

Item 6. Exhibits.

Exhibit Number	Description
10.1*†	Collaboration and License Agreement, dated as of July 20, 2020, by and between the Registrant and MyoKardia, Inc.
10.2*†	First Amendment to the Right of Reference and License Agreement, dated as of September 23, 2020, by and among the Registrant, GlaxoSmithKline Intellectual Property (No. 2) Limited, GlaxoSmithKline LLC and Glaxo Group Limited.
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1+	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2+	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

† Certain portions of this exhibit have been omitted because they are not material and would likely cause competitive harm to the Registrant if disclosed.

* Filed herewith.

+ Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

FULCRUM THERAPEUTICS, INC.

Date: November 10, 2020

By: /s/ Robert J. Gould
Robert J. Gould, Ph.D.
Chief Executive Officer and President, Director

Date: November 10, 2020

By: /s/ Bryan Stuart
Bryan Stuart
Chief Operating Officer (Principal Financial Officer)

Exhibit.10.1

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

COLLABORATION AND LICENSE AGREEMENT

BY AND BETWEEN

FULCRUM THERAPEUTICS, INC.

AND

MYOKARDIA, INC.

COLLABORATION AND LICENSE AGREEMENT

This Collaboration and License Agreement (this "**Agreement**") is entered into as of July 20, 2020 (the "**Effective Date**"), by and between Fulcrum Therapeutics, Inc., a corporation organized under the laws of the State of Delaware ("**Fulcrum**"), and MyoKardia, Inc., a corporation organized under the laws of the State of Delaware ("**MyoKardia**"). MyoKardia and Fulcrum each may be referred to herein individually as a "**Party**" or collectively as the "**Parties**."

RECITALS

WHEREAS, Fulcrum owns or controls certain intellectual property relating to the interrogation, analysis and mapping of novel signaling pathways regulating gene expression within biological systems in support of the identification of gene or protein targets amenable to therapeutic (whether prophylactic, palliative, diagnostic or curative) interventions;

WHEREAS, MyoKardia owns or controls certain intellectual property relating to cardiovascular research and disease and is a biopharmaceutical company dedicated to the discovery, development, and commercialization of therapeutics to treat serious and rare diseases, including cardiomyopathies;

WHEREAS, MyoKardia and Fulcrum desire to enter into this Agreement, pursuant to which MyoKardia and Fulcrum will work to identify one or more target(s) capable of modulating one or more certain gene(s) of interest for the treatment of one or more genetically defined cardiomyopathies and other indications; and

WHEREAS, MyoKardia may subsequently exploit products that bind to and modulate the activity of, or express, one or more selected cardiomyopathy target(s), in accordance with the terms and subject to the conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the mutual promises and covenants set forth below and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereto agree as follows:

Article 1
DEFINITIONS

For purposes of this Agreement, the following capitalized terms will have the following meanings:

1.1 "**AAA**" has the meaning set forth in Section 12.1.3 (Resolution by Mediation).

1.2 "**Abandoned Patent Right**" has the meaning set forth in Section 7.4.3 (Joint Patent Rights).

1.3 "**Acquired Party**" means (a) any Third Party that Fulcrum acquires through an Affiliate Acquisition following the Effective Date, and (b) any of such Third Party's Affiliates (other than Fulcrum or any Affiliate of Fulcrum that existed prior to such Affiliate Acquisition).

1.4 "**Acquired Product**" has the meaning set forth in Section 6.3.5(b) (Skipped Milestones).

1.5 "**Acquiring Party**" means (a) any Third Party that acquires Fulcrum through a Change of Control of Fulcrum following the Effective Date, and (b) any of such Third Party's Affiliates (other than Fulcrum or any Affiliate of Fulcrum that existed prior to such Change of Control).

1.6 "**Act**" has the meaning set forth in Section 7.4.5 (Cooperation).

1.7 "**Additional Cardiomyopathy Target**" means a Cardiomyopathy Target Candidate that modulates the expression of a Potential Project Gene other than the Lead Project Gene selected in accordance with Section 3.6.6 (Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets). An Additional Cardiomyopathy Target will also include any [**]. With respect to the immediately preceding sentence, any [**] intended to be included as described in such sentence shall be identified and documented in writing at the time that Selected Targets are designated in accordance with Section 3.6.2 (Part 1 Validation).

1.8 "**Additional Project Gene**" has the meaning set forth in Section 3.6.6 (Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets).

1.9 "**Additional Project Gene Exclusive Indications**" has the meaning set forth in Section 3.6.6 (Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets).

1.10 "**Affiliate**" means, as of any point in time and for so long as such relationship continues to exist, with respect to a Party, any other Person that controls, is controlled by or is under common control with such Party. For purposes of this definition, "control" means that a Person (a) owns or controls, directly or indirectly, more than fifty percent (50%) of the equity securities of the subject Person entitled to vote in the election of directors (or, in the case of a Person that is not a corporation, for the election of the corresponding managing authority), or (b) possesses, directly or indirectly, the power to direct or cause the direction of the management or policies of such Person (whether through ownership of securities or other ownership interests, by contract or otherwise).

1.11 "**Affiliate Acquisition**" has the meaning set forth in Section 5.5.3 (Exception for Affiliate Acquisition).

1.12 "**Agreement**" has the meaning set forth in the preamble to this Agreement.

1.13 "**Anticipated Research Budget**" has the meaning set forth in Section 4.3 (Reporting).

1.14 "**Applicable Law**" means all applicable laws, statutes, rules, regulations (including any applicable rules, regulations, guidelines, or other requirements of Regulatory Authorities) that may be in effect from time to time.

1.15 "**Approval Milestones**" has the meaning set forth in Section 6.3.5(b) (Skipped Milestones).

1.16 "**Bankruptcy Code**" has the meaning set forth in Section 10.2.4(b) (Termination for Insolvency).

1.17 "**Business Day**" means a day other than a Saturday, Sunday or bank or other public holiday in Boston, Massachusetts or San Francisco, California.

1.18 "**Calendar Quarter**" means the respective periods of three consecutive calendar months ending on March 31, June 30, September 30 or December 31, during the Term, or the applicable part thereof during the first or last calendar quarter of the Term.

1.19 "**Calendar Year**" means any calendar year ending on December 31, or the applicable part thereof during the first or last calendar year of the Term.

1.20 "**Cardiomyopathy Milestone Target**" means an Initial Cardiomyopathy Milestone Target or a Secondary Cardiomyopathy Milestone Target.

1.21 "**Cardiomyopathy Target**" means the Lead Cardiomyopathy Target, a Follow-On Cardiomyopathy Target, or an Additional Cardiomyopathy Target, as applicable.

1.22 "**Cardiomyopathy Target Candidate**" has the meaning set forth in Section 3.6.3 (Part 2 Validation).

1.23 "**CDA**" means the Confidential Disclosure Agreement by and between MyoKardia and Fulcrum, dated as of May 3, 2019.

1.24 "**Cell Toxicity Screen**" means the screen identified as the Cell Toxicity Screen in Figure 2 of the Research Plan.

1.25 "**Change of Control**" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of greater than fifty percent (50%) of the outstanding voting securities of such Party; (b) a merger, consolidation, recapitalization, or reorganization of such Party is consummated that results in shareholders or equity holders of such Party immediately prior to such transaction, ceasing to own at least fifty percent (50%) of the combined outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve a plan of complete liquidation of such Party; or (d) the sale or transfer to a Third Party of all or substantially all of such Party's consolidated assets taken as a whole, through one or more related transactions.

1.26 "**Clinical Proof-of-Concept**" means [**].

1.27 "**Clinical Trial**" means a Phase 1 Clinical Trial, Phase 2 Clinical Trial, Phase 3 Clinical Trial, or any combination thereof.

1.28 "**Collaboration Molecule**" means any therapeutic agent that (a) (i) binds to and modulates the activity of a Cardiomyopathy Target or (ii) expresses, in whole or in part, a

Cardiomyopathy Target ((i) or (ii), "**directed against**" a Cardiomyopathy Target) and (b) (i) is Covered by a Fulcrum Patent Right that claims or discloses a method of using such therapeutic agent, (ii) with respect to which MyoKardia or any of its Affiliates or Licensees has initiated Lead Optimization within [**] after the Final Data Package Delivery Date or (iii) MyoKardia or any of its Affiliates or Licensees has in-licensed or acquired within [**] after the Final Data Package Delivery Date, (whether or not, in the case of clause (ii) or this clause (iii), Covered by a Fulcrum Patent Right); *provided* that [**] (collectively, the "**Exceptions**") will not be deemed a Collaboration Molecule.

1.29 "**Combination Product**" means a Product that is (a) sold in the form of a combination that contains or comprises one or more additional therapeutically active pharmaceutical agents (whether co-formulated or co-packaged or otherwise sold for a single price) other than a Molecule in the Product, or (b) sold for a single price together with any delivery device or component therefor, companion diagnostic, process, service, or therapy other than the Product (each, an "**Other Component**"); or (c) defined as a "combination product" by the FDA pursuant to 21 C.F.R. §3.2(e) or its foreign equivalent.

1.30 "**Commercially Reasonable Efforts**" means, with respect to each Party and its Affiliates, [**].

1.31 "**Competing Product**" has the meaning set forth in Section 5.5.1(b) (Cardiomyopathy Target Candidates).

1.32 "**Competitive Infringement**" has the meaning set forth in Section 7.6.1 (Notice of Competitive Infringement).

1.33 "**Confidential Information**" means, with respect to each Party, all Know-How or other non-public information in whatever form (including, written, oral, or visual), including proprietary information and materials (whether or not patentable) regarding or embodying such Party's or its Representatives' technology, products, services, intellectual property, software, inventions, processes, formulas, technical data, designs, drawings, research and development plans, finances, operations or business information or objectives, that is communicated, provided or made available by or on behalf of the Disclosing Party to the Receiving Party or its Representatives, on or after the Effective Date. In addition, "Confidential Information" will include: (a) confidential information of a Third Party provided or made available by or on behalf of the Disclosing Party to the Receiving Party or its Representatives on or after the Effective Date, which the Disclosing Party has a legal right to disclose to the Receiving Party under terms of confidentiality; and (b) any notes, analyses, compilations, studies, interpretations, memoranda or other documents prepared by the Receiving Party or its Representatives to the extent containing, reflecting or based upon, in whole or in part, any Confidential Information communicated, furnished or made available to the Receiving Party or its Representatives pursuant hereto. The terms and conditions of this Agreement will be considered Confidential Information of both Parties being deemed the Receiving Party of such Confidential Information. Notwithstanding anything to the contrary herein, all Research Data ([**]) and all Data Packages ([**]) will be the Confidential Information of both Parties. All [**] will be the Confidential Information of MyoKardia. Notwithstanding anything to the contrary in the foregoing, all "Confidential Information," as that term is defined in the CDA, is Confidential Information under this Agreement, all "Confidential

Information," as that term is defined in the CDA, disclosed by MyoKardia under the CDA is the Confidential Information of MyoKardia under this Agreement, and all "Confidential Information," as that term is defined in the CDA, disclosed by Fulcrum under the CDA is the Confidential Information of Fulcrum under this Agreement.

1.34 "**Control**" or "**Controlled**" means, as to any Know-How, Patent Right or other intellectual property right, the possession (whether by ownership or license, other than by a license granted pursuant to this Agreement) by a Person of the ability to grant to another Person access, ownership, a license or a sublicense as required herein to such Know-How or Patent Right, without (a) violating the terms of any agreement or other arrangement with any Third Party or (b) requiring the payment of any consideration to any Third Party, except, in the case of this clause (b), to the extent such other Person has agreed to be responsible for the payment of such consideration.

1.35 "**Cover**," "**Covering**" or "**Covers**" means, as to a particular subject matter at issue and a Patent Right, that, in the absence of a license granted under, or ownership of, such Patent Right, the making, using, keeping, selling, offering for sale or importation of such subject matter at issue would infringe a Valid Claim in such Patent Right or, with respect to a Valid Claim that is a claim of a pending patent application, the making, using, keeping, selling, offering for sale or importation of such subject matter at issue would infringe such Valid Claim in such Patent Right if such pending claim were to issue in an issued patent without modification.

1.36 "**Covered Research Expenses**" has the meaning set forth in Section 3.8 (Research Funding).

1.37 "**COVID-19 Pandemic**" means the global novel coronavirus (COVID-19) pandemic.

1.38 "**CPI**" means the Consumer Price Index – Urban Wage Earners and Clerical Workers, U.S. City Average (All Items, 1982-84 = 100) published by the United States Department of Labor, Bureau of Labor Statistics, or any successor index thereto.

1.39 "**CRC Screen**" means the nine (9) point concentration response curve, identified as the CRC Screen in paragraph 5.1(a) of the Research Plan.

1.40 "**CRISPR Confirmation**" means the use of CRISPR technology to confirm an active hit from the Primary Screen by genetic manipulation, as conducted in Part 1 Validation activities.

1.41 "**Data Package**" means the Screen Preparation Data Package, Primary Screen Data Package, Preliminary Validation Data Package, Part 1 Validation Package, Part 2 Validation Summary and the Final Data Package, as applicable.

1.42 "**Development Candidate**" means a Molecule that MyoKardia has deemed suitable, in accordance with its standard internal processes, for advancement into IND-enabling studies and intends to advance into a Clinical Trial.

1.43 "**Development Candidate Designation**" means the earlier of [**].

1.44 "**Development Milestone**" has the meaning set forth in Section 6.3.3 (Development Milestones).

1.45 "**Development Milestone Payments**" has the meaning set forth in Section 6.3.3 (Development Milestones).

1.46 "**directed against**" has the meaning set forth in Section 1.28 (Collaboration Molecule) or Section 1.47 (Disclosed Molecule), as applicable.

1.47 "**Disclosed Molecule**" means any therapeutic agent that (a)(i) binds to and modulates the activity of a Disclosed Target or (ii) expresses, in whole or in part, a Disclosed Target ((a) or (b), "**directed against**" a Disclosed Target) and (b) (i) is Covered by a Fulcrum Patent Right that claims or discloses a method of using such therapeutic agent, (ii) with respect to which MyoKardia or any of its Affiliates or Licensees has initiated Lead Optimization within [**] after the Final Data Package Delivery Date or (iii) MyoKardia or any of its Affiliates or Licensees has in-licensed or acquired within [**] after the Final Data Package Delivery Date; *provided* that any therapeutic agent that meets the criteria of an Exception will not be deemed a Disclosed Molecule.

1.48 "**Disclosed Product**" mean a pharmaceutical product that contains a Disclosed Molecule.

1.49 "**Disclosed Target**" means a Selected Target that is not a Cardiomyopathy Target; *provided* that any target that, through documented evidence, can be shown to have been identified by a Third Party collaborator of MyoKardia without using confidential results generated by the Research Activities shall not be deemed a Disclosed Target hereunder. A Disclosed Target will also include any [**]. With respect to the immediately preceding sentence, any [**] intended to be included as described in such sentence shall be identified and documented in writing at the time that Selected Targets are designated in accordance with Section 3.6.2 (Part 1 Validation).

1.50 "**Disclosing Party**" has the meaning set forth in Section 11.1 (Confidentiality).

1.51 "**Dollar**," "**USD**," or "**$**" means legal tender in the U.S.

1.52 "**Effective Date**" has the meaning set forth in the preamble of this Agreement.

1.53 "**EMA**" means the European Medicines Agency and any successor entity thereto.

1.54 "**Engineered Cell Line**" means a genetically engineered cell line for use in the Primary Screen, carrying a relevant mutation, initially [**].

1.55 "**European Commission**" means the European Commission or any successor entity that is responsible for granting marketing approvals authorizing the sale of pharmaceuticals in the European Union.

1.56 "**European Union**" or "**EU**" means (a) all countries or territories that are officially part of the European Union, as constituted from time to time, and (b) the United Kingdom.

1.57 "**Exceptions**" has the meaning set forth in Section 1.28 (Collaboration Molecule).

1.58 "**Excluded Claim**" has the meaning set forth in Section 12.1.4 (Excluded Claims).

1.59 "**Excluded Field**" means the treatment, prophylaxis or diagnosis of [**].

1.60 "**Excluded Know-How**" means any Know-How to the extent Controlled by any Acquiring Party, which Know-How is (a) Controlled by such Acquiring Party immediately prior to the effective date of the applicable Change of Control or (b) Controlled by such Acquiring Party on or after the effective date of such Change of Control but, in each case ((a) and (b)), is not Controlled by Fulcrum or an Affiliate of Fulcrum (excluding for purposes of this provision, such Acquiring Party and Affiliates of Fulcrum that are Affiliates by virtue of controlling, being controlled by or under common control with such Acquiring Party) and was invented, created or obtained without the direct or indirect use of any Fulcrum Technology.

1.61 "**Excluded Patent Rights**" means any Patent Right to the extent Controlled by any Acquiring Party, which Patent Right is (a) Controlled by such Acquiring Party immediately prior to the effective date of the applicable Change of Control or (b) Controlled by such Acquiring Party on or after the effective date of such Change of Control but in each case ((a) and (b)), is not Controlled by Fulcrum or an Affiliate of Fulcrum (excluding for purposes of this provision, such Acquiring Party and Affiliates of Fulcrum that are Affiliates by virtue of controlling, being controlled by or under common control with such Acquiring Party) and was invented, created or obtained without the direct or indirect use of any Fulcrum Technology.

1.62 "**Excluded Target**" means, as of a particular period in time, a target that is (a) (i) subject to a binding contractual obligation under a written agreement with a Third Party that would conflict with the inclusion of a target as a Selected Target hereunder or (ii) the subject of a *bona fide* development program specific to such target for which Fulcrum has initiated [**] and Fulcrum intends in good faith to continue to pursue or (b) MyoKardia has identified on Schedule 1.62(b); *provided that*, until the selection of Cardiomyopathy Target Candidates, no Selected Targets may become Excluded Targets without MyoKardia's prior written permission.

1.63 "**Excluded Technology**" means the Excluded Patent Rights and the Excluded Know-How.

1.64 "**Exclusivity Period**" has the meaning set forth in Section 5.5.1(a) (Data).

1.65 "**Executive Officers**" means the [**] of Fulcrum, initially [**], and the [**] of MyoKardia, initially [**] or either of their respective designees having sufficient authority to resolve the applicable matter.

1.66 "**Existing Platform Patent Rights**" has the meaning set forth in Section 8.2.1 (Representations and Warranties of Fulcrum).

1.67 "**FD&C Act**" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and the rules and regulations promulgated thereunder.

1.68 "**FDA**" means the United States Food and Drug Administration and any successor entity thereto.

1.69 "**Field**" means all fields of use except, for so long as Fulcrum is contractually prohibited from granting rights with respect thereto, the Excluded Field.

1.70 "**Final Data Package**" has the meaning set forth in Section 3.6.4 (Final Data Package).

1.71 "**Final Data Package Delivery Date**" has the meaning set forth in Section 3.6.4 (Final Data Package).

1.72 "**First Commercial Sale**" means with respect to a Product in a country, the first commercial sale in a country by MyoKardia, its Affiliates, or Licensees of such Product to a Third Party following receipt of Regulatory Approval for such Product; *provided*, that First Commercial Sale does not include (a) any sale to or between MyoKardia, its Affiliates, or Licensees, (b) any use of such Product in Clinical Trials, pre-clinical studies or other development activities, or (c) the disposal or transfer of such Product for a *bona fide* charitable purpose, including expanded access or compassionate use.

1.73 "**First Designation Period**" has the meaning set forth in Section 3.6.5 (Selection of Lead Cardiomyopathy Target).

1.74 "**Follow-On Cardiomyopathy Target**" means a Cardiomyopathy Target Candidate other than the Lead Cardiomyopathy Target that modulates the expression of the Lead Project Gene selected in accordance with Section 3.6.6 (Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets). A Follow-On Cardiomyopathy Target will also include any [**]. With respect to the immediately preceding sentence, any [**] intended to be included as described in such sentence shall be identified and documented in writing at the time that Selected Targets are designated in accordance with Section 3.6.2 (Part 1 Validation).

1.75 "**FTE**" means a full-time equivalent employee (*i.e.*, one fully-committed or multiple partially-committed employees aggregating to one full-time employee) employed or contracted by a Party and assigned to perform specified work, with such commitment of time and effort to constitute one employee performing such work on a full-time basis, which for purposes of activities performed under this Agreement will be [**]. For the avoidance of doubt, FTEs shall not include personnel performing administrative and corporate functions, including but not limited to human resources, legal and investor relations.

1.76 "**FTE Hourly Rate**" means, with respect to any Fulcrum FTE performing Fulcrum Research Activities, a rate of [**], subject to annual increases beginning on January 1, 2021 to reflect, as of December 31 of the then-most recently completed Calendar Year, the increase in the level of the CPI in the U.S. as compared to December 31 of the immediately preceding Calendar Year. The FTE Hourly Rate is "fully burdened" and includes employee salaries, benefits, bonuses and overhead allocated to such employee's work hereunder, but excludes, for the avoidance of doubt, Out-of-Pocket Expenses.

1.77 "**Fulcrum**" has the meaning set forth in the preamble of this Agreement.

1.78 "**Fulcrum Indemnified Party**" has the meaning set forth in Section 9.1.1 (Indemnification by MyoKardia).

1.79 "**Fulcrum Patent Rights**" means the Patent Rights within the Fulcrum Technology.

1.80 "**Fulcrum Platform**" means Fulcrum's proprietary high-throughput discovery platform designed to identify and validate biological drug targets that balance the expression of the genes known to drive or ameliorate disease and Fulcrum's proprietary library of compounds, in each case, as such exist from time to time during the Research Term.

1.81 "**Fulcrum Research Activities**" means any research conducted or to be conducted by or on behalf of Fulcrum (including by an Affiliate or subcontractor of Fulcrum) under the Research Plan.

1.82 "**Fulcrum Technology**" means all Know-How and Patent Rights (a) Controlled by Fulcrum (i) as of the Effective Date or (ii) during the Research Term and, in the case of this clause (ii), (x) developed in the conduct of the Fulcrum Research Activities or (y) otherwise to the extent related to, covering or disclosing any method of modulating the activity of any Target for the treatment of any Lead Project Gene Exclusive Indication or Additional Project Gene Exclusive Indication, and (b) that are necessary to research, develop, manufacture, commercialize, or otherwise exploit Molecules and Products in the Territory in the Field, other than any Excluded Technology. For the avoidance of doubt, the Fulcrum Technology includes Fulcrum's interest in the Joint Technology.

1.83 "**GAAP**" means generally accepted accounting principles as practiced in the United States, consistently applied.

1.84 "**Generic Product**" means, with respect to a Product, and on a Product-by-Product and country-by-country basis, any product (including a "generic product," "biogeneric," "follow-on biologic," "follow-on biological product," "follow-on protein product," "similar biological medicinal product," or "biosimilar product") approved by way of an abbreviated regulatory mechanism by the relevant Regulatory Authority in a country in reference to such Product, that in each case: (a) is sold in the same country (or is commercially available in the same country via import from another country) as such Product by any Third Party that is not a Sublicensee of MyoKardia or its Affiliates and that did not purchase such product in a chain of distribution that included any of MyoKardia or any of its Affiliates or its Sublicensees; and (b) meets the equivalency determination by the applicable Regulatory Authority in such country (including a determination that the product is "comparable," "interchangeable," "bioequivalent," "biosimilar" or other term of similar meaning, with respect to the Product), in each case, as is necessary to permit substitution of such product for the Product under Applicable Law in such country.

1.85 "**Governmental Authority**" means any court, agency, department, authority or other instrumentality of any national, state, county, city or other political subdivision.

1.86 "**IND**" means an Investigational New Drug Application submitted under the FD&C Act, or an analogous application or submission with any analogous agency or Regulatory

Authority outside of the United States for the purposes of obtaining permission to conduct Clinical Trials.

1.87 "**Indemnified Party**" has the meaning set forth in Section 9.1.3 (Procedure).

1.88 "**Indemnifying Party**" has the meaning set forth in Section 9.1.3 (Procedure).

1.89 "**Initial Cardiomyopathy Milestone Target**" means (a) the Lead Cardiomyopathy Target, (b) a Follow-On Cardiomyopathy Target or (c) a Disclosed Target that modulates the expression of the Lead Project Gene.

1.90 "**Initiation**" or "**Initiate**" means, with respect to any Clinical Trial, dosing of the first human subject in such Clinical Trial.

1.91 "**Insolvency Event**" has the meaning set forth in Section 10.2.4(a) (Termination for Insolvency).

1.92 "**Insolvent Party**" has the meaning set forth in Section 10.2.4(a) (Termination for Insolvency).

1.93 "**Joint Know-How**" means the Target Know-How and any other Know-How that is jointly owned by the Parties pursuant to Sections 7.1.1 and 7.1.4(b)(i) (Ownership of Technology).

1.94 "**Joint Patent Rights**" means the Target Patent Rights and any other Patent Rights that Cover Joint Know-How.

1.95 "**Joint Steering Committee"** or "**JSC**" means has the meaning set forth in Section 2.1 (Joint Steering Committee).

1.96 "**Joint Technology**" means the Joint Know-How and the Joint Patent Rights.

1.97 "**Jointly Controlled Patent Rights**" has the meaning set forth in Section 7.4.3 (Joint Patent Rights).

1.98 "**Know-How**" means confidential (as of the time of disclosure) intellectual property, data, results, pre-clinical and clinical protocols and data from studies and Clinical Trials, chemical structures, chemical sequences, information, inventions, know-how, formulas, trade secrets, techniques, methods, processes, procedures and developments; *provided* that Know-How does not include Patent Rights.

1.99 "**Lead Cardiomyopathy Target**" means the Cardiomyopathy Target Candidate selected as the Lead Cardiomyopathy Target in accordance with Section 3.6.5 (Selection of Lead Cardiomyopathy Target). The Lead Cardiomyopathy Target will also include any [**]. With respect to the immediately preceding sentence, any [**] intended to be included as described in such sentence shall be identified and documented in writing at the time that Selected Targets are designated in accordance with Section 3.6.2 (Part 1 Validation).

1.100 "**Lead Cardiomyopathy Target Notice**" has the meaning set forth in Section 3.6.5 (Selection of Lead Cardiomyopathy Target).

1.101 "**Lead Optimization**" means the process of optimizing the properties of structurally-related compounds directed against a Target with the intent of identifying a suitable Development Candidate.

1.102 "**Lead Project Gene**" has the meaning set forth in Section 3.6.5 (Selection of Lead Cardiomyopathy Target).

1.103 "**Lead Project Gene Exclusive Indications**" has the meaning set forth in Section 3.6.5 (Selection of Lead Cardiomyopathy Target).

1.104 "**Liability**" has the meaning set forth in Section 9.1.1 (Indemnification by MyoKardia).

1.105 "**Licensed Product**" means a pharmaceutical product containing a Collaboration Molecule.

1.106 "**Licensee**" means (a) any Sublicensee or (b) any other entity to which MyoKardia or any of its Affiliates, directly or through multiple tiers, grants any rights to commercialize a Product.

1.107 "**Loss of Market Exclusivity**" means an event where, with respect to any Product in any country: (a) one or more Generic Products are being marketed in such country; and (b) aggregate Net Sales of such Product sold in that country during any Calendar Quarter following introduction of such Generic Product in such country have fallen by at least [**] in that country as compared to the average quarterly total aggregate Net Sales of such Product sold in such country during the last [**] prior to the Calendar Quarter in which such Generic Product was first introduced.

1.108 "**Major Market Countries**" means [**].

1.109 "**Medicinal Chemistry Services**" means services performed by Fulcrum, as requested by MyoKardia, outside of the Research Plan relating to the generation and optimization of Collaboration Molecules.

1.110 "**MHRA**" means the Medicines and Healthcare Products Regulatory Agency in the United Kingdom and any successor entity thereto.

1.111 "**Milestone**" means a Development Milestone, Preclinical Milestone, [**] Milestone or Sales Milestone.

1.112 "**Milestone Payment**" means a Development Milestone Payment, Preclinical Milestone Payment, [**] Milestone Payment or Sales Milestone Payment.

1.113 "**Molecule**" means any Collaboration Molecule or Disclosed Molecule.

1.114 "**MyoKardia**" has the meaning set forth in the preamble to this Agreement.

1.115 "**MyoKardia Indemnified Party**" has the meaning set forth in Section 9.1.2 (Indemnification by Fulcrum).

1.116 "**MyoKardia Patent Rights**" means the Patent Rights within the MyoKardia Technology.

1.117 "**MyoKardia Research Activities**" means any research conducted or to be conducted by or on behalf of MyoKardia (including by an Affiliate or subcontractor of MyoKardia) under the Research Plan.

1.118 "**MyoKardia Technology**" means all Know-How and Patent Rights Controlled by MyoKardia or its Affiliates as of the Effective Date or during the Term that are necessary or useful for Fulcrum to perform the Fulcrum Research Activities.

1.119 "**NDA**" means a New Drug Application submitted to the FDA in the United States in accordance with the FD&C Act with respect to a pharmaceutical product or any analogous application or submission with any Regulatory Authority outside of the United States.

1.120 "**Net Sales**" means, for any Calendar Quarter during the Royalty Term and for any country, the total aggregate amount invoiced during such Calendar Quarter in such country by MyoKardia, its Affiliates, Licensees or, to the extent not a Licensee, any of their distributors (the "**Selling Party**") in such country for all sales of the Products to Third Parties (other than to MyoKardia, its Affiliates, or Licensees), after deducting, if not previously deducted, from the amount invoiced or received, the following deductions, in each case, in accordance with GAAP or the Selling Party's accounting standard, actually applied or taken, and specifically allocable to such sales of the Products:

1.120.1 [discounts (including trade, cash and quantity discounts), cash and non-cash coupons, charge back payments and rebates granted to managed health care organizations, hospital or group purchasing organizations, or to federal, state and local governments, their agencies, and purchasers and reimbursers or to customers or required by Applicable Law (including governmental medical assistance programs);

1.120.2 actually granted credits, allowances, repayments, discounts to and chargebacks for claims, spoiled, damaged, or outdated goods, rejections or returns of the Products, including Products returned in connection with recalls or withdrawals;

1.120.3 any sales, value added or similar taxes, insurance, custom duties, excise (including annual fees due under Section 9008 of the United States Patient Protection and Affordable Care Act of 2010 (Pub. L. No. 111-48)) or other similar governmental charges levied directly on the production, sale, transportation, delivery or use of a Product that are paid by or on behalf of the Selling Party, but not including any tax levied with respect to income;

1.120.4 actual freight and insurance costs and other expenses incurred in distributing, warehousing, importing, handling and transporting the Product to distributors or customers;

1.120.5 [**];

1.120.6 [**];

1.120.7 retroactive price reductions or billing corrections;

1.120.8 amounts that are written off as uncollectible; *provided*, that if any such written-off amounts are subsequently collected, such collected amounts will be included in Net Sales in the Calendar Quarter in which they are subsequently collected; and

1.120.9 other specifically identifiable amounts deducted for reasons similar to those listed above in accordance with the Selling Party's accounting standards.

Net Sales does not include (a) any sale of such Product to or between MyoKardia, its Affiliates or Licensees for further sale by such entity (but includes the subsequent sale by such entity to a Third Party), (b) sales by a Third Party distributor who purchases such Product for resale, (c) samples of Product used to promote additional Net Sales, in amounts consistent with normal business practices of a Selling Party, (d) any use of such Product in Clinical Trials, pre-clinical studies or other development activities, or (e) the disposal, use or transfer of such Product at or below cost for a *bona fide* charitable purpose, including expanded access, compassionate use, patient assistance or named patient use.

If a Product is sold as part of a Combination Product in a country in the Territory, then Net Sales for the Product included in such Combination Product in such country will be calculated as follows:

If the Product and the Other Components in such Combination Product are both sold separately in such country, then Net Sales for the Product will be calculated by multiplying actual Net Sales of such Combination Product in such country by the fraction A/(A+B), where: "A" is the gross invoice price in such country of the Product when sold separately; and "B" is the gross invoice price in such country of the Other Components contained in the Combination Product when sold separately.

If the Product is sold separately in such country, but the Other Components contained in the Combination Product are not sold separately in such country, then Net Sales for the Product will be calculated by multiplying actual Net Sales of such Combination Product in such country by the fraction A/C, where "A" is the gross invoice price in such country of the Product when sold separately, and "C" is the gross invoice price of the Combination Product in such country;

If the Product is not sold separately in such country, but the Other Components contained in the Combination Product are sold separately in such country, then Net Sales for the Product will be calculated by multiplying actual Net Sales of such Combination Product by the result of 1 – (B/C), where "B" is the gross invoice price in such country of the Other Components contained in the

Combination Product when sold separately and "C" is the gross invoice price of the Combination Product in such country; or

If neither the Product nor the Other Components contained in the Combination Product are sold separately in such country, then Net Sales will be calculated by multiplying the actual Net Sales of such Combination Product in such country by the fraction A/(A+B) as above, but the gross invoice price in such equation will be determined by agreement of the Parties reached in good faith prior to the end of the accounting period in question based on an equitable method of determining the same that takes into account, in the applicable country, variations in dosage units and the relative fair market value of the Product and each Other Component in the Combination Product. If the Parties are unable to reach such an agreement prior to the end of the applicable accounting period, then either Party may refer such matter for resolution pursuant to Section 12.1.2 (Resolution by Executive Officers) and, if necessary, Section 12.1.3 (Resolution by Mediation).

1.121 **"New Modality"** means any therapeutic agent or approach other than [**].

1.122 "**NMPA**" means the National Medical Products Administration of the People's Republic of China (formerly the China Food and Drug Administration), and local and provincial counterparts thereto, and any and any successor entity(ies) thereto.

1.123 "**Other Component**" has the meaning set forth in Section 1.29 (Combination Product).

1.124 "**Other Enforcement Action**" has the meaning set forth in Section 7.6.4 (Other Enforcement Actions).

1.125 "**Out-of-Pocket Expenses"**, means external expenses that are specifically identifiable and incurred in the performance of activities under this Agreement, including [**]. For the avoidance of doubt, Out-of-Pocket Expenses (a) do not include any costs included in the FTE Hourly Rate and (b) will be passed through to MyoKardia without markup.

1.126 "**Part 1 Validation**" has the meaning set forth in Section 3.6.2 (Part 1 Validation).

1.127 "**Part 1 Validation Criteria**" means the criteria employed by the JSC to evaluate whether certain Selected Targets evaluated in Part 1 Validation should be advanced to Part 2 Validation, as set forth in Figure 2 of the Research Plan, or as otherwise defined by the JSC.

1.128 "**Part 1 Validation Data Package**" has the meaning set forth in Section 3.6.2 (Part 1 Validation).

1.129 "**Part 2 Validation**" has the meaning set forth in Section 3.6.3 (Part 2 Validation).

1.130 "**Part 2 Validation Summary**" has the meaning set forth in Section 3.6.3 (Part 2 Validation).

1.131 "**Party**" or "**Parties**" has the meaning set forth in the Preamble.

1.132 "**Patent Rights**" means any and all (a) patents, (b) pending patent applications, including, all provisional, non-provisional, continuations, continuations-in-part and divisional applications and all patents granted thereon, (c) all reissues, reexaminations, renewals and extensions, and (d) all U.S. and foreign counterparts of any of the foregoing.

1.133 "**Person**" means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, incorporated association, joint venture or similar entity or organization, including a government or political subdivision or department or agency of a government

1.134 "**Phase 1 Clinical Trial**" means, as to a specific Product, study in humans of such Product, designed to satisfy the requirements of 21 C.F.R. § 312.21(a), as amended from time to time, or the corresponding regulation in jurisdictions other than the United States.

1.135 "**Phase 2 Clinical Trial**" means as to a specific Product, a study in humans of such Product, designed to satisfy the requirements of 21 C.F.R. § 312.21(b), as amended from time to time, or the corresponding regulation in jurisdictions other than the United States.

1.136 "**Phase 3 Clinical Trial**" means as to a specific Product, a study in humans of such Product, designed to satisfy the requirements of 21 C.F.R. § 312.21(c), as amended from time to time, or the corresponding regulation in jurisdictions other than the United States.

1.137 "**Platform Patent Rights**" has the meaning set forth in Section 7.1.3.

1.138 "**PMDA**" means the Pharmaceuticals and Medicines Devices Agency in Japan and any successor entity thereto.

1.139 "**Potential Project Genes**" has the meaning set forth in Section 3.3 (Potential Project Genes).

1.140 "**Preclinical Milestone**" has the meaning set forth in Section 6.3.2 (Preclinical Milestones).

1.141 "**Preclinical Milestone Payments**" has the meaning set forth in Section 6.3.2 (Preclinical Milestones).

1.142 "**Preliminary Validation**" has the meaning set forth in Section 3.6.1 (Preliminary Validation).

1.143 "**Preliminary Validation Data Package**" has the meaning set forth in Section 3.6.1 (Preliminary Validation).

1.144 [**].

1.145 [**].

1.146 "**Primary Screen**" means the use of Fulcrum's small molecule probe library to perturb a designated cell type for the purposes of identifying targets that modulate the expression of the Potential Project Genes in the desired direction.

1.147 **"Primary Screen Data Package**" has the meaning set forth in Section 3.5 (Primary Screen).

1.148 "**Product**" means any Licensed Product or Disclosed Product.

1.149 "**Product Patent Right**" has the meaning set forth in Section 7.4.4 (MyoKardia's Second Right).

1.150 "**Receiving Party**" has the meaning set forth in Section 11.1 (Confidentiality).

1.151 "**Regulatory Approval**" means any and all approvals (including the approval by an applicable Governmental Authority in certain countries or territories, including [**], with respect to the price at which a pharmaceutical product is sold and can be reimbursed by healthcare insurers), licenses, registrations or authorizations (or waivers) of any national, supra-national, regional, state or local regulatory agency, department, bureau, commission, council or other governmental entity, necessary for the marketing and sale of a pharmaceutical product in a given regulatory jurisdiction.

1.152 "**Regulatory Authority**" means, with respect to a country in the Territory, any national (e.g., [**]), supra-national (e.g., [**]), regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Authority involved in the regulation of the research, development or other exploitation of any Molecule or Product or granting of Regulatory Approvals for pharmaceutical products in such country or countries.

1.153 "**Regulatory Exclusivity**" means any exclusive marketing rights or data exclusivity rights conferred by any Regulatory Authority with respect to a Product in a country or jurisdiction in the Territory, other than a Patent Right, including exclusivity for Regulatory Approval in the Territory, new chemical entity exclusivity, new clinical data exclusivity, orphan drug exclusivity, pediatric exclusivity, or rights similar thereto in other countries or regulatory jurisdictions.

1.154 "**Replacement Target**" has the meaning set forth in Section 6.3.5(a) (Replacement Target).

1.155 "**Representatives**" means with respect to each Party, its Affiliates and each of their respective officers, directors, employees, consultants, contractors, subcontractors, agents, and sublicensees.

1.156 "**Research Activities**" means the Fulcrum Research Activities and the MyoKardia Research Activities, collectively, performed during the Research Term.

1.157 "**Research Budget**" means [**], with reasonable, documented overages capped at [**], up to a maximum of [**], as such amount may be increased in connection with any amendment to the Research Plan approved by the JSC.

1.158 "**Research Data**" has the meaning set forth in Section 5.5.1(a) (Data).

1.159 "**Research Plan**" has the meaning set forth in Section 3.1 (Research Plan).

1.160 "**Research Term**" means the period commencing on the Effective Date and continuing until [**].

1.161 [**].

1.162 "**Royalty Rates**" has the meaning set forth in Section 6.4.1 (Royalty Rate).

1.163 "**Royalty Term**" means, with respect to a Product in a country, the period commencing on the first sale for use or consumption by an end user of such Product in such country and ending upon the latest of: (a) the expiration of the last Valid Claim of the last Fulcrum Patent Right that the sale of such Product in such country would infringe; (b) the expiration of any applicable Regulatory Exclusivity in such country with respect to such Product; or (c) [**] years from the date of the First Commercial Sale of such Product in such country.

1.164 "**Sales Milestone**" has the meaning set forth in Section 6.3.4 (Cumulative Sales Milestones).

1.165 "**Sales Milestone Payments**" has the meaning set forth in Section 6.3.4 (Cumulative Sales Milestones).

1.166 "**Screen Preparation**" has the meaning set forth in Section 3.4 (Cell Line Scale Up and Screen Preparation).

1.167 "**Screen Preparation Data Package**" has the meaning set forth in Section 3.4 (Cell Line Scale Up and Screen Preparation).

1.168 "**Screened Target**" means, with respect to a Potential Project Gene, any biological target identified through the Primary Screen that modulates such Potential Project Gene.

1.169 "**Second Designation Period**" has the meaning set forth in Section 3.6.6 (Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets).

1.170 "**Secondary Cardiomyopathy Milestone Target**" means (a) an Additional Cardiomyopathy Target or (b) a Disclosed Target that modulates the expression of an Additional Project Gene.

1.171 "**Selected Target**" has the meaning set forth in Section 3.6.2 (Part 1 Validation).

1.172 "**Selling Party**" has the meaning set forth in Section 1.120 (Net Sales).

1.173 "**siRNA Confirmation**" means the use of siRNA technology to confirm an active hit from the Primary Screen by genetic manipulation, as conducted in Part 1 Validation activities.

1.174 "**Sublicensee**" means a Third Party to which MyoKardia (or any of its Affiliates) grants a sublicense under MyoKardia's rights under Section 5.1 (License Grant to MyoKardia);

provided that "Sublicensee" does not include any distributors of MyoKardia or its Affiliates that purchase Products from MyoKardia or its Affiliates in an arm's length transaction and who have no other obligation, including a reporting obligation, to MyoKardia or its Affiliates, with respect to any subsequent use or disposition of such Products.

1.175 "**Target**" means any Cardiomyopathy Target or Disclosed Target.

1.176 "**Target Know-How**" has the meaning set forth in Section 7.1.1.

1.177 "**Target Patent Rights**" has the meaning set forth in Section 7.1.1.

1.178 "**Term**" has the meaning set forth in Section 10.1 (Term).

1.179 "**Territory**" means worldwide.

1.180 "**Third Party**" means any Person other than MyoKardia, Fulcrum or their respective Affiliates.

1.181 "**Threshold Criteria**" means the criteria for Screened Targets to advance to Preliminary Validation as defined by the JSC.

1.182 "**United States**" or "**U.S.**" means the United States of America and all of its districts, territories and possessions.

1.183 "**Valid Claim**" means (a) an issued claim of any Patent Right that has not been permanently revoked, nor held unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction that is unappealable or unappealed in the time allowed for appeal, and which has not been cancelled, withdrawn or abandoned or admitted to be invalid or unenforceable through reissue, disclaimer or otherwise or (b) a pending claim of a patent application or series of related patent applications that has been pending, in the same or substantially the same claims, less than [**] from the date of filing of the earliest patent application from which such patent application claims priority, which claim has not been cancelled, withdrawn or abandoned, or finally rejected by an administrative agency action from which no appeal can be taken.

1.184 "**Withholding Taxes**" means any and all income or other taxes, withholdings or other deductions required by Applicable Law to be withheld or deducted from any of the payments made under this Agreement.

Article 2
COLLABORATION MANAGEMENT

2.1. **Joint Steering Committee**. Within [**] after the Effective Date, the Parties will establish a Joint Steering Committee (the "**JSC**") to coordinate the Parties' activities under the Research Plan, as further set forth in this Article 2 (Collaboration Management).

2.2. **Composition of the JSC**. The Research Plan will be conducted under the direction of the JSC, which will consist of up to [**] representatives of Fulcrum and up to [**]

representatives of MyoKardia. The JSC may change its size from time to time by mutual consent of the Parties, *provided* that the JSC will consist at all times of an equal number of representatives of each of Fulcrum and MyoKardia. Each Party will appoint its respective representatives to the JSC from time to time, and may substitute one or more of its representatives, in its sole discretion, effective upon notice to the other Party of such change. Each Party will have at least one (1) JSC representative who is a senior employee, and all JSC representatives will be employees of the relevant Party and have appropriate expertise and ongoing familiarity with the Research Plan. Additional non-voting representatives (including an alliance manager from each Party) or consultants may from time to time, by decision of the JSC, be invited to attend JSC meetings, subject to all representatives (including the designated voting representatives) and consultants entering into binding, written confidentiality obligations and assignment of inventions (which may have been executed prior to the Effective Date) that are consistent with the requirements on the Parties under Article 11 (Confidentiality) and Section 7.2 (regarding assignment of inventions). Each Party will bear its own expenses relating to attendance at JSC meetings by its representatives.

2.3. **JSC Chairperson**. The JSC chairperson will be an employee of MyoKardia.

2.4. **JSC Responsibilities**. The JSC will have the following responsibilities with respect to the Research Plan:

2.4.1. acting as liaison between the Parties to ensure open and regular communication channels, and more particularly to ensure the Parties are informed of, and have a forum to discuss, the ongoing progress of the Research Plan during the Research Term;

2.4.2. coordinating and monitoring activities under the Research Plan;

2.4.3. reviewing and approving any amendments to the Research Plan, including (a) any revision or expansion of work under any amendment to the Research Plan, (b) the estimated timelines, as applicable, for the conduct of particular activities under the Research Plan, (c) the responsibilities of each Party under the Research Plan, and (d) the budget applicable to any revised or expanded work under any amendment to the Research Plan;

2.4.4. reviewing reports and updates provided by Parties regarding the progress of the Research Plan;

2.4.5. reviewing the Screen Preparation Data Package and aligning on readouts (including positive and negative controls) and deciding at what point to advance to performance of the Primary Screen;

2.4.6. (a) reviewing the data in the Primary Screen Data Package, (b) requesting and reviewing annotated expression data for certain Screened Targets identified from the Primary Screen that did not meet the Threshold Criteria, where the JSC determines that consideration of the biology and relevant pathways justifies such a request (e.g., potential positive and negative controls), (c) determining the Threshold Criteria for Screened Targets to advance to Preliminary Validation, and (d) determining the number of and which

compounds or Screened Targets should advance to Preliminary Validation following the completion of the Primary Screen of the Potential Project Genes under the Research Plan;

2.4.7. (a) reviewing the data in Preliminary Validation Data Package, (b) selecting up to [**] Screened Targets (or more, if mutually agreed by the Parties) to advance to Part 1 Validation, and (c) determining the Part 1 Validation Criteria for Screened Targets;

2.4.8. selecting one (1) patient-derived cell line to be used in conjunction with Part 1 Validation under the Research Plan;

2.4.9. (a) selecting CRISPR Confirmation or siRNA Confirmation as the optimal method of genetic validation to be utilized in Part 1 Validation by Fulcrum and (b) identifying any confirmation of hit selectivity by characterization of downstream genes and any additional cytotoxicity assays to be performed by Fulcrum as part of Part 1 Validation;

2.4.10. reviewing the data from the Part 1 Validation Data Package and identifying which of the up to [**] Selected Targets across up to [**] Potential Project Genes meet the Part 1 Validation Criteria set forth in Figure 2 of the Research Plan;

2.4.11. reviewing the data from the Part 2 Validation Summary;

2.4.12. reviewing the data from the Final Data Package;

2.4.13. maintaining a list identifying the Lead Cardiomyopathy Target, Follow-On Cardiomyopathy Targets, Additional Cardiomyopathy Targets, Disclosed Targets, Replacement Targets, Potential Project Genes, the Lead Project Gene and Additional Project Genes and providing such list to the Parties upon dissolution of the JSC;

2.4.14. approving any of Fulcrum's Third Party subcontractors that may perform any responsibilities under the Research Plan;

2.4.15. extending the term of the JSC until the first IND submission for a Collaboration Molecule by MyoKardia;

2.4.16. attempting to resolve matters on an informal basis; and

2.4.17. performing such other activities as the Parties agree in writing will be the responsibility of the JSC.

The JSC will only have the roles and responsibilities assigned to it in this Section 2.4 (JSC Responsibilities). For avoidance of doubt, the JSC will not have the authority to modify or waive the terms of this Agreement or to amend, modify or limit the final decision-making authority of MyoKardia as set forth in this Agreement. For clarity, any amendment of the Research Plan shall be effective only if agreed in writing and signed on behalf of each Party.

2.5. **Meetings**. The first JSC meeting will be held within [**] after the Effective Date, and the JSC will thereafter meet in accordance with a schedule established by mutual written agreement of the Parties, but no less frequently than [**]. The JSC may meet by teleconference,

videoconference, or in person, as determined from time to time by the JSC. The JSC chairperson will (a) set agendas for meetings with solicited input from other JSC representatives; (b) coordinate with the JSC secretary the delivery of draft minutes to the JSC for review and adoption; and (c) conduct meetings, including ensuring that objectives for each meeting are set and achieved. The JSC chairperson will have no greater authority on the JSC than any other representative of the JSC. The JSC will appoint a secretary for each meeting, who can be either a JSC representative or a non-voting representative or consultant invited to attend in accordance with Section 2.2 (Composition of the JSC). The JSC secretary will prepare minutes of the meeting, which will provide a description in reasonable detail of the discussions held at the meeting and a list of any actions, decisions or determinations approved by the JSC. The JSC secretary will circulate draft minutes promptly after each meeting. The JSC secretary will solicit and, with the cooperation of the Parties, ensure that any reasonable comments of a member of the JSC are incorporated into such minutes before being adopted by the JSC as final. Minutes of a meeting of the JSC will be adopted by the JSC no later than the next meeting of the JSC. The JSC secretary will have no greater authority on the JSC than any other representative of the JSC.

2.6. **Appointment of Subcommittees, Project Teams and Collaboration Managers**. The JSC will be empowered to create such subcommittees and project teams as it may deem appropriate or necessary. Each such subcommittee and project team will report to the JSC, which will have authority to approve or reject recommendations or actions proposed thereby subject to the terms of this Agreement. The provisions of Sections 2.2 (Composition of the JSC) and 2.5 (Meetings) will apply to each subcommittee, *mutatis mutandis*, unless otherwise determined by the JSC.

2.7. **JSC Decision-Making**.

2.7.1. For each meeting of the JSC, attendance by at least half of the representatives of each Party will constitute a quorum. All decisions of the JSC must be made by unanimous vote of the JSC, with the representatives of each Party collectively having one (1) vote on behalf of such Party. In the event that the JSC does not unanimously approve any action, the JSC shall take no action on the matter at issue and will use good faith efforts to reach consensus pursuant to Section 2.7.2.

2.7.2. The JSC shall use good faith efforts to reach consensus on matters within its decision-making authority. If the JSC is unable to reach a consensus with respect to any such matter for a period in excess of [**], then such matter will be submitted to the Executive Officers of MyoKardia and Fulcrum for resolution.

2.7.3. If the Executive Officers of MyoKardia and Fulcrum are unable to resolve such matter within [**] after escalation to the Executive Officers, then the Executive Officer of MyoKardia will have the deciding vote over the resolution of such matter.

2.7.4. Notwithstanding the foregoing, MyoKardia may not exercise its final decision-making authority under Section 2.7 (JSC Decision-Making) (a) to amend the Research Plan and the budget included therein in a manner that would (i) expand the scope or increase the cost of the Fulcrum Research Activities, [**], or (ii) cause delays of greater than [**] in the performance of the Research Activities, (b) to require Fulcrum to take or

decline to take any action that could reasonably be expected to result in a violation of any Applicable Law, any agreement with any Third Party or the infringement of intellectual property rights of Third Parties, (c) in a manner that excuses MyoKardia from any of its obligations specifically enumerated under this Agreement or otherwise agreed in writing by the Parties, (d) to expand or narrow the responsibilities of the JSC, (e) to amend, modify or waive any term of this Agreement, (f) to determine whether a Milestone has been achieved or (g) to determine whether a Party has breached or is in breach of this Agreement.

2.8. **Dissolution of JSC**. The JSC will be dissolved upon the expiration of the Research Term or the earlier termination of this Agreement, unless extended as set forth in Section 2.4.15.

Article 3
RESEARCH ACTIVITIES

3.1. **Research Plan**. Subject to the terms and conditions of this Agreement, Fulcrum and MyoKardia agree to collaborate to discover novel genetic modifiers of underlying drivers of genetic cardiomyopathy. The research plan for this collaboration is set forth in Schedule 3.1 (the "**Research Plan**"). All of the Research Activities to be conducted by or on behalf of the Parties under this Agreement during the Research Term are set forth at a high level in the Research Plan. Subject to Section 2.7 (JSC Decision-Making), the Research Plan may be amended at any time during the Research Term by the JSC. Each Party will use Commercially Reasonable Efforts to conduct the Research Activities allocated to it in the Research Plan in a professional and timely manner and in compliance with all Applicable Laws. Upon expiration of the Research Term, the Parties will have no further obligation to conduct any Research Activities, including the screening and target validation activities set forth in Section 3.6 (Target Validation and Selection of Cardiomyopathy Target(s)).

3.2. **Progress Reports**. During the Research Term, each Party will furnish to the JSC, no later than [**] before each scheduled meeting of the JSC, an update on such Party's progress under the Research Plan with respect to the performance of such Party's Research Activities, including a high level summary of any results, data and other Know-How generated by such Party under the Research Plan. With respect to any such progress updates to the JSC, [**].

3.3. **Potential Project Genes**. MyoKardia will designate [**] different genes, which have been identified by MyoKardia as genes previously implicated in the scientific and medical literature as underlying drivers for certain genetically defined cardiomyopathies (the "**Potential Project Genes**"), within the first [**] following the Effective Date, for purposes of this Agreement.

3.4. **Cell Line Scale Up and Screen Preparation**. Fulcrum will (a) scale up and differentiate one (1) Engineered Cell Line in an amount sufficient to conduct the Primary Screen and (b) undertake other preparation activities necessary to define a robust and reproducible set of experimental conditions to enable a more efficient high-throughput Primary Screen, as set forth in more detail in the Research Plan (collectively ((a) and (b)), the "**Screen Preparation**"). Fulcrum will furnish a data package to the JSC that will contain the pilot data, assay performance metrics and other data from the Screen Preparation ("**Screen Preparation Data Package**"). The JSC, in accordance with Article 2 (Collaboration Management), will review the Screen Preparation Data

Package and align on readouts (including positive/negative controls) and decide at what point to advance to Fulcrum's performance of the Primary Screen.

3.5. **Primary Screen**. Fulcrum will conduct the Primary Screen on each Potential Project Gene for the purpose of identifying one or more Screened Targets that modulate such Potential Project Gene. Within [**] after completion of such Primary Screen, Fulcrum will furnish a data package to the JSC that will contain the information described in paragraph 4(d) of the Research Plan for [**] Potential Project Genes, including the [**] (the "**Primary Screen Data Package**") [**]. The JSC, in accordance with Article 2 (Collaboration Management), will review the Primary Screen Data Package. The JSC also may request, and Fulcrum will provide [**].

3.6. **Target Validation and Selection of Cardiomyopathy Target(s)**.

3.6.1. **Preliminary Validation**. From review of the Primary Screen Data Package and other data provided pursuant to Section 3.5 (Primary Screen), the JSC will select approximately [**] (or such number as determined by the JSC) compounds to advance to CRC Screen and Cell Toxicity Screen ("**Preliminary Validation**"). Fulcrum will conduct Preliminary Validation for all such compounds and Screened Targets for each Potential Project Gene. Within [**] after completion of Preliminary Validation, Fulcrum will deliver a report to the JSC containing all data resulting from the Preliminary Validation for all Screened Targets for all Potential Project Genes ("**Preliminary Validation Data Package**").

3.6.2. **Part 1 Validation.** The Parties, through the JSC and in accordance with Article 2 (Collaboration Management), will review the data in the Preliminary Validation Data Package to select up to [**] (or more, if mutually agreed by the Parties) such Screened Targets (each, a "**Selected Target**") from which the JSC shall determine that all or a subset shall advance to Part 1 Validation. Promptly following designation of the Selected Targets, Fulcrum will (a) provide the JSC with the identity of all Selected Targets, (b) notify the JSC as to whether any Screened Targets that were considered for advancement to Part 1 Validation are Excluded Targets (which, in such case, pursuant to Section 3.6.8 (Excluded Target) such Screened Targets shall not be selected by the JSC as a Selected Target) and [**]. Following designation of the Selected Targets and the JSC decision as to which Selected Targets to advance forward, each Party will initiate and conduct the Part 1 Validation activities described in paragraph 5.2 and Figure 2 of the Research Plan that are assigned to such Party for each Selected Target for each Potential Project Gene, which for Fulcrum will include [**] ("**Part 1 Validation**"). For MyoKardia, Part 1 Validation activities include [**]. Within [**] after a Party's completion of its Part 1 Validation activities, such Party will deliver a report containing all data resulting from such Part 1 Validation for all Selected Targets and for all Potential Project Genes to the JSC (each, a "**Part 1 Validation Data Package**").

3.6.3. **Part 2 Validation**. The Parties, through the JSC and in accordance with Article 2 (Collaboration Management) will review the data from the Part 1 Validation Data Package and identify which of the [**] Selected Targets across up to [**] Potential Project Genes meet the Part 1 Validation Criteria set forth in Figure 2 of the Research Plan (such targets, "**Cardiomyopathy Target Candidates**"). MyoKardia will select which

Cardiomyopathy Target Candidates to advance to the validation activities identified in paragraph 5.3 and Figure 2 of the Research Plan ("**Part 2 Validation**") and [**]. On a Cardiomyopathy Target Candidate-by-Cardiomyopathy Target Candidate basis, [**] from Part 2 Validation for such Cardiomyopathy Target Candidate to the JSC for review and discussion ("**Part 2 Validation Summary**").

3.6.4. **Final Data Package**. Upon completion of the Research Plan, the Parties shall work together to produce a final data package that will contain (a) all data generated in the performance of Fulcrum Research Activities, (b) all data generated in the performance of MyoKardia Research Activities [**] and (c) such other information identified in the Research Plan as to be included in such final data package (to the extent not provided in a previous Data Package), to provide to the JSC and allow MyoKardia to fully consider whether any of the Cardiomyopathy Target Candidates should be designated a Cardiomyopathy Target; *provided* that, Fulcrum will not be required to undertake any additional research activities to produce such other information not set forth in the Research Plan, and, to the extent, any such additional research activities would be required, Fulcrum will not be required to deliver such other information (the "**Final Data Package**"). In the event that the Final Data Package does not contain all such information and either Party notifies the other Party thereof, such Party will promptly provide such missing items and, notwithstanding any earlier delivery by such Parties, the delivery date for the Final Data Package will be deemed for all purposes under this Agreement to be the date upon which the Parties have delivered a complete Final Data Package including all such items, as memorialized by the Parties in writing, which date will, if the Parties have not otherwise memorialized such delivery, be deemed to occur on [**] following the last date on which either Party has provided any missing items to the other Party and neither Party has notified the other of any additional missing items (the "**Final Data Package Delivery Date**"). Fulcrum will provide reasonable assistance to MyoKardia in interpreting all data in the Final Data Package. Promptly following the Final Data Package Delivery Date, Fulcrum will destroy MyoKardia's materials provided under this Agreement, including any Engineered Cell Line, and MyoKardia's Confidential Information, except that one copy of such Confidential Information can be kept for archival and legal purposes.

3.6.5. **Selection of Lead Cardiomyopathy Target.** MyoKardia will have the sole discretion to designate one (1) Cardiomyopathy Target Candidate as the Lead Cardiomyopathy Target by written notice ("**Lead Cardiomyopathy Target Notice**") delivered to Fulcrum no later than the date that is [**] (such period, the "**First Designation Period**"). In the Lead Cardiomyopathy Target Notice, MyoKardia will also identify the (a) Potential Project Gene for which expression is modulated by the Lead Cardiomyopathy Target (the "**Lead Project Gene**") and (b) the genetically defined cardiomyopathies scientifically shown to be related to the Lead Project Gene (the "**Lead Project Gene Exclusive Indications**").

3.6.6. **Selection of Follow-On Cardiomyopathy Targets and Additional Cardiomyopathy Targets.** MyoKardia may, in its sole discretion, designate (a) [**] Follow-On Cardiomyopathy Target(s) or (b) [**] Additional Cardiomyopathy Target(s) by written notice(s) delivered to Fulcrum no later than the date that is [**] following the Final Data Package Delivery Date ("**Second Designation Period**"). Such notice will identify

whether such Cardiomyopathy Target is a Follow-On Cardiomyopathy Target or an Additional Cardiomyopathy Target and, with respect to an Additional Cardiomyopathy Target, the (a) Potential Project Gene for which expression is modulated by such Additional Cardiomyopathy Target ("**Additional Project Gene**") and (b) the genetically defined cardiomyopathies scientifically shown to be related to the Additional Project Gene (the "**Additional Project Gene Exclusive Indications**").

3.6.7. **Medicinal Chemistry**.

(a) *By MyoKardia*. [**].

(b) *By Fulcrum*. In the event that MyoKardia desires Fulcrum to provide medicinal chemistry services and Fulcrum is willing to provide such services, then the Parties will thereafter negotiate in good faith to determine the activities, timelines, budgets, deliverables (including technology transfer, as appropriate) and other specifications of any Medicinal Chemistry Services to be performed by Fulcrum, and such matters would be set forth in a separate research plan.

3.6.8. **Excluded Target**. The JSC may not select any Excluded Target as a target or compound to undergo Primary Screen or as a Selected Target or Cardiomyopathy Target Candidate, and MyoKardia may not select an Excluded Target as a Cardiomyopathy Target. For clarity, (a) Fulcrum will not be required to provide any information regarding any Excluded Targets to MyoKardia, conduct target validation activities on such Excluded Target or to include any information with respect to such Excluded Target in any Data Package and (b) MyoKardia will have no payment obligations to Fulcrum with respect to any Excluded Targets. For further clarity, (i) if such target qualifies as an Excluded Target solely pursuant to Section 1.62(a)(ii) (Excluded Target), Fulcrum may notify MyoKardia in writing of Fulcrum's election, in its sole discretion, not to exclude such target from this Agreement, or (ii) if such target qualifies as an Excluded Target solely pursuant to Section 1.62(a)(i) (Excluded Target) and the applicable license, option or other grant of rights is non-exclusive, Fulcrum shall provide written notice of the nature of such license, option or grant of rights (subject to obligations of confidentiality to any Third Party) and, upon MyoKardia's written notice to Fulcrum, such target will no longer constitute an Excluded Target, and if MyoKardia designates such target as a Cardiomyopathy Target, the scope of the grant of rights with respect to such target to MyoKardia under this Agreement will be reduced solely to the extent necessary to comply with the applicable license, option or other grant of rights.

3.7. **Subcontracting**. Either Party may subcontract any of its responsibilities under the Research Plan; *provided* that (i) any subcontractor of a Party will have the requisite expertise to conduct the relevant subcontracted responsibilities, (ii) in the case of subcontracting by Fulcrum to a Third Party, such Third Party is approved by the JSC prior to the engagement of such Third Party subcontractor, and (iii) any agreement between a Party or its Affiliate and a permitted subcontractor pertaining to the Research Activities will be consistent with the provisions of this Agreement, including (A) an obligation to assign or irrevocably exclusively license, worldwide, in all fields, all intellectual property developed in the conduct of the relevant Research Activities

to the subcontracting Party, including assignments by any employee, contractor or agent of such subcontractor (other than intellectual property solely related to improvements to any such subcontractor's background technology, which intellectual property may be non-exclusively licensed to the subcontracting Party), and (B) terms and conditions under which such subcontractor of a Party is obligated to preserve the confidentiality of any Confidential Information of the other Party received by such subcontractor that are at least as restrictive as those described in Article 11 (Confidentiality). The engagement by a Party of any subcontractor under this Section 3.7 (Subcontracting) will not relieve such Party of its obligations under this Agreement, including the Research Plan, and such Party will remain responsible for all acts or omissions by such subcontractor.

3.8. **Research Funding**. Fulcrum will invoice MyoKardia within [**] after the end of each Calendar Quarter for the number of hours that Fulcrum FTEs spent performing the Fulcrum Research Activities in accordance with the Research Plan at the FTE Hourly Rate and all reasonable Out-of-Pocket Expenses incurred by Fulcrum in performing the Fulcrum Research Activities in accordance with the Research Plan (collectively, the "**Covered Research Expenses**"). Such invoices will be reasonably detailed to include: (a) aggregate quarterly FTE hours; (b) the total amount due for FTEs; and (c) reasonably detailed descriptions of Out-of-Pocket Expenses. MyoKardia will pay Two Million Five Hundred Thousand Dollars ($2,500,000) as pre-paid research funding pursuant to Section 6.2 (Prepaid Research Funding). Once the Covered Research Expenses exceed such $2,500,000 threshold in aggregate costs, as evidenced by the cumulative sum of all invoices provided by Fulcrum to MyoKardia, MyoKardia will thereafter reimburse Fulcrum on a Calendar Quarter basis (including the Calendar Quarter in which the Covered Research Expenses exceeded such $2,500,000 threshold) for the Covered Research Expenses incurred by Fulcrum in such Calendar Quarter, up to a maximum of the Research Budget. MyoKardia will pay all undisputed amounts set forth in such invoices within [**] after MyoKardia's receipt thereof. MyoKardia will be responsible for any costs it incurs in the performance of the MyoKardia Research Activities.

3.9. **Records**. Each Party will maintain, or cause to be maintained, records of its activities under the Research Plan in sufficient detail and in good scientific manner appropriate for scientific, patent and regulatory purposes, which will properly reflect all work included in the Research Activities conducted under the Research Plan consistent with its internal procedures and policies. Further, Fulcrum shall maintain records related to the invoices submitted to MyoKardia pursuant to Section 3.8 (Research Funding). The Parties will retain such records for at least [**] after the expiration or termination of this Agreement in its entirety or for such longer period as may be required by Applicable Law. During the Term, MyoKardia will have the right, during normal business hours and upon reasonable notice and at its own expense, to inspect and copy such of Fulcrum's records not more than [**] in any Calendar Year.

3.10. **Use of MyoKardia Materials and Molecules**. For the avoidance of doubt, nothing in this Agreement shall require Fulcrum to use any of MyoKardia's materials or molecules, including in the conduct of the Research Activities [**].

Article 4
RESEARCH, DEVELOPMENT, MANUFACTURING, AND COMMERCIALIZATION OF PRODUCTS

4.1. **General**. Subject to the terms of this Agreement (including Section 4.2 (Diligence Requirements)), MyoKardia will have sole and exclusive control over whether to pursue the research, development, Regulatory Approval, manufacturing, commercialization, or other exploitation of any Molecule or Product and all matters relating thereto, itself or through one or more Affiliates or Third Parties selected by MyoKardia in its sole discretion.

4.2. **Diligence Requirements**. During the Term and following MyoKardia's designation of the Lead Cardiomyopathy Target, MyoKardia will use Commercially Reasonable Efforts to advance [**] Product to Regulatory Approval [**]. After receiving Regulatory Approval for a Product [**], MyoKardia will use Commercially Reasonable Efforts to commercialize such Product in [**]. After receiving Regulatory Approval for a Product [**], MyoKardia will use Commercially Reasonable Efforts to commercialize such Product [**]. Subject to Section 3.7 (Subcontracting) with respect to MyoKardia's responsibilities under the Research Plan, MyoKardia may satisfy its obligations under this Section 4.2 (Diligence Requirements) itself or through one or more Affiliates or Third Parties selected by MyoKardia in its sole discretion.

4.3. **Reporting**. Within [**] after the Effective Date, Fulcrum shall submit to MyoKardia a high-level report setting forth Fulcrum's then current estimate of (a) the aggregate quarterly FTE hours, (b) the total amount due for FTEs, and (c) the total Out-of-Pocket Expenses Fulcrum reasonably anticipates incurring during the conduct of the Fulcrum Research Activities (the "**Anticipated Research Budget**"). Following the receipt of such report, Fulcrum and MyoKardia shall reasonably cooperate to resolve any questions pertaining to the Anticipated Research Budget. Fulcrum shall promptly notify MyoKardia in writing of any material changes to the Anticipated Research Budget and provide an updated report reflecting any such material changes to the Anticipated Research Budget. Following the end of the Research Term and, on a Target-by-Target basis, until the First Commercial Sale of a Product for any Target, MyoKardia shall provide a written report to Fulcrum within [**] after the end of each Calendar Year during the Term that summarizes MyoKardia's exercise of efforts with respect to advancing Products to Regulatory Approval in accordance with Section 4.2 (Diligence Requirements) under this Agreement, including matters relating to seeking Regulatory Approval therefor. Notwithstanding anything to the contrary in this Agreement (including Section 4.2 (Diligence Requirements)), MyoKardia will have no obligation to negotiate agreements to obtain additional rights to satisfy its obligations in Section 4.2 (Diligence Requirements). MyoKardia shall also provide, in such annual reports, a summary of its anticipated activities with respect to the advancing such Products to Regulatory Approval for the following Calendar Year.

4.4. **Applicable Laws**. MyoKardia will, and will require its Affiliates and Licensees to, comply with all Applicable Laws in its and their research, development, manufacture and commercialization of Products.

Article 5
LICENSE GRANTS AND EXCLUSIVITY

5.1. **License Grant to MyoKardia**. Subject to the terms of this Agreement, during the Term, Fulcrum hereby grants, on behalf of itself and its Affiliates, to MyoKardia and its Affiliates an exclusive (even as to Fulcrum, except to the extent necessary for Fulcrum to perform the Fulcrum Research Activities), worldwide, royalty-bearing, sublicensable (through multiple tiers, in accordance with Section 5.2 (Sublicenses)) license under the Fulcrum Technology to research, develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, export, have exported, distribute, have distributed, market, have marketed, promote, have promoted, or otherwise exploit Molecules and Products in the Field in the Territory.

5.2. **Sublicenses**. Subject to the terms of this Agreement, MyoKardia may grant sublicenses of any rights granted to MyoKardia under Section 5.1 (License Grant to MyoKardia) through multiple tiers of sublicenses to one or more Sublicensees. Each such sublicense will be consistent with the terms of this Agreement. MyoKardia will provide a copy of each sublicense agreement to Fulcrum (which agreement may be redacted to remove confidential information not necessary for Fulcrum to ensure compliance with this Agreement) within [**] after the execution of each such sublicense. MyoKardia will remain responsible for each Sublicensee's compliance with the applicable terms of this Agreement and, notwithstanding any sublicense, MyoKardia will remain primarily liable for all of MyoKardia's duties and obligations contained in this Agreement.

5.3. **License Grant to Fulcrum**. Subject to the terms of this Agreement, MyoKardia hereby grants, on behalf of itself and its Affiliates, to Fulcrum and its Affiliates a non-exclusive, non-sublicensable (except to permitted subcontractors, in accordance with Section 3.7 (Subcontracting)) license under the MyoKardia Technology solely to perform the Fulcrum Research Activities during the Research Term.

5.4. **No Implied Licenses; Retained Rights**. Except as expressly provided in this Agreement, no Party will be deemed by estoppel or implication to have granted the other Party any licenses or other right with respect to any intellectual property.

5.5. **Exclusivity**.

5.5.1. **Exclusivity Obligations**.

(a) *Data*. During the Research Term, Fulcrum (i) will use, and will cause its Affiliates and permitted subcontractors to use, any data generated in performance of any Research Activities (including any data in any Data Package) ("**Research Data**") solely to perform the Fulcrum Research Activities in accordance with this Agreement and (ii) will only transfer, make available, deliver or disclose Research Data to such Affiliates and permitted subcontractors performing such Fulcrum Research Activities. If MyoKardia designates a Lead Cardiomyopathy Target, then for [**] after the end of the Research Term ("**Exclusivity Period**"), Fulcrum (A) will not use (and will cause its Affiliates and permitted subcontractors to not use) any Research Data for any reason and (B) will not transfer, make available, deliver or disclose Research Data to any Third Party

for any reason. For clarity, after the Exclusivity Period, Fulcrum may use any Research Data (except for [**]) and may transfer, make available, deliver or disclose Research Data (except [**] to any Third Party for any reason.

(b) *Cardiomyopathy Target Candidates*. During the Research Term following determination of the Cardiomyopathy Target Candidates, and for [**] after the end of the Research Term, Fulcrum will not (and, subject to Section 5.5.2 (Exception for Change of Control) and Section 5.5.3 (Exception for Affiliate Acquisition), will cause its Affiliates not to) work, independently or for or with any Third Party (including via a license, assignment, transfer or other grant of rights to such Third Party), to research, develop, manufacture, commercialize, use, or otherwise exploit any compound or product (i) that is a Molecule or Product directed against the Cardiomyopathy Target Candidates (in whole or in part and in modified or unmodified form) for the treatment, prophylaxis or diagnosis of any indication or (ii) for the treatment of any Lead Project Gene Exclusive Indications or Additional Project Gene Exclusive Indications (any such compound or product in (i) or (ii), a "**Competing Product**"); *provided*, *however*, that any compounds or products that are directed against any Excluded Target(s) will not be considered within the definition of "**Competing Product**", and Fulcrum will not be restricted by this Section 5.5.1 (Exclusivity Obligations) with respect to any such product.

5.5.2. **Exception for Change of Control**. Fulcrum will not be in breach of the restrictions set forth in Sections 5.5.1 (Exclusivity Obligation) if Fulcrum undergoes a Change of Control with an Acquiring Party that is, independently on its own behalf, on the behalf of any Third Party or with any Third Party (including via a license, assignment, transfer or other grant of rights to such Third Party), researching, developing, manufacturing, commercializing, using, or otherwise exploiting any Competing Product immediately prior to the consummation of such Change of Control and continues such exploitation of any Competing Product following the consummation of such Change of Control, as applicable; *provided* that (a) Fulcrum promptly notifies MyoKardia of such Change of Control and all Competing Products, (b) no Fulcrum Technology or Fulcrum Confidential Information is used by or on behalf of such Acquiring Party in connection with any subsequent performance of any such activities with respect to any such Competing Products following the consummation of such Change of Control, and (c) such Acquiring Party institutes commercially reasonable technical and administrative safeguards to ensure the requirements set forth in the foregoing clause (b) are met, including by creating "firewalls" between the personnel working on such Competing Products and the personnel teams charged with working on any Molecule or Product (or component thereof) or having access to data from activities performed under this Agreement or Confidential Information of the Parties; *provided* that personnel of such Acquiring Party that are responsible for financial functions and alliance management may, solely for such purposes, have access to information concerning Molecules and Products solely as necessary to perform such functions.

5.5.3. **Exception for Affiliate Acquisition**. Fulcrum will not be in breach of the restrictions set forth in Sections 5.5.1 (Exclusivity Obligation) if Fulcrum acquires a Third Party (whether such acquisition occurs by way of a purchase of assets, merger,

consolidation, change of control or otherwise) (an "**Affiliate Acquisition**") that is, independently on its own behalf, on the behalf of any Third Party or with any Third Party (including via a license, assignment, transfer or other grant of rights to such Third Party), researching, developing, manufacturing, commercializing, using, or otherwise exploiting any Competing Product, immediately prior to the consummation of such Affiliate Acquisition, as applicable, and continues such exploitation of any Competing Product following the consummation of such Affiliate Acquisition, as applicable; *provided* that (a) Fulcrum promptly notifies MyoKardia of such Affiliate Acquisition and all Competing Products, (b) within [**] after the effective date of such Affiliate Acquisition, Fulcrum will either (i) request that any Competing Product be included in this Agreement as a Molecule or Product, as applicable, on terms to be negotiated by the Parties; *provided* that if the Parties are unable to agree on the terms on which to include any Competing Product in this Agreement within [**] after the effective date of such Affiliate Acquisition, Fulcrum and its Affiliates will take the action specified in either the following clause (ii) or (iii), (ii) notify MyoKardia that the Acquired Party will fully divest its rights in and to such Competing Product, in which case, Fulcrum and the Acquired Party will fully divest their rights in and to any Competing Product within [**] after the effective date of such Affiliate Acquisition, or (iii) notify MyoKardia that Fulcrum and the Acquired Party are ceasing all research, development, manufacture and commercialization activities with respect to any Competing Product, in which case, within [**], after MyoKardia's receipt of such notice, Fulcrum and its Affiliates will cease all such activities, (c) no Fulcrum Technology or Fulcrum Confidential Information is used by or on behalf of such Acquired Party in connection with any subsequent performance of any such activities with respect to any such Competing Products following the consummation of such Affiliate Acquisition, and (d) Fulcrum institutes commercially reasonable technical and administrative safeguards to ensure the requirements set forth in the foregoing clause (c) are met, including by creating "firewalls" between the personnel working on such Competing Products and the personnel teams charged with working on any Molecule or Product (or component thereof) or having access to data from activities performed under this Agreement or Confidential Information of the Parties.

5.5.4. **Exception for Existing Agreements**. Fulcrum will not be in breach of the restrictions set forth in Section 5.5.1(b)(ii) (Exclusivity Obligation) as a result of the exercise by any Third Party of any rights granted by Fulcrum or its Affiliates to such Third Party pursuant to any agreement entered into between Fulcrum or its Affiliates and such Third Party [**]. Fulcrum will not be in breach of the restrictions set forth in Section 5.5.1(b)(i) (Exclusivity Obligation) as a result of the exercise by any Third Party of any rights granted by Fulcrum or its Affiliates to such Third Party pursuant to any agreement entered into between Fulcrum or its Affiliates and such Third Party [**].

Article 6
FINANCIAL PROVISIONS

6.1. **Upfront Payment**. MyoKardia will pay Fulcrum a one-time, non-refundable, non-creditable upfront payment of Ten Million Dollars ($10,000,000) within [**] after receipt by MyoKardia of a corresponding invoice from Fulcrum, which shall be issued no earlier than the Effective Date.

6.2. **Prepaid Research Funding**. MyoKardia will pay Fulcrum a non-refundable payment of Two Million Five Hundred Thousand Dollars ($2,500,000) as prepaid research funding within [**] after receipt by MyoKardia of a corresponding invoice from Fulcrum, which shall be issued no earlier than the Effective Date.

6.3. **Milestone Payments**.

6.3.1. **[**] Milestone**. Upon delivery of a [**] by Fulcrum to MyoKardia pursuant to Section [**] (such achievement the "[**] **Milestone**"), MyoKardia will pay Fulcrum a non-refundable payment of [**] within [**] after receipt by MyoKardia of a corresponding invoice from Fulcrum ("[**] **Milestone Payment**"); *provided*, *however*, that if [**], MyoKardia will pay Fulcrum the [**] Milestone Payment [**] within [**] after receipt by MyoKardia of a corresponding invoice from Fulcrum. For the avoidance of doubt, the [**] Milestone Payment shall be payable only once.

6.3.2. **Preclinical Milestones**. Subject to the remainder of this Section 6.3 (Milestone Payments), on a Cardiomyopathy Target-by-Cardiomyopathy Target basis, MyoKardia will make each of the one-time milestone payments set forth in Table 6.3.2 ("**Preclinical Milestone Payments**") for the first achievement of each preclinical milestone event set forth in Table 6.3.2 ("**Preclinical Milestone**") by the first Collaboration Molecule directed against such Cardiomyopathy Target to achieve such event, pursuant to and in the amounts set forth in column (a), (b) or (c), depending upon whether such Cardiomyopathy Target is (a) the Lead Cardiomyopathy Target, (b) a Follow-On Cardiomyopathy Target or (c) an Additional Cardiomyopathy Target, respectively. MyoKardia will notify Fulcrum of the achievement of a Preclinical Milestone by MyoKardia, its Affiliates or its Licensees, in writing within [**] after such achievement. MyoKardia will pay Fulcrum the amount of the Preclinical Milestone Payment corresponding to such Preclinical Milestone within [**] following the receipt of a corresponding invoice from Fulcrum. For the avoidance of doubt, each Preclinical Milestone Payment will be payable only once with respect to a Cardiomyopathy Target, on the first achievement of the Preclinical Milestone by the applicable Product directed against such Cardiomyopathy Target (if at all), notwithstanding the number of times one or more Products directed against the same such Cardiomyopathy Target may achieve such Preclinical Milestone. For the further avoidance of doubt, in no event will the aggregate Preclinical Milestone Payments payable by MyoKardia under this Section 6.3.2 (Preclinical Milestones) exceed: (i) [**] for Collaboration Molecules directed against the Lead Cardiomyopathy Target; (ii) on a Follow-On Cardiomyopathy Target-by-Follow-On Cardiomyopathy basis, [**] for Collaboration Molecules directed against a Follow-On Cardiomyopathy Target; and (iii) on an Additional Cardiomyopathy Target-by-Additional

Cardiomyopathy Target basis, [**] for Collaboration Molecules directed against an Additional Cardiomyopathy Target.

Table 6.3.2 – Preclinical Milestones and Preclinical Milestone Payments			
Preclinical Milestone	**Preclinical Milestone Payments for the [First Collaboration Molecule that is Directed Against:**		
	(a) the Lead Cardiomyopathy Target	***(b) a Follow-On Cardiomyopathy Target***	***(c) an Additional Cardiomyopathy Target***
1. [**]	[**]	[**]	[**]
2. [**]	[**]	[**]	[**]

6.3.3. **Development Milestones**. Subject to the remainder of this Section 6.3 (Milestone Payments), on a Cardiomyopathy Milestone Target-by-Cardiomyopathy Milestone Target basis, MyoKardia will make each of the one-time milestone payments set forth in Table 6.3.3 ("**Development Milestone Payments**") for the first achievement of each development milestone event set forth in Table 6.3.3 ("**Development Milestone**") by the first Product directed against such Cardiomyopathy Milestone Target to achieve such event, pursuant to and in the amounts set forth in column (a), (b) or (c), depending upon whether such Cardiomyopathy Milestone Target is an Initial Cardiomyopathy Milestone Target (and, in such case, whether such Cardiomyopathy Milestone Target is the first Initial Cardiomyopathy Milestone Target to achieve the applicable Development Milestone) or a Secondary Cardiomyopathy Milestone Target. MyoKardia will notify Fulcrum of the achievement of a Development Milestone by MyoKardia, its Affiliates or its Licensees, in writing within [**] after such achievement. MyoKardia will pay Fulcrum the amount of the Development Milestone Payment corresponding to such Development Milestone within [**] following the receipt of a corresponding invoice from Fulcrum. For the avoidance of doubt, each Development Milestone Payment will be payable only once with respect to a Cardiomyopathy Milestone Target, on the first achievement of the Development Milestone by the applicable Product directed against such Cardiomyopathy Milestone Target (if at all), notwithstanding the number of times one or more Products directed against such Cardiomyopathy Milestone Target may achieve such Development Milestone. For the further avoidance of doubt, in no event will the aggregate Development Milestone Payments payable by MyoKardia under this Section 6.3.3 (Development Milestones) exceed: (i) [**] with respect to the first achievement of each Development Milestone, collectively, by a Product directed against any Initial Cardiomyopathy Milestone Target; (ii) except as set forth in the foregoing clause (i), on an Initial Cardiomyopathy Milestone Target-by-Initial Cardiomyopathy Milestone Target basis, [**] for Products directed against an Initial Cardiomyopathy Milestone Target; and (iii) on a Secondary Cardiomyopathy Milestone Target-by-Secondary Cardiomyopathy Milestone

Target basis, [**] for Products directed against a Secondary Cardiomyopathy Milestone Target.

Table 6.3.3 – Development Milestones and Development Milestone Payments			
Development Milestone	**Development Milestone Payment for the first Product Directed Against:**		
	(a) the first Initial Cardiomyopathy Milestone Target	***(b) a subsequent Initial Cardiomyopathy Milestone Target***	***(c) a Secondary Cardiomyopathy Milestone Target***
1. [**]	[**]	[**]	[**]
2. [**]	[**]	[**]	[**]
3. [**]	[**]	[**]	[**]
4. [**]	[**]	[**]	[**]
5. [**]	[**]	[**]	[**]
6. [**]	[**]	[**]	[**]

6.3.4. **Cumulative Sales Milestones**. Subject to the remainder of this Section 6.3 (Milestone Payments), on a Cardiomyopathy Milestone Target-by-Cardiomyopathy Milestone Target basis, during the Royalty Term, MyoKardia will make each of the one-time milestone payments set forth in Table 6.3.4 ("**Sales Milestone Payments**") for the first achievement of each sales milestone event set forth in Table 6.3.4 ("**Sales Milestone**") by the Products directed against such Cardiomyopathy Milestone Target to achieve such event, pursuant to and in the amounts set forth in column (a), (b) or (c), depending upon whether such Cardiomyopathy Milestone Target is an Initial Cardiomyopathy Milestone Target (and, in such case, whether such Cardiomyopathy Milestone Target is the first Initial Cardiomyopathy Milestone Target to achieve the applicable Sales Milestone) or a Secondary Cardiomyopathy Milestone Target. MyoKardia will notify Fulcrum of the achievement of a Sales Milestone by MyoKardia, its Affiliates or its Licensees, in writing within (i) [**] after the achievement of Sales Milestone 1 (First Commercial Sale of a Product); and (ii) [**] after the end of the Calendar Year in which each remaining Sales Milestone is achieved. MyoKardia will pay Fulcrum the amount of the Sales Milestone Payment corresponding to such Sales Milestone within [**] following the receipt of a corresponding invoice from Fulcrum. For the avoidance of doubt, each Sales Milestone Payment will be payable only once with respect to a Cardiomyopathy Milestone Target, on the first achievement of the Sales Milestone by the applicable Product directed against such Cardiomyopathy Milestone Target (if at all), notwithstanding the number of times one or more Products directed against the same such Cardiomyopathy Milestone Target may achieve such Sales Milestone. For the further avoidance of doubt, in no event will the aggregate Sales Milestone Payments payable by MyoKardia under this Section 6.3.4 (Cumulative Sales Milestones) exceed: (A) [**] with respect to the first achievement of

each Sales Milestone, collectively, by a Product directed against any Initial Cardiomyopathy Milestone Target, (B) except as set forth in the foregoing clause (i), on an Initial Cardiomyopathy Milestone Target-by-Initial Cardiomyopathy Milestone Target basis, [**] for Products directed against an Initial Cardiomyopathy Milestone Target; and (C) on a Secondary Cardiomyopathy Milestone Target-by-Secondary Cardiomyopathy Milestone Target basis, [**] for Products directed against a Secondary Cardiomyopathy Milestone Target.

Table 6.3.4 – Sales Milestones and Sales Milestone Payments

Sales Milestone	**Sales Milestone Payment for Products Directed Against:**		
	(a) the first Initial Cardiomyopathy Milestone Target	***(b) a subsequent Initial Cardiomyopathy Milestone Target***	***(c) a Secondary Cardiomyopathy Milestone Target***
1. [**]	[**]	[**]	[**]
2. Cumulative Net Sales of Products are equal to or exceed [**]	[**]	[**]	[**]
3. Cumulative Net Sales of Products are equal to or exceed [**]	[**]	[**]	[**]
4. Cumulative Net Sales of Products are equal to or exceed [**]	[**]	[**]	[**]
5. Cumulative Net Sales of Products are equal to or exceed [**]	[**]	[**]	[**]

6.3.5. **Additional Terms Applicable to Milestones.** The following additional terms apply to some or all of the Milestones and Milestone Payments.

(a) *Replacement Target*. On a Cardiomyopathy Milestone Target-by-Cardiomyopathy Milestone Target basis, if MyoKardia discontinues development of Products directed against such Cardiomyopathy Milestone Target, MyoKardia may select another Cardiomyopathy Target Candidate to replace the terminated Cardiomyopathy Milestone Target at any time during the Exclusivity Period by written notice to Fulcrum (such selected Cardiomyopathy Target Candidate, the "**Replacement Target**"). If any such Replacement Target is so selected after any Preclinical Milestone or Development Milestone has already been achieved with respect to a Product directed against the original Cardiomyopathy Milestone Target, then MyoKardia shall not pay any Milestone Payment upon achievement of the same Milestone by any Product directed against such Replacement Target for which Fulcrum already received a Preclinical Milestone Payment or Development Milestone Payment for a Product directed against the original Cardiomyopathy Milestone Target.

(b) *Skipped Milestones*. Certain Development Milestones, together with the Preclinical Milestones are intended to be successive, and on a Cardiomyopathy Milestone Target-by-Cardiomyopathy Milestone Target basis, if the first Molecule or Product directed against such Cardiomyopathy Milestone Target is not required to undergo the event associated with any such Development Milestone or Preclinical Milestone (or if MyoKardia acquires rights to the first Molecule or Product directed against such Cardiomyopathy Milestone Target from a Third Party, including by license or acquisition, which rights are acquired with respect to a Molecule or Product that is at any stage of development after a Preclinical Milestone or Development Milestone, an "**Acquired Product**"), such skipped milestone will be deemed to have been achieved upon the achievement by such Product of the next successive Milestone; *provided* that,

(i) with respect to Development Milestones 4, 5, and 6 the "**Approval Milestones**"), none of the Approval Milestones will be deemed to have been achieved upon the achievement of any other Approval Milestone; and

(ii) the achievement of Development Milestone 4 will result in the deemed achievement of Development Milestone 3 (if not previously achieved).

Payment for any such skipped Development Milestones or Preclinical Milestones that is owed in accordance with the provisions of Section 6.3.3 (Development Milestones) with respect to a given Product will be due concurrently with the payment for the next successive Development Milestone or Preclinical Milestone by such Product; *provided* that, with respect to an Acquired Product, payment for any such skipped Development Milestones or Preclinical Milestones that is owed in accordance with the provisions of Section 6.3.3 (Development Milestones) will be due and payable with respect to such Acquired Product [**] following achievement of the corresponding Preclinical Milestone or Development Milestone.

6.4. **Royalties**.

6.4.1. **Royalty Rate**. Subject to Section 6.4.4 (Royalty Reductions), on a Product-by-Product basis during the Royalty Term, MyoKardia will pay to Fulcrum royalties based

on the Net Sales in any Calendar Year resulting from the sale of such Product in the Territory at the royalty rates set forth in Table 6.4.1 (the "**Royalty Rates**").

Table 6.4.1 – Royalty Rates	
Calendar Year Net Sales of a Product in the Territory	**Royalty Rate**
Portion of annual Net Sales of such Product in the Territory that is less than or equal to [**]	[**]
Portion of annual Net Sales of such Product in the Territory that is greater than [**], and less than or equal to [**]	[**]
Portion of annual Net Sales of such Product in the Territory that is greater than [**], and less than or equal to [**]	[**]
Portion of annual Net Sales of such Product in the Territory that is greater than [**]	[**]

6.4.2. **Royalty Term**. On a Product-by-Product and country-by-country basis, upon expiration of the Royalty Term for a Product in a country, (a) no further royalty payments will be payable by MyoKardia in respect of sales of such Product in such country, (b) no further Development Milestone Payments or Sales Milestone Payments will be payable by MyoKardia with respect of development or sales of such product in such country, and (c) the license granted to MyoKardia under Section 5.1 (License Grant to MyoKardia) with respect to such Product in such country will automatically become fully paid-up, perpetual, irrevocable, and royalty-free.

6.4.3. **Only One Royalty**. Only one royalty will be due with respect to the sale of the same unit of Product. Only one royalty will be due hereunder on the sale of a Product even if the manufacture, use, sale, offer for sale, importation or exportation of such Product infringes more than one Valid Claim of a Fulcrum Patent Right.

6.4.4. **Royalty Reductions**.

(a) *Valid Claim Adjustment*. On a Product-by-Product and country-by-country basis, in the event that during the Royalty Term there is no Valid Claim of a Fulcrum Patent Right Covering such Product in such country, then, commencing with the first Calendar Quarter there is no Valid Claim of a Fulcrum Patent Right Covering such Product in such country, the applicable Royalty Rate for such Product in such country will be reduced by [**].

(b) *Reduction for Generic Competition*. Subject to Section 6.4.4(d) (Cumulative Adjustments), on a Product-by-Product basis, in the event of Loss of Market Exclusivity with respect to a Product in a country, as from the first Calendar Quarter this Section 6.4.4(b) (Reduction for Generic Competition) applies, the applicable Royalty Rate pursuant to Section 6.4 (Royalty Rate) for annual Net Sales of such Product otherwise due in such country will be reduced by [**] where there is a Loss of Market Exclusivity for such Product in such Calendar Quarter in such country.

(c) *Third Party Obligations*. Subject to Section 6.4.4(d) (Cumulative Adjustments), in the event that MyoKardia determines that patents owned or controlled by a Third Party would be necessary or useful for the development or commercialization of any Product under this Agreement, MyoKardia may deduct [**] of any royalties paid by MyoKardia to such Third Party for sales of such Product thereunder in a given Calendar Quarter in a particular country against the royalties due and payable by MyoKardia to Fulcrum on the Net Sales for such Product in such Calendar Quarter in such country. For clarity, such deduction would be on a Product-by-Product basis (i.e., Third Party royalties by MyoKardia paid with respect to a Product would be stackable only against royalties for the same Product).

(d) *Cumulative Adjustments*. The provisions of Sections 6.4.4(a) (Valid Claim Adjustment), 6.4.4(b) (Reduction for Generic Competition), and 6.4.4(c) (Third Party Obligations) are cumulative; *provided*, *however*, on a country-by-country and Product-by-Product basis, in no event will the royalty reductions for a Product permitted under Sections 6.4.4(a) (Valid Claim Adjustment), 6.4.4(b) (Reduction for Generic Competition), or 6.4.4(c) (Third Party Obligations), alone or together, reduce the royalties due to Fulcrum for such Product pursuant to Section 6.4.1 (Royalty Rates) in a country in a given Calendar Quarter by more than (i) if such Product is not a New Modality, [**] of the applicable royalties that would otherwise be owed on the Net Sales of such Product or (ii) if such Product is a New Modality, [**] of the applicable royalties that would otherwise be owed on the Net Sales of such Product.

6.4.5. [**].

6.4.6. **Royalty Reports**. Following the first sale of a Product giving rise to Net Sales and continuing for the remainder of the Royalty Term, (a) within [**] after the end of each Calendar Quarter, MyoKardia will deliver a report to Fulcrum specifying on a Product-by-Product and country-by-country basis, MyoKardia's preliminary, non-binding, good faith estimates of the royalties payable to Fulcrum on Net Sales of such Products in such countries, and (b) within [**] after the end of each Calendar Quarter, MyoKardia will deliver a report to Fulcrum specifying on a Product-by-Product and country-by-country basis: (i) Net Sales in the relevant Calendar Quarter; (ii) to the extent such Net Sales include sales not denoted in Dollars, a summary of the then-current exchange rate methodology then in use by MyoKardia; (iii) a calculation of any adjustments to such royalties under Section 6.4.4(a) (Valid Claim Adjustment); (iv) the applicable Cardiomyopathy Milestone Target(s) directed against such Product and the applicable Royalty Rate(s) under this Agreement for such Net Sales; and (v) a calculation of the final royalties payable on such Net Sales. All royalty payments due under this Section 6.4 (Royalties) for each Calendar Quarter will be due and payable within [**] after the end of each Calendar Quarter. MyoKardia's reports delivered to Fulcrum under this Section 6.4.6 (Royalty Reports) will be MyoKardia's Confidential Information under this Agreement.

6.5. **Payment Terms; Blocked Payments**. All payments under this Agreement will be paid in Dollars, by wire transfer to an account designated by Fulcrum (which account Fulcrum

may update from time to time in writing). In the case of Net Sales made by MyoKardia and its Affiliates or Licensees in currencies other than Dollars, the rate of exchange to be used in computing the amount of Dollars due for royalty payments will be the rate of exchange utilized by MyoKardia in its worldwide accounting system and calculated in accordance with GAAP. If, by reason of Applicable Laws or regulations in any country, it becomes impossible or illegal for MyoKardia to transfer, or have transferred on its behalf, royalties or other payments to Fulcrum, such payments will be made in any such country in local currency in such country by deposit in a local bank designated by Fulcrum.

6.6. **Withholding Taxes**. If MyoKardia concludes Withholding Taxes are required under the laws of any country within the Territory with respect to payments to Fulcrum, MyoKardia will withhold the required amount and pay it to the appropriate Governmental Authority. In any such case, MyoKardia will promptly provide Fulcrum with original receipts or other evidence and cooperation as reasonably desirable and sufficient to allow Fulcrum to document such Withholding Taxes for purposes of claiming foreign tax credits and similar benefits.

6.7. **Records; Audits**. The Parties will (and will cause their respective Affiliates and sublicensees to) at all times keep and maintain accurate and complete records regarding, in the case of MyoKardia, Net Sales during [**], and in the case of Fulcrum, any costs for Fulcrum FTEs or Out-of-Pocket Expenses covered by the initial prepaid research funding or reimbursed by MyoKardia pursuant to Section 3.8 (Research Funding). Upon [**] prior written notice from the auditing Party, the non-auditing Party will (and will cause its Affiliates and sublicensees to) permit an independent certified public accounting firm of internationally recognized standing, selected by the auditing Party and reasonably acceptable to the non-auditing Party, to examine the relevant books and records of the non-auditing Party, its Affiliates, and sublicensees, as may be reasonably necessary to verify, in the case of MyoKardia, the royalty reports submitted by MyoKardia in accordance with Section 6.4.6 (Royalty Reports), and in the case of Fulcrum, the invoices submitted by Fulcrum in accordance with Section 3.8 (Research Funding). An examination by either Party under this Section 6.7 (Records; Audits) will occur not more than once in any Calendar Year and will be limited to the pertinent books and records for any Calendar Year ending not more than [**] before the date of the request. Further, a Party's (or its Affiliates' or sublicensees') books of records for any Calendar Year may be examined [**]. The accounting firm will be provided access to such books and records at the facility or facilities where such books and records are normally kept and such examination will be conducted during normal business hours. The non-auditing Party (or any Affiliate or sublicensee) may require the accounting firm to sign a customary non-disclosure agreement before providing the accounting firm access to its facilities or records. Upon completion of the audit, the accounting firm will provide both Fulcrum and MyoKardia a written report disclosing whether, in the case of MyoKardia, the reports submitted by MyoKardia, or in the case of Fulcrum, the invoices submitted by Fulcrum, are correct or incorrect and the specific details concerning any discrepancies. If any report submitted by MyoKardia or invoice submitted by Fulcrum results in an underpayment or overpayment, the Party owing the underpaid or overpaid amount will promptly pay such amount to the other Party with interest calculated in accordance with Section 6.8 (Late Payment). The costs and fees of any audit conducted by a Party under this Section 6.7 (Records; Audits) will be borne by the auditing Party, unless, in the case of an audit conducted by Fulcrum, such audit reveals an underpayment of amounts owed to Fulcrum of more than [**] of the amount that was owed by MyoKardia, or in the case of an audit conducted

by MyoKardia, such audit reveals an overpayment of amounts owed to Fulcrum of more than [**] of the amount that was properly payable by MyoKardia in accordance with Section 3.8 (Research Funding), in either case, with respect to the relevant Calendar Year, in which case, the non-auditing Party will reimburse the auditing Party for the reasonable expense incurred by the auditing Party in connection with the audit.

6.8. **Late Payment**. Any undisputed payments or portions thereof due hereunder that are not paid when due will accrue interest from the date due until paid at [**] above the Prime Rate of interest as reported in the Wall Street Journal (or if the Wall Street Journal no longer quotes such rate, as reported in another source mutually agreed by the Parties) on the date payment is due, compounded daily, but not to exceed the maximum permitted by Applicable Law. Any such overdue payment when made will be accompanied by all interest so accrued.

Article 7
INTELLECTUAL PROPERTY

7.1. **Ownership of Technology**. Notwithstanding any provision of this Agreement to the contrary, as between the Parties:

7.1.1. the Parties will jointly own (and may, subject to the licenses granted and other terms and conditions hereunder, exploit without a duty to account to the other Party and without an obligation to seek permission to grant licenses thereunder) all Know-How invented or created solely or jointly by the Parties, their Affiliates or Third Parties acting on their behalf, in the performance of activities under the Research Plan [**], including any such Know-How related to a Target ("**Target Know-How**"), and any Patent Right that claims or discloses any Target Know-How ("**Target Patent Rights**"), except for any Know-How and Patent Rights solely and exclusively owned by MyoKardia in accordance with Section 7.1.2;

7.1.2. MyoKardia will solely and exclusively own (a) all Know-How invented or created solely or jointly by the Parties, their Affiliates or Third Parties acting on their behalf, in the performance of activities under this Agreement to the extent relating to [**], and (b) any Patent Right that claims or discloses any Know-How described in clause (a);

7.1.3. Fulcrum will solely and exclusively own (a) all Know-How invented or created solely or jointly by the Parties, their Affiliates or Third Parties acting on their behalf, in the performance of activities under this Agreement solely to the extent such Know-How relates to the Fulcrum Platform and (b) any Patent Right that claims or discloses any Know-How described in clause (a) (the "**Platform Patent Rights**"); and

7.1.4. except as set forth in Sections 7.1.1, 7.1.2, and 7.1.3, (a) each Party will solely own (i) all Know-How invented or created solely by such Party, its Affiliates or Third Parties acting on its or their behalf in the performance of activities under this Agreement and (ii) any Patent Right that claims any Know-How described in clause (a)(i), and (b) the Parties will jointly own (and may, subject to the licenses granted hereunder, exploit without a duty to account to the other Party and without an obligation to seek permission to grant licenses thereunder) any (i) Know-How jointly invented or created by

the Parties, their Affiliates or Third Parties acting on their behalf, in the performance of activities under this Agreement and (ii) Patent Right that claims Know-How described in clause (b)(i).

7.2. **Cooperation**. Each Party will, and does hereby, assign, and will cause its Affiliates to, and use good faith efforts to cause its and their Representatives to, so assign, to the other Party, without additional compensation, such rights, title and interests in and to any Know-How or Patent Rights as are necessary to fully effect, as applicable, the allocation of ownership set forth in Section 7.1 (Ownership of Technology).

7.3. **Inventorship**. Inventorship of any inventions conceived or reduced to practice in the course of performance of activities pursuant to this Agreement shall be determined in accordance with U.S. patent laws (regardless of where the applicable activities occurred). In the case of unpatentable Know-How, inventorship will be determined under such U.S. patent law principles by treating such Know-How as if it were patentable.

7.4. **Prosecution and Maintenance of Patent Rights**.

7.4.1. **MyoKardia's Sole Right**. As between the Parties, MyoKardia will have the sole right, but not the obligation, at MyoKardia's expense, to control the preparation, filing, prosecution, maintenance and defense of the MyoKardia Patent Rights (other than the Joint Patent Rights).

7.4.2. **Fulcrum's First Right**. As between the Parties, Fulcrum will have the first right, but not the obligation, at Fulcrum's expense, to control the preparation, filing, prosecution, maintenance and defense of the Fulcrum Patent Rights (other than the Joint Patent Rights).

7.4.3. **Joint Patent Rights**. As between the Parties, MyoKardia shall have the right to control the preparation, filing, prosecution, maintenance and defense of any Joint Patent Right relating to a Target at its sole expense and discretion. Otherwise, the Parties shall mutually agree as to which Party shall have the right to control the preparation, filing, prosecution, maintenance and defense of any other Joint Patent Right, and the Parties shall share the costs of such activities ("**Jointly Controlled Patent Rights**"). If MyoKardia no longer wishes to file or maintain any Joint Patent Right, then MyoKardia shall so notify Fulcrum and such Joint Patent Right shall become a Jointly Controlled Patent Right. Immediately upon such notice to Fulcrum, or within [**] before an official action is due or deemed required to the applicable Governmental Authority with respect to such Jointly Controlled Patent Right, Fulcrum will have the second right, but not the obligation, to assume the preparation, filing, prosecution, maintenance and defense of such Jointly Controlled Patent Right upon written notice to MyoKardia and, upon such written notice to MyoKardia, MyoKardia's exclusive license to Fulcrum's rights under such Jointly Controlled Patent Right under Section 5.1 (License Grant to MyoKardia) shall terminate. If either Party decides not to share in the cost of filing or maintaining any Joint Patent Right (an "**Abandoned Patent Right**") and the other Party decides to continue prosecution or maintenance of the Joint Patent Right, then the continuing Party shall have sole discretion (and expense) regarding the prosecution and maintenance of the Abandoned Patent Right.

7.4.4. **MyoKardia's Second Right**. If Fulcrum fails or declines to file or maintain any Fulcrum Patent Right (other than Platform Patent Rights) that solely Covers a Product (each, a "**Product Patent Right**"), then within [**] before a response is due to the applicable Governmental Authority with respect to such Product Patent Right, MyoKardia will have the second right, but not the obligation, at MyoKardia's expense, to assume the preparation, filing, prosecution, maintenance and defense of such Product Patent Right upon written notice to Fulcrum.

7.4.5. **Cooperation**. Each Party will cooperate with the other Party to the extent reasonably necessary for a Party to prosecute the Product Patent Rights or the Joint Patent Rights, at the non-prosecuting Party's cost and expense, including by providing access to relevant records and documents (including laboratory notebooks) and other evidence, and making its employees available during reasonable business hours, executing all such documents and instruments and performing such acts (and causing its relevant Representatives to execute such documents and instruments and to perform such acts) as the prosecuting Party may reasonably request. The prosecuting Party with respect to any of the Product Patent Rights or the Joint Patent Rights in the Territory will give the non-prosecuting Party an opportunity to review any application with respect to such Patent Rights before filing, will consult with the non-prosecuting Party with respect thereto, and will consider any reasonable comments of the non-prosecuting Party with respect thereto. The prosecuting Party will supply the non-prosecuting Party with a copy of the application as filed, together with notice of its filing date and serial number. The prosecuting Party will keep the non-prosecuting Party reasonably informed of the status of the actual and prospective patent filings (including the grant of any such Patent Rights), and will provide advance copies of any official correspondence related to the filing, prosecution and maintenance of such patent filings including (i) all United States and non-United States patent office actions involving the Product Patent Rights or the Joint Patent Rights, (ii) the issuance of each patent included within the Product Patent Rights or the Joint Patent Rights, giving the date of issue and patent number for each such patent, and (iii) each notice pertaining to any patent included within the Product Patent Rights or the Joint Patent Rights which it receives pursuant to the Drug Price Competition and Patent Term Restoration Act of 1984 (the "**Act**"). As between the Parties, MyoKardia will have the sole right, but not the obligation, at MyoKardia's expense, to control any application for patent term extensions or supplementary protection certificates where applicable, with respect to the Product Patent Rights. Fulcrum will cooperate with MyoKardia in applying for any such patent term extensions. MyoKardia will notify Fulcrum of each filing for patent term restoration under the Act and all awards of patent term restoration (extensions) with respect to the Product Patent Rights.

7.5. **Defense of Claims Brought by Third Parties**. If any Third Party brings a claim or otherwise asserts that a Product manufactured, used or sold by MyoKardia, its Affiliates or Licensees infringes such Third Party's Patent Rights or misappropriates such Third Party's Know-How, the Party first having notice of the claim or assertion will promptly notify, but in any event no later than [**] after the receipt of notice of an action, the other Party in writing. Each Party will have the sole right to take action to defend any such claim brought against it by a Third Party; *provided*, *however*, that neither Party will enter into any settlement of any claim described in this Section 7.5 (Defense of Claims Brought by Third Parties) that materially and adversely affects the

other Party's rights or interests without first obtaining such Party's written consent. Nothing in this Section 7.5 (Defense of Claims Brought by Third Parties) will be deemed to relieve either Party of its rights or obligations under Article 9 (Indemnification; Insurance).

7.6. **Enforcement of Patent Rights**.

7.6.1. **Notice of Competitive Infringement**. Each Party will provide to the other Party written notice within [**] after becoming aware of any infringement, unauthorized use, misappropriation or threatened infringement of the Product Patent Rights, by a Third Party that is actually or potentially exploiting a product that is or would be competitive with a Product (a "**Competitive Infringement**").

7.6.2. **MyoKardia's First Right**. As between the Parties, MyoKardia will have the (a) sole right, but not the obligation, to enforce MyoKardia Patent Rights (other than the Joint Patent Rights) against any infringement, unauthorized use, misappropriation or threatened infringement by counsel of its own choice, at its own expense, and (b) the first right, but not the obligation, to enforce the Product Patent Rights and the Joint Patent Rights, against any Competitive Infringement by counsel of its own choice, at its own expense. For the avoidance of doubt, MyoKardia shall not have the right to enforce any Platform Patent Rights.

7.6.3. **Fulcrum's Second Right**. If, within [**] after receipt of notice of any Competitive Infringement, MyoKardia has not enforced the Product Patent Rights or the Joint Patent Rights, against such Competitive Infringement, then Fulcrum will have the second right, but not the obligation, at Fulcrum's expense, to enforce such Product Patent Rights or Joint Patent Rights against such Competitive Infringement, by counsel of its own choice, at its own expense, upon written notice to MyoKardia.

7.6.4. **Other Enforcement Actions**. As between the Parties, each Party will have the right, but not the obligation, to enforce the Joint Patent Rights against the unauthorized use, misappropriation or threatened infringement of the Joint Patent Rights by a Third Party that is actually or potentially exploiting a product that is or would be competitive with each Party's other product(s) (each, an "**Other Enforcement Action**"), without the consent of the other Party.

7.6.5. **Cooperation**. Each Party will cooperate with the other Party to the extent reasonably necessary for a Party to bring any enforcement action pursuant to Section 7.6.2 (MyoKardia's First Right), Section 7.6.3 (Fulcrum's Second Right) or Section 7.6.4 (Other Enforcement Actions), as applicable, at the non-enforcing Party's cost and expense, including by providing access to relevant records and documents (including laboratory notebooks) and other evidence, making its employees available during reasonable business hours, and executing all such documents and instruments and performing such acts (and causing its relevant Representatives to execute such documents and instruments and to perform such acts) as the prosecuting Party may reasonably request. The non-enforcing Party will, and will cause its Affiliates to, assist and cooperate with the enforcing Party, as the enforcing Party may reasonably request from time to time, in connection with any enforcement action under this Section 7.6 (Enforcement of Patent Rights), including

joining in, or being named as a necessary party to, any such enforcement action and executing any settlement agreement as reasonably requested by the enforcing Party; *provided* that the enforcing Party will reimburse the non-enforcing Party for its reasonable and verifiable out-of-pocket costs and expenses incurred in connection with its cooperation pursuant to this sentence. Unless otherwise set forth herein, the enforcing Party will have the right to settle all claims arising from any such enforcement action; *provided* that neither Party will have the right to settle any litigation or claim under this Section 7.6 (Enforcement of Patent Rights) in a manner that (a) imposes any costs or liability on the other Party or its Affiliates or its or their licensees, (b) involves any admission of wrongdoing, fault, or liability by the other Party or its Affiliates or its or their licensees, (c) admits the invalidity or unenforceability (in whole or in part) of intellectual property Controlled by the other Party or its Affiliates or its or their licensees, or (d) imposes restrictions or obligations on the other Party or its Affiliates or licensees not otherwise permitted under this Agreement, in each case ((a) through (d)), without the express written consent of such other Party, which will not be unreasonably withheld, conditioned, or delayed.

7.6.6. **Recovery of Damages**. Unless otherwise agreed by the Parties in writing, any damages or monetary awards recovered with respect to a proceeding under this Section 7.6 (Enforcement of Patent Rights) will be first allocated to reimburse the Parties for their costs and expenses incurred in connection with such proceeding (which amounts shall be allocated *pro rata* if insufficient to cover the totality of such costs and expenses), and the remainder, if any, shall be [**].

7.7. **Trademarks; Copyrights**. MyoKardia will have the sole discretion to select, prosecute, maintain, and enforce all trademarks, trade dress, and copyrights related to the Product(s).

Article 8
REPRESENTATIONS, WARRANTIES AND COVENANTS

8.1. **Mutual Representations and Warranties**. Each of the Parties hereby represents and warrants to the other Party that, as of the Effective Date:

8.1.1. it is duly organized, validly existing and in good standing under the laws of the jurisdiction of its organization;

8.1.2. the execution, delivery and performance of this Agreement by such Party has been duly authorized by all requisite action under the provisions of its charter, bylaws and other organizational documents, and does not require any action or approval by any of its shareholders or other holders of its voting securities or voting interests;

8.1.3. it has the power and authority to execute and deliver this Agreement and to perform its obligations hereunder;

8.1.4. this Agreement has been duly executed and is a legal, valid and binding obligation on each Party, enforceable against such Party in accordance with its terms; and

8.1.5. the execution, delivery, and performance by such Party of this Agreement (including such Party's respective Research Activities) and its compliance with the terms and provisions hereof does not and will not conflict with or result in a breach of or default under any agreement with a Third Party, order, judgment, agreement or instrument to which it is a party.

8.2. **Representations and Warranties of Fulcrum**. Fulcrum hereby represents and warrants to MyoKardia that, as of the Effective Date:

8.2.1. to its knowledge, the practice of the Fulcrum Technology existing as of the Effective Date and the Platform Patent Rights existing as of the Effective Date (the "**Existing Platform Patent Rights**") in the conduct of the Fulcrum Research Activities will not infringe any Patent Right or misappropriate any Know-How of any Third Party;

8.2.2. the Fulcrum Technology existing as of the Effective Date and Existing Platform Patent Rights are solely owned by Fulcrum or one of its Affiliates, free of any encumbrance, lien, or claim of ownership by any Third Party;

8.2.3. all current and former Fulcrum Representatives who have contributed to the creation or development of any Fulcrum Technology existing as of the Effective Date and Existing Platform Patent Rights have executed and delivered to Fulcrum or one of its Affiliates an agreement regarding the protection of proprietary information (including Confidential Information and Know-How) and the assignment to Fulcrum or such Affiliate of any intellectual property that arises from such Representatives' activities for Fulcrum or any of its Affiliates, and, to its knowledge, no current or former Representative is in violation of any such agreement;

8.2.4. it has not granted any right or license to any Affiliate or Third Party that would be inconsistent with or conflict with MyoKardia's rights hereunder, and there are no agreements or arrangements to which Fulcrum or any of its Affiliates is a party relating to Fulcrum Technology, Platform Patent Rights or the Fulcrum Platform that would (a) limit the rights granted to MyoKardia under this Agreement or (b) restrict or result in a restriction in MyoKardia's ability to research, develop, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, export, have exported, distribute, have distributed, market, have marketed, promote, have promoted, or otherwise exploit the Molecules or Products in the Field in the Territory in accordance with this Agreement;

8.2.5. there is no action, claim, demand, suit, proceeding, arbitration, grievance, citation, summons, subpoena, inquiry or investigation of any nature, civil, criminal, regulatory or otherwise, in law or in equity, pending or, to its knowledge after reasonable inquiry, threatened against Fulcrum or any of its Affiliates, in each case relating to the activities or transactions contemplated by this Agreement or that would impair Fulcrum's ability to perform its obligations under this Agreement; and

8.2.6. (a) all targets that meet the definition of "Excluded Targets" as of the Effective Date are listed on Schedule 1.62(a) and (b) there are no targets listed on Schedule 1.62(a) that do not meet the definition of "Excluded Targets" as of the Effective Date.

8.3. **Mutual Covenants**. During the Term, each Party covenants to the other Party that such Party:

8.3.1. will comply with Applicable Law in the performance of its respective obligations under this Agreement;

8.3.2. will not, and will cause its Affiliates not to, grant any right or license to any Third Party that would be inconsistent with or in conflict with, or take any action that would materially conflict with, (a) the rights it granted to the other Party under this Agreement or (b) its obligations to the other Party under this Agreement; and

8.3.3. will not knowingly engage directly, in any material capacity in connection with this Agreement, any Person who either has been debarred by the FDA, is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any such similar sanction of the EMA or another Regulatory Authority; and will inform the other Party in writing promptly if it or any Person engaged by such Party or any of its Affiliates who is performing material activities under this Agreement is debarred or is the subject of a conviction described in Section 306 of the FD&C Act, or if any action, suit, claim, investigation or legal or administrative proceeding is pending or, to such Party's knowledge, is threatened, relating to the debarment or conviction of such Party, any of its Affiliates or any such Person performing services hereunder or thereunder.

8.4. **Covenants of Fulcrum**. During the Term, Fulcrum covenants to MyoKardia that:

8.4.1. Fulcrum will promptly notify MyoKardia in writing if it receives written notice that the practice of the Fulcrum Technology or the Fulcrum Platform (to the extent the Fulcrum Platform is or was used in the Research Activities) infringes or will infringe any Patent Right or misappropriate any Know-How of any Third Party;

8.4.2. Fulcrum will maintain Control of all Fulcrum Technology that is or becomes such on the Effective Date or during the Term, and will not take any action during the Term that would materially adversely affect the rights to the Fulcrum Technology granted to MyoKardia in this Agreement;

8.4.3. Fulcrum will provide to MyoKardia all data from the performance of the Fulcrum Research Activities which data is related to any biological target against which any compound in the Fulcrum Platform has activity, except Excluded Targets, which, for the avoidance of doubt, will not include the identity of any biological target discovered in the course of the Research Activities except as provided in accordance with Section 3.6.2 (Part 1 Validation).

8.4.4. if, during the Term, Fulcrum controls any Person (with control being determined for this purpose in accordance with Section 1.10 (Affiliates)), then Fulcrum and any such Person will, at all times during which such relationship exists, be party to an

intercompany license agreement pursuant to which Fulcrum Controls all intellectual property owned or licensed to such Person that would otherwise be included in Fulcrum Technology if owned by Fulcrum; and

8.4.5. all Fulcrum Representatives that are in a position to contribute to the creation or development of any Fulcrum Technology have executed or will execute and deliver to Fulcrum or one of its Affiliates an agreement regarding the protection of proprietary information (including Confidential Information and Know-How) and the assignment to Fulcrum or such Affiliate of any intellectual property that arises from such Representatives' activities for Fulcrum or any of its Affiliates.

8.5. **Disclaimer**. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS AGREEMENT, NEITHER PARTY NOR ITS AFFILIATES MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS, IMPLIED OR OTHERWISE, TO THE OTHER PARTY, INCLUDING ANY WARRANTY OF MERCHANTABILITY, NON-INFRINGEMENT OR FITNESS FOR A PARTICULAR PURPOSE. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING AND WITHOUT LIMITING MYOKARDIA'S OBLIGATIONS UNDER SECTION 4.2 (DILIGENCE REQUIREMENTS), MYOKARDIA MAKES NO REPRESENTATION OR WARRANTY REGRADING WHETHER ANY MOLECULE OR PRODUCT WILL BE DEVELOPED OR COMMERCIALIZED SUCCESSFULLY OR WHETHER ANY PARTICULAR LEVEL OF SALES WILL BE ACHIEVED WITH REGARD TO ANY PRODUCT. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING, FULCRUM MAKES NO REPRESENTATION OR WARRANTY REGARDING THE OUTCOME OF THE RESEARCH ACTIVITIES, INCLUDING THAT THE RESEARCH ACTIVITIES WILL BE SUCCESSFUL.

Article 9
INDEMNIFICATION; INSURANCE

9.1. **Indemnification**.

9.1.1. **Indemnification by MyoKardia**. MyoKardia will indemnify Fulcrum and its Representatives (each, a "**Fulcrum Indemnified Party**") from and against any liability, loss, damage or expense (including reasonable attorneys' fees and expenses) (collectively, "**Liability**") that the Fulcrum Indemnified Party may incur or otherwise be required to pay to one or more Third Parties in connection with any Third Party suit, investigation, claim or demand resulting from or arising out of:

(a) any claims arising out of the research, development, manufacture, commercialization or use of any Product by, on behalf of, or under the authority of, MyoKardia, including all claims involving death or bodily injury;

(b) the conduct of the MyoKardia Research Activities;

(c) any claims arising out of the use of the Research Data by or on behalf of MyoKardia;

(d) the breach by MyoKardia of any of its representations, warranties or covenants set forth in this Agreement; or

(e) the negligence or willful misconduct of an MyoKardia Indemnified Party.

and except, in each case, to the extent such claims fall within the scope of Fulcrum's indemnification obligations under Section 9.1.2 (Indemnification by Fulcrum).

9.1.2. **Indemnification by Fulcrum**. Fulcrum will indemnify MyoKardia and its Representatives (each, a "**MyoKardia Indemnified Party**") from and against any Liability that the MyoKardia Indemnified Party may incur or otherwise be required to pay to one or more Third Parties in connection with any Third Party suit, investigation, claim or demand resulting from or arising out of:

(a) the conduct of the Fulcrum Research Activities (other than any Fulcrum Research Activities solely to the extent included in the Research Plan through an amendment to the Research Plan that is approved solely by MyoKardia, over Fulcrum's objection, by MyoKardia's Executive Officer in the exercise of MyoKardia's right to determine decisions of the JSC pursuant to Section 2.7.3 (subject to Section 2.7.4));

(b) any claims arising out of the use of the Research Data by or on behalf of Fulcrum;

(c) the breach by Fulcrum of any of its representations, warranties or covenants set forth in this Agreement; or

(d) the negligence or willful misconduct of a Fulcrum Indemnified Party.

and except, in each case, to the extent such claims fall within the scope of MyoKardia's indemnification obligations under Section 9.1.1 (Indemnification by MyoKardia).

9.1.3. **Procedure**. Each Party will notify the other Party in writing if it becomes aware of a claim for which such Party may seek indemnification hereunder. If any proceeding (including any governmental investigation) is instituted against a Party with respect to which indemnity may be sought pursuant to Sections 9.1.1 (Indemnification by MyoKardia) or 9.1.2 (Indemnification by Fulcrum), as applicable, such Party (the "**Indemnified Party**") will give prompt written notice of the indemnity claim to the other Party (the "**Indemnifying Party**") and provide the Indemnifying Party with a copy of any complaint, summons or other written notice that the Indemnified Party receives in connection with any such claim. An Indemnified Party's failure to deliver such written notice in a timely manner will relieve the Indemnifying Party of liability to the Indemnified Party under Sections 9.1.1 (Indemnification by MyoKardia) or 9.1.2 (Indemnification by Fulcrum), as applicable, only to the extent such delay is prejudicial to the Indemnifying Party's ability to defend such claim. The Indemnified Party will permit the Indemnifying Party to control any litigation relating to such claim and the disposition of such claim by

negotiated settlement or otherwise (subject to this Section 9.1 (Indemnification)). The Indemnifying Party will act reasonably and in good faith with respect to all matters relating to such claim and will not settle or otherwise resolve such claim without the Indemnified Party's prior written consent, which will not be unreasonably withheld, conditioned, or delayed; *provided* that such consent will not be required with respect to any settlement that includes a full and complete release of the Indemnified Party and involves only the payment of monetary awards for which the Indemnifying Party will be fully-responsible. The Indemnified Party will cooperate with the Indemnifying Party in the Indemnifying Party's defense of any claim for which indemnity is sought under this Agreement, at the Indemnifying Party's cost and expense.

9.2. **Insurance**. Each Party will maintain, at its cost, reasonable insurance against liability and other risks associated with its activities contemplated by this Agreement and will furnish to the other Party evidence of such insurance upon request. Notwithstanding the foregoing, either Party may self-insure to the extent that it self-insures for its other activities.

9.3. **No Consequential Damages**. EXCEPT WITH RESPECT TO LIABILITY ARISING FROM A BREACH OF THE CONFIDENTIALITY AND NON-USE PROVISIONS OF ARTICLE 11 (CONFIDENTIALITY) OR SECTION 5.5 (EXCLUSIVITY), OR TO THE EXTENT SUCH PARTY MAY BE REQUIRED TO INDEMNIFY THE OTHER PARTY UNDER THIS ARTICLE 9 (INDEMNIFICATION; INSURANCE), IN NO EVENT WILL EITHER PARTY OR ITS REPRESENTATIVES BE LIABLE UNDER THIS AGREEMENT FOR ANY SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE, INCLUDING LOSS OF PROFITS OR REVENUE, SUFFERED BY THE OTHER PARTY OR ANY OF ITS REPRESENTATIVES.

Article 10
TERM; TERMINATION

10.1. **Term**. The term of this Agreement will commence on the Effective Date and continue on a Product-by-Product and country-by-country basis until the expiration of the last-to-expire Royalty Term for such Product in such country (the period from the Effective Date until the expiration of this Agreement with respect to all Products in all countries, the "**Term**") unless this Agreement is terminated earlier in accordance with this Article 10 (Term; Termination). Notwithstanding the foregoing, if MyoKardia has not designated a Cardiomyopathy Target Candidate as a Lead Cardiomyopathy Target by the end of the First Designation Period, then this Agreement will automatically terminate.

10.2. **Termination**.

10.2.1. **Termination for Convenience**. MyoKardia may terminate this Agreement for convenience at any time in its entirety or on a Target-by-Target, Product-by-Product or Molecule-by-Molecule basis by providing written notice of its intent to terminate to Fulcrum, in which case, such termination will be effective [**] after Fulcrum's receipt of such written notice.

10.2.2. **For Safety**. MyoKardia may terminate this Agreement in its entirety or on a Target-by-Target, Product-by-Product or Molecule-by-Molecule basis, effective immediately upon written notice to Fulcrum in the event of:

(a) withdrawal or suspension of any Regulatory Approval for a Product directed against a Target in a Major Market Country;

(b) a Regulatory Authority notifies MyoKardia or its Affiliate that there is a safety issue regarding any Molecule or Product directed against a Target; or

(c) MyoKardia in good faith determines that it is not advisable to continue to develop or commercialize any Product directed against a Target as a result of a safety concern regarding the use thereof for the indication to which such Product is directed, based on specific results generated in connection with the development conducted hereunder by either Party, safety reports, evidence provided in scientific publications or other objective evidence from credible sources.

10.2.3. **Termination for Material Breach**. In the event either Party commits a material breach of its obligations under this Agreement and fails to cure that breach within [**] (or, in the case of a payment breach, [**]) after receiving written notice thereof, then the non-breaching Party may terminate this Agreement in its entirety if the breach is not specific to a Target, Product or Molecule, or on a Target-by-Target, Product-by-Product or Molecule-by-Molecule basis with respect to the Target to which the breach relates, immediately upon written notice to the breaching Party upon the expiration of such cure period; *provided*, *however*, that if such breach (other than a payment breach) is capable of being cured but cannot be cured within such [**] period and the breaching Party initiates actions to cure such breach within such period and thereafter diligently pursues such actions, then the cure period will be extended for so long as the breaching Party is diligently pursuing such actions not to exceed [**]. In the event that the breaching Party disputes in good faith the non-breaching Party's grounds for terminating this Agreement under this Section 10.2.3 (Termination for Material Breach), then the Parties will refer such dispute to the Executive Officers pursuant to Section 12.1.2 (Resolution by Executive Officers) and, if necessary, Section 12.1.3 (Resolution by Mediation), and any cure period provided for under this Section 10.2.3 (Termination for Material Breach) will be tolled during the pendency of such dispute.

10.2.4. **Termination for Insolvency**.

(a) If either Party makes an assignment for the benefit of creditors, appoints or suffers appointment of a receiver or trustee over all or substantially all of its property, files a petition under any bankruptcy or insolvency act or has any such petition filed against it that is not discharged within [**] after the filing thereof (each, an "**Insolvency Event**"), the other Party may terminate this Agreement in its entirety by providing written notice to the Party subject to the Insolvency Event (such Party, the "**Insolvent Party**"), in which case, this Agreement will terminate on the date on which the Insolvent Party receives such written notice; *provided* that

no termination shall be permitted pursuant to this clause (a) if the Insolvent Party (i) does not reject this Agreement or otherwise disavow its obligations hereunder, (ii) continues to perform its obligations hereunder during such Insolvency Event, and (iii) assumes this Agreement or otherwise affirms its obligations hereunder on or before any deadline for doing so during such Insolvency Event.

(b) All licenses and rights to licenses granted under or pursuant to this Agreement by Fulcrum to MyoKardia are, and will otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code (the "**Bankruptcy Code**"), licenses of rights to "intellectual property" as defined under Section 101(35A) of the Bankruptcy Code. The Parties agree that MyoKardia, as a licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that that upon commencement of a bankruptcy proceeding by or against Fulcrum under the Bankruptcy Code, MyoKardia will be entitled to a complete duplicate of, or complete access to (as MyoKardia deems appropriate), all such intellectual property and all embodiments of such intellectual property as may be necessary for MyoKardia to exercise its rights and licenses in accordance with this Agreement. Such intellectual property and all embodiments of such intellectual property will be promptly delivered to MyoKardia (i) upon any such commencement of a bankruptcy proceeding and upon written request by MyoKardia, unless Fulcrum assumes this Agreement or otherwise affirms its obligations hereunder on or before any deadline for doing so during such bankruptcy proceeding, or (ii) if not delivered under (i) above, upon the rejection of this Agreement by or on behalf of Fulcrum and upon written request by the MyoKardia. Fulcrum (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) agrees not to interfere with the exercise by MyoKardia or its Affiliates of its rights and licenses to such intellectual property and such embodiments of intellectual property in accordance with this Agreement, and agrees to assist MyoKardia and its Affiliates in obtaining such intellectual property and such embodiments of intellectual property in the possession or control of Third Parties as reasonably necessary or desirable for MyoKardia to exercise such rights and licenses in accordance with this Agreement. The foregoing provisions are without prejudice to any rights MyoKardia may have arising under the Bankruptcy Code or other Applicable Law.

10.3. **Alternative in Lieu of Termination**. Notwithstanding anything to the contrary set forth in this Agreement, if MyoKardia has the right to terminate this Agreement pursuant to Section 10.2.3 (Termination for Material Breach) in its entirety or on a Target-by-Target, Product-by-Product or Molecule-by-Molecule basis with respect to the Target, Product or Molecule to which the breach relates (after the expiration of all relevant cure periods and resolution of any dispute with respect to MyoKardia's right to terminate this Agreement), or Section 10.2.4 (Termination for Insolvency), MyoKardia may either elect, in lieu of terminating this Agreement, for the rights and obligations of the Parties under this Agreement to remain in full force and effect, including the licenses and rights granted by Fulcrum to MyoKardia under Section 5.1 (License Grant to MyoKardia); *provided* that, with respect to such Product or Molecule or all Products or Molecules directed against such Target (or, in the case of a right to terminate the Agreement in its entirety,

with respect to all Products), MyoKardia's financial obligations under Sections 6.3 (Milestone Payments) and 6.4 (Royalties) will be reduced by [**] of what they would otherwise be if calculated in accordance with such Section [**].

10.4. **Consequences of Termination of this Agreement**.

10.4.1. **Effects of Termination**. Upon any termination of this Agreement in its entirety or with respect to a Target, Product or Molecule:

(a) all license rights granted by Fulcrum to MyoKardia pursuant to this Agreement, including the license rights granted to MyoKardia under Section 5.1 (License Grant to MyoKardia) will terminate in their entirety or with respect to such Target, Product or Molecule, as applicable, as of the effective date of termination;

(b) any sublicenses granted by MyoKardia with respect to the license rights terminated pursuant to Section 10.4.1 (Effects of Termination) will remain in full force and effect upon termination of this Agreement in its entirety or with respect to such Target, Product or Molecule, as applicable; *provided* that (i) as of the effective date of such termination, the applicable Sublicensee is not in breach of its sublicense agreement, (ii) the applicable Sublicensee agrees to be bound directly to Fulcrum under the terms of this Agreement to the extent of the sublicensed rights, and (iii) Fulcrum will not be required to assume any obligations or liabilities beyond those contemplated by this Agreement as a result of this Section 10.4.1 (Effects of Termination); and

(c) MyoKardia may sell any existing of all such Product(s) or all such Products directed against such Target, as applicable, in MyoKardia's existing inventory, on order from an supplier or in the process of being manufactured, in each case, for a period of up to [**] after the effective date of termination of this Agreement or with respect to such Target, subject to MyoKardia's obligations to make corresponding payments with respect to any such sales pursuant to Article 6 (Financial Provisions). After expiration of such [**] period, MyoKardia shall discontinue selling any such Product(s) or all such Products directed against such Target, as applicable.

10.4.2. **Effect of Termination or Expiration**. Each Party will promptly return all Confidential Information of the other Party as provided in upon any expiration or termination of this Agreement.

10.4.3. **Surviving Provisions**. The following provisions will survive any expiration or termination of this Agreement for the period of time specified in such provision, or if not specified, then they will survive indefinitely: Article 1 (Definitions), Article 7 (Intellectual Property), Article 9 (Indemnification; Insurance), Article 11 (Confidentiality), Article 12 (Dispute Resolution), and Article 13 (Miscellaneous), and Section 3.9 (Records); Section 5.1 (License Grant to MyoKardia) (solely to the extent provided in Section 6.4.2(c) (Royalty Term)), Section 6.3 (Milestone Payments) through

Section 6.8 (Late Payment) (with respect to payment obligations that accrued prior to expiration of the [**] period in Section 10.4.1(c) for Products), Section 8.5 (Disclaimer), Section 10.2.4 (Termination for Insolvency), Section 10.4 (Consequences of Termination of this Agreement). Termination of this Agreement will not relieve the Parties of any liability which accrued under this Agreement prior to the effective date of such termination nor preclude either Party from pursuing all rights and remedies it may have under this Agreement or at law or in equity with respect to any breach of this Agreement. The remedies provided in this Article 10 (Term; Termination) are not exclusive of any other remedies a Party may have in law or equity.

Article 11
CONFIDENTIALITY

11.1. **Confidentiality**. Each Party (the "**Receiving Party**") receiving any Confidential Information of the other Party (the "**Disclosing Party**") will: (a) keep the Disclosing Party's Confidential Information confidential; (b) not publish, or allow to be published, and will not otherwise disclose, or permit the disclosure of, the Disclosing Party's Confidential Information; and (c) not use, or permit to be used, the Disclosing Party's Confidential Information for any purpose, except, in each case, to the extent expressly permitted under this Agreement or otherwise agreed by the Parties in writing. In protecting the Confidential Information of the Disclosing Party, the Receiving Party will use the same degree of care it uses for its own Confidential Information of a similar nature, but in no event less than a reasonable degree of care.

11.2. **Authorized Disclosure**.

11.2.1. Notwithstanding Section 11.1 (Confidentiality), the Receiving Party may disclose the Disclosing Party's Confidential Information and the existence and terms of this Agreement to the extent such disclosure is reasonably necessary to:

(a) file or prosecute patent applications as contemplated by this Agreement; *provided* that the Receiving Party will give reasonable advance notice of such disclosure to the Disclosing Party and take such measures to ensure confidential treatment of such Confidential Information as is reasonably requested by the Disclosing Party;

(b) prosecute or defend litigation as contemplated by or to enforce this Agreement;

(c) perform its obligations and to exploit its licenses and other rights under this Agreement;

(d) provide relevant information to its advisors (including financial advisors, attorneys, and accountants), actual or potential acquisition or strategic partners, collaborators, licensors, licensees, financing sources or investors, lenders and other financing sources and underwriters who have a legitimate business reason to know such Confidential Information; *provided* that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein (but of

duration customary in confidentiality agreements entered into for a similar purpose) (which may include professional ethical obligations);

(e) respond to a valid order of a court of competent jurisdiction or other supra-national, federal, national, regional, state, provincial or local governmental or regulatory body of competent jurisdiction; *provided*, *however*, that if the Receiving Party provides the Disclosing Party with prior written notice of any such disclosure and takes reasonably practicable and legally permissible actions to limit disclosure of and seek protective orders or other confidential treatment for such Confidential Information; or

(f) comply with Applicable Law, including securities laws and the rules of any securities exchange or market on which a Receiving Party's securities are listed or traded; *provided* that the Receiving Party provides the Disclosing Party with prior written notice of any such disclosure (to the extent reasonably practicable and legally permissible), and, in the case of disclosures other than those required by securities laws and the rules of any securities exchange or market on which a receiving Party's securities are listed or traded, the Receiving Party provides reasonable assistance to the Disclosing Party to limit disclosure of or seek confidential treatment for such Confidential Information, and, in the case of disclosures required by securities laws and the rules of any securities exchange or market on which a Receiving Party's securities are listed or traded, the Receiving Party takes reasonable steps, upon the advice of securities counsel, to limit disclosure of or seek confidential treatment for such Confidential Information.

11.2.2. In addition to the foregoing, the Receiving Party may disclose the Disclosing Party's Confidential Information to its Representatives who have a need to know such Confidential Information in connection with the Receiving Party's performance of its obligations under this Agreement; *provided* that such Representatives are (a) informed of the confidential nature of the Disclosing Party's Confidential Information and (b) bound by written obligations of confidentiality and non-use at least as restrictive as those set forth herein, and the Receiving Party remains liable for the compliance of such Representatives with the terms hereof.

11.3. **Exceptions**. The Receiving Party's obligations of non-disclosure and non-use under this Agreement will not apply to any portion of the Disclosing Party's Confidential Information that the Receiving Party can demonstrate, by competent proof:

11.3.1. is generally known to the public at the time of disclosure or becomes generally known through no wrongful act on the part of the Receiving Party;

11.3.2. is in the Receiving Party's possession prior to the time of disclosure, other than as a result of the Receiving Party's breach of any legal obligation with respect to such Confidential Information;

11.3.3. becomes known to the Receiving Party on a non-confidential basis through disclosure by sources other than the Disclosing Party having the legal right to disclose such Confidential Information; or

11.3.4. is independently developed by the Receiving Party without reference to or reliance upon the Disclosing Party's Confidential Information as evidenced by contemporaneously written records.

Specific aspects or details of Confidential Information shall not be deemed to be within the public domain or in the possession of the Receiving Party merely because such Confidential Information is embraced by general disclosures in the public domain or in the possession of the Receiving Party. In addition, any combination of Confidential Information shall not be considered in the public domain or in the possession of the Receiving Party merely because individual elements thereof are in the public domain or in the possession of the Receiving Party unless the combination and its principles are in the public domain or in the possession of the Receiving Party.

11.4. **Expiration or Termination of this Agreement**. Following expiration or termination of this Agreement, at the Disclosing Party's election, the Receiving Party will promptly (but no more than [**] after such request) return or destroy, all data, files, records and other materials containing or comprising the Disclosing Party's Confidential Information, except to the extent such Confidential Information is necessary or useful to conduct surviving obligations or exercise surviving rights. Notwithstanding the foregoing, (a) the Receiving Party will be permitted to retain one (1) copy of the confidential information of the other Party for archival and legal compliance purposes, and (b) the Receiving Party will not be required to delete or destroy any electronic back-up tapes or other electronic back-up files that have been created solely by the Receiving Party's automatic or routine archiving and back-up procedures, to the extent created and retained in a manner consistent with its or their standard archiving and back-up procedure; *provided* in each case such retained information will continue to be subject to the confidentiality and non-use obligations set forth under this Article 11 (Confidentiality). The confidentiality and non-use obligations set forth under this Article 11 (Confidentiality) will survive expiration or termination of this Agreement for [**] from the effective date of such expiration or termination.

11.5. **Public Announcement**. Promptly following the Effective Date, the Parties will jointly issue a mutually agreed press release regarding the signing of this Agreement in the form attached hereto as Schedule 11.5. Except (a) as set forth in the preceding sentence or Section 11.2 (Authorized Disclosure) (b) as may be expressly permitted under this Section 11.5 (Public Announcement), neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party. Notwithstanding the foregoing, (i) MyoKardia may make scientific publications or public announcements concerning its research, development, manufacture or commercialization activities with respect to any Product under this Agreement without Fulcrum's prior written approval but subject to MyoKardia's obligations under this Article 11 (Confidentiality), and (ii) Fulcrum may announce the achievement of any Milestone or the payment of any Milestone Payment without MyoKardia's prior written approval; *provided* that (A) Fulcrum shall not disclose details sufficient to identify any Target, or any Molecule or Product without MyoKardia's prior written consent, unless required by Applicable Law and (B) Fulcrum shall provide MyoKardia reasonable advance notice of any such proposed announcement,

and will incorporate such reasonable comments and revisions to protect the Confidential Information of MyoKardia as reasonably requested by MyoKardia, and (iii) Fulcrum will not otherwise make any publications, presentations or public announcements of any kind regarding any of the activities contemplated under this Agreement or the results of such activities without MyoKardia's prior written consent in each instance. Neither Party will use the other Party's or its Affiliates' name or logo in any label, press release or product advertising, or for any other promotional purpose, without first obtaining the other Party's prior written consent.

Article 12
DISPUTE RESOLUTION

12.1. **Dispute Resolution**. If any dispute or disagreement arises between the Parties with respect to any matter under this Agreement, they will follow the following procedures in an attempt to resolve the dispute or disagreement:

12.1.1. **Resolution by the JSC**. The Party claiming that such a dispute exists will give notice in writing to the other Party of the nature of the dispute, and the JSC will meet to try to resolve the dispute.

12.1.2. **Resolution by Executive Officers**. If the JSC is not able to resolve such dispute within [**] after receipt of such notice, then the dispute will be submitted to the Executive Officers of MyoKardia and Fulcrum for resolution.

12.1.3. **Resolution by Mediation**. If the Executive Officers are not able to resolve such dispute within [**] after escalation to the Executive Officers, then, except for (a) any matter for which MyoKardia has final decision-making authority under Section 2.7 (JSC Decision-Making) and (b) any Excluded Claim, the Parties shall enter into confidential non-binding mediation in the State of Delaware in accordance with the Commercial Mediation Rules of the American Arbitration Association ("**AAA**"). The Parties shall mutually approve a mediator who has the requisite experience and qualifications, and if the Parties are unable to mutually approve a mediator, then a mediator having the requisite experience and qualifications shall be appointed in accordance with such Commercial Mediation Rules. The mediation shall be held within [**] of the selection of the mediator. A representative of each Party with authority to resolve the dispute shall participate in the mediation. Each Party agrees to use reasonable efforts to make its current employees available, if reasonably needed, as the mediator may determine. The fees and expenses related to the services provided by the mediator in connection with any mediation hereunder shall be paid one-half by each party, except that each Party shall pay its own attorneys' fees and expenses. If the Parties are unable to resolve their dispute through mediation within [**] after selection of the mediator, either Party may invoke any remedy available to it under law or equity to resolve the dispute.

12.1.4. **Excluded Claims**. Notwithstanding the other provisions of this Section 12.1 (Dispute Resolution), any dispute that involves the (i) breach of the confidentiality and non-use provisions of Article 11 (Confidentiality), (ii) the scope, validity, enforceability, inventorship or infringement of Patent Rights, (iii) applicability of the Bankruptcy Code, or (iv) compliance by the Parties with any Applicable Laws

governing antitrust, anti-monopoly or competition law, whether or not statutory, (each an "**Excluded Claim**") will not be subject to the provisions of Section 12.1.3 (Resolution by Mediation).

12.2. **Injunctive Relief**. Notwithstanding any provision to the contrary set forth in this Agreement, the Parties each stipulate and agree that a breach of Article 5 (License Grants and Exclusivity) or Article 11 (Confidentiality) by a Party may cause irrevocable harm for which monetary damages would not provide a sufficient remedy, and in such case, the non-breaching Party will be entitled to equitable relief, including specific performance, temporary or permanent restraining orders, preliminary injunction, or permanent injunction, or other equitable relief without the posting of any bond or other security, from any court of competent jurisdiction, in each case, without first submitting to the dispute resolution procedures set forth in Section 12.1 (Dispute Resolution).

Article 13
MISCELLANEOUS

13.1. **Assignment**. This Agreement will not be assignable by any Party to any Third Party without the written consent of the non-assigning Party. Notwithstanding the foregoing, (a) without the written consent of MyoKardia, Fulcrum may assign this Agreement or its rights and obligations under this Agreement to (i) a Third Party in connection with a Change of Control, or (ii) an Affiliate (both only for so long as such Affiliate remains an Affiliate), in each case ((i) and (ii)), that agrees in writing to be bound by the terms of this Agreement and (b) without the written consent of Fulcrum, MyoKardia may assign this Agreement or its rights and obligations under this Agreement in whole or on a Target-by-Target or Product-by-Product basis to an Affiliate (only for so long as such Affiliate remains an Affiliate) or its or their Sublicensees or to any successor in interest (whether by merger, acquisition, asset purchase or otherwise) to all or substantially all or of the business to which this Agreement (or the applicable Target or Product) relates; *provided* that such Affiliate, such Sublicensee or such successor in interest agrees in writing to be bound by the terms of this Agreement. This Agreement will be binding upon the successors and permitted assigns of the Parties and the name of a Party appearing herein will be deemed to include the names of such Party's successors and permitted assigns to the extent necessary to carry out the intent of this Agreement. Any assignment not in accordance with this Section 13.1 (Assignment) will be void.

13.2. **Representation by Legal Counsel**. Each Party hereto represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, no presumption will exist or be implied against the Party that drafted such terms and provisions.

13.3. **Notices**. All notices which are required or permitted hereunder will be in writing and sufficient if delivered personally, sent by email or sent by nationally-recognized overnight courier, addressed as follows:

If to Fulcrum:

Fulcrum Therapeutics, Inc.
26 Landsdowne Street
Cambridge, MA 02139
Attention: Bryan Stuart
Email: [**]

with a copy that will not constitute notice to:

Ropes & Gray LLP
Attn: Marc A. Rubenstein
Prudential Tower
800 Boylston Street
Boston, MA 02199
Email: marc.rubenstein@ropesgray.com

If to MyoKardia:

MyoKardia, Inc.
1000 Sierra Point Parkway
Brisbane, CA 94005
Attention: Jake Bauer
Email: [**]

MyoKardia, Inc.
1000 Sierra Point Parkway
Brisbane, CA 94005
Attention: Legal Department
Email: [**]

with a copy to that will not constitute notice to:

Goodwin Procter LLP
100 Northern Avenue
Boston, MA 02210
Attention: Richard A. Hoffman
Email: RHoffman@goodwinlaw.com

or to such other address as the Party to whom written notice is to be given may have furnished to the other Party in writing in accordance herewith. Any such written notice will be deemed to have been given and received by the other Party: (a) when delivered if personally delivered; or (b) on receipt if sent by email or overnight courier.

13.4. **Amendment**. No amendment, modification or supplement of any provision of this Agreement will be valid or effective unless made in writing and signed by a duly authorized officer of each of the Parties.

13.5. **Waiver**. No provision of this Agreement will be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The waiver by either Party of any breach of any provision hereof by the other Party will not be construed to be a waiver of any succeeding breach of such provision or a waiver of the provision itself.

13.6. **Severability**. If any clause or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same will not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement will be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement will be construed as if such clause of portion thereof had never been contained in this Agreement, and there will be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by Applicable Law.

13.7. **Descriptive Headings**. The descriptive headings of this Agreement are for convenience only and will be of no force or effect in construing or interpreting any of the provisions of this Agreement.

13.8. **Governing Law**. This Agreement, and all claims arising under or in connection therewith, will be governed by and interpreted in accordance with the substantive laws of the State of Delaware, without regard to conflict of law principles thereof; *provided*, *however*, that with respect to matters involving the enforcement, validity or scope of intellectual property rights, the intellectual property laws of the applicable country will apply. Each of the Parties agrees that this Agreement has been entered into by the Parties in express reliance upon 6 Del. C. § 2708. To the extent that any right of a Party requires enforcement through court process, such enforcement will then be subject to the exclusive jurisdiction of the state courts of Delaware and federal courts of competent jurisdiction located in Wilmington, Delaware. Each of the Parties hereby irrevocably and unconditionally agrees (a) to be subject to the exclusive jurisdiction of the state courts of Delaware and federal courts located in Wilmington, Delaware; (b) (i) to the extent such Party is not otherwise subject to service of process in the State of Delaware, to appoint and maintain an agent in the State of Delaware as such Party's agent for acceptance of legal process and to notify the other Party of such appointment and any change to such appointment from time to time, in each case promptly after such appointment or change or request by the other Party, and (ii) that, to the fullest extent permitted by Applicable Law, service of process may also be made on such Party by prepaid certified mail with a proof of mailing receipt validated by post mail constituting evidence of valid service; and (c) that service made pursuant to the foregoing clause (b)(i) or (b)(ii) will, to the fullest extent permitted by Applicable Law, have the same legal force and effect as if served upon such Party personally within the State of Delaware. Each of the Parties hereby irrevocably and unconditionally waives any objection to the jurisdiction of or venue in the state courts of Delaware and federal courts located in Wilmington, Delaware.

13.9. **Entire Agreement**. This Agreement constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and cancels and supersedes all prior negotiations, correspondence, understandings and agreements, whether oral or written, between the Parties respecting the subject matter of this Agreement, including the CDA.

13.10. **Independent Contractors**. The Parties are independent contractors under this Agreement. Nothing contained herein will be deemed to create an employment, agency, joint venture or partnership relationship between the Parties or any of their agents or employees, or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party. Neither Party will have any express or implied power to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

13.11. **Force Majeure**. Each Party will be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by a *force majeure* event and the nonperforming Party promptly provides notice of the prevention to the other Party. Such excuse will be continued so long as the condition constituting *force majeure* continues and the nonperforming Party uses commercially reasonable efforts to remove the condition. For purposes of this Agreement, *force majeure* will include conditions beyond the reasonable control of the Parties, including an act of God or terrorism, voluntary or involuntary compliance with any regulation, law or order of any government, war, civil commotion, labor strike or lock-out, epidemic, pandemic, failure or default of public utilities or common carriers, destruction of production facilities or materials by fire, earthquake, storm or like catastrophe. Notwithstanding the foregoing, as of the Effective Date, the COVID-19 Pandemic is not a *force majeure*. If a *force majeure* causes a material failure or delay in the performance by Fulcrum of obligation sunder the Research Plan under this Agreement for more than [**], MyoKardia may, at its option, terminate this Agreement.

13.12. **Interpretation**. Except where the context expressly requires otherwise, (a) the use of any gender herein will be deemed to encompass references to either or both genders, and the use of the singular will be deemed to include the plural (and vice versa), (b) the words “include”, “includes” and “including” will be deemed to be followed by the phrase “without limitation”, (c) the word “will” will be construed to have the same meaning and effect as the word “shall”, (d) any definition of or reference to any agreement, instrument or other document herein will be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person will be construed to include the Person’s successors and assigns, (f) the words “herein”, “hereof” and “hereunder”, and words of similar import, will be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (g) all references herein to Sections, Exhibits or Schedules will be construed to refer to Sections, Exhibits or Schedules of this Agreement, and references to this Agreement include all Exhibits and Schedules hereto, (h) the word “notice” means notice in writing (whether or not specifically stated) and will include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder “agree,” “consent” or “approve” or the like will require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging), (j) references to any specific law, rule or regulation, or article, section or other division thereof, will be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof, (k) the term “or” will be interpreted in the inclusive sense commonly associated with the term “and/or” and (l) unless otherwise specified, “day” means a calendar day.

13.13. **No Third Party Beneficiaries, Rights or Obligations**. There are no Third Party beneficiaries hereunder and the provisions of this Agreement are for the exclusive benefit of the Parties. No provision of this Agreement will be deemed or construed in any way to result in the creation of any rights or obligations in any Person not a Party to this Agreement, and no other Person or entity shall have any right or claim against either Party by reason of these provisions or be entitled to enforce any of these provisions against either Party.

13.14. **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

13.15. **Counterparts**. This Agreement may be executed in two counterparts, each of which will be an original and both of which will constitute together the same document. Counterparts may be signed and delivered by digital transmission (*e.g.*,.pdf), each of which will be binding when received by the applicable Party.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their representatives thereunto duly authorized as of the Effective Date.

MYOKARDIA, INC.	**FULCRUM THERAPEUTICS, INC.**
By: _____	By: _____
Name:	Name:
Title:	Title:

SCHEDULE 1.62

EXCLUDED TARGETS

[**]

SCHEDULE 3.1

RESEARCH PLAN

[**]

SCHEDULE 11.5

PRESS RELEASE

[*Attached.***]**

Exhibit 10.2

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

FIRST AMENDMENT TO THE RIGHT OF REFERENCE AND LICENSE AGREEMENT

This **FIRST AMENDMENT TO THE RIGHT OF REFERENCE AND LICENSE AGREEMENT** ("**Amendment**") is made and entered into, effective as of September 23, 2020, by and between GlaxoSmithKline Intellectual Property (No. 2) Limited, a company organized under the laws of England and Wales and having a place of business at 980 Great West Road, Brentford, Middlesex TW8 9GS England ("**GIP2**"), GlaxoSmithKline LLC, a Delaware limited liability company having a place of business at 1250 S. Collegeville Road, Collegeville, PA 19426-0989 ("**GSK LLC**") and Glaxo Group Limited, a company organized under the laws of England and Wales and having a place of business at 980 Great West Road, Brentford, Middlesex TW8 9GS England ("**GGL"**; together with GIP2 and GSK LLC", collectively referred to herein as "**GSK**") and Fulcrum Therapeutics, Inc., a Delaware corporation having a place of business at 26 Landsdowne Street, Cambridge, MA 02139 ("**Fulcrum**"). GSK and Fulcrum are herein collectively referred to as the "**Parties**".

BACKGROUND

WHEREAS, the Parties entered into a Right of Reference and License Agreement dated as of February 8, 2019 (the "**Original Agreement**") pursuant to which Fulcrum obtained from GSK a right of reference to the Losmapimod IND and an exclusive license under the GSK Intellectual Property to exploit losmapimod and pharmaceutical products containing losmapimod and agreed to, among other things, pay GSK development and regulatory milestones as provided in Section 3.2 of the Original Agreement;

WHEREAS, in [**] 2020 Fulcrum informed GSK that it intended to [**], and under the terms of the Original Agreement, [**] would trigger the milestone set forth in Section 3.2(b) pertaining to the [**] (the "**[**] Milestone**");

WHEREAS, Fulcrum and GSK have agreed to amend the Original Agreement to provide that the [**] Milestone will not be paid upon the [**] but will be paid upon the occurrence of other events as provided in this Amendment.

NOW THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, GSK and Fulcrum agree as follows:

1. Definitions in Amendment. In this Amendment, capitalized terms not otherwise defined herein shall have the meaning given to them in the Original Agreement or in Section 2 of this Amendment.

2. Revised Defined Term. The preamble of the Original Agreement is hereby amended by deleting the language "(this "Agreement")" from the first and second lines thereof

and Section 1.1 of the Original Agreement is hereby amended by adding the following definition after the definition of "Affiliate":

"Agreement" means the Right of Reference and License Agreement, dated as of February 8, 2019, made and entered into by and between GSK and Fulcrum, as amended from time to time."

3. Revised [**] Milestone. Section 3.2(b) of the Original Agreement is hereby amended by deleting the phrase "[**]" in the box under the column labelled "Milestone Event" in that Section and replacing it with the following:

"[**]."

4. Except for the changes expressly mentioned in this Amendment, all other terms and conditions of the Agreement shall remain unchanged and continue to be in full force and effect.

5. This Amendment may be executed in any number of counterparts, each of which shall be an original as against the Party whose signature appears thereon, but all of which taken together shall constitute one and the same instrument.

6. The provisions of Section 10.4 of the Original Agreement (Controlling Law and Dispute Resolution) shall apply equally to this Amendment.

[Signature page follows – the rest of this page intentionally left blank]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be duly executed, as of the date first above written.

GLAXOSMITHKLINE INTELLECTUAL PROPERTY (NO. 2) LIMITED

By: /s/ Adam Walker

Name: Adam Walker

Title: Director

GLAXOSMITHKLINE LLC

By: /s/ Hatixhe Hoxha

Name: Hatixhe Hoxha

Title: Assistant Secretary

GLAXO GROUP LIMITED

By: /s/ Adam Walker

Name: Adam Walker

Title: Director

FULCRUM THERAPEUTICS, INC.

By: /s/ Robert J. Gould

Name: Robert J. Gould

Title: President & CEO

(*Signature Page to Right of Reference and License Agreement*)

Exhibit 31.1

CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Robert J. Gould, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Fulcrum Therapeutics, 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)) 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) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);

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

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

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

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

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

Date: November 10, 2020

By: /s/ Robert J. Gould
Robert J. Gould, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Bryan Stuart, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Fulcrum Therapeutics, 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)) 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) (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);

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

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

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

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

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

Date: November 10, 2020

By: /s/ Bryan Stuart
Bryan Stuart
Chief Operating Officer
(Principal Financial 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 on Form 10-Q of Fulcrum Therapeutics, Inc. (the "Company") for the period ended September 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Robert J. Gould, President and Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 10, 2020

By: /s/ Robert J. Gould
Robert J. Gould, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

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 on Form 10-Q of Fulcrum Therapeutics, Inc. (the "Company") for the period ended September 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Bryan Stuart, Chief Operating Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 10, 2020

By: /s/ Bryan Stuart
Bryan Stuart
Chief Operating Officer
(Principal Financial Officer)