UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

(Mark One)

x 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

o 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: 001-37467

Catabasis Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**26-3687168**
(State or Other Jurisdiction of Incorporation or Organization)	(IRS Employer Identification No.)
100 High Street Floor 28 Boston, Massachusetts	**02110**
(Address of Principal Executive Offices)	(Zip Code)

(617) 349-1971

(Registrant's Telephone Number, Including Area Code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value per share	**CATB**	**The Nasdaq Stock Market LLC**

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** o

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** o

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a 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	o	Accelerated filer	o
Non-accelerated filer	x	Smaller reporting company	x
		Emerging growth company	x

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 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** o **No** ☒

As of October 30, 2020, there were 20,084,337 shares of the registrant’s Common Stock, par value $0.001 per share, outstanding.

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CAUTIONARY NOTE CONCERNING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. 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 and objectives of management and expected market growth are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- our expectations regarding our ability to stop development activities related to edasalonexent and wind down material costs related thereto;
- our expectations regarding our plans to explore and evaluate strategic options;
- our intellectual property position and strategy;
- our estimates regarding expenses, capital requirements and needs for additional financing;
- our expectations regarding possible litigation and investigations; and
- the impact of government laws and regulations.

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 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 that we may make or enter into.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

Catabasis Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share data)

(Unaudited)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 52,856	$ 9,899
Short-term investments	-	26,345
Prepaid expenses and other current assets	2,656	2,714
Total current assets	55,512	38,958
Right-of-use asset	1,178	2,349
Other assets	160	473
Total assets	$ 56,850	$ 41,780
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,408	$ 1,197
Accrued expenses	4,924	2,610
Current portion of operating lease liabilities	648	1,225
Total current liabilities	6,980	5,032
Long-term portion of operating lease liabilities	559	1,028
Total liabilities	7,539	6,060
Commitments (Note 6)		
Stockholders' equity:		
Preferred stock, $0.001 par value per share, 5,000,000 shares authorized and no shares issued and outstanding	-	-
Common stock, $0.001 par value per share, 150,000,000 shares authorized; 20,077,337 and 12,433,600 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	20	12
Additional paid-in capital	301,210	259,305
Accumulated other comprehensive loss	-	-
Accumulated deficit	(251,919)	(223,597)
Total stockholders' equity	49,311	35,720
Total liabilities and stockholders' equity	$ 56,850	$ 41,780

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

Catabasis Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 7,806	$ 4,697	$ 19,845	$ 14,054
General and administrative	3,057	1,985	8,612	6,287
Total operating expenses	10,863	6,682	28,457	20,341
Loss from operations	(10,863)	(6,682)	(28,457)	(20,341)
Other income (expense):				
Interest and investment income	4	214	231	697
Other expense, net	(3)	(46)	(96)	(39)
Total other income, net	1	168	135	658
Net loss	$ (10,862)	$ (6,514)	$ (28,322)	$ (19,683)
Net loss per share - basic and diluted	$ (0.56)	$ (0.56)	$ (1.59)	$ (1.80)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	19,424,866	11,624,232	17,769,738	10,945,765

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

Catabasis Pharmaceuticals, Inc.
Condensed Consolidated Statements of Comprehensive Loss
(In thousands)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Net loss	$ (10,862)	$ (6,514)	$ (28,322)	$ (19,683)
Other comprehensive income:				
(Loss) gain on short-term investments	(1)	(1)	-	4
Total other comprehensive income:	(1)	(1)	-	4
Comprehensive loss	$ (10,863)	$ (6,515)	$ (28,322)	$ (19,679)

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

Catabasis Pharmaceuticals, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(In thousands, except shares)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Common stock, shares				
Balance, beginning of period	18,823,601	11,553,937	12,433,600	7,141,996
Issuance of common stock in at-the-market offerings	1,253,736	161,349	2,353,737	564,590
Issuance of common stock and warrants in public offerings	-	-	5,290,000	4,000,000
Issuance of common stock upon exercise of common stock warrants	-	-	-	8,700
Balance, end of period	20,077,337	11,715,286	20,077,337	11,715,286
Common stock, par value				
Balance, beginning of period	$ 19	$ 12	$ 12	$ 7
Issuance of common stock in at-the-market offerings	1	-	3	1
Issuance of common stock and warrants in public offerings	-	-	5	4
Balance, end of period	$ 20	$ 12	$ 20	$ 12
Additional paid-in capital				
Balance, beginning of period	$ 291,897	$ 253,821	$ 259,305	$ 232,243
Issuance of common stock in at-the-market offerings	8,938	1,049	16,267	3,223
Issuance of common stock and warrants in public offerings	-	-	24,554	18,501
Issuance of common stock upon exercise of common stock warrants	-	-	-	54
Stock-based compensation expense	375	378	1,084	1,227
Balance, end of period	$ 301,210	$ 255,248	$ 301,210	$ 255,248
Accumulated deficit				
Balance, beginning of period	$ (241,057)	$ (210,473)	$ (223,597)	$ (197,304)
Net loss	(10,862)	(6,514)	(28,322)	(19,683)
Balance, end of period	$ (251,919)	$ (216,987)	$ (251,919)	$ (216,987)
Accumulated other comprehensive loss				
Balance, beginning of period	$ 1	$ 1	$ -	$ (4)
Realized (loss) gain on short-term investments	(1)	(1)	-	4
Balance, end of period	$ -	$ -	$ -	$ -
Total stockholders' equity	$ 49,311	$ 38,273	$ 49,311	$ 38,273

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

Catabasis Pharmaceuticals, Inc.
Condensed Consolidated Statements of Cash Flows
(In thousands)

(Unaudited)

	Nine Months Ended September 30,	
	2020	**2019**
Operating activities		
Net loss	$ (28,322)	$ (19,683)
Reconciliation of net loss to net cash used in operating activities:		
Non-cash items	1,127	1,277
Changes in assets and liabilities:		
Prepaid expenses and other current assets	36	(671)
Other assets	85	(25)
Right-of-use asset- operating	125	(36)
Accounts payable	211	682
Accrued expenses	2,314	(287)
Other liabilities	-	(56)
Net cash used in operating activities	(24,424)	(18,799)
Investing activities		
Purchases of short-term investments	(42,777)	(123,355)
Sales and maturities of short-term investments	69,110	122,777
Purchases of property and equipment	(23)	-
Net cash provided by (used in) investing activities	26,310	(578)
Financing activities		
Proceeds from public offerings, net of issuance costs	24,559	18,505
Proceeds from at-the-market offering, net of issuance costs	16,270	3,289
Proceeds from exercise of common stock warrants	-	54
Net cash provided by financing activities	40,829	21,848
Net increase in cash, cash equivalents and restricted cash	42,715	2,471
Cash, cash equivalents and restricted cash, beginning of period	10,376	15,407
Cash, cash equivalents and restricted cash, end of period	$ 53,091	$ 17,878
Non-cash financing activities:		
At-the-market offering issuance costs included in current liabilities	$ -	$ 65

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

1. Organization and Operations

The Company

Catabasis Pharmaceuticals, Inc (the "Company") is a biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics. On October 26, 2020, the Company announced that the Phase 3 PolarisDMD trial of the Company's lead product candidate, edasalonexent, for the treatment of Duchenne muscular dystrophy (DMD) did not meet the primary and secondary endpoints of the trial. Based on these results, the Company announced that it was stopping activities related to the development of edasalonexent, including the Company's ongoing open-label extension trial, and that it plans to work with external advisors to explore and evaluate strategic options. The Company was incorporated in the State of Delaware on June 26, 2008.

Liquidity

The Company has entered into various sales agreements with Cowen and Company LLC ("Cowen"), pursuant to which the Company could issue and sell shares of common stock, par value of $0.001 per share, under at-the-market offering programs (the "ATM Programs"). The Company pays Cowen 3% of the gross proceeds from any common stock sold through these sales agreements. As of September 30, 2020, the Company has $27.9 million remaining available under its current sales agreement.

During the nine months ended September 30, 2020, the Company sold an aggregate of 2,353,737 shares of common stock pursuant to the ATM Program at an average price of $7.13 per share, for gross proceeds of $16.8 million and net proceeds of $16.3 million after deducting sales commissions and offering expenses.

On January 30, 2020, the Company entered into an underwriting agreement with Oppenheimer & Co. Inc. relating to an underwritten public offering (the "January 2020 Financing") of 5,290,000 shares of common stock at a price to the public of $5.00 per share, including 690,000 shares issued upon the exercise in full by Oppenheimer & Co. Inc. of its overallotment option. This resulted in gross proceeds of $26.5 million, and net proceeds of $24.6 million.

As of September 30, 2020, the Company had an accumulated deficit of $251.9 million. The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since its inception.

The Company is subject to a number of risks, including, but not limited to, the ability to successfully execute on its exploration and evaluation of strategic options, the successful discovery and development of any future drug candidates that the Company may pursue, raising additional capital, development by potential competitors of new technological innovations, protection of proprietary technology, regulatory approval and market acceptance of any products the Company may develop, and the COVID-19 pandemic. The Company anticipates that it will continue to incur significant operating losses as it explores and evaluates strategic options.

As of September 30, 2020, the Company had available cash and cash equivalents of $52.9 million. Based on the Company's current operating plan, the Company believes it has sufficient cash and cash equivalents to fund operations for at least twelve months following the issuance of these condensed consolidated financial statements.

The Company has not generated any product revenues and has financed its operations primarily through public offerings and private placements of its equity securities. There can be no assurance that the Company will be able to obtain additional debt, equity or other financing or generate product revenue or revenues from collaborative partners, on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on the Company's business, results of operations, and financial condition.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying financial statements and the related disclosures are unaudited and have been prepared in accordance with United States generally accepted accounting principles ("U.S. GAAP"). Additionally, certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted from this report. Accordingly, these condensed financial statements should be read in conjunction with the financial statements as of and for the year ended December 31, 2019 and notes thereto included in the 2019 Annual Report on Form 10-K.

The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited financial statements. In the opinion of the Company's management, the accompanying unaudited interim condensed consolidated financial statements contain all adjustments, including those adjustments that are of a normal and recurring nature, which are necessary to fairly present the Company's results for the interim periods presented. The results for the three and nine months ended September 30, 2020 are not necessarily indicative of the results for the year ending December 31, 2020, or for any future period.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary, Catabasis Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the Company's condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the condensed consolidated financial statements and accompanying notes. Actual results could differ from such estimates.

The Company utilizes certain estimates to record expenses relating to research and development contracts. These contract estimates, which are primarily related to the length of service of each contract and the amount of service provided as of each measurement date, are determined by the Company based on input from internal project management, as well as from the Company's service providers.

Stock-Based Compensation

During the three and nine months ended September 30, 2020 and 2019, the Company recorded stock-based compensation expense, which was allocated as follows in the condensed consolidated statements of operations (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 154	$ 162	$ 491	$ 462
General and administrative	221	216	593	765
Total	$ 375	$ 378	$ 1,084	$ 1,227

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the Company's dilutive net loss per share calculation, stock options and warrants to purchase common stock were considered to be common stock equivalents but were excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive; therefore, basic and diluted net loss per share were the same for all periods presented.

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

	Three and Nine Months Ended September 30,	
	2020	2019
Stock options	1,336,262	843,183
Common stock warrants	6,193,749	6,193,749
	7,530,011	7,036,932

Cash, Cash Equivalents and Restricted Cash

The reconciliation of cash, cash equivalents and restricted cash reported within the applicable balance sheet that sum to the total of the same such amount shown in the statement of cash flows is as follows:

	September 30,	
	2020	**2019**
Cash and cash equivalents	$ 52,856	$ 17,765
Restricted cash (1)	235	113
Total	$ 53,091	$ 17,878

(1) Included in prepaid expenses and other current assets and other assets.

Recent Accounting Pronouncements - Adopted

In August 2018, the Financial Accounting Standards Board ("FASB") issued Accounting Standard Update ("ASU") 2018-13, *Fair Value Measurement (Topic 820)*. This standard includes amendments regarding changes in unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and disclosure requirements of measurement uncertainty. This amendment was effective for annual reporting periods beginning after December 15, 2019. Adoption of the standard did not have a material impact on the Company's consolidated financial statements.

Recent Accounting Pronouncements - Not Yet Adopted

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and adopted by the Company as of the specified effective date.

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments-Credit Losses* (Topic 326). This standard requires a financial asset to be presented at amortized cost basis at the net amount expected to be collected. It also requires that credit losses relating to available-for-sale debt securities should be recorded through an allowance for credit losses. In November 2019, the FASB issued an amendment making this ASU effective for annual reporting periods beginning after December 15, 2022 for smaller reporting companies., early adoption is permitted. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 2, "Summary of Significant Accounting Policies," in the 2019 Annual Report on Form 10-K, and there were no significant changes to such policies in the three and nine months ended September 30, 2020 that had a material impact on the Company's results of operations or financial position.

3. Financial Instruments

The tables below present information about the Company's assets and liabilities that are measured at fair value on a recurring basis as of September 30, 2020 and December 31, 2019 and indicate the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value. In general, fair values determined by Level 1 inputs utilize observable inputs such as quoted prices in active markets for identical assets or liabilities. Fair values determined by Level 2 inputs utilize data points that are either directly or indirectly observable, such as quoted prices, interest rates and yield curves. Fair values determined by Level 3 inputs utilize unobservable data points for which there is little or no market data, which require the Company to develop its own assumptions for the asset or liability. There were no transfers between fair value measurement levels during the nine months ended September 30, 2020 or 2019.

The Company's investment portfolio may include fixed income securities that do not always trade on a daily basis. As a result, the pricing services used by the Company apply other available information as applicable through processes such as benchmark yields, benchmarking of like securities, sector groupings and matrix pricing to prepare valuations. The Company validates the prices provided by its third party pricing services by obtaining market values from other pricing sources and analyzing pricing data in certain instances. The Company has from time to time invested in certain reverse repurchase agreements which are collateralized by deposits in the form of U.S. Government Securities and Obligations for an amount no less than 102% of their value. The Company has not recorded an asset or liability for the collateral as the Company was not permitted to sell or re-pledge the collateral. The collateral had at least the prevailing credit rating of U.S. Government Treasuries and Agencies. The Company utilized a third party custodian to manage the exchange of funds and ensure that collateral received is maintained at 102% of the value of the reverse repurchase agreements on a daily basis.

Below is a summary of assets measured at fair value on a recurring basis (in thousands):

	As of September 30, 2020			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market funds	$ 46,995	$ -	$ -	$ 46,995
Total assets	$ 46,995	$ -	$ -	$ 46,995

	As of December 31, 2019			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market funds	$ 5,432	$ -	$ -	$ 5,432
Corporate debt securities	-	1,937	-	1,937
Short-term investments:				
Commercial paper	-	1,993	-	1,993
Corporate debt securities	-	3,352	-	3,352
Reverse repurchase agreements	-	21,000	-	21,000
Total assets	$ 5,432	$ 28,282	$ -	$ 33,714

At September 30, 2020, and December 31, 2019, cash equivalents approximated their fair value due to their short-term nature.

4. Short-Term Investments

The Company did not hold any short-term investments at September 30, 2020. The following table summarizes the short-term investments held at December 31, 2019 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2019				
Commercial paper	$ 1,993	$ -	$ -	$ 1,993
Corporate debt securities	3,352	-	-	3,352
Reverse repurchase agreements	21,000	-	-	21,000
Total	$ 26,345	$ -	$ -	$ 26,345

The contractual maturities of all short-term investments held at December 31, 2019 were one year or less. There were four short-term investments in an unrealized loss position at December 31, 2019, none of which had been in an unrealized loss position for more than 12 months. The aggregate fair value of these investments was approximately $3.4 million. The Company did not hold any investments with other-than-temporary impairments at December 31, 2019.

Gross realized gains and losses on the sales of short-term investments are included in other income, net. Unrealized holding gains or losses for the period that have been included in accumulated other comprehensive income, as well as gains and losses reclassified out of accumulated other comprehensive income into other income, net were not material to the Company's condensed consolidated results of operations. The cost of investments sold or the amount reclassified out of the accumulated other comprehensive income into other income, net is based on the specific identification method for purposes of recording realized gains and losses. All proceeds in the three and nine-month periods ended September 30, 2020 and 2019 related to maturities of underlying investments. The gains on proceeds from maturities of short-term investments were not material to the Company's condensed consolidated results of operations for the three and nine months ended September 30, 2020 and 2019.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2020	December 31, 2019
Accrued contracted research costs	$ 2,456	$ 737
Accrued compensation	1,483	1,365
Accrued professional fees	555	370
Accrued other	430	138
Total	$ 4,924	$ 2,610

6. Commitments

Future minimum payments required under the Company's non-cancelable operating lease as of September 30, 2020 are summarized as follows (in thousands):

Period Ending December 31,	Amount
2020	$ 182
2021	740
2022	438
Total minimum lease payments	$ 1,360

Rent expense was $0.2 million and $0.1 million for the three months ended September 30, 2020 and 2019, respectively. Rent expense was $0.7 million and $0.2 million for the nine months ended September 30, 2020 and 2019, respectively. Lease payments were $0.2 million and $0.4 million for the three months ended September 30, 2020 and 2019, respectively. Lease payments were $1.3 million and $1.1 million for the nine months ended September 30, 2020 and 2019, respectively.

7. Stockholders' Equity

Preferred Stock

As of September 30, 2020, the Company had 5,000,000 shares of preferred stock authorized for issuance, $0.001 par value per share, none of which are issued or outstanding. Preferred stock may be issued from time to time in one or more series, each series to have such terms as stated or expressed in the resolutions providing for the issue of such series adopted by the board of directors of the Company. Preferred stock which may be redeemed, purchased or acquired by the Company may be reissued except as otherwise provided by law.

Common Stock Warrants

The following table presents information about warrants to purchase Common Stock issued and outstanding at September 30, 2020:

Year Issued	Warrants Outstanding	Exercise Price	Date of Expiration
2014	1,227	$ 122.12	8/26/2021
2015	1,227	$ 122.12	3/30/2022
2018	4,199,995	$ 12.00	6/21/2023
2019	1,991,300	$ 6.25	2/7/2024
Total	6,193,749		
Weighted average exercise price		$ 10.19	
Weighted average life in years			2.93

8. Common Stock Reserved for Future Issuance

The Company has reserved for future issuance the following shares of common stock:

	September 30, 2020	December 31, 2019
Warrants for the purchase of common stock	6,193,749	6,193,749
Options outstanding to purchase common stock	1,336,262	785,832
Options available for future issuance to purchase common stock	1,974,563	525,484
Shares reserved for the employee stock purchase plan	148,951	112,481
Total	9,653,525	7,617,546

9. Stock Incentive Plans

A summary of the Company's stock option activity and related information follows:

	Shares	Weighted-Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2019	785,832	$ 16.48	8.13	$ 470
Granted	583,500	$ 5.55		
Cancelled or forfeited	(32,589)	$ 26.59		
Expired	(481)	$ 32.10		
Outstanding at September 30, 2020	1,336,262	$ 11.46	8.31	$ 964
Vested and exercisable at September 30, 2020	482,969	$ 21.82	6.89	$ 227

There were no options exercised in the three or nine months ended September 30, 2020 and 2019. The total grant date fair value of options vested for the three months ended September 30, 2020 and 2019 was $0.3 million and $0.5 million, respectively. The total grant date fair value of options vested for the nine months ended September 30, 2020 and 2019 was $1.0 million and $1.3 million, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the three months ended September 30, 2020 and 2019 was $4.85 and $4.10, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the nine months ended September 30, 2020 and 2019 was $3.72 and $3.25, respectively.

At September 30, 2020, the total unrecognized compensation expense related to unvested stock option awards was $2.8 million. The Company expects to recognize that cost over a weighted-average period of approximately 2.7 years.

10. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence for certain estimates and to identify matters that require additional disclosure. Subsequent events have been evaluated as required.

On October 26, 2020, the Company announced that the Phase 3 PolarisDMD trial of the Company's lead product candidate, edasalonexent, for the treatment of DMD did not meet the primary and secondary endpoints of the trial. Based on these results, the Company announced that it was stopping activities related to the development of edasalonexent, including the Company's ongoing open-label extension trial, and that it plans to work with external advisors to explore and evaluate strategic options. The Company anticipates that it will incur less than $0.5 million of additional expenses related to the early termination of contracts.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and the related notes and other financial information included elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto included in our most recent Annual Report on Form 10-K. 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. You should review the "Risk Factors" section of this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to 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 biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics.

On October 26, 2020, we announced that the Phase 3 PolarisDMD trial of our lead product candidate, edasalonexent for the treatment of Duchenne muscular dystrophy, or DMD, did not meet the primary endpoint, which was a change from baseline in the North Star Ambulatory Assessment over one year of edasalonexent compared to placebo. In addition, we announced that the secondary endpoint timed function tests (time to stand, 10-meter walk/run and 4-stair climb) did not show statistically significant improvements. Based on these results, we stopped activities related to the development of edasalonexent, including the ongoing GalaxyDMD open-label extension trial. The Phase 3 Polaris DMD trial was a one-year placebo-controlled trial designed to evaluate the safety and efficacy of edasalonexent in boys ages 4-7 (up to 8th birthday) with DMD. The trial enrolled 131 boys across eight countries, with any mutation type, who were not on steroids. Edasalonexent was well-tolerated, consistent with the safety profile seen to date. The majority of adverse events were mild in nature and the most common treatment-related adverse events were diarrhea, vomiting, abdominal pain and rash. There were no treatment-related serious adverse events and no dose reductions. The global COVID-19 pandemic had no meaningful impact on the trial or its results. Data from the Phase 3 PolarisDMD trial will be further analyzed and are expected to be presented at an upcoming scientific conference and published.

In addition to edasalonexent, we have developed CAT-5571 and have completed investigational new drug, or IND, application-enabling activities. In August 2020, we announced that we entered into an agreement with the Bill & Melinda Gates Medical Research Institute to study CAT-5571 as a potential oral therapy to promote autophagy and clear persistent lung infections in patients with both drug-sensitive and drug-resistant tuberculosis.

In light of our decision to stop activities related to the development of edasalonexent, we plan to work with external advisors to explore and evaluate strategic options going forward, which may potentially result in changes to our business strategy and future operations. Potential strategic options that may be evaluated include a merger, business combination, in-licensing, out-licensing or other strategic transaction. On November 12, 2020, we announced that we engaged Ladenburg Thalmann & Co., Inc. as our strategic financial advisor. Pending any decision to change our strategic direction, our current operating plan provides for us to stop all activities related to the development of edasalonexent and to explore and evaluate strategic options. We do not have a defined timeline for the exploration and evaluation of strategic options and cannot confirm that the process will result in any strategic option being announced or consummated. We cannot provide any commitment regarding when or if this strategic evaluation process will result in any type of transaction, and there can be no assurance that such activities will result in any agreements or transactions that will enhance stockholder value. We do not intend to discuss or disclose further developments during this process unless and until our board of directors has approved a specific action or we otherwise determined that further disclosure is appropriate.

Since our inception in June 2008, we have devoted substantially all of our resources to developing our proprietary platform technology, identifying potential product candidates, undertaking preclinical studies and conducting clinical trials for three clinical-stage compounds, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, and providing general and administrative support for these operations. To date, we have primarily financed our operations through private placements of our preferred stock, registered offerings of our common stock, including our initial public offering, or IPO, as well as a secured debt financing. From our inception through September 30, 2020, we raised an aggregate of $315.9 million through various private placements of preferred stock, our IPO, debt financing as well as various other registered equity offerings, including underwritten public offerings, at-the-market, or ATM, offerings, and stock option and warrant exercises.

As of September 30, 2020, we had cash and cash equivalents of $52.9 million. Over the next several quarters, we expect that our research and development expenses will be significantly reduced due to stopping activities related to the development of edasalonexent. We expect to continue to incur costs associated with operating as a public company. Based on our current operating plan, we believe our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements for at least the next twelve months. However, because of the numerous uncertainties associated with our exploration and evaluation of strategic options and our future business strategies and operations, we are unable to predict our future levels of expenses with certainty and we could exhaust our available capital resources sooner than we expect. See "—Liquidity and Capital Resources."

We may need substantial additional funding to support our continuing operations and pursue any future business strategies arising from our exploration and evaluation of strategic options. In addition, we expect that we will not be able to achieve profitability unless we determine to pursue development of additional product candidates, and we expect that we would require substantial additional funding to complete development of, and ultimately commercialize, any such product candidate. Even if we are able to generate product sales from any such future product candidates, we may not become profitable. To the extent that we seek to raise additional capital, we expect that a majority of such proceeds would be derived from the sale of equity. We may be unable to raise additional funds when needed on favorable terms, or at all. If we fail to raise capital as, and when, needed, we may be unable to continue our operations at planned levels and be forced to modify our business strategies and reduce or terminate our operations.

Financial Overview

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including drug discovery efforts, and the development of any future product candidates, which include:

- employee-related expenses including salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with third parties, including contract research organizations that conduct clinical trials and research and development and preclinical activities on our behalf;
- the cost of consultants;
- the cost of acquiring, developing and manufacturing study materials; and
- facilities and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

Research and development costs are expensed as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

We typically use our employee, consultant and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, other internal costs or external consultant costs to specific product candidates or development programs. We record our research and development expenses net of any research and development tax incentives we are entitled to receive from government authorities.

The following table summarizes our research and development expenses by program (in thousands):

	Nine Months Ended September 30,	
	2020	**2019**
Edasalonexent	$ 13,765	$ 9,375
CAT-5571	10	11
Costs not directly allocated to programs:		
Employee expenses including cash compensation, benefits and stock-based compensation	4,421	3,740
Facilities	427	168
Consultants and professional expenses, including stock-based compensation	924	504
Other	298	256
Total costs not directly allocated to programs	6,070	4,668
Total research and development expenses	$ 19,845	$ 14,054

Since inception of the edasalonexent and the CAT-5571 programs, total direct expenses to support the programs have been $63.2 million and $4.2 million, respectively.

Based on the results of the Phase 3 PolarisDMD trial of edasalonexent for the treatment of DMD, we are stopping all activities related to the development of edasalonexent, including the ongoing GalaxyDMD open-label extension trial, and we are not actively pursuing the development of any product candidate at this time. We expect to continue to incur significant research and development expenses in connection with stopping activities related to the edasalonexent program, although we expect that our research and development expenses will be significantly reduced over the next several quarters. We plan to work with Ladenburg Thalmann & Co., Inc., our strategic financial advisor, to explore and evaluate strategic options going forward, which may potentially result in changes to our business strategy and future operations. Potential strategic options that may be evaluated include a merger, business combination, in-licensing, out-licensing or other strategic transaction. Our research and development expenses beyond 2020 will be heavily dependent on the outcome of our exploration and evaluation of strategic options. If we determine to pursue development of additional product candidates, the successful development of any such product candidates would be highly uncertain and we cannot reasonably estimate at this time the nature, timing and costs of the efforts that would be necessary to complete the development of any such product candidates. We are also unable to predict when, if ever, material net cash inflows would commence from any such potential product candidates. This is due to the fact that we would need to raise substantial additional capital to fund the clinical development of any such product candidates and the numerous risks and uncertainties associated with developing product candidates, including the uncertainties of:

- establishing an appropriate safety profile with IND-enabling toxicology studies;
- successful enrollment in, and completion of clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for any future product candidates;
- launching commercial sales of any future product candidates, if and when approved, whether alone or in collaboration with others; and
- a continued acceptable safety profile of any future product candidates following approval.

A change in the outcome of any of these variables with respect to the development of any future product candidates, or the occurrence of any of the development, commercialization, financial and other applicable risks set forth in Part II, Item 1A – Risk Factors, included elsewhere in this Quarterly Report on Form 10-Q, would significantly change the costs and timing associated with the development of that product candidate.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, commercial, business development, legal and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting and consulting services.

We expect our general and administrative expenses to remain relatively consistent through the remainder of 2020. Our general and administrative expenses beyond 2020 will be heavily dependent on the outcome of our exploration and evaluation of strategic options.

Other Income (Expense)

Other income (expense), net consists of interest income earned on our cash, cash equivalents, and short-term investments, foreign currency fluctuations, and net amortization expense on short-term investments.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with United States generally accepted accounting principles. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

During the nine months ended September 30, 2020, there were no material changes to our critical accounting policies as reported in our 2019 Annual Report on Form 10-K.

Results of Operations

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

The following table summarizes our results of operations for the three months ended September 30, 2020 and 2019, together with the dollar change in those items (in thousands):

	Three Months Ended September 30,		Period-to-Period Change
	2020	2019	
Operating expenses:			
Research and development	$ 7,806	$ 4,697	$ 3,109
General and administrative	3,057	1,985	1,072
Total operating expenses	10,863	6,682	4,181
Loss from operations	(10,863)	(6,682)	(4,181)
Other income, net	1	168	(167)
Net loss	$ (10,862)	$ (6,514)	$ (4,348)

Research and Development Expenses

Research and development expenses increased by $3.1 million to $7.8 million for the three months ended September 30, 2020 from $4.7 million for the three months ended September 30, 2019, an increase of 66%. The increase in research and development expenses was attributable to a $2.8 million increase in costs to support our edasalonexent program due to activities associated with conducting ongoing clinical trials, a $0.1 million increase in employee related expenses, a $0.2 million increase in other expenses such as consulting fees and the research and development portion of facilities.

General and Administrative Expenses

General and administrative expenses increased by $1.1 million to $3.1 million for the three months ended September 30, 2020 from $2.0 million for the three months ended September 30, 2019, an increase of 54%. The increase was attributable to a $0.6 million increase in consulting and other professional services associated with ongoing commercialization activities, a $0.3 million increase in employee related expenses, a $0.1 million increase in insurance expense, and a $0.1 million increase in the general and administrative portion of facilities.

Other Income, Net

Other income, net decreased by $167,000 to $1,000 for the three months ended September 30, 2020 from $168,000 for the three months ended September 30, 2019, which was attributable to a decrease in interest and investment income due to lower interest rates.

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

The following table summarizes our results of operations for the nine months ended September 30, 2020 and 2019, together with the dollar change in those items (in thousands):

	Nine Months Ended September 30,		Period-to-Period Change
	2020	2019	
Operating expenses:			
Research and development	$ 19,845	$ 14,054	$ 5,791
General and administrative	8,612	6,287	2,325
Total operating expenses	28,457	20,341	8,116
Loss from operations	(28,457)	(20,341)	(8,116)
Other income, net	135	658	(523)
Net loss	$ (28,322)	$ (19,683)	$ (8,639)

Research and Development Expenses

Research and development expenses increased by $5.8 million to $19.9 million for the nine months ended September 30, 2020 from $14.1 million for the nine months ended September 30, 2019, an increase of 41%. The increase in research and development expenses was attributable to a $4.4 million increase in costs to support our edasalonexent program due to activities associated with conducting ongoing clinical trials, a $0.7 million increase in employee related expenses, a $0.4 million increase in consulting fees and a $0.3 million increase in the research and development portion of facilities expense.

General and Administrative Expenses

General and administrative expenses increased by $2.3 million to $8.6 million for the nine months ended September 30, 2020 from $6.3 million for the nine months ended September 30, 2019, an increase of 37%. The increase was attributable to a $1.5 million increase in consulting and other professional services primarily related to ongoing commercialization activities, a $0.3 million increase in employee related expenses, $0.3 million increase in insurance and general office expenses, and a $0.2 million increase in the general and administrative portion of facilities expense.

Other Income, Net

Other income, net decreased by $0.5 million to $0.1 million for the nine months ended September 30, 2020 from $0.7 million for the nine months ended September 30, 2019. This was attributable to a $0.5 million decrease in interest and investment income due to lower interest rates.

Liquidity and Capital Resources

From our inception through September 30, 2020, we raised an aggregate of $315.9 million, through various private placements of preferred stock, our IPO, as well as various other registered equity offerings, including underwritten public offerings, ATM programs, and stock option and warrant exercises. As of September 30, 2020, we had $52.9 million in cash and cash equivalents.

January 2020 Financing

On January 30, 2020, we entered into an underwriting agreement with Oppenheimer & Co. Inc. relating to an underwritten public offering, or the January 2020 Financing, of 5,290,000 shares of common stock at a price to the public of $5.00 per share, including 690,000 shares issued upon the exercise in full by Oppenheimer & Co. Inc. of its overallotment option. This resulted in gross proceeds of $26.5 million, and net proceeds of $24.6 million.

At-the-Market Offering

During the nine months ended September 30, 2020, we sold an aggregate of 2,353,737 shares of common stock pursuant to our ATM programs, at a weighted average price of $7.13 per share, for gross proceeds of $16.8 million and net proceeds of $16.3 million.

Cash Flows

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

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

	Nine Months Ended September 30,	
	2020	**2019**
Net cash used in operating activities	$ (24,424)	$ (18,799)
Net cash provided by (used) in investing activities	26,310	(578)
Net cash provided by financing activities	40,829	21,848
Net increase in cash, cash equivalents and restricted cash	$ 42,715	$ 2,471

Net Cash Provided by (Used in) Operating Activities

Net cash used in operating activities was $24.4 million for the nine months ended September 30, 2020 and consisted primarily of a net loss of $28.3 million adjusted for non-cash items of $1.1 million and a net decrease in operating assets of $2.8 million, which resulted primarily from an increase in accrued expenses of $2.3 million, an increase in accounts payable of $0.2 million, and a decrease in prepaid expense, other assets and operating lease of $0.3 million.

Net cash used in operating activities was $18.8 million for the nine months ended September 30, 2019 and consisted primarily of a net loss of $19.7 million adjusted for non-cash items, including stock-based compensation and depreciation and amortization expense of $1.3 million and a net increase in operating assets of $0.4 million, which resulted primarily from an increase in prepaid expenses of $0.7 million, a decrease in accrued expenses of $0.3 million and an increase long-term other assets and the net operating leases of $0.1 million, partially offset by an increase in accounts payable of $0.7 million.

Net Cash Used in Investing Activities

Net cash provided by investing activities was $26.3 million for the nine months ended September 30, 2020 and consisted of proceeds from maturities of short-term investments of $69.1 million partially offset by purchases of short-term investments of $42.8 million. Net cash used in investing activities was $0.6 million for the nine months ended September 30, 2019 and consisted of purchases of short-term investments of $123.4 million substantially offset by proceeds from maturities of short-term investments of $122.8 million.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $40.8 million during the nine months ended September 30, 2020, which was primarily attributable to net proceeds of $24.6 million from the January 2020 Financing and net proceeds of $16.3 million from our ATM programs. Net cash provided by financing activities was $21.8 million during the nine months ended September 30, 2019, which was primarily attributable to net proceeds of $18.5 million from our February 2019 public offering and net proceeds of $3.3 million from our ATM programs.

Funding Requirements

Absent a change in our strategic plan, our current operating plan consists primarily of stopping activities related to our edasalonexent program and exploring and evaluating strategic options.

As of September 30, 2020 we had an accumulated deficit of $251.9 million. We have been primarily involved with research and development activities and have incurred operating losses and negative cash flows from operations since our inception. While we are stopping activities related to the edasalonexent program, we still expect to continue to incur significant expenses and operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We anticipate that we will continue to incur significant expenses and operating losses as we implement our review of strategic options and we may incur increased expenses if and to the extent we:

- are unable to stop activities related to the edasalonexent program on our anticipated timelines or we incur unexpected material expenses during this process;
- initiate and continue research and preclinical and clinical development efforts for any future product candidate;
- seek to identify and develop any future product candidate;
- seek regulatory and marketing approvals for any future product candidate that successfully completes clinical trials, in the United States and other markets;
- establish sales, marketing, market access, distribution, supply chain and other commercial infrastructure in the future to commercialize products for which we may obtain marketing approval, if any;
- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio;
- need to respond to any investigations or inquiries, or defend against any litigation, that may result from the announcement of the results of our Phase 3 PolarisDMD trial; and
- hire and retain additional personnel or add information systems, equipment or physical infrastructure to support our operations, including to help us comply with our obligations as a public company.

As of September 30, 2020, we had available cash and cash equivalents of $52.9 million. Based on our current operating plan, we believe our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements for at least the next twelve months. However, because of the numerous uncertainties associated with our ongoing exploration and evaluation of strategic options and our future business strategies and operations, we are unable to predict our future levels of expenses with certainty and we could exhaust our available capital resources sooner than we expect. Our future funding requirements will depend on many factors, including:

- our ability to successfully complete our exploration and evaluation of strategic options and implement any such options;
- our ability to eliminate all material edasalonexent-related expenses on the timelines that we anticipate;
- our ability to limit our ongoing operating costs as we work to explore and evaluate strategic options;
- the progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, any future product candidates, including potential future clinical trials;
- the impact of the COVID-19 pandemic on our operations, business and prospects;
- our ability to enter into and the terms and timing of any additional collaborations, licensing or other arrangements that we may establish;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking regulatory approvals for any future product candidates;
- the costs of commercialization activities for any future product candidates for which we pursue marketing approval to the extent such costs are not the responsibility of any future collaborators;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of any future product candidate;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the costs of responding to any investigations or inquiries, or defending against any litigation, that may result from the announcement of the results of our Phase 3 PolarisDMD trial; and
- the costs of operating as a public company, including additional costs that we would incur if we cease to be an emerging growth company or smaller reporting company, as defined in U.S. Securities and Exchange Commission regulations.

We may need substantial additional funding to support our continuing operations and pursue any future business strategies arising from our exploration and evaluation of strategic options. In addition, we expect that we will not be able to achieve profitability unless we determine to pursue development of additional product candidates, and we expect that we would require substantial additional funding to complete development of, and ultimately commercialize, any such product candidate. 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 marketing approval and achieve product sales. In addition, any future product candidates, if approved, may not achieve commercial success.

We do not have any committed external source of funds. To the extent that we seek to raise additional capital, we expect that a majority of such proceeds would be derived from the sale of equity, which may result in the dilution of our existing stockholders' ownership interests, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise funds through collaborations, strategic alliances 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 to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity, debt or other financings when needed, we may be unable to continue our operations at planned levels and be forced to modify our business strategies and reduce or terminate our operations.

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 under applicable Securities and Exchange Commission rules.

Contractual Obligations

As of September 30, 2020, there had been no material changes to our contractual obligations and commitments disclosed under Management's Discussion and Analysis of Financial Condition and Results of Operations in the 2019 Annual Report on Form 10-K.

Item 3. Qualitative and Quantitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of September 30, 2020, we had cash and cash equivalents of $52.9 million and, as of December 31, 2019, we had cash, cash equivalents and short-term investments of $36.2 million. Our cash equivalents as of September 30, 2020 consisted of money market funds. Our cash equivalents as of December 31, 2019 consisted of money market funds and corporate debt securities. Our short-term investments as of December 31, 2019 consisted of corporate debt securities, U.S. reverse repurchase agreements and commercial paper. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio and interest income.

As of September 30, 2020 and December 31, 2019, we had no material liabilities denominated in foreign currencies.

Item 4. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Securities Exchange Act of 1934, or the Exchange Act) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized, and reported within the time periods specified in the U.S. Securities and Exchange Commission's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this Quarterly Report on Form 10-Q. 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 management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2020, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

Changes in Internal Control over Financial Reporting.

During the three months ended September 30, 2020, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1A. Risk Factors

We operate in a dynamic and rapidly changing business environment that involves risks and substantial uncertainty. The following discussion addresses risks and uncertainties that could cause, or contribute to causing, actual results to differ from expectations in material ways. In evaluating our business, investors should pay particular attention to the risks and uncertainties described below and in other sections of this Quarterly Report on Form 10-Q and in our subsequent filings with the Securities and Exchange Commission, or SEC. These risks and uncertainties, or other events that we do not currently anticipate or that we currently deem immaterial also may affect our results of operations, cash flows and financial condition. The trading price of our common stock could also decline due to any of these risks, and you could lose all or part of your investment.

Our business has been almost entirely dependent on the success of edasalonexent as a potential treatment for DMD, a program for which we recently stopped all activities on due to the failure to meet the primary or secondary endpoints of the Phase 3 PolarisDMD trial.

On October 26, 2020, we announced that we were stopping all activities related to the edasalonexent program and that we plan to work with external advisors to explore and evaluate strategic options. Potential strategic options that may be evaluated include a merger, business combination, in-licensing, out-licensing or other strategic transaction We have engaged Ladenburg Thalmann & Co., Inc., a strategic financial advisor, to support our exploration and evaluation of strategic options that could maximize both near and long-term value for our stockholders. We do not have a defined timeline for the exploration and evaluation of strategic options and cannot confirm that the process will result in any strategic option being announced or consummated. We cannot provide any commitment regarding when or if this strategic evaluation process will result in any type of transaction, and there can be no assurance that such activities will result in any agreements or transactions that will enhance stockholder value. If we determine to engage in a transaction as a result of our exploration and evaluation of strategic options, our future business, prospects, financial position and operating results could be significantly different than those in historical periods or projected by our management. Because of the significant uncertainty regarding our future plans, we are not able to accurately predict the impact of a potential change in our existing business strategy. We do not intend to discuss or disclose further developments during this process unless and until our board of directors has approved a specific action or we otherwise determined that further disclosure is appropriate.

Pending the results of our exploration and evaluation of strategic options, our current operating plan provides for stopping all activities related to the edasalonexent program and focusing on activities necessary to explore and evaluate strategic options.

Risks Related to Our Financial Position and Need for Additional Capital

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

Our net losses were $28.3 million for the nine months ended September 30, 2020 and $26.3 million and $25.9 million for the years ended December 31, 2019 and 2018, respectively. As of September 30, 2020, we had an accumulated deficit of $251.9 million. We have not generated any revenues from product sales, have not completed the development of any product candidate and may never have a product candidate approved for commercialization. We have financed our operations to date primarily through private placements of our preferred stock, registered offerings of our common stock, including our initial public offering, or IPO, our June 2018 and February 2019 registered offerings of common stock and common stock warrants and our January 2020 registered offering of common stock, our at-the-market programs, and a secured debt financing, and have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical development programs. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

Since 2018, we have devoted a significant portion of our financial resources and efforts to the development of edasalonexent as a potential treatment for DMD. On October 26, 2020, based on the results of the Phase 3 PolarisDMD trial, we announced that we were stopping all activities related to the edasalonexent program and that we plan to work with external advisors to explore and evaluate strategic options.

While we are stopping activities related to the edasalonexent program, we still expect to continue to incur significant expenses and operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We anticipate that we will continue to incur significant expenses and operating losses as we implement our exploration and evaluation of strategic options and we may incur increased expenses if and to the extent we:

- are unable to stop activities related to the edasalonexent program on our anticipated timelines or we incur unexpected material expenses during this process;
- initiate and continue research and preclinical and clinical development efforts for any future product candidate;
- seek to identify and develop any future product candidate;
- seek regulatory and marketing approvals for any future product candidate that successfully completes clinical trials, in the U.S. and other markets;
- establish sales, marketing, market access, distribution, supply chain and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any;
- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio;
- need to respond to any investigations or inquiries, or defend against any litigation, that may result from the announcement of the results of our Phase 3 PolarisDMD trial; and
- hire and retain additional personnel or add information systems, equipment or physical infrastructure to support our operations, including to help us comply with our obligations as a public company.

To become and remain profitable, we or any potential future collaborators must develop and eventually commercialize at least one product candidate with significant market potential. This will require that we or our collaborators be successful in a range of challenging activities, including completing preclinical studies and clinical trials of one or more product candidates, obtaining marketing approval for one or more these product candidates, manufacturing, marketing and selling those products for which we or our collaborators may obtain marketing approval and satisfying any post-marketing requirements. We or our collaborators may never succeed in any or all of these activities and, even if we or our collaborators do succeed, we or our collaborators may never generate revenue that is significant or large enough to achieve profitability. 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 decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause investors to lose all or part of their investments in us.

If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our operations and may not achieve our strategic objectives.

Absent a change in our strategic plan, our current operating plan consists primarily of stopping activities related to our edasalonexent program and exploring and evaluating strategic options. Based on this operating plan, we believe our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements for at least the next twelve months. Our estimate as to how long we expect our cash and cash equivalents securities to be able to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements will depend on many factors, including:

- our ability to successfully complete our exploration and evaluation of strategic options and implement any such options;
- our ability to eliminate all material edasalonexent-related expenses on the timelines that we anticipate;
- our ability to limit our ongoing operating costs as we work to explore and evaluate strategic options;
- the progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, any future product candidates, including potential future clinical trials;
- the impact of the COVID-19 pandemic on our operations, business and prospects;
- our ability to enter into and the terms and timing of any additional collaborations, licensing or other arrangements that we may establish;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking regulatory approvals for any future product candidates;
- the costs of commercialization activities for any future product candidates for which we pursue marketing approval to the extent such costs are not the responsibility of any future collaborators;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of any future product candidate;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;

- the costs of responding to any investigations or inquiries, or defending against any litigation, that may result from the announcement of the results of our Phase 3 PolarisDMD trial; and
- the costs of operating as a public company, including additional costs that we would incur if we cease to be an emerging growth company or smaller reporting company, as defined in U.S. Securities and Exchange Commission regulations.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. Accordingly, we would need to obtain additional funding if, in the future we initiate new research, preclinical and clinical development efforts for and seek marketing approval for, any future product candidate, and would expect our expenses to increase in connection with each of these activities. If we obtain marketing approval for any future product candidate, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future partner or collaborator, and these activities would require substantial additional funding. Furthermore, we have incurred and will continue to incur significant additional costs associated with operating as a public company.

Adequate additional funding may not be available to us on acceptable terms, on a timely basis or at all. Our failure to raise capital on acceptable terms as and when needed could have a material adverse effect on our business, results of operations, financial condition and ability to pursue our business strategy.

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

We may seek to raise additional capital to acquire, develop and commercialize future product candidates or to pursue other strategic options. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, our existing stockholders' ownership interest may be substantially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. For example, our June 2018 and February 2019 registered offerings of common stock and common stock warrants and our January 2020 registered offering of common stock were highly dilutive to existing stockholders' ownership interests. Further, exercise of the common stock warrants sold in our June 2018 and February 2019 offerings could result in additional dilution upon exercise. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing additional financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of any future product candidate.

If we raise additional funds through collaborations or marketing, distribution, licensing or royalty arrangements with third parties, 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 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.

We have a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We began operations in 2008. Our operations to date have been limited to financing and staffing our company and developing our technology and conducting preclinical research and clinical trials for our product candidates. We have not yet demonstrated an ability to successfully conduct pivotal clinical trials, obtain marketing 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. Accordingly, our investors should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early stages of development, especially clinical-stage biopharmaceutical companies such as ours. Predictions 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 pharmaceutical products.

Risks Related to the Discovery, Development and Commercialization of Our Product Candidates

Our approach to the discovery and development of product candidates has been based on our SMART Linker drug discovery platform, which is unproven, and we do not know whether we will be able to develop any products of commercial value.

We have been focused on discovering and developing novel small molecule drugs by applying our SMART Linker drug discovery platform. We have not yet succeeded and may never succeed in demonstrating efficacy and safety for any of our product candidates in a Phase 3 clinical trial or in obtaining marketing approval thereafter. For example, although we have discovered and evaluated numerous compounds using our SMART Linker drug discovery platform, no product created using the SMART Linker drug discovery platform has ever been approved for sale. Moreover, we have recently announced we are stopping all activities for edasalonexent, our most advance SMART Linker product candidate, due to the failure to meet the primary or secondary endpoints of the Phase 3 PolarisDMD trial.

We do not have a product candidate that we are actively developing. If we determine to continue operating our business based on our SMART Linker drug discovery platform or otherwise, and we are unable to identify a product candidate to advance through research and development efforts, our business would be materially harmed.

As a result of stopping all activities related to our edasalonexent program, we do not have any product candidate in clinical development. If, following our exploration and evaluation of strategic options, we determine to continue operating our business based on our SMART Linker drug discovery platform or otherwise, we will need to evaluate such product candidates, or any other product candidates that we may be able to acquire, to determine which, if any, are suitable for moving into further preclinical or clinical development. If we determine that our platform cannot generate any product candidates that appear to be suitable for development, or we are unable to acquire any product candidates that appear to be suitable for development, our business would be materially harmed.

Our SMART Linker drug discovery platform may fail to help us discover and develop additional potential product candidates.

A significant portion of the research that we have conducted to date and may in the future conduct, involves the development of new compounds using our SMART Linker drug discovery platform. We suspended efforts to discover additional compounds while we completed our Phase 3 PolarisDMD clinical trial, and any new drug discovery that we are conduct using our SMART Linker drug discovery platform may not be successful in creating compounds that have commercial value or therapeutic utility. Our SMART Linker drug discovery platform may initially show promise in identifying potential product candidates, yet fail to yield viable product candidates for clinical development or commercialization for a number of reasons, including:

- compounds created through our SMART Linker drug discovery platform may not demonstrate improved efficacy, safety or tolerability;
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;
- competitors may develop alternative therapies that render our potential product candidates non-competitive or less attractive; or
- a potential product candidate may not be capable of being produced at an acceptable cost.

Any future research program to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, either because our SMART Linker platform is not successful or because we do not develop alternative methods to identify compounds for development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

The COVID-19 pandemic has had, and continues to have, significant impacts worldwide, and may delay the initiation of future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this ongoing pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, both of which could result in adverse effects on our business and operations and ability to raise capital.

The COVID-19 pandemic has had, and continues to have, significant impacts worldwide causing 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, including widespread emergency orders requiring business and residents to curtail non-essential activities, 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. If we seek to pursue the development of additional product candidates, the COVID-19 pandemic could delay the initiation of future clinical trials, disrupt regulatory activities or have other adverse effects on our business or operations. The future progression and unpredictability of the pandemic and its effects on our business and operations are highly uncertain and will depend on future developments that cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the duration of the outbreak, additional surges in the number of cases or deaths from COVID-19, travel restrictions and actions to contain the outbreak or treat its impact, such as social distancing and quarantines or lock-downs in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease.

The pandemic has also caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to evaluate and pursue any strategic options that we elect to explore, 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 continue to 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.

We have never obtained marketing approval for a product candidate and we may be unable to obtain, or may be delayed in obtaining, marketing approval for any future product candidate we may seek to develop.

We have never obtained marketing approval for a product candidate. If we determine to develop a product candidate in the future, it is possible that the FDA may refuse to accept for substantive review any NDAs that we submit for such product candidates or may conclude after review of our data that our applications are insufficient to obtain marketing approval of such product candidate. If the FDA does not accept or approve any NDAs we submit, it may require that we conduct additional clinical, nonclinical or manufacturing validation studies and submit that data before it will reconsider our applications. Depending on the extent of these or any other FDA-required studies, approval of any NDA or application that we submit may be delayed by several years or may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA to approve our NDAs.

Any delay in obtaining, or an inability to obtain, marketing approvals would prevent us from commercializing any future product candidate, generating revenues and achieving and sustaining profitability. If any of these outcomes occur, we may be forced to abandon our development efforts for any future product candidates, which could significantly harm our business.

Results of preclinical studies and early clinical trials may not be predictive of results of future clinical trials.

The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of clinical trials do not necessarily predict success in future clinical trials. Many companies, including us, in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face similar setbacks. The design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. Even if we, or any future collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of any future product candidate, the development timeline and regulatory approval and commercialization prospects for such product candidate, and, correspondingly, our business and financial prospects would be negatively impacted.

The regulatory approval processes for product candidates that target rare diseases are uncertain.

Due to the lack of precedent, broad discretion of regulatory authorities, and a multitude of unique factors that impact the regulatory approval process, the likelihood of the approval of any product candidate that targets rare diseases, which we have historically targeted, is uncertain, and we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned investigational new drug applications and NDAs for any such product candidates, in a timely manner, or at all. Further, the FDA may determine, after evaluation of our data and analyses, that such data and analyses do not support an NDA submission, filing or approval. Due to this lack of predictability, we may not have the resources necessary to meet regulatory requirements and successfully complete a potentially protracted, expensive and wide-ranging approval process for commercialization of product candidates for rare diseases.

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.

We have limited financial and managerial resources and have expended significant resources on the development of our edasalonexent program, which has proven to be unsuccessful. Our current priorities are to stop activities related to our edasalonexent program and to explore and evaluate strategic options. If we determine to develop additional product candidates based on our SMART Linker drug discovery platform, or if we acquire other product candidates, we would seek to develop them for specific indications that we identify as most likely to succeed, in terms of both their potential for marketing approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may 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 future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. 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 the product candidate.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials we initiate will be conducted as planned or completed on schedule, or at all. Further, the clinical development of product candidates is susceptible to the risk of failure at any stage of drug development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. It is possible that even if a product candidate that we choose to develop has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in any clinical trials we conduct, we may fail to detect toxicity of or intolerability caused by a product candidate, or mistakenly believe that a product candidate is toxic or not well tolerated when that is not in fact the case.

In addition to the risk of failure inherent in drug development, any compounds that we may develop in the future using our SMART Linker drug discovery platform may be particularly susceptible to failure to the extent they are based on compounds that others have previously studied or tested, but did not progress in development due to safety, tolerability or efficacy concerns or otherwise. Our failure to successfully complete clinical trials of a product candidate and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of a product candidate would significantly harm our business.

If clinical trials of a product candidate fail to satisfactorily demonstrate safety and efficacy to the FDA and other comparable foreign regulators, we, or any future collaborators, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Comparable foreign regulatory authorities, such as the European Medicines Agency, or the EMA, impose similar restrictions. We, and any future collaborators, may never receive such approvals. We, and any future collaborators, must complete extensive preclinical development and clinical trials to demonstrate the safety and efficacy in humans of any product candidate that we may choose to develop before we, or they, will be able to obtain these approvals.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. Any inability to complete preclinical and clinical development successfully could result in additional costs to us, or any future collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if (1) we, or any future collaborators, are required to modify our trial designs, such as required modifications with respect to patient populations, endpoints, comparators or trial duration, (2) we, or any future collaborators, are required to conduct additional clinical trials or other testing of a product candidate beyond the trials and testing that we, or they contemplate, (3) we, or any future collaborators, are unable to successfully complete clinical trials of a product candidate or other testing, (4) the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or (5) there are unacceptable safety concerns associated with a product candidate, we, or any future collaborators, may:

- be delayed in obtaining marketing approval for such product candidate;
- 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 that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

Adverse events or undesirable side effects caused by, or other unexpected properties of, any of any future product candidate may be identified during development that could delay or prevent their marketing approval or limit their use.

Adverse events or undesirable side effects caused by, or other unexpected properties of, any future product candidate could cause us, any future collaborators, an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of one or more of such product candidate and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. If any such product candidate is associated with adverse events or undesirable side effects or has properties that are unexpected, we, or any future collaborators, may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound.

If we, or any future collaborators, experience any of a number of possible unforeseen events in connection with clinical trials of any future product candidate, potential marketing approval or commercialization of such product candidate could be delayed or prevented.

We, or any future collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent marketing approval or commercialization of any future product candidate, including:

- clinical trials may produce unfavorable or inconclusive results;
- we, or any future collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials may be larger than we, or any future collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or any future collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or any future collaborators, anticipate;
- the cost of planned clinical trials may be greater than we anticipate;
- our third-party contractors or those of any future collaborators, including those manufacturing such product candidate or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of any future collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or any future collaborators in a timely manner or at all;
- regulators or institutional review boards may not authorize us, any future collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we, or any future collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial’s duration;
- we, or any future collaborators, may have to delay, suspend or terminate clinical trials for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate, or travel bans or other restrictions imposed by applicable governmental authorities due to the ongoing COVID-19 pandemic;

- regulators or institutional review boards may require that we, or any future collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar drug or drug candidate;
- the FDA or comparable foreign regulatory authorities may disagree with our, or any future collaborators', clinical trial designs or our or their interpretation of data from preclinical studies and clinical trials;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we, or any future collaborators, enter into agreements for clinical and commercial supplies;
- the supply or quality of raw materials or manufactured product candidates or other materials necessary to conduct clinical trials may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply, due to, among other things, the ongoing COVID-19 pandemic; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

In addition, we may conduct clinical trials outside of the United States. Unforeseen global instability, including political instability, or instability from an outbreak of pandemic or contagious disease, such as the ongoing COVID-19 pandemic, in or around any countries in which we conduct clinical trials, could affect our ability to enroll patients in clinical trials in these countries, prevent patients already enrolled from completing such clinical trials, and/or cause other trial delays or otherwise adversely impact such clinical trials.

Product development costs for us, or any future collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of any future product candidate. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we, or any future collaborators, may have the exclusive right to commercialize product candidates or allow our competitors, or the competitors of any future collaborators, to bring products to market before we, or any future collaborators, do and impair our ability, or the ability of any future collaborators, to successfully commercialize product candidates and may harm our business and results of operations. In addition, many of the factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any future product candidates.

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

We, or any future collaborators, may not be able to initiate or continue clinical trials for any product candidate if we, or they, are unable to locate and enroll, and maintain the enrollment of, a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities, such as the EMA. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors, including:

- the size and nature of the patient population;
- the severity of the disease under investigation;
- the proximity of patients to clinical sites;
- the eligibility criteria for the trial;
- the design of the clinical trial;
- efforts to facilitate timely enrollment;
- competing clinical trials; and
- clinicians' and patients' perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

Our ability to successfully complete any future clinical trial for any product candidate for the treatment of any rare disease or any other indication will be dependent upon our ability to enroll, and maintain the enrollment of, a sufficient number of patients with such disease, which will be subject to a number of risks and uncertainties. For example, rare diseases have small patient populations and often have only a limited number of specialist physicians that regularly treat such patients. Further, these specialized sites typically treat a range of diseases and, at any point in time, may have constrained resources and capacity to handle clinical trials. In addition, other companies may be conducting clinical trials or may have announced plans for future clinical trials that are seeking, or are likely to seek, to enroll patients with the disease and patients are generally only able to enroll in a single trial at a time. The small population of patients, competition for these patients and the limited trial sites and their constrained resources may make it difficult for us to enroll enough patients, and to maintain the enrollment of enough patients, to complete clinical trials for any such future product candidate.

The clinical trials that we may conduct may also have inclusion criteria that further limit the population of patients that we are able to enroll. These inclusion criteria could further limit the available patient pool and present challenges to clinical trial enrollment.

Our inability, or the inability of any future collaborators, to enroll a sufficient number of patients for any clinical trials that we or they may determine to pursue could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in any such clinical trials may result in increased development costs for the applicable product candidates, delay or halt the development of and approval processes for any future product candidates and jeopardize our, or any future collaborators', ability to commence sales of and generate revenues from any future product candidates, which could cause the value of our company to decline.

Business disruptions could delay completion of future clinical trials, seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations, and those of third-party research institution collaborators, contract research organizations, contract manufacturing operations, and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics such as the ongoing COVID-19 pandemic, and other natural or man-made disasters or business interruptions, for which we may be partly uninsured. In addition, to the extent we determine to pursue development of future product candidates, we expect that we will rely on third-party research institution collaborators for conducting research and development of such product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could delay completion of any clinical trials for such product candidates, seriously harm our operations and financial condition and increase our costs and expenses.

If any future product candidate 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, or that of any future collaborators, to market the drug could be compromised.

Clinical trials of product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that clinical trials for any future product candidate, or those of any future collaborator, may indicate an apparent positive effect of such product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the drug or seize the drug;
- we, or any future collaborators, may be required to recall the drug, change the way the drug is administered or conduct additional clinical trials;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular drug;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a "black box" warning or a contraindication;
- we, or any future collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or any future collaborators, could be sued and held liable for harm caused to patients;
- we may become the subject of government investigations, which would be expensive to manage and potentially result in the imposition of fines, injunctions or the imposition of civil or criminal penalties;
- the drug may become less competitive; and
- our reputation may suffer.

Any of these events could have a material and adverse effect on our operations and business and could adversely impact our stock price.

Even if a product candidate 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 the product candidate may be smaller than we estimate.

We have never commercialized a product. Even if any future product candidate of ours is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. For example, physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies.

Efforts to educate the medical community and third-party payors on the benefits of future product candidates may require significant resources and may not be successful. If any of future product candidate of ours is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of any future product candidates of ours, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- whether the product is designated under physician treatment guidelines as a first-line therapy or as a second- or third-line therapy and whether there is an existing standard of care;
- limitations or warnings, including distribution or use restrictions, contained in the product's approved labeling;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the strength of sales, marketing, market access and distribution support;
- the approval of other new products for the same indications;
- changes in the standard of care for the targeted indications for the product;
- the timing of market introduction of our approved products as well as competitive products;
- availability and amount of reimbursement from government payors, managed care plans and other third-party payors;
- adverse publicity about the product or favorable publicity about competitive products; and
- potential product liability claims.

The potential market opportunities for product candidates are difficult to estimate precisely. Any estimates we make as to the potential market opportunities for any future product candidates will be predicated on many assumptions, including industry knowledge and publications, third-party research reports and other surveys. These assumptions will involve the exercise of significant judgment on the part of our management, will be inherently uncertain and the reasonableness of these assumptions may not have been assessed by an independent source. If any such assumptions prove to be inaccurate, the actual markets for any such future product candidate could be smaller than our estimates of the potential market opportunities.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing any future product candidates that we may develop if and when those product candidates are approved.

We currently do not have a formal sales, marketing or distribution infrastructure and have limited experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we would need to either develop a sales and marketing organization or outsource these functions to third parties. We would plan to use a combination of focused in-house sales and marketing capabilities and third-party collaboration, licensing and distribution arrangements to sell any products that receive marketing approval.

We generally expect that we would seek to retain full commercialization rights for products that we can commercialize with a specialized sales force and to retain co-promotion or similar rights when feasible in indications requiring a larger commercial infrastructure. The development of sales, marketing and distribution capabilities will require substantial resources, will be time-consuming and could delay any product launch. If the commercial launch of a product for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we could have prematurely or unnecessarily incurred these commercialization costs. This may be costly, and our investment could be lost if we cannot retain or reposition our sales and marketing personnel. In addition, we may not be able to hire or retain a sales force that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, at such time as we need to, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to a product, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently.

We may collaborate with third parties for commercialization of any products that require a large sales, marketing and product distribution infrastructure. We intend to potentially commercialize product candidates through collaboration, licensing and distribution arrangements with third parties. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no 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, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates that receive marketing approval.

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

The development and commercialization of new drug products is highly competitive. We expect that we, and any future collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to any product candidates that we, or they, may seek to develop or commercialize in the future.

Our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective, have fewer or more tolerable side effects or are less costly than any product candidates that we may develop, which could render any future product candidates obsolete and noncompetitive.

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, or any future collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or any future collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or any future collaborators, are able to enter the market.

Our potential future competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing 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 or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and 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 FDA or comparable foreign regulatory authorities approve generic versions of any future products that receive marketing approval, or such authorities do not grant such products appropriate periods of data exclusivity before approving generic versions of our products, our sales could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a “reference-listed drug” in the FDA’s publication, “Approved Drug Products with Therapeutic Equivalence Evaluations.” Manufacturers may seek approval of generic versions of reference-listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference-listed drug and that the generic version is bioequivalent to the reference-listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug may be typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference-listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference-listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference-listed drug. We do not know if the FDA will treat the active ingredients in any future product candidates of ours as NCEs and, therefore, afford them five years of NCE data exclusivity if they are approved. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that any such products of ours may face from generic versions of such products could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

Even if we, or any future collaborators, are able to commercialize any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or healthcare reform initiatives that could harm our business.

The commercial success of any future product that we may develop will depend substantially, both domestically and abroad, on the extent to which the costs of such product will be paid by third-party payors, including government health administration authorities and private health coverage insurers. If coverage and reimbursement is not available, or reimbursement is available only to limited levels, we, or any future collaborators, may not be able to successfully commercialize such product. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or any future collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement for products can differ significantly from payor to payor.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Many countries outside the United States 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 and can be lengthy, involve extensive negotiations and potentially result in price caps, significant discounts or other budgetary control measures, which could correspondingly impact pricing and reimbursement in other markets through so-called informal or formal reference pricing schemes. These reviews and negotiations could ultimately result in a pricing and reimbursement structure for a drug that a company deems inadequate and therefore elects not launch in such markets. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or any future collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may 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 or the ability of any future collaborators to recoup our or their investment in one or more product candidates, even if any future product candidates obtain marketing approval.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any future collaborators, to commercialize any product will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from third-party payors. Third-party payors decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of any future collaborators to sell any products we develop profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of any future collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or any future collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If the prices for any future products decrease or if governmental and other third-party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any future collaborator, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any product candidate for which we, or any future collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

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

We face an inherent risk of product liability claims as a result of the clinical testing of product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we or any future collaborators commercially sell any product that we may or they may develop. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of any future product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for any future 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 resulting litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to explore and evaluate strategic options; and
- the inability to commercialize any products that we may develop.

Although we maintain general liability insurance of $5.0 million in the aggregate and clinical trial liability insurance of $10.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of any future product candidates, which could adversely affect our business, financial condition, results of operations and prospects.

Risks Related to Our Dependence on Third Parties

We may seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

Drug development and commercialization of product candidates require substantial cash to fund expenses. If we determine to pursue the development of additional product candidates, we may seek one or more collaborators for the development and commercialization of such product candidates. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. In addition, any loan and security agreements or collaboration agreements that we enter into in the future may contain, restrictions on our ability to enter into potential collaborations or to otherwise develop specified compounds.

We face significant competition in seeking appropriate collaborators and strategic partners. Whether we reach a definitive agreement for a collaboration or strategic partnership will depend, among other things, upon our assessment of the other party’s resources and expertise, the terms and conditions of the proposed transaction and the proposed party’s evaluation of a number of factors. Those factors may include the potential differentiation of a partner’s product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator or strategic partner may also be considering alternative transaction types and structures that may be more attractive than the one with us.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop the product candidate or bring it to market and generate product revenue.

If we enter into collaborations with third parties for the development and commercialization of a product candidate, our prospects with respect to such product candidate will depend in significant part on the success of those collaborations.

If we enter into collaborations for the development and commercialization of a product candidate, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of such product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of a product candidate or may elect not to continue or renew development or commercialization programs, based on clinical trial results, 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 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;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination 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 maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for 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 future collaborator of ours is involved in a business combination or a sale or other transaction involving our collaboration, it or the party with which it entered into a business combination, sale or other transaction could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We expect to rely on third parties to conduct our clinical trials. If they do not perform satisfactorily, our business could be significantly harmed.

If we determine to pursue the development of additional product candidates, we expect that we will not independently conduct clinical trials of such product candidates. We would expect rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct clinical trials of any such product candidate. Any of these third parties could terminate its engagement with us under certain circumstances or encounter, for example, business challenges, such as a loss of business or the COVID-19 pandemic, or enter into transactions, such as business combinations, that temporarily or permanently impact the amount or type of resources that they are able or willing to devote to our engagement. We might not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new contract research organization begins work. As a result, delays would likely occur, which could materially impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Further, our reliance on these third parties for clinical development activities would limit our control over these activities, but we would remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of a product candidate, we would 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 current Good Clinical Practices, or cGCPs, 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. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or any of our third-party contractors fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving a product candidate, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. Many of these risks are heightened by the COVID-19 pandemic. We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties that may conduct clinical trials on our behalf would not be our employees, and except for remedies available to us under our agreements with such contractors, we would not be able to control whether or not they devote sufficient time, skill and resources to our development programs, a risk that would be exacerbated during the COVID-19 pandemic as any such third parties try to address the impact of the pandemic on their own businesses and financial condition. Any such contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical programs. 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 may not be able to obtain, or may be delayed in obtaining, marketing approvals for the applicable product candidates. If that occurs, we would not be able to, or may be delayed in our efforts to, successfully commercialize such product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be impaired.

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

We plan to contract with third parties for the manufacture and distribution of product candidates for any future development and commercialization efforts. This reliance on third parties, particularly those we rely on solely to source our drug substance, drug product and certain key raw materials, would increase the risk that we would not have sufficient quantities of product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We currently have no manufacturing facilities and limited personnel with manufacturing experience. If we determine to pursue the development of additional product candidates, we plan to rely on contract manufacturers to produce both drug substance and drug product required for any future clinical trials. We would also plan to continue to rely upon contract manufacturers, and, potentially collaboration partners, to manufacture commercial quantities of our products, if approved. Reliance on such third-party contractors entails risks, including:

- manufacturing delays if our third-party contractors give greater priority to the supply of other products over any future product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the possible termination or nonrenewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the possible breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

We may rely on a single third-party contract manufacturer to supply our active pharmaceutical ingredient and a single third-party contract manufacturer to manufacture required finished product for any future preclinical studies, clinical trials and commercial production. If any such future sole source manufacturer or its facilities should become unavailable to us for any reason, we would likely incur significant delays in manufacturing active pharmaceutical ingredient and finished product as identifying, contracting with and qualifying effective replacements would be a lengthy and time-consuming process. Such delays, if we did not have adequate supplies of safety stock, would potentially delay the timetables of our preclinical studies, clinical trials and commercialization plans.

Any manufacturing problem or delay or the loss of a future contract manufacturer could be disruptive to our operations, delay our clinical trials or regulatory reviews or approvals and, if our products are approved for sale, result in lost sales. Additionally, if we determine to pursue the development of additional product candidates, we plan to rely on third parties to supply the raw materials needed to manufacture such product candidates. Any reliance on such suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. Any unanticipated disruption to future contract manufacture caused by problems at suppliers or their facilities could delay shipment of product candidates, delay clinical trials, increase our cost of goods sold and result in lost sales.

Our ability to obtain clinical and commercial supplies of any future product candidate could also be disrupted if the operations of any future third-party contract manufacturers, particularly any sole source manufacturers, are affected by a man-made or natural disaster or other business interruption, including from the COVID-19 pandemic. There can be no assurance that our supply of research and development, preclinical study, clinical trial and commercial drug substance, drug product, key raw materials and other materials will not be limited, interrupted, restricted in certain geographic regions, or be of satisfactory quality as a result of the pandemic or any other business interruption. If we are at any time unable to provide an uninterrupted supply of any future product candidates or, following regulatory approval, any products to patients, we may lose patients, physicians may elect to utilize competing therapeutics instead of our products, significant revenues and market opportunities and our clinical trials may be adversely affected, which could materially and adversely affect our clinical trial outcomes.

At the appropriate time during the clinical development of any future product candidates, we would plan to enter into long-term agreements with third-party contract manufacturers for the commercial production and distribution of drug substance, drug product and other components of our commercial supply chain. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under current good manufacturing practices, or cGMPs, that are capable of manufacturing any future product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization efforts.

Third-party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States. Facilities used by our third-party manufacturers must be approved by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of product candidates for use or sale in foreign countries. We would not control the manufacturing process and would be completely dependent on our third-party manufacturers for compliance with the applicable regulatory requirements for the manufacture of product candidates. If any such future manufacturers cannot successfully manufacture material that conforms to our specifications or the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, they would not be able to secure the applicable approval for their manufacturing facilities. If these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities or manufacturers, which could result in delays in obtaining approval for the applicable product candidate.

In addition, contract manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing approval. Some of these inspections may be unannounced. Failure by any future manufacturer of ours to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could adversely affect supplies of product candidates and significantly harm our business, financial condition and results of operations.

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

Risks Related to Our Intellectual Property

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

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to any future proprietary product candidates. If we do not adequately protect our intellectual property, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, patents are granted to the party who was the first to file a patent application. However, prior to March 16, 2013, in the United States, patents were granted to the party who was the first to invent the claimed subject matter. 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 at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in post-grant review procedures, oppositions, derivations, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity 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 products. In addition, 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.

Patent applications may not result in patents being issued which protect any future product candidates, in whole or in part, or which effectively prevent others from commercializing competitive 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 or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does.

Even if 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. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with any of our future products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

Along with patent protection, we also rely on trade secret protection for certain aspects of technology platform, including certain aspects of our SMART Linker drug discovery platform. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third party, or those to whom they communicate such technology or information, 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, our business and competitive position could be harmed.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. 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 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 shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing any future product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell any future product candidates and use our SMART Linker drug discovery platform without infringing the intellectual property and other proprietary rights of third parties. If any third-party patents or patent applications are found to cover any future product candidates or their methods of use, we may not be free to manufacture or market such product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to any future product candidates, including interference proceedings before the USPTO. Third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that any future product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. 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 access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing any future product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or the Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act included a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, for example, via post grant review and inter partes review proceedings at the USPTO. In addition, the Leahy-Smith Act transformed the U.S. patent system into a "first to file" system. The first-to-file provisions, however, only became effective in March 2013. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our or our collaboration partners' patent applications and the enforcement or defense of our or our collaboration partners' issued patents, all of which could harm our business, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. 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. 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. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering any future product candidates, our competitive position would be adversely affected.

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive. The requirements for patentability may differ in certain countries, particularly in developing countries. 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 may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents or where any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. 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, if our ability to enforce our patents to stop infringing activities in those jurisdictions is inadequate. 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.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

Patent term may be inadequate to protect our competitive position on our products for an adequate amount of time.

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. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property or claiming ownership of what we regard as our own intellectual property.

Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including members of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. 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. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically 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, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

Risks Related to Regulatory Approval 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 or any future collaborators from obtaining approvals for the commercialization of any future product candidates. As a result, we cannot predict when or if, and in which territories, we, or any future collaborators, will obtain marketing approval to commercialize a product candidate.

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of drug products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities, which regulations differ from country to country. We, and any future collaborators, are not permitted to market product candidates in the United States or in other countries until we, or they, receive approval of an NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Product candidates in the development phase are subject to the risks of failure inherent in drug development. We have not submitted an application for or received marketing approval for any product candidate in the United States or in any other jurisdiction. We have limited experience in conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of an NDA.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It 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.

In addition, 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. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are 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, or any future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of any future collaborators to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

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

In order to market and sell products in the European Union and many other jurisdictions, we, and any future 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 marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We, and any future collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020. Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult to date. To date, only an outline of a trade agreement has been reached and much remains open. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any future product candidates, which could significantly and materially harm our business.

We, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for any future product candidates.

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 annually in the United States. We, or any future collaborators, may seek orphan drug designations and may be unable to obtain such designations.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period 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 the 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, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because FDA has taken the position that, under certain circumstances, another drug with the same active moiety can be approved for the same condition. Specifically, the FDA's regulations provide that it can approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

In August 2017, the 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.

Even if we, or any future collaborators, obtain marketing approvals for a product candidate, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for any product candidate for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

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, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or any future collaborators, receive marketing approval for one or more of any future product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any product candidates for which we, or any future collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Any product candidates for which we, or any future collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, 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, 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 Risk Evaluation and Mitigation Strategy.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or any future collaborators, do not market any of our future product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions 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;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Under the Cures Act and the Trump Administration's regulatory reform initiatives, the FDA's policies, regulations and guidance may be revised or revoked and that could prevent, limit or delay regulatory approval of any future product candidates, which would impact our ability to generate revenue.

In December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and spur innovation, but its ultimate implementation is unclear. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump Administration may impact our business and industry. Namely, 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. An under-resourced FDA could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. In January 2017, President Trump issued an executive order, applicable to all executive agencies including the FDA, which requires 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 and approximate the total costs or savings associated with each new regulation or repealed regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within OMB in February 2017, the administration indicates that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. In addition, on February 24, 2017, President Trump issued an executive order directing each affected agency to designate an agency official as a "Regulatory Reform Officer" and establish a "Regulatory Reform Task Force" to implement the two-for-one provisions and other previously issued executive orders relating to the review of federal regulations. Subsequently, on October 9, 2019, the President issued Executive Order 13,892, which is titled "Promoting the Rule of Law Through Transparency and Fairness in Civil Administrative Enforcement and Adjudication." This Order declares that "guidance documents may not be used to impose new standards of conduct on persons outside the executive branch." It is difficult to predict how these various requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize product candidates and affect the prices we, or they, may obtain.

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, among other things, prevent or delay marketing approval of any future product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that 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, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, 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 Medicare Modernization Act 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 Medicare Modernization Act may result in a similar reduction in payments from private payors.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the Affordable Care Act, or ACA, became law in 2010 and includes the following provisions of potential importance to any future product candidates that we may choose to develop:

- an annual, non-deductible fee on any entity that manufactures, or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% 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 new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new 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. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several 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 future product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

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 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 Tax Cuts and Jobs Act of 2017, 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, became 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 Congress may consider other legislation to replace elements of the ACA during the next Congressional session.

The Trump Administration has also taken executive actions to undermine or delay implementation of the ACA. Since January 2017, President Trump has 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 administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. 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. On April 27, 2020, the U.S. Supreme Court overruled this decision. 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. In addition, Centers for Medicare & Medicaid Services 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.

In addition, 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 Tax Cuts and Jobs Act, 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 recently 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. On December 18, 2019, that court affirmed the lower court's ruling that the individual mandate portion of the ACA was 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 June 25, 2020, the Trump Administration and a coalition of 18 states asked the U.S. Supreme Court to strike down the entirety of the ACA, and in November 2020 the court heard oral argument in the case, but it has not yet issued a ruling. It is unclear how such litigation and other efforts to repeal and replace the ACA will impact the ACA and our business.

We will continue to evaluate the effect that the ACA and its possible repeal and replacement could have on our business. It is possible that such initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to amend the ACA is uncertain in many respects, it is also possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop commercialize product candidates.

Further, there have been several recent U.S. congressional inquiries and proposed and enacted federal and 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, the Trump administration has pressed for drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. 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.

In addition, on May 11, 2018, the Administration issued a plan to lower drug prices. Under this blueprint for action, the 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' ads 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. In addition, on December 23, 2019, the Trump Administration published a proposed rulemaking that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to FDA for review and approval. Applicants would be required to demonstrate 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, 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).

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 health care 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 health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing.

Moreover, legislative and regulatory proposals have also been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical drugs. 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 drug candidates, if any, may be. In addition, increased scrutiny by the Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any collaborators to more stringent drug labeling and post-marketing testing and other requirements.

Our relationships with customers and third-party payors, among others, will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our arrangements with third-party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. These include the following:

Anti-Kickback Statute. The federal healthcare Anti-Kickback Statute prohibits, among other things, persons and entities 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;

False Claims Laws. The federal false claims laws impose criminal and civil penalties 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;

HIPAA. The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters, and, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;

Transparency Requirements. Federal laws require applicable manufacturers of covered drugs, biologics, devices and supplies to report payments and other transfers of value to physicians and teaching hospitals and ownership and investment interests by physicians; and

Analogous State and Foreign Laws. Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader in scope, can apply to our business activities, including sales or marketing arrangements, and claims involving healthcare items or services and are generally broad and are enforced by many different federal and state agencies as well as through private actions. 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 require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State 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 pre-empted 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 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 government funded healthcare programs.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with applicable requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

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 European Union 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 European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with the GDPR's 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.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could significantly harm our business.

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

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

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

In some countries, such as 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, or any future collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our product 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 materially harmed.

A fast track designation by the FDA may not actually lead to a faster development, regulatory review or approval process.

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet needs for this condition, the treatment sponsor may apply for FDA fast track designation. Fast track designation does not ensure a faster development, regulatory review or approval process compared to conventional FDA procedures. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

We may seek and be granted rare pediatric disease designation by the FDA but that designation would not expedite or ensure approval of the applicable product candidate nor would it guarantee that we would receive a Priority Review Voucher if that product candidate is approved by the FDA.

The FDA has awarded rare pediatric disease Priority Review Vouchers to sponsors of drug candidates to treat rare pediatric disease products, if the treatment sponsors apply for this designation and meet certain criteria. Under this program, upon the approval of a qualifying NDA or biologics license application, or BLA, for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease Priority Review Voucher that can be used to obtain priority review for a subsequent NDA or BLA. The Priority Review Voucher may be sold or transferred an unlimited number of times. A rare pediatric disease designation does not guarantee that an NDA will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. It also does not ensure expedited review or approval by the FDA of the product candidate. With passage of the 21st Century Cures Act in December 2016, the Rare Pediatric Disease Priority Review Voucher program was reauthorized until 2020. In addition, if a product candidate is designated before October 1, 2020, it is eligible to receive a voucher if it is approved before October 2022.

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, 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. 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.

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, which we collectively refer to as the Trade Control Laws.

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. Likewise, any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control Laws by U.K., U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Risks Related to Employee Matters, Managing Growth and Information Technology

Our future success depends on our ability to retain our senior management and key employees.

We are highly dependent on members of our senior management, including Jill C. Milne, Ph.D., our President and Chief Executive Officer, Joanne Donovan, M.D., Ph.D., our Chief Medical Officer, Andrew Nichols, Ph.D., our Chief Scientific Officer, Andrew Komjathy, our Chief Commercial Officer, Noah Clauser, our Chief Financial Officer, and Ben Harshbarger, J.D., our Senior Vice President, General Counsel.

If we are unable to retain our executive officers or other key employees, replacing them may be difficult and may take an extended period of time because of the nature of our current business strategy and the limited number of individuals in our industry with the relevant breadth of skills and experience. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate replacements for our executive officers or key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel.

We rely on consultants and advisors, including financial, legal, scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy and to explore and evaluate strategic options. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us.

Security breaches and other disruptions to our information technology systems could compromise our information, disrupt our business and expose us to liability, which would cause our business and reputation to suffer.

In the ordinary course of our business, we collect, process and store sensitive data, including intellectual property, as well as our proprietary business information, employee data and personally identifiable information of clinical trial participants. We also rely to a large extent on information technology systems to operate our business. We have outsourced elements of our confidential information processing and information technology structure, and as a result, we are managing independent vendor relationships with third parties who may or could have access to our confidential information. The secure maintenance of this information is important to our operations and business strategy. Despite our security measures, our information technology infrastructure, and that of our vendors and third-party providers, may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. We, our vendors and third-party providers could be susceptible to third party attacks on our, and their, information security systems, which attacks are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives and expertise, including organized criminal groups, hacktivists, nation states and others. While we have invested in information technology security measures and the protection of confidential information, there can be no assurance that our efforts will prevent service interruptions or security breaches. Although we are not aware of any material information security incidents to date, we have detected common types of attempts to attack our information technology systems and data using means that have included phishing. Any service interruptions or security breaches of our information technology systems may substantially impair our ability to operate our business and could compromise our networks, or those of our vendors and third-party providers, and the information stored could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure, or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation, any of which could adversely affect our business.

Risks Related to Our Common Stock

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

Our shares of common stock began trading on The Nasdaq Global Market, or Nasdaq, in June 2015. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price for our common stock and thereby affect the ability of our stockholders to sell their shares. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

If we were to be delisted from The Nasdaq Stock Market, it could make trading in our stock more difficult.

There are various quantitative listing requirements for a company to remain listed on the Nasdaq, including maintaining a minimum bid price of $1.00 per share. No assurance can be given that we will continue to remain compliant with the minimum bid price requirement or Nasdaq's other continued listing requirements. For example, in August 2018, we received a deficiency letter from the Listing Qualifications Department of Nasdaq notifying us that, for the preceding 30 consecutive business days, the bid price for our common stock had closed below the minimum $1.00 per share requirement for continued inclusion on the Nasdaq Global Market as required by Nasdaq Listing Rule 5450(a)(1). In order to regain compliance, on December 28, 2018, we effected a one-for-ten reverse split of our common stock. In addition, following our announcement on October 26, 2020 that, based on the results of the Phase 3 PolarisDMD trial, we were stopping all activities related to the edasalonexent program and that we plan to explore and evaluate strategic options, the trading price of our common stock on the Nasdaq Global Market dropped to a closing price of $1.56 per share on October 27, 2020, and closed at $1.39 per share on November 10, 2020, which substantially increases the likelihood that we may not remain compliant with the minimum bid price requirement or Nasdaq's other continued listing requirements, which could put us at risk of being delisted from the Nasdaq Global Market. Any delisting would likely have a negative effect on the price of our common stock and would impair stockholders' ability to sell or purchase their common stock when they wish to do so.

The price of our common stock is likely to be highly volatile, which could result in substantial losses for our stockholders.

Our stock price is likely to be highly volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our investors may lose some or all of their investments. The market price for our common stock may be influenced by many factors, including:

- announcement of updates regarding, or the results of, our exploration and evaluation of strategic options;
- the timing and results of clinical trials of any future product candidate;
- commencement or termination of collaborations for any development programs we may pursue;
- failure or discontinuation of any of any development programs we may pursue;
- the success of existing or new competitive products or technologies;
- results of clinical trials of product candidates of competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to a product candidate or clinical development program;
- the results of any additional efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or recommendations by securities analysts that cover our stock;
- announcement or expectation of additional financing efforts;
- announcement of collaborations, licenses, acquisitions or other comparable forms of transactions;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those 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, including political instability, or instability from an outbreak of pandemic or contagious disease, such as the ongoing COVID-19 pandemic; and
- the other factors described in this "Risk Factors" section.

Additionally, securities class action litigation has often been brought against a company following a decline in the market price of its securities. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until the last day of our fiscal year following the fifth anniversary of our IPO, subject to specified conditions. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, 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 expect to continue to take advantage of some or all of the available exemptions until we cease to be an emerging growth company on January 1, 2021. Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company, which would allow us to take advantage of many of the same exceptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of SOX Section 404 and reduced disclosure obligations regarding executive compensation. Investors may find our common stock less attractive as a result of our reliance 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.

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, and particularly after we are no longer an "emerging growth company" or a "smaller reporting company," we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The SOX, 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. We expect that we will need to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being a public company and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements increase our legal and financial compliance costs and make some activities more time-consuming and costly. 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 SOX Section 404 we are required to furnish reports by our management on our internal control over financial reporting with our Annual Reports on Form 10-K with the SEC. Commencing January 1, 2021, when we are no longer an emerging growth company, we may also be required to include attestation reports on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. As of September 30, 2020, we had outstanding 20,077,337 shares of common stock. As of September 30, 2020, we had outstanding warrants to purchase 4,200,000 shares of common stock at an exercise price of $12.00 per share, and 1,991,300 shares of common stock at an exercise price of $6.25 per share. These warrants are fully exercisable, and we have registered the issuance of shares upon exercise of these warrants under registration statements. As a result, the shares issuable upon exercise of these warrants can be freely sold in the public marked upon issuance, subject to volume limitations applicable to affiliates. Additionally, we have an ongoing sales agreement with Cowen and Company LLC, pursuant to which we could issue and sell shares of common stock under at-the-market offering programs.

The number of shares of common stock underlying our outstanding warrants is significant in relation to our currently outstanding common stock, which could have a negative effect on the market price of our common stock and make it more difficult for us to raise funds through future equity offerings.

As part of our June 2018 equity financing we issued warrants to purchase an aggregate of 4,200,000 shares of common stock at an exercise price of $12.00 per share, all of which are outstanding, and as part of our February 2019 equity financing we issued warrants to purchase an aggregate of 2,000,000 shares of common stock at an exercise price of $6.25 per share, of which warrants to purchase 1,991,300 shares remain outstanding. Upon exercise in full of these outstanding warrants, the shares issuable upon exercise would represent a significant portion of our outstanding common stock. The warrants were fully exercisable upon issuance and remain exercisable for five years from their respective dates of issuance. We have registered the issuance of shares upon exercise of these warrants under a registration statements under the Securities Act of 1933, as amended, or the Securities Act, and, accordingly, such shares can be freely sold into the public market upon issuance, subject to volume limitations applicable to affiliates. Sales of these shares could cause the market price of our common stock to decline significantly. Furthermore, if our stock price rises, the holders of these warrants may be more likely to exercise their warrants and sell a large number of shares, which could negatively impact the market price of our common stock and reduce or eliminate any appreciation in our stock price that might otherwise occur.

We may also find it more difficult to raise additional equity capital while these warrants are outstanding. At any time during which these warrants are likely to be exercised, we may be unable to obtain additional equity capital on more favorable terms from other sources. In addition, the exercise of these warrants would result in a significant increase in the number of our outstanding shares of common stock, which could have the effect of significantly diluting the interest of our current stockholders, and following such exercise the former holders of such warrants could have significant influence over our company as a result of the shares of common stock they acquire upon such exercise.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future, accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. Any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be investors' sole source of gain for the foreseeable future.

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

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our investors 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 all members of the board are not elected at one time;
- 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 the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- 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 a special meeting of 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 certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, 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. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders.

Our certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District 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 and officers.

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 does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or any action asserting a claim against us governed by the internal affairs doctrine. These exclusive forum provisions 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 provisions 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. This exclusive forum provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act of 1934, which provides for exclusive jurisdiction of the federal courts. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision and asserts claims under the Securities Act of 1933, inasmuch as Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder; provided, that with respect to claims under the Securities Act, our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

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

The trading market for our common stock will likely depend, in part, on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will cover us or provide favorable coverage. If one or more analysts downgrade our stock or change their opinion of our stock, our share price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibits Index below:

Exhibit Number	Exhibit
10.1	Catabasis Pharmaceuticals, Inc. Amended and Restated Executive Severance Benefits Plan, effective October 7, 2020
31.1	Certification of principal executive officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of 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 Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of The Sarbanes-Oxley Act of 2002, by the Registrant's principal executive officer and principal financial officer
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Label Linkbase Document
101.PRE	XBRL Taxonomy Presentation Linkbase Document

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.

Catabasis Pharmaceuticals, Inc.

Date: November 12, 2020

By: /s/ NOAH C. CLAUSER
Noah C. Clauser
Chief Financial Officer (Principal Financial Officer)

Exhibit 10.1

CATABASIS PHARMACEUTICALS, INC.

Amended and Restated Severance Benefits Plan

1. Establishment of Plan. Catabasis Pharmaceuticals, Inc., a Delaware corporation, hereby establishes this amended and restated unfunded severance benefits plan (the "Plan") that is intended to be a welfare benefit plan within the meaning of Section 3(1) of ERISA. The Plan is in effect for Covered Employees who experience a Covered Termination occurring after the Effective Date and before the termination of this Plan. This Plan supersedes any and all (i) severance plans and separation policies applying to Covered Employees that may have been in effect before the Effective Date with respect to any termination that would, under the terms of this Plan, constitute a Covered Termination and (ii) the provisions of any agreements between any Covered Employee and the Company that provide for severance benefits solely as such agreements relate to severance benefits.

2. Purpose. The purpose of the Plan is to establish the conditions under which Covered Employees will receive the severance benefits described herein if employment with the Company (or its successor in a Change in Control (as defined below)) terminates under the circumstances specified herein. The severance benefits paid under the Plan are intended to assist employees in making a transition to new employment and are not intended to be a reward for prior service with the Company.

3. Definitions. For purposes of this Plan,

(a) "Base Salary" shall mean, for any Covered Employee, such Covered Employee's base rate of pay as in effect immediately before a Covered Termination (or prior to the Change of Control, if greater) and exclusive of any bonuses, overtime pay, shift differentials, "adders," any other form of premium pay, or other forms of compensation.

(b) "Benefits Continuation" shall have the meaning set forth in Section 8(a) hereof.

(c) "Board" shall mean the Board of Directors of the Company.

(d) "Cause" shall mean any of: (a) your conviction of, or plea of guilty or nolo contendere to, any crime involving dishonesty or moral turpitude or any felony; or (b) a good faith finding by the Board that you have (i) engaged in dishonesty, willful misconduct or gross negligence that has a material adverse effect on the Company, (ii) committed an act that materially injures or would reasonably be expected to materially injure the reputation, business or business relationships of the Company, (iii) materially breached the terms of any restrictive covenants or confidentiality agreement with the Company (and not cured the same within any cure period applicable to such covenants or confidentiality agreement), or (iv) failed or refused to comply in any material respect with the Company's material policies or procedures and in a manner that materially injures or would reasonably be expected to materially injure the reputation, business or business relationships of the Company, provided that in the case of clause (iv) you were given written notice of such violation or failure by the Board and a period of 30 days to cure (provided that the Board reasonably determines that such violation or failure is curable).

(e) "Change in Control" shall mean the occurrence of any of the following events, provided that such event or occurrence constitutes a change in the ownership or effective control of the Company, or a change in the ownership of a substantial portion of the assets of the Company, as defined in Treasury Regulation §§1.409A-3(i)(5)(v), (vi) and (vii): (i) the acquisition by an individual, entity or group (within the meaning of Section 13(d)(3) or 14(d)(2) of the Securities Exchange Act of 1934 (the "Exchange Act")) (a "Person") of beneficial ownership of any capital stock of the Company if, after such acquisition, such Person beneficially owns (within the meaning of Rule 13d-3 under the Exchange Act) 50% or more of either (x) the then-outstanding shares of common stock of the Company (the "Outstanding Company Common Stock") or (y) the combined voting power of the then-outstanding securities of the Company entitled to vote generally in the election of directors (the "Outstanding Company Voting Securities"); provided, however, that for purposes of this subsection (i), the following acquisitions shall not constitute a Change in Control: (1) any acquisition directly from the Company or (2) any acquisition by any entity pursuant to a Business Combination (as defined below) which complies with clauses (x) and (y) of subsection (iii) of this definition; or (ii) a change in the composition of the Board that results in the Continuing Directors (as defined below) no longer constituting a majority of the Board (or, if applicable, the Board of Directors of a successor corporation to the Company), where the term "Continuing Director" means at any date a member of the Board (x) who was a member of the Board on the date of the initial adoption of the Plan by the Board or (y) who was nominated or elected subsequent to such date by at least a majority of the directors who were Continuing Directors at the time of such nomination or election or whose election to the Board was recommended or endorsed by at least a majority of the directors who were Continuing Directors at the time of such nomination or election; provided, however, that there shall be excluded from this clause (y) any individual whose initial assumption of office occurred as a result of an actual or threatened election contest with respect to the election or removal of directors or other actual or threatened solicitation of proxies or consents, by or on behalf of a person other than the Board; or (iii) the consummation of a merger, consolidation, reorganization, recapitalization or share exchange involving the Company or a sale or other disposition of all or substantially all of the assets of the Company (a "Business Combination"), unless, immediately following such Business Combination, each of the following two conditions is satisfied: (x) all or substantially all of the individuals and entities who were the beneficial owners of the Outstanding Company Common Stock and Outstanding Company Voting Securities immediately prior to such Business Combination beneficially own, directly or indirectly, more than 50% of the then-outstanding shares of common stock and the combined voting power of the then-outstanding securities entitled to vote generally in the election of directors, respectively, of the resulting or acquiring corporation in such Business Combination (which shall include, without limitation, a corporation which as a result of such transaction owns the Company or substantially all of the Company's assets either directly or through one or more subsidiaries) (such resulting or acquiring corporation is referred to herein as the "Acquiring Corporation") in substantially the same proportions as their ownership of the Outstanding Company Common Stock and Outstanding Company Voting Securities, respectively, immediately prior to such Business Combination and (y) no Person (excluding any employee benefit plan (or related trust) maintained or sponsored by the Company or by the Acquiring Corporation) beneficially owns, directly or indirectly, 50% or more of the then-outstanding shares of common stock of the Acquiring Corporation, or of the combined voting power of the then-outstanding securities of such corporation entitled to vote generally in the election of directors (except to the extent that such ownership existed prior to the Business Combination); or (iv) the liquidation or dissolution of the Company.

(f) "Change in Control Termination" shall mean a termination of the Covered Employee's employment by the Company without Cause or by the Covered Employee for Good Reason, in either case within the 12 months following a Change in Control.

(g) "COBRA" shall mean the Consolidated Omnibus Budget Reconciliation Act.

(h) "Code" shall mean the Internal Revenue Code of 1986, as amended.

(i) "Company" shall mean Catabasis Pharmaceuticals, Inc. or, following a Change in Control, any successor thereto.

(j) "Compensation Committee" shall mean the Compensation Committee of the Board.

(k) "Covered Employees" shall mean all Regular Full-Time Employees (both exempt and non-exempt) who (i) are Executives as of the Effective Date or (ii) become Executives after the Effective Date and are designated by the Board or the Compensation Committee to be a Covered Employee under this Plan, who experience a Covered Termination and who are not designated as ineligible to receive severance benefits under the Plan as provided in Section 5 hereof. For the avoidance of doubt, neither Temporary Employees nor Part-Time Employees are eligible for severance benefits under the Plan. An employee's full-time, part-time or temporary status for the purpose of this Plan is determined by the Plan Administrator upon review of the employee's status immediately before termination. Any person who is classified by the Company as an independent contractor or third party employee is not eligible for severance benefits even if such classification is modified retroactively.

(l) "Covered Termination" shall mean (i) a Non-Change in Control Termination or (ii) a Change in Control Termination.

(m) "Effective Date" shall mean October 7, 2020.

(n) "ERISA" shall mean the Employee Retirement Income Security Act of 1974, as amended.

(o) "Executive" shall mean any employee of the Company holding the title of Vice President or above.

(p) "Good Reason" is defined as: (i) a material diminution in the employee's base compensation; (ii) a material diminution in the employee's authority, duties, or responsibilities; (iii) a material change in the geographic location at which the employee must perform the services; or (iv) any other action or inaction that constitutes a material breach by the Company of any agreement under which the employee provides services; provided, however, that in any case the employee has not consented to the condition which would otherwise give rise to a Good Reason. In order to establish a "Good Reason" for terminating employment, an employee must provide written notice to the Company of the existence of the condition giving rise to the Good Reason, which notice must be provided within 90 days of the initial existence of such condition, the Company must fail to cure the condition within 30 days thereafter, and an employee's termination of employment must occur no later than one year following the initial existence of the condition giving rise to Good Reason.

(q) "Non-Change in Control Termination" shall mean a termination of the Covered Employee's employment by the Company without Cause or by the Covered Employee for Good Reason prior to or more than 12 months following a Change in Control.

(r) "Other C-Level Officer" shall mean any Executive (other than the Chief Executive Officer) designated by the Board or the Compensation Committee as an Other C-Level Officer for purposes of the Plan, including Other C-Level Officers who were designated as such prior to the Effective Date.

(s) "Part-Time Employees" shall mean employees who are not Regular Full-Time Employees and are treated as such by the Company.

(t) "Participants" shall mean Covered Employees.

(u) "Plan Administrator" shall have the meaning set forth in Section 15 hereof.

(v) "Release" shall have the meaning set forth in Section 6 hereof.

(w) "Release Effective Date" shall have the meaning set forth in Section 13(c)(i) hereof.

(x) "Regular Full-Time Employees" shall mean employees, other than Temporary Employees, normally scheduled to work at least 40 hours a week unless the Company's local practices, as from time to time in force, whether or not in writing, establish a different hours threshold for regular full-time employees.

(y) "Severance Pay" shall have the meaning set forth in Section 7 hereof.

(z) "Severance Period" shall mean the applicable severance period determined under the chart in Section 7 hereof based on the type of Covered Termination and the title/role of the Covered Employee.

(aa) "Temporary Employees" are employees treated as such by the Company, whether or not in writing.

4. Coverage. A Covered Employee may be entitled to receive severance benefits under the Plan if such employee experiences a Covered Termination. In order to receive severance benefits under the Plan, Covered Employees must meet the eligibility and other requirements provided below in Sections 5 and 6 of the Plan.

5. Eligibility for Severance Benefits. The following employees will *not* be eligible for severance benefits, except to the extent specifically determined otherwise by the Plan Administrator: (a) an employee who is terminated for Cause; (b) an employee who retires, terminates employment as a result of an inability to perform his or her duties due to physical or mental disability or dies; (c) an employee who voluntarily terminates his or her employment, except in the case of a Covered Termination for Good Reason; (d) an employee who is employed for a specific period of time in accordance with the terms of a written employment agreement; and (e) an employee who promptly becomes employed by another member of the controlled group of entities of which the Company (or its successor in the Change in Control) is a member as defined in Sections 414(b) and (c) of the Code.

6. Release; Timing of Severance Benefits. Receipt of any severance benefits under the Plan requires that the Covered Employee: (a) comply with the provisions of any applicable noncompetition, nonsolicitation, and other obligations to the Company; and (b) execute and deliver a suitable waiver and release under which the Covered Employee releases and discharges the Company and its affiliates from and on account of any and all claims that relate to or arise out of the employment relationship between the Company and the Covered Employee (the "Release") which Release becomes binding within 60 days following the Covered Employee's termination of employment. The Severance Pay will be paid in accordance with the terms of the Plan and the Company's regular pay practices in effect from time to time and the Benefits Continuation will be paid in the amount and at the time premium payments are made by other participants in the Company's health benefit plans with the same coverage. The payments shall be made or commence on the first payroll date after the Release Effective Date.

7. Cash Severance. A Covered Employee entitled to severance benefits under this Plan shall be entitled to the continuation of such employee's monthly Base Salary for the Severance Period indicated below ("Severance Pay"), based upon his or her title/role.

Title/Role of Covered Employee	Non-Change in Control Termination Severance Period	Change in Control Termination Severance Period
Chief Executive Officer	12 months	18 months
Other C-Level Officer	12 months	12 months
Vice Presidents (of any level) who are not Other C-Level Officers	Six months	Nine months

For purposes of this Section 7 and Section 8 below, a Covered Employee's title/role shall be such employee's title/role immediately prior to the Covered Termination or, if such employee's title/role was changed in connection with the Change in Control, immediately prior to the Change in Control.

8. Other Severance Benefits. In addition to the foregoing Severance Pay, the severance benefits under the Plan shall include the following benefits:

(a) Company contributions to the cost of COBRA coverage on behalf of the Covered Employee and any applicable dependents for no longer than the Covered Employee's applicable Severance Period if the Covered Employee elects COBRA coverage, and only so long as such coverage continues in force. Such costs shall be determined on the same basis as the Company's contribution to Company-provided health and dental insurance coverage in effect for an active employee with the same coverage elections; provided that if the Covered Employee commences new employment and is eligible for a new group health plan, the Company's continued contributions toward health and dental coverage shall end when the new employment begins ("Benefits Continuation").

(b) Any unpaid annual bonus in respect to any completed bonus period which has ended prior to the date of the Participant's Covered Termination and which the Board or the Compensation Committee deems granted to the Participant in its discretion pursuant to the Company's contingent compensation program, payable at the same time as annual bonuses are paid to other employees of the Company or, if later, upon the Release Effective Date.

(c) In the case of the Chief Executive Officer upon a Covered Termination, a bonus amount equal to one-half of the average annual bonus paid to the Chief Executive by the Company over the three calendar years preceding the calendar year in which such Covered Termination occurs, which bonus shall be prorated by multiplying the amount by a fraction, the numerator of which is the number of days in the calendar year in which such termination of employment occurs that have elapsed since January 1 through the date of such termination and the denominator of which is 365, payable in a lump sum on the Release Effective Date.

9. **Equity Awards**. In the case of a Change in Control Termination, any unvested equity awards shall become fully vested and exercisable, or free from forfeiture or repurchase, effective upon the Release Effective Date. Except as set forth in the foregoing sentence, the treatment of a Covered Employee's equity awards with the Company upon a Covered Termination shall be dictated by the terms of the applicable award agreements.

10. **Recoupment.** If a Covered Employee fails to comply with the terms of the Plan, including the provisions of Section 6 above, the Company may require payment to the Company of any benefits described in Sections 7 and 8 above that the Covered Employee has already received to the extent permitted by applicable law and with the "value" determined in the sole discretion of the Plan Administrator. Payment is due in cash or by check within 10 days after the Company provides notice to a Covered Employee that it is enforcing this provision. Any benefits described in Sections 7 and 8 above not yet received by such Covered Employee will be immediately forfeited.

11. **Death.** If a Participant dies after the date of his or her Covered Termination but before all payments or benefits to which such Participant is entitled pursuant to the Plan have been paid or provided, payments will be made to any beneficiary designated by the Participant prior to or in connection with such Participant's Covered Termination or, if no such beneficiary has been designated, to the Participant's estate. For the avoidance of doubt, if a Participant dies during such Participant's applicable Severance Period, Benefits Continuation will continue for the Participant's applicable dependents for the remainder of the Participant's Severance Period.

12. **Withholding.** The Company may withhold from any payment or benefit under the Plan: (a) any federal, state, or local income or payroll taxes required by law to be withheld with respect to such payment; (b) such sum as the Company may reasonably estimate is necessary to cover any taxes for which the Company may be liable and which may be assessed with regard to such payment; and (c) such other amounts as appropriately may be withheld under the Company's payroll policies and procedures from time to time in effect.

13. **Section 409A.** It is expected that the payments and benefits provided under this Plan will be exempt from the application of Section 409A of the Code, and the guidance issued thereunder ("Section 409A"). The Plan shall be interpreted consistent with this intent to the maximum extent permitted and generally, with the provisions of Section 409A. A termination of employment shall not be deemed to have occurred for purposes of any provision of this Plan providing for the payment of any amounts or benefits upon or following a termination of employment (which amounts or benefits constitute nonqualified deferred compensation within the meaning of Section 409A) unless such termination is also a "separation from service" within the meaning of Section 409A and, for purposes of any such provision of this Plan, references to a "termination," "termination of employment" or like terms shall mean "separation from service". Neither the Participant nor the Company shall have the right to accelerate or defer the delivery of any payment or benefit except to the extent specifically permitted or required by Section 409A.

Notwithstanding the foregoing, to the extent the severance payments or benefits under this Plan are subject to Section 409A, the following rules shall apply with respect to distribution of the payments and benefits, if any, to be provided to Participants under this Plan:

(a) Each installment of the payments and benefits provided under this Plan will be treated as a separate "payment" for purposes of Section 409A. Whenever a payment under this Plan specifies a payment period with reference to a number of days (*e.g.*, "payment shall be made within 10 days following the date of termination"), the actual date of payment within the specified period shall be in the Company's sole discretion. Notwithstanding any other provision of this Plan to the contrary, in no event shall any payment under this Plan that constitutes "non-qualified deferred compensation" for purposes of Section 409A be subject to transfer, offset, counterclaim or recoupment by any other amount unless otherwise permitted by Section 409A.

(b) Notwithstanding any other payment provision herein to the contrary, if the Company or appropriately-related affiliates become publicly-traded and a Covered Employee is deemed on the date of termination to be a "specified employee" within the meaning of that term under Code Section 409A(a)(2)(B) with respect to such entity, then each of the following shall apply:

(i) With regard to any payment that is considered "non-qualified deferred compensation" under Section 409A payable on account of a "separation from service," such payment shall be made on the date which is the earlier of (A) the day following the expiration of the six month period measured from the date of such "separation from service" of the Covered Employee, and (B) the date of the Covered Employee's death (the "Delay Period") to the extent required under Section 409A. Upon the expiration of the Delay Period, all payments delayed pursuant to this provision (whether otherwise payable in a single sum or in installments in the absence of such delay) shall be paid to or for the Covered Employee in a lump sum, and all remaining payments due under this Plan shall be paid or provided for in accordance with the normal payment dates specified herein; and

(ii) To the extent that any benefits to be provided during the Delay Period are considered "non-qualified deferred compensation" under Section 409A payable on account of a "separation from service," and such benefits are not otherwise exempt from Section 409A, the Covered Employee shall pay the cost of such benefits during the Delay Period, and the Company shall reimburse the Covered Employee, to the extent that such costs would otherwise have been paid by the Company or to the extent that such benefits would otherwise have been provided by the Company at no cost to the Covered Employee, the Company's share of the cost of such benefits upon expiration of the Delay Period. Any remaining benefits shall be reimbursed or provided by the Company in accordance with the procedures specified in this Plan.

(c) To the extent that severance benefits pursuant to this Plan are conditioned upon a Release, the Covered Employee shall forfeit all rights to such payments and benefits unless such release is signed and delivered (and no longer subject to revocation, if applicable) within 60 days following the date of the termination of the Covered Employee's employment with the Company. If the Release is no longer subject to revocation as provided in the preceding sentence, then the following shall apply:

(i) To the extent any severance benefits to be provided are not "non-qualified deferred compensation" for purposes of Section 409A, then such benefits shall commence upon the first scheduled payment date immediately after the date the Release is executed and no longer subject to revocation (the "Release Effective Date"). The first such cash payment shall include all amounts that otherwise would have been due prior thereto under the terms of this Agreement applied as though such payments commenced immediately upon the termination of the Covered Employee's employment with the Company, and any payments made after the Release Effective Date shall continue as provided herein. The delayed benefits shall in any event expire at the time such benefits would have expired had such benefits commenced immediately following the termination of the Covered Employee's employment with the Company.

(ii) To the extent any such severance benefits to be provided are "non-qualified deferred compensation" for purposes of Section 409A, then the Release must become irrevocable within 60 days of the date of termination and benefits shall be made or commence upon the date provided in Section 6, provided that if the 60th day following the termination of the Covered Employee's employment with the Company falls in the calendar year following the calendar year containing the date of termination, the benefits will be made no earlier than the first business day of that following calendar year. The first such cash payment shall include all amounts that otherwise would have been due prior thereto under the terms of this Agreement had such payments commenced immediately upon the termination of Covered Employee's employment with the Company, and any payments made after the first such payment shall continue as provided herein. The delayed benefits shall in any event expire at the time such benefits would have expired had such benefits commenced immediately following the termination of the Covered Employee's employment with the Company.

(d) The Company makes no representations or warranties and shall have no liability to any Participant or any other person, other than with respect to payments made by the Company in violation of the provisions of this Plan, if any provisions of or payments under this Plan are determined to constitute deferred compensation subject to Section 409A of the Code but not to satisfy the conditions of that section.

14. Section 280G. Notwithstanding any other provision of this Plan, except as set forth in Section 14(b), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the following provisions shall apply**:**

(a) The Company shall not be obligated to provide to the Covered Employee any portion of any "Contingent Compensation Payments" (as defined below) that the Covered Employee would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for the Covered Employee. For purposes of this Section 14, the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(b) Notwithstanding the provisions of Section 14(a), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by the Covered Employee if the Eliminated Payments (determined without regard to this sentence) were paid to the Covered Employee (including state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of the Covered Employee's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 14(b) shall be referred to as a "Section 14(b) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(c) For purposes of this Section 14 the following terms shall have the following respective meanings:

(i) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(ii) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(d) Any payments or other benefits otherwise due to the Covered Employee following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 14(d). Within 30 days after each date on which the Covered Employee first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify the Covered Employee (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 14(b) Override is applicable. Within 30 days after delivery of such notice to the Covered Employee, the Covered Employee shall deliver a response to the Company (the "Covered Employee Response") stating either (A) that the Covered Employee agrees with the Company's determination pursuant to the preceding sentence or (B) that the Covered Employee disagrees with such determination, in which case the Covered Employee shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 14(b) Override is applicable. In the event that the Covered Employee fails to deliver a Covered Employee Response on or before the required date, the Company's initial determination shall be final. If the Covered Employee states in the Covered Employee Response that the Covered Employee agrees with the Company's determination, the Company shall make the Potential Payments to the Covered Employee within three business days following delivery to the Company of the Covered Employee Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If the Covered Employee states in the Covered Employee Response that the Covered Employee disagree with the Company's determination, then, for a period of 60 days following delivery of the Covered Employee Response, the Covered Employee and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in Boston, Massachusetts, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Covered Employee Response, make to the Covered Employee those Potential Payments as to which there is no dispute between the Company and the Covered Employee regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(e) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the Contingent Compensation Payment Ratio (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by the Covered Employee for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by the Covered Employee in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1 Q/A-24(b) or (c)).

(f) The provisions of this Section 14 are intended to apply to any and all payments or benefits available to the Covered Employee under this Plan or any other agreement or plan of the Company under which the Covered Employee receives Contingent Compensation Payments

15. Plan Administration.

(a) **Plan Administrator**. The Plan Administrator shall be the Board or the Compensation Committee; provided, however, that the Board or the Compensation Committee may in its sole discretion appoint a new Plan Administrator to administer the Plan following a Change in Control. The Plan Administrator shall also serve as the Named Fiduciary of the Plan under ERISA. The Plan Administrator shall be the "administrator" within the meaning of Section 3(16) of ERISA and shall have all the responsibilities and duties contained therein.

The Plan Administrator can be contacted at the following address:

Catabasis Pharmaceuticals, Inc.
100 High Street, 28th Floor
Boston, MA 02110

(b) **Decisions, Powers and Duties**. The general administration of the Plan and the responsibility for carrying out its provisions shall be vested in the Plan Administrator. The Plan Administrator shall have such powers and authority as are necessary to discharge such duties and responsibilities which also include, but are not limited to, interpretation and construction of the Plan, the determination of all questions of fact, including, without limit, eligibility, participation and benefits, the resolution of any ambiguities and all other related or incidental matters, and such duties and powers of the plan administration which are not assumed from time to time by any other appropriate entity, individual or institution. The Plan Administrator may adopt rules and regulations of uniform applicability in its interpretation and implementation of the Plan.

The Plan Administrator shall discharge its duties and responsibilities and exercise its powers and authority in its sole discretion and in accordance with the terms of the controlling legal documents and applicable law, and its actions and decisions that are not arbitrary and capricious shall be binding on any employee, any employee's spouse or other dependent or beneficiary and any other interested parties whether or not in being or under a disability.

16. Indemnification. To the extent permitted by law, all employees, officers, directors, agents and representatives of the Company shall be indemnified by the Company and held harmless against any claims and the expenses of defending against such claims, resulting from any action or conduct relating to the administration of the Plan, whether as a member of the Compensation Committee or otherwise, except to the extent that such claims arise from gross negligence, willful neglect, or willful misconduct.

17. Plan Not an Employment Contract. The Plan is not a contract between the Company and any employee, nor is it a condition of employment of any employee. Nothing contained in the Plan gives, or is intended to give, any employee the right to be retained in the service of the Company, or to interfere with the right of the Company to discharge or terminate the employment of any employee at any time and for any reason. No employee shall have the right or claim to benefits beyond those expressly provided in this Plan, if any. All rights and claims are limited as set forth in the Plan.

18. Severability. In case any one or more of the provisions of this Plan (or part thereof) shall be held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect the other provisions hereof, and this Plan shall be construed as if such invalid, illegal or unenforceable provisions (or part thereof) never had been contained herein.

19. Non-Assignability. No right or interest of any Covered Employee in the Plan shall be assignable or transferable in whole or in part either directly or by operation of law or otherwise, including, but not limited to, execution, levy, garnishment, attachment, pledge or bankruptcy.

20. Integration with Other Pay or Benefits Requirements. The severance payments and benefits provided for in the Plan are the maximum benefits that the Company will pay to Covered Employees on a Covered Termination, except to the extent otherwise specifically provided in a separate agreement. To the extent that the Company owes any amounts in the nature of severance benefits under any other program, policy or plan of the Company that is not otherwise superseded by this Plan, or to the extent that any federal, state or local law, including, without limitation, so-called "plant closing" laws, requires the Company to give advance notice or make a payment of any kind to an employee because of that employee's involuntary termination due to a layoff, reduction in force, plant or facility closing, sale of business, or similar event, the benefits provided under this Plan or the other arrangement shall either be reduced or eliminated to avoid any duplication of payment. The Company intends for the benefits provided under this Plan to partially or fully satisfy any and all statutory obligations that may arise out of an employee's involuntary termination for the foregoing reasons and the Company shall so construe and implement the terms of the Plan.

21. Amendment or Termination. The Board may amend, modify, or terminate the Plan at any time in its sole discretion; provided, however, that (a) any such amendment, modification or termination made prior to a Change in Control that adversely affects the rights of any Covered Employee shall be unanimously approved by the Board, (b) no such amendment, modification or termination may affect the rights of a Covered Employee then receiving payments or benefits under the Plan without the consent of such person, and (c) no such amendment, modification or termination made after a Change in Control shall be effective until the date that is one year following such Change in Control. The Board, with the support of the Compensation Committee, intends to review the Plan at least annually.

22. Governing Law. The Plan and the rights of all persons under the Plan shall be construed in accordance with and under applicable provisions of ERISA, and the regulations thereunder, and the laws of the Commonwealth of Massachusetts (without regard to conflict of laws provisions) to the extent not preempted by federal law.

Exhibit 31.1

CERTIFICATION

I, Jill C. Milne, certify that:

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

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

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

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

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize, and report financial information; and
   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2020

/s/ JILL C. MILNE, PH.D.
Jill C. Milne, Ph.D.
President and Chief Executive Officer (Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Noah C. Clauser, certify that:

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

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

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

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

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize, and report financial information; and
   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 12, 2020

/s/ NOAH C. CLAUSER
Noah C. Clauser
Chief Financial 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 Catabasis Pharmaceuticals, 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 officers of the Company hereby certify, pursuant to 18 U.S.C. Section 1350, that:

(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 12, 2020

/s/ JILL C. MILNE, PH.D.
Jill C. Milne, Ph.D.
President and Chief Executive Officer (Principal Executive Officer)

Date: November 12, 2020

/s/ NOAH C. CLAUSER
Noah C. Clauser
Chief Financial Officer (Principal Financial Officer)