Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended March 31, 2019

OR

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

For the transition period from _____ to _____

Commission File No. 001-35890

Millendo Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**45-1472564**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
301 North Main Street, Suite 100 Ann Arbor, Michigan	**48104**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's telephone number, including area code: (734) 845-9000

(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§229.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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

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	MLND	The Nasdaq Capital Market, LLC

The number of shares of Registrant's Common Stock, $0.001 par value per share, outstanding as of May 10, 2019 was 13,385,797.

INDEX TO FORM 10-Q

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

Unless the context suggests otherwise, references in this Quarterly Report on Form 10-Q, or Quarterly Report, to "Millendo," "the Company," "we," "us," and "our" refer to Millendo Therapeutics, Inc. and, where appropriate, its subsidiaries.

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These forward-looking statements reflect our plans, estimates and beliefs and include, but are not limited to, statements about our plans to develop and commercialize our product candidates; the progress and timing of our ongoing and planned clinical trials for our product candidates, including our plans to amend the protocol for our Phase 2b classic congenital adrenal hyperplasia study and the timing of topline results from the Phase 2b portion of our Phase 2b/3 clinical trial of livoletide in Prader Willi syndrome ("PWS") patients; the timing of and our ability to obtain and maintain regulatory approvals for our product candidates; and our estimates regarding future revenue, if any, future expenses, the funding of our operations, including whether our existing cash, cash equivalents and marketable securities will be sufficient to fund our current operating plans into the second half of 2020 and through the topline results of the Phase 2b portion of our Phase 2b/3 clinical trial of livoletide in PWS patients, as well as our future capital requirements and needs for additional financing. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "anticipates," "believes," "could," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "would" and similar expressions intended to identify forward-looking statements. Forward-looking statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties. Because of these risks and uncertainties, the forward-looking events and circumstances discussed in this report may not transpire. These risks and uncertainties include, but are not limited to, the risks included in this Quarterly Report on Form 10-Q under Part II, Item 1A, "Risk Factors."

Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our estimates and assumptions only as of the date of this document. You should read this document with the understanding that our actual future results may be materially different from what we expect. Except as required by law, we do not undertake any obligation to update or revise any forward-looking statements contained in this report, whether as a result of new information, future events or otherwise.

PART I – Financial Information
Item 1 – Financial Statements

MILLENDO THERAPEUTICS, INC.

Consolidated Balance Sheets
(Unaudited)
(in thousands except share and per share amounts)

	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 65,587	$ 73,286
Short-term restricted cash	45	45
Marketable securities	1,400	4,385
Prepaid expenses and other current assets	4,174	3,373
Refundable tax credit	1,530	2,333
Total current assets	72,736	83,422
Long-term restricted cash	439	439
Operating lease right of use assets	797	—
Other assets	333	213
Total assets	$ 74,305	$ 84,074
Liabilities and stockholders' equity		
Current liabilities:		
Current portion of debt	$ 191	$ 189
Accounts payable	2,344	1,998
Accrued expenses	5,746	7,630
Operating lease liabilities — current	1,004	—
Total current liabilities	9,285	9,817
Debt, net of current portion	325	383
Operating lease liabilities	817	—
Other liabilities	192	752
Total liabilities	10,619	10,952
Commitments and contingencies (Note 6)		
Stockholders' equity:		
Preferred stock, $0.001 par value: 5,000,000 shares authorized; no shares issued and outstanding	—	—
Common stock, $0.001 par value: 100,000,000 shares authorized; 13,357,999 shares issued and outstanding	13	13
Additional paid-in capital	235,815	234,876
Accumulated deficit	(174,452)	(164,086)
Accumulated other comprehensive income	139	148
Total stockholders' equity attributable to Millendo Therapeutics, Inc.	61,515	70,951
Equity attributable to noncontrolling interests	2,171	2,171
Total stockholders' equity	63,686	73,122
Total liabilities and stockholders' equity	$ 74,305	$ 84,074

See accompanying notes to unaudited interim consolidated financial statements

MILLENDO THERAPEUTICS, INC.

Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)
(in thousands except share and per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Operating expenses:		
Research and development	$ 6,204	$ 2,769
General and administrative	4,453	1,619
Loss from operations	10,657	4,388
Other expenses:		
Interest expense (income), net	$ (315)	$ (9)
Other loss	24	7
Net loss	$ (10,366)	$ (4,386)
Net loss attributable to noncontrolling interest	—	125
Net loss attributable to common stockholders	$ (10,366)	$ (4,261)
Net loss per share of common stock, basic and diluted	$ (0.78)	$ (6.00)
Weighted-average shares of common stock outstanding, basic and diluted	13,357,999	710,390
Other comprehensive income:		
Foreign currency translation adjustment	$ (9)	$ 19
Comprehensive loss	$ (10,375)	$ (4,242)
Comprehensive income attributable to noncontrolling interest	$ —	$ 3
Comprehensive loss attributable to Millendo Therapeutics, Inc.	$ (10,375)	$ (4,245)

See accompanying notes to unaudited interim consolidated financial statements

MILLENDO THERAPEUTICS, INC.

Consolidated statements of convertible preferred stock, redeemable noncontrolling interests and stockholders' (deficit) equity
(Unaudited)
(in thousands except share amounts)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Stockholders' (Deficit) Equity attributable to Millendo Therapeutics, Inc.	Total Equity Attributable to Noncontrolling Interests	Total Stockholders' (Deficit) Equity
	Shares	Amount						
Balance at January 1, 2019	13,357,999	$ 13	$ 234,876	$ (164,086)	$ 148	$ 70,951	$ 2,171	$ 73,122
Stock-based compensation expense	—	—	939	—	—	939	—	939
Foreign currency translation adjustment	—	—	—	—	(9)	(9)	—	(9)
Net income (loss)	—	—	—	(10,366)	—	(10,366)	—	(10,366)
Balance at March 31, 2019	13,357,999	$ 13	$ 235,815	$ (174,452)	$ 139	$ 61,515	$ 2,171	$ 63,686

	Convertible Preferred Stock		Redeemable Noncontrolling	Stockholders' Equity (Deficit)									
				Common Stock		Common-1 Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Stockholders' (Deficit) Equity attributable to Millendo Therapeutics, Inc.	Total Equity Attributable to Noncontrolling Interests	Total Stockholders' (Deficit) Equity
	Shares	Amount	Interests	Shares	Amount	Shares	Amount						
Balance at January 1, 2018	90,848,515	$ 132,922	$ 10,584	246,347	$ —	464,043	$ —	$ 6,192	$ (136,894)	$ 8	$ (130,694)	$ 2,171	$ (128,523)
Stock-based compensation expense	—	—	—	—	—	—	—	177	—	—	177	—	177
Foreign currency translation adjustment	—	—	3	—	—	—	—	—	—	16	16	—	16
Net income (loss)	—	—	(125)	—	—	—	—	—	(4,261)	—	(4,261)	—	(4,261)
Balance at March 31, 2018	90,848,515	$ 132,922	$ 10,462	246,347	$ —	464,043	$ —	$ 6,369	$ (141,155)	$ 24	$ (134,762)	$ 2,171	$ (132,591)

See accompanying notes to unaudited interim consolidated financial statements

MILLENDO THERAPEUTICS, INC.

Consolidated Statements of Cash Flows
(Unaudited)
(in thousands)

	Three Months Ended March 31,	
	2019	**2018**
Operating activities:		
Net loss	$ (10,366)	$ (4,386)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	8	6
Stock-based compensation expense	939	177
Amortization of operating lease right of use assets	(68)	—
Unrealized foreign currency gain	—	(3)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(221)	(166)
Accounts payable	398	(202)
Accrued expenses and other liabilities	(1,291)	(423)
Cash used in operating activities	(10,601)	(4,997)
Investing activities:		
Purchase of property and equipment	(10)	(1)
Proceeds from sale of marketable securities	2,986	—
Net cash paid in Alizé asset purchase	—	(524)
Cash provided by (used in) investing activities	2,976	(525)
Financing activities:		
Repayment of debt	(45)	(43)
Payment of financing costs	(15)	—
Cash used in financing activities	(60)	(43)
Effect of foreign currency exchange rate changes on cash	(14)	30
Net decrease in cash, cash equivalents and restricted cash	(7,699)	(5,535)
Cash, cash equivalents and restricted cash at beginning of period	73,770	17,623
Cash, cash equivalents and restricted cash at end of period	$ 66,071	$ 12,088
Supplemental schedule of non-cash financing activities:		
Financing costs in accounts payable and accrued expenses	$ 60	$ 47

See accompanying notes to unaudited interim consolidated financial statements

MILLENDO THERAPEUTICS, INC.
Notes to Unaudited Interim Consolidated Financial Statements

1. Organization and description of business

Description of Business

Millendo Therapeutics, Inc., a Delaware corporation, together with its subsidiaries, is a late-stage biopharmaceutical company focused on developing novel treatments for orphan endocrine diseases where current therapies do not exist or are insufficient. The Company is currently advancing two product candidates for orphan endocrine diseases. The Company's most advanced product candidate, livoletide (AZP-531), is a potential treatment for Prader-Willi syndrome ("PWS"), a rare and complex genetic endocrine disease characterized by hyperphagia, or insatiable hunger. The Company is also developing nevanimibe (ATR-101) as a potential treatment for patients with classic congenital adrenal hyperplasia ("CAH"), a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses. The Company is also investigating nevanimibe for the treatment of patients with endogenous Cushing's syndrome ("CS"), a rare endocrine disease characterized by excessive cortisol production from the adrenal glands.

The Company's operations to date have focused on organization and staffing, business planning, raising capital, acquiring technology and assets, and conducting preclinical studies and clinical trials. The Company does not have any product candidates approved for sale and has not generated any revenue from product sales. The Company's product candidates are subject to long development cycles and the Company may be unsuccessful in its efforts to develop, obtain regulatory approval for or market its product candidates.

The Company is subject to a number of risks including, but not limited to, the need to obtain adequate additional funding for the ongoing and planned clinical development of its product candidates. Because of the numerous risks and uncertainties associated with pharmaceutical products and development, the Company is unable to accurately predict the timing or amount of funds required to complete development of its product candidates, and costs could exceed the Company's expectations for a number of reasons, including reasons beyond the Company's control.

Merger with OvaScience

In December 2018, OvaScience, Inc., a Delaware corporation ("OvaScience"), now known as Millendo Therapeutics, Inc. (the "Company"), completed its merger (the "Merger") with privately-held Millendo Therapeutics, Inc. ("Private Millendo"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated August 8, 2018, as amended on September 25, 2018 and November 1, 2018 (the "Merger Agreement"), whereby Orion Merger Sub, Inc., a Delaware corporation and wholly-owned subsidiary of OvaScience (the "Merger Sub"), merged with and into Private Millendo, with Private Millendo continuing as a wholly owned subsidiary of OvaScience.

Under the terms of the Merger Agreement, OvaScience issued shares of its common stock to Private Millendo's stockholders, at an exchange ratio of 0.0744 shares of OvaScience common stock, for each share of Private Millendo common stock outstanding immediately prior to the Merger. OvaScience also assumed all of the stock options outstanding under the Private Millendo 2012 Equity Incentive Plan, as amended (the "Private Millendo Plan"), with such stock options henceforth representing the right to purchase a number of shares of OvaScience's common stock equal to 0.0744 multiplied by the number of shares of Private Millendo common stock previously represented by such options.

The Company's shares of common stock listed on The Nasdaq Capital Market, previously trading through the close of business on Friday, December 7, 2018 (the "Merger Date") under the ticker symbol "OVAS," commenced trading on The Nasdaq Capital Market, under the ticker symbol "MLND," on Monday, December 10, 2018.

The Merger was accounted for as a reverse acquisition and recapitalization, with Private Millendo being treated as the accounting acquirer. As such, the results of operations and cash flows prior to the Merger Date, relate to Private Millendo and its subsidiaries. Subsequent to the Merger Date the information relates to the consolidated entities of Millendo Therapeutics, Inc. All share and per share amounts in the unaudited interim consolidated financial statements

and related notes have been retroactively adjusted, where applicable, for all periods presented to give effect to the exchange ratio applied in connection with the Merger.

Liquidity

The Company has incurred net losses since inception and it expects to generate losses from operations for the foreseeable future primarily due to research and development costs for its potential product candidates. As of March 31, 2019, the Company had cash, cash equivalents and marketable securities of $67.0 million and an accumulated deficit of $174.5 million.

In April 2019, the Company entered into an "at-the-market" ("ATM") equity distribution agreement with Citigroup Global Markets Inc. acting as sole agent with an aggregate offering value of up to $50.0 million which allows the Company to sell its common shares through the facilities of the Nasdaq Capital Market. Subject to the terms of the equity distribution agreement, the Company is able to determine, at its sole discretion, the timing and number of shares to be sold under this ATM facility.

The Company will likely require additional capital in the future through equity or debt financings, partnerships, collaborations, or other sources to carry out the Company's planned development activities. If additional capital is not secured when required, the Company may need to delay or curtail its operations until such funding is received. Various internal and external factors will affect whether and when the Company's product candidates become approved drugs. The regulatory approval and market acceptance of the Company's proposed future products (if any), length of time and cost of developing and commercializing these product candidates and/or failure of them at any stage of the drug approval process will materially affect the Company's financial condition and future operations. The Company believes its cash, cash equivalents and marketable securities at March 31, 2019 are sufficient to fund operations into the second half of 2020.

2. Basis of presentation and summary of significant accounting policies

Basis of presentation and consolidation principles

The accompanying unaudited interim consolidated financial statements include the accounts of Millendo Therapeutics, Inc. and its subsidiaries, and all intercompany amounts have been eliminated. The unaudited interim consolidated financial statements have been prepared in conformity with U.S. generally accepted accounting principles ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB"). The unaudited interim consolidated financial statements include the accounts of the Company's subsidiaries in which the Company holds a controlling financial interest as of the financial statement date.

Unaudited interim financial statements

The Company has prepared the accompanying unaudited interim consolidated financial statements based on Securities and Exchange Commission ("SEC") rules that permit reduced disclosure for interim periods. These unaudited interim consolidated financial statements include, in the Company's opinion, all adjustments, consisting only of normal recurring adjustments that the Company considers necessary for a fair presentation of its consolidated financial position and results of operations for these periods. The Company's historical results are not necessarily indicative of the results to be expected in the future and the Company's operating results for the three months ended March 31, 2019 are not necessarily indicative of the results that may be expected for the year ending December 31, 2019.

The consolidated balance sheet at December 31, 2018 was derived from audited financial statements, but does not include all disclosure required by GAAP. The accompanying unaudited interim consolidated financial statements should be read in conjunction with the annual audited consolidated financial statements and related notes as of and for the year ended December 31, 2018 included in the Company's Annual Report on Form 10-K filed with the SEC on April 1, 2019.

Since the date of such financial statements, there have been no changes to the Company's significant accounting policies except as follows:

Leases

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842),* which requires the Company as the lessee to recognize most leases on the balance sheet thereby resulting in the recognition of right of use assets and lease obligations for those leases currently classified as operating leases. ASU 2016-02 became effective for the Company on January 1, 2019.

The Company adopted ASU 2016-02 using a modified retrospective transition approach as of January 1, 2019 and will not restate its comparative period financial information for effects of the standard or make the new required lease disclosures for periods before the date of adoption. The Company has elected to adopt the package of transition practical expedients and, therefore, have not reassessed (1) whether existing or expired contracts contain a lease, (2) lease classification for existing or expired leases or (3) the accounting for initial direct costs that were previously capitalized. As a result of the adoption of ASC 842, the Company recognized an operating lease liability of $2.0 million based on the present value of the minimum rental payments of the leases and a corresponding operating lease right of use ("ROU") asset of $0.9 million within the Company's consolidated balance sheet. The Company's ROU asset is exclusive of any early lease termination obligations whereby future contractual lease payments exceed estimated sublease income.

Under ASC 842, the Company determines if an arrangement is a lease at contract inception. A lease exists when a contract conveys to the customer the right to control the use of identified property, plant, or equipment for a period of time in exchange for consideration. The definition of a lease embodies two conditions: (1) there is an identified asset in the contract that is land or a depreciable asset (i.e., property, plant, and equipment), and (2) the customer has the right to control the use of the identified asset.

The lease liabilities are initially and subsequently measured at the present value of the unpaid lease payments at the lease commencement date. When readily determinable, the Company uses the implicit rate in determining the present value of lease payments. When leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the lease commencement date, including the lease term.

The ROU asset is initially measured at cost, which comprises the initial amount of the lease liability adjusted for lease payments made at or before the lease commencement date, plus any initial direct costs incurred less any lease incentives received. For operating leases, the ROU asset is subsequently measured throughout the lease term at the carrying amount of the lease liability, plus initial direct costs, plus (minus) any prepaid (accrued) lease payments, less the unamortized balance of lease incentives received. Lease expense for lease payments is recognized on a straight-line basis over the lease term.

The Company has noncancelable operating leases for office and laboratory space which have remaining lease terms between two and seven years. In connection with the Merger, the Company assumed a sublease agreement for office and laboratory space located in Waltham, Massachusetts. The term of the sublease expires in November 2020. In February 2019 and October 2018, the Company entered into two additional noncancelable operating leases for office space; one that the Company took possession of in April 2019, and the other that the Company will take possession of in July 2019.

As of March 31, 2019, the lease liabilities and the ROU asset are $1.8 million and $0.8 million, respectively. The discount rate used to account for the Company's operating leases under ASC 842 is the Company's estimated incremental borrowing rate of 7.0%. The Company has options to extend certain of its leases for another five to nine years. These options to extend were not recognized as part of the Company's measurement of the ROU assets and operating lease liabilities for the three months ended March 31, 2019 nor were the future payments related to the leases the Company entered into in February 2019 and October 2018.

Rent expenses related to the Company's operating leases was approximately $67,000 and $48,000 for the three months ended March 31, 2019 and 2018, respectively. Cash paid for amounts included in the measurement of the lease liabilities was approximately $0.2 million and the Company received approximately $71,000 in sublease payments related to its Waltham, Massachusetts lease during the three months ended March 31, 2019. The weighted average remaining term of

the Company's noncancelable operating leases is 2.45 years. Future minimum rental payments under the Company's noncancelable operating leases at March 31, 2019 is as follows (amounts in thousands):

2019	$ 773
2020	969
2021	45
2022	45
2023	45
Thereafter	90
Total	1,967
Present Value Adjustment	(146)
Lease liability at March 31, 2019	$ 1,821

Future minimum rental payments under the Company's noncancelable operating leases at December 31, 2018 is as follows (in thousands):

2019	$ 1,208
2020	1,327
2021	414
2022	425
2023	436
Thereafter	293
	$ 4,103

Use of estimates

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and reported amounts of expenses during the reporting period. Actual results could differ from those estimates. Due to the uncertainty of factors surrounding the estimates or judgments used in the preparation of the consolidated financial statements, actual results may materially vary from these estimates. Estimates and assumptions are periodically reviewed and the effects of revisions are reflected in the financial statements in the period they are determined to be necessary.

Net loss per share

Basic loss per share of common stock is computed by dividing net loss attributable to common stockholders by the weighted-average number of shares of common stock (including shares of common-1 stock during the three months ended March 31, 2018) outstanding during each period. Diluted loss per share of common stock includes the effect, if any, from the potential exercise or conversion of securities, such as convertible preferred stock, preferred stock warrants, restricted stock, and stock options, which would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share, the weighted-average number of shares of common stock remains the same for both calculations due to the fact that when a net loss exists, dilutive shares are not included in the calculation as the impact is anti-dilutive.

The following potentially dilutive securities have been excluded from the computation of diluted weighted-average shares of common stock outstanding, as they would be anti-dilutive:

	Three months ended March 31,	
	2019	2018
Stock options	2,381,288	701,678
Convertible preferred stock	—	6,759,109
Common stock warrants	17,125	—
Preferred stock warrants	—	17,125
BSA and BSPCE warrants	156,719	156,719
	2,555,132	7,634,631

Recent accounting pronouncements

In August 2018, the FASB issued ASU No. 2018-13, *Disclosure Framework - Changes to the Disclosure Requirements for Fair Value Measurement (Topic 820)*. ASU 2018-13 resulted in certain modifications to fair value measurement disclosures, primarily related to level 3 fair value measurements. The new standard is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019, and early adoption is permitted. The Company is currently evaluating the potential impact of the adoption of this standard on its disclosures.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230) Classification of Certain Cash Receipts and Cash Payments,* which makes eight targeted changes to how cash receipts and cash payments are presented and classified in the statement of cash flows. This standard was effective January 1, 2019 and required adoption on a retrospective basis unless it was impracticable to apply, in which case the Company would be required to apply the amendments prospectively as of the earliest date practicable. The Company adopted this standard which did not have a material impact on its consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments – Credit Losses (Topic 326)*, which replaces the incurred loss impairment methodology in current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. Additionally, ASU 2016-13 requires a financial asset measured at amortized cost basis to be presented at the net amount expected to be collected through the use of an allowance of expected credit losses. ASU 2016-13 is effective for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years, and requires a modified retrospective approach. The Company is in the process of evaluating the impact of this new guidance on its consolidated financial statements and disclosures.

3. Fair value measurements

The following table presents the Company's assets and liabilities that are measured at fair value on a recurring basis (amounts in thousands):

	March 31, 2019		
	(Level 1)	(Level 2)	(Level 3)
Assets			
Money market funds (included in cash and cash equivalents)	$ 53,365	$ —	$ —
Marketable securities - U.S. government agency	$ —	$ —	$ —
Marketable securities - Corporate debt securities	$ —	$ 1,400	$ —

	December 31, 2018		
	(Level 1)	(Level 2)	(Level 3)
Assets			
Money market funds (included in cash and cash equivalents)	$ 25,145	$ —	$ —
Marketable securities - U.S. government agency	$ —	$ 2,994	$ —
Marketable securities - Corporate debt securities	$ —	$ 1,391	$ —

Prior to the Merger in December 2018, the Company's preferred stock warrants were liability classified and remeasured at each reporting period using level 3 inputs. There were no changes in the fair value of the preferred stock warrant liability during the three months ended March 31, 2018.

4. Accrued expenses

Accrued expenses consist of (amounts in thousands):

	March 31, 2019	December 31, 2018
Compensation and related benefits	$ 2,064	$ 3,537
Professional fees	1,598	1,140
Preclinical and clinical costs	1,524	1,811
Lease termination	—	630
Other	560	512
Total	$ 5,746	$ 7,630

In connection with the adoption of ASC 842 on January 1, 2019, the lease termination balance as of December 31, 2018 was reclassified into the Company's operating lease liability.

5. Debt

Bpifrance Reimbursable Advance

In December 2017, in connection with its acquisition of Alizé Pharma SAS ("Alizé"), the Company assumed €0.7 million of debt that Alizé had outstanding with Bpifrance Financing ("Bpifrance"). The original advance amount of €0.8 million ("the Bpifrance Advance") was provided to Alizé as an innovation aid that required Alizé to carry out certain activities related to its livoletide clinical development program and incur a certain level of program expenditures. No interest is charged or accrued under the advance.

The Company is required to make quarterly principal payments, which began in December 2016 and continue through September 2021. The quarterly principal payments escalate over the repayment period beginning with €17,500 per quarter and increasing to €50,000 through maturity. In addition to the quarterly payments, beginning January 1, 2016, Bpifrance may require the Company to pay, by no later than March 31st of each year, a reimbursement annuity equal to 20% of the proceeds generated by the Company from license, assignment or use of livoletide. Under no circumstance, however, would the Company be required to reimburse to Bpifrance principal amounts greater than the original advance it received.

The Company is permitted to repay the Bpifrance Advance at any time, at which point it would be released from all commitments and obligations under the Bpifrance Advance agreement. The Bpifrance Advance Agreement does not contain any ongoing financial covenants.

During the three months ended March 31, 2019 and 2018, the Company made principal payments of $45,000 and $43,000, respectively. At March 31, 2019, the balance outstanding was $0.5 million (€0.5 million).

6. Litigation

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, penalties, and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated.

On November 9, 2016, a purported shareholder derivative action was filed in the Business Litigation Session of the Suffolk County Superior Court in the Commonwealth of Massachusetts (*Cima v. Dipp*, No. 16-3443-BLS1 (Mass. Sup. Ct.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) and OvaScience as a nominal defendant alleging breaches of fiduciary duty, unjust enrichment, abuse of control, gross mismanagement and corporate waste for purported actions related to OvaScience's January 2015

follow-on public offering. On February 22, 2017, the court approved the parties' joint stipulation to stay all proceedings in the action until further notice. Following a status conference in December 2017, the stay was lifted. On January 25, 2018, at the parties' request, the court entered a second order staying all proceedings in the action until further order of the court. The Company believes that the complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On March 24, 2017, a purported shareholder class action lawsuit was filed in the U.S. District Court for the District of Massachusetts (*Dahhan v. OvaScience, Inc.*, No. 1:17-cv-10511-IT (D. Mass.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) alleging violations of Sections 10(b) and 20(a) of the Exchange Act (the "Dahhan Action"). On July 5, 2017, the court entered an order approving the appointment of Freedman Family Investments LLC as lead plaintiff, the firm of Robins Geller Rudman & Dowd LLP as lead counsel and the Law Office of Alan L. Kovacs as local counsel. Plaintiff filed an amended complaint on August 25, 2017. The Company filed a motion to dismiss the amended complaint, which the court denied on July 31, 2018. On August 14, 2018, the Company answered the amended complaint. The parties presently are engaged in discovery. The Company believes that the amended complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. A resolution of this lawsuit adverse to the Company or the other defendants could have a material effect on the Company's consolidated financial position and results of operations. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On July 27, 2017, a purported shareholder derivative complaint was filed in the U.S. District Court for the District of Massachusetts (*Chiu v. Dipp*, No. 1:17-cv-11382-IT (D. Mass.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) as a nominal defendant alleging breach of fiduciary duty, unjust enrichment and violations of Section 14(a) of the Exchange Act alleging that compensation awarded to the director defendants was excessive and seeking redress for purported actions related to OvaScience's January 2015 follow-on public offering and other public statements. On September 26, 2017, the plaintiff filed an amended complaint which eliminated all claims regarding allegedly excessive director pay and additionally alleged claims of abuse of control and corporate waste. On October 27, 2017, the defendants filed a motion to dismiss the amended complaint. The court heard oral argument on the motion to dismiss on April 5, 2018. On April 13, 2018, the court granted the defendants' motion to dismiss the amended complaint for failure to state a claim for relief under Section 14(a). The court also dismissed the plaintiffs' pendent state law claims without prejudice, based on lack of subject matter jurisdiction. On April 25, 2018, the plaintiffs moved for leave to amend the complaint, and to stay this case pending the outcome of the Dahhan Action. The Company does not believe that the proposed amended complaint cures the defects in the current complaint, but informed plaintiffs' counsel that, in the interest of judicial economy, defendants would not oppose the proposed amendment if the court would consider staying the case pending the resolution of the Dahhan Action. On April 27, 2018, the court granted the plaintiffs' motion for leave to amend the complaint and for a stay. On April 30, 2018, the plaintiffs filed their second amended complaint. On May 23, 2018, the court entered an order staying this case pending the resolution of the Dahhan Action. The Company believes that the complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

Between October 16, 2018 and November 21, 2018, five putative class action lawsuits were filed in various federal District Courts against OvaScience, Inc. and the OvaScience Board of Directors related to OvaScience's proposed merger with Millendo Therapeutics, Inc.: *Cunningham v. Kroeger, et al.*, No. 1:18-cv-01595 (D. Del. filed Oct. 16, 2018); *Adlard v. OvaScience, Inc., et al.*, No. 1:18-cv-12332 (D. Mass. filed Nov. 6, 2018); *Wheby v. OvaScience, Inc., et al.*, No. 1:18-cv-1811 (D. Del. filed Nov. 16, 2018); *Cuenca Aubets v. OvaScience, Inc., et al.*, No. 1:18-cv-10882 (S.D.N.Y. filed Nov. 20, 2018); and *Kim v. OvaScience, Inc., et al.*, No. 1:18-cv-10939 (S.D.N.Y. filed Nov. 21, 2018). The Complaints each alleged violations of Section 14(a) of the Securities Exchange Act of 1934 and Rule 14a-9 promulgated thereunder, and as against the individual defendants, violations of Section 20(a) of the Securities Exchange Act of 1934. The Cunningham plaintiff alleged that OvaScience's Form S-4 Registration Statement filed on September 26, 2018 omitted or misrepresented material information regarding OvaScience's proposed merger with Millendo Therapeutics, Inc. The Adlard, Wheby, Cuenca Aubets and Kim plaintiffs alleged that OvaScience's Definitive Proxy Statement on Schedule 14A filed on November 6, 2018, omitted or misrepresented material information regarding OvaScience's proposed merger with Millendo Therapeutics, Inc. OvaScience subsequently supplemented its disclosures. The Cunningham plaintiff voluntarily dismissed his complaint on December 10, 2018, and the Wheby plaintiff

voluntarily dismissed his complaint on February 28, 2019. On March 18, 2019, the court dismissed the Cuenca Aubets and Kim actions for failure to serve. The Company is in negotiations with counsel for the plaintiffs regarding their demands for attorneys' fees. There can be no assurance that the negotiations will be successful. If the negotiations are not successful, the Company may be required to litigate the fee applications and/or the underlying actions.

In addition to the matters described above, the Company may be a party to litigation and subject to claims incident to the ordinary course of business from time to time. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors.

7. Stock-based compensation

In December 2018, the Company assumed Private Millendo's 2012 Stock Plan, as amended (the "Millendo Plan"). There were 1,494,431 authorized shares of common stock to be issued under the Millendo Plan. In addition, the Company's 2012 Stock Incentive Plan, as amended (the "2012 Plan") will continue. The number of shares of the Company's common stock that are reserved for issuance under the 2012 Plan is equal to the sum of (1) 96,883 shares of common stock issuable under the 2012 Plan plus the number of shares of the Company's common stock subject to outstanding awards under the 2011 Stock Incentive Plan (the "2011 Plan"), that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right (up to 45,308 shares) plus (2) an annual increase, to be added on the first day of each year beginning in 2013 and each subsequent anniversary until the expiration of the 2012 Plan, equal to the lowest of 65,000 shares of its common stock, 4.0% of the number of shares of the Company's common stock outstanding on the first day of the year and an amount determined by the Company's board of directors.

The Millendo Plan and the 2012 Plan provide for the issuance of stock options, stock appreciation rights, restricted stock units and other stock-based or cash awards to purchase shares of common stock to eligible employees, officers, directors and consultants. As of March 31, 2019 there were 259,091 shares of common stock available for future issuance under both plans in the aggregate. The amount, terms of grants, and exercisability provisions are determined and set by the Company's board of directors.

The Company measures employee and nonemployee stock-based awards at grant-date fair value and records compensation expense on a straight-line basis over the vesting period of the award. Stock-based awards issued to nonemployees are revalued until the award vests.

The Company recorded stock-based compensation expense in the following expense categories of its accompanying consolidated statements of operations and comprehensive loss for the three months ended March 31, 2019 and 2018 (amounts in thousands):

	Three months ended March 31,	
	2019	2018
Research and development	$ 423	$ 71
General and administrative	516	106
Total	$ 939	$ 177

Stock options

Options issued under both plans may have a contractual life of up to 10 years and may be exercisable in cash or as otherwise determined by the board of directors. Vesting generally occurs over a period of not greater than four years.

The following table summarizes the activity related to stock option grants to employees and nonemployees for the three months ended March 31, 2019:

	Shares	Weighted average exercise price per share	Weighted-average remaining contractual life (years)
Outstanding at December 31, 2018	1,764,287	$ 26.81	8.0
Granted	705,133	9.21	
Forfeited	(88,132)	59.41	
Outstanding at March 31, 2019	2,381,288	$ 20.39	7.8
Vested and exercisable at March 31, 2019	891,961	$ 34.63	5.1
Vested and expected to vest at March 31, 2019	2,381,288	$ 20.39	7.8

As of March 31, 2019, the unrecognized compensation cost related to 1,489,327 unvested stock options expected to vest was $10.4 million. This unrecognized compensation will be recognized over an estimated weighted-average amortization period of 3.3 years. There were no options exercised during the three months ended March 31, 2019. The aggregate intrinsic value of options outstanding and options exercisable as of March 31, 2019 was $11.1 million and $5.6 million, respectively. The options granted during the three months ended March 31, 2019 had an estimated weighted average grant date fair value of $6.45. There were no options granted during the three months ended March 31, 2018. The grant date fair value of each option grant was estimated during the three months ended March 31, 2019 using the following assumptions within the Black-Scholes option-pricing model:

	Three months ended March 31, 2019
Expected term (in years)	6.05
Expected volatility	80 %
Risk-free interest rate	2.55 %
Expected dividend yield	0 %

At the time of the Alizé acquisition in December 2017, Alizé had 6,219 non-employee (BSA) warrants and 5,360 employee (BSPCE) warrants outstanding, which have weighted-average exercise prices of €80.06 and €83.40, respectively. As of March 31, 2019, all BSAs and BSPCEs were vested. As of March 31, 2019, there were an aggregate of 156,719 shares of common stock issuable upon the exercise of the warrants with a weighted-average exercise price of $6.76 per share. These instruments are included in the equity attributable to noncontrolling interests.

8. Subsequent events

In April 2019, the Company entered into a lease agreement for office space in Lexington, Massachusetts to expand its operations. Monthly rental payments will commence in May 2019 and continue through the lease term, which is scheduled to end in September 2020. The Company has the right to terminate the lease at any time and upon prior written notice. The lease does not provide for an extension or renewal. The Company will make future rental payments of $74,000 and $96,000 in fiscal 2019 and 2020, respectively.

In May 2019, the Company funded an escrow totaling approximately $1.0 million in connection with a sublease agreement for office space located in Waltham, Massachusetts assumed in connection with the Merger. The escrowed amount represents the difference in the annual base rent payable under the sublease agreement and the annual base rent payable under the overlease for the remaining term of the sublease agreement. The escrowed amount will be released on a monthly basis over the remaining lease term as the annual base rent payments are made by the Company.

Subsequent events were evaluated through the filing date of this Quarterly Report.

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

You should read the following discussion of our financial condition and results of operations in conjunction with our interim unaudited consolidated financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our annual audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission on April 1, 2019. In addition to historical financial information, the following discussion contains forward-looking statements that reflect our plans, estimates, beliefs and expectations that involve risks and uncertainties. Our actual results and the timing of events could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to these differences include those discussed below and elsewhere in this Quarterly Report and in our Annual Report on Form 10-K, particularly in Item 1A. "Risk Factors" and "Special Note Regarding Forward-Looking Statements."

Overview

We are a late-stage biopharmaceutical company focused on developing novel treatments for orphan endocrine diseases where current therapies do not exist or are insufficient. We are currently advancing two product candidates for orphan endocrine diseases. Our most advanced product candidate, livoletide (AZP-531), is a potential treatment for Prader-Willi syndrome ("PWS"), a rare and complex genetic endocrine disease characterized by hyperphagia, or insatiable hunger, that contributes to serious complications, a significant burden on patients and caregivers and early mortality. In a randomized, double-blind, placebo-controlled Phase 2 clinical trial in 47 patients with PWS, we observed that administration of livoletide once daily was associated with a clinically meaningful improvement in hyperphagia, as well as a reduction in appetite. In a pre-specified analysis of 38 home-resident PWS patients from the Phase 2 trial, we observed a larger and statistically significant decrease in hyperphagia following administration of livoletide as compared to placebo. In March 2019, we announced that we initiated a pivotal Phase 2b/3 clinical trial of livoletide in PWS patients, with topline results from the Phase 2b portion of the study expected in the first half of 2020.

We are also developing nevanimibe (ATR-101) with a primary focus on treating patients with classic congenital adrenal hyperplasia ("CAH"), a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses. These chronic high doses of cortisol can result in side effects that include diabetes, obesity, hypertension and psychological problems. When on suboptimal doses of cortisol, female CAH patients can experience hirsutism, infertility and menstrual irregularity, and male CAH patients can experience testicular atrophy, infertility and testicular tumors, making it difficult for physicians to appropriately treat CAH without causing adverse consequences. We reported results from our Phase 2 clinical trial of nevanimibe in patients with CAH in March 2018 and initiated a Phase 2b trial in the third quarter of 2018. We are also investigating nevanimibe for the treatment of patients with endogenous Cushing's syndrome ("CS"), a rare endocrine disease characterized by excessive cortisol production from the adrenal glands.

Since inception, we have incurred significant operating losses and negative operating cash flows and there is no assurance that we will ever achieve or sustain profitability. Our net losses were $10.4 million and $4.4 million for the three months ended March 31, 2019 and 2018, respectively. As of March 31, 2019, we had an accumulated deficit of $174.5 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future.

Recent Developments

Livoletide (AZP-531) for the treatment of Prader-Willi syndrome (PWS)

We have initiated pre-clinical activities in support of the development of a multi-dose pen device to improve patient and caregiver convenience and further simplify administration of livoletide (AZP-531), which we are developing as a potential treatment for PWS, a rare and complex genetic endocrine disease characterized by hyperphagia, or insatiable hunger, that contributes to serious complications, a significant burden on patients and caregivers and early mortality.

Nevanimibe for the treatment of classic congenital adrenal hyperplasia (CAH)

The nevanimibe Phase 2b CAH study began in the third quarter of 2018 and is ongoing. Preliminary data with a starting dose of 1000 mg BID (twice per day) has demonstrated lower tolerability than expected based on prior clinical experience with nevanimibe. Three of six subjects in the Phase 2b CAH study discontinued the study after beginning treatment as a result of non-serious adverse events, primarily of rash, dysuria, or diarrhea. Patients treated at 500 mg BID in previously completed studies tolerated nevanimibe well. We believe that dose escalation from a starting dose of 500 mg BID to higher planned doses may allow patients to better tolerate the therapy. We plan to amend the Phase 2b CAH protocol. Enrollment will be paused while a protocol amendment is submitted to appropriate regulatory authorities and ethics committees. Patients currently enrolled in the study will continue per protocol and may dose escalate to 1500 mg BID and 2000 mg BID. To date, two subjects have received both 1500 mg BID and 2000 mg BID and have completed or are near completing the 12-week study duration.

Previous experience with nevanimibe includes a Phase 2 CAH study where patients started at 125 mg BID and dose escalated to 1000 mg BID, and a healthy volunteer drug-drug interaction study with nevanimibe at 500 mg BID for 11 days. In addition, the Phase 1 ACC study dosed subjects over 2.5 times above the highest dose being explored in the Phase 2b CAH study.

We are currently evaluating the timeline implications of the proposed protocol amendment.

Merger

On December 7, 2018, OvaScience, Inc., or OvaScience, now known as Millendo Therapeutics, Inc., completed its reverse merger or, the Merger, with what was then known as "Millendo Therapeutics, Inc.," or Private Millendo, in accordance with the terms of the Agreement and Plan of Merger and Reorganization dated as of August 8, 2018, as amended on September 25, 2018 and November 1, 2018, or the Merger Agreement. OvaScience's shares of common stock listed on The Nasdaq Capital Market, previously trading through the close of business on Friday, December 7, 2018 under the ticker symbol "OVAS," commenced trading on The Nasdaq Capital Market, under the ticker symbol "MLND," on Monday, December 10, 2018.

Immediately following the Merger, Private Millendo became a wholly-owned subsidiary of OvaScience. Upon consummation of the Merger, or the Closing, OvaScience adopted the business plan of Private Millendo and discontinued the pursuit of OvaScience's business plan pre-Closing. The Merger was accounted for as a reverse recapitalization with Private Millendo as the accounting acquirer. On the Merger date, the primary pre-combination assets of OvaScience was cash, cash equivalents and marketable securities. At the time of the Merger, OvaScience had net assets of $38.0 million, which were comprised primarily of cash, cash equivalents and marketable securities.

Components of Results of Operations

Research and development expense

Research and development expense consists primarily of costs incurred in connection with the development of our product candidates. We expense research and development costs as incurred. These expenses include:

- personnel expenses, including salaries, benefits and stock-based compensation expense;
- costs of funding research performed by third parties, including pursuant to agreements with contract research organizations, or CROs, as well as investigative sites and consultants that conduct our preclinical studies and clinical trials;
- expenses incurred under agreements with contract manufacturing organizations, or CMOs, including manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical study and clinical trial materials;

- payments made under our third-party licensing agreements, other than amounts classified as acquired in-process research and development expenses;
- consultant fees and expenses associated with outsourced professional scientific development services;
- expenses for regulatory activities, including filing fees paid to regulatory agencies; and
- allocated expenses for facility costs, including rent, utilities, depreciation and maintenance.

Milestone payment obligations incurred prior to regulatory approval of a product candidate, which are accrued when the event requiring payment of the milestone occurs are included in research and development expense.

We typically use our employee, consultant and infrastructure resources across our development programs. We track certain outsourced development costs by product candidate, but do not allocate all personnel costs or other internal costs to specific product candidates.

The following table summarizes our research and development expenses by product candidate, personnel expense and other expenses for the three months ended March 31, 2019 and 2018:

	Three Months Ended March 31,			
	2019	2018	Change	
	(dollars in thousands)			
Nevanimibe expenses	$ 818	$ 1,268	$ (450)	(35.5)%
Livoletide expenses	3,003	438	2,565	585.6
Personnel expenses	1,879	929	950	102.3
Other expenses	504	134	370	276.1
Total	$ 6,204	$ 2,769	$ 3,435	124.1 %

We expect our research and development expense will increase for the foreseeable future as we seek to advance development of our product candidates. The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of livoletide or nevanimibe. We are also unable to predict when, if ever, material net cash inflows may commence from sales of livoletide, nevanimibe or any future product candidates that we may develop due to the numerous risks and uncertainties associated with clinical development, including risks and uncertainties related to:

- the number of clinical sites included in the trials;
- the length of time required to enroll suitable patients;
- the number of patients that ultimately participate in the trials;
- the number of doses patients receive;
- the duration of patient follow-up and number of patient visits;
- the results of our clinical trials;
- the establishment of commercial manufacturing capabilities;
- the receipt of marketing approvals; and
- the commercialization of product candidates.

We may never succeed in obtaining regulatory approval for livoletide, nevanimibe or any future product candidates we may develop. Product candidates in later stages of clinical development, like livoletide, generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

General and administrative expense

General and administrative expense consists primarily of personnel expenses, including salaries, benefits and stock-based compensation expense, for employees in executive, finance, accounting, business development, legal and human resource functions. General and administrative expense also includes corporate facility costs, including rent, utilities, depreciation and maintenance, not otherwise included in research and development expense, as well as legal fees related to intellectual property and corporate matters and fees for accounting, recruiting and consulting services.

We anticipate that our general and administrative expense will increase as a result of increased headcount, expanded infrastructure and higher accounting, legal, consulting and investor relations fees, as well as increased director and officer insurance premiums, associated with being a public company. We also anticipate that our general and administrative expense will increase as we support additional clinical trials for livoletide and nevanimibe. In addition, if and when we believe that regulatory approval of livoletide or nevanimibe appears likely, we anticipate an increase in headcount and expense as a result of our preparation for commercial operations.

Interest expense (income), net

Interest income represents amounts earned on our cash, cash equivalents and marketable securities balances.

Results of operations

Comparison of the three months ended March 31, 2019 and 2018

	Three Months Ended March 31,			
	2019	2018	Change	
	(dollars in thousands)			
Operating expenses:				
Research and development	$ 6,204	$ 2,769	$ 3,435	124.1 %
General and administrative	4,453	1,619	2,834	175.0
Loss from operations	10,657	4,388	6,269	142.9
Other expenses:				
Interest expense (income), net	(315)	(9)	(306)	*
Other loss	24	7	17	242.9
Net loss	$ (10,366)	$ (4,386)	$ (5,980)	136.3 %

*Not meaningful

Research and development expense

Research and development expense increased by $3.4 million to $6.2 million for the three months ended March 31, 2019 from $2.8 million for the three months ended March 31, 2018. The following table summarizes our research and development expenses for the three months ended March 31, 2019 and 2018:

	Three Months Ended March 31,			
	2019	2018	Change	
	(dollars in thousands)			
Preclinical and clinical development expense	$ 4,093	$ 1,706	$ 2,387	139.9 %
Compensation expense, other than stock-based compensation	1,456	858	598	69.7
Stock-based compensation expense	423	71	352	*
Other expenses	232	134	98	73.1
Total research and development expense	$ 6,204	$ 2,769	$ 3,435	124.1 %

*Not meaningful

The increase in total research and development expense is attributable to:

- a $1.0 million increase in compensation and stock-based compensation expenses as a result of our increase in research and development headcount and additional options granted;
- a $0.1 million increase in other expense due to facility and other overhead expenses; and
- a $2.4 million increase in preclinical and clinical development expense mainly related to the development of livoletide.

General and administrative expense

General and administrative expense increased by $2.8 million to $4.5 million for the three months ended March 31, 2019 from $1.6 million for the three months ended March 31, 2018. The increase was primarily due to a $1.4 million increase in professional fees incurred mainly related to being a publicly traded company, a $0.8 million increase in compensation and stock-based compensation expense as a result of our increase in general and administrative headcount and changes to compensation arrangements, and a $0.6 million increase in insurance, rent and facility related expenses due to increased headcount and operating as a public company.

Interest expense (income), net

Interest income increased by $0.3 million to $0.3 million for the three months ended March 31, 2019 from $9,000 for the three months ended March 31, 2018. The increase was primarily due to larger cash, cash equivalent and marketable securities balances we had immediately following the Merger.

Other loss

Other loss increased by $17,000 to $24,000 for the three months ended March 31, 2019 primarily due to higher foreign currency losses as a result of exchange rate fluctuations on transactions denominated in a currency other than our functional currency.

Liquidity and Capital Resources

The following table sets forth the primary uses of cash and cash equivalents for each year set forth below:

	Three Months Ended March 31,	
	2019	2018
	(in thousands)	
Net cash used in operating activities	$ (10,601)	$ (4,997)
Net cash provided by (used in) investing activities	2,976	(525)
Net cash used in financing activities	(60)	(43)
Effect of foreign currency exchange rate changes on cash	(14)	30
Net decrease in cash, cash equivalents and restricted cash	$ (7,699)	$ (5,535)

Uses of funds

Operating activities

During the three months ended March 31, 2019, we used $10.6 million of cash in operating activities. Cash used in operating activities reflected our net loss of $10.4 million and a net increase in operating assets and liabilities of $1.1 million, offset by non-cash charges of $0.9 million, principally related to stock-based compensation.

During the three months ended March 31, 2018, we used $5.0 million of cash in operating activities. Cash used in operating activities reflected our net loss of $4.4 million and a net increase in operating assets and liabilities of $0.8 million, offset by non-cash charges of $0.2 million, principally related to stock-based compensation.

Investing activities

During the three months ended March 31, 2019, we received $3.0 million in net proceeds from the sale of marketable securities and paid $10,000 in purchases of property and equipment.

During the three months ended March 31, 2018, we made remaining payments of $0.5 million in connection with the asset acquisition of Alizé.

Financing activities

During the three months ended March 31, 2019, we used cash of $45,000 in principal loan repayments and $15,000 in the payment of financing costs.

During the three months ended March 31, 2018, we used cash of $43,000 in principal loan repayments.

Funding requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we may would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

In April 2019, we entered into an "at-the-market", or ATM, equity distribution agreement with Citigroup Global Markets Inc. acting as sole agent with an aggregate offering value of up to $50.0 million, which allows us to sell our common

shares through the facilities of the Nasdaq Capital Market. Subject to the terms of the equity distribution agreement, we are able to determine, at our sole discretion, the timing and number of shares to be sold under this ATM facility.

As of March 31, 2019, we had cash, cash equivalents and marketable securities of $67.0 million, which we believe are sufficient to fund our planned operations into the second half of 2020. Our existing cash, cash equivalents and marketable securities are currently expected to be sufficient to fund our current operating plans through the topline results of the Phase 2b portion of our livoletide pivotal Phase 2b/3 PWS study.

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

- the outcome of our Phase 2b CAH protocol amendment submissions to appropriate regulatory authorities and ethics committees, and the timing of responses to these submissions;
- the scope, progress, results and costs of preclinical studies and clinical trials;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional 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 or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

As of March 31, 2019, there were no other material changes in commitments under contractual obligations, compared to the contractual obligations disclosed in our Annual Report.

Subsequent to March 31, 2019, we entered into a lease agreement for office space in Lexington, Massachusetts to expand our operations. Monthly rental payments will commence in May 2019 and continue through the lease term, which is scheduled to end in September 2020. We have the right to terminate the lease at any time and upon prior written notice. The lease does not provide for an extension or renewal. We will make future rental payments of $74,000 and $96,000 in fiscal 2019 and 2020, respectively.

In May 2019, we funded an escrow totaling approximately $1.0 million in connection with a sublease agreement for office space located in Waltham, Massachusetts assumed in connection with the Merger. The escrowed amount represents the difference in the annual base rent payable under the sublease agreement and the annual base rent payable under the overlease for the remaining term of the sublease agreement. The escrowed amount will be released on a monthly basis over the remaining lease term as the annual base rent payments are made by us.

Off-Balance Sheet Arrangements

We did not have any off balance sheet arrangements as of March 31, 2019, as defined in Item 303(a)(4)(ii) of Regulation S-K.

Critical Accounting Policies

Other than as described under Note 2 to our unaudited interim consolidated financial statements, the Critical Accounting Policies and Significant Judgments and Estimates included in our Form 10-K for the year ended December 31, 2018, filed with the SEC on April 1, 2019, have not materially changed.

Item 3. ***Quantitative and Qualitative Disclosures about Market Risk***

Not required for smaller reporting companies.

Item 4. ***Controls and Procedures***

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures (as defined in the Securities Exchange Act of 1934 Rules 13a-15(e) or 15d-15(e)) as required by paragraph (b) of Exchange Act Rules 13a-15 or 15d-15,as of March 31, 2019. Based on the evaluation of our disclosure controls and procedures as of March 31, 2019, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in internal control over financial reporting during the quarter ended March 31, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on Controls

Our disclosure controls and procedures and internal control over financial reporting are designed to provide reasonable assurance of achieving the desired control objectives. Our management recognizes that any control system, no matter how well designed and operated, is based upon certain judgments and assumptions and cannot provide absolute assurance that its objectives will be met. Similarly, an evaluation of controls cannot provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been detected.

PART II

Item 1. ***Legal Proceedings***

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, penalties, and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated.

On November 9, 2016, a purported shareholder derivative action was filed in the Business Litigation Session of the Suffolk County Superior Court in the Commonwealth of Massachusetts (*Cima v. Dipp*, No. 16-3443-BLS1 (Mass. Sup. Ct.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) and OvaScience as a nominal defendant alleging breaches of fiduciary duty, unjust enrichment, abuse of control, gross mismanagement and corporate waste for purported actions related to OvaScience's January 2015 follow-on public offering. On February 22, 2017, the court approved the parties' joint stipulation to stay all proceedings in the action until further notice. Following a status conference in December 2017, the stay was lifted. On January 25, 2018, at the parties' request, the court entered a second order staying all proceedings in the action until further order of the court. The Company believes that the complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On March 24, 2017, a purported shareholder class action lawsuit was filed in the U.S. District Court for the District of Massachusetts (*Dahhan v. OvaScience, Inc.*, No. 1:17-cv-10511-IT (D. Mass.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) alleging violations of Sections 10(b) and 20(a) of the Exchange Act (the "Dahhan Action"). On July 5, 2017, the court entered an order approving the appointment of Freedman Family Investments LLC as lead plaintiff, the firm of Robins Geller Rudman & Dowd LLP as lead counsel and the Law Office of Alan L. Kovacs as local counsel. Plaintiff filed an amended complaint on August 25, 2017. The Company filed a motion to dismiss the amended complaint, which the court denied on July 31, 2018. On August 14, 2018, the Company answered the amended complaint. The parties presently are engaged in discovery. The Company believes that the amended complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. A resolution of this lawsuit adverse to the Company or the other defendants could have a material effect on the Company's consolidated financial position and results of operations. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

On July 27, 2017, a purported shareholder derivative complaint was filed in the U.S. District Court for the District of Massachusetts (*Chiu v. Dipp*, No. 1:17-cv-11382-IT (D. Mass.)) against certain former officers and directors of OvaScience and one current director of the Company (a former director of OvaScience) as a nominal defendant alleging breach of fiduciary duty, unjust enrichment and violations of Section 14(a) of the Exchange Act alleging that compensation awarded to the director defendants was excessive and seeking redress for purported actions related to OvaScience's January 2015 follow-on public offering and other public statements. On September 26, 2017, the plaintiff filed an amended complaint which eliminated all claims regarding allegedly excessive director pay and additionally alleged claims of abuse of control and corporate waste. On October 27, 2017, the defendants filed a motion to dismiss the amended complaint. The court heard oral argument on the motion to dismiss on April 5, 2018. On April 13, 2018, the court granted the defendants' motion to dismiss the amended complaint for failure to state a claim for relief under Section 14(a). The court also dismissed the plaintiffs' pendent state law claims without prejudice, based on lack of subject matter jurisdiction. On April 25, 2018, the plaintiffs moved for leave to amend the complaint, and to stay this case pending the outcome of the Dahhan Action. The Company does not believe that the proposed amended complaint cures the defects in the current complaint, but informed plaintiffs' counsel that, in the interest of judicial economy, defendants would not oppose the proposed amendment if the court would consider staying the case pending the resolution of the Dahhan Action. On April 27, 2018, the court granted the plaintiffs' motion for leave to amend the complaint and for a stay. On April 30, 2018, the plaintiffs filed their second amended complaint. On May 23, 2018, the court entered an order staying this case pending the resolution of the Dahhan Action. The Company believes that the complaint is without merit and intends to defend against the litigation. There can be no assurance, however, that the Company will be successful. At present, the Company is unable to estimate potential losses, if any, related to the lawsuit.

Between October 16, 2018 and November 21, 2018, five putative class action lawsuits were filed in various federal District Courts against OvaScience, Inc. and the OvaScience Board of Directors related to OvaScience's proposed

merger with Millendo Therapeutics, Inc.: *Cunningham v. Kroeger, et al.*, No. 1:18-cv-01595 (D. Del. filed Oct. 16, 2018); *Adlard v. OvaScience, Inc., et al.*, No. 1:18-cv-12332 (D. Mass. filed Nov. 6, 2018); *Wheby v. OvaScience, Inc., et al.*, No. 1:18-cv-1811 (D. Del. filed Nov. 16, 2018); *Cuenca Aubets v. OvaScience, Inc., et al.*, No. 1:18-cv-10882 (S.D.N.Y. filed Nov. 20, 2018); and *Kim v. OvaScience, Inc., et al.*, No. 1:18-cv-10939 (S.D.N.Y. filed Nov. 21, 2018). The Complaints each alleged violations of Section 14(a) of the Securities Exchange Act of 1934 and Rule 14a-9 promulgated thereunder, and as against the individual defendants, violations of Section 20(a) of the Securities Exchange Act of 1934. The Cunningham plaintiff alleged that OvaScience's Form S-4 Registration Statement filed on September 26, 2018 omitted or misrepresented material information regarding OvaScience's proposed merger with Millendo Therapeutics, Inc. The Adlard, Wheby, Cuenca Aubets and Kim plaintiffs alleged that OvaScience's Definitive Proxy Statement on Schedule 14A filed on November 6, 2018, omitted or misrepresented material information regarding OvaScience's proposed merger with Millendo Therapeutics, Inc. OvaScience subsequently supplemented its disclosures. The Cunningham plaintiff voluntarily dismissed his complaint on December 10, 2018, and the Wheby plaintiff voluntarily dismissed his complaint on February 28, 2019. On March 18, 2019, the court dismissed the Cuenca Aubets and Kim actions for failure to serve. The Company currently is in negotiation with counsel for the plaintiffs regarding their demands for attorneys' fees. There can be no assurance that the negotiations will be successful. If the negotiations are not successful, the Company may be required to litigate the fee applications and/or the underlying actions.

In addition to the matters described above, the Company may be a party to litigation and subject to claims incident to the ordinary course of business from time to time. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. ***Risk Factors***

You should carefully consider the risks described below, as well as general economic and business risks and the other information in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2018. The occurrence of any of the events or circumstances described below or other adverse events could have a material adverse effect on our business, results of operations and financial condition and could cause the trading price of our common stock to decline. Additional risks or uncertainties not presently known to us or that we currently deem immaterial may also harm our business.

Risks Related to the Reverse Merger

The risks arising with respect to the historic OvaScience business and operations may be different from what we anticipate, which could lead to significant, unexpected costs and liabilities and could materially and adversely affect our business going forward.

It is possible that we may not have fully anticipated the extent of the risks associated with the recent Reverse Merger we completed with OvaScience. After the Reverse Merger, OvaScience's historic business was discontinued, but prior to the transaction OvaScience had a significant operating history. As a consequence, we may be subject to claims, demands for payment, regulatory issues, costs and liabilities that were not and are not currently expected or anticipated. Notwithstanding our exercise of due diligence pre-transaction and winding down of the OvaScience business post-transaction, the risks involved with taking over a business with a significant operating history and the costs and liabilities associated with these risks may be greater than we anticipate. We may not be able to contain or control the costs or liabilities associated with OvaScience's historic business, which could materially and adversely affect our business, liquidity, capital resources or results of operation.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses and negative operating cash flows and there is no assurance that we will ever achieve or sustain profitability. Our net loss was $84.6 million and $27.2 million for the years ended December 31, 2017 and 2018, respectively, and $10.4 million for three months ended March 31, 2019.

As of March 31, 2019, we had an accumulated deficit of $174.5 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We have devoted substantially all of our efforts to the acquisition of and preclinical and clinical development of our product candidates, livoletide and nevanimibe, as well as to building our management team and infrastructure. It could be several years, if ever, before we have a commercialized product and our commercialized products, if any, may not be profitable. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly in connection with our ongoing activities such as:

- continuing the ongoing and planned clinical development of livoletide and nevanimibe;
- initiating preclinical studies and clinical trials for any additional diseases for our current product candidates and any future product candidates that we may pursue;
- building a portfolio of product candidates through the acquisition or in-license of drugs or product candidates and technologies;
- developing, maintaining, expanding and protecting our intellectual property portfolio;
- manufacturing, or having manufactured, clinical and commercial supplies of our product candidates;
- seeking marketing approvals for our current and future product candidates that successfully complete clinical trials;
- establishing a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;
- hiring additional administrative, clinical, regulatory and scientific personnel; and
- incurring additional costs associated with operating as a public company.

In order to become and remain profitable, we will need to develop and eventually commercialize, on our own or with collaborators, one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of livoletide and nevanimibe, developing commercial scale manufacturing processes, obtaining marketing approval, manufacturing, marketing and selling any current and future product candidates for which we may obtain marketing approval, and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenue from product sales or achieve profitability.

Because of the numerous risks and uncertainties associated with pharmaceutical products and development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. For example, enrollment for our Phase 2b CAH clinical trial will be paused while a protocol amendment is submitted to appropriate regulatory authorities and ethics committees. We are unable to predict the outcome of these submissions or the timing of responses to these submissions. If we are required by the U.S. Food and Drug Administration, or FDA, or other regulatory authorities such as the European Medicines Agency, or EMA, to perform studies and trials in addition to those currently expected, or if there are any delays in the development or in the completion of any planned or future preclinical studies or clinical trials of our current or future product candidates, our expenses could increase and profitability could be further delayed.

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

We have a limited operating history and have never generated any revenue from product sales, which may make it difficult to assess our future viability.

We are a clinical stage biopharmaceutical company with a limited operating history. Our operations to date, with respect to the development of our product candidates, have been limited to organizing and staffing the business, business planning, raising capital, acquiring our product candidates and other assets and conducting preclinical and clinical development of our product candidates. We have not yet demonstrated an ability to successfully complete clinical development of a product candidate, obtain marketing approval, manufacture a commercial-scale drug (or arrange for a third-party to do so on our behalf), or conduct sales and marketing activities necessary for successful commercialization. Consequently, our predictions about our future success or viability may not be as accurate as they could be if we had more experience developing product candidates.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with any future collaborations, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, livoletide, nevanimibe and any additional product candidates that we may pursue in the future. We do not anticipate generating revenue from product sales for the next several years, if ever. Our ability to generate revenue from product sales depends heavily on our or any future collaborators' success in:

- timely and successful completion of clinical development of our current product candidates;
- obtaining and maintaining regulatory and marketing approvals for livoletide, nevanimibe and any future product candidates for which we successfully complete clinical trials;
- launching and commercializing any product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualifying for coverage and adequate reimbursement by government and third-party payors for our current or any future product candidates, if approved, both in the United States and internationally, and reaching acceptable agreements with such government and third-party payors on pricing terms;
- developing, validating and maintaining a commercially viable, sustainable, scalable, reproducible and transferable manufacturing process for livoletide, nevanimibe or any future product candidates that are compliant with current good manufacturing practices, or cGMP;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide an adequate amount and quality of drugs and services to support our planned clinical development, as well as the market demand for livoletide, nevanimibe and any future product candidates, if approved;
- obtaining market acceptance, if and when approved, of livoletide, nevanimibe or any future product candidates as a viable treatment option by physicians, patients, third-party payors and others in the medical community;
- effectively addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter, and performing our obligations pursuant to such arrangements;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference or infringement claims; and

- attracting, hiring and retaining qualified personnel.

We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain capital when needed may force us to delay, limit or terminate certain of our development programs, future commercialization efforts or other operations.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue to develop, and if approved, commercialize, livoletide and nevanimibe. Additionally, if we obtain marketing approval for our product candidates, we expect to incur significant expenses related to manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company.

As of March 31, 2019, our cash, cash equivalents and marketable securities were $67.0 million. Our existing cash, cash equivalents and marketable securities are currently expected to be sufficient to fund our current operating plans into the second half of 2020, which we expect will be sufficient to fund our operating plans through the topline results of the Phase 2b portion of our livoletide pivotal Phase 2b/3 PWS study. However, our operating plans may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, and marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or a combination of these approaches. In any event, we will require additional capital to pursue preclinical and clinical activities, regulatory approval and the commercialization of our current and future product candidates. Even if we believe we have sufficient capital for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations. If we elect to do so, additional capital may not be available to us on acceptable terms, if at all. Our ability to access additional capital, and as a result our operating results and liquidity needs, could be negatively affected by market fluctuations and economic downturn. Any additional capital raising efforts may divert our management from its day-to-day activities, which may adversely affect our ability to develop and commercialize our current and future product candidates.

Raising additional capital by issuing equity or debt securities may cause dilution to our existing stockholders, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.

Until such time as we can generate substantial revenue from product sales, if ever, we expect to finance our cash needs through a combination of equity and debt financings, strategic alliances and license and development agreements in connection with any future collaborations. To the extent that we raise additional capital by issuing equity securities, our existing stockholders' ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. Equity and debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as redeeming our shares, making investments, incurring additional debt, making capital expenditures or declaring dividends.

The incurrence of indebtedness could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants therein, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely affect our ability to conduct our business.

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

We may be required to make payments under licenses applicable to livoletide and nevanimibe.

We have certain milestone and royalty payments related to livoletide and nevanimibe. We acquired worldwide, exclusive rights to nevanimibe pursuant to our license agreement with the Regents of the University of Michigan, or the University of Michigan, entered into in June 2013, or the UM License Agreement. Under the terms of the UM License Agreement, we are obligated to make significant milestone and royalty payments in connection with the attainment of certain development steps and the sale of resulting products with respect to nevanimibe, as well as other material obligations. In addition, pursuant to an assignment agreement for certain patents and patent applications relating to livoletide, we are also required to pay royalties on commercial sales and licensing of livoletide to the assignors. If milestone or other non-royalty obligations become due, we may not have sufficient funds available to meet our obligations, which will materially adversely affect our business operations and financial condition.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with respect to our own product candidates for additional indications and other product candidates or diseases that later prove to have greater commercial potential. Our resource allocation decisions may ultimately not result in successful clinical development programs and may cause us to fail to capitalize on other viable product candidates, commercial products or profitable market opportunities. In addition, our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through sale, collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights.

Risks Related to Development and Commercialization

Our future success is dependent on the successful clinical development, regulatory approval and subsequent commercialization of livoletide, nevanimibe and any future product candidates. If we are not able to obtain the required regulatory approvals, we will not be able to commercialize our current or future product candidates and our ability to generate revenue will be adversely affected.

We do not have any drugs that have received regulatory approval and may never be able to develop marketable product candidates. We expect that a substantial portion of our efforts and expenses for the foreseeable future will be devoted to the clinical development of livoletide and nevanimibe, and as a result, our business currently depends heavily on the successful development, regulatory approval and commercialization of these product candidates. We cannot be certain that livoletide or nevanimibe will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. The research, testing, manufacturing, safety, efficacy, labeling, approval, sale, marketing and distribution of our product candidates are, and will remain, subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar foreign regulatory authorities. Failure to obtain regulatory approval for livoletide or nevanimibe in the United States or other jurisdictions will prevent us from commercializing and marketing livoletide or nevanimibe.

The Phase 2b portion of our recently initiated Phase 2b/3 PWS trial may or may not be sufficient to support FDA approval depending on the data. Additionally, the FDA may require additional data (for example, in children) in order to support an NDA approval in PWS in the United States.

Even if we were to successfully obtain approval from the FDA and comparable foreign regulatory authorities for our product candidates, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for our product candidates, or any approval contains significant limitations, on our own or with any future collaborators, we may not be able to obtain sufficient funding or

generate sufficient revenue to continue the development of any other product candidate that we may in-license, develop or acquire in the future.

Furthermore, even if we obtain regulatory approval for livoletide or nevanimibe, we will still need to develop a commercial infrastructure, or otherwise develop relationships with collaborators to commercialize, establish a commercially viable pricing structure and obtain approval for adequate reimbursement from third-party and government payors. If we, or our collaborators, are unable to successfully commercialize livoletide or nevanimibe, we may not be able to generate sufficient revenue to continue our business.

Preclinical studies or earlier clinical trials are not necessarily predictive of future results and the results of our clinical trials may not support our livoletide or nevanimibe claims.

Our product candidates, livoletide and nevanimibe, are still in development and will require extensive clinical testing before we are prepared to submit an NDA or other similar application for regulatory approval. Enrollment for our Phase 2b CAH clinical trial will be paused while a protocol amendment is submitted to appropriate regulatory authorities and ethics committees. We are unable to predict the outcome of these submissions or the timing of responses to these submissions. We cannot predict with any certainty if or when we might submit an NDA for regulatory approval for livoletide or nevanimibe for the treatment of any indication or whether any such application will be approved by the relevant regulatory authority. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. For instance, the FDA or foreign regulatory authorities may not agree with our proposed endpoints for any clinical trials of livoletide or nevanimibe, even if validated in prior clinical trials of similar product candidates, which may delay the commencement of our future clinical trials. The FDA or foreign regulatory authorities may also not agree with our proposed trial designs or dosing regimens, which may likewise prevent or delay the commencement of our future clinical trials. The clinical trial process is also time-consuming. We estimate that clinical trials of livoletide and nevanimibe for each of the indications that we are pursuing will take the next several years to complete. Failure can occur at any stage of the trials, and we could encounter problems that cause us to abandon or repeat clinical trials. Further, we may encounter challenges in the clinical development of product candidates for reasons unrelated to the observed safety or efficacy of such product candidates in prior clinical trials. In addition, because we may at times pursue the treatment of multiple indications for a single product candidate, setbacks or failures in, or termination of, clinical development for one indication may have a negative impact on the clinical development for the treatment of other indications.

Success in preclinical testing and early clinical trials does not ensure that later and pivotal clinical trials will generate the same results, or otherwise provide adequate data to demonstrate the safety and efficacy of a product candidate. Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later or pivotal clinical trials. Our approach to targeting orphan endocrine diseases where current therapies do not exist or are insufficient, is novel and unproven, and as such, the cost and time needed to develop livoletide and nevanimibe is difficult to predict and our efforts may not be successful. If we do not observe favorable results in future or planned clinical trials of livoletide and nevanimibe, we may decide to delay or abandon development of livoletide and nevanimibe, which could harm our business, financial condition and results of operations. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials.

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

Before obtaining marketing approval from regulatory authorities for the sale of livoletide, nevanimibe and any future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. For example, enrollment for our Phase 2b CAH clinical trial will be paused while a protocol amendment is submitted to appropriate regulatory authorities and ethics committees. We are unable to predict the outcome of these submissions or the timing of responses to these submissions. A failure of one or more clinical trials

can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- failure to obtain regulatory approval to commence a trial;
- unforeseen safety issues;
- determination of dosing issues;
- lack of effectiveness during clinical trials;
- inability to reach agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites;
- slower than expected rates of patient recruitment, failure to recruit adequate numbers of suitable patients to participate in our clinical trials or failure to maintain participation of recruited patients in clinical trials;
- failure to manufacture sufficient quantities of a product candidate for use in clinical trials;
- inability to monitor patients adequately during or after treatment; and
- inability or unwillingness of medical investigators to follow our clinical protocols.

Further, we, the FDA, an institutional review board ("IRB"), or other regulatory authority may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including, for example, the FDA's good clinical practice ("GCP"), regulations, that we are exposing participants to unacceptable health risks, or if the FDA or other regulatory authority, as the case may be, finds deficiencies in our investigational new drug ("IND"), application or other submissions, or the manner in which the clinical trials are conducted. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our current and future product candidates could be harmed, and our ability to generate revenue from our current or future product candidates, once approved, may be delayed or eliminated. In addition, any delays in our clinical trials could increase our costs, slow down the approval process and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

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

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 our current product candidates or any future product candidates that we may develop.

We have never obtained marketing approval for a product candidate. It is possible that the FDA may refuse to accept for substantive review any NDAs that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates. If the FDA does not accept or

approve our NDAs for any of our product candidates, it may require that we conduct additional clinical trials, preclinical studies 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 trials or 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 it has available. It is also possible that additional trials or 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 our product candidates, generating revenues and achieving and sustaining profitability. If any of these outcomes occurs, we may be forced to abandon our development efforts for our product candidates, which could significantly harm our business, prospects, operating results and financial condition.

Enrollment and retention of patients in clinical trials is a competitive, expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

Identifying and qualifying patients to participate in our clinical trials is critical to our success. We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials, including our recently initiated Phase 2b/3 clinical trial of livoletide in PWS patients, depends on many factors, including: the size of the patient population, the nature of the trial protocol, our ability to recruit clinical trial investigators with the appropriate competencies and experience, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same disease, the proximity of patients to clinical sites and the eligibility criteria for the trials, our ability to obtain and maintain patient consents and the risk that patients enrolled in clinical trials will drop out of the trials before completion.

The competitive nature of clinical trials in the pharmaceutical and biotechnology industries may make it difficult for us to recruit a sufficient number of patients to complete any of our clinical trials, or may increase costs. We may not be able to initiate or continue to support clinical trials of our product candidates for one or more indications, or any future product candidates, if we are unable to locate and enroll a sufficient number of eligible participants in these trials as required by the FDA or other regulatory authorities. For example, in our Phase 2 clinical trial for CS, which is ongoing, we have experienced slower than anticipated enrollment, which may make continuation of the CS study and further development of nevanimibe for the treatment of CS impractical. The estimated prevalence of CS is 20,000 cases in the United States (across all ages), and only a subset of this group satisfies the enrollment criteria for our Phase 2 clinical trial. We also face competition for patients for this trial and there are approved products available to patients with CS. Even if we are able to enroll a sufficient number of patients in our clinical trials, if the pace of enrollment is slower than we expect, the development costs for our product candidates may increase and the completion of our trials may be delayed or our trials could become too expensive or impractical to complete.

Our ability to enroll and retain patients in clinical trials of livoletide may be adversely impacted by the fact that livoletide is administered by subcutaneous injection. Furthermore, any negative results we may report in clinical trials of our product candidates may make it difficult or impossible to recruit and retain patients in other clinical trials of those product candidates. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop livoletide and nevanimibe, or could render further development impossible. In addition, we may rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

During the conduct of clinical trials, clinical investigators monitor changes in patients' health, including illnesses, injuries and discomforts. Often, it is not possible to determine whether or not the product candidate being investigated caused these conditions, and regulatory authorities may draw different conclusions or require additional testing to

confirm these determinations if they occur. In addition, it is possible that as we test livoletide, nevanimibe or any other product candidate in larger, longer and more extensive clinical programs, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be observed or reported by subjects. If clinical testing indicates that livoletide, nevanimibe or any future product candidate has side effects or causes serious or life-threatening side effects, we may need to change the design of ongoing clinical trials or adjust dosing levels in ongoing or future clinical trials, and the development of the product candidate may be delayed or terminated entirely. For example, in recent years clinical trials by other companies evaluating product candidates for treatment of PWS, which employed a different mechanism of action than livoletide, have resulted in serious adverse events, including patient deaths, and the eventual termination of the clinical trial and/or clinical development program. Further, if the product candidate has received regulatory approval, such approval may be revoked, which would materially harm our business, prospects, operating results and financial condition.

Moreover, if we elect or are required to modify, delay, suspend or terminate any clinical trial for our product candidates, the commercial prospects of our product candidates may be harmed and our ability to generate revenue through their sale may be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

We face substantial competition, and our operating results will suffer if we fail to compete effectively.

The commercialization of new drugs is competitive, and we may face worldwide competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies and ultimately generic companies. Our competitors may develop or market therapies that are more effective, safer or less costly than any that we are commercializing, or may obtain regulatory or reimbursement approval for their therapies more rapidly than we may obtain approval for ours.

We are aware of a number of companies that are working to develop drugs that would compete, directly or indirectly, against livoletide for the treatment of PWS and nevanimibe for the treatment of classic congenital adrenal hyperplasia or CAH, and endogenous Cushing's syndrome, or CS.

Soleno Therapeutics, Inc. is currently developing diazoxide choline controlled release, an ATP-sensitive potassium channel agonist, and Levo Therapeutics, Inc. is pursuing development of carbetocin, a long-acting analogue of oxytocin, for the treatment of PWS. Each of Saniona AB, GLWL Research Inc. and Insys Therapeutics, Inc. have also announced or initiated smaller trials in PWS for the treatment of hyperphagia. There are also a number of compounds in preclinical development.

We are aware of three other companies developing treatments for patients with CAH: Diurnal Group PLC is developing an exogenous cortisol treatment with a modified release intended to more closely match the physiological release profile of cortisol but recently announced a failed Phase 3 study and placed their U.S. development activities on hold. Neurocrine Biosciences, Inc. has initiated a Phase 2 clinical trial targeting CRF 1 antagonist in a Phase 2 clinical trial, and Spruce Biosciences, Inc. is developing a CRF 1 antagonist in a Phase 2 clinical trial. Novartis AG is currently marketing Signifor and Corcept Therapeutics Inc. is currently marketing Korlym, both for the treatment of subsets of CS patients. There are several other product candidates currently in clinical development for CS, including by Novartis, Corcept, HRA Pharma, SA and StrongBridge BioPharma plc. Many of our existing or potential competitors may have substantially greater financial, technical and human resources than we do, and significantly greater experience in the discovery and development of product candidates, including in the recruitment of patients for clinical trials, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs that have been approved for marketing. If we are not able to compete effectively against existing and potential competitors, our business and financial condition may be harmed.

Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors. Competition may reduce the number and types of patients available to us to participate in clinical trials, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors.

Competition may further increase as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, drugs that are more effective or less costly than any product candidate that we may develop.

Any inability to successfully complete clinical development of a product candidate could result in additional costs or impair or eliminate our ability to generate revenue from future sales of such product candidate, if approved, or from any regulatory and commercialization milestone with respect to such product candidate. In addition, if we make manufacturing or formulation changes to livoletide or nevanimibe, we may need to conduct additional testing to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize livoletide or nevanimibe, or allow our competitors to bring comparable drugs to market before we do, which could impair our ability to successfully commercialize livoletide or nevanimibe, and may harm our business, financial condition and results of operations.

Established pharmaceutical and biotechnology companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make livoletide or nevanimibe less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing and receiving FDA or other regulatory authority approval, or commercializing drugs before we do, which would have an adverse impact on our business and results of operations.

The availability of our competitors' products could limit the demand and the price we are able to charge for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would harm our business, prospects, financial condition and results of operations.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and even if we obtain approval for a product candidate in one country or jurisdiction, we may never obtain approval for, or commercialize, that product candidate in any other jurisdiction, which would limit our ability to realize our full market potential.

Prior to obtaining approval to commercialize a product candidate in any jurisdiction, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the FDA or foreign regulatory agencies may believe the clinical trials do not show the appropriate balance of safety and efficacy in the indication being sought or may interpret the data differently than we do, and deem the results insufficient to demonstrate the appropriate balance of safety and efficacy at the level required for product approval. Further, the regulatory authorities may not complete their review processes in a timely manner, or we may otherwise not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Further, in order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States does not ensure approval by regulatory authorities in any other country or jurisdiction. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials, which could be costly and time consuming. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and

maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

The FDA or any foreign regulatory bodies can delay, limit or deny approval of our product candidates or require us to conduct additional preclinical or clinical testing or abandon a program for many reasons, including:

- the FDA or the applicable foreign regulatory agency's disagreement with the design or implementation of our clinical trials;
- negative or ambiguous results from our clinical trials or results that may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- our inability to demonstrate to the satisfaction of the FDA or the applicable foreign regulatory body that our product candidates are safe and effective for the proposed indication;
- the FDA's or the applicable foreign regulatory agency's disagreement with the interpretation of data from preclinical studies or clinical trials;
- our inability to demonstrate the clinical and other benefits of our product candidates outweigh any safety or other perceived risks;
- the FDA's or the applicable foreign regulatory agency's requirement for additional preclinical studies or clinical trials;
- the FDA's or the applicable foreign regulatory agency's disagreement regarding the formulation, labeling or the specifications of our product candidates;
- the FDA's or the applicable foreign regulatory agency's failure to approve the manufacturing processes or facilities of third-party manufacturers with which we contract, including failure of such manufacturers to pass the required pre-approval inspections; or
- the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Even if we eventually complete clinical testing and receive approval of an NDA or foreign marketing application for our product candidates, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, or the implementation of a Risk Evaluation and Mitigation Strategy, or REMS, which may be required to ensure safe use of the drug after approval. The FDA or the applicable foreign regulatory agency also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or applicable foreign regulatory agency may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would negatively impact our business and results of operations.

If we are not able to obtain orphan drug designations or exclusivity for any of our current or future product candidates for which we seek such designation, the potential profitability of any such product candidates could be limited.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product as an orphan drug if the treatment is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer

than 200,000 individuals in the United States. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for a disease for which it receives the designation, then the product is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same product for the same disease for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use as the drug in question.

We have received orphan drug designation for livoletide from the FDA and EMA for the treatment of PWS. Nevanimibe has received orphan drug designation from the FDA for the treatment of CAH and CS and the EMA for the treatment of CAH. We may also seek orphan drug designation, where applicable, for our current product candidates in additional indications or for our future product candidates. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for any of our current or future product candidates, in any applicable indication. Even if we were to obtain orphan drug designation for a product candidate, we may not obtain orphan exclusivity and that exclusivity may not effectively protect the product candidate from the competition of different products or drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same product for the same disease if the FDA concludes that the later product is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the United States also may be lost if the FDA later determines that the request for designation was materially defective, the prevalence of the orphan disease is found to increase such that the qualifying criterion is no longer met or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition. The failure to obtain an orphan drug designation for any product candidates we may develop and seek it for, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable product candidates to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

If we are not able to obtain required regulatory approvals, we will not be able to commercialize livoletide or nevanimibe, and our ability to generate revenue will be harmed.

Livoletide and nevanimibe and the activities associated with their development and commercialization, including their design, research, testing, manufacture, safety, efficacy, recordkeeping, labeling, packaging, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by similar regulatory authorities outside the United States. Failure to obtain marketing approval for livoletide and nevanimibe or failure to meet post-marketing requirements will prevent us from commercializing them.

We have not yet received approval from regulatory authorities to market any product candidate in any jurisdiction, and it is possible that none of livoletide, nevanimibe or any future product candidates will ever obtain the appropriate regulatory approvals necessary for us to commence product sales. Neither we nor any future collaborator is permitted to market any of our product candidates in the United States until we receive regulatory approval of an NDA from the FDA.

The time required to obtain approval of an NDA by the FDA is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. Prior to submitting an NDA to the FDA or an equivalent application to other foreign regulatory authorities for approval of livoletide for the treatment of PWS and for approval of nevanimibe for the treatment of CAH and CS, respectively, we will need to complete its currently planned registration clinical trials for each, and additional trials that the FDA may require us to complete.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with livoletide or nevanimibe, we may:

- be delayed in obtaining marketing approval for livoletide or nevanimibe, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose restrictions on its distribution in the form of REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Furthermore, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions.

We may rely on third-party CROs and consultants to assist us in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each disease to establish the safety and efficacy of livoletide, nevanimibe and any future product candidate for that disease. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities.

Even if we obtain regulatory approval for livoletide, nevanimibe or future product candidates, we will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approval for livoletide, nevanimibe or future product candidates, the approved product will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. For example, we must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising and the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling. In addition, any regulatory approvals that we receive for livoletide, nevanimibe or future product candidates may also be subject to REMS limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the drug.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requiring recall or withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of livoletide, nevanimibe or future product candidates, a regulatory authority may, among other things:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;

- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize livoletide and nevanimibe, and harm our business, financial condition and results of operations.

In addition, the FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could suspend or restrict regulatory approval of livoletide and nevanimibe. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. 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 harm our business, financial condition and results of operations.

Even if one of our product candidates receives marketing approval, it may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

Even if one of our product candidates receives marketing approval, it may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If any such product candidate does not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of a product candidate, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments;
- the success of our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our products;
- effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our drugs, once approved, for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;

- the availability of third-party coverage and adequate reimbursement, and patients' willingness to pay out-of-pocket in the absence third-party coverage or adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our drugs, once approved, together with other medications

If the market opportunities for our product candidates are smaller than we believe they are, our product revenues may be adversely affected and our business may suffer.

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our products, if and when approved, may require significant resources and may never be successful. Further, patient populations suffering from PWS, CAH and CS, and other indications we may target in the future, are small and have not been established with precision. If the actual number of patients is smaller than we estimate for any disease that we are targeting, or if we cannot raise awareness of these diseases and diagnosis is not improved, our revenue and ability to achieve profitability may be adversely affected. For example, since the patient populations for PWS, CAH and CS are small, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs and achieve profitability. For PWS, CAH and CS, then, we may not maintain or obtain sufficient sales volume at a price high enough to justify our product development efforts and our sales and marketing and manufacturing expenses. Because we expect sales of livoletide and nevanimibe, if approved, to generate substantially all of our product revenue for the foreseeable future, the failure of either of these product candidates to find market acceptance would harm our business.

If we are unable to establish sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates, if approved.

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market any product that may be approved, we must build our sales, distribution, marketing, managerial and other non-technical capabilities, or make arrangements with third parties to perform these services. There can be no assurance we will be able to do so in a cost-effective manner, on terms favorable to us, or at all.

While we may seek the aid of global or regional collaborators to provide additional resources for larger indications or to co-commercialize our product candidates in the European Union and certain other territories, we expect to build a focused sales, distribution and marketing infrastructure to market our product candidates in the United States itself, if approved. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any product launch, which would adversely impact its commercialization.

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

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to educate adequate numbers of physicians as to the benefits or our drug products;
- the inability of reimbursement professionals to negotiate arrangements, for formulary access, reimbursement, and other acceptance by payors;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;

- the lack of complementary medicines to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and

- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

Further, we do not anticipate having the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain markets overseas. Therefore, our future success will depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in a product and such collaborator's ability to successfully market and sell the product. We intend to pursue collaborative arrangements regarding the sale and marketing of our product candidates, if approved, for certain markets overseas; however, we cannot assure you that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that we will have effective sales forces. To the extent that we depend on third parties for marketing and distribution, any revenue we receive will depend upon the efforts of such third parties, and there can be no assurance that such efforts will be successful.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of our product candidates, we may be forced to delay our potential commercialization or reduce the scope of our sales or marketing activities for them. If we elect to increase our expenditures to fund commercialization activities itself, we will 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 will not be able to bring our product candidates to market or generate product revenue. We could enter into arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be ideal and we may be required to relinquish rights to our product candidates or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business and results of operations.

If we are unable to establish adequate sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates and may not become profitable. We will be competing with many companies that currently have extensive and well-funded sales and marketing operations. Without an internal team or the support of a third-party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies.

Even if we obtain and maintain approval for our current and future product candidates from the FDA, we may nevertheless be unable to obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. If approved, sales of livoletide, nevanimibe and any future product candidate outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, if approved, is also subject to approval. Obtaining approval for livoletide, nevanimibe or any future product candidate in the European Union from the European Commission following the opinion of the EMA, if we choose to submit a marketing authorization application there, would be a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of livoletide, nevanimibe or any future product candidate in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for livoletide, nevanimibe or any future product candidate may be withdrawn. If we fail to comply

with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of livoletide, nevanimibe or any future product candidate will be negatively impacted, and our business, prospects, financial condition and results of operations could be harmed.

We are exposed to a variety of risks associated with our international operations.

Since the closing date of the Merger, we have been engaged in the process of winding up various subsidiaries of OvaScience, some or all of which are in foreign jurisdictions. We expect to incur additional costs to complete this process. Moreover, even if we successfully wind up these entities, we may be exposed to liability in these foreign jurisdictions as a result of their historical operations.

In addition, in December 2017, we acquired Alizé, a biopharmaceutical company based in Lyon, France. As of March 31, 2019, we had 27 employees located in the United States and seven employees located in France. Our global operations expose us to numerous and sometimes conflicting legal, tax and regulatory requirements, and violations or unfavorable interpretation by the respective authorities of these regulations could harm our business. Risks associated with international operations include the following, and these risks may be more pronounced if we seek to commercialize livoletide, nevanimibe or any future product candidates outside of the United States:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- changes in diplomatic and trade relationships;
- anti-corruption laws, including the FCPA, and its equivalent in foreign jurisdictions, such as the UK Bribery Act;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

In addition, there are complex regulatory, tax, labor, and other legal requirements imposed by both the European Union and many of the individual countries in and outside of Europe, with which we may need to comply. Many biopharmaceutical companies have found the process of marketing their own products in foreign countries to be very challenging.

Furthermore, in some countries, 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 candidate. In addition, there can be considerable pressure by governments and other stakeholders

on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after coverage and reimbursement have been obtained. Reference pricing used by various countries and parallel distribution or arbitrage between low-priced and high-priced countries, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies, which is time-consuming and costly. If coverage and reimbursement of our product candidates are unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed.

Legal, political and economic uncertainty surrounding the planned exit of the U.K., from the European Union, or EU, may be a source of instability in international markets, create significant currency fluctuations, adversely affect our operations in the U.K. and pose additional risks to our business, revenue, financial condition, and results of operations.

On June 23, 2016, the U.K. held a referendum in which a majority of the eligible members of the electorate voted for the U.K. to leave the EU. The U.K.'s withdrawal from the EU is commonly referred to as Brexit. The lack of clarity over which EU laws and regulations will continue to be implemented in the U.K. after Brexit (including financial laws and regulations, tax and free trade agreements, intellectual property rights, data protection laws, supply chain logistics, environmental, health and safety laws and regulations, immigration laws and employment laws) may negatively impact foreign direct investment in the U.K., increase costs, depress economic activity and restrict access to capital. The uncertainty concerning the U.K.'s legal, political and economic relationship with the EU after Brexit may be a source of instability in the international markets, create significant currency fluctuations, and/or otherwise adversely affect trading agreements or similar cross-border co-operation arrangements (whether economic, tax, fiscal, legal, regulatory or otherwise) beyond the date of Brexit.

These developments, or the perception that any of them could occur, have had, and may continue to have, a significant adverse effect on global economic conditions and the stability of global financial markets, and could significantly reduce global market liquidity and limit the ability of key market participants to operate in certain financial markets. In particular, it could also lead to a period of considerable uncertainty in relation to the U.K. financial and banking markets, as well as on the regulatory process in Europe. Asset valuations, currency exchange rates and credit ratings may also be subject to increased market volatility. The long-term effects of Brexit will depend on any agreements (or lack thereof) between the U.K. and the EU and, in particular, any arrangements for the U.K. to retain access to EU markets either during a transitional period or more permanently.

Such a withdrawal from the EU is unprecedented, and it is unclear how the U.K.'s access to the European single market for goods, capital, services and labor within the EU, or single market, and the wider commercial, legal and regulatory environment, will impact us. We may also face new regulatory costs and challenges that could have an adverse effect on our operations. Depending on the terms of the U.K.'s withdrawal from the EU, the U.K. could lose the benefits of global trade agreements negotiated by the EU on behalf of its members, which may result in increased trade barriers that could make our doing business in the U.K. more difficult. Furthermore, there are likely to be changes to the way in which marketing approvals are granted in the U.K., which could add time and expense to the process by which our product candidates receive and maintain regulatory approval in the U.K. and across the EEA in future.

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

We face an inherent risk of product liability exposure related to the testing of our current and future product candidates, and may face an even greater risk if we commercialize any product candidate that it may develop. If we cannot successfully defend ourselves against claims that any such product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidate that we may develop;
- loss of revenue;

- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidate that it may develop;
- injury to our reputation and significant negative media attention; and
- increased marketing costs to attempt to overcome any injury to our reputation or negative media attention.

In addition, we face an inherent risk of product liability exposure related to OvaScience's prior use of fertility treatments in humans. Product liability claims involving OvaScience's activities may be brought for significant amounts because OvaScience's potential fertility treatments involved mothers and children. For example, it is possible that we will be subject to product liability claims that assert that OvaScience's potential fertility treatments have caused birth defects in children or that such defects are inheritable. These claims could be made many years into the future based on effects that were not observed or observable at the time of birth. If we cannot successfully defend against claims that OvaScience's potential fertility treatments caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in, among other things, significant costs to defend the related litigation; substantial monetary awards or payments to trial participants or patients; loss of revenue; and the diversion of management's resources.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

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

OvaScience is 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. OvaScience's prior operations involved the use of hazardous and flammable materials, including chemicals and biological materials. OvaScience's prior operations also produced hazardous waste products. OvaScience generally contracted with third parties for the disposal of these materials and wastes. In the event of contamination or injury resulting from OvaScience's use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with OvaScience's storage or disposal of biological, hazardous or radioactive materials.

Risks Related to Regulatory Compliance

Our current and future relationships with investigators, health care professionals, consultants, third-party payors and customers may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Our operations may be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and Physician Payments Sunshine Act and regulations. These laws may

constrain our current and future business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our products for which we obtain marketing approval. In addition, we may be subject to patient privacy laws by both the federal government and the states and other countries in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers, formulary managers, and others on the other hand. In addition, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively PPACA, amended the intent requirement of the federal Anti-Kickback Statute, establishing that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. PPACA provides, and recent government cases against pharmaceutical and medical device manufacturers support the view, that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the federal civil False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal civil and criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and certain health care providers, known as covered entities, and their business associates who create, use or disclose individually identifiable health information on their behalf;

- federal transparency laws, including the federal Physician Payments Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to: (i) payments or other "transfers of value" made to physicians and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members;

- state and foreign law equivalents of each of the above federal laws, such as state anti-kickback, self-referral, and false claims laws which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical manufacturers to comply with the industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers; state laws that require pharmaceutical manufacturers to file reports with states regarding marketing information, such as the tracking and reporting of gifts, compensation and other remuneration and items of value provided to healthcare professionals and entities; state laws that require the reporting of information related to drug pricing; and state and local laws requiring the registration of pharmaceutical sales and medical representatives; and

- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. However, because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including significant administrative, civil and criminal penalties, damages, fines, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance with these laws, exclusion from participation in government health care programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm and the curtailment or restructuring of our operations, any of which could harm our ability to operate our business and our results of operations. Similar sanctions and penalties, as well as individual imprisonment, also can be imposed upon executive officers and employees, including criminal sanctions against executive officers under the so-called "responsible corporate officer" doctrine, even in situations where the executive officer did not intend to violate the law and was unaware of any wrongdoing.

The risk of us being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and its provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain a robust and expandable system to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company such as we may run afoul of one or more of the requirements.

Coverage and adequate reimbursement may not be available for our current or future product candidates, which could make it difficult for us to sell them profitably, if approved.

Market acceptance and sales of any product candidates that we commercialize, if approved, will depend in part on the extent to which coverage and reimbursement for these drugs and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any product candidates that we develop will be made on a plan-by-plan basis. As a result, the coverage determination process is often a time-consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained. One payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each plan determines whether it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a formulary generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize livoletide, nevanimibe and any future product candidates that we develop.

Additionally, there have been a number of legislative and regulatory proposals to change the healthcare system in the United States and in some foreign jurisdictions that could affect our ability to sell any future product candidates profitably. These legislative and regulatory changes may negatively impact the coverage and available reimbursement for livoletide, nevanimibe and any future product candidates we may commercialize, following approval, if obtained.

Healthcare legislative reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

In March 2010, PPACA was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry. PPACA, among other things: (i) addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; (ii) increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations; (iii) establishes annual fees and taxes on manufacturers of certain branded prescription drugs; (iv) expands the availability of lower pricing under the 340B drug pricing program by adding new entities to the program; and (v) establishes a new Medicare Part D coverage gap discount program, in which manufacturers must, as of January 1, 2019, agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since PPACA was enacted, the U.S. federal government also has announced delays in the implementation of key provisions of PPACA. Additionally, there have been judicial and Congressional challenges to certain aspects of PPACA, as well as efforts by the Trump administration to repeal or replace certain aspects of PPACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of PPACA or otherwise circumvent some of the requirements for health insurance mandated by PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under PPACA have been signed into law. The Tax Cuts and Jobs Act of 2017, or Tax Act, included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, among other things, amended the PPACA, 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". More recently, in July 2018 CMS published a final rule permitting further collections and payments to and from certain PPACA qualified health plans and health insurance issuers under the PPACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the PPACA will impact the PPACA. We continue to evaluate the potential impact of PPACA and its possible repeal or replacement on our business.

We expect that PPACA, 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 are able to charge for any approved drug in the United States. For example, 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 cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further 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. Further, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services, or HHS, has already started the process of soliciting feedback on some of these measures and, at the same, is immediately implementing others under its existing authority. For example, on January 31, 2019, the HHS Office of Inspector General proposed modifications to federal Anti-Kickback Statute safe harbors which, among other things, may affect rebates paid by manufacturers to Medicare Part D plans, the purpose of which is to further reduce the cost of drug products to consumers. In addition, CMS issued a final rule, effective on or about July 9, 2019, that requires direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product if it is equal to or greater than $35 for a monthly supply or usual course of treatment. Prescription drugs and biological products that are in violation of these requirements will be included on a public list. Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, such measures are designed to encourage importation from other countries and bulk purchasing. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

In addition, other legislative changes have been adopted since PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2018, among other legislative amendments, will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

Additional changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under the health insurance exchanges and fraud and abuse and enforcement. Continued implementation of PPACA and the passage of additional laws and regulations may result in the expansion of new programs such as Medicare payment for performance initiatives, and may impact existing government healthcare programs, such as by improving the physician quality reporting system and feedback program. For each state that does not choose to expand its Medicaid program, there likely will be fewer insured patients overall, which could impact the sales, business and financial condition of manufacturers of branded prescription drugs. Where patients receive insurance coverage under any of the new options made available through PPACA, the possibility exists that manufacturers may be required to pay Medicaid rebates on their resulting drug utilization, a decision that could impact manufacturer revenues.

Regulatory, legislative or self-regulatory/standard developments regarding privacy and data security matters could adversely affect our ability to conduct our business.

We are subject to and affected by laws, rules, regulations and industry standards related to data privacy and security, and restrictions or technological requirements regarding the collection, use, storage, security, retention or transfer of data. In the United States, the rules and regulations to which we may be subject include federal laws and regulations enforced by the Federal Trade Commission, the Department of Health & Human Services, and state privacy, data security, and breach notification laws, as well as regulator enforcement positions and expectations. Internationally, governments and agencies have adopted and could in the future adopt, modify, apply or enforce additional laws, policies, regulations, and standards covering privacy and data security that may apply to our business. New regulation or legislative actions regarding data privacy and security (together with applicable industry standards) may increase our costs of doing business. In addition to privacy and data security regulations currently in force in the jurisdictions where we operate, the European Union General Data Protection Regulation, or GDPR, went into effect in May 2018. The GDPR contains numerous requirements and changes from existing European Union, or EU, law, including more robust obligations on data processors and data controllers and heavier documentation requirements for data protection compliance programs. Specifically, the GDPR will introduce numerous privacy-related changes for companies operating in the EU, including greater control over personal data-by-data subjects (e.g., the "right to be forgotten"), increased data portability for EU consumers, data breach notification requirements, and increased fines. In particular, under the GDPR, fines of up to €20 million or up to 4% of the annual global revenue of the noncompliant company, whichever is greater, could be imposed for violations of certain of the GDPR's requirements. The GDPR requirements apply not only to third-party transactions, but also to transfers of information between us and our subsidiaries, including employee information. However, despite our ongoing efforts to bring our practices into compliance before the effective date of the GDPR, we may not be successful either due to various factors within our control, such as limited financial or human resources, or other factors outside our control. It is also possible that local data protection authorities may have different interpretations of the GDPR, leading to potential inconsistencies amongst various EU member states. Any failure or alleged failure (including as a result of deficiencies in our policies, procedures, or measures relating to privacy, data security, marketing, or communications) by us to comply with laws, regulations, policies, legal or contractual obligations, industry standards, or regulatory guidance relating to privacy or data security, may result in governmental investigations and enforcement actions, litigation, fines and penalties, additional regulatory oversight and reporting obligations or adverse publicity. We expect that there will continue to be new proposed laws, regulations and industry standards relating to privacy and data protection in the United States, the European Union, and in other jurisdictions, and we cannot determine the impact such future laws, regulations and standards may have on our business.

Future laws, regulations, standards and other obligations or any changed interpretation of existing laws or regulations could impair our ability to operate our business and negatively impact our results of operations.

Risks Related to Our Intellectual Property

We rely on the availability of licenses for intellectual property from third parties and these licenses may not be available to us on commercially reasonable terms, or at all.

We rely upon the UM License Agreement to certain patent rights and proprietary technology from the University of Michigan that are important or necessary to the development of nevanimibe. As of March 31, 2019, with respect to nevanimibe patent rights, we owned two issued U.S. patents, two pending U.S. patent applications, and a number of patent applications in other jurisdictions, and we jointly owned, with the University of Michigan, three issued U.S. patents, one pending U.S. patent application, and a number of patent applications in other jurisdictions. In addition, as of March 31, 2019, with respect to livoletide patent rights, we owned four issued U.S. patents, one pending U.S. patent application, and a number of patents and pending patent applications in other jurisdictions. There is no guarantee that any of the foregoing patent applications will result in issued patents, or that any current patents or patent applications, if issued, will include claims that are sufficiently broad to cover our product candidates or future products, or to provide meaningful protection from our competitors in all territories in which we may wish to develop or commercialize our products in the future. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent they are covered by valid and enforceable patents or are effectively maintained as trade secrets within our organization. If third parties disclose or misappropriate our proprietary rights, it may have a material adverse effect on our business.

The licenses granted under the UM License Agreement are revocable under certain circumstances including if we cease to do business, fail to make the payments due thereunder, commit a material breach of the agreement that is not cured within a certain time period after receiving written notice or fail to meet certain specified development and commercial timelines. In such an event, our ability to compete in the market may be diminished. Termination of the UM License Agreement may result in us having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean we are unable to develop or commercialize nevanimibe. Additionally, the UM License Agreement and other licenses we may enter into in the future may not provide exclusive rights to use such intellectual property and technology at all, in all relevant fields of use and/or in all territories in which we may wish to develop or commercialize our product candidates in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products, including in territories included in the UM License Agreement.

Licenses to additional third-party patents and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could harm our business and financial condition.

Our intellectual property licenses and agreements with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

We currently depend, and will continue to depend, on the UM License Agreement. In addition, pursuant to an assignment agreement for certain patents and patent applications relating to livoletide, we are also required to pay royalties on commercial sales and licensing of livoletide to the assignors. Further, the assignors under this assignment agreement have a right to repurchase the assigned intellectual property at a certain price in the event we do not, upon receiving notice, use reasonable efforts to develop, introduce for sale and promote products derived from the assigned intellectual property. Such reasonable efforts involve spending an annual amount of at least CDN$100,000 in research and development related to livoletide, actively pursuing the registration, licenses and permits necessary to market livoletide and actual commercialization of livoletide, if approved. Further development and commercialization of livoletide and nevanimibe may, and development of any future product candidates may, require us to enter into additional license, assignment or collaboration agreements. The agreements under which we currently hold or license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If any of our current or future licenses or agreements or material relationships or any in-licenses upon which our current or future licenses and intellectual property are based are terminated or breached, we may:

- lose our rights to develop and market our current and any future product candidates;
- lose our rights to patent protection for our current or any future product candidates;
- experience significant delays in the development or commercialization of our current or any future product candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

These risks apply to any agreements that we may enter into in the future for livoletide, nevanimibe or for any future product candidates. If we experience any of the foregoing, it would have a material adverse effect on our business, financial condition and results of operations.

If we fail to comply with our obligations in the agreements under which we hold or license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license and intellectual property rights that are important to our business.

Further, we cannot provide any assurances that third-party patents or other intellectual property rights do not exist, which might be enforced against our current product candidates, resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, prospects, financial condition and results of operations.

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. 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 our current and future product candidates in the United States and other countries in which we plan to develop and commercialize such product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

Pursuant to the UM License Agreement, we obtained an exclusive, worldwide license to develop, manufacture and commercialize nevanimibe. However, the UM License Agreement permits the University of Michigan, and other non-profit research institutions which are granted such rights from the University of Michigan, to manufacture and research nevanimibe for internal research, public service and internal educational purposes, all of which could result in new patentable inventions concerning the manufacture or use of nevanimibe. In addition, pursuant to an assignment agreement for certain livoletide patents and patent applications, certain individuals at the Erasmus University Medical Center and the University of Turin were granted non-exclusive rights to use the assigned intellectual property for non-commercial research with our prior written consent, all of which could result in new patentable inventions concerning the manufacture or use of livoletide.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current and future product candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our current and future product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and companion diagnostic under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current and future product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize future drugs. Any such outcome could have a material adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law

does. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically published 18 months after filing, or in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. 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.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent and Trademark Office, or USPTO, recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. Any further changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents and patent applications or narrow the scope of our potential patent protection.

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

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and product candidates, or limit the duration of the patent protection of our technology and product candidates. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from the earliest filing date of a non-provisional patent application. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such drugs. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, we owned and licensed patent portfolio may not provide it with sufficient rights to exclude others from commercializing drugs similar or identical to that of us.

We jointly own patents and patent applications with third parties. Our ability to exploit or enforce these patent rights, or to prevent the third-party from granting licenses to others with respect to these patent rights, may be limited in some circumstances.

We jointly own certain patents and patent applications with third parties. In the absence of an agreement with each co-owner of jointly owned patent rights, we will be subject to default rules pertaining to joint ownership. Some countries require the consent of all joint owners to exploit, license or assign jointly owned patents, and if we are unable to obtain

that consent from the joint owners, we may be unable to exploit the invention or to license or assign our rights under these patents and patent applications in those countries. For example, we secured exclusive rights from the University of Michigan for certain patents and patent applications that they jointly own with us related to nevanimibe. Additionally, in the United States, each co-owner may be required to be joined as a party to any claim or action we may wish to bring to enforce these patent rights, which may limit our ability to pursue third-party infringement claims.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States in several stages over the lifetime of our owned and licensed patents and/or applications and any patent rights it may own or license in the future. We rely on our outside counsel or our licensing partners to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules.

There are situations, however, in which non-compliance 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.

In such an event, potential competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

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

Given the amount of time required for the development, testing and regulatory review of new product candidates such as livoletide and nevanimibe, 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 extension of the term of one U.S. patent that includes at least one claim covering the composition of matter of an FDA-approved drug, an FDA-approved method of treatment using the drug. The extended patent term cannot exceed the shorter of five years beyond the non-extended expiration of the patent or 14 years from the date of the FDA approval of the drug. 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. Further, we may not elect to extend the most beneficial patent to us or the claims underlying the patent that we choose to extend could be invalidated. If any of the foregoing occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing its clinical and preclinical data and launch their drug earlier than might otherwise be the case.

Intellectual property rights do not necessarily address all potential threats to our business.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business. The following examples are illustrative:

- others may be able to make compounds, or livoletide and nevanimibe formulations that are similar to our livoletide and nevanimibe formulations but that are not covered by the claims of the patents that we own or control;

- we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;

- we might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive drugs for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

We do not have broad composition of matter patent protection with respect to nevanimibe.

We own certain patents and patent applications with claims directed to the form of nevanimibe and to specific methods of using nevanimibe and it expects to have marketing exclusivity from the FDA and EMA for a period of seven and ten years, respectively, because nevanimibe has not been approved in these markets. However, we do not have composition of matter protection in the United States and elsewhere broadly covering nevanimibe. We may be limited in our ability to list our patents in the FDA's Orange Book if the form of the compound used is materially different from what is claimed in our patents, or if the use of its product, consistent with its FDA-approved label, would not fall within the scope of our patent claims. Also, our competitors may be able to offer and sell products so long as these competitors do not infringe any other patents that we (or third parties) hold, including patents with claims directed to the forms and manufacture of nevanimibe and/or method of use patents. In general, patents covering certain forms of a compound and method of use patents are more difficult to enforce than broad composition of matter patents because, for example, of the risks that the FDA may approve different forms of subject compounds or alternative uses of the subject compounds not covered by the method of use patents, and others may engage in off-label sale or use of the subject compounds. Physicians are permitted to prescribe an approved product for uses that are not described in the product's labeling. Although off-label prescriptions may infringe its method of use patents, the practice is common across medical specialties and such infringement is difficult to prevent or prosecute. FDA approval of uses that are not covered by our patents would limit our ability to generate revenue from the sale of nevanimibe, if approved for commercial sale. Off-label sales would limit our ability to generate revenue from the sale of nevanimibe, if approved for commercial sale.

Third parties may initiate legal proceedings, which are expensive and time consuming, alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse impact on the success of our business.

Our commercial success depends, in part, upon our ability, and the ability of our future collaborators, to develop, manufacture, market and sell livoletide, nevanimibe and any future product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to livoletide, nevanimibe and any future product candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a material

adverse effect on our ability to commercialize livoletide, nevanimibe and any future product candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third-party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing and marketing our product candidate and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidate. 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 or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing livoletide, nevanimibe or any future product candidates or force us to cease some or all of our business operations, which would have a material adverse effect on our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business. Even if we prevail in such infringement claims, patent litigation can be expensive and time consuming, which would harm our business, financial condition and results of operations.

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of ours patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third-party may also cause the third-party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third-party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could have material adverse effect on our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees. Even if we prevail in such infringement claims, patent litigation can be expensive and time consuming, which would harm our business, financial condition and results of operations.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or

developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

The United States has recently enacted and implemented wide-ranging patent reform legislation. 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. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, federal courts, 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 patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

We may not be able to protect our intellectual property rights throughout the world, which could have a material adverse effect on our business.

Filing, prosecuting and defending patents covering livoletide, nevanimibe and any future product candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop our own drugs and, further, may export otherwise infringing drugs to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These drugs may compete with our drugs in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

If we rely on third parties to manufacture and commercialize livoletide, nevanimibe or any future product candidates, or if we collaborate with third parties for the development of livoletide, nevanimibe or any future product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of third-party collaborators. A competitor's discovery of our trade secrets would harm our business.

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

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages we may lose valuable intellectual property rights or personnel. 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 it is our approach to require our employees and contractors who may be involved in the conception or 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, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

Risks Related to Our Dependence on Third Parties

We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of livoletide and nevanimibe, and any future product candidate.

We have no experience in drug formulation or manufacturing and do not own or operate, and we do not expect to own or operate, facilities for product manufacturing, storage and distribution, or testing. We will rely on a contract manufacturing organization, or CMO, to produce additional livoletide active pharmaceutical ingredient, or API, for us for clinical use. We also currently rely on CMOs to produce nevanimibe for our clinical trials. Additionally, we rely on CMOs with respect to the manufacture of drug product for our clinical trials, including for filing and packaging. Any significant delay in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replenish the supply or replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates. If we or our manufacturer are unable to purchase these raw materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenue from the sale of our product candidates.

We will need to rely on third-party manufacturers to supply us with sufficient quantities of livoletide and nevanimibe to be used, if approved, for the commercialization of each. The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit our NDA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMP requirements for manufacture of drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Further, our reliance on third-party manufacturers entails risks, to which we would not be subject if we manufactured product candidates ourselves, including:

- inability to meet our product specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;

- issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- failure to comply with cGMP and similar foreign standards;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on a limited number of sources, and in some cases, single sources for product components;
- lack of qualified backup suppliers for those materials that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier;
- inability to find replacement manufacturers or suppliers, if necessary, on terms favorable to us, in a timely manner, or at all;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our products under specified storage conditions and in a timely manner.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products once approved. Some of these events could be the basis for FDA or other regulatory authority action, including injunction, recall, seizure, or total or partial suspension of production.

We may in the future enter into collaborations with third parties to develop our product candidates. If these collaborations are not successful, our business could be harmed.

We may enter into collaborations with third parties in the future. We may in the future determine to collaborate with other pharmaceutical and biotechnology companies for development and potential commercialization of our product candidates. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of several factors. If we license rights to our product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If any such potential future collaborations do not result in the successful development and commercialization of product candidates, or if one of our future collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, the development of our product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our future collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization apply to the activities of our potential future collaborators.

We may not be successful in finding strategic collaborators for continuing development of livoletide or nevanimibe, or successfully commercializing or competing in the market for certain diseases.

We may seek to develop strategic partnerships for developing and commercializing livoletide or nevanimibe, due to capital costs required to develop the product candidate, manufacturing constraints or anticipated commercialization costs. We may not be successful in our efforts to establish such a strategic partnership or other alternative arrangements for livoletide or nevanimibe because our research and development pipeline may be insufficient or third parties may not view livoletide or nevanimibe as having the requisite potential to demonstrate safety and efficacy. In addition, we may be restricted under an existing collaboration agreement from entering into a future agreement with a potential collaborator. We cannot be certain that, following a strategic transaction or license, we will achieve an economic benefit that justifies such transaction.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of our product candidates, reduce or delay the development programs, delay potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop livoletide or nevanimibe, which could harm our business, financial condition and results of operations.

We rely on third parties to conduct, supervise and monitor our clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We currently do not have the ability to independently conduct preclinical studies and clinical trials that comply with the regulatory requirements known as good laboratory practice, or GLP, or GCP, respectively. We also do not currently have the ability to independently conduct large clinical trials. We intend to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and we expect to have limited influence over their actual performance.

We intend to rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We expect to control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our studies or trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs will be required to comply with GLP and GCP, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our product candidates that are in preclinical and clinical development, respectively. The regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Although we rely on CROs to conduct any future GLP-compliant preclinical and preclinical studies and current or planned GCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical studies and clinical trials is conducted in accordance with our investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. If we or our CROs fail to comply with GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

While we will have agreements governing their activities, our CROs are and will not be our employees, and we will not control whether or not they devote sufficient time and resources to our future clinical and preclinical programs. These CROs 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 harm our business. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or

accuracy of the clinical data they obtain is compromised due to the failure to adhere to its clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

If our relationships with these CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business and financial condition. Further, we currently rely on two CROs to conduct our ongoing clinical trials and may engage one of these same CROs to conduct additional clinical trials on our behalf. To the extent that these CROs fail to comply with GLP or their contractual obligations to us for any reason, the negative impact on our business and financial condition could be more profound than if we relied on a greater number of CROs.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

Recent acquisitions and potential future acquisitions could prove difficult to integrate, disrupt our business, dilute stockholder value and strain our resources.

We completed our acquisition of Alizé Pharma SAS, or Alizé, through which we acquired livoletide, our PWS product candidate, in December 2017. In the future, we may acquire additional companies, technologies or product candidates that we believe could complement or expand our business. Integrating the operations of acquired businesses successfully or otherwise realizing any of the anticipated benefits of acquisitions involves a number of potential challenges. The failure to meet these integration challenges could seriously harm our financial condition and results of operations. Realizing the benefits of acquisitions depends in part on the integration of operations and personnel. These integration activities are complex and time-consuming, and we may encounter unexpected difficulties or incur unexpected costs, including with respect to:

- diversion of management attention from ongoing business concerns to integration matters;
- coordinating clinical and preclinical development plans;
- consolidating and rationalizing information technology and accounting platforms and administrative infrastructures;
- complexities associated with managing the geographic separation of the combined businesses and consolidating multiple physical locations;
- discontinuation of operations of OvaScience and contingent liabilities we assumed in connection with the Merger;
- reconciling different corporate cultures; and
- retaining scientific and other key employees.

Acquired businesses may have liabilities, adverse operating issues or other matters of concern arise following the acquisition that we fail to discover through due diligence prior to the acquisition. Further, our acquisition targets may not have as robust internal controls over financial reporting as would be expected of a public company. Acquisitions may also result in the recording of goodwill and other intangible assets that are subject to potential impairment in the future that could harm our financial results. We may also become subject to new regulations as a result of an acquisition, including if we acquire operations in a country in which we do not already operate. If we fail to properly evaluate

acquisitions or unanticipated issues arise following the acquisition, we may incur costs in excess of what we anticipate and may not otherwise achieve the anticipated benefits of any such acquisitions.

We are highly dependent on the services of our key executives and personnel, including Julia C. Owens, Ph.D., our chief executive officer, Louis Arcudi III, our chief financial officer, and Pharis Mohideen, MD, our chief medical officer, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.

We are highly dependent on Drs. Owens and Mohideen and Mr. Arcudi. The employment agreements we have with these officers do not prevent such persons from terminating their employment with us at any time. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

In addition, we are dependent on our continued ability to attract, retain and motivate highly qualified additional management, clinical and scientific personnel. If we are not able to retain our management and to attract, on acceptable terms, additional qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or grow.

We may not be able to attract or retain qualified personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. Many of the other pharmaceutical companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles, are located in geographies with a larger biotechnology industry presence and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract, retain and motivate high-quality personnel and consultants to accomplish our business objectives, the rate and success at which we can discover and develop product candidates and our business will be limited and we may experience constraints on our development objectives.

Our future performance will also depend, in part, on our ability to successfully integrate newly hired executive officers into our management team and our ability to develop an effective working relationship among senior management. Our failure to integrate these individuals and create effective working relationships among them and other members of management could result in inefficiencies in the development and commercialization of our product candidates, harming future regulatory approvals, sales of our product candidates and our results of operations. Additionally, we do not currently maintain "key person" life insurance on the lives of our executives or any of our employees.

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

As of March 31, 2019, we had 34 employees, 32 of whom were full-time and two of whom were part-time employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal and other resources. Our management may need to divert a disproportionate amount of our attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational inefficiencies, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of our current and future product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and grow revenue could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates, develop a scalable infrastructure and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Our employees, independent contractors, principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in other jurisdictions, provide accurate information to the FDA and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us and we are not successful in defending itself or asserting our rights, those actions could have a negative impact on our business, financial condition and results of operations, including the imposition of significant fines or other sanctions.

We may be delayed in our receipt of certain tax benefits that Alizé historically received as a French technology company.

As a French technology company, Alizé historically benefited from certain tax advantages, including the French research tax credit (*credit d'impot recherche*), or CIR. The CIR is a French tax credit aimed at stimulating research and development, and can offset French corporate income tax due. Alizé has historically received CIR reimbursements promptly following filing for such reimbursements with applicable French taxing authorities. During the year ended December 31, 2017, claims were made totaling $1.0 million, which we received in the first quarter of 2019. During the year ended December 31, 2018, claims were made totaling $1.4 million. Following our acquisition of Alizé, the combined business may no longer qualify as a French small or medium size enterprise, and, accordingly, the combined business may be subject to a three-year waiting period for reimbursement of CIRs, which could adversely affect the combined business's results of operations and cash flows.

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

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we are not aware of any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

We may be exposed to significant foreign exchange risk.

We incur portions of our expenses, and may in the future derive revenue, in currencies other than the U.S. dollar, in particular, the euro. As a result, we are exposed to foreign currency exchange risk as our results of operations and cash flows are subject to fluctuations in foreign currency exchange rates. We currently do not engage in hedging transactions to protect against uncertainty in future exchange rates between particular foreign currencies and the euro. Therefore, for

example, an increase in the value of the euro against the U.S. dollar could be expected to have a negative impact on our operating expenses as euro denominated expenses, if any, would be translated into U.S. dollars at an increased value. We cannot predict the impact of foreign currency fluctuations, and foreign currency fluctuations in the future may adversely affect our financial condition, results of operations and cash flows.

Risks Related to Ownership of Our Common Stock and Our Status as a Public Company

The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could incur substantial losses.

Our stock price may be volatile. The stock market in general and the market for biopharmaceutical 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, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- the commencement, enrollment or results of our clinical trials or changes in the development status of our product candidates;
- any delay in our regulatory filings for any product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results from, delays in or termination of clinical trials;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- unanticipated serious safety concerns related to the use of our product candidates;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the structure of healthcare payment systems;
- changes in the market valuations of similar companies;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors' general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;

- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we continue to have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

Future sales of our common stock in the public market could cause our share price to decline.

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to the restrictions and limitations described below. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

In connection with the Merger, stockholders holding approximately 58.4% of our common stock outstanding are subject to lock-up restrictions restricting their sale or transfer of our shares until June 6, 2019, or the Lock-Up Period, and, will, after the expiration of such Lock-Up Period, have the right, subject to various conditions and limitations, to include their shares of our common stock in registration statements relating to our securities. Additionally, 1,866,574 of our shares are currently registered for resale and are freely tradeable on Form S-3 and the holders of approximately 9,090,379 shares of our common stock, or their transferees, have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

In addition, on April 5, 2019, we filed a registration statement on Form S-8 under the Securities Act registering the issuance of previously unregistered shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under this registration statement on Form S-8 are available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and the restrictions of Rule 144 in the case of our affiliates.

Provisions in our certificate of incorporation and by-laws 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 certificate of incorporation and by-laws 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 common stockholders might otherwise receive a premium price 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 not all members of the board are 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 stockholder proposals that can be acted on at stockholder meetings and for nominations to our board of directors;
- limit who may call stockholder meetings;
- prohibit actions by our stockholders by written consent;
- require that stockholder actions be effected at a duly called stockholders meeting;
- 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 percent of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or by-laws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns 15 percent or more 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 15 percent or more of our outstanding voting stock, unless the merger or combination is approved in a manner prescribed by the statute.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent our other stockholders from influencing significant corporate decisions.

As of March 31, 2019, our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates, in the aggregate, beneficially own 60.8% of our outstanding common stock. As a result, these persons, acting together, can significantly influence all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current market price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders.

We are at risk of securities class action and similar litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of our securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. We remain the subject of various securities class action lawsuits and shareholder derivative lawsuits that were filed against OvaScience and certain of its officer and directors, as described in more detail in Item 3, Legal Proceedings. These lawsuits, as well as any similar lawsuits initiated in the future, could result in substantial cost and a diversion of management's attention and resources, which could harm our business.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Sarbanes-Oxley Act and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. Notwithstanding that we do not qualify for the relief afforded by Instruction 1 to Item 308 of Regulation S-K to newly public companies, our management has not assessed nor attested to our internal control over financial reporting as is set forth in Item 308 of Regulation S-K promulgated under the Exchange Act, and Section 404 of the Sarbanes-Oxley Act as of December 31, 2018, the end of our last fiscal year. We were unable to conduct the required assessment primarily due to the Merger occurring in the fourth quarter of 2018 and the substantial change in operational focus, management and the internal control environment following the Merger. We intend to do our first internal control assessment as of December 31, 2019.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the Securities and Exchange Commission, or SEC, or other regulatory authorities.

We expect to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance with our public company responsibilities and corporate governance practices.

As a relatively new public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Capital Market and other applicable securities rules and regulations impose various requirements on public companies. Our management and other personnel need to devote a substantial amount of time to compliance with these requirements. Moreover, these rules and regulations increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain directors' and officers' liability insurance, compared to when we were a private company, which could make it more difficult for us to attract and retain qualified members of our board of directors. We cannot predict or estimate the amount of additional costs we will continue to incur as a public company or the timing of such costs.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law the Tax Act which significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes

to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), effective for net operating losses incurred in taxable years beginning after December 31, 2017, limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new federal tax law is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain how various states will respond to the newly enacted federal tax law. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge you to consult with your legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We are subject to taxation in more than one tax jurisdiction. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of the newly enacted federal income tax law, changes in the mix of our profitability from jurisdiction to jurisdiction, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

We might not be able to utilize a significant portion of our net operating loss carryforwards.

As of December 31, 2018, we had federal and state net operating loss carryforwards of $249.6 million and $249.2 million, respectively. The federal and state net operating loss carryforwards will begin to expire, if not utilized, by 2031. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the newly enacted federal income tax law, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain how various states will respond to the newly enacted federal tax law. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future.

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any existing or future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

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

Recent Sales of Unregistered Securities

We did not sell any unregistered securities during the three months ended March 31, 2019.

Issuer Purchases of Equity Securities

We did not repurchase any securities during the three months ended March 31, 2019.

Item 3. Defaults upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Not applicable.

Item 6. Exhibits

The following exhibits are incorporated by reference or filed as part of this report.

Exhibit Number	Description	SEC File No.
3.1*	Restated Certificate of Incorporation of the Registrant, as amended	
3.2	Third Amended and Restated Bylaws, as Amended, of the Registrant (incorporated by reference from Exhibit 3.1 to the Current Report on Form 8-K filed with the Securities and Exchange Commission on August 9, 2018 File No. 001-35890)	
10.1	Lease Agreement (incorporated by reference from Exhibit 10.1 to the Current Report on Form 8-K filed with the Securities and Exchange Commission on February 7, 2019 File No. 001-35890)	
10.2	Amended and Restated Lease Extension and Modification Agreement (incorporated by reference from Exhibit 10.2 to the Current Report on Form 8-K filed with the Securities and Exchange Commission on February 7, 2019 File No. 001-35890)	
31.1*	Certification of Chief Executive Officer (Principal Executive Officer) pursuant to Section 302 of Sarbanes-Oxley Act of 2002	
31.2*	Certification of Chief Financial Officer (Principal Financial Officer) pursuant to Section 302 of Sarbanes-Oxley Act of 2002	
32.1+	Certification of Chief Executive Officer (Principal Executive Officer) and Chief Financial Officer (Principal Financial Officer) pursuant to Section 906 of Sarbanes-Oxley Act of 2002	
101.INS	XBRL Instance Document	
101.SCH	XBRL Taxonomy Extension Schema Document	
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document	
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document	
101.LAB	XBRL Taxonomy Extension Label Linkbase Document	
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document	

* Filed herewith.

+ This certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing of the registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

SIGNATURE

Pursuant to the requirements of Section 13 or 15(d) 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.

MILLENDO THERAPEUTICS, INC.

By: /s/ Julia C. Owens, Ph.D.
Julia C. Owens, Ph.D.
President and Chief Executive Officer (Principal Executive Officer)

By: /s/ Louis Arcudi III
Louis Arcudi III
Chief Financial Officer (Principal Financial and Accounting Officer)

Date: May 15, 2019

Exhibit 3.1

RESTATED CERTIFICATE OF INCORPORATION

OF

OVASCIENCE, INC.

OvaScience, Inc. (the "Corporation"), a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware, does hereby certify as follows:

The original Certificate of Incorporation was filed with the Secretary of State of the State of Delaware on April 5, 2011 under the name OvaStem, Inc.

A resolution was duly adopted by the Board of Directors of the Corporation pursuant to Sections 242 and 245 of the General Corporation Law of the State of Delaware setting forth this Restated Certificate of Incorporation and declaring such Restated Certificate of Incorporation advisable. The stockholders of the Corporation duly approved and adopted this Restated Certificate of Incorporation by written consent in accordance with Sections 228, 242 and 245 of the General Corporation Law of the State of Delaware.

Accordingly, the Certificate of Incorporation of this Corporation, as previously amended and restated, is hereby further amended and restated in its entirety to read as follows:

FIRST: The name of the Corporation is OvaScience, Inc.

SECOND: The address of the Corporation's registered office in the State of Delaware is 901 N. Market Street, Suite 705, in the City of Wilmington, County of New Castle, 19801. The name of its registered agent at that address is Delaware Corporate Services Inc.

THIRD: The nature of the business or purposes to be conducted or promoted by the Corporation is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law of the State of Delaware.

FOURTH: The total number of shares of all classes of stock which the Corporation shall have authority to issue is 105,000,000 shares, consisting of (i) 100,000,000 shares of Common Stock, $0.001 par value per share ("Common Stock"), and (ii) 5,000,000 shares of Preferred Stock, $0.001 par value per share ("Preferred Stock").

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

A COMMON STOCK.

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights of the holders of the Preferred Stock of any series as may be designated by the Board of Directors upon any issuance of the Preferred Stock of any series.

2. Voting. The holders of the Common Stock shall have voting rights at all meetings of stockholders, each such holder being entitled to one vote for each share thereof held by such holder; provided, however, that, except as otherwise required by law, holders of Common Stock shall not be entitled to vote on any amendment to this Certificate of Incorporation (which, as used herein, shall mean the certificate of incorporation of the Corporation, as amended from time to time, including the terms of any certificate of designations of any series of Preferred Stock) that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together as a class with the holders of one or more other such series, to vote thereon pursuant to this Certificate of Incorporation. There shall be no cumulative voting.

The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by the affirmative vote of the holders of a majority of the stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law of the State of Delaware.

3. Dividends. Dividends may be declared and paid on the Common Stock from funds lawfully available therefor as and when determined by the Board of Directors and subject to any preferential dividend or other rights of any then outstanding Preferred Stock.

4. Liquidation. Upon the dissolution or liquidation of the Corporation, whether voluntary or involuntary, holders of Common Stock will be entitled to receive all assets of the Corporation available for distribution to its stockholders, subject to any preferential or other rights of any then outstanding Preferred Stock.

B PREFERRED STOCK.

Preferred Stock may be issued from time to time in one or more series, each of such series to have such terms as stated or expressed herein and in the resolution or resolutions providing for the issue of such series adopted by the Board of Directors of the Corporation as hereinafter provided. Any shares of Preferred Stock which may be redeemed, purchased or acquired by the Corporation may be reissued except as otherwise provided by law.

Authority is hereby expressly granted to the Board of Directors from time to time to issue the Preferred Stock in one or more series, and in connection with the creation of any such series, by adopting a resolution or resolutions providing for the issuance of the shares thereof and by filing a certificate of designations relating thereto in accordance with the General Corporation Law of the State of Delaware, to determine and fix the number of shares of such series and such voting powers, full or limited, or no voting powers, and such designations, preferences and relative participating, optional or other special rights, and qualifications, limitations or restrictions thereof, including without limitation thereof, dividend rights, conversion rights, redemption privileges and liquidation preferences, as shall be stated and expressed in such resolutions, all to the full extent now or hereafter permitted by the General Corporation Law of the State of Delaware. Without limiting the generality of the foregoing, the resolutions providing for issuance of any series of Preferred Stock may provide that such series shall be superior or rank equally or be junior to any other series of Preferred Stock to the extent permitted by law.

The number of authorized shares of Preferred Stock may be increased or decreased (but not below the number of shares then outstanding) by the affirmative vote of the holders of a majority of the voting power of the capital stock of the Corporation entitled to vote thereon, voting as a single class, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law of the State of Delaware.

FIFTH: Except as otherwise provided herein, the Corporation reserves the right to amend, alter, change or repeal any provision contained in this Certificate of Incorporation, in the manner now or hereafter prescribed by statute and this Certificate of Incorporation, and all rights conferred upon stockholders herein are granted subject to this reservation.

SIXTH: In furtherance and not in limitation of the powers conferred upon it by the General Corporation Law of the State of Delaware, and subject to the terms of any series of Preferred Stock, the Board of Directors shall have the power to adopt, amend, alter or repeal the By-laws of the Corporation by the affirmative vote of a majority of the directors present at any regular or special meeting of the Board of Directors at which a quorum is present. The stockholders may not adopt, amend, alter or repeal the By-laws of the Corporation, or adopt any provision inconsistent therewith, unless such action is approved, in addition to any other vote required by this Certificate of Incorporation, by the affirmative vote of the holders of at least seventy-five percent (75%) of the votes that all the stockholders would be entitled to cast in any annual election of directors or class of directors. Notwithstanding any other provisions of law, this Certificate of Incorporation or the By-laws of the Corporation, and notwithstanding the fact that a lesser percentage may be specified by law, the affirmative vote of the holders of at least seventy-five percent (75%) of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors shall be required to amend or repeal, or to adopt any provision inconsistent with, this Article SIXTH.

SEVENTH: Except to the extent that the General Corporation Law of the State of Delaware prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty, no director of the Corporation shall be personally liable to the Corporation or its stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability. No amendment to or repeal of this provision shall apply to or have any effect on the liability or alleged liability of any director of the Corporation for or with respect to any acts or omissions of such director occurring prior to such amendment or repeal. If the General Corporation Law of the State of Delaware is amended to permit further elimination or limitation of the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law of the State of Delaware as so amended.

EIGHTH: The Corporation shall provide indemnification as follows:

1. Actions, Suits and Proceedings Other than by or in the Right of the Corporation. The Corporation shall indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the Corporation) by reason of the fact that he or she is or was, or has agreed to become, a director or officer of the Corporation, or is or was serving, or has agreed to serve, at the request of the Corporation, as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan) (all such persons being referred to hereafter as an "Indemnitee"), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), liabilities, losses, judgments, fines (including excise taxes and penalties arising under the Employee Retirement Income Security Act of 1974), and amounts paid in settlement actually and reasonably incurred by or on behalf of Indemnitee in connection with such action, suit or proceeding and any appeal therefrom, if Indemnitee acted in good faith and in a manner which Indemnitee reasonably believed to be in, or not opposed to, the best interests of the Corporation, and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful. The termination of any action, suit or proceeding by judgment, order, settlement, conviction or upon a plea of nolo contendere or its equivalent, shall not, of itself, create a presumption that Indemnitee did not act in good faith and in a manner which Indemnitee reasonably believed to be in, or not opposed to, the best interests of the Corporation, and, with respect to any criminal action or proceeding, had reasonable cause to believe that his or her conduct was unlawful.

2. Actions or Suits by or in the Right of the Corporation. The Corporation shall indemnify any Indemnitee who was or is a party to or threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the Corporation to procure a judgment in its favor by reason of the fact that Indemnitee is or was, or has agreed to become, a director or officer of the Corporation, or is or was serving, or has agreed to serve, at the request of the Corporation, as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred by or on behalf of Indemnitee in connection with such action, suit or proceeding and any appeal therefrom, if Indemnitee acted in good faith and in a manner which Indemnitee reasonably believed to be in, or not opposed to, the best interests of the Corporation, except that no indemnification shall be made under this Section 2 in respect of any claim, issue or matter as to which Indemnitee shall have been adjudged to be liable to the Corporation, unless, and only to the extent, that the Court of Chancery of Delaware or the court in which such action or suit was brought shall determine upon application that, despite the adjudication of such liability but in view of all the circumstances of the case, Indemnitee is fairly and reasonably entitled to indemnity for such expenses (including attorneys' fees) which the Court of Chancery of Delaware or such other court shall deem proper.

3. Indemnification for Expenses of Successful Party. Notwithstanding any other provisions of this Article EIGHTH, to the extent that an Indemnitee has been successful, on the merits or otherwise, in defense of any action, suit or proceeding referred to in Sections 1 and 2 of this Article EIGHTH, or in defense of any claim, issue or matter therein, or on appeal from any such action, suit or proceeding, Indemnitee shall be indemnified against all expenses (including attorneys' fees) actually and reasonably incurred by or on behalf of Indemnitee in connection therewith. Without limiting the foregoing, if any action, suit or proceeding is disposed of, on the merits or otherwise (including a disposition without prejudice), without (i) the disposition being adverse to Indemnitee, (ii) an

adjudication that Indemnitee was liable to the Corporation, (iii) a plea of guilty or nolo contendere by Indemnitee, (iv) an adjudication that Indemnitee did not act in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the Corporation, and (v) with respect to any criminal proceeding, an adjudication that Indemnitee had reasonable cause to believe his or her conduct was unlawful, Indemnitee shall be considered for the purposes hereof to have been wholly successful with respect thereto.

4. Notification and Defense of Claim. As a condition precedent to an Indemnitee's right to be indemnified, such Indemnitee must notify the Corporation in writing as soon as practicable of any action, suit, proceeding or investigation involving such Indemnitee for which indemnity will or could be sought. With respect to any action, suit, proceeding or investigation of which the Corporation is so notified, the Corporation will be entitled to participate therein at its own expense and/or to assume the defense thereof at its own expense, with legal counsel reasonably acceptable to Indemnitee. After notice from the Corporation to Indemnitee of its election so to assume such defense, the Corporation shall not be liable to Indemnitee for any legal or other expenses subsequently incurred by Indemnitee in connection with such action, suit, proceeding or investigation, other than as provided below in this Section 4. Indemnitee shall have the right to employ his or her own counsel in connection with such action, suit, proceeding or investigation, but the fees and expenses of such counsel incurred after notice from the Corporation of its assumption of the defense thereof shall be at the expense of Indemnitee unless (i) the employment of counsel by Indemnitee has been authorized by the Corporation, (ii) counsel to Indemnitee shall have reasonably concluded that there may be a conflict of interest or position on any significant issue between the Corporation and Indemnitee in the conduct of the defense of such action, suit, proceeding or investigation or (iii) the Corporation shall not in fact have employed counsel to assume the defense of such action, suit, proceeding or investigation, in each of which cases the fees and expenses of counsel for Indemnitee shall be at the expense of the Corporation, except as otherwise expressly provided by this Article EIGHTH. The Corporation shall not be entitled, without the consent of Indemnitee, to assume the defense of any claim brought by or in the right of the Corporation or as to which counsel for Indemnitee shall have reasonably made the conclusion provided for in clause (ii) above. The Corporation shall not be required to indemnify Indemnitee under this Article EIGHTH for any amounts paid in settlement of any action, suit, proceeding or investigation effected without its written consent. The Corporation shall not settle any action, suit, proceeding or investigation in any manner which would impose any penalty or limitation on Indemnitee without Indemnitee's written consent. Neither the Corporation nor Indemnitee will unreasonably withhold or delay its consent to any proposed settlement.

5. Advance of Expenses. Subject to the provisions of Section 6 of this Article EIGHTH, in the event of any threatened or pending action, suit, proceeding or investigation of which the Corporation receives notice under this Article EIGHTH, any expenses (including attorneys' fees) incurred by or on behalf of Indemnitee in defending an action, suit, proceeding or investigation or any appeal therefrom shall be paid by the Corporation in advance of the final disposition of such matter; provided, however, that the payment of such expenses incurred by or on behalf of Indemnitee in advance of the final disposition of such matter shall be made only upon receipt of an undertaking by or on behalf of Indemnitee to repay all amounts so advanced in the event that it shall ultimately be determined by final judicial decision from which there is no further right to appeal that Indemnitee is not entitled to be indemnified by the Corporation as authorized in this Article EIGHTH; and provided further that no such advancement of expenses shall be made under this Article EIGHTH if it is determined (in the manner described in Section 6) that (i) Indemnitee did not act in good faith and in a manner he or she reasonably believed to be in, or not opposed to, the best interests of the Corporation, or (ii) with respect to any criminal action or proceeding, Indemnitee had reasonable cause to believe his or her conduct was unlawful. Such undertaking shall be accepted without reference to the financial ability of Indemnitee to make such repayment.

6. Procedure for Indemnification and Advancement of Expenses. In order to obtain indemnification or advancement of expenses pursuant to Section 1, 2, 3 or 5 of this Article EIGHTH, an Indemnitee shall submit to the Corporation a written request. Any such advancement of expenses shall be made promptly, and in any event within 60 days after receipt by the Corporation of the written request of Indemnitee, unless (i) the Corporation has assumed the defense pursuant to Section 4 of this Article EIGHTH (and none of the circumstances described in Section 4 of this Article EIGHTH that would nonetheless entitle the Indemnitee to indemnification for the fees and expenses of separate counsel have occurred) or (ii) the Corporation determines within such 60-day period that Indemnitee did not meet the applicable standard of conduct set forth in Section 1, 2 or 5 of this Article EIGHTH, as the case may be. Any such indemnification, unless ordered by a court, shall be made with respect to requests under Section 1 or 2 only as authorized in the specific case upon a determination by the Corporation that the

indemnification of Indemnitee is proper because Indemnitee has met the applicable standard of conduct set forth in Section 1 or 2, as the case may be. Such determination shall be made in each instance (a) by a majority vote of the directors of the Corporation consisting of persons who are not at that time parties to the action, suit or proceeding in question ("disinterested directors"), whether or not a quorum, (b) by a committee of disinterested directors designated by majority vote of disinterested directors, whether or not a quorum, (c) if there are no disinterested directors, or if the disinterested directors so direct, by independent legal counsel (who may, to the extent permitted by law, be regular legal counsel to the Corporation) in a written opinion, or (d) by the stockholders of the Corporation.

7. Remedies. The right to indemnification or advancement of expenses as granted by this Article EIGHTH shall be enforceable by Indemnitee in any court of competent jurisdiction. Neither the failure of the Corporation to have made a determination prior to the commencement of such action that indemnification is proper in the circumstances because Indemnitee has met the applicable standard of conduct, nor an actual determination by the Corporation pursuant to Section 6 of this Article EIGHTH that Indemnitee has not met such applicable standard of conduct, shall be a defense to the action or create a presumption that Indemnitee has not met the applicable standard of conduct. In any suit brought by Indemnitee to enforce a right to indemnification, or brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall have the burden of proving that Indemnitee is not entitled to be indemnified, or to such advancement of expenses, under this Article EIGHTH. Indemnitee's expenses (including attorneys' fees) reasonably incurred in connection with successfully establishing Indemnitee's right to indemnification, in whole or in part, in any such proceeding shall also be indemnified by the Corporation. Notwithstanding the foregoing, in any suit brought by Indemnitee to enforce a right to indemnification hereunder it shall be a defense that the Indemnitee has not met any applicable standard for indemnification set forth in the General Corporation Law of the State of Delaware.

8. Limitations. Notwithstanding anything to the contrary in this Article EIGHTH, except as set forth in Section 7 of this Article EIGHTH, the Corporation shall not indemnify an Indemnitee pursuant to this Article EIGHTH in connection with a proceeding (or part thereof) initiated by such Indemnitee unless the initiation thereof was approved by the Board of Directors of the Corporation. Notwithstanding anything to the contrary in this Article EIGHTH, the Corporation shall not indemnify an Indemnitee to the extent such Indemnitee is reimbursed from the proceeds of insurance, and in the event the Corporation makes any indemnification payments to an Indemnitee and such Indemnitee is subsequently reimbursed from the proceeds of insurance, such Indemnitee shall promptly refund indemnification payments to the Corporation to the extent of such insurance reimbursement.

9. Subsequent Amendment. No amendment, termination or repeal of this Article EIGHTH or of the relevant provisions of the General Corporation Law of the State of Delaware or any other applicable laws shall adversely affect or diminish in any way the rights of any Indemnitee to indemnification under the provisions hereof with respect to any action, suit, proceeding or investigation arising out of or relating to any actions, transactions or facts occurring prior to the final adoption of such amendment, termination or repeal.

10. Other Rights. The indemnification and advancement of expenses provided by this Article EIGHTH shall not be deemed exclusive of any other rights to which an Indemnitee seeking indemnification or advancement of expenses may be entitled under any law (common or statutory), agreement or vote of stockholders or disinterested directors or otherwise, both as to action in Indemnitee's official capacity and as to action in any other capacity while holding office for the Corporation, and shall continue as to an Indemnitee who has ceased to be a director or officer, and shall inure to the benefit of the estate, heirs, executors and administrators of Indemnitee. Nothing contained in this Article EIGHTH shall be deemed to prohibit, and the Corporation is specifically authorized to enter into, agreements with officers and directors providing indemnification rights and procedures different from those set forth in this Article EIGHTH. In addition, the Corporation may, to the extent authorized from time to time by its Board of Directors, grant indemnification rights to other employees or agents of the Corporation or other persons serving the Corporation and such rights may be equivalent to, or greater or less than, those set forth in this Article EIGHTH.

11. Partial Indemnification. If an Indemnitee is entitled under any provision of this Article EIGHTH to indemnification by the Corporation for some or a portion of the expenses (including attorneys' fees), liabilities, losses, judgments, fines (including excise taxes and penalties arising under the Employee Retirement Income

Security Act of 1974) or amounts paid in settlement actually and reasonably incurred by or on behalf of Indemnitee in connection with any action, suit, proceeding or investigation and any appeal therefrom but not, however, for the total amount thereof, the Corporation shall nevertheless indemnify Indemnitee for the portion of such expenses (including attorneys' fees), liabilities, losses, judgments, fines (including excise taxes and penalties arising under the Employee Retirement Income Security Act of 1974) or amounts paid in settlement to which Indemnitee is entitled.

12. Insurance. The Corporation may purchase and maintain insurance, at its expense, to protect itself and any director, officer, employee or agent of the Corporation or another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan) against any expense, liability or loss incurred by him or her in any such capacity, or arising out of his or her status as such, whether or not the Corporation would have the power to indemnify such person against such expense, liability or loss under the General Corporation Law of the State of Delaware.

13. Savings Clause. If this Article EIGHTH or any portion hereof shall be invalidated on any ground by any court of competent jurisdiction, then the Corporation shall nevertheless indemnify each Indemnitee as to any expenses (including attorneys' fees), liabilities, losses, judgments, fines (including excise taxes and penalties arising under the Employee Retirement Income Security Act of 1974) and amounts paid in settlement in connection with any action, suit, proceeding or investigation, whether civil, criminal or administrative, including an action by or in the right of the Corporation, to the fullest extent permitted by any applicable portion of this Article EIGHTH that shall not have been invalidated and to the fullest extent permitted by applicable law.

14. Definitions. Terms used herein and defined in Section 145(h) and Section 145(i) of the General Corporation Law of the State of Delaware shall have the respective meanings assigned to such terms in such Section 145(h) and Section 145(i).

NINTH: This Article NINTH is inserted for the management of the business and for the conduct of the affairs of the Corporation.

1. General Powers. The business and affairs of the Corporation shall be managed by or under the direction of the Board of Directors.

2. Number of Directors; Election of Directors. Subject to the rights of holders of any series of Preferred Stock to elect directors, the number of directors of the Corporation shall be established by the Board of Directors. Election of directors need not be by written ballot, except as and to the extent provided in the By-laws of the Corporation.

3. Classes of Directors. Subject to the rights of holders of any series of Preferred Stock to elect directors, the Board of Directors shall be and is divided into three classes, designated Class I, Class II and Class III. Each class shall consist, as nearly as may be possible, of one-third of the total number of directors constituting the entire Board of Directors. The Board of Directors is authorized to assign members of the Board of Directors already in office to Class I, Class II or Class III at the time such classification becomes effective.

4. Terms of Office. Subject to the rights of holders of any series of Preferred Stock to elect directors, each director shall serve for a term ending on the date of the third annual meeting of stockholders following the annual meeting of stockholders at which such director was elected; provided that each director initially assigned to Class I shall serve for a term expiring at the Corporation's first annual meeting of stockholders held after December 31, 2012; each director initially assigned to Class II shall serve for a term expiring at the Corporation's second annual meeting of stockholders held after December 31, 2012; and each director initially assigned to Class III shall serve for a term expiring at the Corporation's third annual meeting of stockholders held after December 31, 2012; provided further, that the term of each director shall continue until the election and qualification of his or her successor and be subject to his or her earlier death, resignation or removal.

5. Quorum. The greater of (a) a majority of the directors at any time in office and (b) one-third of the number of directors fixed pursuant to Section 2 of this Article NINTH shall constitute a quorum of the Board of Directors. If at any meeting of the Board of Directors there shall be less than such a quorum, a majority of the

directors present may adjourn the meeting from time to time without further notice other than announcement at the meeting, until a quorum shall be present.

6. Action at Meeting. Every act or decision done or made by a majority of the directors present at a meeting duly held at which a quorum is present shall be regarded as the act of the Board of Directors unless a greater number is required by law or by this Certificate of Incorporation.

7. Removal. Subject to the rights of holders of any series of Preferred Stock, directors of the Corporation may be removed only for cause and only by the affirmative vote of the holders of at least seventy-five percent (75%) of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors.

8. Vacancies. Subject to the rights of holders of any series of Preferred Stock, any vacancy or newly created directorship in the Board of Directors, however occurring, shall be filled only by vote of a majority of the directors then in office, although less than a quorum, or by a sole remaining director and shall not be filled by the stockholders. A director elected to fill a vacancy shall hold office until the next election of the class for which such director shall have been chosen, subject to the election and qualification of a successor and to such director's earlier death, resignation or removal.

9. Stockholder Nominations and Introduction of Business, Etc. Advance notice of stockholder nominations for election of directors and other business to be brought by stockholders before a meeting of stockholders shall be given in the manner provided by the By-laws of the Corporation.

10. Amendments to Article. Notwithstanding any other provisions of law, this Certificate of Incorporation or the By-laws of the Corporation, and notwithstanding the fact that a lesser percentage may be specified by law, the affirmative vote of the holders of at least seventy-five percent (75%) of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors shall be required to amend or repeal, or to adopt any provision inconsistent with, this Article NINTH.

TENTH: Stockholders of the Corporation may not take any action by written consent in lieu of a meeting. Notwithstanding any other provisions of law, this Certificate of Incorporation or the By-laws of the Corporation, and notwithstanding the fact that a lesser percentage may be specified by law, the affirmative vote of the holders of at least seventy-five percent (75%) of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors shall be required to amend or repeal, or to adopt any provision inconsistent with, this Article TENTH.

ELEVENTH: Special meetings of stockholders for any purpose or purposes may be called at any time by only the Board of Directors, the Chairman of the Board or the Chief Executive Officer, and may not be called by any other person or persons. Business transacted at any special meeting of stockholders shall be limited to matters relating to the purpose or purposes stated in the notice of meeting. Notwithstanding any other provisions of law, this Certificate of Incorporation or the By-laws of the Corporation, and notwithstanding the fact that a lesser percentage may be specified by law, the affirmative vote of the holders of at least seventy-five percent (75%) of the votes which all the stockholders would be entitled to cast in any annual election of directors or class of directors shall be required to amend or repeal, or to adopt any provision inconsistent with, this Article ELEVENTH.

IN WITNESS WHEREOF, this Restated Certificate of Incorporation, which restates, integrates and amends the certificate of incorporation of the Corporation, and which has been duly adopted in accordance with Sections 228, 242 and 245 of the General Corporation Law of the State of Delaware, has been executed by its duly authorized officer this 30th day of April, 2013.

OVASCIENCE, INC.

By: /s/ Michelle Dipp, M.D., Ph.D.
Michelle Dipp, M.D., Ph.D.
President and Chief Executive Officer

CERTIFICATE OF AMENDMENT
TO THE
RESTATED
CERTIFICATE OF INCORPORATION
OF
OVASCIENCE, INC.

OvaScience, Inc. (the "***Corporation***"), a corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware, as amended (the "***DGCL***"), hereby certifies as follows:

A. The name of the Corporation is OvaScience, Inc., and the original certificate of incorporation of the Corporation was filed with the Secretary of State of the State of Delaware on April 5, 2011 under the name OvaStem, Inc. A Restated Certificate of Incorporation of the Corporation was filed with the Secretary of State of the State of Delaware on April 30, 2013 (the "***Prior Certificate***").

B. This Certificate of Amendment to the Restated Certificate of Incorporation (the "***Certificate of Amendment***") amends the Prior Certificate, and has been duly adopted by the Corporation's Board of Directors and stockholders in accordance with the provisions of Sections 141, 211 and 242 of the DGCL.

C. Article FOURTH of the Prior Certificate is hereby amended to add the following Section C:

> "C. Immediately upon the filing of this Certificate of Amendment to the Restated Certificate of Incorporation with the Secretary of State of the State of Delaware every fifteen (15) shares of Common Stock issued and outstanding (or held in treasury) immediately prior to such filing shall be automatically reclassified and combined into one (1) validly issued, fully paid and non-assessable share of Common Stock. The aforementioned reclassification shall be referred to collectively as the "Reverse Split."
>
> The Reverse Split shall occur without any further action on the part of the Corporation or the stockholders of the Corporation and whether or not certificates representing such stockholders' shares prior to the Reverse Split are surrendered for cancellation. No fractional interest in a share of Common Stock shall be deliverable upon the Reverse Split. All shares of Common Stock (including fractions thereof) issuable upon the Reverse Split held by a holder prior to the Reverse Split shall be aggregated for purposes of determining whether the Reverse Split would result in the issuance of any fractional share. Any fractional share resulting from such aggregation upon the Reverse Split shall be rounded down to the nearest whole number. Each holder who would otherwise be entitled to a fraction of a share of Common Stock upon the Reverse Split (after aggregating all fractions of a share to which such stockholder would otherwise be entitled) shall, in lieu thereof, be entitled to receive a cash payment in an amount equal to the product of such fraction to which the stockholder would otherwise be entitled multiplied by the closing price of the Corporation's Common Stock as reported on the Nasdaq Capital Market on the trading day immediately preceding the filing of this Certificate of Amendment to the Restated Certificate of Incorporation with the Secretary of State of the State of Delaware (as adjusted to give effect to the Reverse Split), rounded up to the nearest whole cent. The Corporation shall not be obliged to issue certificates evidencing the shares of Common Stock outstanding as a result of the Reverse Split or cash in lieu of fractional shares, if any, unless and until the certificates evidencing the shares held by a holder prior to the Reverse Split are either delivered to the Corporation or its transfer agent, or the holder notifies the Corporation or its transfer agent that such certificates have been lost, stolen or destroyed and executes an agreement satisfactory to the Corporation to indemnify the Corporation from any loss incurred by it in connection with such certificates."

D. The Certificate of Amendment so adopted reads in full as set forth above and is hereby incorporated by reference. All other provisions of the Prior Certificate remain in full force and effect.

IN WITNESS WHEREOF, OvaScience, Inc. has caused this Certificate of Amendment to be signed by Christopher Kroeger, M.D., M.B.A., a duly authorized officer of the Corporation, on December 6, 2018.

OVASCIENCE, INC.

By: /s/ Christopher Kroeger, M.D., M.B.A.
Name: Christopher Kroeger, M.D., M.B.A.
Title: *President and Chief Executive Officer*

CERTIFICATE OF AMENDMENT
TO THE
RESTATED
CERTIFICATE OF INCORPORATION
OF
OVASCIENCE, INC.

OvaScience, Inc. (the "***Corporation***"), a corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware, as amended (the "***DGCL***"), hereby certifies as follows:

A. The name of the Corporation is OvaScience, Inc., and the original certificate of incorporation of the Corporation was filed with the Secretary of State of the State of Delaware on April 5, 2011 under the name OvaStem, Inc. A Restated Certificate of Incorporation of the Corporation was filed with the Secretary of State of the State of Delaware on April 30, 2013 (the "***Prior Certificate***"). A certificate of Amendment to the Prior Certificate was filed with the Secretary of State of the State of Delaware on December 6, 2018.

B. This Certificate of Amendment to the Restated Certificate of Incorporation (the "***Certificate of Amendment***") amends the Prior Certificate, as amended, and has been duly adopted by the Corporation's Board of Directors and stockholders in accordance with the provisions of Sections 141, 211 and 242 of the DGCL.

C. Article FIRST of the Prior Certificate, as amended, is hereby amended and restated to read as follows:

"FIRST: The name of the Corporation is Millendo Therapeutics, Inc."

D. The Certificate of Amendment so adopted reads in full as set forth above and is hereby incorporated by reference. All other provisions of the Prior Certificate, as amended, remain in full force and effect.

IN WITNESS WHEREOF, OvaScience, Inc. has caused this Certificate of Amendment to be signed by Julia C. Owens, Ph.D., a duly authorized officer of the Corporation, on December 7, 2018.

OVASCIENCE, INC.

By: /s/ Julia C. Owens, Ph.D.
Name: Julia C. Owens, Ph.D.
Title: *President and Chief Executive Officer*

EXHIBIT 31.1

CERTIFICATION

I, Julia C. Owens, Ph.D., certify that:

1. I have reviewed this Form 10-Q of Millendo Therapeutics, Inc.;

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

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

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

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

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

Date: May 15, 2019

/s/ Julia C. Owens, Ph.D.
Julia C. Owens, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Louis Arcudi III, certify that:

1. I have reviewed this Form 10-Q of Millendo Therapeutics, Inc.;

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

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

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

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

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

Date: May 15, 2019

/s/ Louis Arcudi III
Louis Arcudi III
Chief Financial Officer
(Principal Financial Officer)

EXHIBIT 32.1

STATEMENT PURSUANT TO 18 U.S.C. § 1350

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Julia C. Owens, Ph.D., President and Chief Executive Officer (Principal Executive Officer) of Millendo Therapeutics, Inc. (the "Company") and Louis Arcudi III, Chief Financial Officer (Principal Financial Officer) of the Company, each hereby certifies that, to the best of his or her knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the period ended March 31, 2019, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report") fully complies with the requirements of Section 13(a) or 15(d) of the Exchange Act; and

(2) The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Julia C. Owens, Ph.D.
Julia C. Owens, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: May 15, 2019

/s/ Louis Arcudi III
Louis Arcudi III
Chief Financial Officer
(Principal Financial Officer)

Date: May 15, 2019

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not incorporated by reference into any filing of Millendo Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.