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, 2017

OR

☐ **Transition report pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

For the transition period from _____ to _____.

Commission File Number: 001-37833

Audentes Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**46-1606174**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**

600 California Street, 17th Floor
San Francisco, California 94108
(Address of principal executive offices and zip code)

(415) 818-1001
(Registrant's telephone number, including area code)

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

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

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

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

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

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

As of May 9, 2017, there were 27,726,614 shares of the Registrant's Common Stock, $0.00001 par value per share, outstanding.

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PART I

ITEM 1. CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(in thousands, except shares and per share amounts)

	March 31, 2017	December 31, 2016
Assets	*Unaudited*	
Current assets:		
Cash and cash equivalents	$ 18,764	$ 36,359
Short-term investments	62,964	68,524
Restricted cash	820	730
Prepaid expenses and other current assets	3,358	2,824
Total current assets	85,906	108,437
Restricted cash - long-term	3,280	3,020
Property and equipment, net	19,489	18,936
Goodwill	3,631	3,631
Intangible assets	8,000	8,000
Other assets	1,433	33
Total assets	$ 121,739	$ 142,057
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,506	$ 2,424
Accrued liabilities	7,063	9,871
Deferred rent	134	265
Total current liabilities	8,703	12,560
Deferred rent and asset retirement obligation - long-term	2,778	2,486
Contingent acquisition consideration payable	4,470	4,380
Deferred tax liability, net	3,260	3,260
Total liabilities	19,211	22,686
Stockholders' equity:		
Preferred stock, $0.00001 par value, 10,000,000 shares authorized as of March 31, 2017 and December 31, 2016; 0 shares issued and outstanding as of March 31, 2017 and December 31, 2016, respectively	—	—
Common stock, $0.00001 par value, 300,000,000 shares authorized as of March 31, 2017 and December 31, 2016; 21,767,984 and 21,731,259 shares issued and outstanding as of March 31, 2017 and December 31, 2016, respectively	—	—
Additional paid-in capital	221,083	219,811
Accumulated deficit	(118,526)	(100,411)
Accumulated other comprehensive loss	(29)	(29)
Total stockholders' equity	102,528	119,371
Total liabilities and stockholders' equity	$ 121,739	$ 142,057

See accompanying notes to unaudited interim condensed consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except shares and per share amounts)

	Three Months Ended March 31,	
	2017	**2016**
Operating expenses:		
Research and development	$ 14,587	$ 7,906
General and administrative	3,658	2,632
Total operating expenses	18,245	10,538
Loss from operations	(18,245)	(10,538)
Interest income, net	147	97
Other expense, net	(17)	(23)
Net loss	(18,115)	(10,464)
Unrealized gains on short-term investments	—	14
Comprehensive loss	$ (18,115)	$ (10,450)
Net loss per share, basic and diluted	$ (0.83)	$ (4.85)
Weighted-average number of shares used in computing net loss per share, basic and diluted	21,755,134	2,159,065

See accompanying notes to unaudited interim condensed consolidated financial statements.

AUDENTES THERAPEUTICS, INC.
Condensed Consolidated Statements of Cash Flows
(in thousands)

	Three Months Ended March 31,	
	2017	**2016**
	Unaudited	
Cash flows from operating activities:		
Net loss	$ (18,115)	$ (10,464)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	732	78
Stock-based compensation	1,055	327
(Accretion of discount) amortization of premium on investments	(25)	133
Accretion of asset retirement obligation	8	82
Change in fair value of contingent acquisition consideration payable	90	90
Other	45	31
Changes in operating assets and liabilities:		
Additions to restricted cash	(350)	-
Prepaid expenses and other current assets	(491)	(1,402)
Other assets	(1,400)	-
Accounts payable	(1,230)	(2,323)
Accrued liabilities	(3,029)	(450)
Deferred rent	153	1,049
Net cash used in operating activities	(22,557)	(12,849)
Cash flows from investing activities:		
Purchases of property and equipment	(752)	(2,145)
Proceeds from sales and maturities of marketable securities	32,010	17,229
Purchases of marketable securities	(26,430)	(14,656)
Net cash provided by investing activities	4,828	428
Cash flows from financing activities:		
Proceeds from exercise of stock options	134	76
Net cash provided by financing activities	134	76
Net decrease in cash and cash equivalents	(17,595)	(12,345)
Cash and cash equivalents at beginning of period	36,359	72,058
Cash and cash equivalents at end of period	$ 18,764	$ 59,713
Noncash investing and financing activities:		
Change in accounts payable, accrued liabilities and facility lease obligations related to property and equipment purchases	$ 506	$ 3,415
Issuance of common stock warrant related to debt financing facility	$ 83	$ —

See accompanying notes to unaudited interim condensed consolidated financial statements.

1. Organization and Basis of Presentation

Audentes Therapeutics, Inc., or the Company, was incorporated in the State of Delaware on November 13, 2012. The Company is a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. The Company operates in one business segment, with its corporate headquarters located in San Francisco, California and its manufacturing and research operations located in South San Francisco, California.

The accompanying consolidated financial statements include the accounts of Audentes Therapeutics, Inc., and its wholly owned subsidiary, Audentes Therapeutics UK Ltd. All intercompany balances and transactions have been eliminated in consolidation.

Liquidity

In the course of its development activities, the Company has sustained operating losses and expects such losses to continue over the next several years. The Company's ultimate success largely depends on the outcome of its research and development activities. The Company has incurred net losses from operations since inception and as of March 31, 2017 had an accumulated deficit of $118.5 million. The Company intends to raise additional capital through the issuance of additional equity and potentially through strategic alliances with partner companies. In April 2017, the Company completed a follow-on equity financing (see Note 12). If additional financing is not available at adequate levels or on acceptable terms, the Company may need to reevaluate its operating plans. Management believes its currently available resources will provide sufficient funds to enable the Company to meet its operating plans for at least the next twelve months. However, if the Company's anticipated operating results are not achieved in future periods, planned expenditures may need to be reduced in order to extend the time period over which the then-available resources would be able to fund the Company's operations.

2. Summary of Significant Accounting Policies

There were no significant changes to the accounting policies during the three months ended March 31 2017, from the significant accounting policies described in Note 2 of the "Notes to Consolidated Financial Statements" in the Company's Annual Report on Form 10-K for the year ended December 31, 2016.

Basis of Preparation

The accompanying unaudited interim condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America, or U.S. GAAP, and applicable rules and regulations of the SEC regarding interim financial reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP have been condensed or omitted, and accordingly the balance sheet as of December 31, 2016 has been derived from audited financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements. These unaudited interim condensed consolidated financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for a fair presentation of the Company's financial information. The results of operations for the three months ended March 31, 2017 are not necessarily indicative of the results to be expected for the year ending December 31, 2017 or for any other interim period or for any other future year.

The accompanying unaudited interim condensed consolidated financial statements and related financial information should be read in conjunction with the audited financial statements and the related notes thereto for the year ended December 31, 2016 included in the Company's audited financial statements filed in its Annual Report on Form 10-K for the year ended December 31, 2016.

Use of Estimates

The accompanying unaudited interim condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities, as of the date of the financial statements, and the reported amounts of any expenses during the reporting period. On an ongoing basis, management evaluates its estimates, including those related to accrued liabilities, acquisition contingent consideration, fair value of assets, common stock, income taxes, and stock-based compensation. Management bases its estimates on historical experience, and on various other market-specific relevant assumptions that management believes to be reasonable, under the circumstances. Actual results may differ from those estimates.

Concentration of Manufacturing and Third-Party Services Risk

The Company is subject to certain risks with respect to sources of supply of manufactured materials and drug product for use in its preclinical and clinical studies. Due to the technical aspects of manufacturing drug product for gene therapies, there exist few alternative sources of manufacturing. The Company is reliant upon its own internal manufacturing capability and a small number of third-party manufacturers to produce drug product in sufficient quantities and quality to conduct its research and development activities.

Additionally, the Company maintains collaborative research and development arrangements with the University of Florida and the University of Pennsylvania. These institutions provide certain services to the Company including preclinical study support. Disruptions in the ability or willingness of these institutions to perform these services could cause significant delays and may cause the Company to incur additional costs for its product development activities as there are few alternative sources having the requisite expertise to perform the services provided by these institutions.

Recent Accounting Pronouncements

In January 2016, the FASB issued ASU No. 2016-01, *Financial Instruments - Overall (Subtopic 825- 10): Recognition and Measurement of Financial Assets and Financial Liabilities* (ASU 2016-01). ASU 2016-01 addresses certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. ASU 2016-01 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, which for the Company is January 1, 2018. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements and related disclosures.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842).* Under the new guidance, (with the exception of leases with terms of 12 months or less) at the commencement date, lessees will be required to recognize a lease liability and a right-of-use asset. Lessor accounting is largely unchanged, while lessees will no longer be provided with a source of off-balance sheet financing. Public business entities should apply the amendments in ASU 2016-02 for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years (January 1, 2019, for the Company). Early application is permitted. Lessees (for capital and operating leases) must apply a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The modified retrospective approach would not require any transition accounting for leases that expired before the earliest comparative period presented. The Company continues to evaluate the impact that the standard will have on its consolidated financial statements and related disclosures, however it is the Company's expectation that adoption of the pronouncement will have a material impact to its consolidated financial statements and related disclosures.

In March 2016, the FASB issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting*, which amends the accounting for employee share-based payment transactions to require recognition of the tax effects resulting from the settlement of stock-based awards as income tax expense or benefit in the income statement in the reporting period in which they occur. In addition, ASU 2016-09 requires that all tax-related cash flows resulting from share-based payments, including the excess tax benefits related to the settlement of stock-based awards, be classified as cash flows from operating activities in the statement of cash flows. It also requires that cash paid by directly withholding shares for tax withholding purposes be classified as a financing activity in the statement of cash flows. ASU 2016-09 also allows companies to make an accounting policy election to either estimate the number of awards that are expected to vest, consistent with current U.S. GAAP, or account for forfeitures when they occur. The new standard is effective for annual reporting periods beginning after December 15, 2016 with early adoption permitted. The Company adopted ASU No. 2016-09 on January 1, 2017 and will continue to account for forfeitures by estimating the number of awards that are expected to vest. Prior to the adoption of ASU 2016-09, tax attributes related to stock option windfall deductions were not recorded until they resulted in a reduction of cash tax payable. As of December 31, 2016, there were no exclusions of windfall deductions for federal or state purposes that required recording during the three months ended March 31, 2017.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payment*, which clarifies the classification within the statement of cash flows for certain transactions, including debt extinguishment costs, zero-coupon debt, contingent consideration related to business combinations, insurance proceeds, equity method distributions and beneficial interests in securitizations. It also clarifies that cash flows with aspects of multiple classes of cash flows or that cannot be separated by source or use should be classified based on the activity that is likely to be the predominant source or use of cash flows for the item. This new standard is effective for annual reporting periods beginning after December 15, 2017 and interim periods within those fiscal years. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements and related disclosures.

In November 2016, the FASB issued ASU 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash*. Under ASU 2016-18, the statement of cash flows will show the changes in the total cash, cash equivalents and amounts generally described as restricted

cash. As a result, entities will no longer have to determine how to classify transfers to or from restricted cash within the statement of cash flows. An entity will be required to reconcile the total cash, cash equivalents and amounts generally described as restricted cash on the statement of cash flows to amounts in the balance sheet and disclose the nature of any restriction on its cash, cash equivalents or amounts generally described as restricted cash. This new standard is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, and early adoption is permitted. The guidance will be applied retrospectively. If it is impractical for an entity to do so, the entity will apply the guidance prospectively as of the earliest date that is practicable. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements and related disclosures.

3. Short-Term Investments

Available-for-sale securities are as follows:

	March 31, 2017			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Commercial paper	$ 30,704	$ —	$ —	$ 30,704
Corporate securities	14,278	—	(16)	14,262
U.S. government agency securities	18,011	—	(13)	17,998
Total available-for-sale securities	$ 62,993	$ -	$ (29)	$ 62,964

	December 31, 2016			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Market Value
	(in thousands)			
Commercial paper	$ 29,428	$ —	$ —	$ 29,428
Corporate securities	19,601	—	(23)	19,578
Agency discount instruments	2,997	1	—	2,998
U.S. government agency securities	22,021	—	(6)	22,015
Total available-for-sale securities	$ 74,047	$ 1	$ (29)	$ 74,019

All available-for-sale securities as of March 31, 2017 and December 31, 2016 had maturities of less than a year.

4. Fair Value Measurements

Fair value accounting is applied for all financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis (at least annually). Financial instruments include cash and cash equivalents, restricted cash, accounts payable and accrued liabilities that approximate fair value due to their relatively short maturities.

Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

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

Level 2 – Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

Level 3 – Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

Assets Measured at Fair Value

Financial assets subject to fair value measurements on a recurring basis and the level of inputs used in such measurements are as follows:

	March 31, 2017			
	Fair Value Measurements Using			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 14,259	$ 14,259	$ —	$ —
Commercial paper	30,704	—	30,704	—
Corporate securities	14,262	—	14,262	—
U.S. government agency securities	17,998	—	17,998	—
Total financial assets	$ 77,223	$ 14,259	$ 62,964	$ —

	December 31, 2016			
	Fair Value Measurements Using			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 26,439	$ 26,439	$ —	$ —
Commercial paper	29,428	—	29,428	—
Corporate securities	19,578	—	19,578	—
Agency discount instruments	2,998	—	2,998	—
U.S. government agency securities	22,015	—	22,015	—
Total financial assets	$ 100,458	$ 26,439	$ 74,019	$ —

The total financial assets listed above do not included cash held in the Company's primary operating bank accounts of $4.5 million and $4.4 million as of March 31, 2017 and December 31, 2016, respectively.

Liabilities Measured at Fair Value

The Company's financial liabilities are valued based upon observable inputs when available or upon estimates made by management. The following tables set forth the fair value of the Company's financial liabilities as of March 31, 2017 and December 31, 2016:

	March 31, 2017			
	Fair Value Measurements Using			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Contingent acquisition consideration payable	$ 4,470	$ —	$ —	$ 4,470
Asset retirement obligation	717	—	—	717
Total financial liabilities	$ 5,187	$ —	$ —	$ 5,187

	December 31, 2016			
	Fair Value Measurements Using			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Contingent acquisition consideration payable	$ 4,380	$ —	$ —	$ 4,380
Asset retirement obligation	709	—	—	709
Total financial liabilities	$ 5,089	$ —	$ —	$ 5,089

The Company's contingent acquisition consideration payable, resulting from the acquisition of Cardiogen Sciences, Inc. in August 2015, is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probability of occurrence, the estimated timing of when the milestone may be attained and assumed discount period and discount rate. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions will be recorded in research and development expense in the consolidated statement of operations and comprehensive loss. The probability-

based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probability of occurrence.

The following is a summary of the contingent acquisition consideration payable, recorded as a non-current liability in the accompanying consolidated balance sheets:

	Amount
	(in thousands)
Balance, December 31, 2016	$ 4,380
Change in fair value of contingent acquisition consideration payable	90
Balance, March 31, 2017	$ 4,470

Under the terms of its sublease for manufacturing facilities, the Company assumed an asset restoration obligation from the previous tenant. The liability is being accreted, or increased, and recorded as rent expense throughout the remainder of the lease term until the full estimated obligation to restore the building to its original condition is recognized in the condensed consolidated balance sheet. The asset retirement obligation is included in facilities lease obligations in the accompanying consolidated balance sheets.

	Amount
	(in thousands)
Balance, December 31, 2016	$ 709
Asset retirement obligation accretion expense	8
Balance, March 31, 2017	$ 717

5. Balance Sheet Components

Property and Equipment, Net

Property and equipment, net, consist of the following:

	March 31, 2017	December 31, 2016
	(in thousands)	
Furniture and office equipment	$ 624	$ 624
Computer equipment	77	77
Software	229	175
Leasehold improvements	11,616	11,568
Laboratory equipment	3,586	2,974
Manufacturing equipment	5,354	5,200
Construction in progress and deposits on equipment	442	25
Total property and equipment	21,928	20,643
Less accumulated depreciation and amortization	(2,439)	(1,707)
Property and equipment, net	$ 19,489	$ 18,936

Property and equipment depreciation expense for the three months ended March 31, 2017 and 2016 was $0.7 million and $0.1 million, respectively.

Accrued Liabilities

Accrued liabilities consist of the following as of March 31, 2017 and December 31, 2016:

	March 31, 2017	December 31, 2016
	(in thousands)	
Accrued payroll and related expenses	$ 1,762	$ 3,164
Accrued research and development expenses	4,450	6,169
Accrued professional services	549	386
Construction in progress	220	-
Other	82	152
Total accrued liabilities	$ 7,063	$ 9,871

Facility Lease Obligations

Long-term deferred rent and asset retirement obligations consist of the following as of March 31, 2017 and December 31, 2016:

	March 31, 2017	December 31, 2016
	(in thousands)	
Deferred rent	$ 2,061	$ 1,777
Asset retirement obligation	717	709
	$ 2,778	$ 2,486

Hercules Loan Agreement

On March 7, 2017, the Company entered into a Loan and Security Agreement for a term loan in an aggregate principal amount of $20 million. Through September 15, 2017, the Company may draw up to an aggregate of $10 million, but cannot draw more than an aggregate of $5 million from June 15, 2017 to September 15, 2017. In addition, beginning on the date the Company initiates enrollment in Phase 1/2 clinical trials for AT132 and AT342 under U.S. Investigational New Drug applications and continuing through December 15, 2017, the Company may draw up to an additional $10 million.

The term loan made pursuant to the Loan Agreement will bear interest at a rate equal to the greater of either (i) 7.95% plus the prime rate as reported in the Wall Street Journal minus 3.75%, and (ii) 7.95%. The term loan matures on December 1, 2020. The Company shall begin to repay the aggregate principal amount that is outstanding in equal monthly installments of principal and interest beginning on July 1, 2018, or if certain clinical development or financing milestones are achieved, beginning on certain dates in 2019, until the term loan maturity or such earlier date when secured obligations are repaid. The Company may voluntarily prepay the term loan, subject to a 2.0% premium for 12 months and a 1.0% premium after 12 months but prior to 24 months. Certain mandatory prepayments are required upon a Change in Control (as defined in the Loan Agreement). Upon the repayment of the term loan, or if no term loan is drawn, on December 16, 2017, the Company shall pay lender the greater of $200,000 or 5.40% of the aggregate amount of the term loans drawn under the agreement. As of March 31, 2017, the Company has not drawn on the Loan Agreement.

The Loan Agreement requires the Company to maintain certain covenants, including those that require it to provide the Lender with certain financial and other information and pay taxes, and restrict its ability to incur other indebtedness, dispose of collateral, make certain investments and distribution, declare dividends, transfer certain assets, merge with other entities, change its name or jurisdiction, maintain certain deposit accounts or take certain actions with respect to subsidiaries. The Company's obligations under the Loan Agreement are secured by substantially all of its assets, which do not include, among certain other items, its intellectual property.

In connection with the entry into the Loan Agreement, the Company issued a warrant to Hercules Technology, exercisable for 9,194 shares of the Company's common stock at an exercise price of $15.13. The warrant is immediately exercisable through the earlier of (i) March 7, 2022 and (ii) the consummation of certain acquisition transactions involving it as set forth in the warrant. The number of shares for which the warrant is exercisable and the associated exercise price are subject to certain proportional adjustments as set forth in the warrant.

Related to the Loan Agreement, the Company recorded deferred financing costs of $0.3 million, which are being amortized through December 16, 2017. During the three months ended March 31, 2017, the Company recorded this amortization to interest expense aggregating $45,000.

6. License and Collaboration Agreements

During the first quarter of 2017, the Company entered into a services and collaboration agreement for the treatment of Crigler-Najjar Syndrome related to the Company's AT342 development program. The agreement has a term of ten years and provides that the Company will pay a specified minimum fee to the service provider for each year that the program is under development that ranges from $0.1 million to $0.4 million per year in addition to payments for services provided. Following commercialization of AT342, the Company is obligated to pay the service provider a 1% royalty based on net sales, as defined under the agreement, subject to a contractual minimum of $0.2 million during the first three years of commercialization.

7. Stock Compensation

Stock-based compensation expense by category was as follows for the three months ended March 31, 2017 and 2016:

	Three Months Ended March 31,	
	2017	2016
	(in thousands)	
Research and development	$ 539	$ 95
General and administrative	516	232
Total stock-based compensation expense	$ 1,055	$ 327
Employees	$ 1,024	$ 307
Non-employees	31	20
Total stock-based compensation expense	$ 1,055	$ 327

Equity Incentive Plans

Under the Company's 2012 Equity Incentive Plan, or the 2012 Plan, a total of 3,107,517 shares were reserved for issuance. In July 2016, the Company ceased granting awards under the 2012 Plan and rolled the remaining 705,862 shares available for grant into the 2016 Equity Incentive Plan, or 2016 Plan, which was adopted on July 18, 2016. Under the terms of the 2012 Plan, options were granted at an exercise price not less than fair market value. For employees holding more than 10% of the voting rights of all classes of stock, the exercise prices for incentive and non-statutory stock options were not to be less than 110% of fair market value, as determined by the board of directors. The terms of options granted under the 2012 Plan do not exceed ten years. As options from the 2012 Plan are forfeited or canceled, they are rolled into the 2016 Plan.

A total of 1,500,000 shares were reserved for issuance under the 2016 Plan in addition to the 705,862 shares rolled into the 2016 Plan from the 2012 Plan. At March 31, 2017, 1,868,246 shares were available for future grant. The number of shares reserved for issuance under the 2016 Plan will increase automatically on January 1 of each calendar year continuing through the tenth calendar year during the term of the 2016 Plan by a number of shares equal to 5% of the total outstanding shares of the Company's common stock as of the immediately preceding December 31. However, the board of directors at its discretion may reduce the amount of increase in any particular year. On January 1, 2017, 1,086,562 additional shares were added to the 2016 Plan reserve for issuance per this provision. Under the terms of the 2016 Plan, in general, options will vest over a four-year period. However, options may vest based on time or achievement of performance conditions. The term of options granted under the 2016 Plan is limited to ten years.

The following table summarizes option activity for the three months ended March 31, 2017:

	Shares Available for Grant	Number of Options Outstanding	Weighted-Average Exercise Price Per Option	Weighted-Average Remaining Contract Term (Years)	Aggregate Intrinsic Value
					(in thousands)
Balance, December 31, 2016	1,891,092	2,534,622	$ 5.60	8.50	$ 32,126
Increase to authorized shares	1,086,562	—	—		
Options granted	(1,150,750)	1,150,750	$ 15.79		
Options exercised	—	(36,725)	$ 3.65		
Options forfeited	41,342	(41,342)	$ 13.24		
Balance, March 31 2017	1,868,246	3,607,305	$ 8.78	8.66	$ 29,872
Exercisable, March 31, 2017		1,099,582	$ 3.62	7.69	$ 14,760
Vested and expected to vest, March 31, 2017		3,340,349	$ 8.47	8.61	$ 28,684

The aggregate intrinsic values of options outstanding, exercisable, vested and expected to vest were calculated as the difference between the exercise price of the options and the fair value of the Company's common stock as of March 31, 2017. During the three months ended March 31, 2017, options to purchase 36,725 shares of common stock with an intrinsic value of approximately $0.5 million were exercised, generating approximately $0.1 million of cash received.

The weighted average grant date fair value of employee options granted during the three months ended March 31, 2017 and 2016 was $10.03 and $4.12 per share, respectively. As of March 31, 2017, the total unrecognized compensation expense related to unvested employee options, net of estimated forfeitures, was approximately $14.5 million, which the Company expects to recognize over an estimated weighted average period of 3.01 years. To the extent the actual forfeiture rate is different from what the Company has estimated, stock-based compensation related to these awards will be different from its expectations.

The fair value of stock options granted to employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Three Months Ended March 31,	
	2017	2016
Expected term (in years)	5.8-6.1	6.1
Expected volatility	77-78%	68%
Risk-free interest rate	2.1-2.2%	1.5%
Expected dividend yield	0%	0%

There were no non-employee options granted during the three months ended March 31, 2017. The weighted-average grant date fair value of non-employee options granted during the three months ended March 31, 2016 was $5.67. Options and awards to non-employees are recorded at fair value and remeasured at the end of each period. As of March 31, 2017, the total unrecognized compensation expense related to unvested non-employee options was approximately $0.2 million, which the Company expects to recognize over an estimated weighted average period of 1.56 years. To the extent the actual forfeiture rate is different from what the Company has estimated, stock-based compensation related to these awards will be different from its expectations.

The fair value of stock options for non-employees was estimated using a Black-Scholes option pricing model with the following assumptions:

	Three Months Ended March 31, 2016
Expected term (in years)	7.7-10.0
Expected volatility	69-71%
Risk-free interest rate	1.7-2.1%
Expected dividend yield	0%

2016 Employee Stock Purchase Plan

On July 19, 2016, the 2016 Employee Stock Purchase Plan, or the 2016 ESPP was adopted. The 2016 ESPP was adopted in order to enable eligible employees to purchase shares of the Company's common stock at a discount. Purchases will be accomplished through participation in discrete offering periods. The Company initially reserved 210,000 shares of common stock for issuance under the 2016 ESPP. The number of shares reserved for issuance under the 2016 ESPP will increase automatically on January 1 of each calendar year beginning after the first offering date and continuing through the first ten calendar years by the number of shares equal to 1% of the total outstanding shares of the Company's common stock as of the immediately preceding December 31.

The ESPP will not become effective until such time as the Compensation Committee determines in the future, and as of March 31, 2017, the initial offering periods had not commenced.

8. Income Taxes

The Company did not record a federal or state income tax provision or benefit for the three months ended March 31, 2017 and 2016 as it has incurred net losses since inception. In addition, the net deferred tax assets generated from the net operating losses have been fully reserved as the Company believes it is not more likely than not that the benefit will be realized.

9. Commitments and Contingencies

In January 2017, the Company entered into a lease agreement for approximately 7,555 square feet of research and development offices in South San Francisco, California with total minimum lease payments of $0.4 million over an approximately three-year term.

In July 2015, the Company entered into a sub-lease agreement for approximately 22,000 square feet of manufacturing space in South San Francisco, California for an initial term that expires in May 2017 with total minimum lease payments due of $0.9 million. In November 2015, the Company purchased an option that was subsequently exercised in May 2016 to enter into a ten-year lease for the existing 22,000 square feet plus approximately 17,000 additional square feet of manufacturing space, which will become effective in June 2017. The Company executed the lease extension in January 2017.

10. Net Loss per Share

Basic net loss per share is calculated by dividing net loss by the weighted-average number of common shares outstanding during the period and excludes any potential dilutive effects of common stock equivalents. Diluted net loss per share is computed giving effect to all potential dilutive common shares, including common stock issuable upon exercise of stock options, convertible preferred stock, and unvested restricted common stock. As the Company had net losses for the three months ended March 31, 2017 and 2016, all potential common shares were determined to be anti-dilutive and were therefore excluded from the calculation of diluted net loss per share.

The following outstanding shares of common stock equivalents were excluded from the computation of diluted net loss per share of common stock for the periods presented because including them would have been anti-dilutive:

	Three Months Ended March 31,	
	2017	2016
Convertible preferred stock (on an as-if-converted basis)	—	13,820,301
Stock options to purchase common stock	3,607,305	2,279,086
Common stock warrants	9,194	-
	3,616,499	16,099,387

11. Related Party Transactions

There were no related party transactions in the three months ended March 31, 2017. Aggregate payments in connection with related party transactions totaled approximately $8,000 during the three months ended March 31, 2016 and consisted of cost reimbursements to certain investors.

12. Subsequent Events

Follow-On Offering

On April 24, 2017, the Company completed an underwritten public offering of 5,200,000 shares of common stock. As part of the offering, on April 27, 2017 the Company issued an additional 755,151 shares of common stock representing the underwriters' exercise of a majority of their option to purchase additional shares. All shares were offered by the Company at a price to the public of $14.50 per share. The aggregate net proceeds received by the Company were $80.7 million, net of underwriting discounts, commissions and estimated offering expenses.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

NOTE ABOUT FORWARD-LOOKING STATEMENTS

This quarterly report contains forward-looking statements. All statements other than statements of historical fact are "forward-looking statements" for purposes of this Quarterly Report on Form 10-Q. These forward-looking statements may include, but are not limited to, statements regarding our future results of operations and financial position, business strategy, market size, potential growth opportunities, timing and results of preclinical and clinical development activities, and potential regulatory approval and commercialization of product candidates. In some cases, forward looking-statements may be identified by terminology such as "believe," "may," "will," "should", "predict", "goal", "strategy", "potentially," "estimate," "continue," "anticipate," "intend," "could," "would," "project," "plan," "expect," "seek" and similar expressions and variations thereof. These words are intended to identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this report may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this report to conform these statements to actual results or to changes in our expectations, except as required by law.

As used in this Quarterly Report on Form 10-Q, the terms "Audentes," "the Company," "we," "us," and "our" refer to Audentes Therapeutics, Inc. and, where appropriate, its consolidated subsidiary, unless the context indicates otherwise.

You should read the following discussion and analysis of our financial condition and results of operations together with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this report and with our audited consolidated financial statements and related notes thereto for the year ended December 31, 2016, included in our Annual Report on Form 10-K.

Overview

We are a biotechnology company focused on developing and commercializing gene therapy products for patients suffering from serious, life-threatening rare diseases caused by single gene defects. We believe that gene therapy has powerful potential to treat these diseases through delivery of a functional copy of the affected gene to cells, resulting in production of the normal protein. We have built a compelling portfolio of product candidates, including AT132 for the treatment of X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. The Investigational New Drug applications, or INDs, for both AT132 and AT342 are active, and our collaborating institution, the University of Florida, has an active IND to conduct a proof-of-concept study of AT982 delivered via intra-muscular injection in adults with Pompe disease. We are conducting IND-enabling preclinical studies for the systemic administration of AT982 for the treatment of Pompe disease and exploratory preclinical studies evaluating intrathecal delivery of AT982. We plan to file an IND for the systemic administration of AT982 in the first half of 2018. We are also conducting IND-enabling studies of AT307 and plan to file an IND in the second half of 2017. We expect to have preliminary clinical data from the AT132, AT342 and AT982 programs in the second half of 2017. We maintain full global rights to all our product candidates.

We have developed a proprietary in-house cGMP manufacturing capability that we believe provides us with a core strategic advantage, enabling superior control over development timelines, costs and intellectual property. Our manufacturing facility is located in South San Francisco in a building that we have improved to support our research, process development and manufacturing capabilities in accordance with current Good Manufacturing Practices, or cGMP, requirements. We believe we have established a comprehensive platform for production of our adeno-associated virus vector, or AAV, product candidates and plan continued investment to further optimize our manufacturing capabilities to cost-effectively produce high-quality AAV vectors at both clinical and commercial scale. We initiated cGMP manufacturing of our products in our facility in the second half of 2016.

We have built our portfolio of product candidates in part by engaging in strategic transactions with third parties. In July 2013, we entered into a license agreement with REGENXBIO Inc., or REGENXBIO, pursuant to which we obtained intellectual property rights related to AT132 and AT982. In January 2014, we entered into a collaborative development agreement with Genethon, pursuant to which we acquired intellectual property rights related to AT132 in exchange for granting Genethon the exclusive right to manufacture materials for preclinical and early clinical development, subject to Genethon's ability to supply required quantities in accordance with applicable timelines, and the funding for certain research and development activities related to AT132. In July 2015, we entered into a license with the University of Florida Research Foundation, or UFRF, pursuant to which we obtained intellectual property rights related to AT982. In August 2015, in connection with our acquisition of Cardiogen Sciences, Inc., or Cardiogen, we acquired a license agreement with Fondazione Salvatore Maugeri, or FSM, pursuant to which we obtained a license to FSM's intellectual property rights related to AT307 and certain other products that we may develop related to the treatment of several additional inherited arrhythmias. In November 2015, we entered into two additional license agreements with REGENXBIO, pursuant to which we obtained intellectual property rights related to AT307 and AT342. In May 2016, we entered into a license and collaboration agreement with The Trustees of the University of Pennsylvania, or the University of Pennsylvania, pursuant to which we obtained a license to develop and commercialize a gene therapy product for Crigler-Najjar. Upon execution of the license and collaboration agreement with the University of Pennsylvania, we met the conditions of a contractual milestone under our Crigler-Najjar license agreement with REGENXBIO, and made a required payment of $0.4 million to REGENXBIO. We paid the University of Pennsylvania an upfront fee of $0.5 million, $6.0 million for certain preclinical development activities and a $0.7 million milestone payment as a result of our active IND for AT342. We may be required to make additional milestone payments and pay royalties and other amounts to third parties pursuant to our license and collaboration agreements as we further develop and commercialize our product candidates.

Since our inception, we have devoted substantially all of our resources to: identifying, acquiring, and developing our product candidate portfolio; organizing and staffing our company; raising capital; developing our manufacturing capabilities; and providing general and administrative support for these operations. We have never generated revenue and have incurred significant net losses since inception. We do not expect to receive any revenue from any product candidates that we develop until we obtain regulatory approval and commercialize our product candidates or enter into collaborative agreements with third parties. Our net losses were $59.7 million and $26.5 million for the years ended December 31, 2016 and 2015, respectively, and $18.1 million and $10.5 million for the three months ended March 31, 2017 and 2016, respectively. As of March 31, 2017, we had an accumulated deficit of $118.5 million. We expect to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially as we:

- invest significantly to further develop and seek regulatory approval for our existing product candidates;
- further expand our pipeline of potential product candidates;
- continue to develop our proprietary in-house manufacturing facility and capabilities;
- hire additional clinical, scientific, management and administrative personnel;
- seek regulatory and marketing approvals for any product candidates that we may develop;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any drugs for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other assets and technologies; and
- add additional operational, financial and management information systems and processes to support our ongoing development efforts, any future manufacturing or commercialization efforts and our administrative and compliance obligations as a public company.

We have funded our operations to date primarily from the issuance and sale of our convertible preferred stock and through the issuance and sale of our common stock pursuant to our initial public offering, or IPO, in July 2016 and our recently completed follow-on offering. As of March 31, 2017, we had cash, cash equivalents and short-term investments of $81.7 million.

On April 24, 2017, we completed an underwritten public offering of 5,200,000 shares of common stock. As part of the underwritten public offering, on April 27, 2017 we issued an additional 755,151 shares of common stock representing the underwriters' exercise of a majority of their option to purchase additional shares. All shares were offered by us at a price to the public of $14.50 per share. The aggregate net proceeds received by us from the offering were $80.7 million, net of underwriting discounts, commissions and estimated offering expenses.

On March 7, 2017, we entered into a Loan and Security Agreement with Hercules Capital, Inc., or Hercules, pursuant to which Hercules has made available to us a term loan in an aggregate principal amount of $20 million. To date, we have not drawn any

amounts under the loan agreement. In connection with entry into the loan agreement, we issued a warrant to Hercules exercisable for 9,194 shares of our common stock.

To fund our current operating plans, we will need additional capital, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our preclinical and clinical development efforts. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Financial Operations Overview

Research and Development Expenses

Research and development program expenses consist primarily of external costs incurred for the development of our product candidates, which include:

- expenses incurred under agreements with consultants, third-party contract organizations and investigative clinical trial sites that conduct research and development activities on our behalf;
- laboratory and vendor expenses related to the execution of preclinical studies and clinical trials;
- costs related to production of preclinical and clinical materials, including fees paid to contract manufacturers and manufacturing input costs for use in internal manufacturing processes; and
- costs related to in-licensing of rights to develop and commercialize our product candidate portfolio.

Personnel, non-program and unallocated program expenses include costs associated with activities performed by our internal research and development organization and generally benefit multiple programs. These costs are not separately allocated by product candidate and consist primarily of:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- facilities and other expenses, which include expenses for rent and maintenance of facilities, depreciation and amortization expense;
- lab supplies and equipment used for internal research and development activities;
- internal manufacturing expenses; and
- the change in fair value of contingent acquisition consideration payable.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks performed by others using information and data provided to us by our vendors, collaborators and third-party service providers. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and as services are performed.

The largest component of our operating expenses has historically been our investment in research and development activities. However, we do not allocate personnel and other costs, such as salaries, benefits, stock-based compensation expense and indirect internal program costs to product candidates on a program-specific basis.

The following table summarizes our research and development expenses incurred during the respective periods:

	Three Months Ended March 31,	
	2017	2016
AT132 direct program costs	$ 3,924	$ 1,551
AT342 direct program costs	1,879	548
AT982 direct program costs	(106)	581
AT307 direct program costs	29	177
Personnel, non-program, and unallocated program costs	8,861	5,049
Total research and development expenses	$ 14,587	$ 7,906

We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in manufacturing, as our

programs advance into later stages of development and we begin to conduct clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, facilities costs, including rent and maintenance of facilities, depreciation and amortization expense and other expenses for outside professional services, including legal, human resources, audit and accounting services. Personnel costs consist of salaries, bonuses, payroll taxes, benefits and stock-based compensation expense. We expect our general and administrative expenses to increase for the foreseeable future due to anticipated increases in headcount to advance our product candidates and as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, The NASDAQ Global Market, additional insurance expenses, investor relations activities and other administration and professional services.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents and investments.

Other Income, net

Other income, net consists of foreign currency transaction gains and losses incurred during the period.

Critical Accounting Polices and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies related to business combinations, contingent consideration payable, accrued research and development costs, and stock-based compensation expense are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

There have been no significant changes in our critical accounting policies and estimates as compared to the critical accounting policies and estimates disclosed in Management's Discussion and Analysis of Financial Condition and Operations included in our Annual Report on Form 10-K for the year ended December 31, 2016.

Recent Accounting Pronouncements

Except as described in Note 2 to the Unaudited Interim Condensed Consolidated Financial Statements under the heading "Recent Accounting Pronouncements," there have been no new accounting pronouncements or changes to accounting pronouncements during the three months ended March 31, 2017, as compared to the recent accounting pronouncements described in our Annual Report on Form 10-K for the year ended December 31, 2016, that are significant to us.

Results of Operations

Comparison of the three months ended March 31, 2017 and 2016

	Three Months Ended March 31,		
	2017	2016	Change
	(in thousands)		
Operating expenses			
Research and development	$ 14,587	$ 7,906	$ 6,681
General and administrative	3,658	2,632	1,026
Total operating expenses	18,245	10,538	7,707
Loss from operations	(18,245)	(10,538)	(7,707)
Interest income, net	147	97	50
Other expense, net	(17)	(23)	6
Net loss	$ (18,115)	$ (10,464)	$ (7,651)

Research and Development

Research and development expenses increased by $6.7 million, or 85%, to $14.6 million for the three months ended March 31, 2017. The increase was primarily due to a $2.4 million increase in expenses related to our AT132 program and a $1.3 million increase in expenses related to our AT342 program, as we conducted additional preclinical studies, increased manufacturing of study materials and additional consulting and initiation costs in preparation for clinical trials, a $2.4 million increase in personnel costs and a $0.4 million increase for facility costs primarily due to increased headcount and investment in manufacturing, a $0.3 million increase in other expenses related to expanded research and development, and a $0.4 million increase for stock-based compensation expense, while our AT982 program expenses decreased by $0.7 million as we renegotiated certain service agreements related to the program. We anticipate research and development expenses to continue to increase as we expand operations for all our programs.

General and Administrative

General and administrative expenses increased by $1.0 million, or 39%, to $3.7 million for the three months ended March 31, 2017. The increase was primarily due to a $0.3 million increase in personnel costs and a $0.3 million increase in stock-based compensation expense due to increased headcount, a $0.1 million increase in facilities-related costs, and a $0.2 million increase in insurance costs. Many of these increases are a result of becoming a public company and we expect to continue to incur additional expenses related to our operations as a public company.

Interest Income, net

Interest income, net increased by $50,000, or 52%, to $147,000 for the three months ended March 31, 2017, as we invested the funds received from our IPO into short duration fixed-income securities and recognized amortization of deferred financing costs related to our debt financing facility as interest expense.

Liquidity, Capital Resources and Plan of Operations

Since our inception in 2012 through March 31, 2017, our operations have been financed solely by net proceeds of $135.8 million from the sale of shares of our convertible preferred stock and $75.2 million from the sale of common stock from our IPO. As of March 31, 2017, we had $81.7 million in cash, cash equivalents, and short-term investments and an accumulated deficit of $118.5 million.

On April 24, 2017, we completed an underwritten public offering of 5,200,000 shares of common stock. As part of the underwritten public offering, on April 27, 2017 we issued an additional 755,151 shares of common stock representing the underwriters' exercise of a majority of their option to purchase additional shares. All shares were offered by us at a price to the public of $14.50 per share. The aggregate net proceeds received by us from the offering were $80.7 million, net of underwriting discounts, commissions and estimated offering expenses.

On March 7, 2017, we entered into a Loan and Security Agreement with Hercules, pursuant to which Hercules has made available to us a term loan in an aggregate principal amount of $20 million. To date, we have not drawn any amounts under the loan agreement. In connection with entry into the loan agreement, we issued a warrant to Hercules exercisable for 9,194 shares of our common stock.

To fund our current operating plans, we will need additional capital, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our preclinical and clinical development efforts. We cannot assure you that we will ever be profitable or generate positive cash flow from operating

activities.Our primary use of cash is to fund operating expenses, which consist of research and development expenditures and general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe that our existing cash, cash equivalents and investments will be sufficient to meet our anticipated cash and capital expenditure requirements for at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. We will continue to require additional financing to advance our current product candidates through clinical development, to develop, acquire or in-license other potential product candidates and to fund operations for the foreseeable future. We will continue to seek funds through equity or debt financings, collaborative or other arrangements with corporate sources, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms, or at all. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies.

Further, our operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

If we need to raise additional capital to fund our operations, funding may not be available to us on acceptable terms, or at all. If we are unable to obtain adequate financing when needed, we may have to delay, reduce the scope of or suspend one or more of our clinical trials, research and development programs or commercialization efforts. We may seek to raise any necessary additional capital through a combination of public or private equity offerings, debt financings, and collaborations or licensing arrangements. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If additional funding is required, there can be no assurance that additional funds will be available to us on acceptable terms on a timely basis, if at all. If we are unable to raise capital, we will need to curtail planned activities to reduce costs. Doing so will likely have an unfavorable effect on our ability to execute our business plans.

The following table summarizes our cash flows for the periods indicated:

	Three Months Ended March 31,	
	2017	2016
	(in thousands)	
Cash used in operating activities	$ (22,557)	$ (12,849)
Cash provided by investing activities	4,828	428
Cash provided by financing activities	134	76
Net decrease in cash and cash equivalents	$ (17,595)	$ (12,345)

Cash Flows from Operating Activities

Cash used in operating activities for the three months ended March 31, 2017 was $22.6 million. Our net loss was $18.1 million, which was partially offset by noncash charges of $1.9 million, consisting primarily of $1.1 million of stock-based compensation expense, $0.7 million of depreciation and amortization expense, and a $0.1 million change in the fair value of the contingent acquisition consideration liability. The change in our net operating assets was primarily the result of an increase in our prepaid expenses, primarily for research and development contracts, and other current assets by $0.5 million, a $1.4 million increase in long-term deposits and an increase in our accounts payable and accrued liabilities by a net $4.3 million. In addition, we transferred $0.4 million to restricted cash.

Cash used in operating activities for the three months ended March 31, 2016 was $12.8 million. Our net loss was $10.5 million, which was partially offset by noncash charges of $0.7 million consisting primarily of $0.3 million of stock-based compensation expense, $0.1 million from amortization of discounts on investments, a $0.1 million change in the fair value of the contingent acquisition consideration liability, and $0.1 million of depreciation and amortization expense. The change in our net operating assets and liabilities for the period was due primarily to an increase in prepaid expenses of $1.4 million and an increase in accounts payable and accrued liabilities of $2.8 million as our operations expanded.

Cash Flows from Investing Activities

Cash provided by investing activities was $4.8 million for the three months ended March 31, 2017, primarily due to the sale or maturity of marketable securities of $32.0 million, partially offset by purchases of marketable securities of $26.4 million and purchases of property and equipment of $0.8 million.

Cash provided by investing activities was $0.4 million for the three months ended March 31, 2016, primarily related to the maturity or sale of investments of $17.2 million, partially offset by purchases of marketable securities for $14.7 million and purchases of property and equipment for $2.1 million.

Cash Flows from Financing Activities

Cash provided by financing activities for the three months ended March 31, 2017 and 2016 were related to proceeds from the exercise of stock options of $0.1 million and $0.1 million, respectively.

Off-Balance Sheet Arrangements

At March 31, 2017, we were not a party to any off-balance sheet arrangements that have, or are reasonably likely to have, a current or future effect on our financial condition, changes in financial condition, revenue or expenses, results of operations, liquidity, capital expenditures or capital resources.

Contractual Obligations and Other Commitments

Debt Obligations

On March 7, 2017, we entered into a Loan and Security Agreement with Hercules, pursuant to which Hercules has made available to us a term loan in an aggregate principal amount of $20 million, which may be drawn as certain times as specified in the loan agreement. The term loan will bear interest at a rate equal to the greater of either (i) 7.95% plus the prime rate as reported in the Wall Street Journal minus 3.75%, and (ii) 7.95%. The term loan matures on December 1, 2020. We will begin to repay the aggregate principal amount that is outstanding, if any, in equal monthly installments of principal and interest beginning on July 1, 2018, or if certain clinical development or financing milestones are achieved, beginning on certain dates in 2019, until the term loan maturity or such earlier date when secured obligations are repaid. We may voluntarily prepay the term loan, subject to a 2.0% premium for 12 months and a 1.0% premium after 12 months but prior to 24 months. Certain mandatory prepayments are required upon a Change in Control (as defined in the Loan Agreement). Upon the repayment of the term loan, or if no term loan is drawn, on December 16, 2017, we shall pay lender the greater of $200,000 or 5.40% of the aggregate amount of the term loans drawn under the agreement. As of March 31, 2017, we have not drawn on the loan agreement.

Lease Agreements

In January 2017, we entered into a lease agreement for approximately 7,555 square feet of research and development offices in South San Francisco, California with total minimum lease payments of $0.4 million over an approximately three-year term.

In July 2015, we entered into a sub-lease agreement for approximately 22,000 square feet of manufacturing space in South San Francisco, California for an initial term that expires in May 2017 with total minimum lease payments due of $0.9 million. In November 2015, we purchased an option that was subsequently exercised in May 2016 to enter into a ten-year lease for the existing 22,000 square feet plus approximately 17,000 additional square feet of manufacturing space, which will become effective in June 2017. We executed the lease extension in January 2017.

License and Collaboration Agreements

During the three months ended March 31, 2017, we entered into a services and collaboration agreement for the treatment of Crigler-Najjar Syndrome related to our AT342 development program. The agreement has a term of ten years and provides that we will pay a specified minimum fee to the service provider for each year that the program is under development that ranges from $0.1 million to $0.4 million per year in addition to payments for services provided. Following commercialization of AT342, we are obligated to pay the service provider a 1% royalty based on net sales, as defined under the agreement, subject to a contractual minimum of $0.2 million during the first three years of commercialization.

Other Contracts

We also enter into contracts in the normal course of business with various third parties for services related to preclinical research studies, clinical trials, testing, manufacturing and other services. These contracts generally provide for termination upon notice, and therefore we believe that our non-cancelable obligations under these agreements are not material.

ITEM 3. QUALITATIVE AND QUANTITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities. We had cash, cash equivalents and short-term investments of $81.7 million and $104.9 million as of March 31, 2017 and December 31, 2016, respectively, which consisted of bank deposits, money market funds and marketable securities. Such interest-earning instruments carry a degree of interest rate risk; however, historical fluctuations in interest income have not been significant for us. We had no debt outstanding as of March 31, 2017 or December 31, 2016.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, have evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of March 31, 2017.

Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended March 31, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II

ITEM 1. LEGAL PROCEEDINGS

We are not currently a party to any pending legal proceedings. From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputational harm and other factors.

ITEM 1A. RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this report, including our unaudited interim condensed consolidated financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to Product Development and Regulatory Approval

We are very early in our development efforts. All of our product candidates are still in preclinical development. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and all of our lead product candidates are still in preclinical development. We, or our collaborators, have only recently completed initial preclinical studies for our AT132, AT342, AT982 and AT307 programs. We have invested substantially all of our efforts and financial resources in the identification and preclinical development of our current product candidates, AT132 for X-Linked Myotubular Myopathy, or XLMTM, AT342 for the treatment of Crigler-Najjar Syndrome, or Crigler-Najjar, AT982 for the treatment of Pompe disease and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia, or CASQ2-CPVT. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require preclinical and clinical development, regulatory approval in multiple jurisdictions, obtaining preclinical, clinical and commercial manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. Our product candidates must be authorized for marketing by the U.S. Food and Drug Administration, or the FDA, or certain other foreign regulatory agencies, such as the European Medicines Agency, or EMA, before we may commercialize our product candidates.

The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies, including Good Laboratory Practices, or GLP toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, and successful enrollment and completion of clinical trials under current Good Clinical Practices, or GCPs;
- effective Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- positive results from our clinical programs that are supportive of safety and effectiveness and provide an acceptable risk-benefit profile of our product candidates in the intended patient populations;
- receipt of regulatory approvals from applicable regulatory authorities;
- successful development of our internal manufacturing processes, including process development and scale-up activities to supply drug product for pre-clinical studies, clinical trials and commercial sale
- where applicable, establishment of arrangements with third-party contract manufacturing organizations, or CMOs, for clinical and large-scale commercial supply;
- establishment and maintenance of patent and trade secret protection and regulatory exclusivity for our product candidates;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;

- effectively competing against other therapies available in the market;
- establishment and maintenance of adequate reimbursement from third-party payors for our products;
- enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We have not tested any of our product candidates in clinical trials. Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.

Though viral vectors similar to ours have been evaluated by others in clinical trials, our product candidates have never been evaluated in human clinical trials, and we may experience unexpected or adverse results in the future. We will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Other companies conducting gene therapy clinical trials, which we believe serve as proof-of-concept for our product candidates, utilize adeno-associated viral vectors, or AAV vectors, similar to ours. For example, in April 2017, a company developing gene therapy products publicly reported positive top-line results from a Phase 1/2 trial of a product candidate intended to treat spinal muscular atrophy, a rare neuromuscular disease. However, this study and others like it should not be relied upon as evidence that our planned clinical trials will succeed. Trial designs and results from previous trials are not necessarily predictive of our future clinical trial designs or results, and initial positive results we may observe may not be confirmed upon full analysis of the complete trial data. In addition, the positive results we have observed for our product candidates in preclinical animal models may not be predictive of results from our future clinical trials in humans. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials.

Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. For example, we may want to use the RECENSUS retrospective medical chart review as a historical control for our planned Phase 1/2 ASPIRO trial of AT132. However, because the patient population, supportive care or other factors may be different than those used in the ASPIRO trial, we may be unable to use the RECENSUS study to demonstrate statistical significance of results in our planned ASPIRO trial, which may delay the development of AT132. Even if we demonstrate statistical significance, regulatory agencies may not accept the use of the historical control. Regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. We cannot be certain that we will not face similar setbacks.

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.

From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, manufacturing and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of preclinical studies and clinical trials and the submission of regulatory filings. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are, and will be, based on a variety of assumptions. The actual timing of these milestones can vary significantly compared to our estimates, in some cases for reasons beyond our control. We may experience numerous unforeseen events during, or as a result of, any future clinical trials that we conduct that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- the FDA and other governmental health authorities, Institutional Review Boards, or IRBs, or ethics committees may not authorize or may delay authorizing us or our investigators to commence a clinical trial or conduct a clinical trial at all or at a prospective trial site, such as by requiring us to conduct additional preclinical studies and to submit additional data or imposing other requirements before permitting us to initiate a clinical trial;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

- clinical trials of our product candidates may produce negative or inconclusive results and we may decide, or regulators may require us, to conduct preclinical studies in addition to those we currently have planned or additional clinical trials or we may decide to abandon drug development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, IRBs or ethics committees may require that we or our investigators, suspend or terminate clinical trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to health risks;
- the cost of planned clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs or ethics committees to suspend or terminate the trials, or reports may arise from preclinical or clinical testing of other gene therapies that raise safety or efficacy concerns about our product candidates.

For instance, safety signals have been observed at the highest dose in non-GLP mouse disease model studies of AT132 and AT982 that we conducted. In both programs, we have completed large animal GLP studies in which similar safety signals were not observed. We continue to conduct preclinical studies across our portfolio of product candidates. If we observe unexpected safety signals in these studies, we may decide, or regulatory authorities may require us, to delay or halt further development of our product candidates.

Our product candidates are based on a novel AAV gene therapy technology with which there is little clinical experience, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Currently, no gene therapy products have been approved in the United States and only two gene therapy products have been approved in Europe.

Our product candidates are based on gene therapy technology and our future success depends on the successful development of this novel therapeutic approach. We cannot assure you that any development problems we or other gene therapy companies experience in the future related to gene therapy technology will not cause significant delays or unanticipated costs in the development of our product candidates, or that such development problems can be solved. In addition, the clinical study requirements of the FDA, EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied product candidates. Further, as we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, EMA or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. To date, no gene therapy product has been approved in the United States and only two gene therapy products have been approved in Europe, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union, or EU, or other jurisdictions. Further, approvals by EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, the FDA established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. These and other regulatory review agencies, committees and advisory groups and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions.

The FDA, the National Institutes of Health, or NIH, the EMA and other regulatory agencies have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates, which may be difficult to predict.

The FDA, NIH, other regulatory agencies at both the federal and state level in the United States, U.S. congressional committees, and the EMA and other foreign governments, have expressed interest in further regulating the biotechnology industry, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Any such further regulation may delay or prevent commercialization of some or all of our product candidates. For example, in 1999, a patient died during a gene therapy clinical trial that utilized an adenovirus vector and it was later discovered that adenoviruses could generate an extreme immune system reaction that can be life-threatening. In January 2000, the FDA halted that trial and began investigating 69 other gene therapy trials underway in the United States, 13 of which required remedial action. In 2003, the FDA suspended 27 additional gene therapy trials involving several hundred patients after learning that some patients treated in a clinical trial in France had subsequently developed leukemia. While new AAV vectors have been developed to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop.

Regulatory requirements in the United States and abroad governing gene therapy products have changed frequently and may continue to change in the future. Our planned clinical trials will be subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or RAC. As of April 2016, the new NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, including gene therapy, provide the opportunity for one or more oversight bodies (IRB or the Institutional Biosafety Committee, or IBC,) to request a public RAC review based on their own review of the protocol and NIH requirements. Regardless of the request for public review, NIH makes their own assessment as to whether the protocol would significantly benefit from a public RAC review. The NIH's recommendations are shared with the FDA and the oversight bodies. The RAC can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and has not objected to its initiation or has notified the sponsor that the study may begin. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or has recommended against an in-depth, public review. If there is a public RAC review, the receipt of the final recommendation letter concludes the protocol registration process and then oversight body approval can be issued. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, the EMA governs the development of gene therapies in the EU and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

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

Prior to commercialization, our product candidates must be approved by the FDA pursuant to a BLA in the United States and by the EMA and similar regulatory authorities outside the United States. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have no experience in submitting 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 therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Approval of our product candidates may be delayed or refused for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical programs or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- our manufacturing facilities, or those of third-party manufacturers with which we contract or procure certain service or raw materials, may not be adequate to support approval of our product candidates; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or Risk Evaluation and Mitigation Strategies, or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially and adversely affect our business, financial condition, results of operations and prospects.

Further, the regulatory authorities may require concurrent approval or the CE mark, indicating conformity with applicability with European Community directives, of a companion diagnostic device. For the product candidates we currently are developing, we believe that diagnoses based on symptoms, in conjunction with existing genetic tests developed and administered by laboratories certified under the Clinical Laboratory Improvement Amendments, or CLIA, are sufficient to diagnose patients and will be permitted by the FDA. For future product candidates, however, it may be necessary to use FDA-cleared or FDA-approved diagnostic tests to diagnose patients or to assure the safe and effective use of product candidates in trial subjects. The FDA refers to such tests as *in vitro* companion diagnostic devices. In August 2014, the FDA issued a final guidance document describing the agency's current thinking about the development and regulation of *in vitro* companion diagnostic devices. The final guidance articulates a policy position that, when an *in vitro* diagnostic device is essential to the safe and effective use of a therapeutic product, the FDA generally will require approval or clearance of the diagnostic device at the same time that the FDA approves the therapeutic product. At this point, it is unclear how the FDA will apply this policy to our current or future gene therapy product candidates. Should the FDA deem genetic tests used for diagnosing patients for our therapies to be *in vitro* companion diagnostics requiring FDA clearance or approval, we may face significant delays or obstacles in obtaining approval of a BLA for our product candidates. In the EU, the European Commission has proposed substantial revisions to the current regulations governing *in vitro* diagnostic medical devices. If adopted in their current form, these revisions may impose additional obligations on us that may impact the development and authorization of our product candidates in the EU.

We may never obtain FDA approval for any of our product candidates in the United States, and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to eventually market any of our product candidates in any particular foreign jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a jurisdiction-by-jurisdiction basis regarding safety and efficacy. Approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. 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. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including 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 our products will be unrealized.

Delays in establishing that our manufacturing process and facility comply with cGMPs or disruptions in our manufacturing process may delay or disrupt our development and commercialization efforts. To date, no gene therapy product has received approval from the FDA so the requirements for the manufacture of a gene therapy product are uncertain.

We have established relationships with research facilities, CMOs and our collaborators to manufacture and supply our product candidates for preclinical and clinical studies, and we have invested in our own state-of-the-art cGMP manufacturing facility in South San Francisco, California. In this new facility, we are developing and implementing novel in-house production technologies to supply our planned pre-clinical and clinical trials. As we scale our internal manufacturing capabilities, we plan to transition all process development and manufacturing activities to our own facilities. We are currently manufacturing the clinical supply for ASPIRO, the phase 1/2 study of AT132, and VALENS, the phase 1/2 study of AT342, in our internal manufacturing facility. The IND we submitted for AT342 included a description of a manufacturing process and drug product that was manufactured by an external CMO. Before we may use drug product manufactured in our own facility to dose subjects in VALENS, we will need to submit an IND amendment to the FDA notifying the change to our internal manufacturing process and facility and provide data that shows that internally manufactured AT342 drug product is comparable to AT342 produced by the external CMO. We plan to submit this IND amendment after completion of additional pre-clinical studies to confirm similar pharmacologic activity and safety between externally and internally manufactured AT342 drug product. If we are unable to confirm the activity and safety of the two drug products in pre-clinical studies, or if the FDA does not agree with our assessment of new data contained in the planned IND amendment, we may experience a delay in the planned initiation of VALENS. The drug product planned to be used in the investigator sponsored proof-of-concept study of AT982 has been manufactured by the University of Florida in a facility that we believe complies with cGMPs.

Before we may initiate a clinical trial or commercialize any of our product candidates, we must demonstrate to the FDA that the chemistry, manufacturing and controls for our gene therapy product candidates meet applicable requirements. A manufacturing authorization must be obtained from the appropriate EU regulatory authorities. Because no gene therapy product has yet been approved in the United States, there is no manufacturing facility that has demonstrated the ability to comply with FDA requirements, and, therefore, the timeframe for demonstrating compliance to the FDA's satisfaction is uncertain. Delays in establishing that our manufacturing process and facility comply with cGMPs or disruptions in our manufacturing processes, implementation of novel in-house technologies or scale-up activities, may delay or disrupt our development efforts.

We expect that development of our own manufacturing facility will provide us with enhanced control of material supply for both clinical trials and the commercial market, enable the more rapid implementation of process changes and allow for better long-term margins. However, we have limited experience as a company in developing a manufacturing facility and may never be successful in developing our own manufacturing capability. Additionally, given that cGMP gene therapy manufacturing is a nascent industry, there are only a small number of CMOs with the experience necessary to manufacture our product candidates and we may have difficulty finding or maintaining relationships with such CMOs or hiring experts for internal manufacturing and, accordingly, our production capacity may be limited. Even if we are successful, our manufacturing capabilities could be affected by cost-overruns, unexpected delays, equipment failures, lack of capacity, labor shortages, natural disasters, power failures and numerous other factors that could prevent us from realizing the intended benefits of our manufacturing strategy and have a material adverse effect on our business.

In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before any of our product candidates can obtain marketing approval. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMPs, and perform extensive audits of vendors, contract laboratories and suppliers. If we, or any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMPs, we may experience delays or disruptions in manufacturing while we work to remedy the noncompliance or while we work to identify suitable replacement vendors. If we or our CMOs are unable to reliably produce products to specifications acceptable to the FDA or other regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CMOs will be able to manufacture the approved product to specifications acceptable to the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product or to meet potential future demand. Any of these challenges could delay initiation of, or completion

of, clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidate, impair commercialization efforts, increase our cost of goods and have an adverse effect on our business, financial condition, results of operations and growth prospects.

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

Our business model is centered on applying our expertise in rare diseases by establishing focused selection criteria to develop and advance a broad portfolio of gene therapy product candidates through development into commercialization. We may not be able to continue to identify and develop new product candidates in addition to the pipeline of product candidates that our research and development efforts to date have resulted in. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

Our product candidates based on gene therapy technology may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

As discussed above, there have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other trials using other vectors. While new AAV vectors have been developed to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which, while not necessarily adverse to the patient's health, could substantially limit the effectiveness and durability of the treatment. In previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a T-cell response, whereby after the vector is within the target cells, the cellular immune response system triggers the removal of transduced cells by activated T-cells. If our vectors demonstrate a similar effect, we may decide or be required to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we can demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of the product outweigh its risks, which may include, among other things, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these occurrences may harm our business, financial condition and prospects significantly.

The diseases we seek to treat have low prevalence and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue if approved.

Genetically defined diseases generally, and especially those for which our current product candidates are targeted, have low incidence and prevalence. For example, we estimate that the incidence of XLMTM is approximately one in 50,000 male births, that the incidence of Crigler-Najjar is approximately one in 1,000,000 births, that the incidence of Pompe disease is one in 40,000 births, and that there are approximately 6,000 people in North America, Europe and other addressable markets with CASQ2-CPVT. In addition, some of our potential patients may have neutralizing antibodies to the AAV capsid serotypes we employ, which may affect the therapeutic efficacy of our product candidates. This could pose obstacles to the timely recruitment and enrollment of a sufficient number of eligible patients into our trials. Patient enrollment may be affected by other factors including:

- the ability to identify and recruit patients that meet study eligibility criteria;
- the severity of the disease under investigation;
- design of the study protocol;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- our efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients with these diseases for our planned clinical trials would result in significant delays and could require us to not initiate or abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

Additionally, our projections of both the number of people who have XLMTM, Crigler-Najjar, Pompe disease and CASQ2-CPVT, as well as the people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. The total addressable market opportunity for our product candidates will ultimately depend upon, among other things, the final labeling for each of our product candidates, if our product candidates are approved for sale in our target indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients globally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. Our products may potentially be dosed on a one-time basis, which means that patients who enroll in our clinical trials may not be eligible to receive our products on a commercial basis if they are approved, leading to lower revenue potential.

A Breakthrough Therapy Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We plan to seek a Breakthrough Therapy Designation for our product candidates if the clinical data support such a designation for one or more product candidates. A breakthrough therapy is defined as a drug or biologic that is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug, or biologic in our case, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited the FDA review procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product no longer meets the conditions for qualification.

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

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

We may also seek accelerated approval for products that have obtained Fast Track Designation. Under the FDA's accelerated approval program, the FDA may approve a biologic for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. For biologics granted accelerated approval, post-marketing confirmatory trials are required to describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. These confirmatory trials must be completed with due diligence and, in some cases, the FDA may require that the trial be designed and/or initiated prior to approval. Moreover, the FDA may withdraw approval of any product candidate or indication approved under the accelerated approval pathway if, for example:

- the trial or trials required to verify the predicted clinical benefit of the product candidate fail to verify such benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the biologic;
- other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use;
- we fail to conduct any required post-approval trial of the product candidate with due diligence; or
- we disseminate false or misleading promotional materials relating to the product candidate.

We may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity, for AT132, AT342, AT982 and AT307, and may be unsuccessful in obtaining Orphan Drug Designation or transfer of designations obtained by others for our other current or future product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs, or biologics in our case, intended to treat relatively small patient populations as orphan drugs. Under the U.S. Orphan Drug Act, the FDA may designate a biologic as an orphan drug if it is intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax credits for qualified clinical research costs, and prescription drug user fee waivers. Similarly, in the EU, the European Commission grants Orphan Drug Designation after receiving the opinion of the EMA's Committee for Orphan Medicinal Products on an Orphan Drug Designation application. In the EU, Orphan Drug Designation is intended to promote the development of biologics that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or the product would be a significant benefit to those affected). In the EU, Orphan Drug Designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a biologic with an Orphan Drug Designation subsequently receives the first marketing approval for the indication for which it has such designation, the biologic is entitled to a period of marketing exclusivity, which precludes EMA or the FDA from approving another marketing application for the same biologic and indication for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us for products that constitute the same active moiety and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time. The applicable period is seven years in the United States and ten years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for Orphan Drug Designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

As part of our business strategy, we have sought and received Orphan Drug Designation for AT132, AT342, AT982 and AT307 in the United States and Europe. However, Orphan Drug Designation does not guarantee future orphan drug marketing exclusivity.

Additionally, even though we have obtained an Orphan Drug Designation for AT132, AT342, AT982 and AT307, and even if we obtain orphan drug exclusivity for these product candidates and other product candidates, that exclusivity may not effectively protect AT132, AT342, AT982 and AT307 from competition because drugs with different active moieties can be approved for the same condition. Even after an orphan drug is approved, the FDA can also subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We rely on third parties to conduct our preclinical studies, will rely on them to conduct clinical trials and rely on them to perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

Although we have recruited a team that has experience with clinical trials, as a company we have no experience in conducting clinical trials. Moreover, we do not have the ability to independently conduct preclinical studies and clinical trials, and we have relied upon, and plan to continue to rely upon medical institutions, clinical investigators, contract laboratories and other third parties, or our CROs, to conduct preclinical studies and future clinical trials for our product candidates. We expect to rely heavily on these parties for execution of preclinical and future clinical trials for our product candidates and control only certain aspects of their activities. Nevertheless, we will be responsible for ensuring that each of our preclinical and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards and our reliance on CROs will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our preclinical studies and clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We and our CROs will be required to comply with regulations, including GCPs for conducting, monitoring, recording and reporting the results of preclinical and clinical trials to ensure that the data and results are scientifically credible and accurate and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any drugs in clinical development. The FDA enforces GCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable GCPs, 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. We cannot assure you that, upon inspection, the FDA will determine that any of our future clinical trials will comply with GCPs. In addition, our clinical trials must be conducted with product candidates produced in accordance with the requirements in GMP regulations. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action.

Although we intend to design our planned clinical trials for our product candidates, for the foreseeable future CROs will conduct all of our planned clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less day-to-day control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, any preclinical studies or clinical trials with which such CROs are associated with may be extended, delayed or terminated. In such cases, we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates in the subject indication could be harmed, our costs could increase and our ability to generate revenue could be delayed.

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

Our product candidates and the activities associated with their development and potential commercialization, including their testing, manufacture, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMPs, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities and requirements regarding the distribution of samples to physicians and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of any approved product. The FDA closely regulates the post-approval marketing and promotion of drugs and biologics to ensure drugs and biologics are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding use of their products. If we promote our product candidates beyond their potentially approved indications, we may be subject to enforcement action for off-label promotion. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

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

- restrictions on such product candidates, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of any approved product from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of product candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our product candidates;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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

Our product candidates for which we intend to seek approval may face competition from biosimilars sooner than anticipated.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as interchangeable based on its similarity to an existing reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product is approved under a biologics license application, or BLA. To date a handful of biosimilar products and no interchangeable products have been approved under the BPCIA. However, the law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates are approved as a biological product under a BLA it should qualify for the 12-year period of exclusivity. However, there is a risk that the FDA will not consider any of our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. Finally, there has been public discussion of potentially decreasing the period of exclusivity from the current 12 years. If such a change were to be enacted, our product candidates, if approved, could have a shorter period of exclusivity than anticipated.

Our strategy of obtaining rights to key technologies through in-licenses may not be successful.

We seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies, including those related to gene delivery. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies. We cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

The in-licensing and acquisition of these technologies is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

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

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the Affordable Care Act, or the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The current federal administration has indicated an intent to repeal the ACA. The President has indicated an intent to address prescription drug pricing and recent Congressional hearings have brought increased public attention to the costs of prescription drugs. These actions and the uncertainty about the future of the ACA and healthcare laws may put downward pressure on pharmaceutical pricing and increase our regulatory burdens and operating costs.

Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

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

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we obtain marketing approval.

Restrictions under applicable U.S. federal and state healthcare laws and regulations may include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- federal false claims laws, including the federal False Claims Act, imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, knowingly and willfully executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health, or HITECH, Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of people and entities with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payment Sunshine Act requires applicable manufacturers of covered 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 payments and other transfers of value to physicians and teaching hospitals, as well as certain ownership and investment interests held by physicians and their immediate family, which includes annual data collection and reporting obligations; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

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

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of product candidates from government-funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

Risks Related to Manufacturing and Commercialization

Gene therapy products are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in our development or commercialization programs or otherwise harm our business.

The manufacturing processes used to produce our product candidates are complex, novel and have not been validated for commercial use. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of our suppliers.

Our product candidates require processing steps that are more complex than those required for most small molecule drugs. Moreover, unlike small molecules, the physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product is consistent from lot-to-lot or will perform in the intended manner. Accordingly, we employ multiple steps to control the manufacturing process to assure that the process works reproducibly and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving

adequate quantities and quality of clinical-grade materials that meet the FDA, the EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We also may encounter problems hiring and retaining the experienced scientific, quality assurance, quality-control and manufacturing personnel needed to operate our manufacturing processes, which could result in delays in production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or facilities could result in delays in our planned clinical trials and increased costs, and could make us a less attractive collaborator for potential partners, including larger biotechnology companies and academic research institutions, which could limit our access to additional attractive development programs. It could also require us to find alternative manufacturing processes, which may be unavailable to us on attractive terms, or at all. Problems in our manufacturing process could restrict our ability to meet potential future market demand for our products.

We and our collaborators, third-party manufacturers and suppliers use biological materials and may use hazardous materials, and any claims relating to improper handling, storage or disposal of these materials could be time consuming or costly.

We and our collaborators, third-party manufacturers and suppliers may use hazardous materials, including chemicals and biological agents and compounds that could be dangerous to human health and safety or the environment. Our operations and the operations of our third-party manufacturers and suppliers also produce hazardous waste products. Federal, state and local laws and regulations govern the use, generation, manufacture, storage, handling and disposal of these materials and wastes. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development efforts. In addition, we cannot eliminate the risk of accidental injury or contamination from these materials or wastes. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

Any contamination in our or our third parties' manufacturing process, shortages of raw materials or reagents or failure of any of our key suppliers to deliver necessary components of our platform could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our or our third-party vendor's ability to produce our gene therapies on schedule and could therefore harm our results of operations and cause reputational damage.

The raw materials required in our and our third-party vendors manufacturing processes are derived from biological sources. We cannot assure you that we or our third-party vendors have, or will be able to obtain on commercially reasonable terms, or at all, sufficient rights to these materials derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the clinical and commercial manufacturing of our product candidates, which could materially and adversely affect our operating results and development timelines.

We rely on third-party suppliers for the supply and manufacture of certain components of our technology. Should our ability to procure these material components from our suppliers be compromised, our ability to continuously operate would be impaired until an alternative supplier is sourced, qualified and tested, which could limit our ability to produce a clinical and commercial supply of our product candidates and harm our business.

We do not have complete control over any current or future third-party manufacturers' processes and compliance with applicable regulations.

While we are transitioning the manufacturing of our product candidates to our internal facility, we continue to utilize third-party manufacturers and may in the future utilize other third-party manufacturers. Third-party manufacturers may not have the experience or ability to produce our product candidates at clinical or commercial scales within our planned timeframe and cost parameters, and such manufacturers may run into technical or scientific issues that we may be unable to resolve in a timely manner or with available funds. Additionally, the manufacturing of product candidates for clinical and commercial purposes must comply with the cGMP and applicable non-U.S. regulatory requirements. The cGMP requirements govern quality control and documentation policies and procedures. Third-party manufacturers' must demonstrate to the FDA that they can make the product candidate in accordance with the cGMP requirements as part of a pre-approval inspection prior to FDA approval of the product candidate. Failure to pass a pre-approval inspection might significantly delay FDA approval of our product candidates. If any third-party manufacturer fails to comply with FDA or applicable non-U.S. regulatory requirements, we would be subject to possible regulatory action, which could limit the jurisdictions in which we are permitted to sell our products. As a result, our business, financial condition and results of operations may be materially harmed.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from FDA in the United States, the EMA in the EU and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do 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 gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and adequate reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

We face significant competition in an environment of rapid technological change and it is possible that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and financial condition, and our ability to successfully market or commercialize our product candidates.

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, competition and a strong emphasis on intellectual property. We are aware of several companies focused on developing gene therapies in various indications as well as several companies addressing other methods for modifying genes and regulating gene

expression. We may also face competition from large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

For the treatment of XLMTM, Valerion Therapeutics, LLC is studying VAL-0620, a fusion protein consisting of an antibody linked to MTM1. Preclinical evaluation of this approach in the MTM1 murine model demonstrated improvements in both muscle structure and function, as reported in a 2013 publication. This program has not been reported by Valerion Therapeutics, LLC to have progressed to clinical development.

For the treatment of Crigler-Najjar, the current standard of care is phototherapy, and upon disease progression, liver transplant. There are currently no products approved specifically for the treatment of Crigler-Najjar. Genethon, a French not-for-profit organization, is developing an AAV-UGT1A1 gene therapy for the treatment of Crigler-Najjar syndrome, and has announced plans to initiate clinical development. Promethera has received orphan drug designation from the FDA and European Commission for the treatment of Crigler-Najjar syndrome for HepaStem, a product that comprises heterologous human adult liver progenitor cells. Promethera previously completed a Phase 1/2 study that enrolled patients with Crigler-Najjar syndrome or ornithine transcarbamylase deficiency. No further development in Crigler-Najjar syndrome has been announced for HepaStem. Additionally, Alexion and Moderna are collaborating to develop a messenger RNA product candidate for the treatment of Crigler-Najjar, but Alexion has announced delays in the program while Moderna evaluates new formulations.

For the treatment of Pompe disease, the current standard of care is ERT with recombinant GAA protein. Genzyme Corporation currently markets MYOZYME and LUMIZYME, which are ERTs for the treatment of Pompe disease. Multiple companies, including Genzyme Corporation, Amicus Therapeutics, Inc., Valerion Therapeutics, LLC and Oxyrane UK Limited are currently reported to be developing next generation ERT to treat Pompe disease. The furthest advanced of these is neoGAA from Genzyme Corporation. In addition, there are currently multiple academic institutions and companies researching alternative gene therapy approaches to treating Pompe disease. We do not believe these approaches utilize AAV9 capsids and none are currently reported to be in clinical development.

For the treatment of CASQ2-CPVT, patients commonly receive nadolol or propranolol as first-line treatment, sometimes with the addition of a calcium channel blocker. Flecainide, a sodium channel blocker, and implantable cardioverter defibrillators, are also used in the treatment of CASQ2-CPVT. Although infrequent, refractory cases may receive a heart transplant. There are no known investigational therapies in development for CASQ2-CPVT.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources than we do, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any product candidates that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, if ever. Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities can include completing preclinical studies and initiating and completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products that are approved 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 revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

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

Our target indications, including XLMTM, Crigler-Najjar, Pompe disease and CASQ2-CPVT, are indications with small patient populations. In order for products that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such products must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are

unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those products and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial when and if they achieve regulatory approval. Therefore, we expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any of our product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. However, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Further, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. It is difficult to predict what CMS will decide with respect to reimbursement for novel products such as ours since there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

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

In addition to CMS and private payors, professional organizations such as the American Medical Association, or the AMA, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If in the future we are unable to establish U.S. or global sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing our product candidates if they are approved and we may not be able to generate any revenue.

We currently do not have a marketing or sales team for the marketing, sales and distribution of any of our product candidates that are able to obtain regulatory approval. In order to commercialize any product candidates after approval, we must build on a territory-by-territory basis marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. If our product candidates receive regulatory

approval, we may decide to establish an internal sales or marketing team with technical expertise and supporting distribution capabilities to commercialize our product candidates, which will be expensive and time-consuming and will require significant attention of our executive officers to manage. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of any of our product candidates that we obtain approval to market.

With respect to the commercialization of all or certain of our product candidates, we may choose to collaborate, either globally or on a territory-by-territory basis, with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. If we are unable to enter into such arrangements when needed on acceptable terms, or at all, we may not be able to successfully commercialize any of our product candidates that receive regulatory approval or any such commercialization may experience delays or limitations. If we are not successful in commercializing our product candidates, either on our own or through collaborations with one or more third parties, our future product revenue will suffer and we may incur significant additional losses.

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

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

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing or competing in the market for certain indications.

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

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

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

In addition, our collaborations with the University of Pennsylvania and the University of Florida, and any future collaborations that we enter into, may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in

some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Risks Related to Our Financial Position

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

We are an early-stage biotechnology company with a limited operating history on which to base your investment decision. Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring and developing product and technology rights and conducting preclinical research and development activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates, and have funded our operations to date through proceeds from sales of our preferred stock.

We have incurred net losses in each year since our inception. We incurred a net loss of $18.1 million and $10.5 million for the three months ended March 31, 2017 and 2016, respectively. As of March 31, 2017, we had an accumulated deficit of $118.5 million. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as we intend to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated general and administrative expenses, will likely result in us incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital.

We expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of our products. This additional financing may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our product development efforts or other operations.

We will require substantial future capital in order to complete planned and future preclinical and clinical development for AT132, AT342, AT982, AT307 and other future product candidates, if any, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical studies and clinical trials of our lead product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. 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 would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. As of March 31, 2017, our cash, cash equivalents and short-term investments were $81.7 million, and we received net proceeds of $80.7 million from our April 2017 offering. We expect that the net proceeds from our April 2017 offering, together with our existing cash, cash equivalents and short-term investments, will enable us to fund our operating expenses and capital expenditure requirements through late 2019.

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

- the costs associated with the scope, progress and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the costs associated with the development of our internal manufacturing facility and processes;
- the costs related to the extent to which we enter into partnerships or other arrangements with third parties in order to further develop our product candidates;
- the costs and fees associated with the discovery, acquisition or in-license of product candidates or technologies;
- our ability to establish collaborations on favorable terms, if at all;
- the costs of future commercialization activities, if any, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;

- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

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, which may not be available to us on acceptable terms, or at all.

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

We are a preclinical company formed in November 2012. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring our technology, identifying potential product candidates and undertaking research and preclinical studies of our product candidates and establishing licensing arrangements. We have not yet demonstrated the ability to complete and report clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development and commercial activities. We may not be successful in such a transition.

Our ability to utilize our net operating loss carryforwards may be subject to limitation.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. As of December 31, 2016, we had federal net operating loss carryforwards of $77.3 million, which begin to expire in 2033. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," 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, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Risks Related to Intellectual Property

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

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the United States and other countries for our current product candidates and future products, as well as our core technologies, including our manufacturing know-how. We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business by seeking, maintaining and defending our intellectual property, whether developed internally or licensed from third parties. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy. Additionally, we intend to rely on regulatory protection afforded through rare drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

Our in-licensed patents and patent applications are directed to the compositions of matter and methods of use related to various aspects of our product candidates as well as certain aspects of our manufacturing capabilities. 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.

The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our current and future product candidates or otherwise provide any competitive advantage. The FSM and Genethon patent families were filed only in the United States, and therefore these patent families will not provide patent protection outside the United States. While other patent families include foreign counterparts, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. In addition, none of the patent applications licensed from the University of Florida Research Foundation relating to gene therapy for Pompe disease have matured into issued patents in the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. 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. If any of our AT132, AT342, AT982 or AT307 product candidates are approved by the FDA as a biological product under a BLA in the United States, we believe the product would qualify for a 12-year period of exclusivity. For example, if our AT132 product was approved by the FDA as a biological product under a BLA in 2020, we believe it would qualify for a 12-year period of exclusivity, which would expire in 2032, or two years before the Genethon patent family will expire in the United States absent patent term adjustment or patent term extension. Similarly, if our AT307 product was approved by the FDA as a biological product under a BLA in 2020, we believe it would qualify for a 12-year period of exclusivity, which would expire in 2032, the same year the FSM patent family will expire in the United States absent patent term adjustment or patent term extension. Moreover, our exclusive license is subject to retained rights, which may adversely impact our competitive position. As a result, our licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to our product candidates, including biosimilar versions of such products. In addition, the patent portfolio licensed to us is, or may be, licensed to third parties, such as outside our field, and such third parties may have certain enforcement rights. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

Other parties have developed technologies that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether the inventors of our licensed patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Further, we cannot assure you that all of the potentially relevant prior art relating to our licensed patents and patent applications has been found. If such prior art exists, it can invalidate a patent or prevent a patent from issuing from a pending patent application. As a result, the issuance, scope, validity and commercial value of our patent rights cannot be predicted with any certainty.

In addition, the patent prosecution process is expensive and time-consuming, and we or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We cannot provide any assurances that we will be able to pursue or obtain additional patent protection based on our research and development efforts, or that any such patents or other intellectual property we generate will provide any competitive advantage. Patent prosecution is a lengthy process and the scope of the claims initially submitted for examination may be significantly narrowed by the time they issue, if at all. Moreover, we do not have the right to control the preparation, filing and prosecution of patent applications, or to control the maintenance of the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be filed, prosecuted or maintained in a manner consistent with the best interests of our business.

Even if we acquire patent protection that we expect should enable us to maintain competitive advantage, third parties, including competitors, may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose our rights to those challenged patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability and our licensed patents may be challenged in courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our pending licensed patent applications. We may become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review, interference, or similar proceedings in the United States or abroad, challenging the patent rights of others from whom we have obtained licenses to such rights. Furthermore, our licensed patents may be challenged in district court. Competitors may claim that they invented the inventions claimed in such issued patents or patent applications prior to the inventors of our licensed patents, or may

have filed patent applications before the inventors of our licensed patents did. A competitor may also claim that we are infringing its patents and that we therefore cannot practice our technology as claimed under our licensed patents, if issued. As a result, one or more claims of our licensed patents may be narrowed or invalidated.

Even if they are unchallenged, our licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our licensed patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, even if we have a valid and enforceable patent, we may not be able to exclude others from practicing our invention if the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. Moreover, a third party may develop a competitive product that provides benefits similar to one or more of our product candidates but that uses a vector or an expression construct that falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Although currently all of our patents and patent applications are in-licensed, similar risks would apply to any patents or patent applications that we may own or in-license in the future.

If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.

If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates or technology from third parties, we could lose license rights that are important to our business. We currently hold licenses or other rights for certain intellectual property, such as from REGENXBIO relating to various AAV vectors, from Genethon related to XLMTM, from the University of Pennsylvania relating to Crigler-Najjar, from the University of Florida Research Foundation relating to Pompe disease, and from the Fondazione Salvatore Maugeri relating to various nucleic acid sequences associated with single mutation arrhythmias related to CASQ2-CPVT.

Under our existing license agreements, we are subject to various obligations, including diligence obligations such as development and commercialization obligations, as well as potential royalty payments and other obligations. If we fail to comply with any of these obligations or otherwise breach our license agreements, our licensors may have the right to terminate the applicable license in whole or in part. Generally, the loss of any one of our current licenses, or any other license we may acquire in the future, could harm our business, prospects, financial condition and results of operations.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- whether and the extent to which inventors are able to contest the assignment of their rights to our licensors.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. In addition, if disputes arise as to ownership of licensed intellectual property, our ability to pursue or enforce the licensed patent rights may be jeopardized. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

All of our current product candidates are licensed from or based upon licenses from third parties. If any of these license or sublicense agreements are terminated or interpreted to narrow our rights, our ability to advance our current product candidates or develop new product candidates based on these technologies will be materially adversely affected.

We now depend, and will continue to depend, on licenses and sublicenses from third parties and potentially on other strategic relationships with third parties for the research, development, manufacturing and commercialization of our current product candidates. If any of our licenses or relationships or any in-licenses on which our licenses are based are terminated or breached, we may:

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

Additionally, even if not terminated or breached, our intellectual property licenses or sublicenses 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.

If we experience any of the foregoing, it could have a materially adverse effect on our business and could force us to cease operations which could cause you to lose all of your investment.

We are required to pay certain royalties under our license agreements with third-party licensors, and we must meet certain milestones to maintain our license rights.

Under our license agreements with REGENXBIO, the University of Florida Research Foundation, the University of Pennsylvania and FSM, we will be required to pay royalties based on our net revenues from sales of our products utilizing the technologies and products. These royalty payments could adversely affect the overall profitability for us of any products that we may seek to commercialize. In order to maintain our license rights under these license agreements, we will need to meet certain specified milestones, subject to certain cure provisions, in the development of our product candidates and in the raising of funding. In addition, these agreements contain development obligations and we may not be successful in meeting all of the obligations in the future on a timely basis or at all. We may need to outsource and rely on third parties for many aspects of the clinical development, sales and marketing of our products covered under our license agreements. Delay or failure by any such third parties could adversely affect the continuation of our license agreements with third-party licensors. For example, our Exclusive License Agreement with the University of Florida Research Foundation provides that the University of Florida Research Foundation has the right to terminate the agreement if we do not meet certain deadlines.

Third parties may initiate legal proceedings alleging claims of intellectual property infringement, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and future products 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 frequent litigation regarding patents and other intellectual property rights. We may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates, future products and technology, including interference or *inter partes* review proceedings before the USPTO. Our competitors or other third parties may assert infringement or misappropriation claims against us, alleging that our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents. For example, we do not know which processes we will use for commercial manufacture of our future products, or which technologies owned or controlled by third parties may prove important or essential to those processes. Given the vast number of patents in our field of technology, we cannot be certain or guarantee that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. Many companies have filed, and continue to file, patent applications related to gene therapy and orphan diseases. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Furthermore, because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates or future products. If a patent holder believes the manufacture, use, sale or importation of one of our product candidates or future products infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our technology.

Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our licensed patent portfolio may therefore have no deterrent effect.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale, importation or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our future products or the manufacture or use of our future products.

Third parties may assert infringement claims against us based on existing intellectual property rights and intellectual property rights that may be granted in the future. If we were to challenge the validity of an issued U.S. patent in court, such as an issued U.S. patent of potential relevance to some of our product candidates or future products or manufacture or methods of use, we would need to overcome a statutory presumption of validity that attaches to every U.S. patent. This means that in order to prevail, we would have to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on questions of infringement or validity.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. If we are found, or believe there is a risk we may be found, to infringe a third party's intellectual property rights, we could be required or may choose to obtain a license from such third party to continue developing and marketing our products and technology. However, we may not be able to obtain any such license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. Without such a license, we could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our future products or force us to cease some of our business operations, which could materially harm our business. Alternatively, we may need to redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. If we lose a foreign patent lawsuit, alleging our infringement of a competitor's patents, we could be prevented from marketing our therapeutics in one or more foreign countries and/or be required to pay monetary damages for infringement or royalties in order to continue marketing. Claims that we have misappropriated the confidential information, trade secrets or other intellectual property of third parties could have a similar negative impact on our business. Any of these outcomes would have a materially adverse effect on our business.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our future products or processes. Patent litigation is costly and time-consuming, and some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. We may not have sufficient resources to bring these actions to a successful conclusion. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

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

In addition to the protection afforded by patents, we rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our consultants and employees. We may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, however, despite the existence generally of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

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

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents often must be paid to the USPTO and foreign patent agencies over the lifetime of the patent. While an unintentional lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, we may not be able to stop a competitor from marketing drugs that are the same as or similar to our product candidates, which would have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Many of the intellectual property rights we have licensed are generated through the use of U.S. government funding and are therefore subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Further, licensing partners may not prosecute patents in certain jurisdictions in which we may obtain commercial rights, thereby precluding the possibility of later obtaining patent protection in these countries. For example, the FSM and Genethon patent families were only filed in the United States, and therefore these patent families will not provide patent

protection outside the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

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

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

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a "first to file" system. The first-to-file provisions, however, only became effective in March 2013. Accordingly, it is not yet 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 make it more difficult to obtain patent protection for our inventions and 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 harm our business, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. For example, in Association for Molecular Pathology v. Myriad Genetics, Inc., the Supreme Court ruled that a "naturally occurring DNA segment is a product of nature and not patent eligible merely because it has been isolated," and invalidated Myriad Genetics's patents on the BRCA1 and BRCA2 genes. Certain claims of our licensed patents relate to isolated AAV vectors, capsid proteins, or nucleic acids. To the extent that such claims are deemed to be directed to natural products, or to lack an inventive concept above and beyond an isolated natural product, a court may decide the claims are invalid under Myriad. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants or advisors, and the employees, consultants or advisors of our licensors, are currently, or were previously, employed at or affiliated with universities, hospitals 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. Moreover, some of our and our licensors' employees, consultants or advisors are or have been affiliated with multiple institutions. There is no guarantee that such institutions will not challenge our or our licensors' intellectual property ownership rights. 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 policy 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 Employee Matters, Managing Growth and Other Risks Related to Our Business

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As our development, manufacturing and commercialization plans and strategies develop, and as we fully transition our operations as a public company, we expect to need and are actively recruiting additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA and international regulatory review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and other resources, and a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management and manufacturing. We cannot assure you that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

We are highly dependent on the research and development, clinical and business development expertise of Matthew Patterson, our President and Chief Executive Officer, Dr. Suyash Prasad, our Chief Medical Officer, Dr. John Gray, our Head of Research and Development, Natalie Holles, our Chief Operating Officer, and Thomas Soloway, our Chief Financial Officer, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment letter agreements or employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and manufacturing strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Recruiting and retaining qualified scientific, clinical, manufacturing and, if needed, sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Failure to succeed in clinical trials may make it more challenging to recruit and retain qualified scientific personnel.

If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. We have begun the process of documenting, reviewing and improving our internal controls and procedures for compliance with Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, which will require annual management assessment of the effectiveness of our internal control over financial reporting beginning with the year ended December 31, 2017.

During the audit of our financial statements for the years ended December 31, 2015 and 2014 a material weakness was identified in our internal control over financial reporting. Under standards established by the Public Company Accounting Oversight Board, a material weakness is a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected and corrected on a timely basis. The material weakness that was identified related to a lack of sufficient accounting resources and personnel that limited our ability to adequately segregate duties, establish defined accounting policies and procedures and perform timely reviews of account reconciliations.

During 2016 we implemented measures to improve our internal control over financial reporting to address the underlying causes of the previously identified material weakness, including (i) the hiring of our Chief Financial Officer and other accounting personnel, (ii) establishing new accounting policies and procedures, (iii) implementing a new enterprise accounting system, and (iv)

implementing appropriate disclosure controls and procedures We believe that the remediation steps outlined above were sufficient to remediate the previously identified material weakness in internal control over financial reporting as discussed above.

We, and our independent registered public accounting firm, were not required to perform an evaluation of our internal control over financial reporting as of December 31, 2016 in accordance with the provisions of the Sarbanes-Oxley Act. Accordingly, we cannot assure you that we have identified all, or that we will not in the future have additional, material weaknesses.

Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our stock price and make it more difficult for us to raise new capital or effectively market and sell our product candidates once they are approved for commercial sale.

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

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company, which we expect will increase after we are no longer an "emerging growth company." In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the Securities and Exchange Commission and The NASDAQ Stock Market LLC, or NASDAQ, have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an emerging growth company until the earlier of (i) the last day of the fiscal year in which we have total annual gross revenue of $1 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of our initial public offering; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;

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

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

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and operating results.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business, including, for example our August 2015 acquisition of Cardiogen. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures, and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or operating results.

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

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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

Natural disasters could severely disrupt our operations and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our 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 have not experienced 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.

Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

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 the regulations of FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, 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 may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

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

We will face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and will face an even greater risk if we commercialize any of our product candidates. If we cannot successfully defend ourselves against claims that our 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 candidates that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;

- loss of revenue; and
- the inability to commercialize any product candidates that we may develop.

We currently maintain product liability insurance coverage of up to $10.0 million, which may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage when we begin clinical trials 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.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other partners from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products abroad once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

Risks Related to Our Common Stock

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

Our stock price is likely to be volatile. The stock market in general and the market for biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the price at which you purchased them. The market price for our common stock may be influenced by many factors, including:

- the success of competitive drugs or technologies;
- results of preclinical studies or clinical trials of our product candidates or those of our competitors;
- unanticipated or serious safety concerns related to the use of any of our product candidates;
- adverse regulatory decisions, including failure to receive regulatory approval for any of our product candidates;
- regulatory or legal developments in the United States and other countries;
- the size and growth of our prospective patient populations;
- developments concerning our collaborators, our external manufacturers or in-house manufacturing capabilities;
- inability to obtain adequate product supply for any product candidate for preclinical studies, clinical trials or future commercial sale or inability to do so at acceptable prices;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or drugs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the biotechnology sector;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock depends in part on the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable evaluations of our company or our stock, the price of our stock could decline. If one or more of these analysts ceases to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Our executive officers, directors, principal stockholders and their affiliates have significant influence over our company, which will limit your ability to influence corporate matters and could delay or prevent a change in corporate control.

Our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially own a majority of our outstanding capital stock. Therefore, this group of stockholders will have the ability to control us through this ownership position, and these stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents or approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

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

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting our planned clinical trials, manufacturing and commercialization efforts, expanded research and development activities and costs associated with operating as a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

We have never declared or paid any cash dividends on our common stock and do not currently intend to do so for the foreseeable future. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will be limited to the appreciation of stock. Therefore, the success of an investment in shares of our common stock will depend upon any future appreciation in value of the stock. We cannot guarantee you that shares of our common stock will appreciate in value or even maintain the price at which our stockholders have purchased their shares.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to us or our stockholders, any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws or any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein and

the claim not being one which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery or for which the Court of Chancery does not have subject matter jurisdiction. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision may limit our stockholders' ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, employees or agents, which may discourage such lawsuits against us and our directors, officers, employees and agents even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have a material adverse effect on our business, financial condition or results of operations.

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

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your 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 so that not all members of our board are elected at one time;
- permit only the board of directors to establish the number of directors and fill vacancies on the board;
- provide that directors may only be removed "for cause" and only with the approval of two-thirds of our stockholders;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;
- authorize the issuance of "blank check" preferred stock that our board could use to implement a stockholder rights plan, also known as a "poison pill";
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting; and
- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

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

Any of these provisions of our charter documents or Delaware law could, under certain circumstances, depress the market price of our common stock.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

Unregistered Sales of Equity Securities

In connection with the entry into a loan agreement, on March 7, 2017, we issued a warrant to Hercules Technology III, L.P., a Delaware limited partnership, or Hercules Technology, exercisable for the number of shares of our common stock that is equal to the greater of (i) 3.0% of the aggregate amount of the term loan advances funded under the Loan Agreement or (ii) $150,000, in each case divided by the exercise price of $15.13 per share, subject to adjustment from time to time in accordance with the provisions of the warrant. The warrant is immediately exercisable through the earlier of (i) March 7, 2022 and (ii) the consummation of certain acquisition transactions involving us as set forth in the warrant. The number of shares for which the warrant is exercisable and the associated exercise price are subject to certain proportional adjustments as set forth in the warrant.

Neither we nor Hercules Technology engaged any investment advisors with respect to the issuance of the warrant and no finders' fees were paid to any party in connection therewith. The issuance of the warrant was made in reliance on the exemption from registration contained in Section 4(a)(2) of the Securities Act and Rule 506(b) of Regulation D thereunder.

Use of Proceeds

On July 19, 2016, our Registration Statement on Form S-1 (File No. 333-208842) relating to the initial public offering of our common stock was declared effective by the SEC.

There has been no material change in the expected use of the net proceeds from our initial public offering, as described in our final Prospectus filed with the SEC on July 20, 2016 pursuant to Rule 424(b)

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

Not applicable.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

The exhibits filed or furnished as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

SIGNATURES

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

AUDENTES THERAPEUTICS, INC.

Date: May 11, 2017

By: /s/ Matthew Patterson
Matthew Patterson
President and Chief Executive Officer
(Principal Executive Officer)

Date: May 11, 2017

By: /s/ Thomas Soloway
Thomas Soloway
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed/Furnished Herewith
		Form	File No.	Exhibit Filing Date	
4.1	Warrant Agreement with Hercules Technology III, L.P. dated March 7, 2017.	10-K	001-37833	4.3	March 13, 2017
10.1	Net Commercial Lease, effective June 1, 2017, by and between the Registrant and JCN Partners.	10-K	001-37833	10.11	March 13, 2017
10.2	Net Commercial Lease, effective May 1, 2017, by and between the Registrant and 546 Eccles Avenue, a California Limited Partnership.	10-K	001-37833	10.12	March 13, 2017
10.3	First Amendment to Lease Agreement, effective May 1, 2017, by and between the Registrant and 546 Eccles Avenue, a California Limited Partnership.	10-K	001-37833	10.13	March 13, 2017
10.4	Loan and Security Agreement, dated March 7, 2017, by and between the Registrant, each of its Qualified Subsidiaries, the several banks and other financial institutions party thereto, and Hercules Capital, Inc.	10-K	001-37833	10.25	March 13, 2017
10.5†	Second Amendment to Exclusive License and Collaboration Agreement, dated March 21, 2017, by and between the Registrant and The Trustees of the University of Pennsylvania.	8-K	001-37833	10.01	April 3, 2017
31.1	Certification of Principal Executive Officer, pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
31.2	Certification of Principal Financial Officer, pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
32.1*	Certification of Chief Executive Officer, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2*	Certification of Chief Financial Officer, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
101.INS	XBRL Instance Document.				X
101.SCH	XBRL Taxonomy Extension Schema Document.				X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.				X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.				X
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document.				X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.				X

* This certification is deemed not filed for purpose of section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act or the Exchange Act.

† Registrant has omitted and filed separately with the SEC portions of the exhibit pursuant to a confidential treatment order granted under Rule 24b-2 promulgated under the Exchange Act.

Exhibit 31.1

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew Patterson, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Audentes 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)) for the registrant and have:

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

b) 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

c) 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 11, 2017

/s/ Matthew Patterson
Matthew Patterson
Chief Executive Officer
(Principal Executive Officer*)*

Exhibit 31.2

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Thomas Soloway, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Audentes 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)) for the registrant and have:

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

b) 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

c) 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 11, 2017

/s/ Thomas Soloway
Thomas Soloway
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

I, Matthew Patterson, Chief Executive Officer of Audentes Therapeutics, Inc. (Company), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

- the Quarterly Report on Form 10-Q of the Company for the quarter ended March 31, 2017 (Report), as filed with the Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company for the periods presented therein.

Date: May 11, 2017

/s/ Matthew Patterson
Matthew Patterson
Chief Executive Officer
(Principal Executive Officer*)*

Exhibit 32.2

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

I, Thomas Soloway, Chief Financial Officer of Audentes Therapeutics, Inc. (Company), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

- the Quarterly Report on Form 10-Q of the Company for the quarter ended March 31, 2017 (Report), as filed with the Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company for the periods presented therein.

Date: May 11, 2017

/s/ Thomas Soloway
Thomas Soloway
Chief Financial Officer
(Principal Financial Officer*)*