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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 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: 001-37693

AveXis, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**90-1038273**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

2275 Half Day Rd, Suite 200
Bannockburn, Illinois 60015
(Address of principal executive offices, including zip code)

(847) 572-8280
(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 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐		Accelerated filer ☐
Non-accelerated filer	☒	(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 Exchange Act). Yes ☐ No ☒

As of May 11, 2017, there were 27,794,558 shares of the registrant's common stock outstanding.

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AveXis, Inc.

Quarterly Report on Form 10-Q

For the Quarter Ended March 31, 2017

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PART 1. FINANCIAL INFORMATION

Item 1. Financial Statements

AveXis, Inc.

Condensed Consolidated Balance Sheets
(unaudited)

	March 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 204,453,779	$ 240,429,839
Prepaid expenses and other current assets	7,269,779	4,750,469
Total current assets	211,723,558	245,180,308
Property and equipment, net	33,682,337	24,200,582
Other long-term assets	1,836,157	1,194,541
Total assets	$ 247,242,052	$ 270,575,431
Liabilities, redeemable common stock and stockholders' equity		
Current liabilities:		
Accounts payable	$ 11,789,322	$ 3,197,274
Accrued expenses	8,625,514	16,794,003
Accrued indemnification obligation	4,519,700	4,452,500
Total current liabilities	24,934,536	24,443,777
Other long-term liabilities	596,037	—
Total liabilities	$ 25,530,573	$ 24,443,777
Common stock; par value $0.0001 per share, 100,000,000 shares authorized; 27,743,174 shares issued and outstanding at March 31, 2017; 27,700,054 shares issued and outstanding at December 31, 2016	2,774	2,770
Additional paid-in capital	392,989,983	387,691,208
Accumulated deficit	(171,281,278)	(141,562,324)
Total stockholders' equity	$ 221,711,479	$ 246,131,654
Total liabilities, redeemable common stock and stockholders' equity	$ 247,242,052	$ 270,575,431

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

AveXis, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)

	Three Months Ended March 31,	
	2017	2016
Revenue	$ —	$ —
Operating expenses:		
General and administrative	9,637,807	4,823,913
Research and development	20,327,087	16,065,089
Total operating expenses	29,964,894	20,889,002
Loss from operations	(29,964,894)	(20,889,002)
Interest income	245,940	52,895
Net loss and comprehensive loss	$ (29,718,954)	$ (20,836,107)
Basic and diluted net loss per common share	$ (1.07)	$ (1.24)
Weighted-average basic and diluted common shares outstanding	27,733,701	16,774,718

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

AveXis, Inc.

Condensed Consolidated Statements of Cash Flows
(unaudited)

	Three Months Ended March 31,	
	2017	2016
Cash flows from operating activities		
Net loss	$ (29,718,954)	$ (20,836,107)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	244,137	11,045
Stock-based compensation	5,083,878	13,543,547
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(2,532,858)	(420,180)
Other long-term assets	(641,616)	(731,663)
Accounts payable	6,525,937	580,688
Accrued expenses	(7,154,442)	169,971
Accrued indemnification obligation	67,200	—
Net cash used in operating activities	(28,126,718)	(7,682,699)
Cash flows from investing activities		
Capital expenditures	(8,064,242)	(3,659,718)
Net cash used in investing activities	(8,064,242)	(3,659,718)
Cash flows from financing activities		
Proceeds from issuance of common stock, net of issuance costs	—	98,169,703
Payments of deferred offering costs	—	(883,713)
Proceeds from exercise of stock options	214,900	—
Proceeds from exercise of stock warrants	—	42,000
Net cash provided by financing activities	214,900	97,327,990
Net (decrease) increase in cash and cash equivalents	(35,976,060)	85,985,573
Cash and cash equivalents, beginning of period	240,429,839	62,251,860
Cash and cash equivalents, end of period	$ 204,453,779	$ 148,237,433
Supplemental cash flow information:		
Purchases of property and equipment in accounts payable and accrued liabilities	$ 5,841,793	$ 2,060,392
Deferred offering costs recorded in accounts payable and accrued liabilities	$ —	$ 290,848

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

AveXis, Inc.

Notes to Condensed Consolidated Financial Statements
(unaudited)

1. Background

AveXis, Inc. was formed on March 8, 2010 in the state of Delaware as Biolife Cell Bank, LLC. In January 2012, the Company converted from a limited liability company to a corporation, Biolife Cell Bank, Inc. In January 2014, the Company amended and restated its Certificate of Incorporation to change its name to AveXis, Inc. ("AveXis" or "the Company").

The Company is a clinical-stage gene therapy company dedicated to developing and commercializing gene therapy treatments for patients suffering from rare and life-threatening neurological genetic diseases. The Company's initial product candidate, AVXS-101, is a gene therapy product candidate currently in a Phase 1 clinical trial for the treatment of spinal muscular atrophy, ("SMA"), Type 1, the leading genetic cause of infant mortality.

Initial Public Offering

On February 10, 2016, the Company completed an initial public offering ("IPO"), which resulted in the issuance and sale of 4,750,000 shares of its common stock at a public offering price of $20.00 per share, resulting in net proceeds of approximately $88,350,000 after deducting underwriting discounts. Upon the closing of the IPO, the 3,278,938 shares of Class B-1 preferred stock, 326,557 shares of Class B-2 preferred stock, 2,365,020 shares of Class C preferred stock and 3,105,000 of Class D preferred stock were automatically converted into shares of the Company's common stock.

On March 3, 2016, the underwriters of the Company's IPO exercised their over-allotment option to purchase an additional 527,941 shares of the Company's common stock at the initial public offering price of $20.00 per share, less underwriting discounts and commissions, resulting in additional net proceeds of approximately $9,800,000.

Underwritten Public Offering

On September 13, 2016, the Company completed an underwritten public offering (the "Follow-On Offering") of 4,887,500 shares of its common stock, 4,597,645 shares of which were issued and sold by the Company, including the exercise in full by the underwriters of their option to purchase 637,500 shares from the Company, and 289,855 shares of which were sold by PBM Capital Investments, LLC ("PBM"), an existing stockholder of the Company, each at a public offering price of $34.50 per share. After deducting the underwriting discounts and commissions, the net proceeds to the Company were approximately $149.1 million. The Company did not receive proceeds from the sale of the common stock by PBM.

2. Summary of Significant Accounting Policies

Basis of Presentation

The condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") for interim financial reporting and the rules and regulations of the U.S. Securities and Exchange Commission ("SEC") and, therefore, do not include all information and footnote disclosures normally included in the annual consolidated financial statements. In the opinion of management, the unaudited condensed consolidated financial statements reflect all adjustments of a normal recurring nature that are necessary for a fair statement of the results for the interim periods presented. Interim results are not necessarily indicative of results for a full year. The December 31, 2016 condensed consolidated balance sheet data contained within this Form 10-Q was derived from audited consolidated financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 ("Annual Report on Form 10-K"), but does not include all disclosures required by GAAP.

Significant Accounting Policies

There have been no material changes in the Company's significant accounting policies as of and for the three months ended March 31, 2017, as compared with the significant accounting policies described in the Company's Annual Report on Form 10-K.

Use of Estimates

The preparation of the condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and reported amounts of revenues, if any, and expenses during the reporting period. Actual results could differ from those estimates. In the Company's opinion, the accompanying unaudited condensed consolidated financial statements include all normal recurring adjustments necessary for a fair presentation of the Company's financial position, results of operations and cash flows for the interim periods presented.

Recent Accounting Pronouncements

In May 2014, the FASB and the International Accounting Standards Board (IASB) issued Accounting Standard Update ("ASU") No. 2014-09, Revenue from Contracts with Customers (Topic 606). The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. In doing so, companies will need to use more judgment and make more estimates than under previous guidance. Early adoption is permitted after December 15, 2016, and the standard is effective for public entities for annual reporting periods beginning after December 15, 2017 and interim periods therein. The Company has not recognized revenue through March 31, 2017. The Company will not early adopt this standard and will evaluate the adoption of Topic 606 when the Company begins to recognize revenue and determine the effects it may have on the Company's consolidated financial statements.

In November 2015, the FASB issued ASU No. 2015-17, Balance Sheet Classification of Deferred Taxes ("ASU 2015-17"). To simplify presentation, the new guidance requires that all deferred tax assets and liabilities, along with any related valuation allowance, be classified as noncurrent on the balance sheet. As a result, each jurisdiction will now only have one net noncurrent deferred tax asset or liability. ASU 2015-17 is effective for public business entities in fiscal years beginning after December 15, 2016, including interim periods within those years. Early adoption is permitted for all entities as of the beginning of an interim or annual reporting period. The Company is evaluating the adoption of ASU 2015-17, but has not determined the effects it may have on the Company's consolidated financial statements.

In February 2016, the FASB issued Accounting Standards Codification ("ASC") No. 2016-02, Leases ("ASC 842"). The guidance requires lessees to recognize almost all leases on their balance sheet as a right-of-use asset and a lease liability. For income statement purposes, the FASB retained a dual model, requiring leases to be classified as either operating or finance. Lessor accounting is similar to the current model, but updated to align with certain changes to the lessee model and the new revenue recognition standard. Existing sale-leaseback guidance, including guidance for real estate, is replaced with a new model applicable to both lessees and lessors. ASC 842 is effective for fiscal years beginning after December 15, 2018. The Company is evaluating the adoption of ASC 842, but has not determined the effects it may have on the Company's consolidated financial statements.

In August 2016, the FASB issued Accounting Standards Update, or ASU, 2016-15, Statement of Cash Flows (Topic 230). ASU 2016-15 is intended to reduce the diversity in practice regarding how certain transactions are classified within the statement of cash flows. ASU 2016-15 is effective for public business entities for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted with retrospective application. The Company is evaluating the adoption of ASU 2016-15, but has not determined the effects it may have on the Company's consolidated financial statements.

3. Property and Equipment, Net

Property and equipment, net, consists of the following:

	March 31, 2017	December 31, 2016
Office furniture and equipment	$ 4,259,838	$ 337,753
Leasehold improvements	4,457,609	777,456
Construction in progress	25,285,482	23,161,828
Property and equipment, gross	34,002,929	24,277,037
Less: accumulated depreciation	(320,592)	(76,455)
Property and equipment, net	$ 33,682,337	$ 24,200,582

Depreciation expense was $244,137 and $11,045 for the three months ended March 31, 2017 and 2016, respectively.

Construction in progress increased by $2,123,654 for the three months ended March 31, 2017.

4. Accrued Expenses

Accrued expenses consist of the following:

	March 31, 2017	December 31, 2016
Accrued manufacturing development costs	$ 3,052,803	$ 7,167,430
Accrued construction in progress	1,819,086	2,978,498
Accrued payroll, bonus and deferred compensation	956,323	3,841,171
Accrued clinical trial costs	874,758	388,935
Accrued professional fees	571,985	382,874
Accrued consulting	425,329	1,204,601
Accrued business taxes	364,381	226,044
Deferred rent	266,741	97,362
Accrued severance	15,527	167,065
Other	278,581	340,023
Accrued expenses	$ 8,625,514	$ 16,794,003

5. Accrued Indemnification Obligation

In January 2014, the Company issued 2,334,391 shares of restricted common stock to a member of its Board of Directors (Dr. Brian Kaspar, see Note 6) pursuant to a consulting agreement for scientific advisory services to be performed by the director on behalf of the Company. In connection with the restricted stock purchase agreement, the Company agreed to indemnify Dr. Kaspar for any taxes, interest, fines, penalties or other costs and expenses that Dr. Kaspar may incur in the future should the Internal Revenue Service succeed in a tax determination that the stock price paid by Dr. Kaspar (which was par value) was lower than the fair market value of the stock on the date of grant. The indemnification term is in effect for six years after the due date of the tax return for the year in which the stock was issued. In January 2016, the Company entered into an employment agreement with Dr. Kaspar.

In connection with the preparation of the Company's audited consolidated financial statements for the year ended December 31, 2014, the Company determined that the per share fair value of the Company's common stock on January 28, 2014, the grant date, was $1.51.

As a result, the Company issued Dr. Kaspar an amended Form 1099 for the 2014 tax year reflecting an aggregate fair value of the restricted stock grant of $3,535,419. Due to the indemnity obligation contained in Dr. Kaspar's restricted stock purchase agreement, the Company will ultimately be required to reimburse Dr. Kaspar for the taxes he will pay following the amendment of Dr. Kaspar's 2014 personal income tax return. As a result, the Company has concluded that payment of such indemnity is probable as of December 31, 2016 and March 31, 2017.

Additionally, the Company intends to gross-up such indemnification payment for the tax that will be payable by Dr. Kaspar on the indemnity payment.

As a result, the Company has accrued balances of $4,519,700 and $4,452,500 at March 31, 2017 and December 31, 2016, respectively, representing the Company's best estimate of the ultimate tax indemnification and gross-up payment to be made to Dr. Kaspar. The increase in the accrued indemnification obligation was due to the accrual of additional interest through March 31, 2017. The Company expects to pay this entire amount in 2017.

6. Stock-Based Compensation

2014 Stock Plan (the "2014 Plan") and 2016 Equity Incentive Plan (the "2016 Plan")

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

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2016	2,577,111	$ 22.01	8.79	$ 66,466,245
Granted	201,250	$ 64.44		
Exercised	(43,120)	$ 4.98		
Cancelled or forfeited	(1,100)	$ 34.90		
Outstanding at March 31, 2017	2,734,141	$ 25.40	8.66	$ 138,600,297
Exercisable at March 31, 2017	828,183	$ 16.28	8.29	$ 49,483,455
Exercisable and expected to vest at March 31, 2017	2,734,141	$ 25.40	8.66	$ 138,600,297

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in-the-money at March 31, 2017 and December 31, 2016.

For the three months ended March 31, 2017 and 2016, the total number of stock options exercised was 43,120 and 0, respectively, resulting in total proceeds of $214,900 and $0, respectively.

As of March 31, 2017 and December 31, 2016, there was $25,179,386 and $20,583,606, respectively, of unrecognized stock-based compensation expense related to stock option awards that is expected to be recognized over a weighted-average period of 1.4 and 1.4 years, respectively.

The Company has recorded total stock-based compensation expense related to the issuance of stock option awards under the Plans in the consolidated statements of operations and comprehensive loss as follows:

	Three Months Ended March 31,	
	2017	2016
Research and development	$ 1,977,864	$ 1,089,580
General and administrative	2,764,557	2,083,205
	$ 4,742,421	$ 3,172,785

Stock Options Granted to Employees

The weighted-average grant date fair value of options granted during the three months ended March 31, 2017 and 2016 was $47.20 and $11.16, respectively, on the date of grant using the Black-Scholes option-pricing model with the following weighted-average assumptions:

	Three Months Ended March 31,	
	2017	2016
Expected volatility	86.88%	90.00%
Risk-free interest rate	1.94%	1.57%
Expected terms (in years)	6.08	6.08
Expected dividend yield	0.00%	0.00%

Options generally expire ten years following the date of grant. Options typically vest over a period of two to four years, but vesting provisions can vary by award based on the discretion of the Board of Directors. Certain awards issued by the Company include performance conditions that must be achieved in order for vesting to occur. Options to purchase common stock carry an exercise price equal to the estimated fair value of the Company's common stock on the date of grant. Options to purchase shares of the Company's common stock may be exercised by payment of the exercise price in cash, by the delivery of previously acquired shares of common stock having a fair value equal to the exercise price payable or the withholding of common shares equal to the fair value of the aggregate exercise price. Upon the termination of service of a holder of stock options awarded under the Plan, all unvested options are immediately forfeited and vested options may be exercised within three months of termination.

Service-Based Restricted Stock Units

As of March 31, 2017 and December 31, 2016, there were 56,900 and 57,500, respectively, outstanding service-based restricted stock units granted to employees. During the three months ended March 31, 2017, 15,233 service-based restricted stock units vested and remained outstanding, 600 were forfeited and cancelled and the Company recognized stock-based compensation expense of $341,457 during the three months ended March 31, 2017. No service-based restricted stock units were issued and outstanding as of March 31, 2016. At March 31, 2017 and 2016, there was $885,716 and $0, respectively, of unrecognized compensation cost related to service-

based unvested restricted stock units that will be recognized as expense over a weighted-average period of 1.3 years. A summary of the status of the Company's restricted stock units at March 31, 2017 and of changes in service-based restricted stock units outstanding under the 2016 Plan for the three months ended March 31, 2017 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value Per Share
Outstanding at December 31, 2016	57,500	$ 34.90
Granted	—	—
Forfeited and cancelled	(600)	34.90
Outstanding at March 31, 2017	56,900	$ 34.90

The Company granted restricted stock units with service-based vesting terms. The outstanding service-based restricted stock units vest over a period of three years. For awards that vest subject to the satisfaction of service requirements, compensation expense is measured based on the fair value of the RSUs on the date of grant and is recognized as expense on a straight-line basis, net of estimated forfeitures, over the requisite service period. All service-based restricted stock units issued vest over time as stipulated in the individual service-based restricted stock unit agreements.

Performance-Based Restricted Stock Units

On March 20, 2017, the Company granted to certain individuals a total of 49,332 performance-based restricted stock units. As of March 31, 2017, all 49,332 of these performance-based restricted stock units were outstanding, none had vested and the weighted average grant date fair value of all shares was $79.75 per share. There were no performance-based restricted stock units issued and outstanding during the first quarter ended March 31, 2016.

Restricted Stock Granted to Non-Employees

In January 2014, the Company issued 2,334,391 shares of restricted common stock to Dr. Brian Kaspar pursuant to a consulting agreement for scientific advisory services. Of these shares, 583,597 common shares were vested at the time of grant and the remaining restricted shares were scheduled to vest in the amount of 25% per year on the second, third and fourth anniversary of the grant date pursuant to a restricted stock purchase agreement, which became effective upon the effectiveness of the consulting agreement.

In January 2016, the Company entered into an employment agreement with Dr. Kaspar. Upon the effectiveness of the employment agreement, Dr. Kaspar's 1,750,794 unvested shares granted pursuant to the restricted stock purchase agreement vested in full. As a result of the vesting of the remainder of this award the Company recorded $10,370,762 of additional stock compensation expense for the year ended December 31, 2016.

Warrants Granted to Non-Employees

During the three month periods ended March 31, 2017, there were no warrants exercised and as a result no proceeds received by the Company. As of March 31, 2017, there were 305,775 common stock warrants vested and outstanding issued to non-employees with a weighted-average exercise price of $2.57.

7. Net Loss Per Common Share

Basic net loss per common share is computed using the weighted average number of common shares outstanding during the period. Diluted net loss per common share is computed using the sum of the weighted average number of common shares outstanding during the period and, if dilutive, the weighted average number of potential shares of common stock, including the assumed exercise of stock options, stock warrants and unvested restricted common stock. The Company applies the two-class method to calculate its basic and diluted net loss per share attributable to common stockholders, as its preferred stock and common stock are participating securities. The two-class method is an earnings allocation formula that treats a participating security as having rights to earnings that otherwise would have been available to common stockholders. However, the two-class method does not impact the net loss per share of common stock as the Company was in a net loss position for each of the periods presented and preferred stockholders do not participate in

losses. For the three months ended March 31, 2017 and 2016, the following potentially dilutive securities have been excluded from the computations of diluted weighted-average shares outstanding because the effect would be anti-dilutive:

	Three Months Ended March 31,	
	2017	2016
Stock options	2,734,141	2,087,617
Stock warrants	305,775	310,220
Unvested restricted stock units	41,667	—
Unvested performance-based restricted stock units	49,332	—
	3,130,915	2,397,837

Amounts in the table above reflect the common stock equivalents of the noted instruments.

The following table summarizes the calculation of the basic and diluted net loss per common share:

	Three Months Ended March 31,	
	2017	2016
Numerator:		
Net loss and comprehensive loss	$ (29,718,954)	$ (20,836,107)
Denominator:		
Weighted-average basic and diluted common shares	27,733,701	16,774,718
Basic and diluted net loss per common share	$ (1.07)	$ (1.24)

8. Commitments and Contingencies

Operating Leases

In March 2014, the Company entered into a lease agreement, which expired in April 2017, for approximately 2,418 square feet of office space in Dallas, Texas.

The Company leases a 15,668 square foot facility for its corporate headquarters in Bannockburn, Illinois, pursuant to a lease that expires in July 2024. The lease agreement provides for annual escalation in rent payments during the lease term. The Company is amortizing the escalation in rental payments on a straight-line basis over the term of the lease.

The Company also leases a 1,318 square foot facility in Columbus, Ohio for research and development activities, pursuant to a lease that expires in March 2019.

In March 2016, the Company entered into a lease agreement, which expires in August 2026, for approximately 48,529 square feet of warehouse and office space Libertyville, Illinois. A portion of the warehouse space was converted into manufacturing space. The lease agreement provides for annual escalation in rent payments during the lease term. The lease agreement provides the Company with a one-time right to terminate the lease effective as of the last day of the ninety-sixth full calendar month of the lease subject to a termination fee. The Company is amortizing the escalation in rental payments on a straight-line basis over the term of the lease.

Future minimum lease payments under all of the Company's leases are as follows:

Year ending December 31,	
2017	$ 400,870
2018	660,572
2019	674,850
2020	689,289
2021	703,894
Thereafter	2,514,993
Total	$ 5,644,468

Guarantees and Indemnifications

The Company has accrued $4,519,700 and $4,452,500 at March 31, 2017 and December 31, 2016, respectively, representing the Company's best estimate of the ultimate tax indemnification and gross-up payment to be made to Dr. Kaspar pursuant to a tax indemnification granted to Dr. Kaspar in connection with a restricted common stock grant (see Note 5).

Additionally, in the normal course of business, the Company has entered into agreements that contain a variety of representations and provide for general indemnification. The Company's exposure under these agreements is unknown because it involves claims that may be made against the Company in the future. To date, the Company has not paid any claims or been required to defend any action related to these indemnification obligations. As of March 31, 2017 and December 31, 2016, the Company did not have any material indemnification claims related to these agreements that were probable or reasonably possible and consequently has not recorded any related liabilities.

Litigation

On September 8, 2016, Sophia's Cure Foundation ("SCF"), a non-profit 501(c)(3) public charity, filed a complaint in U.S. District Court, Southern District of Ohio, naming as defendants Nationwide Children's Hospital ("NCH") and other entities affiliated with NCH, the Company and certain of the Company's present and former executives (the "Complaint"). According to the complaint, in 2012, SCF and Nationwide Children's Hospital Foundation ("NCH Foundation") entered into a donation agreement under which SCF provided NCH a gift of $550,000 to fund clinical work associated with the study of the product candidate that the Company now refers to as AVXS-101 for SMA Type 1 patients, and NCH Foundation agreed in such donation agreement to reference SCF as the "primary sponsor" of such clinical work in all publications issued by NCH Foundation. The complaint also alleges that NCH breached the donation agreement by not naming SCF as the sponsor of the investigational new drug application (the "IND") that it filed for AVXS-101. Additionally, the complaint alleges that the Company and the named Company executives tortiously interfered with SCF's rights under the donation agreement by assuming sponsorship of the IND under the NCH License. There is no contractual relationship between the Company and SCF. The complaint seeks, among other relief, monetary damages of $500 million and equitable relief, including taking steps to designate SCF as the sponsor of the IND. The Company filed a motion to dismiss this action on October 28, 2016. On December 5, 2016, SCF filed an amended complaint, asserting similar allegations against the Company as in the original complaint. The Company filed a motion to dismiss the amended complaint on December 19, 2016. On March 20, 2017, SCF filed an opposition brief to the Company's motion to dismiss, and on April 10, 2017, the Company filed a reply memorandum in support of its motion to dismiss. The Court has not yet ruled on the Company's motion to dismiss. A pre-trial conference was held on May 9, 2017. The Company believes that the complaint is without merit and intends to vigorously defend itself and its current executives from the allegations. The Company views the probability of loss in this matter to be remote.

Lawsuits may be asserted against the Company in the normal course of business. Based on information currently available, management believes that the disposition of any matters, including the matter involving SCF described above, will not have a material adverse effect on the financial position, results of operations or cash flows of the Company.

9. Taxes

Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, including its net operating losses. Based on its history of operating losses, the Company believes that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of March 31, 2017 and December 31, 2016.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes thereto included elsewhere in this Quarterly Report on Form 10-Q (this "Quarterly Report"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2016 (the "Annual Report"), our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Forward-Looking Statements

This Quarterly Report contains forward-looking statements, within the meaning of the Private Securities Litigation Reform Act of 1995, that involve substantial risks and uncertainties. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions intended to identify statements about the future. These statements speak only as of the date of this Quarterly Report and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements include, without limitation, statements about the following:

- the timing, progress and results of preclinical studies and clinical trials for AVXS-101 and any other product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing of and our ability to obtain and maintain regulatory approval of AVXS-101;
- the proposed clinical development pathway for AVXS-101, including the expected trial design for our proposed pivotal clinical trials, and the acceptability of the results of such trials for regulatory approval of AVXS-101 by the FDA or comparable foreign regulatory authorities;
- our expectations regarding timing for meetings with regulatory agencies;
- our expectations regarding the size of the patient populations for our product candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our ability to successfully commercialize AVXS-101;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our needs for or ability to obtain additional financing;
- our ability to identify, license and develop new product candidates;
- our ability to identify, recruit and retain key personnel;
- our and our licensors' ability to protect and enforce our intellectual property protection for AVXS-101, and the scope of such protection;
- our financial performance;
- the development of and projections relating to our competitors or our industry;
- our expectations about the outcome of litigation and controversies with third parties, including the lawsuit filed by Sophia's Cure Foundation;
- the impact of laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

You should refer to "Item 1A. Risk Factors" in our Annual Report, and the discussion in this Management's Discussion and Analysis of Financial Condition and Results of Operations, for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Quarterly Report will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. The forward-looking statements in this Quarterly Report represent our views as of the date of this Quarterly Report. We anticipate that subsequent events and developments may cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Quarterly Report.

You should read this Quarterly Report and the documents that we reference in this Quarterly Report and have filed as exhibits to this Quarterly Report completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

We are a clinical-stage gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from rare and life-threatening neurological genetic diseases. Our initial product candidate, AVXS-101, is our proprietary gene therapy product candidate currently in a Phase 1 clinical trial for the treatment of spinal muscular atrophy, or SMA, Type 1, the leading genetic cause of infant mortality. SMA Type 1 is a lethal genetic disorder characterized by motor neuron loss and associated muscle deterioration, resulting in mortality or the need for permanent ventilation support before the age of two for greater than 90% of patients. The survival motor neuron, or SMN, is a critical protein for normal motor neuron signaling and function. Patients with SMA Type 1 either carry a mutation in their *SMN1* gene or their *SMN1* genes have been deleted, which prevents them from producing adequate levels of functional SMN protein. AVXS-101 is designed to deliver a fully functional human *SMN* gene into the nuclei of motor neurons that then generates an increase in SMN protein levels and we believe this will result in improved motor neuron function and patient outcomes.

In our fully enrolled Phase 1 clinical trial, we treated 15 SMA Type 1 patients, divided into two dosing cohorts, and observed a favorable safety profile and that AVXS-101 is generally well-tolerated. As of January 20, 2017, the trial evaluation date for which we reported top-line results, all patients in the study have survived (in contrast to the independent, peer-reviewed natural history study for patients with SMA Type 1). Additionally, we have observed improved motor function, including the attainment of motor milestones such as the ability to sit unassisted, crawl, stand and walk that are essentially never seen among untreated patients suffering from SMA Type 1. The U.S. Food and Drug Administration, or FDA, and the European Medicines Agency, or EMA, have each granted AVXS-101 orphan drug designation for the treatment of SMA, and the FDA has granted AVXS-101 fast track designation for the treatment of SMA Type 1. The FDA granted breakthrough therapy designation for AVXS-101 for the treatment of SMA Type 1 in pediatric patients, and the EMA granted access into its PRIority MEdicines, or PRIME, program for AVXS-101 for the treatment of SMA Type 1. Pending the outcome of our Type B meeting with the FDA to discuss chemistry, manufacturing and controls, or CMC, held on May 1, 2017, we intend to initiate a pivotal trial of AVXS-101 for the treatment of SMA Type 1 in the United States during the first half of 2017 and in Europe during the second half of 2017, and we also intend to initiate a Phase 1 safety and dosing escalation study of AVXS-101 via intrathecal delivery in patients with SMA Type 2 in the second quarter of 2017. In addition to developing AVXS-101 to treat SMA Type 1, we plan to develop AVXS-101 to treat additional SMA types and develop other novel treatments for rare neurological genetic diseases.

Based on the top-line results of the trial reported at the primary analysis for efficacy as of January 20, 2017, no patient receiving the proposed therapeutic dose of AVXS-101 has experienced an event at 13.6 months. As of January 20, 2017, all patients have experienced improvement in motor function relative to their baseline measurement, as measured by The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders, or CHOP INTEND, a test developed to measure motor skills of patients with SMA Type 1. Mean increases from baseline in CHOP INTEND scores of 7.7 points in the low-dose cohort, or Cohort 1, and 24.7 points in the proposed therapeutic-dose cohort, or Cohort 2, were observed, reflecting improvement in motor function. At the most recent evaluations as of January 20, 2017, eleven out of 12 patients (92%), ten out of 12 patients (83%) and two out of 12 patients (17%) in Cohort 2 have achieved CHOP INTEND scores of at least 40 points, 50 points or 60 points, respectively. Two patients achieved the maximum CHOP INTEND score of 64. A CHOP INTEND score of 60 points is in a range considered to be normal. Based on our observations to date, we believe that increases in CHOP INTEND motor assessments appear to be age-, baseline CHOP INTEND score- and dose-dependent.

The natural history of SMA Type 1 is marked by the inability to achieve or maintain developmental motor milestones. In contrast, as of January 20, 2017, patients in the proposed therapeutic-dose cohort consistently achieved and maintained key developmental motor milestones. As of January 20, 2017, eleven of 12 patients (92%) in Cohort 2 achieved head control, nine of 12 patients (75%) could roll a minimum of 180 degrees from back to both left and right, and eleven of 12 patients (92%) could sit with assistance. For the end-of-study assessment, we evaluated three validated and well-established measures of sitting unassisted for periods of increasing duration. Nine of 12 patients (75%) in Cohort 2 could sit unassisted for at least five seconds, seven of 12 patients (58%) could sit unassisted for at least 10 seconds and five of 12 patients (42%) could sit unassisted for 30 seconds or more. Two patients in Cohort 2 could walk independently, and each had achieved earlier and important developmental milestones such as standing with support, standing alone and walking with support. These milestone achievements were assessed and adjudicated by an independent third party reviewer using video evidence.

On April 25, 2017, we reported that three patients in Cohort 2 achieved additional sitting milestones after the January 20, 2017 evaluation date. Ten of 12 patients (83%) in Cohort 2 could sit unassisted for at least five seconds, nine of 12 (75%) patients could sit unassisted for at least 10 seconds and eight of 12 patients (67%) could sit unassisted for 30 seconds or more in the post-January 20

analysis. The post-January 20 developmental milestones were confirmed internally by video. Detailed Cohort 2 motor milestone data is included in the chart below.

		Motor Milestone Achievement as of January 20, 2017*							
							Sitting Unassisted		
Cohort 2 2.0e14 vg/kg	Age at Gene Transfer (months)	Brings Hand to Mouth	Head Control	Partial Roll(a)	Roll(b)	Sitting with Assistance	≥5 seconds(c)	≥10 seconds(d)	≥30 seconds(e)
E.04	6	X	X	X	X	X	X		
E.05	4	X	X	X	X	X	X	X	X
E.06	2	X	X	X	X	X	X	X	X
E.07	4	X	X	X	X	X	X	X	
E.08	8	X							
E.09	5	X	X	X	X	X	X	X	X
E.10	1	X	X	X	X	X	X	X	X
E.11	2	X	X	X	X	X	X	O	O
E.12	3	X	X	X	X	X	X	X	X
E.13	1	X	X			X	X	X	O
E.14	4	X	X	X	X	X	O	O	O
E.15	2	X	X			X			

(a) Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180º from back in only one direction.
(b) Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180º from back to both left and right.
(c) Sitting unassisted for ≥5 seconds is in accordance with the criteria of item 22 in the Bayley Scales of Infant and Toddler Development – gross motor subtest and surpasses the three-second count used as a basis for sitting (test item 1) in the Hammersmith Functional Motor Scale – Expanded for SMA (HFMSE).
(d) Sitting unassisted for ≥10 seconds is in accordance with the criteria in the World Health Organization – MultiCentre Growth Reference Study.
(e) Sitting unassisted for ≥30 seconds defines functional independent sitting and is in accordance with the criteria of item 26 in the Bayley Scales of Infant and Toddler Development – gross motor subtest.
*Milestone achievements as of January 20, 2017, except those indicated by an O, which were achieved after the January 20, 2017 cutoff.

According to natural history, nearly all Type 1 patients require nutritional and respiratory support by 12 months of age, and most SMA Type 1 patients are not able to swallow or speak effectively. As of January 20, 2017, six of 7 (86%) patients in Cohort 2 that did not require feeding support before treatment continued without feeding support after treatment, and seven of 10 (70%) patients that did not require bi-level positive airway pressure ("BiPAP") support before treatment continued without any BiPAP after treatment. Further, as of January 20, 2017, eleven of 12 (92%) patients in Cohort 2 were fed orally, and six of 12 (50%) patients were exclusively fed orally. Eight of 12 (67%) patients in Cohort 2 were able to speak.

To date, we have funded our research and development and operating activities primarily through equity financings, including $95.3 million and $148.3 million of net proceeds from our initial public offering and our follow-on public offering, respectively, $80.5 million of aggregate net proceeds from private placements of stock prior to our initial public offering, and $1.0 million in proceeds from the issuance of convertible debt. We have not generated any revenue from sales of gene therapy products to date. We have incurred significant annual net operating losses in every year since our inception and expect to continue to incur net operating losses for the foreseeable future. Our net operating losses were $38.5 million and $83.0 million for the years ended December 31, 2015 and 2016, respectively. As of March 31, 2017, we had an accumulated deficit of $171.3 million. We expect to continue to incur significant expenses and increasing operating losses for the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly if and as we continue to develop and conduct clinical trials with respect to AVXS-101; maintain, expand and protect our intellectual property portfolio; establish a commercial infrastructure to support the manufacture, marketing and sale of AVXS-101 if it receives regulatory approval; and hire additional personnel, such as clinical, regulatory, manufacturing, quality control and scientific personnel. In addition, we expect to incur additional costs associated with operating as a public company.

Licensing Agreements

To date, we have entered into three license agreements relating to the development of AVXS-101.

Nationwide Children's Hospital

In October 2013, we entered into an exclusive, worldwide license agreement with Nationwide Children's Hospital, or NCH, under certain patent applications, and a non-exclusive license under certain technical information, for the use of its scAAV9 technology for the treatment of SMA, of all types, or the NCH License. In January 2016, we amended and restated the NCH License in its entirety. Under the NCH License, we initially issued NCH and The Ohio State University, or OSU, 331,053 shares of common stock. Until May 2015, when we had reached a market capitalization of $100 million, we were obligated to issue additional shares to NCH and OSU from time to time to maintain a 3% ownership of the company on a fully-diluted basis. We issued an aggregate of 124,990 additional shares of common stock between October 2013 and May 2015 pursuant to these anti-dilution obligations. With certain exceptions, we are required to make up to $0.1 million in development milestone based payments to NCH. In addition, we are responsible for all clinical trial costs that are not covered by grants or certain other sources.

Following the first commercial sale of a NCH licensed product we must begin paying NCH an aggregate low-single digit royalty on net sales of any products covered by the NCH License, subject to reduction in specified circumstances and annual minimum royalties that increase over time. In addition, we must pay NCH a portion of sublicensing revenue received from our sublicense of the licensed technology at percentages between low-double digits and low-teens.

On November 6, 2015, the FDA approved our sponsorship of the IND and the transfer of the associated regulatory filing from NCH.

REGENXBIO Inc.

In March 2014, we entered into an exclusive license agreement with ReGenX Biosciences, LLC, or ReGenX, predecessor to REGENXBIO Inc., under certain patent rights owned by the Trustees of the University of Pennsylvania and licensed to ReGenX, for the development and commercialization of products to treat spinal muscular atrophy by *in vivo* gene therapy using AAV9, or the ReGenX License. Under the ReGenX License, we paid ReGenX an initial licensing fee of $2.0 million. We are also required to pay ReGenX: annual maintenance fees, up to $12.25 million in milestone fees for all products covered by the ReGenX License, or ReGenX licensed products; mid-single to low-double digit royalty percentages on net sales of ReGenX licensed products, subject to reduction in specified circumstances; and lower mid-double digit percentages of any sublicense fees we receive from sublicensees for the licensed patent rights. As of March 31, 2017, we have paid $2.4 million under the ReGenX License, which includes $0.3 million in aggregate milestone payments.

Asklepios Biopharmaceutical, Inc.

In May 2015, we entered into a non-exclusive, worldwide license agreement with Asklepios Biopharmaceutical, Inc., or AskBio, under certain patents and patent applications, for the use of AskBio's self-complementary AAV genome technology for the treatment of SMA in humans, or the AskBio License. Under the AskBio License, we paid AskBio a one-time upfront license fee of $1 million, payable across stipulated milestones. We are also required to pay ongoing annual maintenance fees, up to a total of $0.6 million in clinical development milestone payments and up to a total of $9 million in commercial milestone payments. Under the terms of the AskBio License, we are required to pay AskBio annual tiered royalties on net sales of any products covered by the AskBio License, on a country-by-country basis, starting at percentages in the low-single digits and increasing to mid-single digits. These royalty rates are subject to potential reduction in specified circumstances, including, in the event we exercise our option to make a specified one-time royalty option fee payment to AskBio. We must also pay AskBio a low-double digit percentage of all consideration we receive from any sublicense of the licensed technology. Through March 31, 2017, we have paid the $1.0 million upfront license fee owed under the AskBio License.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from the commercial sale of gene therapy products, and we do not expect to generate substantial revenue for at least the next few years. In the future, we will seek to generate revenue primarily from product sales and, potentially, collaborations with strategic partners.

Operating Expenses

We classify our operating expenses into two categories: research and development and general and administrative expenses. Personnel costs including salaries, benefits, bonuses and stock-based compensation expense, comprise a significant component of each of these expense categories. We allocate expenses associated with personnel costs based on the nature of work associated with these resources.

Research and Development Costs

Research and development expense consists of expenses incurred while performing research and development activities to discover and develop potential gene therapy treatments. This includes conducting preclinical studies and clinical trials, investment in our manufacturing facility, manufacturing equipment and manufacturing development efforts and activities related to regulatory filings for product candidates. We recognize research and development expenses as they are incurred. Up-front fees incurred in obtaining technology licenses for research and development activities are expensed as incurred if the technology licensed has no alternative future use. Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits and any employee stock-based compensation, for our scientific personnel performing research and development activities;
- stock-based compensation expense related to restricted common stock grants and stock warrant issuances to consultants assisting us in the research and development of our product candidate;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- costs related to developing processes and analytical methods to manufacture and test product from a significant number of small scale and full scale engineering runs;
- fees paid to consultants and other third parties who support our product candidate development;
- other costs incurred in seeking regulatory approval of our product candidates; and
- allocated facility-related costs and overhead.

We typically utilize our employee, consultant and infrastructure resources across our development programs. To date, substantially all of our research and development expenses have been associated with AVXS-101.

We plan to increase our research and development expense for the foreseeable future as we continue our effort to develop and manufacture AVXS-101 and to advance the development of future product candidates, subject to the availability of sufficient funding.

The successful development of product candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or costs required to complete the remaining development of AVXS-101 or any future product candidates. This is due to the numerous risks and uncertainties associated with the development of product candidates.

General and Administrative Expense

General and administrative expense consists primarily of salaries and personnel-related costs, including employee benefits and any stock-based compensation, for employees performing functions other than research and development. This includes personnel in executive, business operations, finance and administrative support functions. Other general and administrative expenses include facility-related costs not otherwise allocated to research and development expense, professional fees for auditing, tax and legal services, expenses associated with obtaining and maintaining patents and costs of our information systems.

We expect that our general and administrative expense will increase as we continue to operate as a public reporting company and continue to develop and potentially commercialize AVXS-101 and our future product candidates. We believe that these increases likely will include increased costs for director and officer liability insurance, costs related to the hiring of additional personnel and increased fees for outside consultants, lawyers and accountants. We also expect to incur increased costs to comply with corporate governance, internal controls, investor relations, disclosure and similar requirements applicable to public reporting companies.

Interest Income

Interest income primarily consists of any interest income earned on our cash and cash equivalents.

Income Taxes

To date, we have not been required to pay U.S. federal or state income taxes because we have not generated taxable income.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our consolidated 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 our consolidated financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates and judgments on an ongoing basis. We base our estimates on 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.

While our significant accounting policies are more fully described in our consolidated financial statements appearing in our Annual Report, we believe that the following accounting policies are critical to the process of making significant judgments and estimates in the preparation of our consolidated financial statements and understanding and evaluating our reported financial results. There have been no material changes in our significant accounting policies as of and for the three months ended March 31, 2017.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development and manufacturing activities, including salaries, employee stock-based compensation and benefits, stock-based compensation expense related to restricted common stock grants and stock warrant issuances to consultants assisting us in the research and development of our product candidates, third-party license fees, and external costs of outside vendors engaged to conduct preclinical development activities and clinical trials.

Upfront and milestone payments made to third parties who perform research and development services on our behalf are expensed as services are rendered or when they no longer have alternative future use. Costs incurred in obtaining technology licenses are charged to research and development expense as acquired in-process research and development if the technology licensed has not reached technological feasibility and has no alternative future use.

Stock-Based Compensation

We account for stock-based compensation awards in accordance with FASB ASC Topic 718, Compensation—Stock Compensation, or ASC 718. ASC 718 requires all stock-based payments to employees, including grants of employee stock options, to be recognized in the statement of operations based on their fair values. Our stock-based compensation awards have historically consisted of stock options, restricted stock units and shares of restricted stock. In addition, certain other equity transactions involving our directors and executive officers have had a compensatory element, which we also account for as stock-based awards.

Our stock-based awards are subject to service and, in some cases, performance-based vesting conditions. Compensation expense related to awards to employees with only service-based vesting conditions is recognized on a straight-line basis over the requisite service vesting portion of the award as if the award was, in substance, multiple awards, or the Graded Vesting Attribution Method, based on the estimated grant date fair value for each separately vesting tranche. Compensation expense related to awards to non-employees with only service-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, using the Graded Vesting Attribution Method.

Compensation expense related to awards to employees with only performance-based vesting conditions is recognized based on the estimated grant date fair value of the award over the requisite service period using the Graded Vesting Attribution Method to the extent achievement of the performance condition is probable. Compensation expense related to awards to non-employees with only performance-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the requisite service period using the Graded Vesting Attribution Method to the extent achievement of the performance condition is probable.

We calculate the fair value of stock options using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of subjective assumptions, including the expected volatility of our common stock, the assumed dividend yield, the

expected term of our stock options and the risk-free interest rate for a period that approximates the expected term of our stock options. Additionally, for grants prior to our initial public offering, we also estimated the fair value of the underlying common stock on the date of grant. In applying these assumptions, we considered the following factors:

- We do not have sufficient history to estimate the volatility of our common stock. We calculate expected volatility based on reported data for selected similar publicly traded companies for which the historical information is available. We plan to continue to use the guideline peer group volatility information until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants;
- The assumed dividend yield of zero is based on our expectation of not paying dividends for the foreseeable future;
- Our estimates of expected term used in the Black-Scholes option pricing model were based on the estimated time from the grant date to the date of exercise;
- We determine the risk-free interest rate by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant; and
- We estimate forfeitures based on our historical analysis of actual stock option forfeitures. To date, we have had minimal forfeitures, accordingly, we have assumed no forfeiture rate.

Stock-based awards issued to non-employees, consisting of stock warrants and restricted common shares, are accounted for using the fair value method in accordance with ASC 505-50, Equity-Based Payments to Non-Employees. These stock warrants and restricted common shares have been granted in exchange for consulting services to be rendered, and vest according to certain service or performance conditions. In accordance with authoritative guidance, the fair value of non-employee stock-based awards is estimated on the date of grant, and subsequently revalued at each reporting period until the award vests or a measurement date has occurred using the Black-Scholes option-pricing model.

The following summarizes the assumptions we used to estimate the fair value of stock options that we granted for the period indicated:

	Three Months Ended March 31,	
	2017	2016
Expected volatility	86.88%	90.00%
Risk-free interest rate	1.94%	1.57%
Expected terms (in years)	6.08	6.08
Expected dividend yield	0.00%	0.00%

In addition to stock options and stock warrants, we have also incurred stock-based compensation expense in connection with other equity transactions involving employees and directors.

In January 2014, we issued 2,334,391 shares of restricted common stock to Dr. Brian Kaspar, a director of the company, pursuant to a consulting agreement for scientific advisory services to be performed by Dr. Kaspar. Of these shares, 583,597 shares were vested at the time of grant. The remaining shares vested in full on January 1, 2016, upon the effectiveness of Dr. Kaspar's employment agreement. The non-vested shares under the award were revalued each period until they vested. Compensation expense is recorded utilizing the Graded Vesting Attribution Method. The award had a grant date fair value of $3.5 million. We recorded compensation expense of $19.3 million for the year ended December 31, 2015 related to this award. As a result of the vesting in full of the 1,750,794 unvested shares in January 2016, we recorded $10.4 million in research and development expense related to the grant for the year ended December 31, 2016.

The table below summarizes the stock-based compensation expense recognized in our statements of operations by type:

	Three Months Ended March 31,	
	2017	2016
Employee stock options	$ 4,742,421	$ 13,543,547
Employee restricted stock units	341,457	—
Employee performance restricted stock units	—	—
	$ 5,083,878	$ 13,543,547

The table below summarizes the stock-based compensation expense recognized in our statements of operations by classification:

	Three Months Ended March 31,	
	2017	2016
Research and development	$ 2,280,294	$ 11,460,342
General and administrative	2,803,584	2,083,205
	$ 5,083,878	$ 13,543,547

Common Stock Valuation Methodology

Prior to our initial public offering, we were required to make significant assumptions and estimates to determine the fair value of common stock underlying stock option awards at the grant date of the award. Valuation estimates were prepared by management in accordance with the framework of the American Institute of Certified Public Accountants Practice Guide, with the assistance of independent third party valuations, and approved by our Board of Directors. The valuations of our common stock were based on a number of objective and subjective factors, including external market conditions affecting our industry sector, the prices at which we sold shares of our common and preferred stock, and the likelihood of achieving a liquidity event such as an initial public offering.

Tax Indemnity

In connection with the restricted stock purchase agreement with Dr. Kaspar, or RSPA, described in "—Stock-Based Compensation," we are obligated to indemnify Dr. Kaspar against certain adverse tax events with respect to the shares of our common stock he purchased under the RSPA, and such obligation survived the termination of the RSPA effective January 1, 2016. Dr. Kaspar purchased the shares at a price of $0.00007 per share, which was the par value of the shares. Based on our estimate of the fair market value per share of our common stock as of the date of the RSPA of $1.51 per share, Dr. Kaspar purchased these shares at a discount of $1.514 per share. Therefore, we estimate that we are contractually obligated to indemnify Dr. Kaspar for the tax he owes on the imputed income of $3.5 million, based on the difference between the fair market value of the restricted share grant and the purchase price paid. We estimate our total indemnity obligation will be approximately $4.5 million, including gross-up, interest and penalties. As a result, we accrued approximately $4.5 million at March 31, 2017, and approximately $4.5 million at December 31, 2016, representing our best estimate of the ultimate gross-up and tax indemnification that will be payable to Dr. Kaspar. We expect to pay this amount in 2017.

Utilization of Net Operating Loss Carryforwards

As of December 31, 2015 and 2016, we had federal net operating loss, or NOL, carryforwards of $16.5 million and $34.6 million, respectively, which carryforwards may be available to offset future income tax liabilities and begin to expire in 2032.

Under the provisions of the Internal Revenue Code of 1986, as amended, or the Code, the net operating loss carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50 percentage points, as defined under Sections 382 and 383 of the Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on our value immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. We have completed several financings since our inception, which may have resulted in a change in control as defined by Sections 382 and 383 of the Code, or could result in a change in control in the future. If we experience such an ownership change in connection with our previous offerings, including our initial public offering, our subsequent public offering, or future offerings, the tax benefits related to the net operating loss carryforwards may be further limited or lost. During 2016, we performed a detailed analysis of historical and/or current Section 382 ownership changes that may limit the utilization of net operating loss carryovers. From their entire federal net operating loss carryforward of $34.6 million, as of December 31, 2016, approximately $25.6 million is available for immediate use based on the Section 382 analysis performed. The remaining net operating loss carryforward will become available by the end of 2018 unless there is a greater than 50% change in ownership of us. As of December 31, 2016 and 2015, we had state net operating loss carryforwards of $25.9 million and $7.7 million, respectively, which begin to expire in 2027.

We account for income taxes in accordance with FASB ASC Topic 740, Income Taxes, which provides for deferred taxes using an asset and liability approach. We recognize deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference

between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if, based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

We have evaluated the positive and negative evidence bearing upon the realizability of our deferred tax assets, including our NOLs. Based on our history of operating losses, we believe that it is more likely than not that the benefit of our deferred tax assets will not be realized. Accordingly, we have provided a full valuation allowance for deferred tax assets as of March 31, 2017 and December 31, 2016.

Emerging Growth Company Status

Under Section 107(b) of the JOBS Act, an "emerging growth company," or EGC, can delay adopting new or revised accounting standards until such time as those standards 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.

As an EGC, we rely on certain of exemptions and reduced reporting requirements under the JOBS Act, including exemptions from the requirement to provide an auditor's attestation report on our system of internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and from 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, known as the auditor discussion and analysis. We will remain an EGC until the earlier of: the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; December 31, 2021; the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or SEC. Based on our public float as of the date of this report, we currently expect that we will become a large accelerated filer, and cease to be an EGC, as of December 31, 2017.

Recent Accounting Pronouncements

See Note 2 for disclosure of recent accounting pronouncements.

Results of Operations

Comparison of the Three Months Ended March 31, 2017 and 2016

The following table summarizes our results of operations for the three months ended March 31, 2017 and 2016, together with the dollar increase or decrease in those items:

	Three Months Ended March 31,		
	2017	2016	Period-to-Period Change
Net revenue	$ —	$ —	$ —
Operating expenses			
General and administrative	9,637,807	4,823,913	4,813,894
Research and development	20,327,087	16,065,089	4,261,998
Total operating expenses	29,964,894	20,889,002	9,075,892
Interest income	245,940	52,895	193,045
Net loss	$ (29,718,954)	$ (20,836,107)	$ (8,882,847)

General and Administrative

General and administrative expense increased from $4.8 million for the three months ended March 31, 2016, to $9.6 million for the three months ended March 31, 2017. This $4.8 million increase included a $0.7 million increase in non-cash stock-based compensation expense and increases of $2.0 million in salaries and personnel-related costs, $1.4 million in legal, professional and consulting fees, as well as an increase of $0.7 million in other administrative costs driven by increased headcount across all general and administrative functions, from 8 employees as of March 31, 2016 to 28 employees as of March 31, 2017 to support our overall growth.

Research and Development

Research and development expense increased from $16.1 million for the three months ended March 31, 2016, to $20.3 million for the three months ended March 31, 2017. The $4.2 million increase was partially offset by a $9.2 million decrease in non-cash stock-based compensation expense. The increase was primarily attributable to product manufacturing expenses and associated accelerated spending, including increased headcount, in our Libertyville, Illinois product manufacturing facility as well as expenses related to the conclusion of our Phase 1 clinical trial of AVXS-101 in SMA Type 1. More specifically, these increases included $4.7 million in research and development supplies and materials and $4.5 million in third-party clinical and manufacturing research and development expense associated with product manufacturing, $2.1 million in salaries and personnel-related expenses, driven by increased headcount across all research and development and manufacturing functions from 24 employees as of March 31, 2016 to 75 employees as of March 31, 2017, and $2.1 million in other research and development expenses

We anticipate our research and development costs will continue to increase over the next several years due to increased spending on the development of AVXS-101 and future product candidates.

Interest Income

Interest income for the three months ended March 31, 2017 consists of interest earned on our cash and cash equivalents.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have funded our research and development and operating activities primarily through equity financings, including $95.3 million and $148.3 million of net proceeds from our initial public offering and our subsequent public offering, respectively, and $80.5 million of aggregate net proceeds from private placements of stock prior to our initial public offering.

As of March 31, 2017, we had cash and cash equivalents of $204.5 million and had no debt outstanding.

Cash Flows

The following table provides information regarding our cash flows for the three months ended March 31, 2017 and 2016:

	Three Months Ended March 31,	
	2017	2016
Net cash used in operating activities	$ (28,126,718)	$ (7,682,699)
Net cash used in investing activities	(8,064,242)	(3,659,718)
Net cash provided by financing activities	214,900	97,327,990
Net (decrease) increase in cash and cash equivalents	$ (35,976,060)	$ 85,985,573

Operating Activities

For the three months ended March 31, 2017, our net cash used in operating activities of $28.1 million primarily consisted of a net loss of $29.7 million, primarily attributable to our spending on research and development, manufacturing and general and administrative expenses and $3.7 million in net cash used in changes in working capital items, which was partially offset by $5.3 million in adjustments for non-cash items. The $3.7 million in net cash used in changes in working capital represents primarily a $3.2 million increase in prepaid expenses and other current assets and other long-term assets and a $7.1 million decrease in accrued expenses, partially offset by a $6.5 million increase in accounts payable. Adjustments for non-cash items consisted of $5.1 million of stock-based compensation expense and $0.2 million of depreciation and amortization.

For the three months ended March 31, 2016, our net cash used in operating activities of $7.7 million primarily consisted of a net loss of $20.8 million, primarily attributable to our spending on research and development and general and administrative expenses, and $0.4 million in net cash used in changes in working capital items, which was partially offset by $13.5 million in adjustments for non-cash items. Adjustments for non-cash items primarily consisted of $13.5 million of stock-based compensation expense, of which $10.4 million was associated with the vesting in full of the restricted stock grant to Dr. Kaspar. The change in working capital was primarily attributable to an increase in prepaid expenses and other long-term assets, partially offset by an increase in accounts payable and accrued expenses.

Investing Activities

For the three months ended March 31, 2017, net cash used in investing activities consisted of $8.1 million of capital expenditures, primarily related to our manufacturing facility and purchases of property and equipment. For the three months ended March 31, 2016, net cash used in investing activities consisted of $3.7 million of capital expenditures, primarily related to our manufacturing facility and purchases of property and equipment.

Financing Activities

For the three months ended March 31, 2017, net cash provided by financing activities of $0.2 million consisted of proceeds from the exercise of employee stock options. For the three months ended March 31, 2016, net cash provided by financing activities consisted of $97.3 million from our initial public offering.

Future Funding Requirements

To date, we have not generated any revenues from the commercial sale of approved gene therapy products or drug therapies and we do not expect to generate substantial revenue for at least the next few years. If we fail to complete the development of our product candidates in a timely manner or fail to obtain their regulatory approval, our ability to generate future revenue will be compromised. We do not know when, or if, we will generate any revenue from our gene therapy core business. We do not expect to generate significant revenue unless and until we obtain regulatory approval of and commercialize AVXS-101. In addition, we expect our expenses to increase in connection with our ongoing development activities, particularly as we continue the research, development and clinical trials of, and seek regulatory approval for, product candidates, including with respect to development of AVXS-101 for other types of SMA and other product candidates for other diseases. We also expect to continue to incur costs associated with operating as a public company. In addition, subject to obtaining regulatory approval of product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. We anticipate that we will need additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

Based upon our current operating plan, we believe that our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements into the first half of 2019. We intend to devote the majority of our capital resources for clinical development and regulatory approval of AVXS-101. We have based our estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of product candidates.

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

- the progress and results of our studies and clinical trials for AVXS-101;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates;

- the number and development requirements of other product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- the cost and timing of establishing and validating manufacturing processes and facilities, including our own, for development and commercialization of our product candidates, if approved;
- the efforts necessary to institute post-approval regulatory compliance requirements;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval, which may be affected by market conditions, including obtaining coverage and adequate reimbursement of our product candidates from third-party payors, including government programs and managed care organizations, and competition within the therapeutic class to which our product candidates are assigned;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other product candidates and technologies.

Our future commercial revenue, if any, will be derived from sales of therapy products that we do not expect to be commercially available for several years, if at all. Accordingly, we may need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the terms of these equity securities or this debt may restrict our ability to operate. Any future debt financing and equity financing, if available, may involve agreements that include covenants limiting and restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, entering into profit-sharing or other arrangements or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

Contractual Obligations, Commitments and Contingencies

We lease a 15,668 square foot facility for our corporate headquarters in Bannockburn, Illinois, pursuant to a lease that expires in July 2024. The lease agreement provides for annual escalation in rent payments during the lease term. We are amortizing the escalation in rental payments on a straight-line basis over the term of the lease. We also lease a 1,318 square foot facility in Columbus, Ohio for research and development activities, pursuant to a lease that expires in March 2019. In March 2016, we entered into a lease agreement, which expires in August 2026, for approximately 48,529 square feet of warehouse and office space Libertyville, Illinois. A portion of the warehouse space was converted into manufacturing space. The lease agreement provides for annual escalation in rent payments during the lease term. The lease agreement provides us with a one-time right to terminate the lease effective as of the last day of the ninety-sixth full calendar month of the lease subject to a termination fee. We are amortizing the escalation in rental payments on a straight-line basis over the term of the lease. In March 2014, we entered into a lease agreement, which expired in April 2017, for approximately 2,418 square feet of office space in Dallas, Texas.

Future minimum lease payments under all of our leases are as follows:

Year ending December 31,		
2017	$	400,870
2018		660,572
2019		674,850
2020		689,289
2021		703,894
Thereafter		2,514,993
Total	$	5,644,468

During the three months ended March 31, 2017, there were no other material changes outside the ordinary course of our business to the contractual obligations specified in the table of contractual obligations included in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report.

Off-Balance Sheet Arrangements

During the periods presented, we did not have, nor do we currently have, any off-balance sheet arrangements as defined under SEC rules.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Our primary exposure to market risk for our cash and cash equivalents is interest income sensitivity, which is affected by changes in the general level of U.S interest rates. As of March 31, 2017, we had cash and cash equivalents totaling $204.5 million. Cash and cash equivalents consist of cash, deposits with banks and short term highly liquid money market instruments with remaining maturities at the date of purchase of 90 days or less. These instruments are exposed to the impact of interest rate changes which may result in fluctuations to our interest income. The primary objective of our investment activity is to preserve capital to fund our operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of investments in a variety of securities of high credit quality. We do not believe a sudden change in the interest rates would have a material impact on our financial condition or results of operations. A hypothetical 10% change in interest rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, our management, under the supervision and with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2017. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2017, our Chief Executive Officer and Chief Financial Officer have concluded that, as of March 31, 2017, our disclosure controls and procedures were not effective at the reasonable assurance level as a result of the material weaknesses discussed below. Notwithstanding these material weaknesses, our management has concluded that the financial statements included elsewhere in this Quarterly Report present fairly, in all material respects, our financial position, results of operations and cash flows in conformity with generally accepted accounting principles ("GAAP").

In connection with the preparation of our Annual Report on Form 10-K for the year ended December 31, 2016, our management concluded that, as of December 31, 2016, our internal control over financial reporting was not effective, as a result of material weaknesses in our control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our financial statements will not be prevented or detected on a timely basis. The material weaknesses identified in our internal control over financial reporting related to our lack of sufficiently trained professionals with an appropriate level of accounting knowledge, lack of written policies regarding our accounting function and procedures to identify and appropriately account for complex debt and equity agreements or share-based compensation awards, lack of restricted access to key financial systems and records and appropriate segregation of duties.

Status of Remediation of Material Weaknesses

We have taken numerous steps to address these material weaknesses and believe we have made significant progress toward remediating them, primarily through the hiring of multiple additional full-time accounting and financial personnel. With the addition of these personnel and others, we believe we now have sufficient personnel with an appropriate level of accounting knowledge and experience commensurate with our financial reporting requirements.

Based on our risk assessments and ongoing reviews of our financial statements, we have implemented controls over key financial transaction areas, application of GAAP, SEC reporting and associated disclosures and begun the process of formally documenting our key accounting policies and procedures. We have also performed additional review procedures in areas subject to audit adjustments in prior periods and have identified key controls for our significant processes. In addition, effective January 1, 2017, we have converted to a new enterprise resource planning ("ERP") financial system to support improved automation and control over our finance and

accounting functions. Effective with our IPO, we no longer have complex debt and equity agreements.

During the remainder of 2017, we intend to continue our remediation efforts by completing the documentation of our key controls and testing the design and operating effectiveness of these controls. We are actively working to implement effective internal control over financial reporting, which includes remediation of these material weaknesses. However, such compliance is not guaranteed, and we cannot provide any assurance that our internal control over financial reporting will be effective as a result of these efforts.

Except as described above, there were no changes in our internal control over financial reporting during the fiscal quarter ended March 31, 2017 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

For this item, please refer to Note 8, Commitments and Contingencies to the Notes to Unaudited Condensed Consolidated Financial Statements included in Part I, Item 1 of this Quarterly Report, which is incorporated herein by reference.

Item 1A. Risk Factors

The discussion of our business and operations discussed in this report should be read together with the risk factors contained in Item 1A of our Annual Report, as filed with the SEC on March 16, 2017, which describe various risks and uncertainties to which we are or may become subject. These risks and uncertainties have the potential to affect our business, financial condition, results of operations, cash flows, strategies, or prospects in a material and adverse manner.

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

Use of Proceeds from Initial Public Offering of Common Stock

On February 10, 2016, our Registration Statement on Form S-1 (File No. 333-209019) was declared effective by the SEC for our initial public offering, pursuant to which we sold an aggregate of 4,750,000 shares of common stock at a price to the public of $20.00 per share. Goldman, Sachs & Co. and Jefferies LLC acted as joint book-running managers of the offering, BMO Capital Markets Corp. acted as lead manager and Chardan acted as co-manager. We granted the underwriters a 30-day over-allotment option to purchase an additional 712,500 shares of common stock at a price of $20.00 per share, less the underwriting discount. The offering closed on February 17, 2016 with respect to the 4,750,000 shares of common stock.

The net proceeds from this sale, after underwriting discounts and offering expenses, were approximately $88.3 million. On March 9, 2016, the offering closed with respect to an additional 527,941 shares purchased by the underwriters pursuant to the over-allotment option. The net proceeds from this sale, after underwriting discounts, were approximately $9.8 million. In connection with our initial public offering, no payments were made by us to directors, officers or persons owning ten percent or more of our common stock or to their associates or to our affiliates. There has been no material change in the planned use of proceeds from our initial public offering as described in our prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on February 11, 2016.

In connection with our initial public offering, no payments were made by us to directors, officers or persons owning ten percent or more of our common stock or to their associates or to our affiliates. There has been no material change in the planned use of proceeds from our underwritten public offering as described in our prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on February 11, 2016.

Item 3. Default Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

None.

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Item 6. Exhibits

See the Exhibit Index following the signature page to this Quarterly Report for a list of exhibits filed or furnished with this report, which Exhibit Index is incorporated herein by reference.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

AVEXIS, INC.

Date: May 11, 2017

By: /s/ Thomas J. Dee
Thomas J. Dee
Senior Vice President, Chief Financial Officer
(Principal Financial and Principal Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
3.1	Fifth Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K filed on February 17, 2016).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Current Report on Form 8-K filed on February 17, 2016).
4.1	Specimen Stock Certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to Amendment No. 2 to the Registration Statement on Form S-1 filed on February 9, 2016).
31.1	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.

* These certifications are being furnished solely to accompany this Quarterly Report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER

I, Sean P. Nolan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of AveXis, 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) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

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/ Sean P. Nolan
Sean P. Nolan
President and Chief Executive Officer

Exhibit 31.2

Certification of Chief Financial Officer

I, Thomas J. Dee, certify that:

1. I have reviewed this quarterly report on Form 10-Q of AveXis, 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) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

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 J. Dee
Thomas J. Dee
Senior Vice President, Chief Financial Officer

Exhibit 32.1

Certification of Chief Executive Officer and Chief Financial Officer

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Sean P. Nolan, President and Chief Executive Officer, and Thomas J. Dee, Senior Vice President, Chief Financial Officer, of AveXis, Inc. (the "Company"), each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended March 31, 2017 (the "Quarterly Report"), to which this Certification is attached as Exhibit 32.1, fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

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

Date: May 11, 2017

/s/ Sean P. Nolan
Sean P. Nolan
President and Chief Executive Officer

Date: May 11, 2017

/s/ Thomas J. Dee
Thomas J. Dee
Senior Vice President, Chief Financial Officer

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