UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **May 11, 2017**

AVEXIS, INC.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of incorporation)

001-37693	**90-1038273**
(Commission File No.)	(IRS Employer Identification No.)

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

Registrant's telephone number, including area code: **(847) 572-8280**

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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. ☒

Item 2.02. Results of Operations and Financial Condition.

On May 11, 2017, AveXis, Inc. (the "***Registrant***") issued a press release announcing the Registrant's financial results for the first quarter ended March 31, 2017. A copy of this press release is furnished herewith as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information in this Item 2.02 of this Current Report on Form 8-K (including Exhibit 99.1) is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "***Exchange Act***"), or otherwise subject to the liabilities of that Section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Exhibit Description
99.1	Press Release, dated May 11, 2017, titled "AveXis Reports First Quarter 2017 Financial and Operating Results."

SIGNATURES

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

Date: May 11, 2017

AVEXIS, INC.

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

EXHIBIT INDEX

Exhibit Number	Exhibit Description
99.1	Press Release, dated May 11, 2017, titled "AveXis Reports First Quarter 2017 Financial and Operating Results."

Exhibit 99.1

Media Inquiries:
Lauren Barbiero
W2O Group
646-564-2156
lbarbiero@w2ogroup.com

Investor Inquiries:
Jim Goff
AveXis, Inc.
650-862-4134
jgoff@avexis.com

AveXis Reports First Quarter 2017 Financial and Operating Results

— Conference call of April 25 was in lieu of Q1 2017 earnings call —

Chicago, Ill. (May 11, 2017) — AveXis, Inc. (NASDAQ: AVXS), a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, today reported financial results for the first quarter ended March 31, 2017, recent corporate highlights and upcoming milestones.

"We are very pleased with our progress during the first quarter and recent weeks, including the recently reported encouraging results from the closeout of the Phase 1 trial of AVXS-101 in SMA Type 1," said Sean Nolan, President and Chief Executive Officer of AveXis. "Our team is focused on executing our plan to bring AVXS-101 to patients suffering from SMA Type 1 as quickly and safely as possible."

Recent Highlights

Results from the Phase 1 Trial of AVXS-101 in SMA Type 1: The Phase 1, open-label, dose-escalating study was designed to evaluate the safety and tolerability of AVXS-101 in patients with spinal muscular atrophy (SMA) Type 1. The key measures of efficacy were the time from birth to an "event," which was defined as either death or at least 16 hours per day of required ventilation support for breathing for 14 consecutive days in the absence of acute reversible illness or perioperatively, and video confirmed achievement of ability to sit unassisted. Additionally, several exploratory objective measures were assessed, including a standard motor milestone development survey and Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND).

- **Event-free Survival and Safety:** Data as of January 20, 2017, showed no new events, and 15 of 15 (100%) patients event-free at 13.6 months. The expected event-free survival rate at 13.6

months based on the natural history of the disease is 25 percent. The median age at last follow-up was 20.2 months and 30.8 months for patients in the proposed therapeutic dose (Cohort 2) and low-dose (Cohort 1) respectively. AVXS-101 appeared to have a favorable safety profile and to be generally well tolerated, with no new treatment-related safety or tolerability concerns identified.

- **Motor Milestone Achievement:** As of January 20, 2017, 11 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 11 of 12 patients (92%) could sit with assistance.

    - 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.

    - Three patients in Cohort 2 have achieved additional sitting unassisted milestones since 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.

    - As of January 20, 2017, two patients in Cohort 2 could crawl, pull to a stand, and stand and walk independently.

- **Nutritional and Respiratory Support:** As of January 20, 2017, six of seven (86%) patients in Cohort 2 that did not require feeding support before treatment continued without feeding support after treatment; 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, 11 of 12 (92%) patients in Cohort 2 were fed orally, and six of 12 (50%) patients were exclusively fed orally; and eight of 12 (67%) patients were able to speak.

Presented AVXS-101 research at the Annual Meeting of the American Academy of Neurology: Jerry Mendell, M.D., director of the Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital, presented results from the Phase 1 study of AVXS-101 in SMA Type 1 during a plenary session, including video evidence of children achieving motor milestones.

- Dr. Brian Kaspar, Senior Vice President and Chief Scientific Officer of AveXis, presented data from a dose-response study for the cerebrospinal fluid (CSF) delivery of AVXS-101 that offers insight into vector distribution and its correlation with transgene expression, providing guidance for future AAV9-based clinical trials in SMA, as well as other neurodegenerative disorders.

- Dr. Douglas Sproule, Vice President of Clinical Development and Medical Affairs of AveXis, presented data suggesting pre-existing antibodies to AAV9 are quite uncommon in the pediatric population and will not impact use of gene therapy for the vast majority of SMA Type 1 patients.

- Dr. Linda Lowes, Director of Clinical Therapies Research and a member of the Center for Gene Therapy at the Research Institute of Nationwide Children's Hospital, presented research providing further evidence of the correlation between motor function and motor milestone achievement in patients with SMA Type 1, demonstrating that the degree of treatment outcome appears to be influenced by age at dosing and baseline motor function.

Completed Type B chemistry manufacturing and controls (CMC) meeting with the U.S. Food and Drug Administration (FDA): On May 1, 2017, AveXis participated in a Type B CMC meeting with the FDA.

The purpose of the meeting was to present to the agency AveXis' proposed process for producing the intended commercial scale GMP-derived gene therapy product, to gain alignment with the agency on the proposed assay qualification plan, and to gain alignment on the proposed protocol for

demonstrating comparability of the intended commercial scale GMP-derived product with the material administered to patients in the Phase 1 trial of AVXS-101 in SMA Type 1.

The company expects to provide an update on its further plans and development timelines following receipt of the minutes of the CMC Type B meeting, currently anticipated in early June 2017.

Joao Siffert Appointed to Board of Directors: On April 19, 2017, AveXis announced the appointment of Joao Siffert to its Board of Directors, effective upon the completion of the annual meeting of stockholders. Dr. Siffert brings important knowledge to the Board based on his experience in central nervous system drug development and regulatory expertise in both the U.S. and Europe, as well as his experience working with global health care companies.

First Quarter 2017 Financial Results

- **Cash Position:** As of March 31, 2017, AveXis had $204.5 million in cash and cash equivalents.

- **R&D Expenses:** Research and development expenses were $20.3 million for the first quarter of 2017 (which included $2.3 million of non-cash stock-based compensation expense), compared to $16.1 million for the same period in 2016 (which included $11.5 million of non-cash stock-based compensation expense). Excluding the impact attributable to non-cash compensation expense, research and development expenses increased $13.4 million. The increase was primarily attributable to product manufacturing expenses and associated accelerated spending related to the company's Libertyville, Illinois product manufacturing facility, associated increased headcount as well as expenses related to the conclusion of the Phase 1 clinical trial of AVXS-101 in SMA Type 1.

- **G&A Expenses:** General and administrative expenses were $9.6 million for the first quarter of 2017 (which included $2.8 million of non-cash stock-based compensation expense), compared to $4.8 million for the same period in 2016 (which included $2.1 million of stock-based compensation expense), an increase of $4.8 million. Excluding the impact attributable to non-cash compensation expense, general and administrative expenses increased $4.1 million. The increase was primarily attributable to increases in salaries and personnel-related costs, legal, professional and consulting fees and other administrative costs driven by increased headcount across all general and administrative functions to support the company's overall growth.

- **Net Loss:** Net loss was $29.7 million, or $1.07 per share, for the first quarter of 2017, compared to a net loss of $20.8 million, or $1.24 per share, for the first quarter of 2016.

Selected Financial Information

Operating Results:

	Three Months Ended March 31,	
	2017	2016
Revenue		
Total 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)
Weighted-average basic and diluted common shares outstanding	27,733,701	16,774,718
Basic and diluted net loss per common share	$ (1.07)	$ (1.24)

Balance Sheet Information:

	March 31, 2017	December 31, 2016
Cash and cash equivalents	$ 204,453,779	$ 240,429,839
Total assets	247,242,052	270,575,431
Total liabilities	25,530,573	24,443,777
Accumulated deficit	$ (171,281,278)	$ (141,562,324)

Conference Call Information

The AveXis conference call and webcast of April 25, 2017 was conducted in lieu of a first quarter 2017 financial and operating results conference call. AveXis will not host a conference call and webcast related to its first quarter 2017 financial and operating results. The Company expects to host its next conference call and webcast following receipt of the minutes from the CMC meeting with the FDA, expected approximately 30 days following the meeting, which took place May 1, 2017.

About SMA

SMA is a severe neuromuscular disease characterized by the loss of motor neurons leading to progressive muscle weakness and paralysis. SMA is caused by a genetic defect in the SMN1 gene that codes SMN, a protein necessary for survival of motor neurons. The incidence of SMA is approximately one in 10,000 live births.

The most severe form of SMA is Type 1, a lethal genetic disorder characterized by motor neuron loss and associated muscle deterioration, which results in mortality or the need for permanent ventilation support before the age of two for greater than 90 percent of patients. SMA Type 1 is the leading genetic cause of infant mortality.

About AVXS-101

AVXS-101 is a proprietary gene therapy candidate of a one-time treatment for SMA Type 1 and is designed to address the monogenic root cause of SMA and prevent further muscle degeneration by addressing the defective and/or loss of the primary SMN gene. AVXS-101 also targets motor neurons providing rapid onset of effect, and crosses the blood brain barrier allowing an IV dosing route and effective targeting of both central and systemic features.

About AveXis, Inc.

AveXis is a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases. The company's initial proprietary gene therapy candidate, AVXS-101, is in an ongoing Phase 1 clinical trial for the treatment of SMA Type 1. For additional information, please visit www.avexis.com.

Forward-Looking Statements

This press release contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, regarding, among other things, AveXis' research, development and regulatory plans for AVXS-101, including the potential of AVXS-101 to positively impact quality of life and alter the course of disease in children with SMA Type 1 and statements about the effects of SMA Type 1 on developmental milestones and timing of regulatory feedback. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual results to differ materially from those projected in its forward-looking statements. Meaningful factors which could cause actual results to differ include, but are not limited to, the scope, progress, expansion, and costs of developing and commercializing AveXis' product candidates; regulatory developments in the U.S. and EU, as well as other factors discussed in the "Risk Factors" and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of AveXis' Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 16, 2017. In addition to the risks described above and in the Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the SEC, other unknown or unpredictable factors also could affect AveXis' results. There can be no assurance that the actual results or developments anticipated by AveXis will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, AveXis. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

All forward-looking statements contained in this press release are expressly qualified by the cautionary statements contained or referred to herein. AveXis cautions investors not to rely too heavily on the forward-looking statements AveXis makes or that are made on its behalf. These forward-looking statements speak only as of the date of this press release (unless another date is indicated). AveXis undertakes no obligation, and specifically declines any obligation, to publicly update or revise any such forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

Non-GAAP Financial Measure

In addition to disclosing financial results that are determined in accordance with GAAP, in order to understand and evaluate our operating performance, and provide a more complete understanding of factors and trends affecting our business, we also measure the increase in research and development expenses and general and administrative expenses excluding non-cash stock-based compensation

expense. We believe that excluding this expense better reflects the increase in research and development and general and administrative expenses during the period, as compared to the prior period. This non-GAAP financial metric should be considered supplemental to and not a substitute for financial information prepared in accordance with GAAP. Because non-GAAP financial metrics exclude the effect of items that will increase or decrease the company's reported results of operations, we strongly encourage investors to review our consolidated financial statements and periodic reports in their entirety.

# # #