AVEXIS, INC.

FORM 8-K
(Current report filing)

Filed 03/16/17 for the Period Ending 03/16/17

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2275 HALF DAY ROAD
SUITE 160
BANNOCKBURN, IL, 60015

Telephone 972-725-7797
CIK 0001652923

SIC Code 2836 - Biological Products, (No Diagnostic Substances)
Industry Biotechnology & Medical Research
Sector Healthcare
Fiscal Year 12/31
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): March 16, 2017

AVEXIS, INC.
(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of incorporation)

001-37693
(Commission File No.)

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

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

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

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))
Item 2.02. Results of Operations and Financial Condition.

On March 16, 2017, AveXis, Inc. (the “Registrant”) issued a press release announcing the Registrant’s financial results for the fourth quarter and fiscal year ended December 31, 2016, as well as information regarding a conference call to discuss these financial results and the Registrant’s recent corporate highlights and outlook, including certain information related to the Registrant’s clinical data results for AVXS-101. 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 (the “Securities Act”), or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 7.01 Regulation FD Disclosure.

As discussed in Item 2.02 above, the Registrant intends to present certain information related to its clinical data results for AVXS-101 during the March 16, 2017 conference call. A copy of the slides to be presented during the call are furnished herewith as Exhibit 99.2 to this Current Report on Form 8-K and are incorporated herein by reference.

The information in this Item 7.01 of this Current Report on Form 8-K (including Exhibit 99.2) is being furnished and shall not be deemed “filed” for purposes of Section 18 of 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 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

<table>
<thead>
<tr>
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</tr>
</thead>
<tbody>
<tr>
<td>99.1</td>
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</tr>
<tr>
<td>99.2</td>
<td>Slide Presentation dated March 16, 2017, titled “AVXS-101 Topline Phase 1 Clinical Trial Results.”</td>
</tr>
</tbody>
</table>
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: March 16, 2017

AVEXIS, INC.

By: /s/ Sean P. Nolan
Sean P. Nolan
President and Chief Executive Officer
<table>
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AveXis Reports Topline Results from Phase 1 Trial of AVXS-101 in SMA Type 1 and Fourth Quarter and Full Year 2016 Financial and Operating Results

— No new treatment-related safety or tolerability findings —

— No new events reported and 15 of 15 patients event-free at 13.6 months of age; majority of patients receiving proposed therapeutic dose sit unassisted —

— Conference call and webcast March 16 at 4:30 p.m. EDT —

Chicago, Ill. (March 16, 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 topline results from the Phase 1 trial of AVXS-101 in spinal muscular atrophy (SMA) Type 1. The company also reported financial results for the fourth quarter and full year ended December 31, 2016, recent corporate highlights and upcoming milestones.

“The completion of our Phase 1 clinical study of AVXS-101, the first ever gene therapy studied for the treatment of SMA Type 1, is an exciting and eagerly awaited milestone, and we are quite pleased with these data,” said Sean Nolan, President and Chief Executive Officer of AveXis. “The past few months have been productive for AveXis, and we look forward to continuing the momentum with several upcoming corporate catalysts, including the planned Type B CMC meeting with the FDA, as well as ongoing collaborative discussions with regulatory authorities in the United States and Europe to explore the most expeditious pathways for marketing approval of AVXS-101.”
Topline 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 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).

No New Treatment-related Safety or Tolerability Concerns Identified: As of January 20, 2017, AVXS-101 appeared to have a favorable safety profile and to be generally well tolerated, with no new safety or tolerability concerns identified.

- As has been previously reported, a total of five adverse events (AEs) in four patients were deemed treatment-related. Of these, two were serious adverse events (SAEs) experienced by two patients, and three were non-serious AEs experienced by two patients. All consisted of clinically asymptomatic liver enzyme elevations and were resolved with prednisolone treatment. There were no clinically significant elevations of gamma-glutamyl transferase, alkaline phosphatase or bilirubin and, as such, Hy’s Law was not met. Other non-treatment-related AEs were expected and were associated with SMA.
- A cumulative total of 256 AEs (five treatment-related AEs and 251 non-treatment related AEs) were reported as of January 20, 2017, following monitoring and source verification. Of these, 52 were determined to be SAEs and 204 were non-serious AEs. As previously noted, two of the 52 SAEs were deemed treatment-related.
- There were 65 new AEs reported after September 15, 2016, of which 10 were SAEs in three patients and were associated with SMA and were not deemed treatment-related.

No New Events and 15 of 15 Patients Event-Free at 13.6 Months, including 12 of 12 Patients in Proposed Therapeutic-Dose Cohort: As of January 20, 2017, 12 of 12 patients (100%) in the cohort of patients who received the proposed one-time therapeutic dose of AVXS-101 (Cohort 2) had reached 13.6 months of age event-free, where the expected event-free survival rate based on natural history of the disease is 25%. The median age at last follow-up for Cohort 2 was 20.2 months, with the oldest patient at 31.1 months of age.

- As of January 20, 2017, 9 of 9 patients-3 in the low-dose cohort (Cohort 1) and 6 in Cohort 2-reached 20 months of age event free, where the expected event-free rate based on natural history of the disease is 8%.
- As of January 20, 2017, three patients in Cohort 1 reached 13.6 months of age event-free. As has been previously reported, one patient in Cohort 1 had a pulmonary event in the third quarter of 2016. The patient had increased use of bi-level positive airway pressure (BiPAP) in advance of surgery related to hypersalivation, a condition experienced by some SMA patients; the event was determined upon independent review to represent progression of disease and not to be related to the use of AVXS-101. This patient completed the final trial visit in September 2016, and as of that time BiPAP use was below the event threshold.

Rapid and Sustained CHOP INTEND Improvements Above Baseline: As of January 20, 2017, mean increases from baseline in CHOP INTEND scores of 7.7 points in Cohort 1 and 24.7 points in Cohort 2 were observed, reflecting improvement in motor function. In Cohort 2,
there were mean increases in CHOP INTEND of 9.8 points one month after gene therapy and 15.4 points three months following gene therapy.

- 11 out of 12 patients (92%) in Cohort 2 achieved CHOP INTEND scores of at least 40 points.
- 10 out of 12 patients (83%) in Cohort 2 achieved CHOP INTEND scores of at least 50 points.
- 2 out of 12 patients (17%) in Cohort 2 achieved CHOP INTEND scores of at least 60, which is in a range considered to be normal. These two patients achieved the maximum CHOP INTEND score of 64.

Cohort 2 Patients Consistently Achieved and Maintained Key Developmental Motor Milestones: 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. For the end-of-study assessment, AveXis evaluated three validated and well-established measures of sitting unassisted for periods of increasing duration. Nine of 12 patients (75%) 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 could walk independently, and each had achieved earlier and important developmental milestones such as standing with support, standing alone and walking with support.

Detailed proposed therapeutic-dose cohort motor milestone data is included in the chart below:

<table>
<thead>
<tr>
<th>Cohort 2</th>
<th>Age at Gene Transfer (mos)</th>
<th>Brings Hand to Mouth</th>
<th>Head Control</th>
<th>Partial Roll(a)</th>
<th>Roll(b)</th>
<th>Sitting with Assistance</th>
<th>Sitting Unassisted</th>
<th>Motor Milestone Achievement as of January 20, 2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>E.04</td>
<td>6</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.05</td>
<td>4</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.06</td>
<td>2</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
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<td>E.07</td>
<td>4</td>
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<td>X</td>
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<td>X</td>
<td></td>
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</tr>
<tr>
<td>E.08</td>
<td>8</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.09</td>
<td>5</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.10</td>
<td>1</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
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<td>E.11</td>
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<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.12</td>
<td>3</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.13</td>
<td>1</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
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<td>E.14</td>
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<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.15</td>
<td>2</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
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</tr>
</tbody>
</table>

(a) Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back in only one direction.
(b) Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° 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.
For the end-of-study assessment, all motor milestone achievements above were assessed and adjudicated by an independent third-party reviewer using video evidence.

“These topline data in aggregate for this Phase 1 study suggest a one-time infusion of AVXS-101 appears to be well-tolerated, with a favorable safety profile, and indicate the potential for a clinically transformative effect on event-free survival, rapid and sustained increases in motor function and achievement of motor milestones never observed in the natural history of this disease,” said Sukumar Nagendran, MD, Senior Vice President and Chief Medical Officer, AveXis. “We continue to be encouraged by these clinical trial data, and look forward to initiating our pivotal studies of AVXS-101 in SMA Type 1 later this year. We wish to thank the patients and caregivers, the SMA community, health care practitioners and all those who worked to make this trial possible. They are truly pioneers in exploring the potential for gene therapy in SMA Type 1.”

Recent Company Highlights

**AVXS-101 Accepted into PRIME Program:** On January 31, 2017, AveXis announced the European Medicines Agency (EMA) granted access into its PRIority MEdicines (PRIME) program for AVXS-101 for the treatment of SMA Type 1. PRIME is intended to enhance support for the development of medicines — specifically those that may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options — through early and proactive support by EMA to optimize the generation of robust data and development plans, and potentially expedite the assessment of the Marketing Authorization Application so these medicines may reach patients sooner.

**EU Pivotal Trial to Reflect Single-Arm Design:** On February 6, 2017, AveXis announced the planned pivotal study of AVXS-101 in SMA Type 1 in the European Union (EU) will reflect a single-arm design, using natural history of the disease as a comparator, and will enroll approximately 30 patients. This update was based on receipt of the Scientific Advice response from the Scientific Advice Working Party within the Committee for Medicinal Products for Human Use (CHMP) of the EMA. In addition to evaluating safety, the planned pivotal trial is expected to evaluate achievement of motor milestones, specifically patients’ ability to sit unassisted, as well as an efficacy measure defined by the time from birth to an event. The CHMP additionally recommended AveXis discuss the potential for Conditional Marketing Authorization in a future meeting with EMA.

**Rick Modi Appointed to Senior Management Team as CBO:** On February 15, 2017, AveXis announced the appointment of Rick Modi to the executive management team as Senior Vice President, Chief Business Officer. Mr. Modi brings more than 15 years of commercial, business and corporate experience to the position, and is responsible for all aspects of the company’s commercial functions.

**Planned Upcoming Clinical Development Milestones**

- Conduct a Type B meeting with the U.S. Food and Drug Administration (FDA) to discuss chemistry manufacturing and controls (CMC); provide an update based on receipt of meeting minutes in the second quarter of 2017.
- Initiate pivotal trial in U.S. of AVXS-101 via intravenous (IV) delivery in patients with SMA Type 1 in the second quarter of 2017, pending a successful outcome of the Type B CMC meeting.
- Initiate a Phase 1 safety and dose escalation study of AVXS-101 via intrathecal (IT) delivery in patients with SMA Type 2 in the second quarter of 2017, pending a successful outcome of the Type B CMC meeting described above.
Conduct an end-of-Phase 1 meeting with FDA in the second or third quarter of 2017.

Conduct a comprehensive clinical program review with the EMA, to be scheduled in the second or third quarter of 2017.

Initiate pivotal trial in the EU of AVXS-101 using IV delivery in patients with SMA Type 1 in the second half of 2017.

Five preclinical and clinical abstracts will be presented at the Annual Meeting of the American Academy of Neurology in Boston, April 22-28, 2017, including results from the Phase 1 trial of AVXS-101 in SMA Type 1, including developmental milestones videos.

Fourth Quarter and Full Year 2016 Financial Results

- **Cash Position:** As of December 31, 2016, AveXis had $240.4 million in cash and cash equivalents.
- **R&D Expenses:** Research and development expenses were $18.3 million for the fourth quarter of 2016 (which included $1.6 million of non-cash stock-based compensation expense), compared to $8.7 million for the same period in 2015 (which included $5.7 million of non-cash stock-based compensation expense), an increase of $9.6 million. The increase in research and development expenses was primarily attributable to an increase in expenses necessary to support the advancement of the company’s manufacturing product development efforts, clinical and pre-clinical programs, primarily the ongoing trial of AVXS-101 in SMA Type 1, and increases in personnel-related expenses driven by increased headcount across all research and development functions. Partially offsetting the increase in research and development spending was lower non-cash stock-based compensation expense of $4.1 million.
- **G&A Expenses:** General and administrative expenses were $7.2 million for the fourth quarter of 2016 (which included $2.4 million of non-cash stock-based compensation expense), compared to $4.4 million for the same period in 2015 (which included $1.6 million of stock-based compensation expense), an increase of $2.8 million. The increase in general and administrative expenses was primarily attributable to an increase in personnel-related expenses driven by increased headcount across all general and administrative functions, legal and professional fees and other infrastructure costs to support the company’s overall growth, and higher non-cash stock-based compensation expense.
- **Net Loss:** Net loss was $25.4 million, or $0.92 per share, for the fourth quarter of 2016, compared to a net loss of $13.2 million, or $1.82 per share, for the fourth quarter of 2015.

Selected Financial Information

Operating Results:

<table>
<thead>
<tr>
<th></th>
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<tbody>
<tr>
<td><strong>Revenue</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Total revenue</td>
<td>$ —</td>
<td>$ —</td>
<td>$ —</td>
<td>$ —</td>
</tr>
<tr>
<td><strong>Operating Expenses</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>General and administrative</td>
<td>7,198,039</td>
<td>4,428,279</td>
<td>24,522,902</td>
<td>11,079,512</td>
</tr>
<tr>
<td>Research and development</td>
<td>18,349,344</td>
<td>8,737,246</td>
<td>58,891,667</td>
<td>27,493,460</td>
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<tr>
<td>Total Operating Expenses</td>
<td>25,547,383</td>
<td>13,165,525</td>
<td>83,414,569</td>
<td>38,572,972</td>
</tr>
<tr>
<td>Loss from operations</td>
<td>(25,547,383)</td>
<td>(13,165,525)</td>
<td>(83,414,569)</td>
<td>(38,572,972)</td>
</tr>
<tr>
<td><strong>Other (Income)/Expense:</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Interest income</td>
<td>(172,452)</td>
<td>(4,718)</td>
<td>(402,765)</td>
<td>(99,128)</td>
</tr>
<tr>
<td>Total Other (Income) Expense</td>
<td>(172,452)</td>
<td>(4,718)</td>
<td>(402,765)</td>
<td>(99,128)</td>
</tr>
<tr>
<td><strong>Net loss</strong></td>
<td>$ (25,374,931)</td>
<td>$ (13,160,807)</td>
<td>$ (83,011,804)</td>
<td>$ (38,473,844)</td>
</tr>
<tr>
<td>Weighted-average basic and diluted common shares outstanding</td>
<td>27,678,348</td>
<td>7,226,122</td>
<td>22,647,583</td>
<td>7,087,618</td>
</tr>
<tr>
<td><strong>Basic and diluted net loss per common share</strong></td>
<td>$ (0.92)</td>
<td>$ (1.82)</td>
<td>$ (3.67)</td>
<td>$ (5.43)</td>
</tr>
</tbody>
</table>
Balance Sheet Information:

<table>
<thead>
<tr>
<th></th>
<th>December 31, 2016</th>
<th>December 31, 2015</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash and cash equivalents</td>
<td>$240,429,839</td>
<td>$62,251,860</td>
</tr>
<tr>
<td>Total assets</td>
<td>270,575,431</td>
<td>65,084,291</td>
</tr>
<tr>
<td>Total liabilities</td>
<td>24,443,777</td>
<td>6,877,304</td>
</tr>
<tr>
<td>Accumulated deficit</td>
<td>($141,562,324)</td>
<td>($58,550,520)</td>
</tr>
</tbody>
</table>

Conference Call Information

AveXis will host a conference call and webcast at 4:30 p.m. EDT today, March 16, 2017, to discuss the topline Phase 1 trial results for AVXS-101 in SMA Type 1, fourth quarter and full year 2016 financial and operating results, recent business highlights and upcoming development milestones.

Analysts and investors can participate in the conference call by dialing (844) 889-6863 for domestic callers and (661) 378-9762 for international callers, using the conference ID 84652584. The webcast can be accessed live on the Events and Presentations page in the Investors and Media section of the AveXis website, www.AveXis.com. The webcast will be archived on the company’s website until its next quarterly earnings call and will be available for telephonic replay for 14 days following the call by dialing (855) 859-2056 (Domestic) or (404) 537-3406 (International), conference ID 84652584.

About the Phase 1 Trial Design

The Phase 1 open-label, dose-escalation clinical trial of AVXS-101 in patients with SMA Type 1 initiated in April 2014. Enrollment of 15 patients across two dosing cohorts was completed in December 2015. Patients received a one-time intravenous infusion of AVXS-101 over a one-hour period in a peripheral limb vein.

Patients in the low-dose cohort (n=3) received 6.7E13 vg/kg. Patients in the proposed therapeutic dose cohort (n=12) received 2.0E14 vg/kg. Key inclusion criteria included SMA Type 1 patients with clinical symptoms before six months of age, bi-allelic SMN1 gene deletions or point mutations and with two copies of the SMN2 backup gene, as determined by genetic testing. Of note, all patients in the trial had bi-allelic Exon 7 deletions in SMN1. A key exclusion criteria was the presence of c.859G>C point mutation in SMN2 (Exon 7 modifier). Additionally, patients must have been no older than nine months of age (for the first nine patients) and six months of age (for the last six patients) at the time of vector infusion.

The primary outcome measure was safety and tolerability. The key measures of efficacy were 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).

The primary analysis for efficacy was assessed when all patients reached 13.6 months of age. A follow-up safety analysis will be completed when the last patient reaches 24 months post-dose.

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. SMA is the leading genetic cause of infant mortality.

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.

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 SMN1 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, recently completed a 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 expectations regarding timing and planned design of the U.S. and EU pivotal trials of AVXS-101 in patients with SMA Type 1, the overall clinical development of AVXS-101, our expectations regarding timing for meetings with regulatory agencies and the potential benefits of AVXS-101 being accepted into the PRIME program. 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.

###

8
AVXS-101 Topline Phase 1 Clinical Trial Results
Fourth Quarter and Full Year 2016 Financial and Operating Results

March 16, 2017


Disclaimers

Forward-Looking Statements

This presentation contains forward-looking statements, including statements about: the timing, progress and results of preclinical studies and clinical trials for AVXS-101, including statements regarding the timing of initiation of studies or trials and related preparatory work, our expectations regarding timing for meetings with regulatory agencies, our manufacturing strategy and developments, key regulatory and development milestones and our research and development programs. These statements involve substantial known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The forward-looking statements in this presentation represent our views as of the date of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable 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 presentation.
Our Strategy

Vision: Leader in rare and life-threatening neurological genetic diseases

Build pipeline of gene therapy treatments beyond SMA

Expand development of AVXS-101 into SMA Type 2

Establish foundational presence in SMA Type 1
Overview of SMA

SMA is a devastating orphan disease that results in motor neuron loss and progressive weakness: it is the most common genetic cause of infant death.

- Incidence: ~1 in 10,000 live births
- Caused by reduced SMN (survival motor neuron) protein levels from loss of/defective SMN1 gene
- SMA divided into sub-categories, Type 1-4, with Type 1 being most severe
  - Severity correlates with # of copies of SMN2 backup gene
# SMA Types: A Devastating Disease

<table>
<thead>
<tr>
<th></th>
<th>TYPE 1</th>
<th>TYPE 2</th>
<th>TYPE 3</th>
<th>TYPE 4</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>SMN2 Copy Number</strong></td>
<td>Two</td>
<td>Three or Four</td>
<td>Three or Four</td>
<td>Four to Eight</td>
</tr>
<tr>
<td><strong>Onset</strong></td>
<td>Before 6 Months</td>
<td>6-18 Months</td>
<td>Early childhood to early adulthood (juvenile)</td>
<td>Adulthood (20s-30s) usually after 30</td>
</tr>
<tr>
<td><strong>Incidence per Live Birth</strong></td>
<td>Approximately 60%</td>
<td>Approximately 27%</td>
<td>Approximately 13%</td>
<td>Uncommon; limited information available</td>
</tr>
<tr>
<td><strong>Developmental Milestones</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Will never be able to sit without support</td>
<td>• Will never be able to walk or stand without support</td>
<td>• Stand alone and walk but may lose ability to walk in 30s-40s</td>
<td>• Stand alone and walk but may lose ability to walk in 30s-40s (Same as Type 3)</td>
<td></td>
</tr>
<tr>
<td>• Difficulty breathing &amp; swallowing</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Can’t crawl/will never walk</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Survival</strong></td>
<td>&lt;10% Event free* by two years of age</td>
<td>68% alive at age 25</td>
<td>Normal</td>
<td>Normal</td>
</tr>
</tbody>
</table>

*Event = Death or ≥16-hr/day ventilation continuously for ≥2 wks. in the absence of an acute reversible illness
Natural History of SMA Type 1

More than 90% of SMA Type 1 patients will not survive or will need permanent ventilation support by age 2

*Survival for Finkel¹ = no death, or no need for ≥6 hr/day ventilation continuously for ≥2 weeks, in the absence of an acute reversible illness; n = 23 (7 copies of SMN2)

Survival for Kolb² = no death, or no tracheostomy; n = 20

1. FNCR (Finkel)
2. NeuroNEXT (Kolb)

Onset of SMA Type 1 by 6 months

Symptoms may present:
- "Floppy baby" syndrome
- Muscle weakness (legs more than arms)
- Poor head control
- Belly breathing
- Bulbar muscle weakness (weak cry, difficulty swallowing, aspiration)
- Will never sit unsupported

Milestone for a healthy infant

SMA Type 1 survival rates per Finkel¹

SMA Type 1 survival rate per Kolb²

Funeral head steady alone; brings hands to mouth
Falls over in both directions
Sits alone; crawls
Sits alone, may stand alone
Walks alone, may not and walk up stairs with support
Climbs furniture alone; walks and throws a ball
AVXS-101 Targets the Primary SMN Gene

**NORMAL INDIVIDUAL**

- SMN Genes
  - SMN1 Primary
  - SMN2 Back-up

  SMN Protein

**SMA-AFFLICTED INDIVIDUAL**

- SMN Genes
  - SMN1 Primary
  - SMN2 Back-up

  SMN Protein

**SMA-AFFLICTED INDIVIDUAL TREATED WITH AVXS-101**

- SMN Genes
  - SMN1 Primary
  - SMN2 Back-up

  SMN Protein

- AVXS-101 Primary

- Functional SMN Protein
- Non-functional SMN Protein
Our Solution: AVXS-101
An Innovative Treatment Approach for SMA

Gene therapy is the right approach for SMA: Monogenic mutation that drives the pathology

Recombinant AAV9 Capsid Shell

scAAV ITR  Continuous Promoter  Human SMN Transgene  scAAV ITR

KEY COMPONENTS

<table>
<thead>
<tr>
<th>Recombinant AAV9 Capsid Shell</th>
<th>PURPOSE</th>
</tr>
</thead>
<tbody>
<tr>
<td>scAAV ITR (Self-complementary DNA technology)</td>
<td></td>
</tr>
<tr>
<td>Continuous Promoter</td>
<td></td>
</tr>
<tr>
<td>Human SMN Transgene</td>
<td></td>
</tr>
</tbody>
</table>

- Ability to deliver across the blood brain barrier (BBB) and into the spinal cord
- Avoids the need for intrathecal delivery when treating infants
- Non-replicating virus does not modify the existing DNA of the patient
- Enables rapid onset of effect which is key in a quickly deteriorating population
- Activates the transgene to allow for continuous and sustained SMN expression
- Full copy of a stable, functioning SMN gene that is introduced into the cell’s nucleus

Children with SMA Type 1 Do Not Reach Any Major Motor Milestones

Developmental Milestones in Type 1 Spinal Muscular Atrophy
De Sanctis et al. Neuromuscular Disorders, Nov-2016

**DESIGN**
- Retrospective Study from large multi-center datasets (US and EU)
- Patients (n=33) have **genetically confirmed homozygous deletion of exon 7 in SMN1 gene**; categorized according to Dubowitz’s decimal classification (confirmation of SMN1 status and clinical observations)
- Study visits at baseline, every 2-3 months until the age of 12 months, and every 6 months thereafter, when possible
- Hammersmith Infant Neurological Examination (HINE) used to assess intermediate steps leading to full achievement of milestones

**CONCLUSIONS**
- **Prolongation of survival with supportive care does not impact achievement of motor milestones** in SMA Type 1 infants
- SMA Type 1 infants with symptom onset <6 months:
  - Will not reach any major motor milestones, such as sitting, crawling, standing, and walking
  - Any early intermediate milestones in 18 patients will be quickly lost
- The highest milestone achieved is seen in the child’s first visit followed by a rapid decline
- Any improvement or achievement of milestones not usually achieved in a child with SMA Type 1 in a drug intervention trial can be attributed to the drug and not due to survival or enhanced standard of care
### Key Enrollment Criteria

<table>
<thead>
<tr>
<th>Inclusion</th>
<th>Exclusion</th>
</tr>
</thead>
<tbody>
<tr>
<td>- 9 months of age / 6 months of age and younger at day of vector infusion with SMA Type 1 as defined by the following criteria:</td>
<td>- Active viral infection (includes HIV or serology positive for hepatitis B or C)</td>
</tr>
<tr>
<td>- Bi-allelic SMN1 gene deletion or point mutations</td>
<td>- Use of invasive ventilatory support (tracheotomy) or pulse oximetry &lt;95% saturation</td>
</tr>
<tr>
<td>- All enrolled patients carry bi-allelic SMN1 deletions, confirmed by independent laboratory</td>
<td>- Patients with Anti-AAV9 antibodies titers &gt;1:50 as determined by ELISA binding immunoassay</td>
</tr>
<tr>
<td>- 2 copies of SMN2</td>
<td>- Abnormal laboratory values considered to be clinically significant</td>
</tr>
<tr>
<td>- Onset of disease at birth to 6 months of age</td>
<td>- <em>Patients with c.859G&gt;C mutation in SMN2 exon 7 (predicted mild phenotype)</em></td>
</tr>
</tbody>
</table>

### Objectives

**Primary**
- Safety and Tolerability

**Secondary**
- Time from birth until death or time to 14-hour ventilation continuously for 22 weeks in the absence of an acute reversible illness or perioperatively
- Video confirmed achievement of ability to sit unassisted

*Key developmental milestone achievements assessed and adjudicated by external independent reviewer*

### Additional
- CHOP INTEND
- Bayley Motor Scales of Infant/Toddler development – Gross Motor
Event-Free Survival Data – January 20, 2017

Age (months*)

Cohort 1
6.7E13 vg/kg

Cohort 2
2.0E14 vg/kg

PNCR (Finke) 2014 Natural History Study:
75% event-free
50% event-free
25% event-free
8% event-free

Survival Data
- 9/9 reached 20 mo. event-free (8% PNCR)
- 15/15 reached 13.6 mo. event-free (25% PNCR)
- 15/15 reached 10.5 mo. event-free (50% PNCR)
- 15/15 reached 8.1 mo. event-free (75% PNCR)

Age at Last Follow-up
- Cohort 1*: 30.8 months (median)
- Cohort 2*: 20.2 months (median)

*reflects age at Last Trial Visit or most recent pulmonary assessment. EDI's age at Pulmonary Event.
CHOP INTEND vs. Age – January 20, 2017

**COHORT 1 (n=3)**
- Baseline Age (months): 5.9 [median], 6.3 [mean]
- Current Age (months): 30.8 [median], 30.4 [mean]
- Mean CHOP INTEND Increase: 7.7 points

**COHORT 2 (n=12)**
- Baseline Age (months): 3.1 [median], 3.4 [mean]
- Current Age (months): 20.2 [median], 20.7 [mean]
- Mean CHOP INTEND Increase: 24.7 points

Early intervention and close appear to affect response

Dashed line denotes missed or partial assessments
SAFETY AND TOLERABILITY OBSERVATIONS

- No new treatment-related SAEs or AEs observed
- As previously reported, a total of 5 treatment-related AEs in 4 patients have been reported following monitoring and source verification
  - Treatment-related SAEs and AEs were **clinically asymptomatic** elevated liver function enzymes (LFEs) assessed under CTCAE on the basis on laboratory values and **resolved with prednisolone treatment**
    - 2 were SAEs experienced by 2 patients
    - 3 were AEs experienced by 2 patients
- A total 256 AEs (5 treatment-related AEs and 251 non-treatment related AEs) have been reported following monitoring and source verification
  - 52 SAEs and 204 non-serious AEs
  - 65 AEs have occurred since September 15, 2016
    - 10 disease-related SAEs in 3 patients have occurred since September 15, 2016

*No drug-induced liver injury (DILI) as defined by Hy's Law*
Children with SMA Type 1 Never Sit Unassisted

The Natural History of SMA Type 1 is marked by the inability to achieve or maintain developmental milestones.

<table>
<thead>
<tr>
<th>Disease Characteristics</th>
</tr>
</thead>
<tbody>
<tr>
<td>Disease onset &lt;6 months</td>
</tr>
<tr>
<td>Hypotonia and weakness</td>
</tr>
<tr>
<td>Bulbar muscle weakness</td>
</tr>
<tr>
<td>Difficulty breathing and swallowing</td>
</tr>
<tr>
<td>Inexorable progression to nutritional failure</td>
</tr>
<tr>
<td>Inexorable progression to respiratory failure</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Developmental Milestone Prognosis</th>
</tr>
</thead>
<tbody>
<tr>
<td>Progressive decline in motor function soon after birth</td>
</tr>
<tr>
<td>Rapid loss of any early milestones (e.g. head control, hands to mouth)</td>
</tr>
<tr>
<td>Will never be able to sit unassisted</td>
</tr>
<tr>
<td>Will never be able to roll</td>
</tr>
<tr>
<td>Will never be able to crawl, stand, or walk</td>
</tr>
</tbody>
</table>
Motor Milestone Achievement Assessed and Adjudicated by Independent External Reviewer – January 20, 2017

<table>
<thead>
<tr>
<th>Cohort 2</th>
<th>Age of GI (mos)</th>
<th>Motor Milestone Achievement</th>
<th>Sitting with assistance</th>
<th>Sitting Unassisted</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td>brings hand to mouth</td>
<td>head control</td>
<td>partial roll</td>
</tr>
<tr>
<td>E.04</td>
<td>6</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.05</td>
<td>4</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.06</td>
<td>2</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.07</td>
<td>4</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.08</td>
<td>8</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.09</td>
<td>5</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.10</td>
<td>1</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.11</td>
<td>2</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.12</td>
<td>3</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.13</td>
<td>1</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.14</td>
<td>4</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>E.15</td>
<td>2</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
</tbody>
</table>

Two children stand with support, and stand and walk independently.

- Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back in only one direction.
- Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back to both left and right.
- 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 (HFMA-E).
- Sitting unassisted for ≥10 seconds is in accordance with the criteria of item 26 in the Bayley Scales of Infant and Toddler Development – gross motor subtest.
Financial Position (dollars in millions)

Cash and cash equivalents of $240.4 million as of December 31, 2016

<table>
<thead>
<tr>
<th></th>
<th>Three months ended 12/31/16</th>
<th>Three months ended 12/31/15</th>
</tr>
</thead>
<tbody>
<tr>
<td>R&amp;D Expense</td>
<td>$18.3</td>
<td>$8.7</td>
</tr>
<tr>
<td>G&amp;A Expense</td>
<td>$7.2</td>
<td>$4.4</td>
</tr>
<tr>
<td>Net Loss</td>
<td>$25.4</td>
<td>$13.2</td>
</tr>
</tbody>
</table>
Company Milestones

**2017**

- **Q1 2017:** Provide update on EU regulatory pathway and trial design feedback
- **Q2 2017:** Anticipated update/minutes from CMC Type B meeting
- **Q2 2017:** Initiate Phase 1 safety and dosing study in SMA Type 2 via intrathecal (IT) delivery*
- **2H 2017:** Initiate SMA Type 1 pivotal trial in EU*

- **Q1 2017:** 13.6 months of data for all patients in the Phase 1 trial
- **Q2 2017:** Initiate SMA Type 1 pivotal trial in U.S.*
- **Q2/Q3 2017:** Conduct a comprehensive clinical program review with EMA
- **Q2/Q3 2017:** End-of-Phase 1 meeting with FDA

* Assumes positive outcome of CMC Type B meeting
Thank You