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:

### Motor Milestone Achievement as of January 20, 2017

<table>
<thead>
<tr>
<th>Cohort 2 2.0e14 vg/kg</th>
<th>Age at Gene Transfer (mos)</th>
<th>Brings Hand to Mouth</th>
<th>Head Control</th>
<th>Partial Rolla</th>
<th>Rollb</th>
<th>Sitting with Assistance</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>
</tr>
<tr>
<td>E.05</td>
<td>4</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>E.06</td>
<td>2</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>E.07</td>
<td>4</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>E.08</td>
<td>8</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>
</tr>
<tr>
<td>E.10</td>
<td>1</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>E.11</td>
<td>2</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>E.12</td>
<td>3</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>E.13</td>
<td>1</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td></td>
<td>X</td>
</tr>
<tr>
<td>E.14</td>
<td>4</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></td>
<td></td>
</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, development and manufacturing 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>
<th>Three Months Ended December 31,</th>
<th>Year Ended December 31,</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2016</td>
<td>2015</td>
</tr>
<tr>
<td>Revenue</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Total revenue</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>

**Operating Expenses:**

<p>| | | | | |</p>
<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<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>
</tr>
<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>
</tbody>
</table>

**Other (Income)/Expense:**

<p>| | | | | |</p>
<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Other income</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>
</tbody>
</table>
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.

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**Balance Sheet Information:**

<table>
<thead>
<tr>
<th>Section</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>

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**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.

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**Facts & Figures:**

- **Total Other (Income) Expense:** $(172,452) $(4,718) $(402,765) $(99,128)
- **Net loss:** $(25,374,931) $(13,160,807) $(83,011,804) $(38,473,844)
- **Weighted-average basic and diluted common shares outstanding:** 27,678,348 7,226,122 22,647,583 7,087,618
- **Basic and diluted net loss per common share:** $(0.92) $(1.82) $(3.67) $(5.43)
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.

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