

## Community Letter Europe, February 9<sup>th</sup>, 2018

Dear Members of the SMA Community,

This is a response to a request from SMA Europe to share an update on the ongoing AveXis clinical development program.

We recently announced our plans to start three new clinical trials ([STR1VE EU](#), [SPRINT](#), [REACH](#)) in addition to our ongoing US clinical trials ([STR1VE](#), [STRONG](#)).

We have now initiated screening in the US for the remaining patients to be enrolled in the pivotal trial of AVXS-101 for SMA Type 1, known as STR1VE, following FDA review of safety data and early signals of efficacy from the first three patients dosed in the pivotal trial of AVXS-101 for spinal muscular atrophy (SMA) type 1.

For an overview of the studies planned for Europe later this year, please see the summary below.

If you have questions about our studies, please contact us at [medinfo@avexis.com](mailto:medinfo@avexis.com).

The AveXis Team

## Overview of AVXS-101 Clinical Development Program

### *Ongoing AVXS-101 Studies in the US:*

#### **STR1VE**

- **OVERVIEW:** STR1VE is an ongoing study of AVXS-101 in patients with SMA Type 1, studied at multiple centers across the U.S, which began enrolling in September 2017
- **ADMINISTRATION:** In STR1VE, AVXS-101 is given one-time through a needle inserted into a vein, known as an intravenous (IV) infusion.
- **WHO:** STR1VE will enroll a minimum of 15 patients with SMA Type 1 who are less than six months of age at the time of gene therapy, and who have one or two copies of the SMN2 backup gene and bi-allelic SMN1 gene deletion or point mutations.

#### **STRONG**

- **OVERVIEW:** STRONG is an ongoing study of AVXS-101 in patients with SMA Type 2, studied at multiple centers across the U.S.
- **ADMINISTRATION:** In STRONG, AVXS-101 is given one time through what is known as an intrathecal (IT) injection near the lower end of the spinal cord. STRONG is the first time AVXS-101 is being administered this way. Data from STRONG will help us understand how to best design the planned study, REACH.
- **WHO:** STRONG will enroll 27 infants and children who are symptomatic with a genetic diagnosis consistent with SMA, including the bi-allelic deletion of SMN1 and three copies of SMN2 without the SMN2 genetic modifier. Patients enrolled in STRONG must be able to sit but can't stand or walk, and cannot have previously been able to stand or walk.

### *Planned European and international Studies*

#### **STR1VE EU**

- **OVERVIEW:** STR1VE EU is expected to start enrolling patients in the first half of 2018 in patients with SMA Type 1, studied at multiple centers across the European Union.
- **ADMINISTRATION:** In STR1VE EU, AVXS-101 is administered through a one-time IV infusion.
- **WHO:** STR1VE EU will enroll approximately 30 patients with SMA Type 1 who are less than six months of age at the time of gene therapy.

#### **SPRINT**

- **OVERVIEW:** SPRINT is expected to start enrolling patients in the first half of 2018 in pre-symptomatic patients with SMA Types 1, 2 and 3.
- **ADMINISTRATION:** In SPRINT, AVXS-101 is administered through a one-time IV infusion.
- **WHO:** SPRINT is expected to enroll approximately 44 patients with two, three and four copies of SMN2 who are less than six weeks of age and pre-symptomatic at the time of gene therapy.

## REACH

- **OVERVIEW:** REACH is expected to start enrolling patients late in 2018 or early in 2019 in patients with SMA Types 1, 2 and 3.
- **ADMINISTRATION:** In REACH, AVXS-101 is administered through a one-time IT injection. Data from STRONG (the first study of AVXS-101 delivered through IT injection) will help determine the final study design.
- **WHO:** REACH is expected to enroll approximately 50 patients with SMA Types 1, 2 and 3 who are between approximately six months and 18 years of age.

### About AVXS-101

AVXS-101 is a proprietary gene therapy candidate of a one-time treatment for SMA Types 1 and 2, 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 is also designed to target motor neurons, providing rapid onset of effect and crossing the blood brain barrier to allow 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 the pivotal phase of study for the treatment of SMA Type 1, and a Phase 1 trial for SMA Type 2. The company also intends to expand its development of gene therapy into two additional rare neurological monogenic disorders: Rett syndrome (RTT) and a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1) gene.

For additional information, please visit [www.avexis.com](http://www.avexis.com).