May 8, 2018

Dear SMA Community,

In response to your request for an update, please find below more information on the ongoing clinical development program.

AveXis, the gene therapy company developing a new approach to treat SMA known as AVXS-101, recently treated the first patient in a new study known as SPR1NT. SPR1NT is a multi-national study that will evaluate AVXS-101 in approximately 44 patients less than six weeks old with SMA Types 1, 2 or 3 who have 2, 3 or 4 copies of *SMN2* – often called the SMA back-up gene – and have not yet shown symptoms of the disease, also referred to as being "pre-symptomatic."

As with all our studies, the intention of SPR1NT is to further our understanding of both the safety of AVXS-101, and how well AVXS-101 may work in SMA patients. This study is specifically to look at patients who are pre-symptomatic and less than 6 weeks old. We are excited by the progress we are making, bringing us ever closer to our goal of making AVXS-101 available to patients and their families.

For more information regarding the study, please read our press release here or view the listing on ClinicalTrials.gov. If you have any questions about SPR1NT, please contact us at medinfo@avexis.com.

Sincerely,

The AveXis Team

**ABOUT SPR1NT**

- **OVERVIEW**: SPR1NT is a multi-national trial in pre-symptomatic patients with SMA Types 1, 2 and 3 who have 2, 3 or 4, copies of *SMN2* and are less than 6 weeks old.
- **ADMINISTRATION**: In SPR1NT, AVXS-101 is administered through a one-time intravenous (also known as IV) infusion.
- **WHO**: SPR1NT will enroll approximately 44 patients with two, three or four copies of *SMN2* who are less than six weeks of age and pre-symptomatic at the time of gene therapy administration.