26 September 2018

Dear SMA Community,

AveXis, now a Novartis company, is investigating a gene replacement therapy for the treatment of SMA with the goal of addressing the underlying cause by functionally replacing the lost or defective *SMN1* gene. This is a response to your request for an update regarding our ongoing clinical development program.

AveXis recently initiated a Phase 3 study in Europe, called STR1VE-EU, that will evaluate the safety and efficacy of a one-time intravenous infusion of AVXS-101 in approximately 30 patients with SMA Type 1. This is a multi-national trial covering 16 centers in eight countries: Italy, UK, France, Spain, The Netherlands, Sweden, Belgium and Germany. Patients enrolled in the study must be less than six months old and have one or two copies of the SMN back-up gene, known as the *SMN2* gene. Centers in UK and Italy are enrolling patients. More information about the trial and eligibility criteria can be found at www.studysmanow.com. We expect to report data from STR1VE-EU at a future medical congress.

See the table below for information about the full clinical development program.

For more information regarding our trials, please view the listings on ClinicalTrials.gov. If you have any questions about the clinical development program, please contact us at medinfo@avexis.com.

Sincerely,

The AveXis Team

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# Overview of AVXS-101 Clinical Development Program (as of September 2018)

| Study Name | Where | Who | Administration | Status |
|---|---|---|---|---|
| STR1VE | U.S. | • 20 patients with SMA Type 1<br>• Less than six months of age | Intravenous (IV) infusion | Enrollment Complete |
| STR1VE-EU | Europe | • 30 patients with SMA Type 1<br>• Less than six months of age | IV | Enrolling – Italy and UK currently activated |
| SPR1NT | Global | • 44 patients with two, three and four copies of *SMN2*<br>• Less than six weeks of age and pre-symptomatic | IV | Enrolling – US, Canada and Australia currently activated.<br>EU application reviews ongoing |
| STRONG | U.S. | • 27 infants and children who are symptomatic with the bi-allelic deletion of *SMN1* and three copies of *SMN2* without the *SMN2* genetic modifier<br>• Older than six months and less than five years old | Intrathecal (IT) injection | Enrolling |
| **REACH** | Global | • Data from STRONG (the first study of AVXS-101 delivered through IT injection) will help determine the final study design | IT | Planned |