RESULTS FROM A RELATED STUDY

AVXS-101 Clinical Trial for Patients With Spinal Muscular Atrophy (SMA) Type 1

As you consider enrolling your child in this clinical trial, we would like to share an overview of the results from a previous study conducted in children who have SMA Type 1.

Clinical Trial Purpose

AveXis studied 15 infants with SMA Type 1

AveXis sponsored a clinical trial of its gene replacement therapy, AVXS-101, in children with SMA Type 1.

The study took place from May 2014 through December 2017. Fifteen children with SMA Type 1 who were 9 months of age and younger participated in the clinical trial.

AVXS-101 is a one-time treatment. It was given to the participating children through an intravenous (IV) infusion. The gene replacement therapy aims to replace a faulty or missing survival motor neuron 1 (*SMN1*) gene, the underlying cause of SMA.

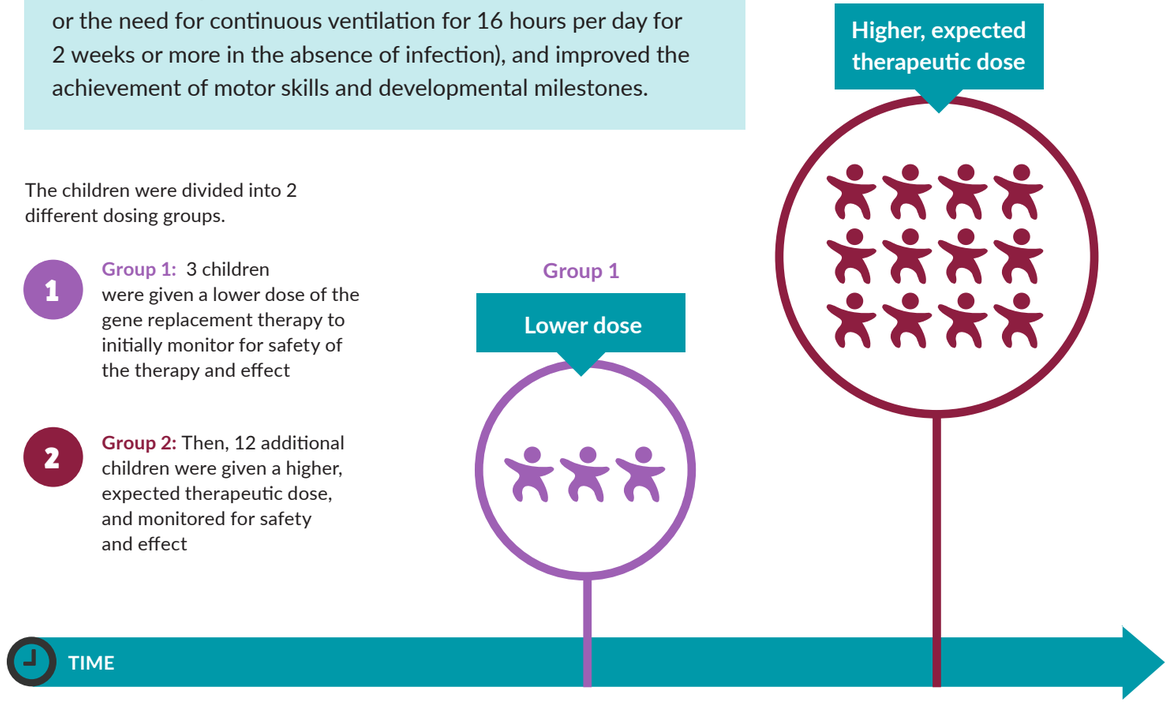
The children were monitored up to 13.6 months of age to see if the medication had its intended effect. They will continue to be monitored for safety of the treatment for up to 2 years after receiving AVXS-101.

The study was done to see if AVXS-101 was safe and well tolerated, delayed time to "events" (which were defined as death or the need for continuous ventilation for 16 hours per day for 2 weeks or more in the absence of infection), and improved the achievement of motor skills and developmental milestones.

The children were divided into 2 different dosing groups.

1. **Group 1:** 3 children were given a lower dose of the gene replacement therapy to initially monitor for safety of the therapy and effect

2. **Group 2:** Then, 12 additional children were given a higher, expected therapeutic dose, and monitored for safety and effect

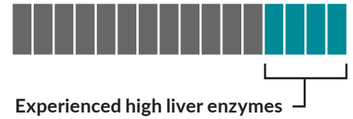


Clinical Trial Results

SAFETY

AVXS-101 appears to be safe and generally well tolerated in children studied to date.

4 of the 15 children experienced higher than normal levels of liver function enzymes. There were no symptoms associated with these elevations, and the issue was resolved by treatment with prednisolone, a steroid.

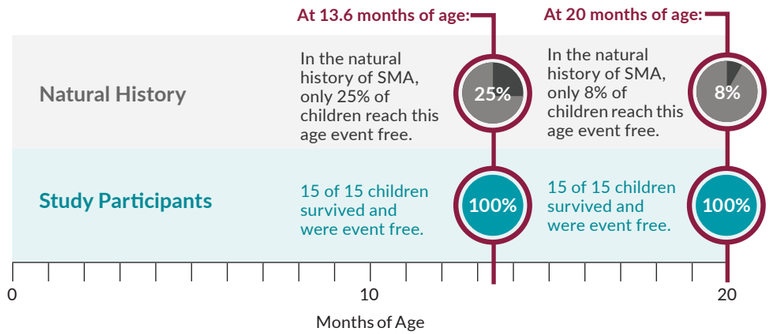


EFFECT

Survival

The children were monitored throughout the study for "events," which were defined as death or the need for continuous ventilation for 16 hours per day for 2 weeks or more in the absence of infection.

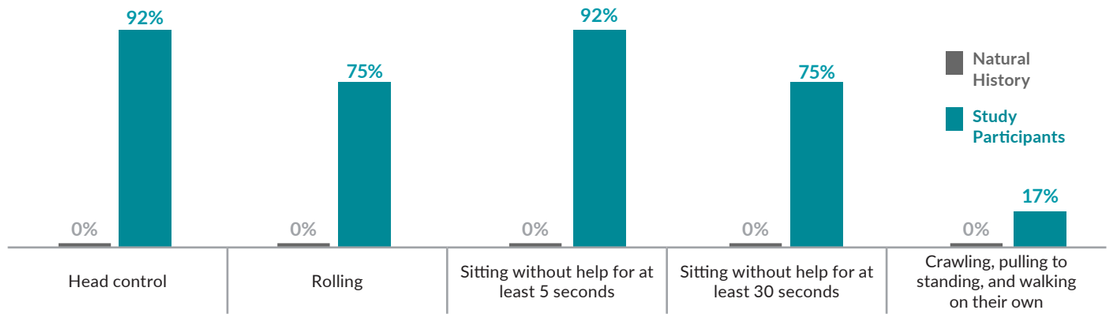
Median age of study participants at time of assessment:
Group 1: 30.7 months
Group 2: 25.7 months



Developmental Milestones

Children in group 2 of the study achieved key developmental milestones not achieved in the natural history of SMA Type 1. The study is ongoing, with the participants continuing to be monitored.

Median age of study participants at time of assessment: 25.7 months



Motor Skills

The study used the CHOP INTEND, or the Children's Hospital of Philadelphia (CHOP) Infant Test of Neuromuscular Disorders (INTEND), to measure the child's motor skills. The higher the score, the more developed the child's motor skills. The maximum score on the test is 64 points, and a typical score for children with SMA Type 1 is 20 to 22 points.

All children in the study saw an increase in their CHOP INTEND score.

11 of 12 children in group 2 exceeded and maintained a score of at least 40.

Outside of the study and in the natural course of SMA Type 1, untreated children do not score higher than 40 after 6 months of age, and most score much worse. Additionally, all children with untreated SMA Type 1 see their scores worsen after their first evaluation.

Breathing and Nutrition

11 of 12 children in group 2 feed by mouth, including **6 fed only by mouth**.

7 of 10 patients who did not require breathing support before dosing continue without any breathing support.

11 of 12 patients in group 2 are speaking.

Most patients with untreated SMA Type 1 require feeding support, and most require breathing support by 12 months of age. Also, few untreated children with SMA Type 1 achieve and maintain the ability to speak.

Sponsored by:

AVXS-101-CL-101