AveXis SMA1 Gene Therapy Trial Show Impressive Results in Most Infants

Some of the information below was copied verbatim from the SMA News Today article

Results from the first clinical trial of AVXS-101, a gene therapy for infants with spinal muscular atrophy (SMA) type 1, reveal dramatically improved survival as well as motor skills in most of the babies involved in the drug's first clinical trial.

The Phase 1 trial (NCT02122952) treated 15 infants with SMA Type 1, with the gene therapy called AVXS-101, developed by AveXis. This treatment consisted of a harmless virus that carried a functional SMN gene. The virus was injected into the bloodstream, and entered the central nervous system to deliver the gene. All children were 6 months old or younger; 12 of the 15 received a higher dose of AVXS-101, while 3 received a lower dose.

Since the trial was an early human study, safety was the main outcome measure. Researchers were also evaluating the treatment's efficacy, mainly as the time from birth to an "event." Researchers defined an event as death or at least 16 hours per day of required breathing support (ventilation) for 14 consecutive days in the absence of other reversible illnesses.

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Data presented by AveXis at the American Academy of Neurology 2017 Annual Meeting which wrapped up on April 28th in Boston, also indicated that infants treated earlier achieved developmental motor milestones more quickly. This has led researchers to urge newborn screening for SMA Type 1 as soon as treatment becomes available.

Studies also showed that antibodies against the gene therapy carrier, which could potentially render the treatment ineffective, were rare in this group.

Survival was defined as eliminating the need for permanent ventilation. It was the focus of a study which also looked at developmental milestones. Researchers analyzed data up to Sept. 15, 2016, the cutoff date. As of that date, all patients were alive, and only one, in the low-dose group, required ventilation at 28.8 months of age. At 13.6 months, none of the babies required ventilation. This is an age at which 75 percent of untreated SMA Type 1 infants have either died or are on ventilation, according to earlier studies.

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Researchers saw improvements in motor skills in the higher-dose group. SMA Type 1 patients rarely score higher than 40 in an assessment of movement ability called CHOP-INTEND. But in the trial, 11 of the 12 children in the high-dose group had higher scores, with many showing head control. In addition, 8 could sit unassisted and 2 could crawl, stand or walk independently, abilities never seen in untreated infants.

An analysis of the CHOP-INTEND scores in the high-dose group, which AveXis has proposed to be used in future trials, showed that 9 out of 12 children had a score of 50 or higher. Since it is extremely rare for SMA Type 1 infants to scores that high, the researchers analyzed how the scores corresponded to the children's ability to sit unassisted. They noted that scores of 50 or higher were more likely in babies that can sit. The treatment, they said, seemed to be more effective when given at an early age and early on in the disease course. Children treated with the higher dose at earlier time points achieved motor milestones more quickly.

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A third presentation focused on antibodies against the viral carrier. Earlier studies have noted that such antibodies are rare in children and young adults. At the start of the study, all children were screened for antibodies, with researchers finding excessive levels in 3 of the 16 infants initially screened.

2 of these infants were later retested, and had sufficiently low levels to be included in the study. Only 1 infant was excluded because of high antibody levels. Findings of initially high antibodies, which may be transferred from the mother during pregnancy or breastfeeding, often fall to lower levels, the study noted. It also suggests that antibodies will not be a major problem, or one that will prevent treatment.

AveXis has earlier released safety data from the study showing the treatment to be safe and well-tolerated. Earlier data also focused on aspects of independent sitting and other motor skills.

My Opinions Regarding These Results

In an earlier video, I made a statement where I said that the main benefactors of these new and upcoming treatments for SMA would be the children. Since they had not encountered as much muscle and bone atrophy as those of us who are adults had experienced, these new treatments would be able to counter many of the effects that SMA would have on them. While the results of this clinical trial from AveXis seem promising, those of us that are adults are wondering whether or not this potential treatment could benefit us.

QUESTION

Would the same results be achievable in adults who have SMA?

ANSWER

While the answer to this question can only be realized over time, in my opinion, I do believe that AveXis will open their AVXS-101 drug to those of us who are adults. Remember, AveXis just finished their Phase 1 trial, so it will be a while before it could be available to us, but I do believe that we will able to take this drug once it gets to the treatment stage.