The effect of Nusinersen and starting age on the improvement of motor performance in Spinal Muscular Atrophy (SMA): Preliminary results

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OBJECTIVES

Expected change in motor function assessment scores with starting age of drug can help predict overall disease progression and explain motor improvement deeply more.

MATERIAL & METHODS

Thirteen children(girl/boy:9/4) with SMA type 1, and 9 children(girl/boy:3/6) with SMA type 2 were followed up. In order to standardize comparison of the motor function change between the first and last doses at which Nusinersen was started, a total of 6 children (girls/boys:4/2) with SMA type 1 followed up with four doses and 5 children(girls/boys:2/3) with SMA type 2 followed up to six doses were selected. The correlation between Nusinersen starting age and improvement were analyzed by using Hammersmith Functional Motor Scale-Extended(HFMSE) and CHOP.

RESULTS

The mean age of the participants was 45.66±16.82 months for SMA type 1 and 116.4±38.24 months for SMA type 2. The mean change of CHOP between 1st and 4th (n=6) dose was 11.33±9.68 in the direction of increase($p<0.035$). The mean change of HFMSE between 1st and 6th(n=5) dose mean was 9.00±10.29 in the direction of increase($p>0.05$). The highest change was between 3rd and 4th in SMA Type 1(4.50±5.46), in SMA Type 2 it was between 1st and 2nd was(5±12.26). No significant correlation was found between the starting age of the drug and the scores in both types($p>0.05$).

Dose differences	SMA Type 1 Mean±SD	SMA Type 2 Mean±SD
1-2	5±12.26	3.33±5.95
2-3	3.20±3.76	3.62±2.06
3-4	2±2.91	4.50±5.46
4-5	2.40±3.28	2.75±5.50
5-6	-0.40±1.81	-1±5.83
6-7	-	3.50±5.19
7-8	-	-0.66±0.57
8-9	-	2±0

CONCLUSIONS

While Nusinersen causes a higher score increase in SMA type 2 at earlier doses, the increase occurs at more advanced doses in SMA type 1. Starting age are needed to be investigated with more detailed assessment tools and more participants.

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