



October 5th, 2020

Dear SMA Community,

Following your request, we would like to share an update on the findings of the recent nusinersen study in adults with SMA, titled Nusinersen safety and effects on motor function in adult spinal muscular atrophy type 2 and 3, published in the Journal of Neurology, Neurosurgery, and Psychiatry. This was an independent study that wasn't funded by Biogen.

This large, real-world study demonstrated that type 3 patients treated with nusinersen gained significant improvements in motor function during the 14 –month study. The most relevant treatment effect was seen in those patients who were unable to walk without assistance at the start of treatment.

Conducted in Italy, the study included 103 patients with SMA type 3 and 13 patients with SMA type 2, representing the largest group of adult type 3 patients investigated to date. Patients were assessed using three scales: HFMSE (Hammersmith Functional Motor Scale Expanded), RULM (Revised Upper Limb Module) and the 6MWT (6-Minute Walk Test).

For patients with SMA type 3, improvements increased cumulatively over the study, with 70% of patients showing clinically meaningful improvements in motor function by month 14. Examples of the motor functions assessed included their ability to lift their arms, walk and climb stairs. Lung function was also significantly improved in type 3 patients at 14 months.

For those with SMA type 2, clinically meaningful improvements were seen in 60% of patients at 14 months. However, due to the small number of type 2 patients in the study, definitive conclusions couldn't be reached in this group (i.e. this trend was not 'significant', meaning that it may have been a result of random chance). However, considering that deterioration of motor control is often expected in patients with severe or advanced disease, the authors suggest that stabilisation of motor function can be considered as a positive effect.

No new safety concerns were identified during the study.

We remain available to address any further questions.

Best Regards,

The Biogen SMA Team