

INTHENews

Spinraza Is Safe and Effective in Adults

A new paper published recently in *The Lancet Neurology* provides evidence that nusinersen (Spinraza) is safe and effective in adults with **spinal muscular atrophy (SMA)**. Although nusinersen was approved by the US Food and Drug Administration (FDA) in 2016 for use in SMA patients of all ages, clinical trials have typically focused on infants and children. In this article titled, "Nusinersen in adults with 5q spinal muscular atrophy: a non-interventional, multicentre, observational cohort study," Dr. Tim Hagenacker and colleagues examined the safety and efficacy of intrathecal nusinersen treatment in an observational cohort study across 10 clinical sites.

Meaningful improvement

Very little data have been generated evaluating the efficacy of nusinersen in adult SMA patients. In one prospective single center study, Maggie Walter and colleagues discovered a mild effect of nusinersen following 10 months of treatment in SMA type 3 patients. Nusinersen was well tolerated and did significantly improve scores on the Six-Minute Walk Test, despite a relatively small sample size. The multicenter approach at 10 clinical sites taken by Hagenacker et al. yielded almost ten-fold more patients that were followed for a longer period.

Using the Hammersmith Functional Motor Scale Expanded (HFMSE) as a primary outcome measure, adults treated with nusinersen saw significantly increased HFMSE scores compared with baseline. Analyses were performed at 6 months, 10 months, and 14 months, with more marked improvements seen at the later time points. Clinically meaningful improvements, defined as ≥ 3 point increase on the HFMSE, were observed in 28% of 124 patients at 6 months, 35% of 92 patients at 10 months, and 40% of 57 patients at 14 months. Importantly, no serious adverse events were reported at the 14-month follow-up, with the most common side effects being headache (35% of patients), back pain (22% of patients), and nausea (11% of patients).

As the authors indicate, "Patients with SMA type 2 or 3 reach adulthood with varying states of motor dysfunction and with slow but ongoing disease progression ... In this study, numerous patients showed clinically meaningful improvements in motor function or showed



stabilisation of the disease, independent of age." The authors go on to say that "these data also strongly encourage clinicians to document natural history data for this age group, when available, as these data could help to further identify the trajectories of progression in treated versus untreated patient. A small improvement could still be clinically meaningful in patients who are at risk of showing deterioration over 12 months, as suggested by the few natural history data available in adults; however, findings should be confirmed in a larger natural history cohort study."

Read the full article [here](#).

An important study

This is the first study to examine nusinersen treatment in a multicenter, real-world population of adult SMA patients, and although the absence of a control group is a limitation, there is a clear beneficial effect of the treatment with a good safety profile. It also highlights the importance of tracking disease progression across the lifespan in SMA, especially as more patients are receiving treatments. These results complement the strong body of evidence for nusinersen safety and efficacy in children with SMA and support continued nusinersen use in adult patients.