IONIS™ InBrief
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Lancet Neurology:
Teens and adults on SPINRAZA® showed clinically meaningful improvement in motor function

Findings of independent study underscore therapy's durability and established long-term safety profile

SPINRAZA® (nusinersen) remains the global foundation of care for treatment of patients living with all forms of spinal muscular atrophy (SMA); over three years of real-world data have proven Spinraza's safety and efficacy in treating pediatric and adult patients. Now, a new independent study adds to that impressive track record, showing that treatment with Spinraza led to clinically meaningful improvement in motor function in teen and adult SMA patients.

The observational cohort study of 139 teens and adults published in Lancet Neurology is the largest study of Spinraza in teens and adults to date. It found that treatment with Spinraza was associated with statistically significant increases in total Hammersmith Functional Motor Scale Expanded (HFMSE) scores, compared to baseline, at six, 10 and 14 months of treatment. Clinically meaningful improvement – an increase of at least three points in the Hammersmith score – was observed in 40 percent of patients at the 14-month assessment. No new safety concerns were identified in the study and no serious adverse events were reported.

The first treatment approved for SMA in infants, children and adults, Spinraza is available in over 50 countries and approximately 10,800 patients have been treated with Spinraza globally.

KEY TAKEAWAY

This observational study provides evidence for the safety and efficacy of nusinersen in a large real-world cohort of adult patients with 5q spinal muscular atrophy. In this study, numerous patients showed clinically meaningful improvements in motor function or showed stabilization of the disease, independent of age.

The study was conducted at 10 academic clinical sites in Germany between July 13, 2017 and May 1, 2019. In all patients, 12 mg of nusinersen was administered intrathecally on days one, 14, 28 and 63 with repeated maintenance injections in accordance with the label. The primary endpoint was the change from baseline in the total HFMSE score. Secondary endpoints were the change from baseline to months six, 10 and 14 in the Revised Upper Limb Module (RULM) score, measuring improvement in arm function, and the six-minute walk test. The results show the safety and efficacy of treatment with Spinraza in adult SMA patients, with statistically significant improvements in motor function at all timepoints of the study.

Researchers noted that the main limitation to their study was the lack of a control group. However, they went on to point out that the need for a control group was not warranted because Spinraza was already approved for the treatment of a severe, chronic progressive disease without limitations related to age or disease classification. They conclude by encouraging future studies of Spinraza to focus on long-term effects of the therapy as well as other motor and motor-related functions such as swallowing and ventilation, and possible individualized treatments, i.e., dosing regimen.