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ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

Switching to Risdiplam Maintains Motor Gains in SMA Patients

Ryan Quigley:
Welcome to *AudioAbstracts* on ReachMD. I'm Ryan Quigley, and today, I'll be discussing a multicenter real-world study evaluating the transition from nusinersen to risdiplam in patients with spinal muscular atrophy. This study was recently published in *Frontiers in Pediatrics*.

For context, spinal muscular atrophy, or SMA, is a progressive genetic motor neuron disease caused by mutations in the *SMN1* gene. Disease-modifying therapies have transformed care for this disease: nusinersen, an intrathecal antisense oligonucleotide, and risdiplam, an oral small molecule that modifies *SMN2* splicing, are both approved in China. However, in practice, patients and clinicians are often faced with a practical question: what happens when someone switches from nusinersen to risdiplam?

That's the clinical gap this study aimed to address.

This was a retrospective analysis across four medical centers in the Jiangsu Province of China. Investigators identified 11 patients with 5q-associated SMA who transitioned from nusinersen to risdiplam. The cohort included patients with type 1, type 2, and type 3 disease. Median age at starting nusinersen was nine years, and the median age at risdiplam initiation was 11 years. The median follow-up after switching was eight months.

Most patients switched for pragmatic reasons. Intrathecal injections are challenging, particularly in those with scoliosis or prior spinal surgery. Some families cited cost differences or treatment convenience. Notably, there was no standardized washout period. The median interval between therapies was zero days, and some patients even had brief overlap.

Motor outcomes were assessed using the Hammersmith Functional Motor Scale–Expanded, or HFMSE, and the Revised Upper Limb Module, known as RULM.

So what changed after the switch?

On the HFMSE, mean scores showed a gradual upward trend over eight months, though differences were not statistically significant. A few patients achieved clinically meaningful gains, defined as at least three points, while a small number experienced declines. Overall, gross motor function appeared largely maintained.

Upper limb function told a clearer story. RULM scores improved significantly compared with pre-treatment baseline and remained stable to modestly improved after the switch. At four months post-switch, seven patients achieved clinically meaningful gains of at least two points. Importantly, no patients showed clinically significant upper limb decline at eight months.

In terms of safety, no serious adverse events were reported during follow-up. One patient experienced weight loss, decreased appetite, and hair loss. No new safety signals emerged, though adverse event reporting relied on retrospective chart review.

There are important limitations here. The sample size was small, given the rare disease. There was no control group. Follow-up was relatively short. And functional assessments across centers may introduce variability, although each patient was evaluated by the same assessor before and after switching.

Still, the signal is consistent with emerging data from other switch studies. In this real-world cohort, transitioning from nusinersen to risdiplam did not lead to loss of motor function. Upper limb function in particular showed sustained or incremental gains.

For clinicians, this offers practical reassurance. In patients facing procedural burden, spinal access challenges, or financial constraints, switching therapies appears feasible and generally safe in the short term.

As more patients live longer with SMA and treatment sequencing becomes more common, understanding real-world outcomes will only grow in importance.

This has been an *AudioAbstract*, and I'm Ryan Quigley. To access this and other episodes in our series, visit ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!

Reference:

Cheng X, Ma Y, Yu LQ, et al. Treatment of risdiplam after nusinersen continuously improves upper limb motor function in spinal muscular atrophy patients: a multicenter experience. *Front Pediatr*. 2026;14:1679549. Published 2026 Jan 26. doi:10.3389/fped.2026.1679549