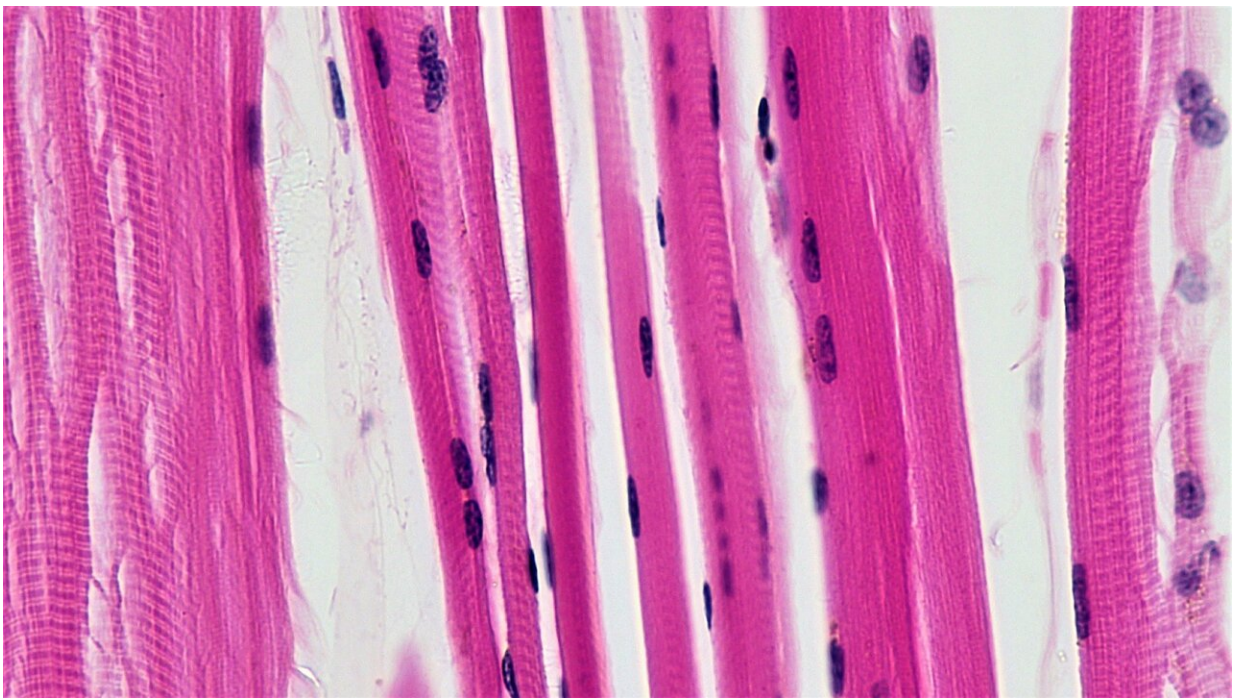


Safety and efficacy data published for novel nusinersen drug delivery method for spinal muscular atrophy patients

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Skeletal muscle fibers. Credit: Berkshire Community College Bioscience Image Library / Public domain

A recently published paper details the safety and efficacy of nusinersen administration via a subcutaneous intrathecal catheter system (SIC) for SMA patients with advanced disease. SMA is a devastating genetic

disease that leads to progressive degeneration of motor neurons that control movement, swallowing, and breathing. The novel SIC system is comprised of an intrathecal catheter that's connected to an implantable infusion port. The SIC system was created to address the complications of nusinersen delivery derived from significant spinal deformities or fusions commonly found in SMA patients.

In the safety study, seventeen (17) participants started therapy between 2.7 and 31.5 years of age and received between 9 and 12 doses of nusinersen via SIC. A separate efficacy study included a subgroup of 11 participants with three copies of SMN2 who were nonambulatory, treatment-naïve, and had complex spine anatomy.

A total of 14 treatment-related adverse events occurred among 12 of the participants. All were related to the SIC and not nusinersen. Four SICs required surgical revision due to mechanical malfunction with or without cerebrospinal fluid leak and one was removed due to a meningitis infection.

Improvements were observed in mean performance on the nine-hole peg test in dominant (15.9%) and nondominant (19.0%) hands and grip strength increased by 44.9%. No significant changes were observed in motor scales, muscle force, lung function, or SMA biomarkers.

The SIC system allows patients, even those with complex spine anatomy, to receive nusinersen treatments in an outpatient setting. It has a technical success rate similar to that of more complex and costly CT-guided administration strategies, but is subject to mechanical failures and infection. For SMA patients with advanced disease, these risks can be balanced against an expectation of small but functionally meaningful improvements of arm and hand function.

The research was published in *Muscle & Nerve*.

More information: Vincent J. Carson et al, Nusinersen by subcutaneous intrathecal catheter for symptomatic spinal muscular atrophy patients with complex spine anatomy, *Muscle & Nerve* (2021). DOI: [10.1002/mus.27425](https://doi.org/10.1002/mus.27425)

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