New nusinersen drug delivery method identified for spinal muscular atrophy patients

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STRASBURG, PA- A new report has identified an alternative method to deliver nusinersen to patients with spinal muscular atrophy (SMA) using a subcutaneous intrathecal catheter system (SIC) configured by connecting an intrathecal catheter to an implantable infusion port. SMA is a devastating genetic disease that leads to progressive degeneration of motor neurons that control movement, swallowing, and breathing. It is the leading genetic cause of infant death worldwide. Nusinersen is the first FDA approved therapy for SMA but must be administered into the cerebrospinal fluid by repeat lumbar puncture every 4 months for life. Unfortunately, the majority of surviving SMA patients have skeletal deformities or spinal hardware that make it difficult to safely and reliably access the cerebrospinal fluid.

The study, by clinicians and researchers at the Clinic for Special Children in Strasburg, PA and the Nemours/A.I. duPont Hospital for Children in Wilmington DE, appears in the *Journal of Pediatric Orthopaedics*. Ten SMA patients underwent implantation of the catheter device and received nusinersen dosing through the SIC. The device implantation took less than two hours and was well tolerated in all patients, with an average hospital stay of less than 55 hours. Once the SIC system was implanted, all subsequent nusinersen doses were administered in less than 20 minutes, requiring only topical anesthetic in an outpatient setting. SIC implantation significantly reduced the cost of drug administration.

This SIC dosing method might also benefit SMA patients without advanced neuromuscular disease or spinal pathology; repeated lumbar puncture can prove challenging for any SMA patient, especially newborns and young children, who have the highest rate of traumatic complications (20-50%) when nusinersen is administered by lumbar puncture.

While the study's observations suggest the SIC system to be relatively safe and without complications, use of the device warrants further multicenter trials. If proven effective in future studies, this method could double the number of patients able to receive nusinersen worldwide and reduce costs five- to ten-fold.

More information: Kevin A. Strauss et al, Preliminary Safety and Tolerability of a Novel Subcutaneous Intrathecal Catheter System for Repeated Outpatient Dosing of Nusinersen to Children and Adults With Spinal Muscular Atrophy, *Journal of Pediatric Orthopaedics* (2018). DOI: 10.1097/BPO.0000000000001247

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