



Contact: Kelly Cullen  
P | 717-687-9407  
E | [kcullen@clinicforspecialchildren.org](mailto:kcullen@clinicforspecialchildren.org)

**FOR IMMEDIATE RELEASE**

## **SAFETY AND EFFICACY DATA PUBLISHED FOR NOVEL NUSINERSEN DRUG DELIVERY METHOD FOR SPINAL MUSCULAR ATROPHY PATIENTS**

STRASBURG, PA – 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.



# Clinic for Special Children

535 Bunker Hill Road, PO Box 128, Strasburg, PA 17579 T 717.687.9407 F 717.687.9237

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 conducted by a team including the study's first author Vincent J. Carson, MD, Millie Young, RNC, Karlla W. Brigatti, MS, CGC, Donna L. Robinson, CRNP, from the Clinic for Special Children, Strasburg, PA; Robert M. Reed, MD, from University of Maryland School of Medicine, Baltimore, MD; Jihee Sohn, PhD, Marco Petrillo, PhD, Wildon Farwell, MD, MPH, from Biogen, Cambridge, MA; Freeman Miller, MD, from Department of Orthopedics, Sidney Kimmel Medical College at Thomas Jefferson University, Philadelphia, PA; and Kevin A. Strauss, MD from the Clinic for Special Children, Strasburg, PA.

This study was funded in part by a grant from Biogen, the manufacturer of nusinersen. J.S., M.P., and W.F. were employed by Biogen throughout the conduct of the study and own stock options in the company.

## **About the Clinic for Special Children**

The Clinic for Special Children (CSC) is a non-profit organization located in Strasburg, PA, which provides primary pediatric care and advanced laboratory services to those who live with genetic or other complex medical disorders. Founded in 1989, the organization provides services to over 1,050 individuals and is recognized as a world-leader in translational and precision medicine. The organization is primarily supported through community fundraising events and donations. For more information, please visit [www.ClinicforSpecialChildren.org](http://www.ClinicforSpecialChildren.org)