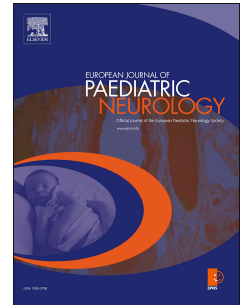


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A 5-center experience with intrathecal administration of Nusinersen in SMA1 in Italy Letter to the Editor of European Journal of Pediatric Neurology regarding the manuscript “Single-center experience with intrathecal administration of Nusinersen in children with spinal muscular atrophy type 1” written by Pechmann and colleagues”



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Letter to the Editor of European Journal of Pediatric Neurology regarding the manuscript "Single-center experience with intrathecal administration of Nusinersen in children with spinal muscular atrophy type 1" written by Pechmann and colleagues"

A 5-center experience with intrathecal administration of Nusinersen in SMA1 in Italy

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Dear Editor,

We read with great interest the experience with intrathecal administration of Nusinersen in children with SMA1 reported by the Department of Neuropediatrics and Muscle Disorders from the University of Freiburg (1). The Authors describe the protocol applied at this single site giving details on the lumbar puncture procedure, sedation and monitoring during the procedure in 20 children with SMA1.

They conclude that the lumbar puncture for intrathecal administration of Nusinersen can be performed without any relevant complications if a standard protocol is applied. This is important because while repetitive intrathecal administrations of drugs is relatively common in oncematology in children, this treatment had never been applied to the neuromuscular field prior to Nusinersen. This is even more so in children with neuromuscular disorders having respiratory muscle weakness which makes them even more fragile and susceptible to respiratory complications during anesthesia if needed.

While we strongly agree with the suggestion that details on the procedure and on the protocol are important for others involved in the treatment of SMA, we also wish to highlight that the settings and some procedural details can be different in different centers according to what is available.

As part of our experience with the EAP (Expanded Access Program) in Italy (1) Nusinersen was administered intrathecally in over 120 children from December 2016 to December 2017. Despite several commonalities (local anesthetic cream on the injection site, Quincke G22 needle, lateral decubitus position, video watching) there were site-specific differences as each Center applied the protocol which best fitted the local setting and organization. (Table 1). Additional health-operators were involved during the procedure according to each site's organization.

Despite the different settings involved (eg surgery room vs out-patient clinic), types of sedation and professionals performing the lumbar puncture, the procedure was successfully performed in all centers but

in all the settings a multidisciplinary approach was needed. While we agree with the Authors' conclusion that a standardized protocol can reduce the risks and feasibility limits of lumbar puncture in SMA1 children, we also strongly believe that this should be only performed in centres who have expertise in SMA and with a multidisciplinary setting. This information is relevant as Nusinersen is becoming now commercially available in many countries and some expertise should be guaranteed in the centers who will be designated to perform the procedures.

Table 1

	The NEMO Clinical Center-Niguarda Hospital, Milan	Istituto Giannina Gaslini, Genoa	The NEMO Clinical Center-Ospedale Gemelli, Rome	Bambino Gesù Children's Hospital, Rome	The NEMO Clinical Center – Policlinico Universitario, Messina
Setting for LP	Surgery room	Multifunctional room	Pediatric Intensive Care Unit	Pediatric Sub Intensive Care Unit	Pediatric Intensive Care Unit
Additional sedation used	Nitrous oxide	Nitrous oxide	Sevorane gas	Midazolam	Midazolam
Health-operator performing LP	Pediatrician or Neurologist or Child neurologist	Child onco-hematologist	Anesthetician	Pediatrician or Pediatric Neurologist	Anesthetician
Additional health-operators during the procedure	Respiratory physiotherapist Anesthetician as needed Nurse	Nurse, Pediatrician, Pediatric neurologist	Nurse, Pediatric neurologist, Respiratory physiotherapist	Nurse, Pediatrician, Pediatric neurologist	Nurse, Neurologist, Pyschologist

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- (1) Astrid Pechmann, Thorsten Langer, Sabine Wider, Janbernd Kirschner. Single-center experience with intrathecal administration of Nusinersen in children with spinal muscular atrophy type 1. *European Journal of Paediatric Neurology* (2017), <https://doi.org/10.1016/j.ejpn.2017.11.001>
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