Anesthetic Considerations for Spinal Muscular Atrophy Patients Undergoing Spinraza (Nusinersen) Therapy

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Background

- Spinal muscular atrophy (SMA) is a motor neuron disorder caused by a defect in the *SMN1* gene that is necessary for muscle survival
- Nusinersen (Spinraza) is the first pharmacological treatment for SMA
- Spinraza is given intrathecally and increases *SMN* protein levels by altering the splicing of *SMN2* messenger RNA

Case Series

- 52 patients were selected to receive Spinraza (Table 1)
- Ages 3 months – 36 years
- SMA types 1 – 3
- 44 patients underwent intrathecal injection of Spinraza
- Four loading doses on days 1, 15, 29, and 58 followed by doses every 120 days

Table 1. Patient Demographics

Patient Characteristic	n
Age at Initial Presentation	
3-12 months	1
1-4 years	14
5-9 years	12
10-17 years	16
>18 years	9
Sex	
Male	25
Female	27
SMA Type	
1	6
2	24
3	22

Pre-operative Process

- Multi-disciplinary evaluation by anesthesia, neurology, pulmonary
- Staggered the patients' dose schedules
- Continuity of care was provided by anesthesia nurse practioners
- Procedure location was based on anticipated difficulty of lumbar puncture (LP) and anticipated anesthetic challenge
- Patients instructed to bring their non-invasive respiratory support devices
- Clear liquids encouraged until 2 hours prior to arrival
- Before the procedure, IV placed, labs drawn to check coagulation studies and platelet count, hydration started, topical lidocaine applied

Results

- Anesthetic management varied from anesthesia standby to general anesthesia with a supraglottic airway in 3 patients
- Most patients received IV anesthetic with midazolam, propofol, or dexmedetomidine and a natural airway
- 11 patients received inhalational anesthetic for at least 1 LP
- 9 patients ages 10 years and older tolerated all LPs with only local anesthesia and midazolam
- 9 patients who initially needed anesthesia required only midazolam and a Child Life Specialist on subsequent injections
- 2 patients had a tracheostomy and 2 used home BiPAP
- No patients required escalation of care due to respiratory support
- Complications
  - Pain at injection site (6)
  - Vomiting (3)
  - Mild headache requiring only supportive care (9)
- 3 patients had unsuccessful LPs
  - 2 required Interventional Radiology
  - 1 had a previous posterior spinal fusion requiring a laminectomy to initiate therapy that was complicated by dural tear and CSF leak requiring ICU admission

Conclusions

- A comprehensive perioperative program can successfully screen and prepare SMA patients for Spinraza therapy and allocate resources efficiently
- Although SMA patients are particularly vulnerable to post-anesthesia respiratory issues, nearly all patients tolerated their brief anesthetic well and were discharged home after observation
- Many patients did well with anesthesia for their first LP, and then after they became familiar with the procedure only required anesthesia standby for subsequent LPs