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Brachial plexus enhancement in acute flaccid myelitis: A novel radiographic finding

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doi: 10.1017/cjn.2019.159

Background: Acute flaccid myelitis (AFM) is a condition which causes acute paralysis in pediatric patients. Although awareness of AFM is increasing, the pathophysiology and full spectrum of clinical, biochemical, and radiographic features remain to be fully elucidated. **Methods:** We report a 5 year-old, previously healthy, male patient who presented with acute right upper extremity weakness following a two day history of fever, cough, and fatigue. The patient underwent extensive inflammatory and infectious workup in addition to MRI imaging of the brain, spinal cord, and bilateral brachial plexuses. **Results:** Infectious and inflammatory workup did not identify a causative agent. The patient was seen to have bilateral asymmetric (R>L) thickening and enhancement of the anterior horn cells of his cervical (C3-C7) spine, consistent with the spinal grey matter lesions previously described in patients with AFM. Enhancement of the corresponding anterior nerve rootlets and bilateral brachial plexuses was also seen. **Conclusions:** Patients with acute flaccid myelitis may demonstrate grey matter enhancement extending beyond the spinal cord to the peripheral nerves and plexuses, a radiographic finding which has not previously been published.

Neuromuscular Disease and EMG

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Time to treatment effect in Spinal Muscular Atrophy Type 1 (SMA1): an indirect comparison of treatments

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doi: 10.1017/cjn.2019.160

Background: SMA1 is a rapidly progressing disease resulting in death/permanent ventilation by 2 years. This study compared clinical trial data evaluating the relationship between treatment timing, time to treatment effect, and clinical outcomes in SMA1 patients **Methods:** A post-hoc indirect treatment comparison was conducted to measure time-to-effect differences in AVXS-101 (CL-101, NCT02122952, cohort 2) vs nusinersen (ENDEAR, NCT02193074) or risdiplam (FIREFISH, NCT02913482) using CHOP-INTEND scores. **Results:** Compared with nusinersen, AVXS-101 more rapidly increased mean CHOP-INTEND score from baseline (9.8- and 14.9-point increase at 1- and 2-months post-AVXS-101 vs ≤5-point increase at 2-months post-nusinersen). Greater survival benefits and lower rates of permanent ventilatory support were also observed in AVXS-101- vs nusinersen-treated patients. Compared with risdiplam treatment, AVXS-101 improved median CHOP-INTEND scores (14.0-point increase at 2-months post-AVXS-101 vs 5.5-point increase at ~2-months post-risdiplam). Treatment differences were maintained through 8-months with additional improvements at all time-points. **Conclusions:** Although patients in these 3 cohorts are not entirely matched (e.g. age, disease severity), useful comparisons can still be made. Based on CHOP-INTEND scores, the treatment effect of AVXS-101 appears to be more rapid vs nusinersen or risdiplam. These findings suggest that timely restoration of SMN protein may be essential for maximizing outcomes in SMA1 patients.

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The value of AVXS-101 gene-replacement therapy for Spinal Muscular Atrophy Type 1 (SMA1)

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doi: 10.1017/cjn.2019.161

Background: SMA1, a rapidly progressing disease, results in muscle weakness, respiratory failure, hospitalization, and early death. This study highlights the value of onasemnogene abeparvovec (AVXS-101) gene-replacement therapy for SMA1. **Methods:** Twelve SMA1 patients received a one-time intravenous proposed therapeutic dose of AVXS-101 (CL-101; NCT02122952). Event-free survival (no death/permanent ventilation), pulmonary/nutritional interventions, swallow function, hospitalization rates, CHOP-INTEND, motor milestones, and safety were assessed (2-year follow-up). **Results:** By study end, all 12 patients survived event-free; 7 did not require non-invasive ventilation; 11 had stable/improved swallowing function (6 exclusively fed by mouth); 11 spoke. On average, patients experienced 1.4 (SD=0.41, range=0–4.8) respiratory hospitalizations/year. The mean proportion of time hospitalized was 4.4% (range=0–18.3%); mean unadjusted rate of hospitalization/year was 2.1 (range=0–7.6), with a mean hospital stay of 6.7 (range=3–12.1) days. CHOP-INTEND increased by 9.8 (SD=3.9) and 15.4 (SD=6.4) points at 1- and 3-months post-treatment. At long-term follow-up, 11 patients sat unassisted, 4 stood with assistance, and 2 walked. Adverse events included elevated serum aminotransferase levels, which were attenuated by prednisolone. **Conclusions:** AVXS-101 in CL-101 resulted in dramatic survival and motor function improvements. The reduced healthcare utilization in treated infants could decrease cost and alleviate patient, caregiver, and societal burden.

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Burden of illness of spinal muscular atrophy (SMA): an update

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doi: 10.1017/cjn.2019.162

Background: In this retrospective claims analysis, real-world healthcare resource use (HRU) and costs among SMA type 1 (SMA1) patients were assessed. **Methods:** SMA1 patients were identified from Symphony Health's Integrated Dataverse® (09/01/2016–08/31/2018). The study period spanned from the index date (date of first SMA1 diagnosis after nusinersen approval [12/23/2016]) until death/end of available data. HRU and costs per-patient-per-year (PPPY; 2018USD) were described during the study period for all