

visit, 22/24 (92%) infants had achieved WHO motor milestones sitting without support and 8/16 (50%; 2 *SMN2*, n=3/11; 3 *SMN2*, n=5/5) on study >13 months achieved walking alone. AEs were reported in 24/25 (96%) infants; most 20/25 (80%) had AEs that were mild/moderate in severity; 9 had serious AEs. Four infants had an AE possibly related to study drug, which resolved despite continued treatment. No new safety concerns were identified. **Conclusions:** Nusinersen continued to benefit infants who initiated treatment in a presymptomatic stage of SMA.

Study Support: Biogen

B.06

Safety and efficacy of nusinersen in infants/children with spinal muscular atrophy (SMA): part 1 of the phase 2 EMBRACE study

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Background: EMBRACE (NCT02462759) Part 1 is a randomized, double-blind, sham-procedure controlled study assessing safety/tolerability of intrathecal nusinersen (12-mg equivalent dose) in symptomatic infants/children with SMA who were not eligible to participate in ENDEAR or CHERISH. **Methods:** Eligible participants had onset of SMA symptoms at ≤ 6 months with 3 *SMN2* copies; onset at ≤ 6 months, age >7 months and 2 copies; or onset at >6 months, age ≤ 18 months, and 2/3 copies. Safety/tolerability was the primary endpoint. Exploratory endpoints included Hammer-smith Infant Neurological Examination Section 2 (HINE-2) motor milestone attainment, change in ventilator use, and growth. **Results:** EMBRACE Part 1 was terminated early based on positive results from ENDEAR. Safety/tolerability was similar to previous trials. More nusinersen-treated (11/14; 79%) vs. sham-treated individuals (2/7; 29%) were HINE-2 motor milestone responders. Between Day 183 and 302, mean (SD) hours of ventilator use changed by +1.236 (3.712) hours in nusinersen-treated (n=12) and +2.123 (3.023) hours in sham-treated individuals (n=7). Similar increases in weight and body length were observed in nusinersen-treated and sham-treated individuals by Day 183. **Conclusions:** In EMBRACE Part 1, nusinersen demonstrated a favorable benefit-risk profile. These results add to the aggregated efficacy, safety/tolerability data of nusinersen in SMA.

Study Supported by: Ionis and Biogen

B.07

Review of patients with Spinal Muscular Atrophy treated with Nusinersen in Ontario

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Background: Spinal Muscular Atrophy (SMA) is an autosomal recessive neurodegenerative disease. In June 2017, Health Canada approved Nusinersen, currently the only available drug for SMA. Since 2016, patients in Ontario have been treated clinically with Nusinersen through different access programs. **Methods:** Retrospective case series of patients with SMA treated clinically with Nusinersen in Ontario, describing clinical characteristics and logistics of intrathecal Nusinersen administration. **Results:** Twenty patients have been treated across four centres. To date, we have reviewed 8 cases at one centre (seven SMA Type I, one SMA Type II). Age at first dose ranged from 3-156 months and disease duration 9-166 months. Patients had received 4-7 doses at last evaluation. Three patients with scoliosis (2 with spinal rods) required fluoroscopy-guided radiologist administration, and 4 required general anesthesia. No complications/adverse events were reported. At last follow up, 5/8 families reported improved daily activities. Of 5 patients with baseline and follow up motor function testing, 3 demonstrated improved scores. One patient died due to respiratory decline at age 9 months, despite improved motor outcome scores. **Conclusions:** We describe the first Canadian post-marketing experience with Nusinersen. Timely dissemination of this information is needed to guide clinicians, hospital administrators, and policy-makers.

CNSS CHAIR'S SELECT ABSTRACTS

C.01

Endoscopic versus open microvascular decompression of trigeminal neuralgia: a systematic review and comparative meta-analysis

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Background: Microvascular decompression (MVD) is commonly used in the treatment of trigeminal neuralgia with positive clinical outcomes. Fully endoscopic microvascular decompression (E-MVD) has been proposed as a minimally invasive, effective alternative, but a comparative review of the two approaches in the literature has not been conducted. **Methods:** We performed a meta-analysis comparing patient outcome rates and complications for both techniques. From a pool of 1,039 studies, 22 articles were selected for review: 12 open MVD and 10 E-MVD. The total number of patients was 6,734. **Results:** Good pain relief was achieved in 81% of MVD and 88% of E-MVD patients, with a mean recurrence rate of 14% and 9% respectively. Average rates of complications in MVD versus E-MVD included facial paresis or weakness, 9%, 3%; hearing loss,