

of most bothersome migraine-associated symptom (MBS). **Results:** 1672 patients were randomized (safety population: n=1436; mITT population: n=1327). Mean age: 40.7 years; white (82.4%); female (87.5%). A significantly greater percentage of ubrogepant- than placebo-treated patients achieved pain freedom 2 hours post initial dose (50mg: 19.2%, adjusted  $P=0.0023$ ; 100mg: 21.2%, adjusted  $P=0.0003$ ; placebo: 11.8%). A significantly greater percentage of ubrogepant patients achieved absence of MBS (50mg: 38.6%, adjusted  $P=0.0023$ , 100mg: 37.7%, adjusted  $P=0.0023$ ; placebo: 27.8%). The adverse event (AE) profile of ubrogepant was similar to placebo. The most common AEs (incidence  $\geq 2\%$  in any treatment group) within 48 hours of initial or optional second dose were nausea, somnolence, and dry mouth (all with incidence  $< 5\%$ ). **Conclusions:** Both co-primary endpoints were met, with clinically meaningful effects on migraine headache pain and MBS. Ubrogepant was well tolerated, with no identified safety concerns.

## CHAIR'S SELECT ABSTRACTS - CHILD NEUROLOGY AND NEUROPHYSIOLOGY

### B.01

#### AVXS-101 gene-replacement therapy (GRT) for spinal muscular atrophy type 1 (SMA1): pivotal phase 3 study (STRIVE) update

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**Background:** SMA1 is a neurodegenerative disease caused by bi-allelic survival motor neuron 1 gene (*SMN1*) deletion/mutation. In the phase 1 study, *SMN* GRT onasemnogene abeparvovec (AVXS-101) improved outcomes of symptomatic SMA1 patients. We report preliminary data of STRIVE, a pivotal study (NCT03306277) evaluating efficacy and safety of a one-time intravenous AVXS-101 infusion. **Methods:** STRIVE is a phase 3, multicenter, open-label, single-arm study in SMA1 patients aged  $< 6$  months (bi-allelic *SMN1* loss, 2x*SMN2*). Primary outcomes: independent sitting for  $\geq 30$  seconds (18 months) and survival (14 months). Secondary outcomes: ability to thrive and ventilatory support (18 months). Exploratory outcomes: CHOP-INTEND and Bayley Scales of Infant and Toddler Development scores. **Results:** Enrollment is complete with 22 patients dosed. Mean age at symptom onset, genetic diagnosis, and enrollment was 1.9 (0–4.0), 2.1 (0.5–4.0), and 3.7 (0.5–5.9) months. At baseline, no patient required ventilatory/nutritional support, and all exclusively fed by mouth. Mean baseline CHOP-INTEND score was 32.6 (17.0–52.0), which increased 6.9 (-4.0–16.0, n=20), 10.4 (2.0–18.0, n=12), and 11.6 (-3.0–23.0, n=9) points at 1, 2, and 3 months; updates provided at congress. **Conclusions:** Preliminary data from STRIVE show rapid motor function improvements in SMA1 patients, paralleling phase 1 findings.

### B.02

#### Long-term neurodevelopmental outcomes in preterm twins

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**Background:** Preterm infants are at risk for adverse neurodevelopmental outcomes, however studies examining preterm twins are limited. The aim of this study was to examine whether preterm monozygotic (MZ) and dizygotic (DZ) twins have similar morbidities and long-term neurodevelopmental outcomes. **Methods:** From a cohort of 225 preterm neonates studied with MRI, 24 MZ and 52 DZ twins were included. Outcomes at 1.5-years, 3-years and 4.5-years were assessed with the Bayley-III, Movement Assessment Battery for Children and Wechsler Preschool and Primary Scale of Intelligence. **Results:** Twin pairs had substantial concordance for retinopathy of prematurity but only moderate-fair concordance for bronchopulmonary dysplasia, infection and brain injury. Differences in cognitive and language scores were stable over time, while motor differences increased. Discordant twins had significantly lower gestational age [ $Mean_1(SD)=26.7(1.38)$ ;  $Mean_2(SD)=29.1(2.1)$ ;  $P<0.001$ ] and birth weight [ $Mean_1(SD)=892.2(291.2)$ ;  $Mean_2(SD)=1208.0(289.4)$ ;  $P=0.001$ ] and a higher incidence of bronchopulmonary dysplasia and retinopathy of prematurity. In discordant twins, cognitive and language score differences decreased over time while motor differences increased. **Conclusions:** Preterm twin pairs have similar neurodevelopmental outcomes through early childhood despite poor concordance for perinatal illness. Discordant twins were born earlier and had more morbidities. Increasing concordance in cognitive and language outcomes over time may reflect the positive impact of early intervention programs.

### B.03

#### Clinical utility of critical care EEG monitoring in a Canadian paediatric centre

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**Background:** Continuous electroencephalographic (cEEG) monitoring is essential to diagnosing non-convulsive seizures (NCS), reported to occur in 7-46% of at-risk critically ill patients. However, cEEG is labour-intensive, and given scarcity of resources at most centres cEEG is feasible in only selected patients. We aim to evaluate the clinical utility of cEEG at our centre in order to optimize further cEEG allocation among critically ill patients. **Methods:** Using a clinical database, we identified critically ill children who underwent cEEG monitoring in 2016, 2017 and 2018. We abstracted underlying diagnoses, indication for cEEG monitoring, cEEG findings, and associated changes in management. **Results:** Over this three year period, 928 cEEGs were performed. Among the 100 studies analyzed to date, primary indications for monitoring were characterization of events of unclear etiology (32%), diagnosis of NCS (30%), and monitoring of therapy for seizures (17%). Seizures were captured in 31% of patients (22% subclinical only, 5% electroclinical only, 4% both),

which resulted in a treatment change in 90% of cases. Non-epileptic events were captured in 26% of patients. **Conclusions:** cEEG yielded clinically meaningful information in 57% of cases, frequently resulting in management changes. Subgroup analyses by cEEG indication and ICU location will be presented.

## B.04

### Alterations in brain structure in pediatric migraine

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**Background:** Migraine is a prevalent and disabling condition with limited understanding in the developing brain. Adults with chronic migraine show structural alterations in pain and sensory processing regions. Similar data is lacking in children and required for early intervention. **Methods:** Case-control feasibility study assessing structural brain differences between adolescents with chronic migraine and healthy controls using 3T Siemens structural volumetric MRI analysis. Fifteen subjects with chronic migraine were compared to 25 age and sex matched healthy controls. Non-parametric statistics performed (Kruskal-Wallis). **Results:** Migraine subjects had reduced volumes in total brain (grey and white matter) (KW  $p < 0.03$ ), total thalamus (KW  $p < 0.01$ ) and hippocampal regions (KW  $p < 0.03$ ). Unilateral (right) cerebellar grey matter volumes were significantly reduced in migraine subjects versus controls (KW  $p < 0.05$ ). No significant differences were found in other regions, including basal ganglia, cortical grey matter and brainstem. **Conclusions:** Total brain, hippocampal and thalamic volumetric reductions are seen in adolescents with chronic migraine. The regions identified are involved in migraine pathogenesis. This volumetric imaging study should improve understanding of the causes and effects of pediatric migraine.

## B.05

### The importance of mental health in improving quality of life in transition-aged patients with epilepsy

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**Background:** Growing evidence has that a suggested that mental health strongly influences quality of life (QoL) in adolescents with epilepsy. In addition, research has suggested that these mental health issues are associated with increased seizure burden and worsened health outcomes. Despite this, and the elevated rate of mental health issues in this population, seizure control tends to be the dominant or sole concern for treating physicians. **Methods:** In order to look at potential predictors of QoL in adolescents we looked at seizure related data, demographic variables, and comorbid conditions in 70 adolescents with epilepsy aged 14 to 18 ( $M = 16.31$ ; 37 males, 33 females) enrolled into an epilepsy transition clinic. **Results:** Regression analysis found that mental health remained a significant and independent predictor of QoL even when other significant seizure related variables were accounted for ( $t(58) = -3.44$ ,  $p = .001$ ). Furthermore, when looking at the individual subscales of patient QoL (e.g., memory, social support, stigma), mental health was consistently found to be

the strongest correlate. **Conclusions:** These results demonstrate that in order to ensure the best outcomes for transition-aged adolescents with epilepsy, it is important to not only manage and treat seizures, but also to assess and treat mental health issues.

## B.06

### Whole exome sequencing in genetic ataxias associated with cerebellar atrophy: the Canadian experience

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**Background:** Cerebellar atrophy is characterized by loss of cerebellar tissue, with evidence on brain imaging of enlarged interfolial spaces compared to the foliae. Genetic ataxias associated with cerebellar atrophy are a heterogeneous group of disorders. We investigated the prevalence in Canada and the diagnostic yield of whole exome sequencing (WES) for this group of conditions. **Methods:** Between 2011 and 2017, WES was performed in 91 participants with cerebellar atrophy as part of one of two national research programs, Finding of Rare Genetic Disease Genes (FORGE) or Enhanced Care for Rare Genetic Diseases in Canada (Care4Rare). **Results:** A genetic diagnosis was established in 58% of cases (53/91). Pathogenic variants were found in 24 known genes, providing a diagnosis for 46/53 participants (87%), and in four novel genes, accounting for 7/53 cases (13%). 38/91 cases (42%) remained unsolved. The most common diagnoses were channelopathies in 12/53 patients (23%) and mitochondrial disorders in 9/53 (17%). Inheritance was autosomal recessive in the majority of cases. Additional clinical findings provided useful clues to some of the diagnoses. **Conclusions:** This is the first report on the prevalence of genetic ataxias associated with cerebellar atrophy in Canada, and the utility of WES for this group of conditions.

## B.07

### An educational video improves consent in pediatric lumbar puncture: a randomized control trial

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**Background:** Lumbar puncture (LP) is a low-risk procedure performed on pediatric patients for a variety of indications. There are no published studies of the nature of the concerns of parents in North America, and no studies examining a process to improve pediatric lumbar puncture consent. **Methods:** 72 patient-parent dyads were enrolled in a randomized control trial to receive standard consent with or without an educational video. A survey was provided to determine parent self-rated understanding of the procedure, their perception of its safety, their perception of the painfulness and their overall comfort with their child undergoing LP. In addition, demographic characteristics and qualitative information about parent concerns were collected. **Results:** Viewing the video significantly increased parent understanding of the procedure ( $p = 0.015$ ) and their perception of its safety