

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 ≥ 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),