A.04

The classification of autosomal recessive cerebellar ataxias: a consensus statement from the society for research on the cerebellum and ataxias task force

M Beaudin (Québec) A Matilla-Dueñas (Badalona) B Soong (Taipei) J Pedroso (São Paulo) OG Barsottini (São Paulo) H Mitoma (Tokyo) S Tsuji (Tokyo) JD Schmahmann (Boston) M Manto (Charleroi) GA Rouleau (Montreal) CJ Klein (Rochester) N Dupre (Québec)*

doi: 10.1017/cjn.2019.86

Background: There is currently no accepted classification of recessive cerebellar ataxias, a group of disorders characterized by important genetic heterogeneity and complex phenotypes. The objective of this task force was to build a consensus and develop a clinical and pathophysiological classification for recessive ataxias. **Methods:** The work of this task force was based on a scoping systematic review of the literature that identified recessive disorders characterized primarily by a cerebellar motor syndrome and cerebellar degeneration. The task force regrouped 12 international ataxia experts who decided on general orientation and specific issues. **Results:** We identified 59 disorders that are classified as primary recessive ataxias. For each of these disorders, we present geographical and ethnical specificities along with distinctive clinical and imagery features. The primary recessive ataxias were organized in a clinical and a pathophysiological classification, and we present a general clinical approach to the patient presenting with ataxia. We also identified a list of 48 complex multisystem disorders in which ataxia is a secondary feature. **Conclusions:** This classification is based on a scoping systematic review of the literature and results from a sconsensus among a panel of international experts. It promotes a unified understanding of recessive cerebellar disorders for clinicians and researchers.

A.05

Five-year clinical and health economic outcomes in patients with late functional improvement post-stroke: A population-based cohort study

A Ganesh (Calgary) R Luengo-Fernandez (Oxford) PM Rothwell (Oxford)*

doi: 10.1017/cjn.2019.87

Background: We recently demonstrated that late functional improvement between 3-12 months post-stroke occurs in about one-fourth of patients with ischaemic stroke. It is unknown whether this improvement is associated with better long-term clinical or health-economic outcomes. **Methods:** In a prospective, population-based cohort of 1-year ischaemic stroke survivors (Oxford Vascular Study;2002-2014), we determined changes in functional status (modified Rankin Scale[mRS], Rivermead Mobility Index[RMI], Barthel Index[BI]) from 3-12 months post-stroke. We examined the association of late improvement (by ≥1 mRS grades, ≥1 RMI points, and/or ≥2 BI points) with 5-year mortality, institutionalization (Cox regressions), and health/social-care costs (generalized linear models), adjusted for age/sex/3-month disability/stroke subtype. **Results:** Among 1,288 1-year survivors, 1,135 had 3-month mRS>0, with 319(28.1%) demonstrating late improvement. 1-year survivors with late mRS improvement had lower 5-year mortality (aHR:0.68,95%CI 0.51-0.91,p=0.009), institutionalization (aHR:0.48,0.33-0.72,p<0.001), and costs (margin -$17,369,-25,271 to -9,469,p<0.001). These associations remained on excluding patients with recurrent strokes during follow-up (e.g. 5-year death/institutionalization aHR:0.59,0.44-0.79,p<0.001) and on examining late improvement per RMI and/or BI (e.g. 5-year death/institutionalization aHR:0.67,0.53-0.84,p=0.001). **Conclusions:** Late functional improvement post-stroke is associated with lower 5-year mortality, institutionalization, and costs. These findings should motivate patients and clinicians to maximize late recovery and encourage payers to consider access to rehabilitative services for at least 1-year post-stroke.

A.06

Assessing inter-rater reliability in localizing sleep-related hypermotor seizures: a video-based survey

*PM Lobbezoo (Utrecht) L Nobili (Genoa) S Gibbs (Montreal)**

doi: 10.1017/cjn.2019.88

Background: Sleep-related hypermotor epilepsy (SHE) is a focal epilepsy characterized by abrupt sleep-related hypermotor seizures (SRHS) with complex semiology. Although difficult to localize within the frontal lobe recent studies using intracerebral EEG recordings have suggested the existence of four distinct semiology patterns (SP) organized in a rostro-caudal manner. It remains unclear however if these SP are clinically useful. **Methods:** We aimed to estimate the inter-rater reliability (IR) of classifying SP in SHE amongst epilepsy and sleep medicine experts. Following a short training session, ten experts were asked to review and classify 40 videos of SRHS in patients with confirmed SHE. IR was calculated using Kappa statistics. **Results:** SP1 and SP4, who are at the opposite ends of the SHE semiology spectrum, had substantial IR (0.82 and 0.67, respectively). Meanwhile, SP2 and SP3 showed fair agreement (0.25 and 0.35, respectively) and represented the major source of variance, with a small difference favouring epilepsy experts. **Conclusions:** Amongst epilepsy and sleep medicine experts, IR of classifying SRHS into four SP was only mildly satisfactory. SP1 and SP4 were shown to be easily recognizable while SP2 and SP3 were frequently confounded. Improvements in SP recognition are needed before widespread clinical use.

A.07

Efficacy, safety, and tolerability of ubrogepant for the acute treatment of migraine: a single-attack phase 3 study, ACHIEVE I

*DW Dodick (Scottsdale) RB Lipton (Bronx) J Ailani (Washington) K Lu (Madison) H Lakkis (Madison) M Finnegan (Madison) JM Trugman (Madison) A Szegedi (Madison) G Davidovic (Marham)**

doi: 10.1017/cjn.2019.89

Background: To evaluate efficacy, safety, and tolerability of ubrogepant, an oral CGRP receptor antagonist, for acute treatment of a single migraine attack. **Methods:** Multicenter, randomized, double-blind, placebo-controlled, parallel-group, single-attack, phase 3 study (NCT02828020). Patients randomized 1:1:1 to placebo, ubrogepant 50mg, or ubrogepant 100mg had 60 days to treat one migraine attack (moderate/severe pain intensity). Co-primary efficacy endpoints: pain freedom 2 hours post initial dose and absence

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 ≥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 (STR1VE) update

JW Day (Stanford) CA Chiriboga (New York) TO Crawford (Baltimore) BT Darras (Boston) RS Finkel (Orlando) AM Connolly (St. Louis) ST Iannaccone (Dallas) NL Kuntz (Chicago) LD Pena (Cincinnati) M Schultz (Madison) PB Shieh (Los Angeles) EC Smith (Durham) DE Feltner (Bannockburn) FG Ogrinc (Bannockburn) TA Macek (Bannockburn) E Kernbauer (Bannockburn) LM Muehring (Bannockburn) J L'Italien (Bannockburn) DM Sproule (Bannockburn) BK Kaspar (Bannockburn) JR Mendell (Columbus)*

doi: 10.1017/cjn.2019.90

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 STR1VE, a pivotal study (NCT03306277) evaluating efficacy and safety of a one-time intravenous AVXS-101 infusion. **Methods:** STR1VE 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 STR1VE show rapid motor function improvements in SMA1 patients, paralleling phase 1 findings.

B.02

Long-term neurodevelopmental outcomes in preterm twins

R Christensen (Toronto) V Chau (Toronto) A Synnes (Vancouver) R Grunau (Vancouver) S Miller (Toronto)*

doi: 10.1017/cjn.2019.91

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

F Din (Toronto) S Lalgudi Ganesan (Toronto) A Ochi (Toronto) H Otsubo (Toronto) C Go (Toronto) R Whitney (Toronto) V Chau (Toronto) M Cortez (Toronto) R Sharma (Toronto) CD Hahn (Toronto)*

doi: 10.1017/cjn.2019.92

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),