incidence may be much higher. Methods: The Alberta Children's Hospital (ACH) Rescue ECLS program cannulates patients who are then transferred to the partner program at Stollery Children's Hospital. Data was systematically collected from all patients cannulated for Rescue ECLS at ACH October 2011 and May 2023. Neuroimaging (CT, MR) performed after cannulation was reviewed for evidence of ischemic and hemorrhagic strokes and hypoxic-ischemic brain injury. Results: Seventy-one patients were included in the Rescue ECLS cohort. Median age at cannulation was 1.74 years (range 0-17.6 years, 51% female). Survival to hospital discharge was 65%. Primary indication for ECLS included cardiac (42%), respiratory (33.3%), extracorporeal cardiopulmonary resuscitation (ECPR; 23.2%) and trauma (1.4%). Seventy four percent of the cohort underwent neuroimaging, of whom 67% had evidence of neurologic injury including stroke (ischemic 67%; hemorrhagic 50%) or hypoxic-ischemic injury (33%). Risk of neurologic injury did not differ by indication for ECLS. Conclusions: Neuroimaging abnormalities are present in most pediatric patients imaged post-cannulation for Rescue ECLS. Further research into modifiable risk factors for specific ECLS-related brain injuries may help to improve outcomes for survivors.

C.5

Altered inflammatory profiles in critically ill children with neurologic involvement

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Background: More than 1 in 4 children admitted to the pediatric ICU (PICU) have suspected neuroinflammation for a variety of reasons. While often beneficial, uncontrolled inflammation can lead to secondary neurologic injuries and interfere with repair mechanisms. Methods: A prospective cohort study was initiated at Alberta Children's Hospital to evaluate neuroinflammation in children admitted to the PICU. Forty-eight cytokines, chemokines and growth factors collected at multiple pre-determined timepoints were analyzed along with data on clinical trajectory. Preliminary exploratory analyses of patients enrolled January 2022-July 2023 were completed. Results: Fifty-three patients were included in the initial analysis. Encephalopathy (18.9%), hypoxia (17%) and TBI (15.1%) were the most common reasons for enrollment. All groups had temporal alterations in serum cytokines, with primary inflammatory brain diseases having the highest levels of innate inflammation (cytokine storm) on admission and day one compared to other subgroups. There was a trend towards normalization of cytokine levels over time. Conclusions: Temporal profiling of cytokine levels can inform on neuroinflammatory pathways contributing to the clinical course in critically ill children. Further analysis is ongoing with the entire cohort to evaluate longitudinal and between-group differences. Improved understanding of altered neuroinflammatory pathways in this population may assist with rationalizing targeted immunotherapies to improve outcomes.

C.6

Sex differences in neurodevelopmental outcomes and brain development from early-life to 8-years in preterm males and females

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Background: Sex is associated with differences in early outcomes with preterm males at greater risk for mortality and morbidity. The objective of this study was to examine preterm sex differences in neurodevelopmental outcomes and brain development from early-life to 8-years. Methods: A prospective cohort of preterm infants born 24-32 weeks gestation were followed to 8-years with standardized measures. MRI scans were performed after birth, term-equivalent age and 8-years. Associations between sex, risk factors, brain volumes, white matter fractional anisotropy (FA) and outcomes were assessed using generalized estimating equations. Results: Preterm males (N=83) and females (N=72) had similar risk factors, brain injury and pain exposure. Sex was a predictor of cognitive scores (P=0.02) and motor impairment (P=0.03), with males having lower cognitive scores and higher motor impairment over time. There was a sex effect for FA (P=0.04), with males having lower FA over time. There were significant sex-brain injury and sex-pain interactions for cognitive and motor outcomes. Conclusions: In this longitudinal study, preterm males had lower cognitive scores and greater motor impairment, which may relate to differences in white matter maturation. Effects of brain injury and pain on outcomes is moderated by sex, indicating a differential response to early-life adversity in preterm males and females.

Clinical Neurophysiology (CSCN)

D.1

Efficacy, safety, and tolerability of subcutaneous efgartigimod in chronic inflammatory demyelinating polyneuropathy: results from the ADHERE trial

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Background: Efgartigimod, a human immunoglobulin G (IgG)1 antibody Fc fragment, blocks the neonatal Fc receptor,

decreasing IgG recycling and reducing pathogenic IgG autoantibody levels. ADHERE assessed the efficacy and safety of efgartigimod PH20 subcutaneous (SC; co-formulated with recombinant human hyaluronidase PH20) in chronic inflammatory demyelinating polyneuropathy (CIDP). Methods: ADHERE enrolled participants with CIDP (treatment naive or on standard treatments withdrawn during run-in period) and consisted of open-label Stage A (efgartigimod PH20 SC once weekly [QW]), and randomized (1:1) Stage B (efgartigimod or placebo QW). Primary outcomes were clinical improvement (assessed with aINCAT, I-RODS, or mean grip strength; Stage A) and time to first aINCAT score deterioration (relapse; Stage B). Secondary outcomes included treatment-emergent adverse events (TEAEs) incidence. Results: 322 participants entered Stage A. 214 (66.5%) were considered responders, randomized, and treated in Stage B. Efgartigimod significantly reduced the risk of relapse (HR: 0.394; 95% CI: 0.25–0.61) versus placebo (p=0.000039). Reduced risk of relapse occurred in participants receiving corticosteroids, intravenous or SC immunoglobulin, or no treatment before study entry. Most TEAEs were mild to moderate; 3 deaths occurred, none related to efgartigimod. Conclusions: Participants treated with efgartigimod PH20 SC maintained a clinical response and remained relapse-free longer than those treated with placebo.

D.2

Cost-effectiveness analysis of efgartigimod vs chronic IVIg for treatment of patients with generalized myasthenia gravis in Canada

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Background: Efgartigimod is a human IgG1 antibody Fc fragment recently approved by Health Canada for patients with acetylcholine receptor antibody positive (AChR-Ab+) generalized myasthenia gravis (gMG). We assessed cost-effectiveness of efgartigimod vs chronic IVIg for adult patients with AChR-Ab+ gMG. Methods: A Markov model estimated costs (treatment and administration, disease monitoring, complications from chronic corticosteroid use, exacerbation and crisis management, adverse events, end-of-life care) and benefits (quality-adjusted life-years [QALYs]). The analysis was conducted from the Canadian healthcare system perspective. Health state transition probabilities were estimated using data from ADAPT, ADAPT+, and a network meta-analysis comparing efgartigimod with chronic IVIg. Utility values were obtained from MyRealWorld MG. Patients with MG-ADL ≥5 who did not die/discontinue were assumed to receive treatment every 4 weeks or every 3 weeks over the lifetime horizon. Results: Over the lifetime horizon, efgartigimod and chronic IVIg were predicted to have total discounted QALYs of 16.80 and 13.35, and total discounted costs of $1,913,294 and $2,170,315, respectively. Efgartigimod dominated chronic IVIg with incremental QALYs of 3.45 and cost savings of $257,020 over the lifetime horizon. Conclusions: Efgartigimod may provide greater benefit at lower costs than chronic IVIg for Canadian patients with AChR-Ab+ gMG, with substantial healthcare system savings over the lifetime horizon.

D3

Interim results for Myasthenia Gravis-resource utilization, epidemiology, survival & treatment patterns (MG-REST) study in Ontario, Canada

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Background: Reliable real-world data on the burden of MG is needed to inform Canadian clinical and policy decisions in the era of new MG therapeutics, including FcRn inhibitors. Given the lack of recent Canadian data on MG disease burden, the MG-REST Study aims to estimate the clinical burden of MG in Ontario. Methods: Ontario administrative data from ICES were utilized for a retrospective population-based cohort study of adults with MG identified through a validated algorithm (April 2013-March 2019) and followed for up to seven years (March 2020) to determine myasthenic crisis characteristics and overall survival (OS). Results: The MG cohort (n=2,601) had an average age of 65.7 years and 53.3% were males. Incidence of first myasthenic crisis was 9%, with 87% of events occurring at/after diagnosis. MG OS was 89%, 85% and 75% at 1-year, 2-years and 5-years, respectively, while OS after first crisis was 60%, 52%, and 39% for the same years. Conclusions: Despite the availability of conventional therapies throughout the study, MG crisis remains a serious, common complication of MG, with decreased survival at 1-year post-crisis (29% difference versus 1-year OS following MG diagnosis). Study highlights MG burden and unmet need for new effective therapies for MG treatment.

D.4

Safety and efficacy of delandistrogene moxeparvovec versus placebo in Duchenne muscular dystrophy (EMBARK): Pivotal Phase 3 primary results

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Background: Duchenne muscular dystrophy (DMD) is caused by DMD gene mutations. Delandistrogene moxeparvovec is an investigational gene transfer therapy, developed to address the underlying cause of DMD. We report findings from Part 1 (52 weeks) of the two-part EMBARK trial (NCT05096221). Methods: Key inclusion criteria: Ambulatory patients aged ≥4-<8 years with a confirmed DMD mutation within exons 18–79 (inclusive); North Star Ambulatory Assessment (NSAA) score >16 and <29 at screening. Eligible patients were randomized 1:1 to intravenous delandistrogene moxeparvovec (1.33×1014 vg/kg) or placebo. The primary endpoint was