pharmacokinetics/pharmacodynamics of different risdiplam dose levels in patients with Types 2/3 SMA. Part 2 assessed the efficacy and safety of the selected dose of risdiplam versus placebo in Type 2 and non-ambulant Type 3 SMA. In Part 2, participants were treated with risdiplam or placebo for 12 months, then received risdiplam in a blinded manner until month 24. At month 24, patients were offered the opportunity to enter the open-label extension phase. Results: Change from baseline in MFM32 total score (Part 2- primary endpoint) in patients treated with risdiplam versus placebo was met at month 12. These increases in motor function were sustained in the second and third year after risdiplam treatment. Here we present 4-year efficacy and safety data from SUNFISH. Conclusions: SUNFISH is ongoing and will provide further long-term efficacy and safety data of risdiplam in a broad population of individuals with SMA.

C.3

Development and validation of a prediction model for perinatal arterial ischemic stroke in term neonates

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Background: Perinatal arterial ischemic stroke (PAIS) is a focal brain injury in term neonates, identified postnatally but presumed to occur around birth. Early risk detection and targeted treatments are limited. We developed and validated a diagnostic risk prediction model from common clinical factors to predict a term neonate's probability of PAIS. Methods: A diagnostic prediction model was developed using multivariable logistic regression. Common pregnancy, delivery, and neonatal clinical factors were collected across four registries. Variable selection was based on peer-reviewed literature. Participant inclusion criteria were term birth and no underlying predisposition to stroke. The primary outcome was discriminative accuracy of the model predicting PAIS, measured by the concordance (C-) statistic. Results: 2571 participants (527 cases, 2044 controls) were eligible for analysis. Nine variables were included in the model – maternal age, tobacco exposure, recreational drug exposure, pre-eclampsia, chorioamnionitis, maternal fever, emergency c-section, low 5-minute Apgar score, and sex – to predict the risk of PAIS in a term neonate. This model demonstrated good discrimination between cases and controls (C-statistic 0.73) and model fit (Hosmer-Lemeshow p=0.20). Conclusions: Clinical variables can be used to develop and internally validate a model of PAIS risk prediction. Identifying high-risk neonates for early screening and treatment could reduce lifelong morbidity.

C.4

Understanding the role of deep brain stimulation for Refractory Status Dystonicus in children: case series and systematic review

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Background: Status dystonicus (SD) is a life-threatening form of dystonia with limited treatments available. We sought to better understand the processes, outcomes, and complications of deep brain stimulation (DBS) for pediatric SD through a systematic review alongside an institutional case series. Methods: Data regarding treatment, stimulation parameters, dystonia severity and outcomes was collected for the case series (n=7) and systematic review (n=70, conducted in accordance with PRISMA guidelines). This was analysed descriptively (rates, outcome measures). For the case series we created probabilistic voxel-wise maps for improvement in dystonia based on brain region stimulated. Results: All patients in our case series and > 95% of patients in the systematic review had resolution of SD with DBS, typically within 2-4 weeks. Most patients in the review (84%) and all patients in the case series had DBS implanted to the globus pallidus internus. In terms of dystonia severity scores, there was a mean improvement of 25% (case series) or 49% (systematic review). Reported mortality was 4% in the systematic review. Conclusions: DBS for pediatric SD is feasible and safe. It allows for increased survival as well as quality of life - however risks still exist. More work is needed to determine timing, eligibility, and stimulation parameters.

C.5

Highlighting a novel, stepwise pathway for the in-hospital management of children with acutely worsening dystonia

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Background: Dystonia is common in children with acquired and inherited neurological disorders. Status dystonicus (SD) is the most severe form of dystonia that can lead to life-threatening complications if not treated promptly. We identified a local provider knowledge gap in the acute management of dystonia, leading to uncertainty and delays in care. To our knowledge, no in-hospital clinical pathway exists for the ward-based management of acute dystonia. We hypothesized that a stepwise clinical pathway would standardize and improve comfort in managing hyperacute dystonia. Methods: We formed a multidisciplinary working group and developed a pathway based on literature review and expert consensus. Aims of the pathway included:

reducing delays in recognition and treatment of acute dystonia, limiting variation in management, and decreasing progression to SD. A survey was administered to providers assessing knowledge and comfort post-implementation. Results: There has been high usability with 58% (18/31) of providers surveyed having used the pathway at least once. Provider comfort has improved, with 89% (25/28) of respondents reporting increased comfort managing SD due to the clarity of the pathway and stepwise directions. Conclusions: The pathway fills a gap in the in-hospital management of dystonia and has led to increased provider comfort.

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D.1

Feasibility and impact of palliative care at any stage of amyotrophic lateral sclerosis

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Background: Although palliative care (PC) is recommended for patients with amyotrophic lateral sclerosis (ALS), many patients receive PC very late or not at all. Our study goals included 1) determing the feasibility of early PC 2) describing patient/caregiver satisfaction with early PC and 3) measuring the impact of early PC on quality of life (QOL) and mood. Methods: Patients followed at the multidisciplinary ALS clinic in Ottawa, Canada and their caregivers were eligible for the study irrespective of duration or severity of disease. All participants completed questionnaires tracking QOL and mood and all were offered a palliative care consultation. Participants completed a satisfaction survey post-PC consultation. Results: 32 patients and 20 caregivers received a PC consultation, conducted virtually. All of them found the consult beneficial and none of the patients reported preferring the consultation later in their illness. The PC consultations were most highly rated by patients with high levels of anxiety and worse bulbar function, and by caregivers of patients with low function. There was no statistically significant change in mood or QOL compared to the 7 participants who declined PC consultation. Conclusions: PC consultations are feasible and beneficial at all stages of illness. Patients with anxiety and bulbar dysfunction may benefit most.

D.2

Cross-sectional axonal excitability and motor unit number index profile in early stages of weakness in ALS

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Background: What is the number and size of motor units, and axonal excitability profile in the early stages of muscle weakness in ALS compared to controls? Methods: We enrolled ALS patients with APB manual strength testing rated four or four-minus (ALS:4-arm) and four-plus (ALS:4+ arm) and control participants >35 years-old from the University of Toronto, University of Alberta and Universidade Federal de Sao Paulo. Mean±SD, one-way ANOVA and ANCOVA of ALSFRS-R, PUMN Score, MUNIX, MUSIX, and nerve-excitability testing using QTRAC TROND protocol were reported. Results: Twenty-five ALS patients and 63 controls were included. Mean MUNIX was significantly lower ($p<0.0001$) and MUSIX was significantly higher ($p<0.001$) in both ALS groups compared to controls. Mean strength-duration time constant in the ALS:4-arm (0.50ms±0.11; $p<0.05$) and superexcitability in both ALS groups (ALS:4- -29.05%±9.24, ALS:4+ -27.67%±8.03; $p<0.05$) were relatively increased, supporting axonal hyperexcitability. Conclusions: Significant motor unit loss measured by MUNIX is already present at the earliest detection of muscle weakness in ALS. Increased MUSIX and altered axonal physiology are associated with axonal sprouting and geometry change(1), along with ion channel dysfunction(2). Future trials targeting muscle weakness in ALS should consider the altered neuronal physiology during early disease stages and utilize neurophysiological biomarkers only in normal-to-mildly weak muscles.

D.3

Peripheral nerve injuries related to walking aid use: a systematic review

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Background: Walking aids such as crutches, canes and walkers are used by 2 million Canadians. Repetitive weight-bearing with walking aids may cause upper limb peripheral nerve injury. The objectives of this review were to: 1) identify types of nerve injuries reported with walking aids; 2) report electrodiagnostic findings; 3) identify typical treatment strategies; and 4) determine expected recovery time for such injuries. Methods: MEDLINE, EMBASE, CINAHL and Cochrane Library were searched for primary data in English published between 1950-2022. Abstracts were reviewed independently by 2 authors. Full-text reviews were independently conducted by 2 authors. Results: The search identified 3746 abstracts, 43 of which underwent full-text review. 31 studies were included. There were 144 cases of peripheral nerve injury. Crutches caused the most injuries (n=21 studies). The ulnar nerve was most commonly injured (n=27 cases). Improper walking aid fit was identified as a risk factor in 74% of cases. Stopping walking aid use was the most common treatment strategy (n=10 studies). Follow-up reports (n=20) indicated 65% of patients experienced recovery at 6 months. Conclusions: Improper walking aid fit and use were identified as major injury risk factors. A national program to teach patients and clinicians how to use walking aids may reduce injury risk.