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: