guidelines. Individual case reports were excluded. We identified interventions which have been used, and selected those with evidence or expert support for use in a protocol. The protocol was reviewed with stakeholders in pediatric neurology and PICU. Results: Reported treatments include high-dose steroids, IV immunoglobulins, tocilizumab, and plasmapheresis. The treatment with strongest evidence is high-dose steroids started within 24 hours of presentation. There is frequently reported use of IV immunoglobulins and plasmapheresis, and growing evidence to support use of tocilizumab (IL-6 blockade) within the first 48 hours. Conclusions: Overall, there is strong expert opinion that treatment should be initiated promptly. We present our centre's protocol to expedite this treatment.

Neuromuscular Disease and EMG

P.070

Delay to diagnosis of Duchenne muscular dystrophy

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Background: Duchenne muscular dystrophy (DMD) typically presents with painless weakness which may contribute to its delayed recognition. Methods: Retrospective chart review was performed for patients with DMD at CHEO over 15 years (2009-2023). Our data will later be combined with that from two other centers. Inclusion criteria: 1) confirmed DMD; 2) symptom onset <6 yo. Exclusion criteria: incomplete records or family history of DMD. Results: We identified 72 DMD patients. Total of N=49 were analyzed. Subjects were excluded for: incomplete data N=10 (e.g. diagnosis at another centre); symptom onset ≥6 yo (N=4); family history (N=9). First symptoms were reported at a mean age of 2.7 yo (range: 0-5.9 yo) with diagnosis at mean age of 5.2 yo (range: 0.5 to 9.6 yo), representing a mean delay of 2.5 years (range: 0-6.8 yrs). Initial symptoms included: weakness (61.2%), sports difficulty (61.2%), calf pseudohypertrophy (10.2%), language difficulties (8.2%) or muscle pain (2.0%). Learning disability was reported in 36 (73.5%) subjects with 7 (14.3%) having autistic spectrum disorder. Conclusions: The mean delay to diagnosis of patients followed at our centre was similar to the United Kingdom (MDSTARnet). We advocate for increased education to identify DMD earlier, particularly given emerging therapies for this disorder.

P.071

The value of genetics, biopsy and EMG in diagnosing congenital myopathies

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Background: Congenital myopathies (CM) are inherited muscle disorders historically classified according to features seen on muscle biopsy and congenital-onset weakness and hypotonia. The aim of our study was to evaluate the benefit of genetic testing, muscle biopsy, NCS/EMG and muscle MRI in obtaining a definite diagnosis for these patients. Methods: A retrospective chart review of all patients diagnosed at a single tertiary-care pediatric hospital over 15 years (2008-2022). REB approval was obtained. Results: Over a period of 15 years, 42 patients with CM were included. All (100%) had genetic testing (i.e. gene panel, WES), 65.9% had muscle biopsy, 67.5% had NCS/EMG and 20% had a muscle MRI. Definite diagnosis was obtained in 38% by genetic testing only, while 42.8% had a diagnosis made by genetic testing supported by the findings of one or more of the other diagnostic tools. Conclusions: Early diagnosis of CM is still essential in congenital myopathies to provide optimal care. Genetic testing is the gold standard for diagnosis, but other diagnostic tools remain valuable in the case of variants of unclear significance.

P.072

Safety and effectiveness of Nipocalimab in adolescent participants in the open label phase 2/3 Vibrance-mg clinical study

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Background: Nipocalimab (a fully human, effectorless anti-neonatal Fc receptor (FcRn) monoclonal antibody) may ameliorate gMG disease manifestations by selectively targeting FcRn IgG recycling and lowering IgG, including pathogenic autoantibodies in generalized myasthenia gravis (gMG). The objective was to evaluate the effectiveness and safety of intravenous nipocalimab added to background standard-of-care therapy in adolescents with gMG. Methods: Seropositive patients (12-<18 years) with gMG (MGFA Class II-IV) on stable therapy but inadequately controlled, were enrolled in a 24-week open label study. Nipocalimab was administered as a 30 mg/kg IV loading dose followed by 15 mg/kg IV every 2 Weeks. Results: Seven adolescents were enrolled; 5 completed 24-weeks of dosing. The mean(SD) age was 14.1(1.86) years; seven were anti-AChR+, six were female. Mean(SD) baseline MG-ADL/QMG scores were 4.29(2.430)/12.50(3.708). Nipocalimab showed a significant reduction in total serum IgG at week-24; the mean(SD) change from baseline to week-24 for total serum IgG was -68.98%(7.561). The mean(SD) change in MG-ADL/QMG scores at week-24 was -2.40(0.418)/-3.80(2.683); 4 of 5 patients achieved minimum symptom expression (MG-ADL score 0-1) by week-24. Nipocalimab was well-tolerated; there were no serious adverse events. There were no clinically meaningful laboratory changes. Conclusions: Nipocalimab demonstrated efficacy and safety in this 6-month trial in seropositive adolescents with gMG.