gross motor (limb/axial motor items) and ocular (ocular/facial muscles) domains. **Results:** Eculizumab-treated patients showed improvements in all four QMG domain scores to week 26. Rapid, sustained improvements were demonstrated across all domains, with a trend toward significant differences between eculizumab and placebo (bulbar, p=0.0628; respiratory, p=0.0682; gross motor, p=0.0114; ocular, p=0.0017). The eculizumab safety profile was consistent with previous reports. **Conclusions:** Eculizumab demonstrated a consistent response across all QMG muscle domains. This aligns with previously reported MG-ADL findings with eculizumab. (NCT01997229).

P.024

Long-term use of patisiran in patients with hereditary transthyretin amyloidosis (hATTR): 12 month efficacy & safety data from a global open label extension (OLE) study

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Background: Hereditary transthyretin-mediated (hATTR) amyloidosis is a multi-systemic, heterogenous, life-threatening disease. Patisiran resulted in significant improvement in neuropathy and QoL at 18-months compared to placebo, and was generally well-tolerated in the Phase 3 APOLLO study. **Methods:** Multi-center, OLE study to evaluate the efficacy and safety of long-term patisiran dosing for ≤ 5 years in hATTR amyloidosis patients with polyneuropathy who have completed the APOLLO study (NCT02510261). Endpoints include safety, tolerability and long-term efficacy of patisiran. Measures of clinical benefit are the same endpoints used in APOLLO including changes in mNIS+7 composite neuropathy impairment score and QoL (Norfolk QoL-DN) **Results:** As of December 2017, 184 of 186 (99%) patients who completed APOLLO and 25 patients from the Ph 2 OLE study enrolled in the Global OLE study. Baseline data for 211(APOLLO/placebo, n=49; APOLLO/patisiran, n=137 and patisiran Ph 2 OLE, n=25) patients included: median age 61 years (26-84); 74% males; 46% V30M. Interim safety data and 12-month efficacy results will be presented. **Conclusions:** The global OLE study includes a diverse population of hATTR amyloidosis patients. Interim data will include the long-term safety and maintenance of effect in patients continuing on patisiran, as well as the impact of treatment with patisiran on patients previously treated with placebo.

P.025

APOLLO, a phase 3 study of patisiran for the treatment of hereditary transthyretin amyloidosis (hATTR): 18-month safety and efficacy in subgroup with cardiac involvement

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Background: Hereditary transthyretin-mediated (hATTR) amyloidosis a hereditary, multi-systemic and life-threatening disease resulting in neuropathy and cardiomyopathy. In the APOLLO study, patisiran, an investigational RNAi therapeutic targeting hepatic TTR production resulted in significant improvement in neuropathy and QoL compared to placebo and was generally well tolerated. **Methods:** APOLLO, a Phase 3 study of patisiran vs. placebo (NCT01960348) prespecified a cardiac subpopulation (n=126 of 225 total) that included patients with baseline left ventricular (LV) wall thickness ≥ 13mm and no medical history of aortic valve disease or hypertension. Cardiac measures included structure and function by electrocardiography, changes in NT-proBNP and 10-MWT gait speed. **Results:** At 18 months, patisiran treatment resulted in a mean reduction in LV wall thickness of 1 mm (p=0.017) compared to baseline, which was associated with significant improvements relative to placebo in LV end diastolic volume (+8.31 mL, p=0.036), global longitudinal strain (-1.37%, p=0.015) and NT-proBNP (55% reduction, p=7.7 x 10-8) (Figure 1). Gait speed was also improved relative to placebo (+0.35 m/sec, p=7.4 x 10-**9**). Rate of death or hospitalization was lower with patisiran. mNIS+7 results in the cardiac subpopulation will also be presented. **Conclusions:** These data suggest patisiran has the potential to halt or reverse cardiac manifestations of hATTR amyloidosis.

P.026

Response to eculizumab in patients with myasthenia gravis recently treated with chronic intravenous immunoglobulin

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Background: Chronic intravenous immunoglobulin (IVIg) is used to treat refractory myasthenia gravis (MG). This subgroup analysis evaluated response to eculizumab in patients receiving chronic IVIg before entry to REGAIN, a phase 3, randomized, double-blind, placebo-controlled study of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalized MG. **Methods:** IVIg was only permitted during REGAIN as rescue therapy; previously treated patients underwent a 4-week washout before randomization. Patients included in this analysis had received chronic IVIg ≥4 times in 1 year, with ≥1 dose within 6 months before REGAIN entry. Exacerbations and MG status changes were assessed. **Results:** Eighteen patients were evaluated; four experienced exacerbations (eculizumab-treated, 1/9; placebo-treated, 3/9). Clinically relevant improvements were larger with eculizumab than placebo, respect-

ively (mean change, standard deviation [SD]: MG Activities of Daily Living score [MG-ADL], -5.3 [4.0] vs -2.1 [2.8]; Quantitative MG score [QMG], -4.1 [6.1] vs -1.3 [3.5]). More patients receiving eculizumab (7/9) had clinically meaningful responses (MG-ADL ≥3 and/or QMG ≥5 points) than those receiving placebo (3/9). Eculizumab safety was consistent with previous reports. Interim data from the open-label extension of REGAIN will be presented. **Conclusions:** In patients previously receiving chronic IVIg, eculizumab showed a trend toward meaningful clinical improvements and fewer exacerbations compared with placebo. (NCT01997229, NCT02301624).

P.027

Incidence of amyotrophic lateral sclerosis in Newfoundland and Labrador

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Background: There is a paucity of research regarding ALS epidemiology in Canada. Previously published data from Newfoundland and Labrador (NL) demonstrate an average incidence of 2.4/100,000 from 2000-2004 (peak 3.3 in 2001, the highest reported in Canada). Local neurologists believe that the incidence has continued to increase. **Methods:** Clinicians affiliated with the electromyography (EMG) lab at the Health Sciences Centre in St. John's compiled a list of patients diagnosed with ALS from 2012-2016, based on recall. Their medical records were reviewed and demographic information collected. This was cross-referenced with new referrals to the ALS Society NL per year. **Results:** Based on new referrals to ALS Society NL the average incidence between 2012-2016 was 2.81/100,000 (peak 3.6 in 2015). Average age-adjusted incidence from the EMG lab was 1.33 (peak 1.73 in 2016). The EMG lab documented a crude incidence of 3.97 in 2018. **Conclusions:** The incidence of ALS in NL is increased compared to the usual incidence of 1-2/100,000 per year. After the preliminary study, the EMG lab maintained more thorough records and an incidence of 3.97/100,000 was found in 2018. This makes a compelling argument for future research which could explore potential genetic or environmental causes for the increased incidence in this population.

P.028

A milder congenital myopathy in the french canadians caused by a novel TNNT1 homozygous missense mutation

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Background: Mutations of the slow skeletal muscle troponin-T1 (*TNNT1*) gene are a rare cause of nemaline myopathy. The phenotype is characterized by severe amyotrophy and contractures. Death from respiratory insufficiency occurs in infancy. We report on four French Canadians with a novel congenital *TNNT1*-related myopathy. **Methods:** Patients underwent MRI of leg muscles, quadriceps biopsy and genetic testing. Wild type or mutated human *TNNT1* mRNAs were co-injected with morpholinos in a zebrafish knockdown model to assess their relative abilities to rescue the morphant phenotype. **Results:** Three adults and one child shared a novel missense homozygous pathogenic variant in the *TNNT1* gene. They developed from childhood slowly progressive limb-girdle weakness with spinal rigidity and contractures. They suffered from restrictive lung disease and recurrent episodes of infection-triggered rhabdomyolysis, which were relieved by dantrolene in one patient. Older patients remained ambulatory into their sixties. MRI of leg muscles showed symmetrical atrophy and fatty infiltration in a proximal-to-distal gradient. Biopsies showed multi-minicores, while nemaline rods were seen in half the patients. Wild type *TNNT1* mRNA rescued the zebrafish morphants but mutant transcripts failed to rescue the morphants. **Conclusions:** This study expands the spectrum of *TNNT1*-related myopathy to include a milder clinical phenotype caused by a functionally-confirmed novel missense mutation.

P.029

Facial onset sensorimotor neuronopathy syndrome (FOSMN) associated with Non-Hodgkin Lymphoma (NHL)

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Background: FOSMN is a recently describe neurological syndrome, characterizes by slow onset of facial sensory abnormalities and motor deficits. The initial description showed a very uniform clinical presentation. Since the initial description there are clinical cases describe in literature with subtle phenotype variations. **Methods:** We describe a clinical case associated with NHL. We will report clinical data, laboratory and neurophysiological findings. **Results:** *Patient initiated with left perioral and mental sensory symptoms on her left side. It spread up to include left V2 area and spread to the right side. After 2 years she developed sensory symptoms on her right hand. Progressed to weakness and atrophy on the right upper limb. Also developed dysarthria, dysphonia, dysphagia, as well as photophobia, anisocoria and double vision. Had thorough work-up and everything unrevealing. Except for Spep that showed increased free kappa. Bone marrow biopsy showed evidence of a clonal cell expansion consistent with indolent lymphoma* **Conclusions:** This case provides evidence of FOSMN associated with NHL. To our knowledge this is a first case describe with NHL. There had been reports with motor neuro diseases phenotype with lymphoma that may represent a paraneoplastic disorder. Our patient expands the clinical presentation. This finding should not lessen the diagnosis of FOSMN.

P.030

The journey with CIDP- a Canadian perspective

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Background: Chronic Inflammatory demyelinating polyradiculoneuropathy is a rare disorder of the peripheral nerves. A disease affecting up to 8.9 out of 100,000 people, and a yearly incidence of 1.6/100,000 people, CIDP is a condition that is treatable but still relatively unknown outside of the neuromuscular community. The purpose of this research, initiated by the GBS/CIDP Foundation, is