Neuromuscular Disease and EMG

P.034

Minimal symptom expression following treatment with efgartigimod in patients with Generalized Myasthenia Gravis

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doi: 10.1017/cjn.2023.138

Background: Efgartigimod is a human IgG1 antibody Fc-fragment that reduces IgG levels through FcRn blockade. A key efficacy indicator in the treatment of IgG autoantibody-mediated generalized myasthenia gravis (gMG) is improvement in MG-ADL score. Methods: The ADAPT phase 3 trial evaluated safety and efficacy of efgartigimod in patients with gMG, including reaching and maintaining of minimal symptom expression (MSE; defined as an MG-ADL total score of 0 or 1). Results: 167 patients (AChR-Ab+, n=129; AChR-Ab-, n=38) were randomized to receive treatment cycles of 4 weekly infusions of efgartigimod or placebo. Significantly more AChR-Ab+ efgartigimod-treated patients achieved MSE during cycle 1 compared to placebo-treated patients (40.0% [n=26/65] vs 11.1% [n=7/63; $P<0.0001$]). In cycle 2, 31.4% (n=16/51) of AChR-Ab+ patients in the efgartigimod cohort achieved MSE compared to none in the placebo cohort. MG-ADL score improved by ≥6 points in 56.9% of AChR-Ab+ efgartigimod-treated patients compared to 20.6% of placebo-treated patients in cycle 1. Most patients achieved MSE by week 4 of a cycle, paralleling early reduction in IgG levels, and MSE duration ranged from 1 to ≥10 weeks. Adverse events were predominantly mild to moderate. Conclusions: Efgartigimod treatment resulted in more patients with AChR-Ab+ gMG achieving both MSE and clinically meaningful MG-ADL improvements.

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Autologous hematopoietic stem cell transplant for the treatment of refractory myasthenia gravis with anti-muscle specific kinase antibodies

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doi: 10.1017/cjn.2023.139

Background: Several case series describe patients with refractory acetylcholine receptor antibody-positive (AChR) myasthenia gravis (MG) treated with hematopoietic stem cell transplant (HSCT). In this report, we describe four patients with anti-muscle-specific kinase (MuSK) MG treated with HSCT. Methods: We reviewed the records of all patients undergoing HSCT for MG in the Alberta Blood and Bone Marrow Transplant Program and identified 4 patients with anti-MuSK MG. Results: All 4 patients had severe disease (Myasthenia Gravis Foundation of America score IVb-V) and were refractory to multiple treatments, including rituximab. 3 patients improved with no clinical manifestations or mild symptoms and remained as such for 2, 3.5, and 5.5 years. In these 3 patients, adverse events ranged from treatable infections and transient dyspnea to persistent fatigue and premature menopause. The average worst Myasthenia Gravis Activities of Daily Living (MG-ADL) scores improved from 14.7 before to 0.3 after HSCT while their mean worst Myasthenia Gravis Quality of Life Questionnaire (MG-QoL15) scores improved from 26.7 to 0. The fourth patient developed pneumonia and passed away from respiratory failure 8 weeks post-transplant. Conclusions: In patients with severe refractory anti-MuSK MG, it may be reasonable to consider HSCT but with an appreciation of the associated risks.

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Are there sex differences in the treatment of myasthenia gravis? a single centre cohort study

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doi: 10.1017/cjn.2023.140

Background: Females with generalized myasthenia gravis (gMG) report lower quality of life (QoL) compared to males. Our objective was to determine whether sex differences in treatment and time to treatment initiation may contribute to this difference. Methods: We performed a single centre retrospective study of people diagnosed with gMG. We used multivariable logistic and Cox regression models to assess the association between sex and study outcomes, adjusting for duration from onset to diagnosis, age at diagnosis, thymoma, and antibody status. Results: 179 people with gMG were included. Mean age at diagnosis was 58.4 years, mean follow-up was 4.8 years, and 58.1% were male. There was no association between sex and odds of starting prednisone (adjusted odds ratio [aOR]=0.58, 95% confidence interval [95%CI]=0.28-1.19, p=0.14) or steroid sparing agents (aOR=0.72, 95%CI=0.39-1.35, p=0.31). Similarly, sex was not associated with time to starting prednisone (adjusted hazard ratio [aHR]=0.74, 95% confidence interval [95%CI]=0.52-1.06, p=0.10) or steroid sparing agents (aHR=0.82, 95%CI=0.55-1.22, p=0.33). Females were more likely to start plasmapheresis (aOR=3.15, 95%CI=1.09-9.07, p=0.03). Conclusions: We found no sex differences in first and second line immunotherapy for gMG that might explain differences in QoL. Females were more likely to initiate plasmapheresis, which may reflect greater disease severity.

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Neurofascin-155 IgG in acute-onset inflammatory polyneuropathy: possible predictor of relapse and recovery

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doi: 10.1017/cjn.2023.141

Background: IgG4 autoantibodies to neurofascin-155 (NF-155) have been described in a subset of patients with chronic

inflammatory demyelinating polyneuropathy (CIDP). While reports suggest an acute onset is more likely than in antibody negative CIDP, little literature exists around the subsequent course of NF-155 positive cases that originally presented with an acute inflammatory demyelinating polyneuropathy (AIDP) phenotype. Methods: Two male patients, ages 51 and 59, presented with similar, <2 week histories of lower extremity weakness. Patients were diagnosed with AIDP and treated with IVIG. Following initial improvement, both patients relapsed. One patient was treated with IVIG and steroids with subsequent improvement; however, he was unable to be weaned from steroids without experiencing recurrence of symptoms. The other patient was not retreated. Testing for NF-155 IgG was sent. Results: The first patient ultimately required Rituximab for stable improvement, the other improved spontaneously. Both patients later had positive tests for NF-155 IgG4 antibodies. Conclusions: Both of our NF-155 positive cases had initial AIDP-like presentations, followed by a relapsing course and excellent eventual recovery. This result, along with limited other available cases, suggest that in patients with an AIDP-like presentation, NF-155 IgG4 autoantibodies could be a marker of disease recurrence, but do not necessarily predict a poor outcome.

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Idiopathic inflammatory myopathies and malignancy screening: a survey of the current practices amongst Canadian neurologists and rheumatologists

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doi: 10.1017/cjn.2023.142

Background: There is a well-established association between idiopathic inflammatory myopathies (IIM) and malignancy. There are no evidence-based guidelines amongst neurologists and rheumatologists on the choice and timing of malignancy investigations. Our aim is to characterize the current gaps and uncertainties amongst neurologists and rheumatologists with malignancy screening in IIM patients. Methods: An online survey consisting of 18 multiple-choice questions related to IIM malignancy screening was distributed to adult neurologists and rheumatologists in Canada. Quantitative and descriptive analysis was performed. Results: The majority of respondents (96%, n=68) performed malignancy screening. There was variability in practice including delegation and choice of screening tests, influence of patient-specific factors, and time and length of repeat testing. Only 18% of respondents were confident in their malignancy screening practices. Between neurologists and rheumatologists, there were differences in the number of IIM patients seen, consideration of patient-specific factors and choice of screening investigations. Further details and data will be presented at the conference. Conclusions: There is a lack of consensus and confidence in the choice and timing of malignancy investigations in IIM, with neurologists and rheumatologists differing in their approaches. Further research is required to better understand the relationship between IIM and malignancy to create expert-led consensus guidelines.

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Development of a checklist for treating adults with Myotonic Dystrophy Type 1: a neuromuscular disease network for Canada (NMD4C) Knowledge Translation Tool

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doi: 10.1017/cjn.2023.143

Background: The Neuromuscular Disease Network for Canada (NMD4C) aims to improve the care of Canadians with neuromuscular diseases. It has identified a need to support clinicians in implementing clinical guidelines with the use of checklists for initial evaluation and clinical follow-ups. The objective of the study was to develop a pragmatic management checklist to support clinical guidelines for diagnosis and follow-up of myotonic dystrophy type 1 (DM1). Methods: A practice-based DM1 checklist will be reviewed by a panel of 35 experts using an online survey. The survey has been drafted using the Appraisal of Guidelines Research and Evaluation tool for assessing Recommendation Excellence (AGREE-REX). The experts will rate: (1) the quality of each checklist recommendation, and (2) the applicability of each recommendation based on their clinical setting. Scores will be compiled and discussed among experts to achieve consensus. Results: The compiled checklist items were organized into three sections: (1) initial evaluation, (2) follow-up visit and (3) general treatment recommendations. Feedback from experts across Canada, results on feasibility, and a finalized checklist will be presented. Conclusions: The development of a feasible treatment checklist is a useful KT tool that DM1 experts across Canada could apply in their own clinical settings.

P.040

Long-term efficacy and safety of ravulizumab in adults with anti-acetylcholine receptor antibody-positive generalized myasthenia gravis

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doi: 10.1017/cjn.2023.144

Background: The 26-week double-blind, randomized, placebo-controlled period (RCP) of the CHAMPION MG study (NCT03920293) demonstrated ravulizumab's efficacy and tolerability in anti-acetylcholine receptor antibody-positive (AChR Ab+) generalized myasthenia gravis (gMG). Methods: In the ongoing open-label extension (OLE), patients receive intravenous ravulizumab (blind loading dose in placebo-treated patients or bridging dose in ravulizumab-treated patients, then 3000–3600 mg according to body weight every 8 weeks) for ≤4 years. Data from RCP baseline up to Week 60 were analyzed. Results: Ravulizumab's long-term efficacy (n=161) and safety (n=169) were assessed. Patients who switched from placebo in the RCP to