Neuromuscular Disease and EMG

P.034

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

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