or an ownership interest from Johnson & Johnson. Dr. Sun has received personal compensation for serving as an employee of Janssen.

Long-term Safety and Efficacy of Efgartigimod in Patients With Generalized Myasthenia Gravis: Interim Results of the ADAPT+ Study

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Objective

To evaluate the safety and efficacy of efgartigimod in patients with generalized myasthenia gravis (MG) enrolled in the ADAPT+ long-term extension study.

Background

Treatment with efgartigimod, a human IgG1 antibody Fc-fragment that blocks neonatal Fc receptor, resulted in clinically meaningful improvement (CMI) in MG-specific outcome measures in the ADAPT phase 3 clinical trial. All patients who completed ADAPT were eligible to enroll in its ongoing open-label, 3-year extension study, ADAPT+.

Design/Methods

Efgartigimod (10 mg/kg IV) was administered in cycles of once-weekly infusions for 4 weeks, with subsequent cycles initiated based on clinical evaluation. Efficacy was assessed during each cycle utilizing Myasthenia Gravis Activities of Daily Living (MG-ADL) and Quantitative Myasthenia Gravis (QMG) scales.

Results

Ninety-one percent of ADAPT patients (151/167) entered ADAPT+. As of February 2021, 106 AChR-Ab+ and 33 AChR-Ab– patients had received at least 1 dose of open-label efgartigimod (including 66 ADAPT placebo [PBO] patients). The mean (SD) study duration was 363 (114) days, resulting in 138 patient-years of observation. Similar incidence rates per patient year (IR/PY) of serious adverse events were seen in ADAPT (efgartigimod: 0.11; placebo: 0.29) compared to ADAPT+ (0.25). Five deaths (acute myocardial infarction, COVID-19 pneumonia/septic shock, bacterial pneumonia/MG crisis, malignant lung neoplasm, and unknown [multiple cardiovascular risk factors identified on autopsy]) occurred; none were considered related to efgartigimod by the investigator. AEs were predominantly mild or moderate. CMI was observed in AChR-Ab+ patients during each cycle (up to 10 cycles) at magnitudes comparable to improvements observed at week 3 of cycle 1 (mean[SE] improvements: MG-ADL, –5.1[0.34]; QMG, –4.7[0.41]). Clinical improvements mirrored maximal reductions in total IgG and AChR-Abs across all cycles.

Conclusions

This analysis suggests the efficacy of long-term treatment with efgartigimod was consistent across multiple cycles. No new safety signals were identified, despite being conducted before vaccine availability during the COVID-19 pandemic.

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Numbers Needed to Treat and Costs Per Improved Outcome Among Treatments for Myasthenia Gravis

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Objective

Assess number needed to treat (NNT) and costs required to achieve improvements in symptoms and functional activities with targeted therapies for myasthenia gravis (MG).

Background

NNT and cost per improved efficacy can help inform comparative clinical efficacy and cost-effectiveness across MG treatments.

Design/Methods

Relative to conventional therapy (CT), NNTs and annual costs for achieving one point improvement in Quantitative Myasthenia Gravis score (QMG), one additional patient with minimal clinically important difference (MCID) in QMG (i.e., = 3 points improvement), and one additional patient achieving minimal symptom expression (MSE; Myasthenia Gravis-

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