

P.044**Efficacy, safety, and tolerability of efgartigimod in AChR-Ab- patients with Generalized Myasthenia Gravis: interim analysis of ADAPT/ADAPT+**

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Background: Efgartigimod, a human IgG1 antibody Fc-fragment, reduces IgG levels through neonatal Fc receptor blockade. Patients with anti-acetylcholine receptor antibody-negative (AChR-Ab-) generalized myasthenia gravis (gMG) comprise 15%-20% of the gMG population and have limited approved treatment options. We evaluated long-term safety and efficacy of efgartigimod in AChR-Ab- patients from ADAPT/ADAPT+ (open-label extension). **Methods:** ADAPT evaluated safety and efficacy of efgartigimod versus placebo in AChR-Ab+ (n=129) and Ab- (n=38) patients with gMG. This integrated analysis includes 37 AChR-Ab- patients who received ≥ 1 dose of efgartigimod in ADAPT/ADAPT+ through October 2020 (median[range] follow-up: 453[85-721] days). Responder status was defined as ≥ 2 -point (MG-ADL) and ≥ 3 -point (QMG) improvement for ≥ 4 consecutive weeks (with first improvement ≤ 1 week after last infusion). **Results:** Among AChR-Ab- patients in ADAPT (cycle 1), 68.4% (13/19) efgartigimod-treated were MG-ADL responders (placebo, 63.2% [12/19]), and 52.6% (10/19) were QMG responders (placebo, 36.8% [7/19]). In the integrated ADAPT/ADAPT+ analysis (cycle 1), AChR-Ab- patients improved from baseline in MG-ADL/QMG scores, with consistent improvements across multiple subsequent cycles. No clinically meaningful differences in safety or efficacy outcomes between AChR-Ab+ and Ab- patients occurred. **Conclusions:** Long-term treatment (median >1 year) with efgartigimod was well tolerated and associated with clinically meaningful improvements in MG-ADL/QMG scores in AChR-Ab- patients.

P.045**Safety profile overview of Efgartigimod Clinical Trials in participants with diverse Diverse IgG-Mediated Autoimmune Diseases**

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Background: Efgartigimod is a human IgG1 antibody Fc-fragment that reduces IgG autoantibody levels through FcRn blockade. This study reports safety of efgartigimod across IgG-mediated disorders. **Methods:** The safety of intravenous efgartigimod was evaluated in 204 efgartigimod-treated subjects with generalized myasthenia gravis (phase 3 ADAPT and 3-year

open-label extension ADAPT+ trials), primary immune thrombocytopenia (phase 3 ADVANCE trial), or pemphigus (open-label phase 2 trial). These studies examined different efgartigimod doses (10–25 mg/kg), including cyclical dosing in generalized myasthenia gravis and continuous weekly dosing in primary immune thrombocytopenia and pemphigus. **Results:** Across all indications and doses studied, efgartigimod demonstrated a consistent safety profile, with treatment-emergent adverse event (TEAE) rates comparable to placebo (ADAPT, 77.4% efgartigimod/84.3% placebo; ADVANCE, 93.0% efgartigimod/95.6% placebo; and 85% in the pemphigus study). Most TEAEs were mild to moderate in severity. Discontinuation rates due to adverse events were consistently low (ADAPT, 3.6% efgartigimod/3.6% placebo; ADVANCE, 3.5% efgartigimod/2.2% placebo; and 3% of pemphigus study participants). In ADAPT+, no increases in TEAEs or infections occurred with additional efgartigimod dosing (≤ 19 cycles). **Conclusions:** Efgartigimod was well tolerated across indications and doses studied. Most TEAEs, including infections, were mild or moderate in severity and did not increase in frequency with recurrent dosing.

P.046**Real-world survival effectiveness of edaravone in amyotrophic lateral sclerosis: a propensity score weighted, registry-based, Canada-wide cohort study**

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Background: ALS is a progressive neurodegenerative disease without a cure and limited treatment options. Edaravone, a free radical scavenger, was shown to slow disease progression in a select group of patients with ALS over 6 months; however, the effect on survival was not investigated in randomized trials. The objective of this study is to describe real-world survival effectiveness over a longer timeframe. **Methods:** This retrospective cohort study included patients with ALS across Canada with symptom onset up to three years. Those with a minimum 6-month edaravone exposure between 2017 and 2022 were enrolled in the interventional arm, and those without formed the control arm. The primary outcome of tracheostomy-free survival was compared between the two groups, accounting for age, sex, ALS-disease progression rate, disease duration, pulmonary vital capacity, bulbar ALS-onset, and presence of frontotemporal dementia or C9ORF72 mutation using inverse propensity treatment weights. **Results:** 182 patients with mean \pm SD age 60 \pm 11 years were enrolled in the edaravone arm and 860 in the control arm (mean \pm SD age 63 \pm 12 years). Mean \pm SD time from onset to edaravone initiation was 18 \pm 10 months. Tracheostomy-free survival will be calculated. **Conclusions:** This study will provide evidence for edaravone effectiveness on tracheostomy-free survival in patients with ALS.