decreasing IgG recycling and reducing pathogenic IgG autoantibody levels. ADHERE assessed the efficacy and safety of efgartigimod PH20 subcutaneous (SC; co-formulated with recombinant human hyaluronidase PH20) in chronic inflammatory demyelinating polyneuropathy (CIDP). Methods: ADHERE enrolled participants with CIDP (treatment naive or on standard treatments withdrawn during run-in period) and consisted of open-label Stage A (efgartigimod PH20 SC once weekly [QW]), and randomized (1:1) Stage B (efgartigimod or placebo QW). Primary outcomes were clinical improvement (assessed with aINCAT, I-RODS, or mean grip strength; Stage A) and time to first aINCAT score deterioration (relapse; Stage B). Secondary outcomes included treatment-emergent adverse events (TEAEs) incidence. Results: 322 participants entered Stage A. 214 (66.5%) were considered responders, randomized, and treated in Stage B. Efgartigimod significantly reduced the risk of relapse (HR: 0.394; 95% CI: 0.25–0.61) versus placebo (p=0.000039). Reduced risk of relapse occurred in participants receiving corticosteroids, intravenous or SC immunoglobulin, or no treatment before study entry. Most TEAEs were mild to moderate; 3 deaths occurred, none related to efgartigimod. Conclusions: Participants treated with efgartigimod PH20 SC maintained a clinical response and remained relapse-free longer than those treated with placebo.

D.2

Cost-effectiveness analysis of efgartigimod vs chronic IVIg for treatment of patients with generalized myasthenia gravis in Canada

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Background: Efgartigimod is a human IgG1 antibody Fc fragment recently approved by Health Canada for patients with acetylcholine receptor antibody positive (AChR-Ab+) generalized myasthenia gravis (gMG). We assessed cost-effectiveness of efgartigimod vs chronic IVIg for adult patients with AChR-Ab+ gMG. Methods: A Markov model estimated costs (treatment and administration, disease monitoring, complications from chronic corticosteroid use, exacerbation and crisis management, adverse events, end-of-life care) and benefits (quality-adjusted life-years [QALYs]). The analysis was conducted from the Canadian healthcare system perspective. Health state transition probabilities were estimated using data from ADAPT, ADAPT+, and a network meta-analysis comparing efgartigimod with chronic IVIg. Utility values were obtained from MyRealWorld MG. Patients with MG-ADL ≥5 who did not die/discontinue were assumed to receive treatment every 4 weeks or every 3 weeks over the lifetime horizon. Results: Over the lifetime horizon, efgartigimod and chronic IVIg were predicted to have total discounted QALYs of 16.80 and 13.35, and total discounted costs of $1,913,294 and $2,170,315, respectively. Efgartigimod dominated chronic IVIg with incremental QALYs of 3.45 and cost savings of $257,020 over the lifetime horizon. Conclusions: Efgartigimod may provide greater benefit at lower costs than chronic IVIg for Canadian patients with AChR-Ab+ gMG, with substantial healthcare system savings over the lifetime horizon.

D3

Interim results for Myasthenia Gravis-resource utilization, epidemiology, survival & treatment patterns (MG-REST) study in Ontario, Canada

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Background: Reliable real-world data on the burden of MG is needed to inform Canadian clinical and policy decisions in the era of new MG therapeutics, including FcRn inhibitors. Given the lack of recent Canadian data on MG disease burden, the MG-REST Study aims to estimate the clinical burden of MG in Ontario. Methods: Ontario administrative data from ICES were utilized for a retrospective population-based cohort study of adults with MG identified through a validated algorithm (April 2013-March 2019) and followed for up to seven years (March 2020) to determine myasthenic crisis characteristics and overall survival (OS). Results: The MG cohort (n=2,601) had an average age of 65.7 years and 53.3% were males. Incidence of first myasthenic crisis was 9%, with 87% of events occurring at/after diagnosis. MG OS was 89%, 85% and 75% at 1-year, 2-years and 5-years, respectively, while OS after first crisis was 60%, 52%, and 39% for the same years. Conclusions: Despite the availability of conventional therapies throughout the study, MG crisis remains a serious, common complication of MG, with decreased survival at 1-year post-crisis (29% difference versus 1-year OS following MG diagnosis). Study highlights MG burden and unmet need for new effective therapies for MG treatment.

D.4

Safety and efficacy of delandistrogene moxeparvovec versus placebo in Duchenne muscular dystrophy (EMBARK): Pivotal Phase 3 primary results

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Background: Duchenne muscular dystrophy (DMD) is caused by DMD gene mutations. Delandistrogene moxeparvovec is an investigational gene transfer therapy, developed to address the underlying cause of DMD. We report findings from Part 1 (52 weeks) of the two-part EMBARK trial (NCT05096221). Methods: Key inclusion criteria: Ambulatory patients aged ≥4-<8 years with a confirmed DMD mutation within exons 18–79 (inclusive); North Star Ambulatory Assessment (NSAA) score >16 and <29 at screening. Eligible patients were randomized 1:1 to intravenous delandistrogene moxeparvovec (1.33×1014 vg/kg) or placebo. The primary endpoint was