gross motor (limb/axial motor items) and ocular (ocular/facial muscles) domains. **Results:** Eculizumab-treated patients showed improvements in all four QMG domain scores to week 26. Rapid, sustained improvements were demonstrated across all domains, with a trend toward significant differences between eculizumab and placebo (bulbar, p=0.0628; respiratory, p=0.0682; gross motor, p=0.0114; ocular, p=0.0017). The eculizumab safety profile was consistent with previous reports. **Conclusions:** Eculizumab demonstrated a consistent response across all QMG muscle domains. This aligns with previously reported MG-ADL findings with eculizumab. (NCT01997229).

P.024

Long-term use of patisiran in patients with hereditary transthyretin amyloidosis (hATTR): 12 month efficacy & safety data from a global open label extension (OLE) study

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Background: Hereditary transthyretin-mediated (hATTR) amyloidosis is a multi-systemic, heterogenous, life-threatening disease. Patisiran resulted in significant improvement in neuropathy and QoL at 18-months compared to placebo, and was generally well-tolerated in the Phase 3 APOLLO study. **Methods:** Multi-center, OLE study to evaluate the efficacy and safety of long-term patisiran dosing for ≤ 5 years in hATTR amyloidosis patients with polyneuropathy who have completed the APOLLO study (NCT02510261). Endpoints include safety, tolerability and long-term efficacy of patisiran. Measures of clinical benefit are the same endpoints used in APOLLO including changes in mNIS+7 composite neuropathy impairment score and QoL (Norfolk QoL-DN) **Results:** As of December 2017, 184 of 186 (99%) patients who completed APOLLO and 25 patients from the Ph 2 OLE study enrolled in the Global OLE study. Baseline data for 211(APOLLO/placebo, n=49; APOLLO/patisiran, n=137 and patisiran Ph 2 OLE, n=25) patients included: median age 61 years (26-84); 74% males; 46% V30M. Interim safety data and 12-month efficacy results will be presented. **Conclusions:** The global OLE study includes a diverse population of hATTR amyloidosis patients. Interim data will include the long-term safety and maintenance of effect in patients continuing on patisiran, as well as the impact of treatment with patisiran on patients previously treated with placebo.

P.025

APOLLO, a phase 3 study of patisiran for the treatment of hereditary transthyretin amyloidosis (hATTR): 18-month safety and efficacy in subgroup with cardiac involvement

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Background: Hereditary transthyretin-mediated (hATTR) amyloidosis a hereditary, multi-systemic and life-threatening disease resulting in neuropathy and cardiomyopathy. In the APOLLO study, patisiran, an investigational RNAi therapeutic targeting hepatic TTR production resulted in significant improvement in neuropathy and QoL compared to placebo and was generally well tolerated. **Methods:** APOLLO, a Phase 3 study of patisiran vs. placebo (NCT01960348) prespecified a cardiac subpopulation (n=126 of 225 total) that included patients with baseline left ventricular (LV) wall thickness ≥ 13mm and no medical history of aortic valve disease or hypertension. Cardiac measures included structure and function by electrocardiography, changes in NT-proBNP and 10-MWT gait speed. **Results:** At 18 months, patisiran treatment resulted in a mean reduction in LV wall thickness of 1 mm (p=0.017) compared to baseline, which was associated with significant improvements relative to placebo in LV end diastolic volume (+8.31 mL, p=0.036), global longitudinal strain (-1.37%, p=0.015) and NT-proBNP (55% reduction, p=7.7 x 10-8) (Figure 1). Gait speed was also improved relative to placebo (+0.35 m/sec, p=7.4 x 10-**9**). Rate of death or hospitalization was lower with patisiran. mNIS+7 results in the cardiac subpopulation will also be presented. **Conclusions:** These data suggest patisiran has the potential to halt or reverse cardiac manifestations of hATTR amyloidosis.

P.026

Response to eculizumab in patients with myasthenia gravis recently treated with chronic intravenous immunoglobulin

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Background: Chronic intravenous immunoglobulin (IVIg) is used to treat refractory myasthenia gravis (MG). This subgroup analysis evaluated response to eculizumab in patients receiving chronic IVIg before entry to REGAIN, a phase 3, randomized, double-blind, placebo-controlled study of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalized MG. **Methods:** IVIg was only permitted during REGAIN as rescue therapy; previously treated patients underwent a 4-week washout before randomization. Patients included in this analysis had received chronic IVIg ≥4 times in 1 year, with ≥1 dose within 6 months before REGAIN entry. Exacerbations and MG status changes were assessed. **Results:** Eighteen patients were evaluated; four experienced exacerbations (eculizumab-treated, 1/9; placebo-treated, 3/9). Clinically relevant improvements were larger with eculizumab than placebo, respect-