In this interim analysis of RAISE-XT, zilucoplan is a heterogeneous, chronic, unpredictable neuromuscular disease. Longer exposure to zilucoplan did not lead to higher E2. In total, 199 patients entered the RAISE-XT study. Secondary efficacy endpoints include change from RAISE-XT baseline to Extension Week 12 (Week E12) during zilucoplan treatment in the extension double-blind parent studies continued to improve further from Week E1 through to Week E12. The primary safety endpoint was incidence of TEAEs. The assessment of TEAEs includes TEAEs from the start of the open-label extension periods of the Phase 2 and Phase 3 studies. Secondary efficacy endpoints include change from RAISE-XT baseline to double-blind period to RAISE-XT E12 to Phase 3 (RAISE; NCT04115293) double-blind, randomized, placebo-controlled study. The study design is shown in Figure 1. A small number of patients received zilucoplan 0.5 mg/kg in Phase 2, the data from these patients are not shown separately here, but are included in the zilucoplan doses group. All patients self-administered daily SC injections of zilucoplan 0.3 mg/kg. The safety and efficacy of zilucoplan in patients with gMG who had previously participated in a parent zilucoplan Phase 2 or 3 study. In this interim analysis, patients who had received a median of 253 days of exposure to zilucoplan. gMG is a heterogeneous, chronic, unpredictable neuromuscular disease characterized by fluctuating fatigable muscle weakness. Zilucoplan is not approved for treatment of myasthenia gravis by any health authority. 8 (8.9) 6 (6.5) 14 (7.0)

MG-ADL score in patients who received placebo vs Week E12 (95% CI).

**Conclusions**

- In this interim analysis of RAISE-XT, zilucoplan demonstrated a favorable long-term safety profile over 24 weeks with no major safety concerns, and good tolerability, as well as improvements in signs and symptoms of the disease activity.
- Longer exposure to zilucoplan did not lead to higher rates of TEAEs overall vs the placebo-switch group.
- In participants who received zilucoplan 0.3 mg/kg treatment during the parent studies, MG-ADL, QMG, MGC, and MG-QoL 15r scores continued to improve further through to Week E12.
- In participants who received placebo during the parent studies, rapid improvements were observed as early as Week 12 after switching to zilucoplan, and continued through to Week E12.
- RAISE-XT is ongoing and additional long-term safety and efficacy data will be assessed.