

Improvements in Myasthenia Gravis Symptoms Patient-Reported Outcomes during long-term cyclic rozanolixizumab treatment

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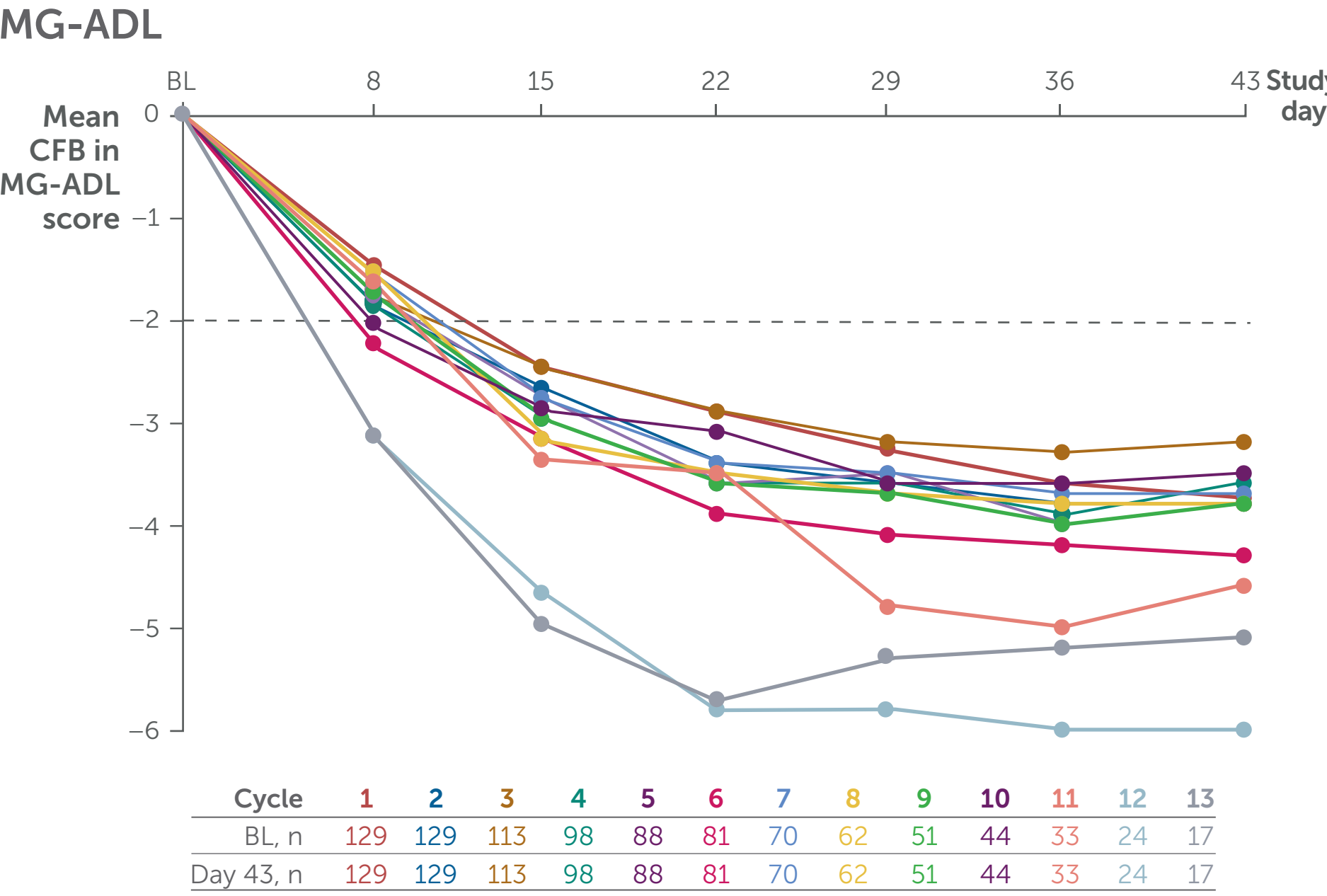
Introduction

- Patients living with MG experience fluctuating and variable symptoms, including muscle weakness, muscle fatigability and fatigue¹
- The MG Symptoms PRO scales provide a granular assessment of patients' experiences of MG symptoms, including physical fatigue and muscle weakness fatigability, both of which are important but not evaluated by the MG-ADL scale¹
- Rozanolixizumab, a humanized IgG4 mAb that targets and blocks FcRn with high affinity, is approved for the treatment of adults with anti-AChR Ab+ or anti-MuSK Ab+ gMG^{2,3}
- In the Phase 3 MycarinG (NCT03971422) and OLE studies (MG0004 [NCT04124965] and MG0007 [NCT04650854]), clinically meaningful improvements in MG-ADL scores were observed with long-term cyclic rozanolixizumab treatment^{4,5} (**Figure 1**)
 - Improvements were also observed in other MG-specific outcomes, including MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue and Bulbar Muscle Weakness scale scores⁵
- Building on these findings, this analysis assessed the effect of long-term cyclic rozanolixizumab treatment, without rescue therapy, on MG Symptoms PRO scale scores across MycarinG and its OLE studies**

Methods

- In MycarinG, patients were aged ≥18 years with MGFA Clinical Class II–IVa anti-AChR Ab+ or anti-MuSK Ab+ gMG with an MG-ADL score ≥3 (for non-ocular symptoms) and a QMG score ≥11
- Following MycarinG, patients could enroll in OLE studies MG0004, then MG0007, or MG0007 directly
 - In MG0004, patients received once-weekly rozanolixizumab 7 mg/kg or 10 mg/kg for ≤52 weeks
 - MG0007 comprised an initial 6-week cycle of rozanolixizumab 7 mg/kg or 10 mg/kg, with further cycles administered upon symptom worsening at the investigator's discretion
- Efficacy data were pooled for patients with ≥2 symptom-driven rozanolixizumab treatment cycles across MycarinG, MG0004 (first 6 weeks) and MG0007; up to 13 cycles are reported
- Safety data were pooled for patients receiving ≥1 rozanolixizumab treatment cycle with a follow-up period of up to 8 weeks across MycarinG and MG0007
- CFB scores in MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue and Bulbar Muscle Weakness were assessed to Day 43 of each cycle
- MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue and Bulbar Muscle Weakness responses without rescue therapy were assessed at Day 43 of each cycle

Figure 1 Rozanolixizumab showed clinically meaningful improvements in MG-ADL scores from baseline to Day 43⁵



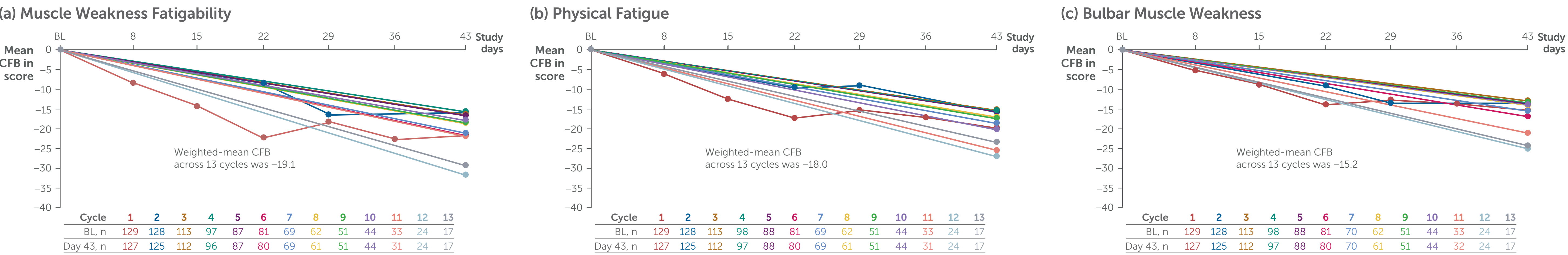
For patients who received rescue therapy, efficacy data collected at or after the time of its initiation were excluded from the analysis, with no imputation of missing data for the respective cycle. The reference value for clinically meaningful change was a 2.0-point improvement in MG-ADL score.

- Responder cut-offs were defined using the reference values for meaningful within-patient change: a 16.67-point improvement for Muscle Weakness Fatigability and a 20.00-point improvement for each of Physical Fatigue and Bulbar Muscle Weakness¹

Results

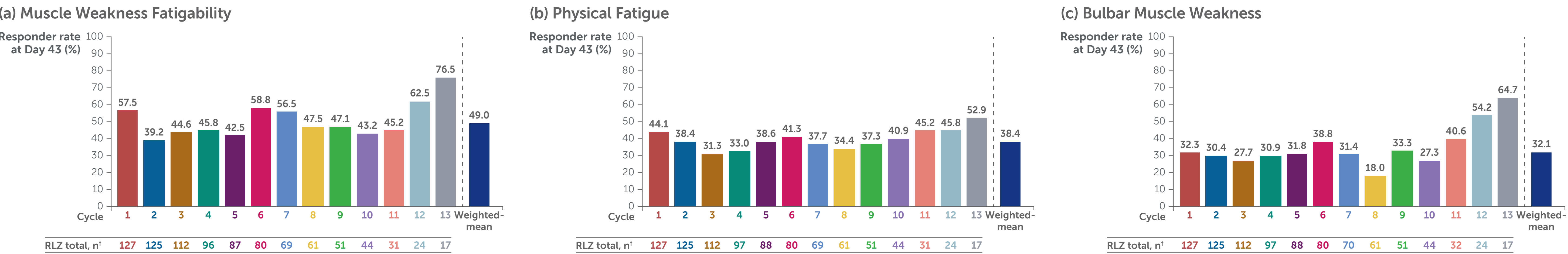
- Overall, 129 patients received ≥2 symptom-driven cycles of rozanolixizumab
- Consistent improvements from baseline in MG Symptoms PRO scale scores were observed across repeated treatment cycles, without rescue therapy, with improvements observed as early as 1 week after the first infusion in Cycle 1 (**Figure 2**)
- The proportion of patients achieving an MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue or Bulbar Muscle Weakness response was broadly consistent within each scale across repeated cycles up to Cycle 11, with variability thereafter (**Figure 3**)
 - Across all cycles, average response rates of 49.0% (Muscle Weakness Fatigability), 38.4% (Physical Fatigue) and 32.1% (Bulbar Muscle Weakness) were observed (weighted-mean)
- Over a median follow-up of 2 years, 93.1% (n=175/188) of patients experienced a TEAE; most were mild or moderate
 - The most common TEAEs were headache (50.0%), diarrhea (33.5%), COVID-19 (21.8%) and pyrexia (20.7%)

Figure 2 Consistent improvements in MG symptoms PRO scale scores were observed across repeated treatment cycles, without rescue therapy, for (a) Muscle Weakness Fatigability, (b) Physical Fatigue and (c) Bulbar Muscle Weakness



For patients who received rescue therapy, efficacy data collected at or after the time of its initiation were excluded from the analysis, with no imputation of missing data for the respective cycle. The reference values for meaningful within-patient change for the MG Symptoms PRO scales were a 16.67-point improvement for Muscle Weakness Fatigability and a 20.00-point improvement for Physical Fatigue and Bulbar Muscle Weakness.

Figure 3 The proportion of patients achieving the responder cut-offs across repeated cycles was broadly consistent up to Cycle 11 within (a) Muscle Weakness Fatigability, (b) Physical Fatigue and (c) Bulbar Muscle Weakness



MG Symptoms PRO Muscle Weakness Fatigability, Physical Fatigue and Bulbar Muscle Weakness responses were defined as a ≥16.67-point, ≥20.00-point and ≥20.00-point within-patient improvement in score from baseline, respectively. Patients with missing data at Day 43 or who received rescue therapy after Day 43 were treated as missing. 'n' represents the number of patients who had completed the relevant MG Symptoms PRO scale assessment at Day 43 in each treatment cycle.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; BL, baseline; CFB, change from baseline; FcRn, neonatal fragment crystallizable receptor; gMG, generalized myasthenia gravis; IgG4, immunoglobulin G subunit 4; mAb, monoclonal antibody; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; OLE, open-label extension; PRO, patient-reported outcome; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; TEAE, treatment-emergent adverse event.

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Summary and conclusions

- The MG Symptoms PRO scales provide a granular, patient-centric assessment of relevant MG symptoms and uniquely capture physical fatigue, which is a key symptom not evaluated by other MG-specific measures
- Repeated cyclic rozanolixizumab treatment led to consistent and clinically meaningful improvements in MG symptoms, as measured by the MG-ADL and MG Symptoms PRO scales
- MG Symptoms PRO responder rates were broadly consistent within each scale across repeated cycles, with some variability observed in later cycles
- These data suggest that repeated cyclic rozanolixizumab treatment may lead to improvement in the symptoms of most relevance to patients with gMG

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