

Efficacy and safety of rozanolixizumab treatment cycles in patients with generalised myasthenia gravis: Final pooled analysis of Phase 3 studies

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Introduction

- The FcRn inhibitor rozanolixizumab significantly improved all MG-specific outcomes after one 6-week treatment cycle and was generally well tolerated in adults with gMG in the Phase 3 MycarinG study (MG0003/NCT03971422)¹
- After MycarinG, patients could enrol in OLE studies MG0004 then MG0007, or MG0007 directly (**Figure 1**); these studies are now complete
 - In MG0004 (NCT04124965), patients received chronic, once-weekly rozanolixizumab for ≤52 weeks
 - In MG0007 (NCT04650854), patients received once-weekly rozanolixizumab in an initial 6-week cycle, with subsequent cycles administered upon symptom worsening at the investigator's discretion
- This analysis assessed the efficacy and safety of rozanolixizumab over multiple symptom-driven cycles in patients with gMG

Methods

- MycarinG enrolled adults with MGFA Disease Class II–IVa anti-AChR Ab+ or anti-MuSK Ab+ gMG, MG-ADL score ≥3 (for non-ocular symptoms) and QMG score ≥11¹
 - The primary endpoint was CFB to Day 43 in MG-ADL score; secondary endpoints included CFB to Day 43 in QMG score¹
- Final data were pooled across MycarinG, MG0004 (first 6 weeks; efficacy outcomes only) and MG0007
 - Efficacy variables (mean CFB to Day 43 in MG-ADL, QMG and MGC scores) were assessed for patients with ≥2 symptom-driven cycles (17 cycles assessed; up to 13 cycles are reported)
 - Safety variables (incidence of TEAEs) were assessed for patients with ≥1 treatment cycle with a follow-up period of ≤8 weeks
 - All analyses are based on observed data

Results

- Overall, 196 patients received ≥1 dose of rozanolixizumab, of whom:
 - 188 received ≥1 treatment cycle (primary safety pool)
 - 129 received ≥2 symptom-driven cycles (primary efficacy pool)

- Baseline characteristics were similar between the two pools and the two dose groups, and were indicative of a population with moderate-to-severe gMG
 - 90.7% of patients had anti-AChR Ab+ gMG and 9.3% had anti-MuSK Ab+ gMG
 - Mean (SD) baseline scores were 8.7 (3.4) for MG-ADL and 16.0 (3.8) for QMG (primary efficacy pool)
- Patients who were in the studies for >12 months received a mean (SD) of 4.1 (1.7) and median of 4.0 cycles in the first year, corresponding to a mean (SD) of 22.0 (8.9) and median of 23.0 infusions (primary safety pool)
 - At the population level, this equates to an expected treatment pattern in the first year of 6 weeks of rozanolixizumab treatment followed by a 6–8-week treatment-free interval, which can be adjusted for individual patients as needed
 - All treated patients (with any follow-up duration) received a mean (SD) annualised number of 2.9 (1.8) cycles per year, corresponding to a mean of 16.0 (10.6) infusions per year
- Mean CFB in MG-ADL, MGC and QMG scores were consistent over time for each cycle, with rapid onset of effect by Day 8 and clinically meaningful improvement sustained from Day 15 to Day 43 (**Figure 2**)
- For most cycles up to Cycle 13, the most frequent treatment-free interval duration was ≥4 to <8 weeks (**Figure 3**)
- Overall, 175/188 (93.1%) patients experienced any TEAE (**Figure 4**); most events were mild or moderate
 - The incidence of TEAEs did not increase with repeated cyclic treatment
 - The most common TEAEs were headache (94/188 [50.0%]), diarrhoea (63/188 [33.5%]) and COVID-19 (41/188 [21.8%]); the studies were conducted during the COVID-19 pandemic
 - There were no anaphylactic reactions or clinically meaningful changes in lipid or albumin levels

Summary and conclusions

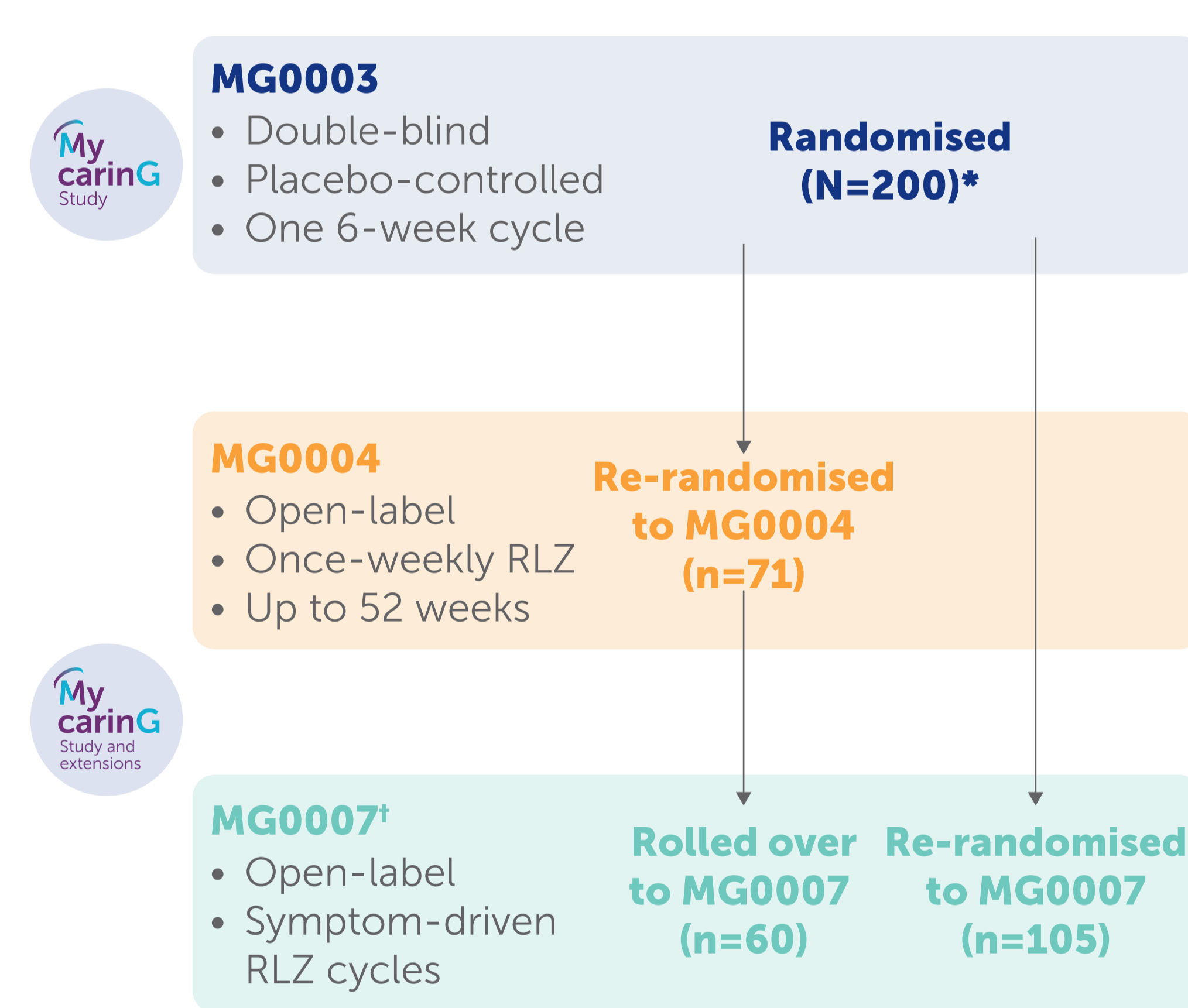
Pooled data from the MycarinG and OLE studies provide insights into the efficacy and safety of rozanolixizumab over multiple symptom-driven cycles in patients with gMG

Rozanolixizumab showed consistent and clinically meaningful improvements across multiple MG-specific outcomes with repeated cyclic treatment for up to 13 cycles

Rozanolixizumab was generally well tolerated, and the data were consistent with the known rozanolixizumab safety profile

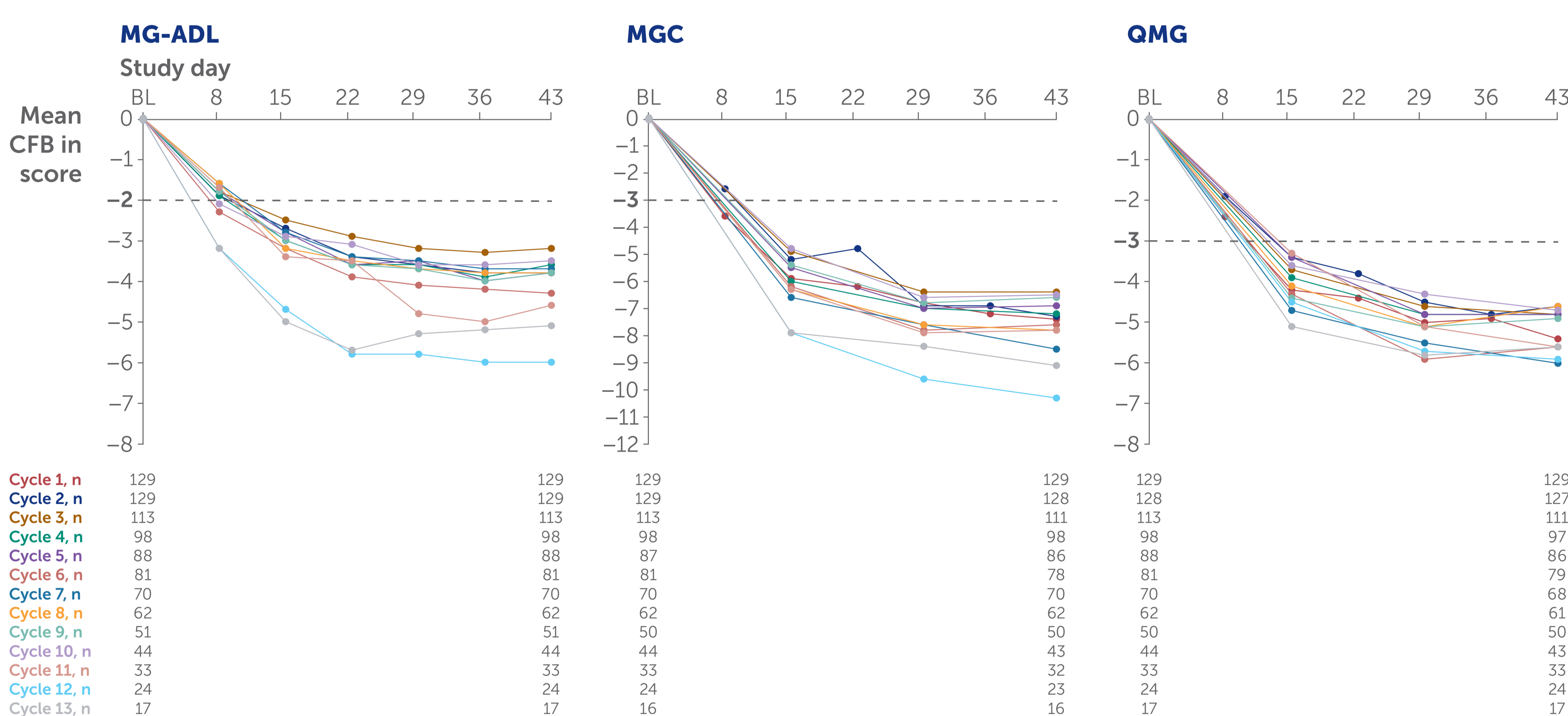
These efficacy and safety data from MycarinG and the OLE studies support the long-term use of repeated cycles of rozanolixizumab in patients with gMG

Figure 1 MycarinG and OLE studies: Design and patient flow



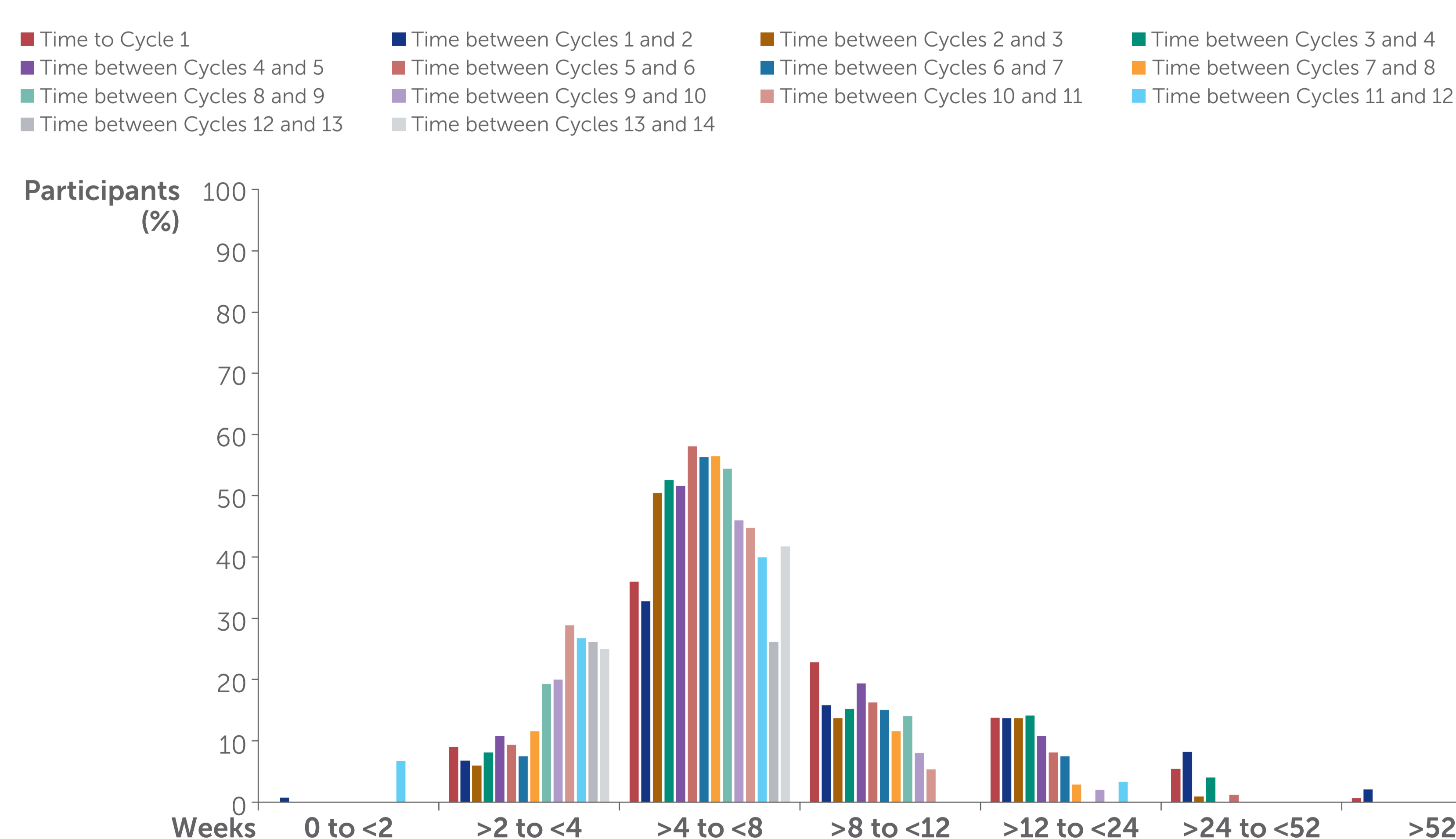
*Placebo (n=67), rozanolixizumab (n=133). ¹After the initial cycle, dose modifications from 10 mg/kg to 7 mg/kg and vice versa were permitted at the beginning of each treatment cycle provided the benefit-risk ratio remained favourable for the patient.

Figure 2 Rozanolixizumab showed clinically meaningful improvements in MG-ADL, MGC and QMG scores from baseline to Day 43



Efficacy data collected at or after the timepoint of rescue use are excluded from the analysis with no imputation of missing data for the respective cycle. Dashed lines correspond to clinically meaningful thresholds of -2 points for MG-ADL and -3 points for MGC and QMG.

Figure 3 The most frequently occurring treatment-free interval duration was ≥4 to <8 weeks



Data are reported for patients who had received RLZ treatment and have initiated or are awaiting a treatment cycle based on symptom worsening at the investigator's discretion. Patients without a symptom-driven cycle after RLZ treatment are censored at the time of dropping out or at the end of the study (MycarinG or MG0007). Number of censored patients: Time to Cycle 1, n=21; Time between Cycles 1 and 2, n=29; Time between Cycles 2 and 3, n=18; Time between Cycles 3 and 4, n=6; Time between Cycles 4 and 5, n=7; Time between Cycles 5 and 6, n=6; Time between Cycles 6 and 7, n=11; Time between Cycles 7 and 8, n=12; Time between Cycles 8 and 9, n=7; Time between Cycles 9 and 10, n=12; Time between Cycles 10 and 11, n=8; Time between Cycles 11 and 12, n=7; Time between Cycles 12 and 13, n=11; Time between Cycles 13 and 14, n=4.

Figure 4 Across all cycles, rozanolixizumab was generally well tolerated

	RLZ 7 mg/kg (N=135) % (n)	RLZ 10 mg/kg (N=133) % (n)	RLZ total (N=188) % (n)
Any TEAE	83.0 (112)	94.7 (126)	93.1 (175)
Serious TEAEs	15.6 (21)	27.1 (36)	29.3 (55)
Permanent discontinuation from study due to TEAEs	8.1 (11)	16.5 (22)	17.6 (33)
Treatment-related TEAEs	48.9 (66)	63.2 (84)	63.8 (120)
Severe TEAEs	13.3 (18)	34.6 (46)	33.0 (62)
TEAEs leading to death*	0.7 (1)	2.3 (3)	2.1 (4)

*All deaths were considered not related to rozanolixizumab by the investigator. 'n' is the number of patients reporting at least one TEAE within the category.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; BL, baseline; CFB, change from baseline; FcRn, neonatal fragment crystallisable receptor; gMG, generalised myasthenia gravis; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; SD, standard deviation; TEAE, treatment-emergent adverse event.

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Reference: 1. Brill V, et al. Lancet Neurol. 2023;22(5):383–394.



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