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

Ali A. Habib[1], Carlo Antozzi[2], Artur Drużdż[3], Julian Grosskreutz[4], Sabrina Sacconi[5], Kimiaki Utsugisawa[6], John Vissing[7], Tuan Vu[8], Fiona Grimson[9], Belinda McDonough[9], Irene Pulido-Valdeolivas[10], Thaïs Tarancón[10], Vera Bril[11] on behalf of the MycarinG and MycarinG OLE study teams

[1]MDA ALS & Neuromuscular Center, Department of Neurology, University of California, Irvine, Orange, CA, USA; [2]Neuroimmunology and Muscle Pathology Unit, Multiple Sclerosis Center, Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Nazionale Neurologico Carlo Besta, Milan, Italy; [3]Department of Neurology, Municipal Hospital, Poznań, Poland; [4]Precision Neurology of Neuromuscular Diseases, Department of Neurology, University of Lübeck, Lübeck, Germany; [5]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [6]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [7]Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark; [8]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [9]UCB, Slough, UK; [10]UCB, Madrid, Spain; [11]Ellen and Martin Prosserman Centre for Neuromuscular Diseases, Toronto General Hospital, University of Toronto, Toronto, Ontario, Canada

MDA Conference 2026, Orlando, FL, USA; March 8–11, 2026

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 enroll 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 ≥11[1]
  - The primary endpoint was CFB to Day 43 in MG-ADL score; secondary endpoints included CFB to Day 43 in QMG score[1]
- 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) annualized 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

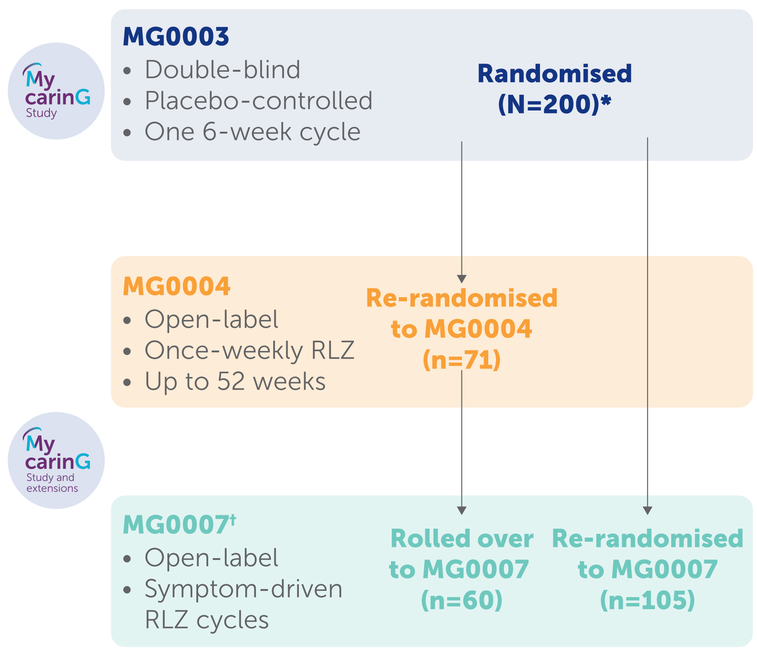
Pooled data from the MycarinG and OLE studies provide insights into the efficacy and safety of rozanolixizumab over multiple symptom-driven cycles in patients with gMG

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

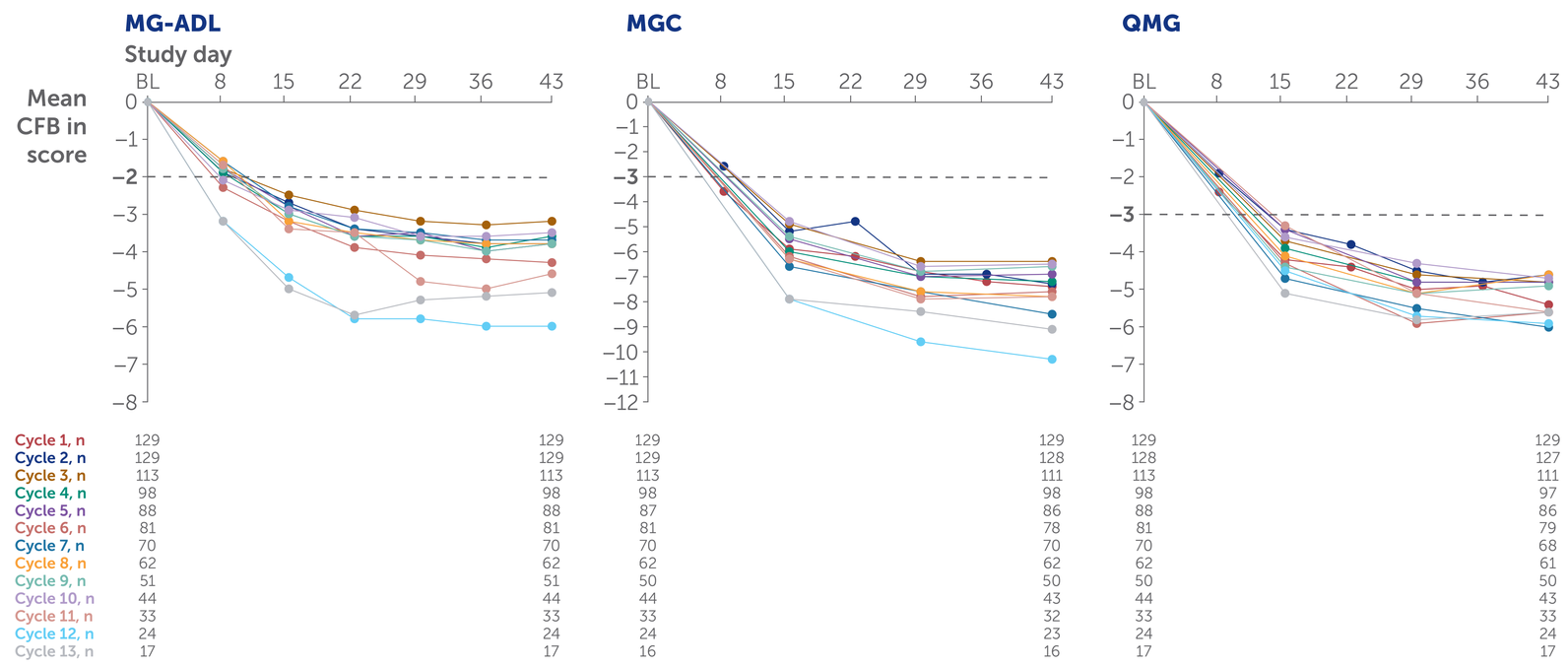
These efficacy and safety data from MycarinG and the OLE studies support the long-term use of repeated cycles of rozanolixizumab in patients with gMG

Figure 1 MycarinG and OLE studies: Design and patient flow



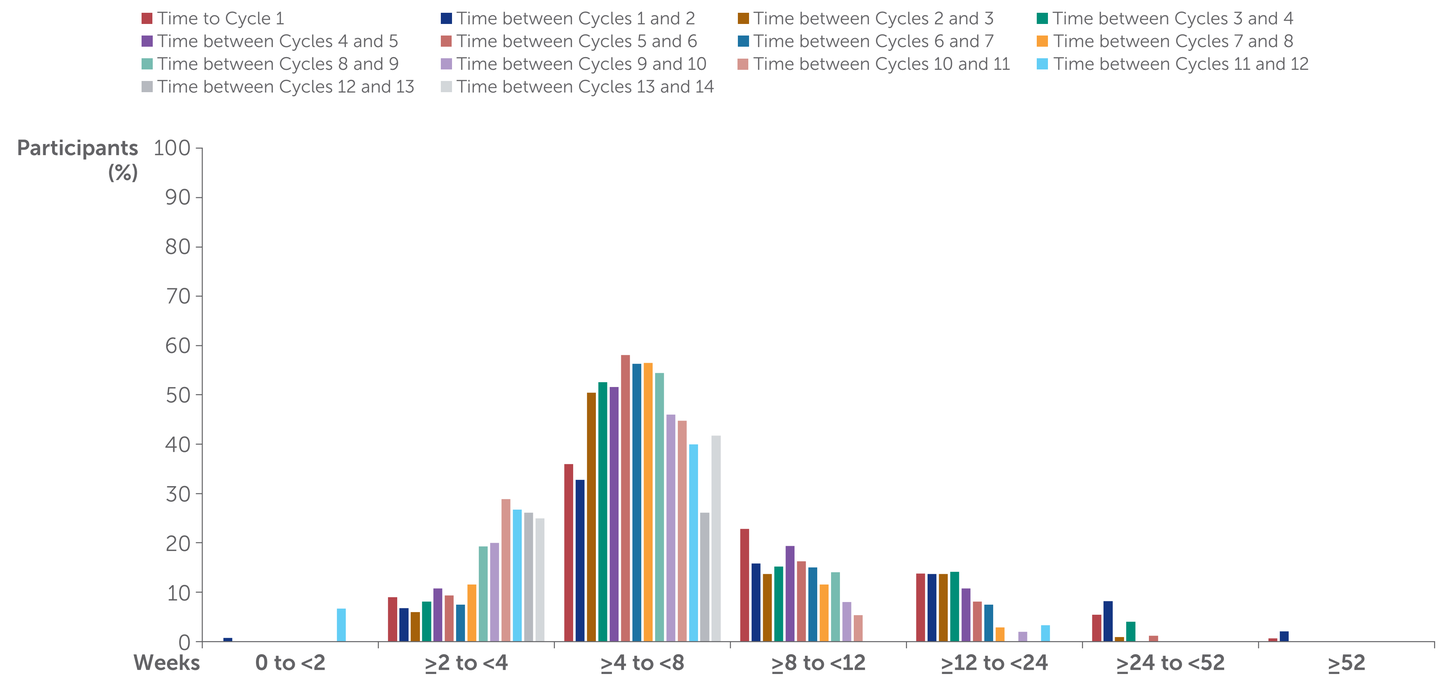
*Placebo (n=67), rozanolixizumab (n=133). †After the initial cycle, dose modifications from 10 mg/kg to 7 mg/kg and vice versa were permitted at the beginning of each treatment cycle provided the benefit–risk ratio remained favourable for the patient.

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



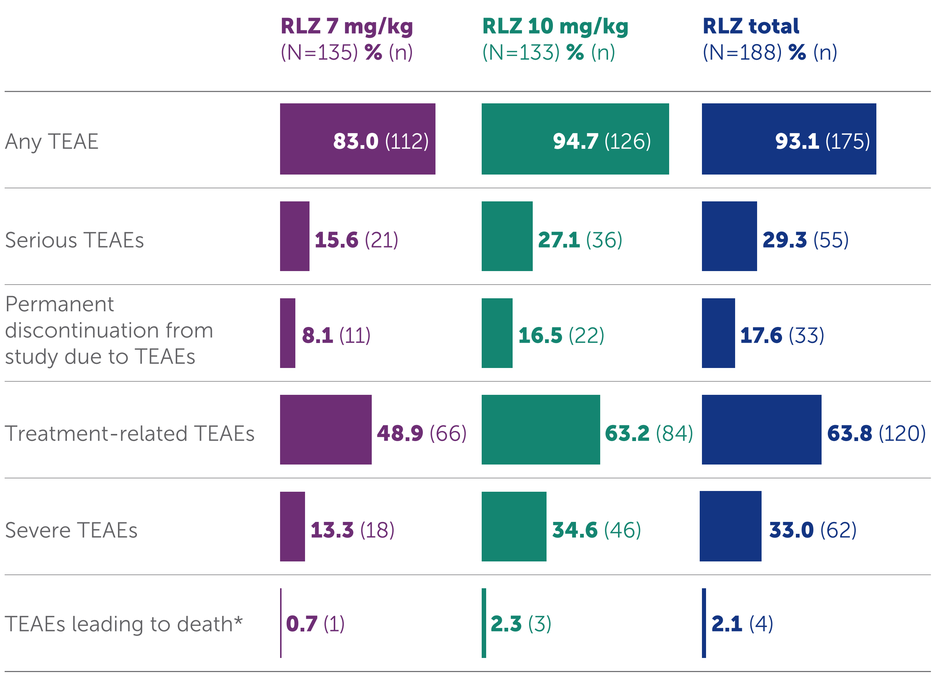
Efficacy data collected at or after the timepoint of rescue use are excluded from the analysis with no imputation of missing data for the respective cycle. Dashed lines correspond to clinically meaningful thresholds of –2 points for MG-ADL and –3 points for MGC and QMG.

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



*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.

Acknowledgements: This study was funded by UCB. The authors acknowledge Millie Hall, BSc, of Ogilvy Health, London, UK, for editorial assistance, which was funded by UCB. The authors thank Veronica Porkess, PhD, of UCB for publication and editorial support. The authors thank the patients and their caregivers, in addition to the investigators and their teams who contributed to this study.

Author disclosures: Ali A. Habib has received research support from Alexion Pharmaceuticals, argenx, Cabaletta Bio, Genentech/Roche, Immunovant, Regeneron Pharmaceuticals, UCB and Viela Bio (now Amgen). He has received honoraria from Alexion Pharmaceuticals, Alpine Immune Sciences, argenx, Genentech/Roche, Immunovant, Inhibrx, NMD Pharma, Regeneron Pharmaceuticals and UCB. Carlo Antozzi has received funding for congress and Institutional Review Board participation from Alexion Pharmaceuticals, argenx, Biogen, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Momenta (now Johnson & Johnson) and UCB. Artur Drużdż and Sabrina Sacconi have nothing to disclose. Julian Grosskreutz has served as a Consultant for Alexion Pharmaceuticals, Biogen and UCB, and his institution has received research support from the Boris Canessa Foundation. Kimiaki Utsugisawa has served as a paid Consultant for argenx, Chugai Pharmaceutical, HanAll Biopharma, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Merck, Mitsubishi Tanabe Pharma, UCB and Viela Bio (now Amgen); he has received speaker honoraria from Alexion Pharmaceuticals, argenx, the Japan Blood Products Organization and UCB. John Vissing has been a Consultant on advisory boards for Amicus Therapeutics, Biogen, Edgewise Therapeutics, Fulcrum Therapeutics, Genethon, Horizon Therapeutics (now Amgen), Lupin, ML Biopharma, Novartis, Regeneron Pharmaceuticals, Roche, Sanofi Genzyme (now Sanofi), Sarepta Therapeutics and UCB. He has received research and travel support and/or speaker honoraria from Alexion Pharmaceuticals, argenx, Biogen, Edgewise Therapeutics, Fulcrum Therapeutics, Lupin, Sanofi Genzyme (now Sanofi) and UCB. He is a Principal Investigator in clinical trials for Alexion Pharmaceuticals, argenx, Atamyo Therapeutics, Genethon, Horizon Therapeutics (now Amgen), Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), ML Biopharma, Novartis, Regeneron Pharmaceuticals, Roche, Sanofi Genzyme (now Sanofi) and UCB. Tuan Vu is the USF Site Principal Investigator for MG clinical trials sponsored by Alexion/AstraZeneca Rare Disease, Amgen, argenx, Cartesian Therapeutics, COUR Pharmaceuticals, Dianthus Therapeutics, Immunovant, Johnson & Johnson, NMD Pharma, Regeneron Pharmaceuticals and UCB, and has served as a speaker for Alexion/AstraZeneca Rare Disease, argenx and CSL Behring. He has performed consulting work for Alexion/AstraZeneca Rare Disease, argenx, Dianthus Therapeutics, ImmunAbs and UCB. Fiona Grimson, Belinda McDonough, Irene Pulido-Valdeolivas and Thaïs Tarancón are employees and shareholders of UCB. Vera Bril is a Consultant for Akcea, Alexion Pharmaceuticals, Alnylam, argenx, CSL, Grifols, Immunovant, Ionis, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Momenta (now Johnson & Johnson), Novo Nordisk, Octapharma, Pfizer, Powell Mansfield, Roche, Sanofi, Takeda Pharmaceuticals and UCB. She has received research support from Akcea, Alexion Pharmaceuticals, argenx, CSL, Grifols, Immunovant, Ionis, Momenta (now Johnson & Johnson), Octapharma, Takeda Pharmaceuticals, UCB and Viela Bio (now Amgen).

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

Please use this QR code to download a PDF of the poster.

These data were previously presented at the 15th MGFA International Conference 2025, The Hague, Netherlands; 13–15 May 2025.