Responder and minimal symptom expression rates with rozanolixizumab in generalised myasthenia gravis: Final pooled analysis of Phase 3 studies

Silvia Falso[1], Carlo Antozzi[2], Artur Drużdż[3], Julian Grosskreutz[4], Ali A. Habib[5], Sabrina Sacconi[6], Kimiaki Utsugisawa[7], John Vissing[8], Tuan Vu[9], Fiona Grimson[10], Thaïs Tarancón[11], Vera Bril[12], on behalf of the MycarinG and MycarinG OLE study teams

[1]Department of Neuroscience, Catholic University of the Sacred Heart, Rome, Italy; [2]Immunotherapy and Apheresis Unit and Neuroimmunology and Muscle Pathology Unit, Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto 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]MDA ALS & Neuromuscular Center, Department of Neurology, University of California, Irvine, Orange, CA, USA; [6]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [7]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [8]Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark; [9]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [10]UCB, Slough, UK; [11]UCB, Madrid, Spain; [12]Ellen and Martin Prosserman Centre for Neuromuscular Diseases, Toronto General Hospital, University of Toronto, Toronto, Ontario, Canada

SIN 2025, Padua, Italy; 24–28 October 2025

Introduction

- In the randomised, placebo-controlled, double-blind Phase 3 MycarinG study (MG0003/NCT03971422), one 6-week treatment cycle with the FcRn inhibitor rozanolixizumab significantly improved MG-specific outcomes versus placebo in patients with gMG[1]
- Following MycarinG, patients could enrol in the now completed OLE studies, MG0004 (NCT04124965) followed by MG0007 (NCT04650854), or MG0007 directly (**Figure 1**)
- This analysis aimed to assess MG-ADL, MGC and QMG responder rates, and achievement of MSE across repeated 6-week rozanolixizumab treatment cycles in patients with gMG

Methods

- MycarinG enrolled adults with MGFA Disease Class II–IVa, anti-AChR Ab+ or anti-MuSK Ab+ gMG with MG-ADL score ≥3 (for non-ocular symptoms) and QMG score ≥11[1]
  - Patients were randomised 1:1:1 to receive rozanolixizumab 7 mg/kg or 10 mg/kg or placebo once weekly for 6 weeks[1]
- In MG0004, patients received chronic, once-weekly rozanolixizumab 7 mg/kg or 10 mg/kg for ≤52 weeks
- In MG0007, patients received an initial 6-week cycle (rozanolixizumab 7 mg/kg or 10 mg/kg), with subsequent cycles administered upon symptom worsening, at the investigator's discretion
- Final efficacy data were pooled across MycarinG, MG0004 (first 6 weeks) and MG0007 for patients with ≥2 symptom-driven cycles
- For safety analysis, final data were pooled across MycarinG and MG0007 for patients with ≥1 treatment cycle followed by an up to 8-week follow-up period
- Efficacy outcomes assessed in this analysis were:
  - The proportion of patients achieving MG-ADL, MGC and QMG response (≥2.0-point, ≥3.0-point and ≥3.0-point improvement from baseline, respectively, without rescue therapy) at Day 43 in each 6-week cycle
  - Achievement of MSE (MG-ADL score of 0 or 1) at any visit for each 6-week cycle and observation period
- The incidence of TEAEs was also evaluated
- All data were summarised descriptively by cycle

Results

- Overall, 196 patients received ≥1 dose of rozanolixizumab
  - 129 patients received ≥2 symptom-driven cycles (efficacy pool)
  - 188 patients received ≥1 treatment cycle with rozanolixizumab (safety pool)
- Baseline characteristics were similar between the efficacy and safety pools, and balanced between the 7 mg/kg and 10 mg/kg groups
- For patients in the safety pool, the mean (SD) and median (range) total time in studies was 1.7 (0.9) and 2.0 (0.1–3.1) years, respectively
  - The mean (SD) annualised number of cycles and infusions were 2.9 (1.8) and 16.0 (10.6) per year, respectively
- High rates of MG-ADL, MGC and QMG response were observed at Day 43 in Cycle 1 for both rozanolixizumab dose groups and were consistently observed for up to 13 cycles (**Figures 2–4**)
- For the rozanolixizumab total group, achievement of MSE was consistent across Cycles 1 to 13 (**Figure 5**)
- In total, 83.0% (112/135) and 94.7% (126/133) of patients receiving rozanolixizumab 7 mg/kg and 10 mg/kg, respectively, experienced a TEAE (**Figure 6**); most events were mild or moderate

Summary and conclusions

This analysis pooled final data across MycarinG, MG0004 and MG0007 to assess MG-ADL, MGC and QMG responder rates and achievement of MSE during repeated 6-week rozanolixizumab treatment cycles in patients with gMG

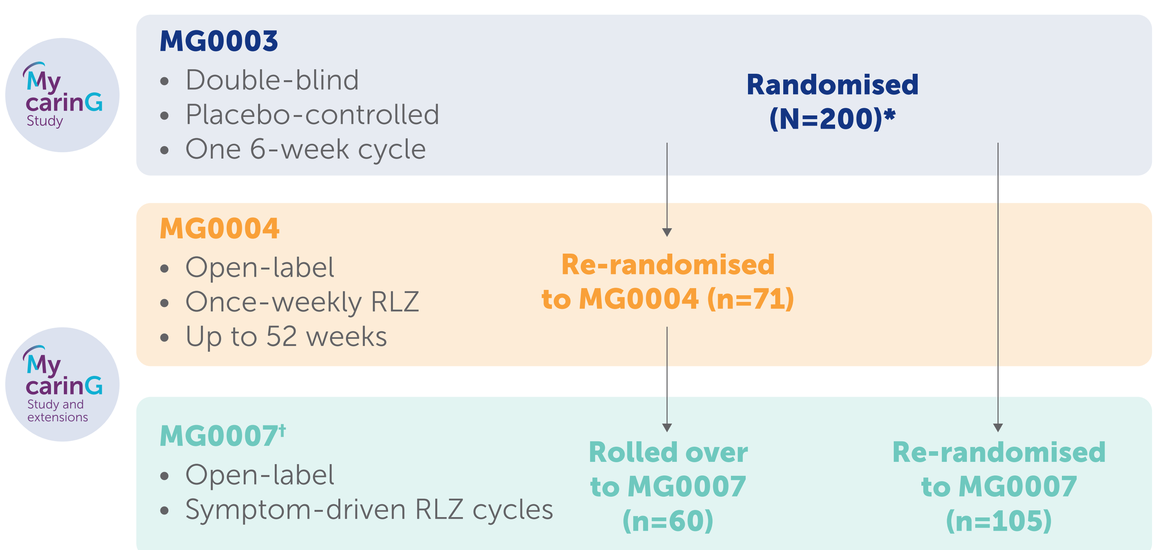
MG-ADL, MGC and QMG responder rates of ≥54.0% were consistently observed across Cycles 1–13

The proportion of patients who achieved MSE remained consistent across cycles for rozanolixizumab total

Rozanolixizumab had an acceptable safety profile and was generally well tolerated

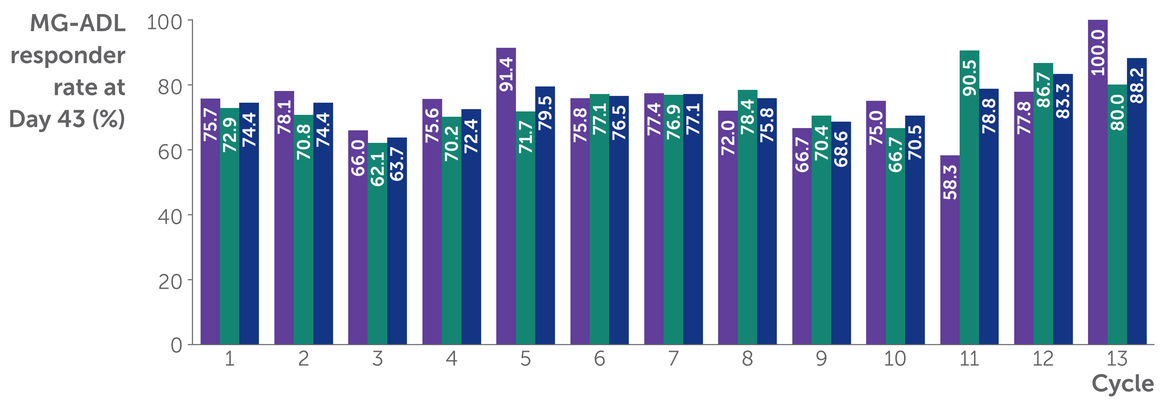
Rozanolixizumab showed consistent and clinically meaningful improvements across multiple MG-specific outcomes with repeated cyclic treatment for up to 13 cycles

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.

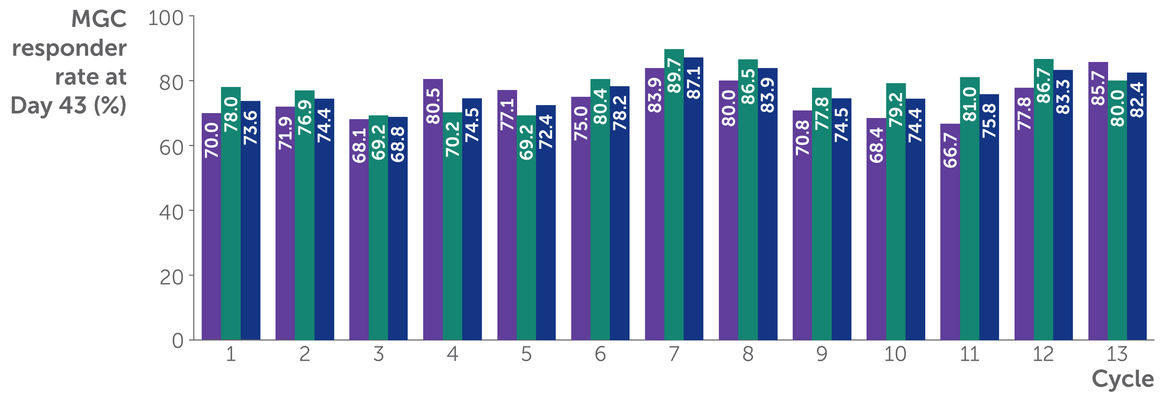
Figure 2 MG-ADL responder rates were high at Day 43 in Cycle 1 and showed a consistent profile over each subsequent cycle



RLZ 7 mg/kg, N*	70	64	47	41	35	33	31	25	24	20	12	9	7
RLZ 10 mg/kg, N*	59	65	66	57	53	48	39	37	27	24	21	15	10
RLZ total, N*	129	129	113	98	88	81	70	62	51	44	33	24	17

Efficacy pool. For rozanolixizumab total, MG-ADL responder rates ranged from 63.7% (72/113) to 88.2% (15/17). *N represents the number of patients who had completed an MG-ADL assessment at baseline (Day 1) and Day 43 in each treatment cycle.

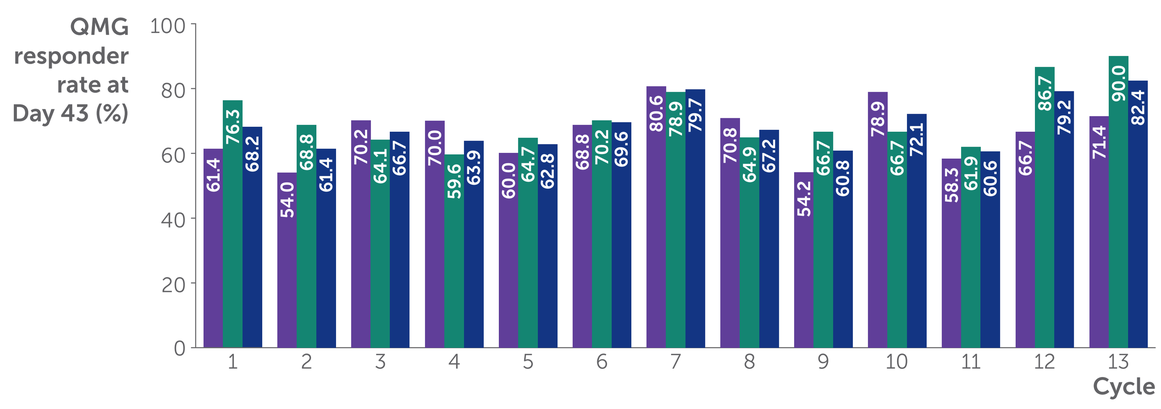
Figure 3 MGC responder rates were high at Day 43 in Cycle 1 and showed a consistent profile over each subsequent cycle



RLZ 7 mg/kg, N*	70	64	47	41	35	32	31	25	24	19	12	9	7
RLZ 10 mg/kg, N*	59	65	65	57	52	46	39	37	27	24	21	15	10
RLZ total, N*	129	129	112	98	87	78	70	62	51	43	33	24	17

Efficacy pool. For rozanolixizumab total, MGC responder rates ranged from 68.8% (77/112) to 87.1% (61/70). *N represents the number of patients who had completed an MGC assessment at baseline (Day 1) and Day 43 in each treatment cycle.

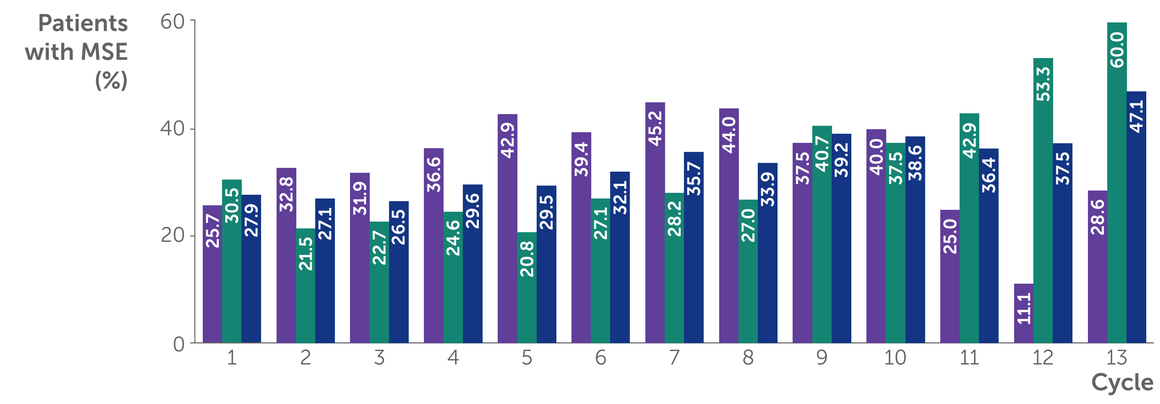
Figure 4 QMG responder rates were high at Day 43 in Cycle 1 and showed a consistent profile over each subsequent cycle



RLZ 7 mg/kg, N*	70	63	47	40	35	32	31	24	24	19	12	9	7
RLZ 10 mg/kg, N*	59	64	64	57	51	47	38	37	27	24	21	15	10
RLZ total, N*	129	127	111	97	86	79	69	61	51	43	33	24	17

Efficacy pool. For rozanolixizumab total, QMG responder rates ranged from 60.6% (20/33) to 82.4% (14/17). *N represents the number of patients who had completed a QMG assessment at baseline (Day 1) and Day 43 in each treatment cycle.

Figure 5 For the rozanolixizumab total group, MSE rates were consistent across Cycles 1 to 13



RLZ 7 mg/kg, N*	70	64	47	41	35	33	31	25	24	20	12	9	7
RLZ 10 mg/kg, N*	59	65	66	57	53	48	39	37	27	24	21	15	10
RLZ total, N*	129	129	113	98	88	81	70	62	51	44	33	24	17

Efficacy pool. For rozanolixizumab total, achievement of MSE ranged from 26.5% (30/113) to 47.1% (8/17). *N represents the number of patients with MSE assessment in the efficacy pool at each treatment cycle.

Figure 6 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)
Temporary discontinuation from study drug due to TEAEs	**15.6** (21)	**22.6** (30)	**24.5** (46)
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)

Safety pool. n is the number of patients reporting at least one TEAE within the category.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; FcRn, neonatal fragment crystallisable receptor; gMG, generalised myasthenia gravis; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MGC, Myasthenia Gravis Composite; MSE, minimal symptom expression; 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 Qudsia Ehsan, 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: Silvia Falso has nothing to disclose. 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 has 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. Ali A. Habib has received research support and/or honoraria from Alexion/AstraZeneca Rare Disease, Amgen, Arcellx, argenx, Cabaletta Bio, Cartesian Therapeutics, COUR Pharmaceuticals, Genentech/Roche, Grifols, Immunovant, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Kyverna, Merck, MGNet, Nkarta, NMD Pharma, Novartis, Regeneron Pharmaceuticals and UCB. Sabrina Sacconi has nothing to disclose. 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 and ImmunAbs. Fiona Grimson 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).

References: 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