Response to rozanolixizumab in patients with generalized myasthenia gravis: Final pooled analysis of MycarinG and open-label extension studies

Tuan Vu[1], Carlo Antozzi[2], Julian Grosskreutz[3], Ali A. Habib[4], Sabrina Sacconi[5], Kimiaki Utsugisawa[6], John Vissing[7], Fiona Grimson[8], Thaïs Tarancón[9], Vera Bril[10]

[1]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, 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]Precision Neurology of Neuromuscular Diseases, Department of Neurology, University of Lübeck, Lübeck, Germany; [4]MDA ALS & Neuromuscular Center, Department of Neurology, University of California, Irvine, Orange, CA, USA; [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]UCB, Slough, UK; [9]UCB, Madrid, Spain; [10]Ellen and Martin Prosserman Centre for Neuromuscular Diseases, Toronto General Hospital, University of Toronto, Toronto, Ontario, Canada

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Introduction

- Rozanolixizumab is a humanized IgG4 mAb FcRn blocker approved for the treatment of adults with anti-AChR Ab+ or anti-MuSK Ab+ gMG[1]
- In the double-blind, placebo-controlled, Phase 3 MycarinG study (MG0003/NCT03971422), one 6-week cycle of rozanolixizumab led to clinically meaningful and statistically significant improvements in MG-specific outcomes versus placebo and was generally well tolerated in adults with gMG[2]
- After MycarinG, patients could enroll in the now completed OLE studies: MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly
- Here, we evaluate the response to repeated rozanolixizumab cycles in patients with gMG using different MG-ADL and QMG responder thresholds

Methods

- In MycarinG, patients were randomized to receive subcutaneous infusions of rozanolixizumab 7 mg/kg, 10 mg/kg or placebo once weekly for 6 weeks
- In MG0004, patients received once-weekly rozanolixizumab 7 mg/kg or 10 mg/kg for up to 52 weeks
- In MG0007, following an initial 6-week rozanolixizumab 7 mg/kg or 10 mg/kg cycle, subsequent cycles were 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 an up to 8-week follow-up period across MycarinG and MG0007
- MG-ADL response was prespecified as a ≥2.0-point improvement in MG-ADL score without rescue therapy at Day 43 in each cycle; ≥3.0-point and ≥5.0-point thresholds were assessed *post hoc*
- QMG response was prespecified as a ≥3.0-point improvement in QMG score without rescue therapy at Day 43 in each cycle; a ≥5.0-point threshold was assessed *post hoc*
- Safety outcomes included the incidence of TEAEs

Results

- Overall, 129 patients received ≥2 symptom-driven cycles of rozanolixizumab
- Baseline demographics and disease characteristics were indicative of a broad population of patients with gMG (**Table 1**)
- Clinically meaningful (≥2.0-point) improvement in MG-ADL score was achieved by ≥63.7% of patients across Cycles 1–13 (**Figure 1**)
  - More stringent ≥3.0-point and ≥5.0-point improvements were achieved by ≥54.0% and ≥30.6% of patients, respectively, across all cycles up to Cycle 13
- Clinically meaningful (≥3.0-point) improvement in QMG score was achieved by ≥60.6% of patients across Cycles 1–13 (**Figure 2**)
  - A ≥5.0-point improvement was achieved by ≥40.2% of patients across all cycles up to Cycle 13
- At the group level, a mean improvement in MG-ADL score of approximately 3.0 points from baseline was maintained over 130 weeks of repeated rozanolixizumab treatment cycles (**Figure 3**)
- Over a total of 1,094 cycles, 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%)

Summary and conclusions

- Across Cycles 1–13, high MG-ADL responder rates were observed; in each cycle, more than 63% of patients achieved a ≥2.0-point improvement in MG-ADL score, and over 30% achieved a ≥5.0-point improvement
- Similar improvements were observed in QMG score – more than 60% of patients achieved a ≥3.0-point improvement, and over 40% achieved a ≥5.0-point improvement in each cycle
- At the group level, long-term efficacy was maintained over 130 weeks of cyclic rozanolixizumab treatment
- Clinically meaningful improvements in MG-specific outcomes were maintained over time, supporting a consistent response to rozanolixizumab across repeated treatment cycles

Figure 1 Rozanolixizumab demonstrated efficacy across 13 cycles using the prespecified and more stringent MG-ADL responder thresholds

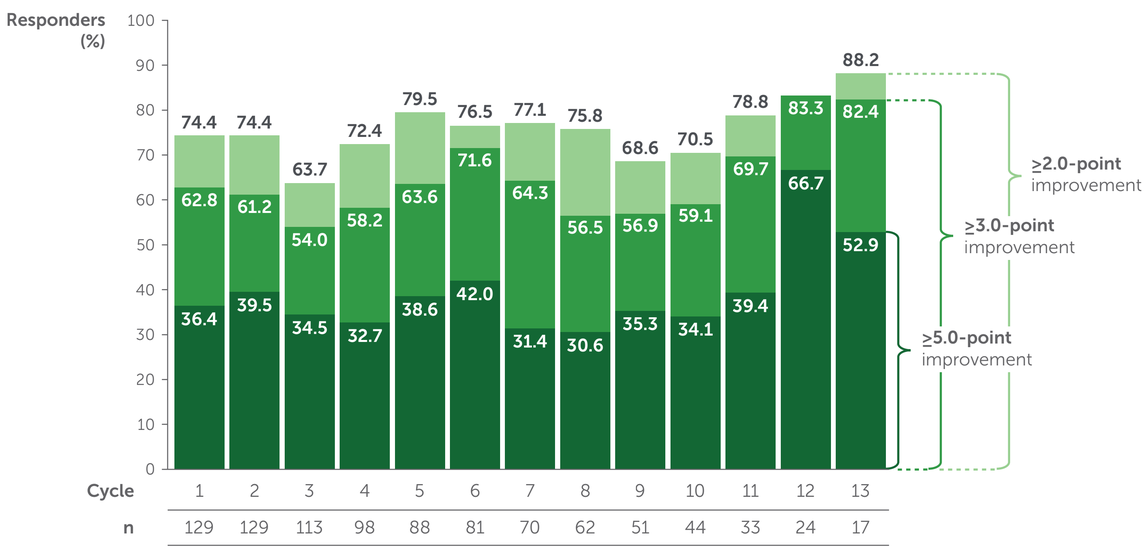


Table 1 Baseline demographics and disease characteristics were indicative of a broad population of patients with gMG

		RLZ total (N=129)
	Age, years, mean (SD)	**50.9** (16.3)
	Sex, female, n (%)	**77** (59.7)
	Thymectomy, yes, n (%)	**55** (42.6)
	Anti-AChR Ab+, n (%)	**117** (90.7)
	Anti-MuSK Ab+, n (%)	**12** (9.3)
	MG-ADL score at baseline, mean (SD)	**8.7** (3.4)
MG-ADL score, n (%)	<5	**13** (10.1)
	≥5	**116** (89.9)
	QMG score at baseline, mean (SD)	**16.0** (3.8)
MGFA Disease Class, n (%)	II	**52** (40.3)
	III	**72** (55.8)
	IV	**5** (3.9)
	Duration of disease from diagnosis, years, mean (SD)	**8.1** (8.5)

Figure 2 Rozanolixizumab demonstrated efficacy across 13 cycles using the prespecified and more stringent QMG responder thresholds

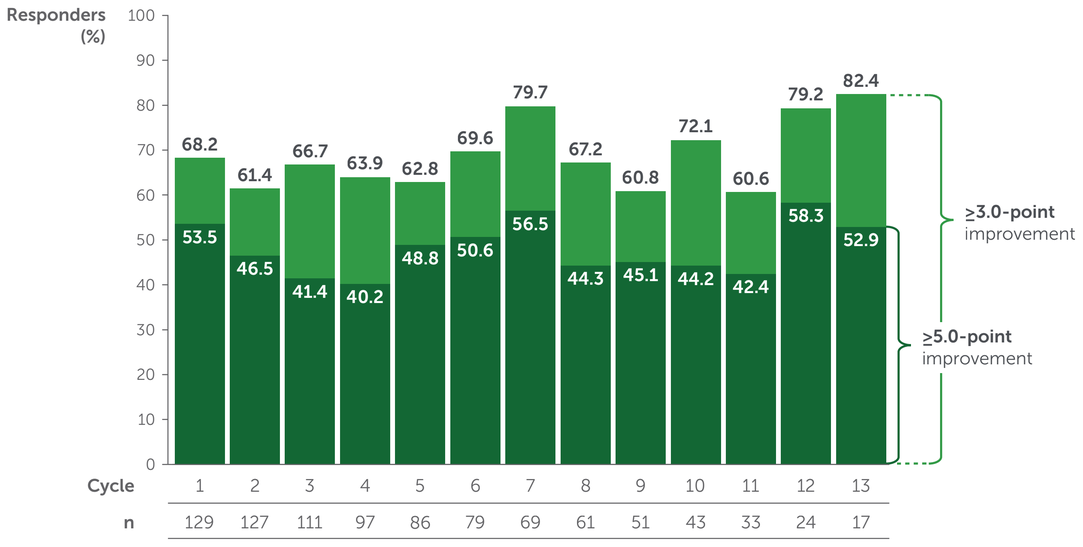
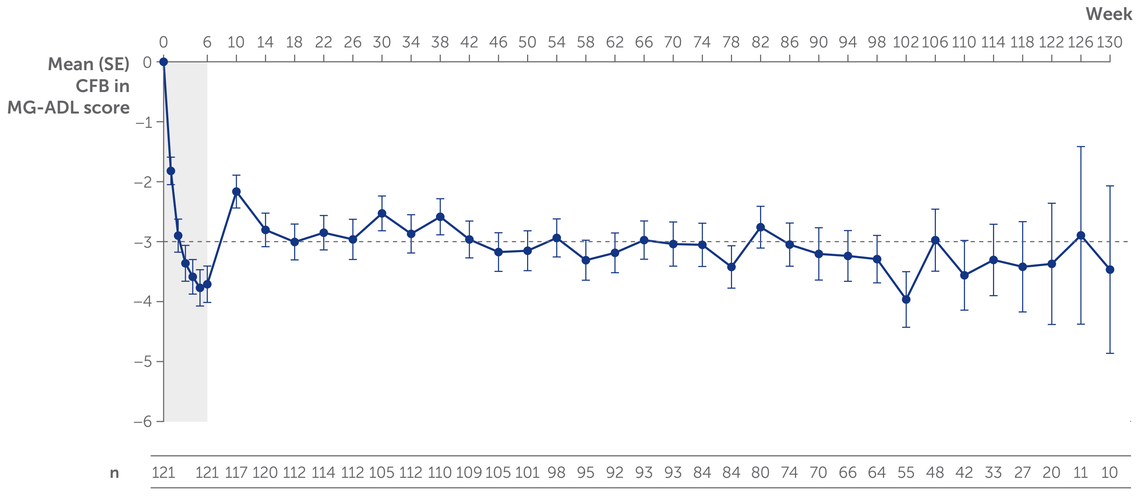


Figure 3 Over 130 weeks of repeated rozanolixizumab treatment cycles, a mean improvement in MG-ADL score of approximately 3.0 points from baseline was maintained



Includes patients in MycarinG and MG0007 who received ≥2 consecutive symptom-driven rozanolixizumab treatment cycles. Data at Weeks 0–6 represent observed MG-ADL scores in the Cycle 1 treatment period. After this, patients followed their own cadence of rozanolixizumab treatment cycles, with average monthly (28-day) MG-ADL scores calculated for each patient. The group-level average was calculated using data from all patients with at least one MG-ADL measurement at that time period, whether on treatment or not. If patients had multiple MG-ADL measurements during the period, their average MG-ADL score over the measurements was used.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; CFB, change from baseline; COVID-19, coronavirus disease 2019; FcRn, neonatal fragment crystallizable receptor; gMG, generalized myasthenia gravis; IgG4, immunoglobulin G4; 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; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; SD, standard deviation; SE, standard error; TEAE, treatment-emergent adverse event.

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References: 1. RYSTIGGO® US PI. https://www.ucb-usa.com/RYSTIGGO-prescribing-information.pdf. Accessed September 2025. 2. Bril V, et al. Lancet Neurol. 2023;22(5):383–394.

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