Repeated cycles of rozanolixizumab treatment in patients with anti-muscle-specific tyrosine kinase antibody-positive generalized myasthenia gravis

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MGFA Scientific Session at AANEM 2025, San Francisco, CA, USA; October 29, 2025

Introduction

- Anti-MuSK Ab+ MG is a rare (5–8% of patients) and frequently severe subtype of MG[1]
- Rozanolixizumab is a humanized IgG4 mAb FcRn blocker approved for the treatment of adults with anti-AChR or anti-MuSK Ab+ gMG[2,3]
- In the randomized, double-blind, Phase 3 MycarinG study (MG0003/NCT03971422), six once-weekly rozanolixizumab (7 mg/kg or 10 mg/kg) infusions demonstrated clinical efficacy versus placebo in adults with anti-AChR or anti-MuSK Ab+ gMG[2]
  - Following MycarinG, patients could enroll in the OLE studies MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly[4]
- Here, we evaluate the efficacy and safety of repeated rozanolixizumab cycles in patients with anti-MuSK Ab+ gMG

Methods

- MycarinG enrolled patients aged ≥18 years with MGFA Disease Class II–IVa gMG, MG-ADL score ≥3 (for non-ocular symptoms) and QMG score ≥11
- In MG0004, patients received chronic, once-weekly rozanolixizumab for ≤52 weeks
- In MG0007, after an initial 6-week cycle of rozanolixizumab, subsequent cycles were based on symptom worsening (investigator's discretion)
- Final rozanolixizumab data were pooled across:
  - MycarinG, MG0004 (first 6 weeks) and MG0007 for patients with ≥2 symptom-driven cycles (efficacy)
  - MycarinG and MG0007 for patients with ≥1 treatment cycle with a subsequent ≤8-week follow-up period (safety; incidence of TEAEs)
- Efficacy outcomes assessed included CFB in MG-ADL, MGC and QMG scores and MG-ADL, MGC and QMG responder rates (Day 43 in each cycle)

Results

- Of 129 patients who received ≥2 symptom-driven rozanolixizumab cycles, 12 had anti-MuSK Ab+ gMG
- Across Cycles 1–9, mean (SD) CFB in MG-ADL score for patients with anti-MuSK Ab+ gMG ranged from –3.0 (3.6) to –7.0 (3.5) (RLZ total; **Figure 1a**)
- In patients with anti-MuSK Ab+ gMG, mean CFB in MGC and QMG scores ranged from –5.8 (7.4) to –13.9 (6.6) and –5.2 (1.9) to –10.6 (6.0), respectively (RLZ total; **Figure 1b** and **Figure 1c**)
- High MG-ADL, MGC and QMG responder rates were observed across Cycles 1–9 in patients with anti-MuSK Ab+ gMG (**Figure 2**), and were comparable to those in the overall population (RLZ total)
- The estimated median (Q1, Q3) treatment-free interval to the first symptom-driven cycle was 75 (36, 209) days in patients with anti-MuSK Ab+ gMG
- Of 188 patients included in the safety pool, 18 had anti-MuSK Ab+ gMG
  - Most TEAEs were mild or moderate (**Table 1**)

Summary and conclusions

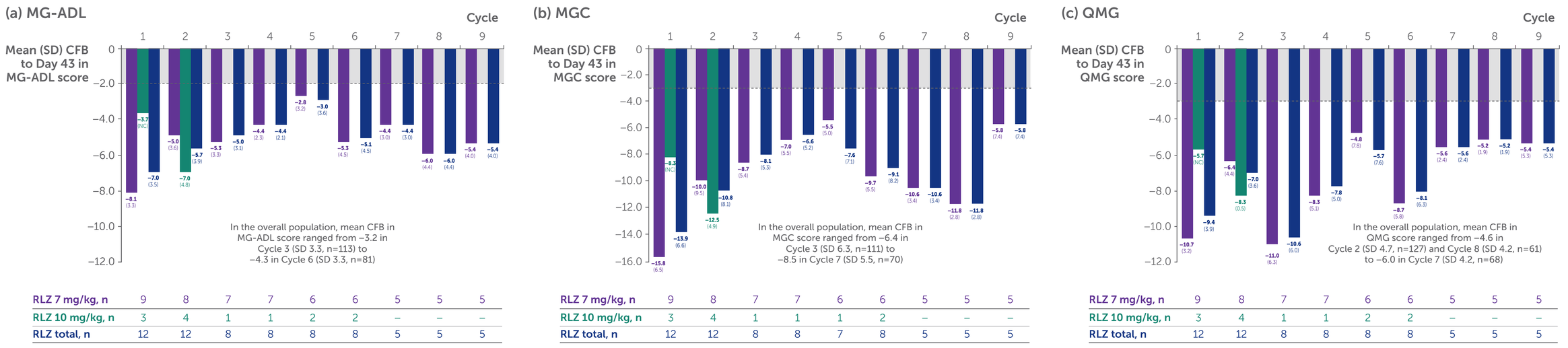
Data were pooled across MycarinG, MG0004 and MG0007 to assess the efficacy and safety of repeated rozanolixizumab cycles in patients with anti-MuSK Ab+ gMG, a frequently severe subtype of MG that can be challenging to treat[1]

Consistent with findings in the overall population, treatment with rozanolixizumab demonstrated efficacy in patients with anti-MuSK Ab+ gMG, which was maintained over repeated treatment cycles

Rozanolixizumab was well tolerated and had an acceptable safety profile over repeated treatment cycles in this subgroup of patients

These data support the long-term use of rozanolixizumab as a potential treatment option for patients with gMG who have anti-MuSK antibodies

Figure 1 Clinically meaningful improvement* from baseline to Day 43 in (a) MG-ADL, (b) MGC, and (c) QMG scores was observed for patients with anti-MuSK Ab+ gMG in Cycle 1 and repeated treatment cycles



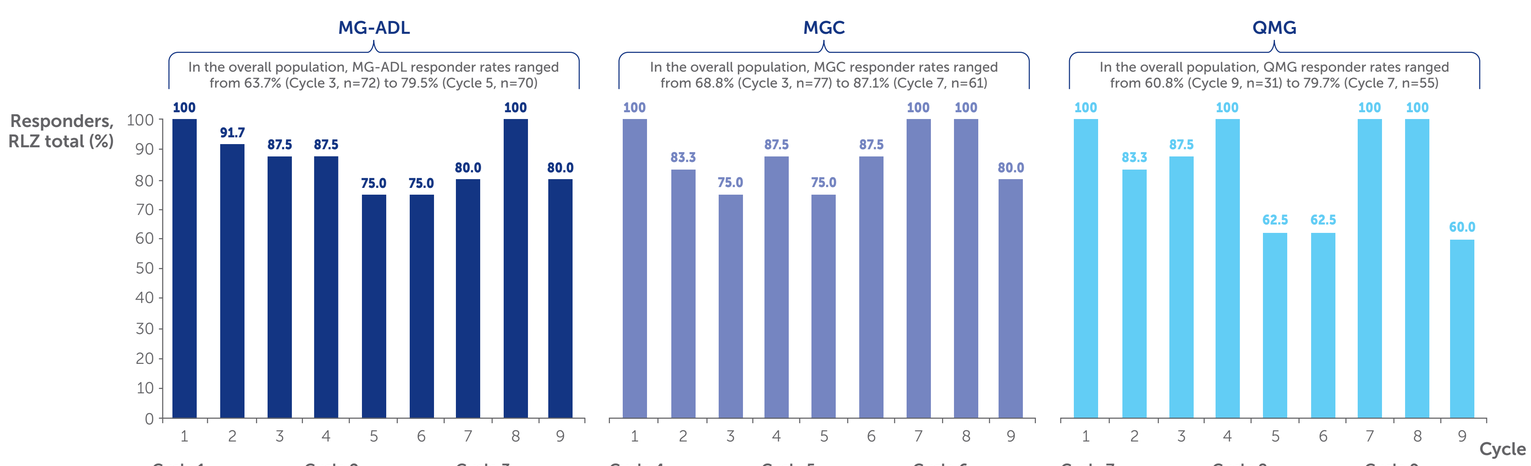
	(a) MG-ADL								
	Cycle 1	Cycle 2	Cycle 3	Cycle 4	Cycle 5	Cycle 6	Cycle 7	Cycle 8	Cycle 9
RLZ 7 mg/kg, n	9	8	7	7	6	6	5	5	5
RLZ 10 mg/kg, n	3	4	1	1	2	2	–	–	–
RLZ total, n	12	12	8	8	8	8	5	5	5

	(b) MGC								
	Cycle 1	Cycle 2	Cycle 3	Cycle 4	Cycle 5	Cycle 6	Cycle 7	Cycle 8	Cycle 9
RLZ 7 mg/kg, n	9	8	7	7	6	6	5	5	5
RLZ 10 mg/kg, n	3	4	1	1	1	2	–	–	–
RLZ total, n	12	12	8	8	7	8	5	5	5

	(c) QMG								
	Cycle 1	Cycle 2	Cycle 3	Cycle 4	Cycle 5	Cycle 6	Cycle 7	Cycle 8	Cycle 9
RLZ 7 mg/kg, n	9	8	7	7	6	6	5	5	5
RLZ 10 mg/kg, n	3	4	1	1	2	2	–	–	–
RLZ total, n	12	12	8	8	8	8	5	5	5

Efficacy pool. Observed data. The MG-ADL, MGC and QMG scores range from 0–24, 0–50 and 0–39, respectively, with higher scores indicating more severe disease. Mean (SD) values were not calculated for n≤2.
*The reference values for clinically meaningful change were a 2.0-point improvement for MG-ADL score and a 3.0-point improvement for MGC and QMG scores.[5]

Figure 2 High responder rates of at least 60% were consistently observed across repeated treatment cycles



Responders by dose group, n/N (%)	Cycle 1		Cycle 2		Cycle 3		Cycle 4		Cycle 5		Cycle 6		Cycle 7		Cycle 8		Cycle 9	
	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg	RLZ 7 mg/kg	RLZ 10 mg/kg
MG-ADL	**9/9** (100)	**3/3** (100)	**7/8** (87.5)	**4/4** (100)	**6/7** (85.7)	**1/1** (100)	**6/7** (85.7)	**1/1** (100)	**5/6** (83.3)	**1/2** (50.0)	**5/6** (83.3)	**1/2** (50.0)	**4/5** (80.0)	**0**	**5/5** (100)	**0**	**4/5** (80.0)	**0**
MGC	**9/9** (100)	**3/3** (100)	**6/8** (75.0)	**4/4** (100)	**5/7** (71.4)	**1/1** (100)	**6/7** (85.7)	**1/1** (100)	**5/6** (83.3)	**1/2** (50.0)	**6/6** (100)	**1/2** (50.0)	**5/5** (100)	**0**	**5/5** (100)	**0**	**4/5** (80.0)	**0**
QMG	**9/9** (100)	**3/3** (100)	**6/8** (75.0)	**4/4** (100)	**6/7** (85.7)	**1/1** (100)	**7/7** (100)	**1/1** (100)	**4/6** (66.7)	**1/2** (50.0)	**4/6** (66.7)	**1/2** (50.0)	**5/5** (100)	**0**	**5/5** (100)	**0**	**3/5** (60.0)	**0**

Efficacy pool. MG-ADL, MGC and QMG response were defined as a ≥2.0-point, ≥3.0-point and ≥3.0-point improvement from baseline without rescue therapy, respectively.

Table 1 Rozanolixizumab was generally well tolerated in patients with anti-MuSK Ab+ gMG; most TEAEs were mild or moderate

	All cycles		
	RLZ 7 mg/kg (N=11)* n (%)	**RLZ 10 mg/kg** (N=13)* n (%)	**RLZ total** (N=18) n (%)
Any TEAE	**9** (81.8)	**9** (69.2)	**14** (77.8)
Serious TEAEs	**1** (9.1)	**2** (15.4)	**3** (16.7)
Permanent discontinuation from study due to TEAEs	**0**	**3** (23.1)	**3** (16.7)
Treatment-related TEAEs	**6** (54.5)	**6** (46.2)	**11** (61.1)
Severe TEAEs	**1** (9.1)	**2** (15.4)	**3** (16.7)
TEAEs leading to death	**0**	**0**	**0**

Safety pool. 'n' is the number of patients reporting at least one TEAE within the category. *Patients who switched rozanolixizumab dose between cycles were allocated to both treatment groups but were only counted once in RLZ total.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; CFB, change from baseline; 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; MGC, Myasthenia Gravis Composite; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; NC, not calculated; OLE, open-label extension; Q[x], quartile [x]; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; SD, standard deviation; TEAE, treatment-emergent adverse event.

Acknowledgments: 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, Slough, UK, 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 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. Artur Drużdż has nothing to disclose. Dale J. Lange has received research support from Novartis Pharmaceuticals. He has received funding for advisory board participation from AbbVie, Inc., Alexion Pharmaceuticals and Calico Life Sciences. He has received funding for consultations from Summus Global. Renato Mantegazza has received funding for travel and meeting attendance or advisory board participation from Alexion Pharmaceuticals, argenx, BioMarin, Catalyst, Regeneron Pharmaceuticals, Sanofi and UCB. Hiroyuki Naito has nothing to disclose. Robert M. Pascuzzi is Professor Emeritus of Neurology at Indiana University and receives compensation for his professional work from Indiana University Health. He has no financial relationship with any pharmaceutical company and receives no compensation from any pharmaceutical company (present or past). Robert M. Pascuzzi speaks at educational seminars on a broad variety of general neurology topics for primary care physicians through the organisation Medical Education Resources (an educational organisation with no links or ties to any pharmaceutical or healthcare business company). Therefore, Robert M. Pascuzzi has no conflicts of interest related to this research, manuscript, presentation, or publication. 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. Jiann-Horng Yeh serves as a Principal Investigator for clinical trials sponsored by Alexion/AstraZeneca Rare Disease, argenx, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Merck, Novartis, Regeneron Pharmaceuticals, UCB and Viela Bio (now Amgen). He has served as a Consultant on advisory boards for Alexion/AstraZeneca Rare Disease, CSL, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine) and Merck. Fiona Grimson, 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).

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