

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

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Introduction

- Anti-MuSK Ab+ MG is a rare (5–8% of patients) and frequently severe subtype of MG^{1,2}
- Rozanolixizumab, a humanized IgG mAb that targets and blocks FcRn with high affinity, is approved for the treatment of adults with anti-AChR Ab+ or anti-MuSK Ab+ gMG^{3,4}
- In the Phase 3 MycarinG (NCT03971422) study, one 6-week cycle of rozanolixizumab (7 mg/kg or 10 mg/kg) led to clinically meaningful improvements in MG-specific outcomes in patients with gMG⁵
- Following MycarinG, patients could enroll in the OLE studies MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly²
- **This analysis evaluated the efficacy and safety of repeated rozanolixizumab cycles in patients with anti-MuSK Ab+ gMG**

Methods

- In MycarinG, patients were aged ≥18 years with MGFA Clinical Class II–IVa anti-AChR Ab+ or anti-MuSK Ab+ gMG, with an MG-ADL score ≥3 (for non-ocular symptoms) and a QMG score ≥11
- In MG0004, patients received once-weekly rozanolixizumab 7 mg/kg or 10 mg/kg for ≤52 weeks
- MG0007 comprised an initial 6-week rozanolixizumab 7 mg/kg or 10 mg/kg cycle, with further 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 rozanolixizumab treatment cycles
- Time to event data for the treatment-free interval were pooled across patients who had received rozanolixizumab, and had either initiated or were awaiting their first symptom-driven rozanolixizumab treatment cycle
- For MG-ADL and QMG, across Cycles 1–9, the following outcomes were assessed *post hoc* (for each cycle and as weighted means across 9 cycles):
 - Mean total scores at baseline and Day 43
 - CFB at Day 43
 - Responder rates at Day 43

- Final safety data were pooled across MycarinG and MG0007 for patients with ≥1 rozanolixizumab treatment cycle, with a treatment-free follow-up period; the incidence of TEAEs was assessed

Results

- Of 129 patients who received ≥2 symptom-driven rozanolixizumab cycles, 12 (9.3%) had anti-MuSK Ab+ gMG (**Table 1**)
- Consistent improvements in mean MG-ADL scores from baseline to Day 43 were observed in patients with anti-MuSK Ab+ gMG:
 - Across Cycles 1–9, mean CFB in MG-ADL scores ranged from –3.0 (n=8; Cycle 5) to –7.0 (n=12; Cycle 1) (**Figure 1a**)
 - Across Cycles 1–9, the overall weighted-mean MG-ADL score was 10.37 at baseline and 5.14 at Day 43, corresponding to a weighted-mean reduction of 5.23 points (**Figure 1a**)
- Similar improvements were observed in mean QMG scores:
 - Across Cycles 1–9, mean CFB in QMG scores ranged from –5.2 (n=5; Cycle 8) to –10.6 (n=8; Cycle 3) (**Figure 1b**)
 - Across Cycles 1–9, the overall weighted-mean QMG score was 16.76 at baseline and 9.23 at Day 43, corresponding to a weighted-mean reduction of 7.54 points (**Figure 1b**)
- Across Cycles 1–9, responder rates in patients with anti-MuSK Ab+ gMG ranged from 75.0% to 100% for MG-ADL (weighted mean: 87.3%) and 60.0% to 100% (weighted mean: 84.5%) for QMG (**Figure 2**)
- The median (Q1–Q3) treatment-free interval to the first symptom-driven cycle, estimated using Kaplan–Meier analysis, was 10.7 (5.1–29.9) weeks in patients with anti-MuSK Ab+ gMG
- Of 188 patients included in the safety pool, 18 (9.6%) had anti-MuSK Ab+ gMG
 - TEAEs were reported in 14/18 (77.8%) patients, of whom 11/14 (78.6%) reported TEAEs of mild or moderate intensity only (**Table 2**)

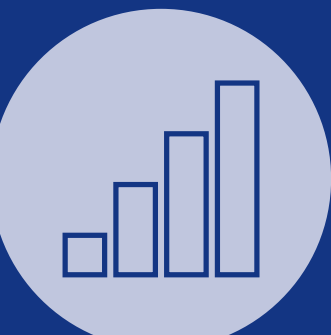
Summary and conclusions



This pooled analysis of data from the MycarinG study and its OLEs assessed the efficacy and safety of repeated rozanolixizumab cycles in patients with anti-MuSK Ab+ gMG, a rare and frequently severe subtype of MG^{1,2}



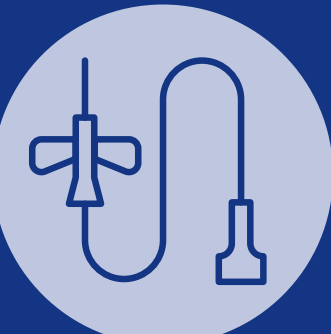
Patients with anti-MuSK Ab+ gMG who received rozanolixizumab treatment consistently had improvements from baseline in MG-ADL and QMG scores across cycles



Across Cycles 1–9, high average responder rates of 87.3% for MG-ADL and 84.5% for QMG were achieved in patients with anti-MuSK Ab+ gMG

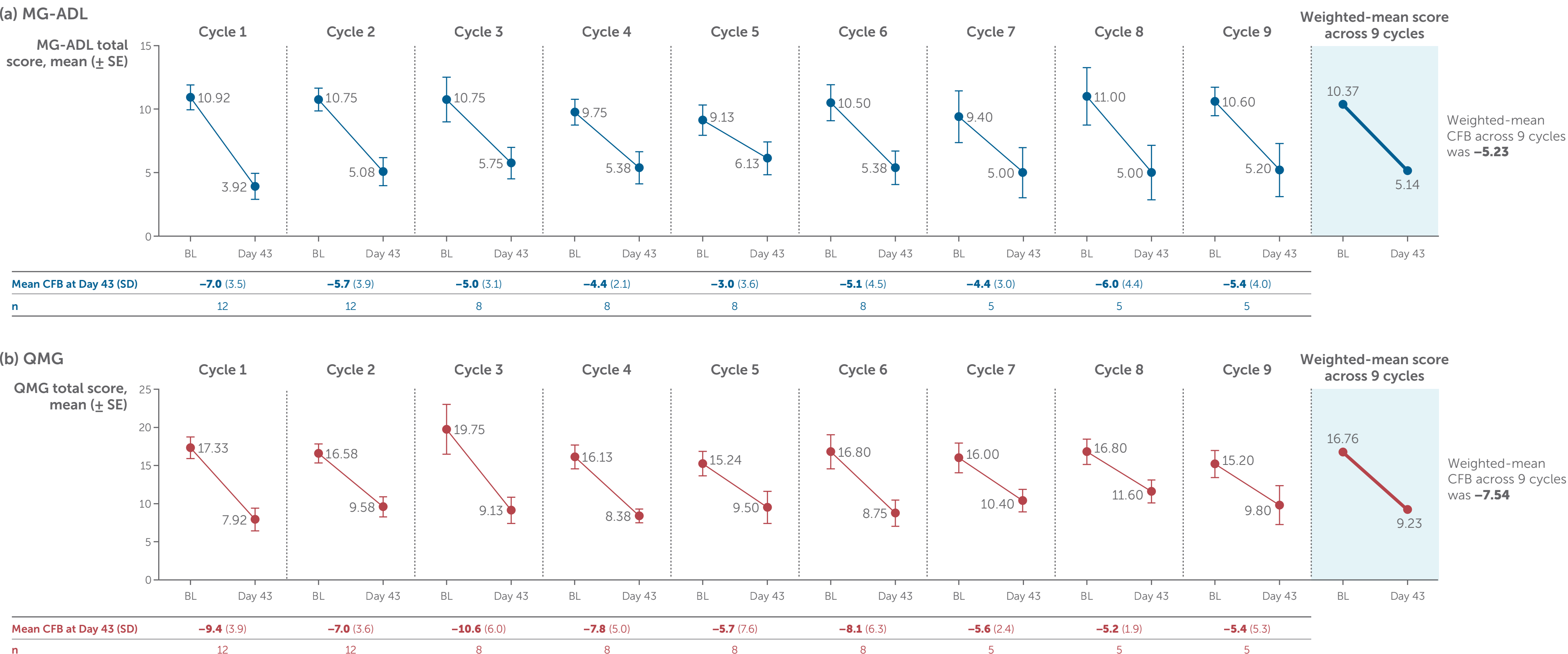


Rozanolixizumab was generally well tolerated, with an acceptable safety profile, in this subgroup of patients



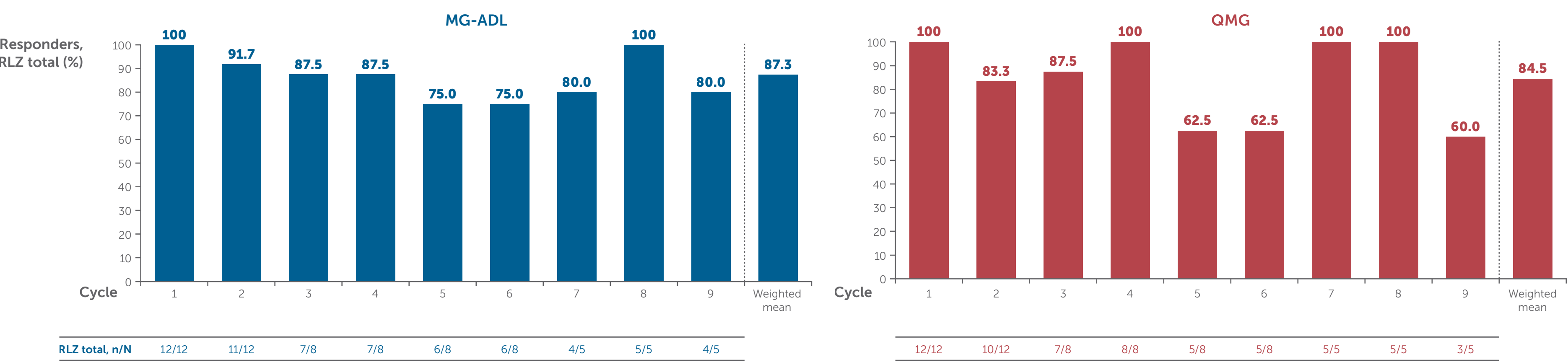
These data support the long-term use of rozanolixizumab as a treatment option for patients with anti-MuSK Ab+ gMG

Figure 1 Reductions in mean MG-ADL and QMG scores were observed in patients with anti-MuSK Ab+ gMG and maintained over repeated treatment cycles



Efficacy data presented for patients with ≥2 symptom-driven cycles. ‘n’ is the number of participants who have assessments recorded at both baseline and Day 43 of the cycle. Due to low patient numbers, results for Cycles 10–13 are not shown (Cycle 10, n=4; Cycles 11 and 12, n=3; Cycle 13, n=2). The small numerical discrepancy between the QMG weighted means is due to the rounding of data.

Figure 2 High average responder rates (across Cycles 1–9) of ≥84.5% for MG-ADL and QMG were observed in patients with anti-MuSK Ab+ gMG



Efficacy data presented for patients with ≥2 symptom-driven cycles. Response at Day 43 of each cycle was defined as an improvement from baseline of ≥2.0 points for MG-ADL and ≥3.0 points for QMG, both without rescue therapy.

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; BL, baseline; CFB, change from baseline; FcRn, neonatal fragment; crystallizable receptor; gMG, generalized myasthenia gravis; IgG4, immunoglobulin G subunit 4; 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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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 organization Medical Education Resources (an educational organization 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. 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 Viala Bio (now Arigen); he has received speaker honoraria from Alexion Pharmaceuticals, argenx, the Japan Blood Products Organization and UCB. Fiammetta Vanoli has received funding for travel, congress attendance, speaking and advisory boards from Alexion Pharmaceuticals, argenx, Biogen 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, Cartesio Therapeutics, COUR Pharmaceuticals, Dianthus Therapeutics, ImmunAbs, Immunovant, Johnson & Johnson, NMD

Table 1 Baseline demographics and disease characteristics were broadly similar between the overall population and the anti-MuSK Ab+ subgroup

	RLZ total (N=129)	RLZ anti-MuSK Ab+ subgroup (N=12)
Age, years, mean (SD)	50.9 (16.3)	46.8 (10.8)
Sex, female, n (%)	77 (59.7)	9 (75.0)
Age at initial gMG diagnosis, years, mean (SD)	43.1 (17.8)	37.5 (10.1)
Duration of disease from diagnosis, years, mean (SD)	8.1 (8.5)	9.8 (8.0)
MGFA Clinical Class, n (%)	II	52 (40.3)
	III	72 (55.8)
	IV	5 (3.9)
		1 (8.3)
MG-ADL score, mean (SD)	8.7 (3.4)	9.8 (3.5)
QMG score, mean (SD)	16.0 (3.8)	17.3 (4.5)
Thymectomy at baseline, n (%)	55 (42.6)	2 (16.7)
Corticosteroids for systemic use	80 (62.0)	8 (66.7)
Baseline gMG medication, n (%)	Immunosuppressants	66 (51.2)
	Parasympathomimetics	113 (87.6)

Table 2 Rozanolixizumab was generally well tolerated in patients with anti-MuSK Ab+ gMG

	RLZ overall population (N=188), n (%)	RLZ anti-MuSK Ab+ subgroup (N=18), n (%)
Any TEAE	175 (93.1)	14 (77.8)
Serious TEAEs	55 (29.3)	3 (16.7)
Patient discontinuation due to TEAEs	33 (17.6)	3 (16.7)
Treatment-related TEAEs	120 (63.8)	11 (61.1)
Severe TEAEs	62 (33.0)	3 (16.7)
TEAEs leading to death [†]	4 (2.1)	0

All cycles (encompassing cycles beyond Cycle 9). Safety data presented from data pooled from MycarinG and MG0007 for patients with ≥1 rozanolixizumab treatment cycle with a treatment-free follow-up period. ‘n’ is the number of patients reporting at least one TEAE within the category. [†]All deaths were considered not related to rozanolixizumab by the investigator.

Pharma, Regeneron Pharmaceuticals, UCB and Vor, and has served as a speaker for Alexion/AstraZeneca Rare Disease, Amgen, argenx and CSL Behring. He has performed consulting work for Alexion/AstraZeneca Rare Disease, argenx, Dianthus Therapeutics, NMD Pharma, Regeneron and Vor. 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 Viala 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. Joel Baldwin is an employee of Cogent Skills Limited working for UCB. Thaïs Tarancón is an employee and shareholder of UCB. Tuan Vu was 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 received research support from Akcea, Alexion Pharmaceuticals, argenx, CSL, Grifols, Immunovant, Ionis, Momenta (now Johnson & Johnson), Octapharma, Takeda Pharmaceuticals, UCB and Viala Bio (now Amgen).

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