# Long-term safety of cyclic rozanolixizumab treatment in patients with generalized myasthenia gravis: A final analysis of Phase 3 studies

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

[1]MDA ALS & Neuromuscular Center, Department of Neurology, University of California, Irvine, Orange, CA, USA; [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]Precision Neurology of Neuromuscular Diseases, Department of Neurology, University of Lübeck, Lübeck, Germany; [4]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [5]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [6]Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark; [7]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [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[1,2]
- This analysis evaluated the incidence of predefined TEAEs in patients who participated in the Phase 3 double-blind MycarinG study (MG0003/NCT03971422)[1] and its open label extension MG0007 (NCT04650854)

## Methods

**MG0003[1]**
- Double-blind
- Placebo-controlled
- One 6-week cycle
- N=200, randomized 1:1:1 to SC placebo, rozanolixizumab 7 mg/kg or 10 mg/kg

Inclusion criteria:[1]
- Aged ≥18 years
- Anti-AChR Ab+ or anti-MuSK Ab+ gMG
- MGFA Disease Class II–IVa
- MG-ADL score ≥3 (for non-ocular symptoms)
- QMG score ≥11

**MG0007**
- Open-label
- Symptom-driven rozanolixizumab cycles
- N=165

Pooled safety data for patients receiving ≥1 rozanolixizumab treatment cycle with a subsequent ≤8-week follow-up period

Predefined TEAEs based on the mechanism of action and structure of rozanolixizumab, earlier clinical trials and predefined MedDRA search criteria included:
- Headaches
- GI disorders
- Infections
- Hypersensitivity reactions
- Anaphylactic reactions

In MG0007, after an initial cycle, subsequent cycles were based on symptom worsening at the investigator's discretion.

## Results

- In total, 188 patients received ≥1 rozanolixizumab treatment cycle
  - Overall, the mean time in studies was 602.3 (SD 331.0) days
  - Of these 188 patients, 130 (69.1%) had >1 year of participation equating to 284.91 PY; they initiated a mean of 4.1 (SD 1.7) cycles (median 4.0 [range 1–7] cycles) in the first year
- Baseline characteristics were similar between the two dose groups
- Across all cycles, TEAEs occurred in 93.1% of patients; most were non-serious and mild or moderate in intensity (**Table 1**)
  - Across 13 cycles, the incidence of TEAEs in each cycle ranged from 42.3% (n=11/26 [Cycle 12]) to 79.3% (n=149/188 [Cycle 1])
- The frequency of predefined TEAEs is shown in **Table 2**
  - There were no anaphylactic reactions
- Excluding headaches and GI disorders, which were more frequent in Cycle 1, the incidence of predefined TEAEs was similar across cycles (**Figure 1**)
- One event of aseptic meningitis led to study discontinuation (Cycle 1; rozanolixizumab 10 mg/kg)
- No clinically meaningful changes in lipid or albumin levels were identified
- Six deaths occurred, all during MG0007; none were considered related to rozanolixizumab by investigators
  - Four patients experienced fatal TEAEs (COVID-19 infection, COVID-19 pneumonia, cardiac failure and pneumonia)
  - Two deaths were attributed to non-TEAEs (myocardial infarction [occurred >6 months after the last dose] and metastatic SCLC [death occurred 804 days after the last dose])

## Summary and conclusions

- Pooled data are reported across MycarinG and MG0007 to evaluate the long-term safety of repeated rozanolixizumab treatment cycles in adults with gMG
- In general, compared with Cycle 1, the incidence of predefined TEAEs did not increase with repeated rozanolixizumab treatment cycles
- There were no clinically meaningful changes in albumin or lipid levels
- These data indicate that long-term rozanolixizumab treatment is well tolerated with an acceptable safety profile that is consistent across repeated treatment cycles

**Table 1** Across all cycles, rozanolixizumab was generally well tolerated and demonstrated an acceptable safety profile

| | All cyles | | |
|---|---|---|---|
| | 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) |
| 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) |

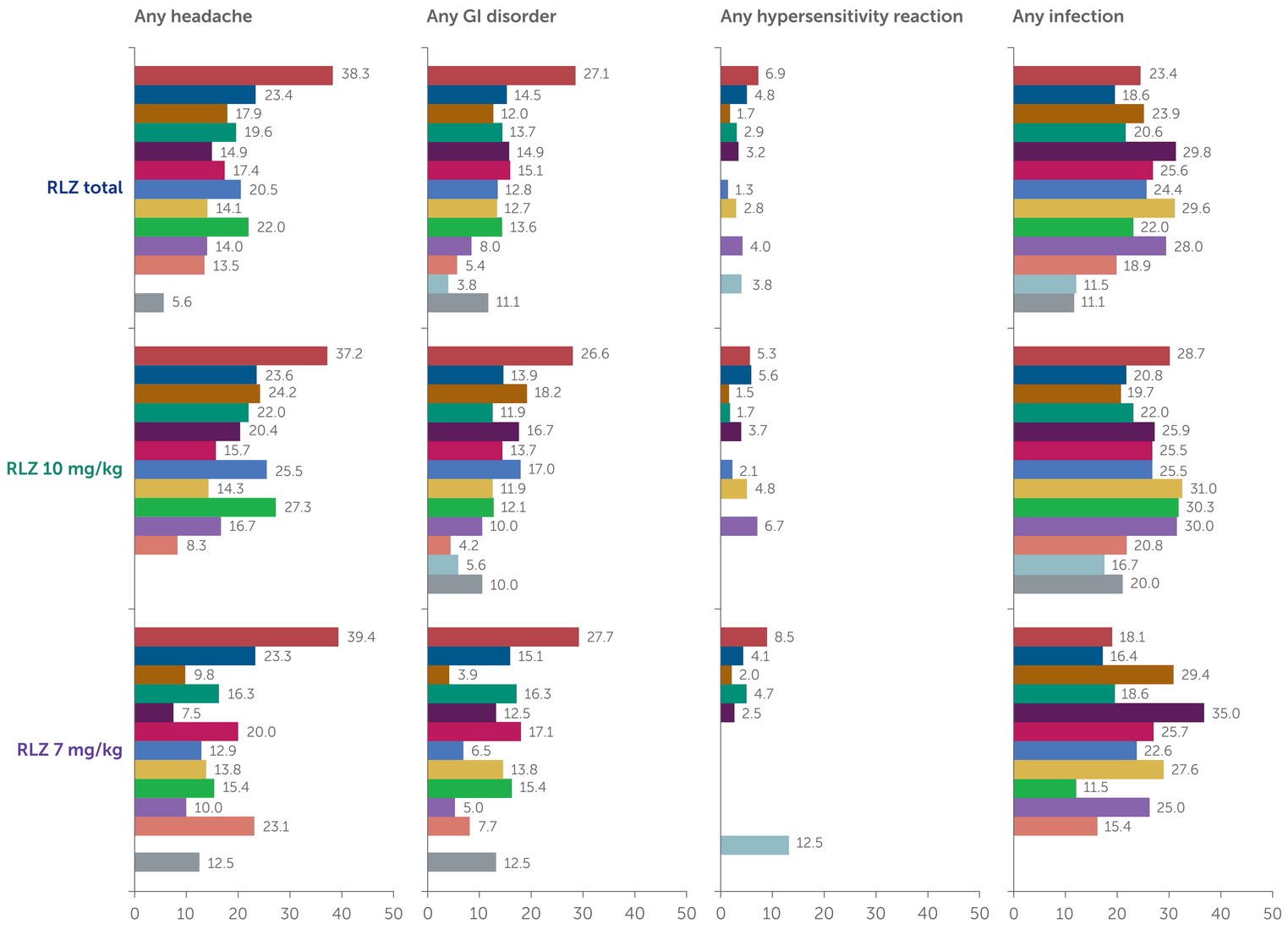
n is the number of patients reporting at least one TEAE within the category.

**Table 2** Across all cycles, most predefined TEAEs were mild or moderate in severity

| | All cycles | | |
|---|---|---|---|
| | RLZ 7 mg/kg (N=135) % (n) | RLZ 10 mg/kg (N=133) % (n) | RLZ total (N=188) % (n) |
| **Any headache*** | **43.7** (59) | **47.4** (63) | **51.6** (97) |
| Serious headache | 0 | 0.8 (1) | 0.5 (1) |
| Severe headache | 0.7 (1) | 5.3 (7) | 4.3 (8) |
| **Any GI disorder†** | **31.9** (43) | **42.1** (56) | **43.6** (82) |
| Serious GI disorder | 1.5 (2) | 0 | 1.1 (2) |
| Severe GI disorder | 0.7 (1) | 2.3 (3) | 2.1 (4) |
| **Any anaphylactic reaction** | **0** | **0** | **0** |
| **Any hypersensitivity reaction‡** | **11.9** (16) | **10.5** (14) | **15.4** (29) |
| Serious hypersensitivity reaction | 0 | 0 | 0 |
| Severe hypersensitivity reaction | 0.7 (1) | 0 | 0.5 (1) |
| **Any infection or infestation§** | **40.7** (55) | **55.6** (74) | **58.0** (109) |
| Serious infection or infestation | 3.0 (4) | 6.0 (8) | 6.4 (12) |
| Severe infection or infestation | 2.2 (3) | 6.0 (8) | 5.9 (11) |
| **Any opportunistic infection‖** | **2.2** (3) | **0.8** (1) | **2.1** (4) |
| Serious opportunistic infection | 0.7 (1) | 0 | 0.5 (1) |
| Severe opportunistic infection | 0.7 (1) | 0 | 0.5 (1) |

n is the number of patients reporting at least one TEAE within the category. *Included headache, migraine and migraine with aura. †The three most common GI disorders overall were diarrhea, nausea and abdominal pain. ‡Excluding injection site reactions, the three most common hypersensitivity reactions were rash, urticaria and eczema. §The three most common infections were COVID-19, upper respiratory tract infection and nasopharyngitis. ‖Included *Blastocystis* infection, ophthalmic herpes simplex, esophageal candidiasis and sinusitis aspergillus.

**Figure 1** In general, compared with Cycle 1, the incidence of headaches, GI disorders, hypersensitivity reactions and infections did not increase with repeated rozanolixizumab treatment cycles




| Cycle | 1 | 2 | 3 | 4 | 5 | 6 | 7 | 8 | 9 | 10 | 11 | 12 | 13 |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| RLZ total, n | 188 | 145 | 117 | 102 | 94 | 86 | 78 | 71 | 59 | 50 | 37 | 26 | 18 |
| RLZ 10 mg/kg, n | 94 | 72 | 66 | 59 | 54 | 51 | 47 | 42 | 33 | 30 | 24 | 18 | 10 |
| RLZ 7 mg/kg, n | 94 | 73 | 51 | 43 | 40 | 35 | 31 | 29 | 26 | 20 | 13 | 8 | 8 |

All hypersensitivity reactions were non-serious and most were mild or moderate. One event of severe rash was reported (Cycle 4; rozanolixizumab 7 mg/kg); the patient was subsequently diagnosed with subacute cutaneous lupus erythematosus.

**Abbreviations:** Ab+, antibody positive; AChR, acetylcholine receptor; FcRn, neonatal fragment crystallizable receptor; GI, gastrointestinal; gMG, generalized myasthenia gravis; IgG4, immunoglobulin G4; mAb, monoclonal antibody; MedDRA, Medical Dictionary for Regulatory Activities; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; PY, patient-years; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; SC, subcutaneous; SCLC, small cell lung cancer; SD, standard deviation; TEAE, treatment-emergent adverse event.

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