Minimal symptom expression in myasthenia gravis: A *post hoc* analysis of Phase 3 rozanolixizumab studies

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Introduction

- gMG is a chronic, autoimmune neuromuscular disease characterised by muscle weakness[1,2]
- Rozanolixizumab, a humanised IgG4 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 study (NCT03971422) and its OLE studies, MG-ADL responder rates (≥2-point improvement in MG-ADL score) of ≥63.7% were observed in patients with gMG across Cycles 1–13 of rozanolixizumab treatment (**Figure 1**)[5]
- MSE is defined as an MG-ADL score of 0 or 1, which is a higher treatment threshold than a ≥2 point improvement in MG-ADL, and a stringent measure of efficacy that is considered a treatment goal in gMG[6]
- **This *post hoc* analysis assessed achievement of MSE in patients receiving repeated treatment cycles of rozanolixizumab, based on their Cycle 1 response**

Methods

- In the MycarinG study, patients were aged ≥18 years with MGFA Disease 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
- After the double-blind study, patients could enrol in OLE studies MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly
  - 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 cycles
- The proportion of patients who achieved MSE (MG-ADL score: 0 or 1) at any time during treatment or observation in each symptom-driven cycle up to Cycle 13 was analysed *post hoc* based on MSE achievement in Cycle 1
- Final safety data were pooled across MycarinG and MG0007 for patients with ≥1 treatment cycle; incidence of TEAEs was assessed

Results

- Overall, 129 patients received ≥2 symptom-driven cycles of rozanolixizumab
- In Cycle 1, 27.9% (36/129) of patients achieved MSE (**Figure 2**)
- In every cycle (1–13), almost all patients who achieved MSE did so within the 6-week treatment period (**Figure 3**)
  - Rapid onset (1 week) was seen in some patients
- In total, 93.1% (175/188) of patients experienced a TEAE; most were mild or moderate in severity
  - The most common TEAEs were headache (50.0%; n=94), diarrhoea (33.5%; n=63), COVID-19 (21.8%; n=41) and pyrexia (20.7%; n=39)

Summary and conclusions

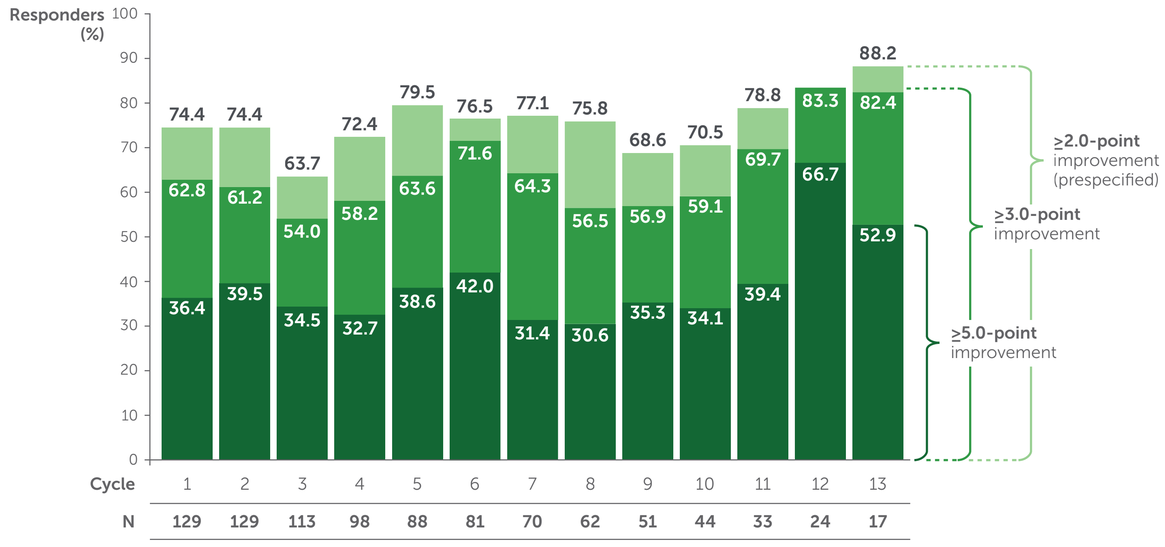
This pooled analysis of data from the MycarinG study and its OLEs assessed MSE achievement in patients receiving repeated treatment cycles of rozanolixizumab, based on their Cycle 1 MSE response

Among Cycle 1 MSE achievers, MSE achievement rates were ≥60.0% in each subsequent cycle (Cycles 2–13)

Among Cycle 1 MSE non-achievers, 19.4% achieved MSE in a subsequent cycle (Cycles 2–13)

In each cycle, almost all patients who achieved MSE did so within the 6-week treatment period. Some patients achieved MSE as early as one week after rozanolixizumab infusion

Figure 1 Rozanolixizumab demonstrated efficacy across 13 symptom-driven cycles using the prespecified and more stringent MG-ADL responder thresholds[5]



Efficacy pool, which included pooled data across patients with ≥2 symptom-driven cycles. N is the number of patients who completed the MG-ADL assessment at Day 43 of each cycle.

Figure 3 Most patients who achieved MSE did so within the 6-week treatment period (only data from Cycle 1 are presented)

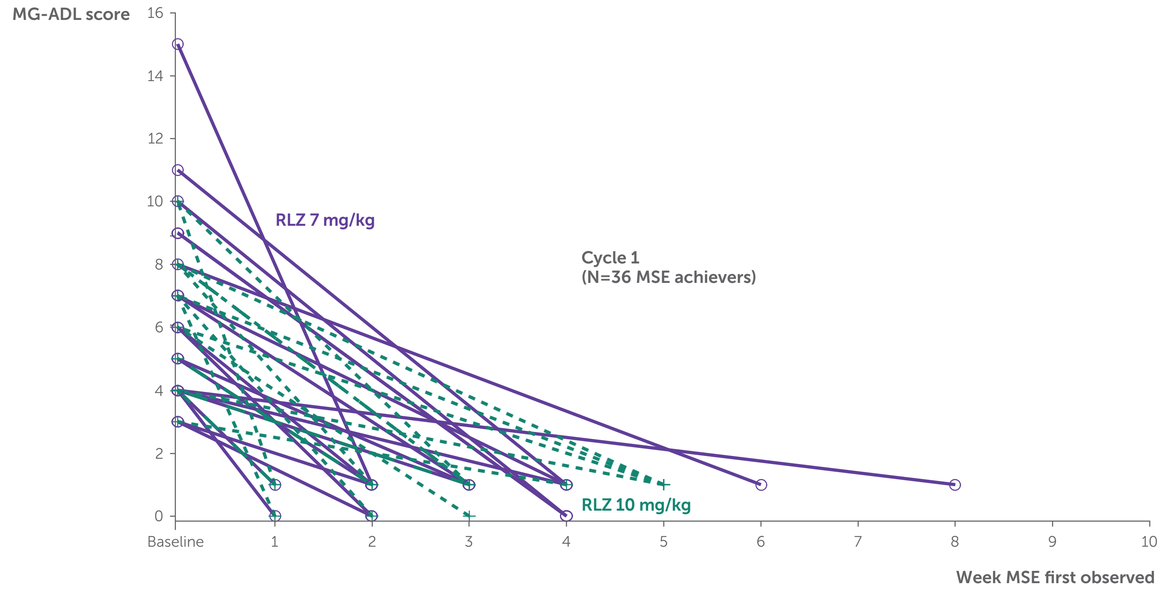
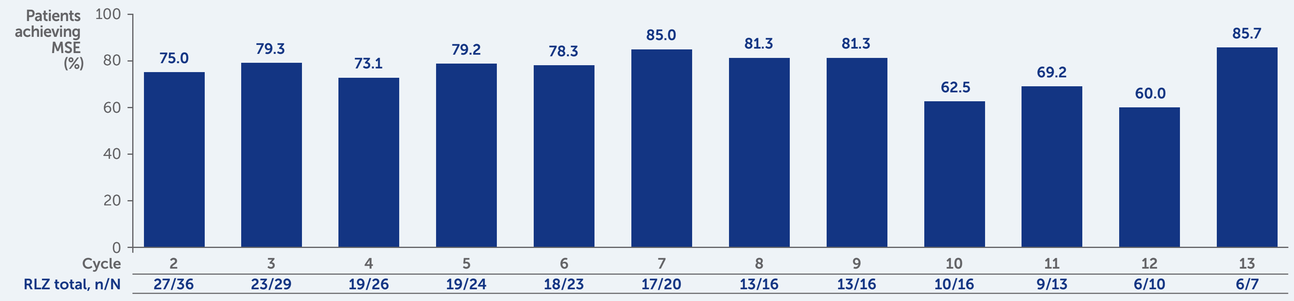


Figure 2 Subsequent MSE achievement rates were ≥60.0% in each cycle (Cycles 2–13), for patients who achieved MSE in Cycle 1 of rozanolixizumab treatment

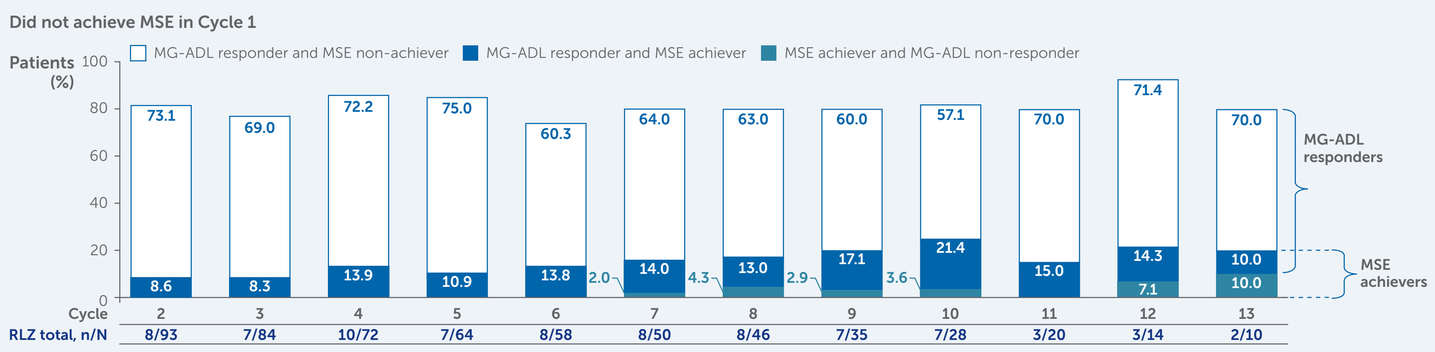
MSE in Cycle 1
RLZ 7 mg/kg: **25.7%** (18/70)
RLZ 10 mg/kg: **30.5%** (18/59)
RLZ total: **27.9%** (36/129)

No MSE in Cycle 1
RLZ 7 mg/kg: **74.3%** (52/70)
RLZ 10 mg/kg: **69.5%** (41/59)
RLZ total: **72.1%** (93/129)

Achieved MSE in Cycle 1



Did not achieve MSE in Cycle 1



- Among 93 (72.1%) Cycle 1 MSE non-achievers, MSE rates were 8.3–25.0% in subsequent cycles, and 57.1–75.0% of patients were MG-ADL responders (≥2 point improvement) without achieving MSE
  - In total, 19.4% of patients achieved MSE in ≥1 subsequent cycle (Cycles 2–13), having received a median of six additional cycles

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; FcRn, neonatal fragment crystallisable receptor; gMG, generalised myasthenia gravis; IgG4, immunoglobulin G4; mAb, monoclonal antibody; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MSE, minimal symptom expression; MuSK, muscle-specific tyrosine kinase; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; TEAE, treatment-emergent adverse event.

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