Self-administration of rozanolixizumab in patients with generalized myasthenia gravis: The MG0020 study

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Introduction

- gMG is a chronic autoimmune disorder requiring long-term treatment[1]
- Rozanolixizumab is a humanized IgG4 mAb FcRn blocker indicated for the treatment of adults with anti-AChR Ab+ or anti-MuSK Ab+ gMG[2]
- Rozanolixizumab is administered by HCPs as a once-weekly SC infusion in 6-week cycles using programmable syringe drivers[2]
- Broadening the administration options of rozanolixizumab would enable patients and caregivers to administer rozanolixizumab in a way that aligns with their individual needs and preferences
  - Manual push and syringe driver are two options that allow patients to self-administer treatment, with manual push being simpler and expected to reduce infusion times[3]
- Here, we assess the self-administration, efficacy, pharmacodynamics and safety of rozanolixizumab using manual push and syringe driver methods in patients with gMG

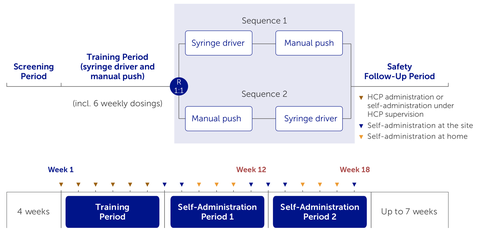
Methods

- MG0020 (NCT05681715) was a Phase 3, open-label, randomized, two-period, two-sequence crossover study (**Figure 1**)
- Patients were aged ≥18 years with a documented diagnosis of gMG, either rozanolixizumab-naïve or non-naïve, with a serum total IgG level of ≥5.5–16.0 g/L and body weight ≥35 kg at screening
- Patients received rozanolixizumab once-weekly during an 18-week Treatment Period comprising:
  - A 6-week Training Period in the two self-administration methods
  - Two 6-week Self-Administration Periods
- At Week 7, following the investigator's confirmation of eligibility to perform self-administration, patients were randomized 1:1 to self-administer rozanolixizumab via either:
  - The syringe driver method, crossing over to the manual push method at Week 13 (Sequence 1), or
  - The manual push method, crossing over to the syringe driver method at Week 13 (Sequence 2)
- Primary endpoint:
  - Successful self-administration of rozanolixizumab, evaluated by an HCP at Weeks 12 and 18, defined as choosing the correct infusion site, administering subcutaneously and delivering the intended dose
- Secondary endpoint:
  - Occurrence of TEAEs
- Additional endpoints:
  - Occurrence of TEAEs leading to permanent withdrawal of rozanolixizumab
  - Successful self-administration of rozanolixizumab at all home self-administration visits
  - Median CFB in total IgG serum concentration
  - Mean CFB in MG-ADL score

Results

- Overall, 62 patients entered MG0020 (safety set) and 55 were randomized (randomized safety set) to Sequence 1 (n=28) or Sequence 2 (n=27)
- Baseline characteristics were well balanced between the sequences (**Table 1**)
- All patients in both treatment sequences successfully self-administered 100% of their rozanolixizumab infusions with manual push and syringe driver at Weeks 12 and 18 (**Figure 2**)
  - Additionally, all patients successfully self-administered rozanolixizumab using either infusion method at all visits, including those at home (randomized safety set)
- In the safety set, mean (SD) duration of study medication exposure during the Treatment Period was 110.0 (28.0) days
- Overall, 75.8% (n=47/62) of patients experienced a TEAE (**Table 2**)
  - Most TEAEs occurred in the Training Period (first 6 weeks) and were of mild or moderate intensity
- Five TEAEs reported by four patients led to permanent withdrawal of rozanolixizumab
  - One event each of metastatic lung cancer, migraine and MG worsening in individual patients, and one event each of pyrexia and headache in the same patient
- A rapid reduction in total IgG was observed after one week; the switch in self-administration method had no impact on IgG levels (**Figure 3**)
- Clinically significant improvements in MG-ADL score (≥2-point improvement from baseline) were observed in both treatment sequences by Week 7; improvements were sustained across the Self-Administration Periods (**Figure 4**)

Figure 1 MG0020 study design



Rozanolixizumab was administered using a fixed dosing regimen (420 mg for body weight 35–<50 kg; 560 mg for bodyweight ≥50 kg) or weight-tiered dosing of 7 mg/kg for Japanese patients. *Patients completing all treatment periods, including the End of Treatment Visit, and moving on to either a post-study access program or commercially available rozanolixizumab during the Safety Follow-Up Period underwent an earlier End of Study Visit prior to this move.

Table 1 Baseline demographic and disease characteristics were generally well balanced between self-administration sequences

		Self-administration population (RSS)			Overall population (SS)
		SRD–MP (N=28)	MP–SRD (N=27)	RLZ total (N=55)	RLZ total (N=62)
Age, years, mean (SD)		**52.9** (16.3)	**53.4** (15.7)	**53.1** (15.9)	**53.3** (15.7)
Sex, female, n (%)		**16** (57.1)	**15** (55.6)	**31** (56.4)	**35** (56.5)
MG-ADL score, mean (SD)		**7.1** (3.9)	**7.5** (3.9)	**7.3** (3.9)	**7.3** (3.9)
MGFA Disease Class, n (%)	I	**1** (3.6)	**0**	**1** (1.8)	**1** (1.6)
	II	**12** (42.9)	**12** (44.4)	**24** (43.6)	**28** (45.2)
	III	**15** (53.6)	**15** (55.6)	**30** (54.5)	**33** (53.2)
	IV–V	**0**	**0**	**0**	**0**
RLZ-naive, n (%)*		**19** (67.9)	**18** (66.7)	**37** (67.3)	**42** (67.7)
Age at initial MG diagnosis, years, mean (SD)		**46.8** (18.9)	**44.6** (18.4)	**45.7** (18.5)	**45.8** (18.3)
Duration of disease, years, mean (SD)[‡]		**6.4** (8.1)	**9.4** (9.4)	**7.9** (8.8)	**7.9** (8.5)
Myasthenic crisis in the past, n (%)		**8** (28.6)	**6** (22.2)	**14** (25.5)	**15** (24.2)
Anti-AChR Ab+, n (%)		**21** (75.0)	**20** (74.1)	**41** (74.5)	**46** (74.2)
Anti-MuSK Ab+, n (%)		**3** (10.7)	**2** (7.4)	**5** (9.1)	**5** (8.1)
Anti-LRP-4 Ab+, n (%)		**0**	**0**	**0**	**1** (1.6)
Prior gMG medications, n (%)[¶]	Parasympathomimetics	–	–	–	**54** (87.1)
	Corticosteroids	–	–	–	**37** (59.7)
	Immunosuppressants	–	–	–	**31** (50.0)

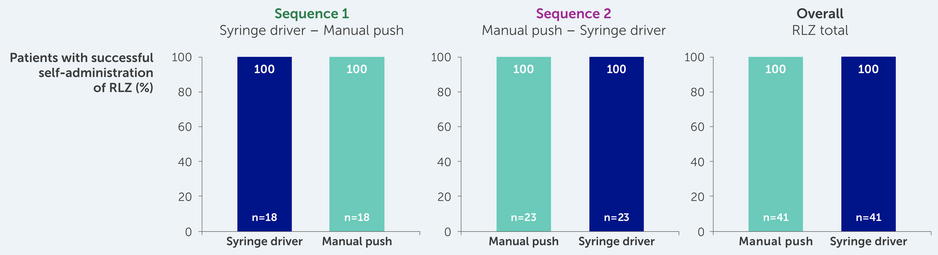
Safety set and randomized safety set. The safety set consisted of all patients who received at least one dose of rozanolixizumab (partial or full). The randomized safety set consisted of all patients who were included in the safety set and were randomized. *Patients were considered rozanolixizumab-naive if they did not receive rozanolixizumab prior to study entry. All non-naïve patients participated in the MG0007 study. †From diagnosis. ‡Prior medications include any medications that started before the first administration of RLZ. Data for prior gMG medications were captured prior to the Training Period and were therefore analyzed in the safety set.

Table 2 The incidence of TEAEs was consistent with the established safety profile of HCP-administered rozanolixizumab

	Training Period (N=62) n (%)	Self-Administration Periods* (N=58) n (%)	Safety Follow-Up† (N=53) n (%)	RLZ total (N=62) n (%)
Any TEAEs‡	**35** (56.5)	**30** (51.7)	**6** (11.3)	**47** (75.8)
Headache	**12** (19.4)	**4** (6.9)	**0**	**13** (21.0)
COVID-19	**4** (6.5)	**2** (3.4)	**0**	**7** (11.3)
Pyrexia	**4** (6.5)	**3** (5.2)	**0**	**6** (9.7)
Diarrhea	**4** (6.5)	**1** (1.7)	**0**	**5** (8.1)
Nasopharyngitis	**0**	**5** (8.6)	**1** (1.9)	**5** (8.1)
Serious TEAEs‡	**1** (1.6)	**4** (6.9)	**2** (3.8)	**7** (11.3)
MG worsening	**1** (1.6)	**0**	**1** (1.9)	**2** (3.2)
TEAEs resulting in permanent withdrawal from RLZ	**3** (4.8)	**1** (1.7)	**0**	**4** (6.5)
Treatment-related TEAEs‖	**19** (30.6)	**8** (13.8)	**2** (3.8)	**22** (35.5)
Severe TEAEs	**1** (1.6)	**2** (3.4)	**1** (1.9)	**4** (6.5)
All deaths¶	**0**	**0**	**0**	**0**

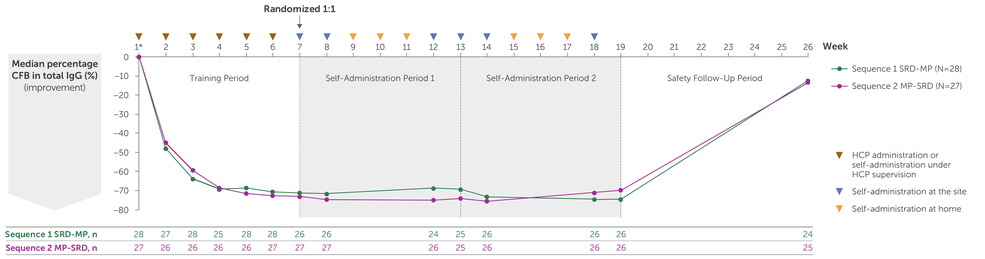
Safety set. *Includes 55 randomized patients and three patients who were not randomized as they were not eligible for self-administration. †Patients who discontinued in the Training Period but completed Safety Follow-Up are excluded. ‡Individual preferred terms listed under 'Any TEAEs' are those occurring in ≥5% of patients in RLZ total. §Individual preferred terms listed under 'Serious TEAEs' are those occurring in >1 patient in RLZ total. ‖Treatment-related was based on the investigator assessment. ¶AEs leading to death.

Figure 2 All patients in both sequences successfully self-administered 100% of their rozanolixizumab infusions



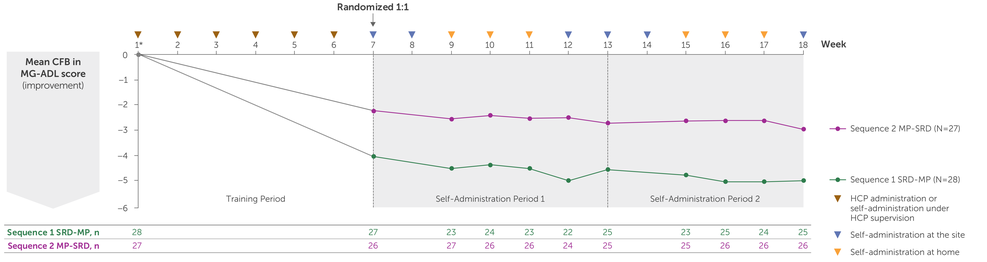
Primary efficacy analyses were performed on the full analysis set, which consisted of all patients who were in the safety set, were randomized and completed both Self-Administration Periods. Percentages are based on the number of patients with non-missing data.

Figure 3 There was a rapid and sustained reduction in total IgG serum concentration



Randomized safety set. IgG values up to and including 8 weeks after the start date of rescue therapy were excluded. Mean (SD) baseline value was 9.85 (2.29) g/L for Sequence 1 (N=28) and 9.76 (2.78) g/L for Sequence 2 (N=27). *Baseline values were defined as the last non-missing measurement before the first administration of rozanolixizumab at Week 1.

Figure 4 Improvements from Baseline in MG-ADL score were observed by the end of the Training Period (Week 7) and remained consistent across both Self-Administration Periods



Randomized safety set. The difference in mean CFB between the two groups occurred purely by chance due to the randomization at Week 7. The two patients with the largest improvement (≥12 points) at the end of the Training Period (Week 7) were both randomly assigned to Sequence 1, while five of the six patients who demonstrated MG-ADL worsening were randomly assigned to Sequence 2. A difference of approximately two points in mean MG-ADL score was observed between the two groups at Week 7 and was preserved during the Self-Administration Periods. Mean (SD) baseline value was 7.07 (3.89) for Sequence 1 (N=28) and 7.52 (3.94) for Sequence 2 (N=27). *Baseline values were defined as the last non-missing measurement before the first administration of rozanolixizumab at Week 1.

Summary and conclusions

MG0020 was a Phase 3, open-label, randomized, crossover study of rozanolixizumab self administration in patients with gMG aged ≥18 years

All patients successfully self-administered rozanolixizumab at all visits, using both manual push and syringe driver methods, in clinic and at home

Efficacy, pharmacodynamics and safety were consistent with the known profile of HCP-administered rozanolixizumab

These findings support manual push and syringe driver self-administration of rozanolixizumab as viable options for patients with gMG

Based on data from MG0020, self-administration of rozanolixizumab via manual push and syringe driver methods following training by an HCP has been approved in the EU and Japan[4–6]

Abbreviations: AE, adverse event; anti-AChR Ab+, anti-acetylcholine antibody positive; anti-LRP-4 Ab+, anti-low-density lipoprotein receptor-related protein 4 antibody positive; anti-MuSK Ab+, anti-muscle-specific tyrosine kinase antibody positive; CFB, change from baseline; COVID-19, coronavirus disease 2019; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; HCP, healthcare professional; IgG, immunoglobulin G; IgG4, immunoglobulin G4; mAb, monoclonal antibody; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MP, manual push; RLZ, rozanolixizumab; RSS, randomized safety set; SC, subcutaneous; SD, standard deviation; SRD, syringe driver; TEAE, treatment-emergent adverse event; SS, safety set.

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