Rozanolixizumab treatment patterns in patients with generalised myasthenia gravis: *Post hoc* analysis

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Introduction

- Rozanolixizumab is a humanised IgG4 monoclonal antibody FcRn inhibitor for the treatment of adults with anti-AChR Ab+ or anti-MuSK Ab+ gMG[1,2]
- In the Phase 3 MycarinG study (NCT03971422), adults with gMG received one 6-week treatment cycle of once-weekly rozanolixizumab or placebo[1]
- Patients from MycarinG who enrolled directly in the MG0007 OLE study (NCT04650854) received one further treatment cycle, then subsequent need-based cycles were given based on symptom worsening initiated at the investigator's discretion[3]
  - Some patients from MycarinG enrolled in a separate OLE study (MG0004, NCT04124965) before switching to MG0007, following which all cycles were based on symptom worsening
  - The use of need-based (symptom-driven) cycles led to variability in the frequency of cycles received by each patient and the duration of treatment-free intervals
- At the time of interim analysis, patients who had participated for at least 1 year had initiated a mean of 4.0 cycles (median 4.0, range 1–7) in the first year. This suggests an expected treatment pattern of 6 weeks' treatment followed by 6–8 weeks' treatment-free interval, that can be adjusted according to the individual needs of the patient
- This *post hoc* analysis aimed to describe the range of rozanolixizumab treatment patterns in more detail and their associations with baseline patient characteristics

Methods

- Patients enrolled in MycarinG were aged ≥18 years with anti-AChR Ab+ or anti-MuSK Ab+ gMG, MGFA Disease Class II–IVa, MG-ADL score ≥3 (for non-ocular symptoms) and QMG score ≥11[1]
- Following MycarinG, patients could enrol in one of two OLE studies: MG0004 (chronic weekly treatment) and MG0007 (cyclic treatment); patients in MG0004 could enrol in MG0007 at any time after ≥6 weeks
- Clustering analysis on the number of cycles per year was conducted using data from patients with ≥1 cycle from MycarinG and MG0007 (data cut-off: 8 July 2022)
  - Clustering is a data-driven approach used to describe between-patient variability in the number of cycles
  - Clustering with the best fit was determined using three metrics: the pseudo-F statistic comparing between-cluster variability and within-cluster variability, the R-squared statistic giving the percentage of variability explained by clustering, and the sample size of each cluster
  - For patients with <12 months in the studies who discontinued, cycles per year was calculated as total number of cycles. For all other patients, cycles per year was calculated as total number of cycles over total follow-up
- Baseline patient characteristics were assessed for association with the number of cycles per year using a multivariate logistic regression model
- The reporting of any TEAEs, serious TEAEs and severe TEAEs by cycle was compared across the three clusters
- All analyses were descriptive

Results

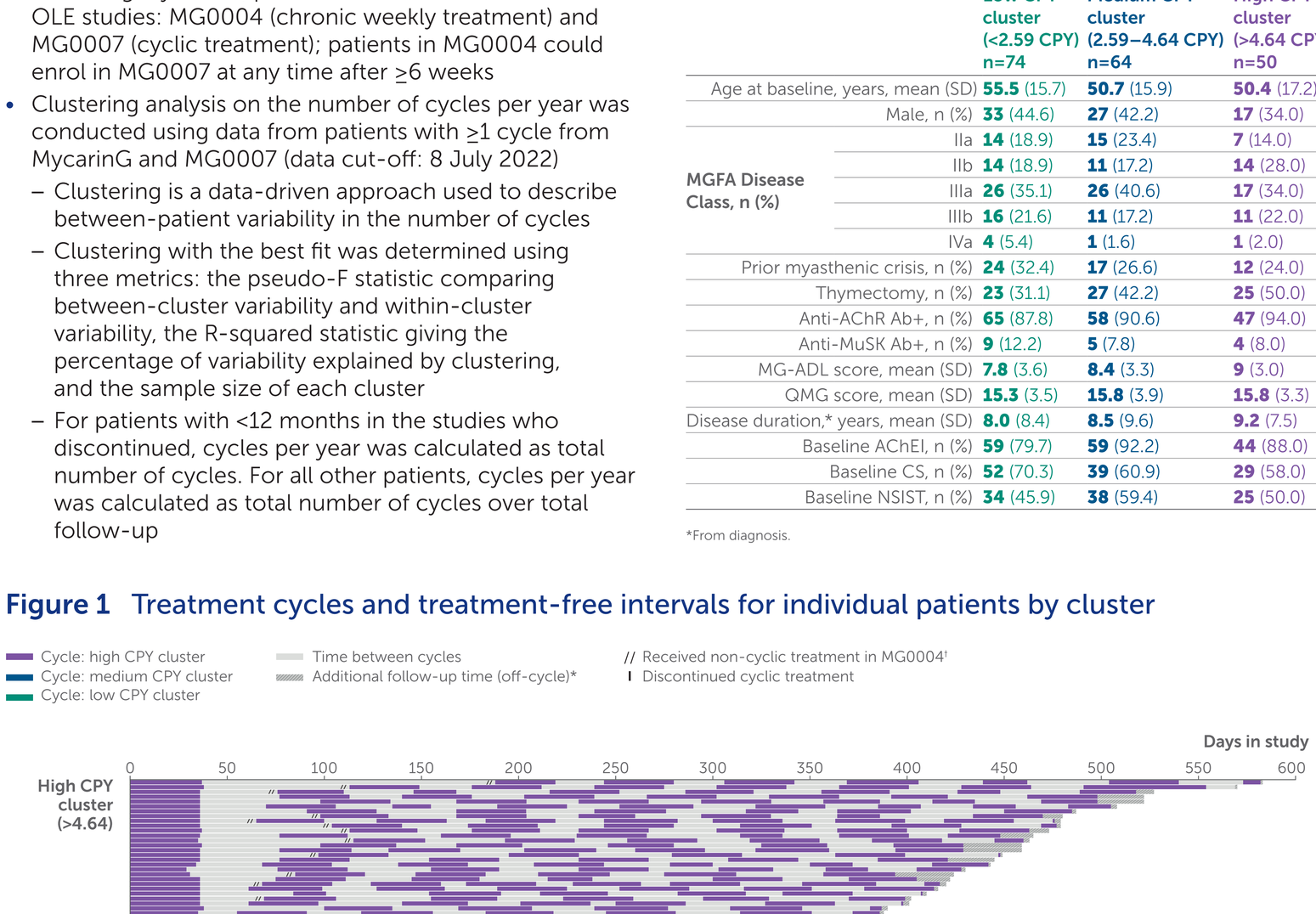
- A total of 188 patients received ≥1 cycle of rozanolixizumab treatment
- The most balanced clustering and optimal goodness-of-fit was achieved using three clusters to describe the number of cycles per year (low: <2.59 [n=74]; medium: 2.59–4.64 [n=64]; high: >4.64 [n=50])
  - The mean (SD) number of cycles per year in each cluster was 1.50 (0.53), 3.59 (0.60) and 5.82 (0.72), respectively
  - The range of cycles per year in each cluster was 0.61–2.54, 2.60–4.64 and 4.77–7.53, respectively
- Treatment-free interval lengths varied between and within patients across the three clusters (**Figure 1**)
- Baseline patient characteristics were generally balanced between the clusters (**Table 1**) and did not predict the cluster in which a patient would be categorised (**Figure 2**)
- Rozanolixizumab was generally well tolerated and, consistent with previous analyses,[4] the incidence of TEAEs did not increase over repeated cycles (**Table 2**)
  - In Cycles 1 and 2, the incidence of serious and severe TEAEs was higher in the low cluster than in the medium and high clusters
  - In the first two cycles, 28.4% (21/74) of patients in the low cluster (but no patients in the medium and high clusters) discontinued due to TEAEs

Table 1 Baseline patient characteristics by cluster

		Low CPY cluster (<2.59 CPY) n=74	Medium CPY cluster (2.59–4.64 CPY) n=64	High CPY cluster (>4.64 CPY) n=50
Age at baseline, years, mean (SD)		**55.5** (15.7)	**50.7** (15.9)	**50.4** (17.2)
Male, n (%)		**33** (44.6)	**27** (42.2)	**17** (34.0)
MGFA Disease Class, n (%)	IIa	**14** (18.9)	**15** (23.4)	**7** (14.0)
	IIb	**14** (18.9)	**11** (17.2)	**14** (28.0)
	IIIa	**26** (35.1)	**26** (40.6)	**17** (34.0)
	IIIb	**16** (21.6)	**11** (17.2)	**11** (22.0)
	IVa	**4** (5.4)	**1** (1.6)	**1** (2.0)
Prior myasthenic crisis, n (%)		**24** (32.4)	**17** (26.6)	**12** (24.0)
Thymectomy, n (%)		**23** (31.1)	**27** (42.2)	**25** (50.0)
Anti-AChR Ab+, n (%)		**65** (87.8)	**58** (90.6)	**47** (94.0)
Anti-MuSK Ab+, n (%)		**9** (12.2)	**5** (7.8)	**4** (8.0)
MG-ADL score, mean (SD)		**7.8** (3.6)	**8.4** (3.3)	**9** (3.0)
QMG score, mean (SD)		**15.3** (3.5)	**15.8** (3.9)	**15.8** (3.3)
Disease duration,* years, mean (SD)		**8.0** (8.4)	**8.5** (9.6)	**9.2** (7.5)
Baseline AChEI, n (%)		**59** (79.7)	**59** (92.2)	**44** (88.0)
Baseline CS, n (%)		**52** (70.3)	**39** (60.9)	**29** (58.0)
Baseline NSIST, n (%)		**34** (45.9)	**38** (59.4)	**25** (50.0)

*From diagnosis.

Figure 1 Treatment cycles and treatment-free intervals for individual patients by cluster



Each row represents an individual patient cycling through successive treatment cycles and treatment-free intervals.

*For patients who were ongoing at the data cut-off date (8 July 2022), follow-up was censored; additional follow-up time for these patients is the difference between the end of last treatment cycle and this date.

†Patients spent an unspecified time period in the MG0004 study and received weekly (non-cyclic) rozanolixizumab treatment after Cycle 1 in MycarinG.

‡Patients in MycarinG who completed the study but did not continue to any OLE study (n=9) were included in the cluster analysis but are not presented in the figure.

Summary and conclusions

In the MycarinG OLE, MG0007, the use of need-based, MG symptom-driven cycles of rozanolixizumab treatment initiated at the investigator's discretion led to variability in the number of cycles received per patient and the duration of treatment-free intervals

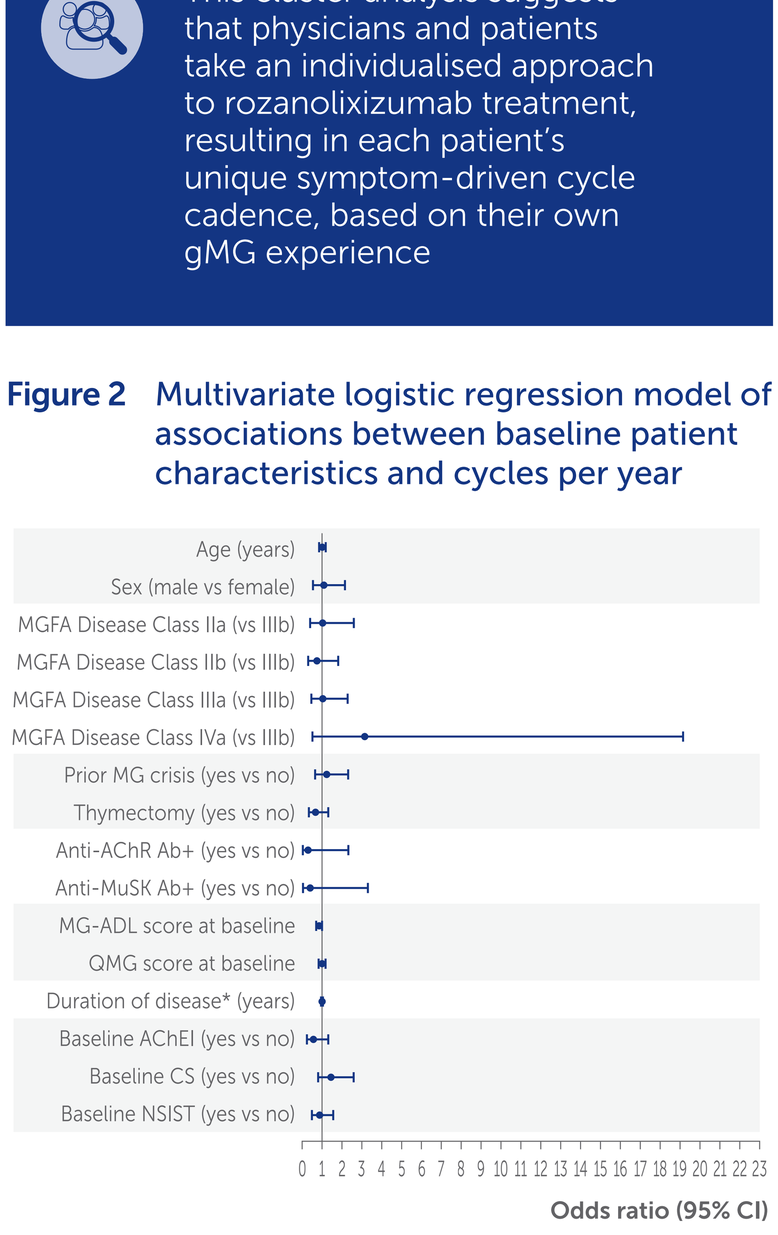
Three treatment clusters based on mean cycles per year demonstrated that rozanolixizumab cycle cadence varies between patients, from approximately 1–7 cycles per year

There were no significant differences in the baseline characteristics of patients across the three clusters

Treatment with rozanolixizumab was well tolerated and the incidence of TEAEs did not increase over repeated cycles, in line with previous analyses; the safety profile differed slightly between the low and medium/high clusters

This cluster analysis suggests that physicians and patients take an individualised approach to rozanolixizumab treatment, resulting in each patient's unique symptom-driven cycle cadence, based on their own gMG experience

Figure 2 Multivariate logistic regression model of associations between baseline patient characteristics and cycles per year



CPY was the response variable (categorical: high, medium and low). BMI at baseline was also included in the model but is not shown. *From diagnosis.

Table 2 Safety summary by cluster

		Low CPY cluster (<2.59 CPY)	Medium CPY cluster (2.59–4.64 CPY)	High CPY cluster (>4.64 CPY)	All patients
		Patients per cycle: n1=74, n2=38, n3=10, n4=1	Patients per cycle: n1=64, n2=55, n3=53, n4=42	Patients per cycle: n1=50, n2=50, n3=50, n4=49	Patients per cycle: n1=188, n2=143, n3=113, n4=92
Any TEAE, n (%)	Cycle 1	**58** (78.4)	**49** (76.6)	**40** (80.0)	**147** (78.2)
	Cycle 2	**26** (68.4)	**36** (65.5)	**38** (76.0)	**100** (69.9)
	Cycle 3	**6** (60.0)	**29** (54.7)	**32** (64.0)	**67** (59.3)
	Cycle 4	**1** (100.0)	**21** (50.0)	**31** (63.3)	**53** (57.6)
Serious TEAEs, n (%)	Cycle 1	**14** (18.9)	**4** (6.3)	**2** (4.0)	**20** (10.6)
	Cycle 2	**7** (18.4)	**0**	**2** (4.0)	**9** (6.3)
	Cycle 3	**1** (10.0)	**3** (5.7)	**1** (2.0)	**5** (4.4)
	Cycle 4	**0**	**3** (7.1)	**2** (4.1)	**5** (5.4)
Severe TEAEs, n (%)	Cycle 1	**17** (23.0)	**3** (4.7)	**3** (6.0)	**23** (12.2)
	Cycle 2	**6** (15.8)	**1** (1.8)	**2** (4.0)	**9** (6.3)
	Cycle 3	**1** (10.0)	**3** (5.7)	**2** (4.0)	**6** (5.3)
	Cycle 4	**0**	**4** (9.5)	**4** (8.2)	**8** (8.7)

nX, number of patients in Cycle X.

Abbreviations: Ab+, antibody positive; AChEI, acetylcholinesterase inhibitor; AChR, acetylcholine receptor; BMI, body mass index; CI, confidence interval; CPY, cycles per year; CS, corticosteroid; FcRn, neonatal fragment crystallisable receptor; (g)MG, (generalised) myasthenia gravis; IgG4, immunoglobulin G subclass 4; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MuSK, muscle-specific tyrosine kinase; NSIST, non-steroidal immunosuppressant therapy; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; SD, standard deviation; TEAE, treatment-emergent adverse event.

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The data were previously presented at the MGFA Scientific Session; Savannah, GA, USA; 15 October 2024