Characterisation of patients with generalised myasthenia gravis on zilucoplan treatment in European Managed Access Programmes

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Introduction

- MG is a rare, chronic, neuromuscular autoimmune disease characterised by fluctuating muscle weakness and fatigue[1,2]
- In the Phase 3 RAISE study (NCT04115293), treatment with zilucoplan, a C5 inhibitor, demonstrated clinically meaningful improvements in multiple MG-specific outcomes in patients with gMG, sustained up to Week 120 in the OLE RAISE-XT study (NCT04225871)[3,4]
- MAPs enable use of treatments prior to authorisation for patients with a severe/debilitating/life-threatening condition, where available treatments are deemed inadequate by patients and physicians and there is no available clinical trial
- **The objective of this study was to describe patient characteristics and zilucoplan treatment outcomes in MAPs from three countries: France, Italy and Spain**

Methods

- This was an observational, retrospective, longitudinal cohort study of adults with gMG who initiated zilucoplan treatment in MAPs in France, Italy and Spain between December 2021 and November 2024, and who consented for their data to be collected in the real-world database (**Figure 1**)
- All analyses were descriptive

Results

- 57 patients from France and 38 from Italy were included
- Data were available for <5 patients from Spain, who had a mean (SD) age of 48.8 (13.6) years; 75.0% were female
  - Given the small number of patients from Spain, further results are not presented
- Patients who received zilucoplan had a long disease duration and high rates of prior hospitalisation and crisis (**Table 1**)
- The majority of patients previously received corticosteroids and pyridostigmine before entering the MAP (**Table 2**)
- In France (N=45), four (8.9%) patients received zilucoplan 16.6 mg, 16 (35.6%) patients received 23.0 mg and 25 (55.6%) patients received 32.4 mg
- In Italy (N=38), five (13.2%) patients received zilucoplan 16.6 mg, 14 (36.8%) patients received 23.0 mg and 19 (50.0%) patients received 32.4 mg
- Mean (SD) treatment duration was 6.6 (4.5) months in France and 8.1 (4.2) months in Italy
- MG-ADL score improvement with zilucoplan was sustained over time for both France and Italy (**Figure 2**)
- Garches score improved over time for patients in France (**Figure 3**)
- Quality of life, as measured by MG-QoL15r, improved over follow-up for 11 patients in Italy, but data are not presented as patient numbers were low for this endpoint
  - MG-QoL15r data were not available for France
- A third to a half of patients reduced their corticosteroid dose during follow-up after receiving zilucoplan (**Table 3**)
- 13/57 (22.8%) patients in France discontinued zilucoplan treatment and 13/29 (44.8%) patients discontinued in Italy
- 19/57 (33.3%) patients had a TEAE in France and 12/64 (18.8%) patients had a TEAE in Italy (**Table 4**)
  - One (1.8%) death occurred in France, attributed to complications of pneumonia, unrelated to zilucoplan (**Table 4**)

Limitations

- The primary purpose of a MAP is to provide treatments for patients who are otherwise unable to access them, not data collection
- Patients in the MAP could optionally provide consent for additional data collection. It was not mandatory for clinicians to complete additional data entry. As a result, data are limited beyond the baseline assessment
- The small sample size meant that only descriptive analyses were conducted
- The time on treatment during the MAP depended on when the patient joined the program and the timing of national reimbursement in the participating country. In some instances, this was a limited duration

Summary and conclusions

This observational, retrospective, longitudinal cohort study assessed adults with gMG who initiated zilucoplan treatment in MAPs in France, Italy and Spain

MG-specific outcomes, including MG-ADL, improved over follow-up for patients in France and Italy; however, data should be interpreted with caution due to low patient numbers

A third to a half of patients reduced their corticosteroid dose during follow-up after receiving zilucoplan

These data characterise patients receiving zilucoplan in a MAP in France and Italy who otherwise would not have been able to access the treatment

Figure 1 Zilucoplan has been provided in MAPs in Europe and Australia

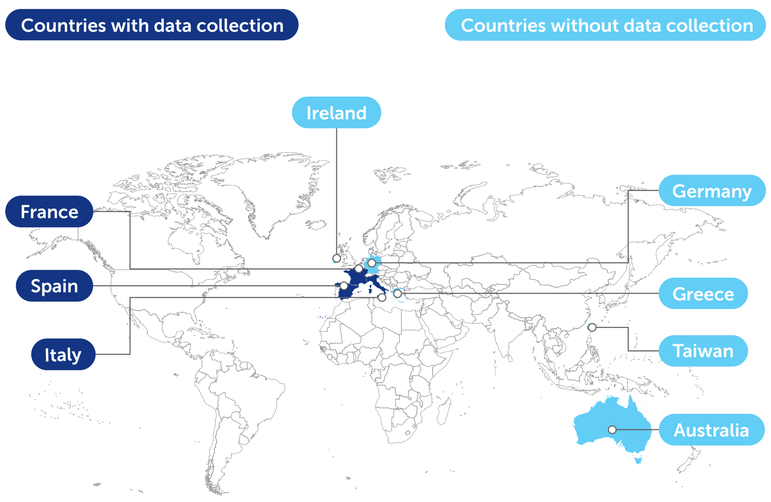


Table 1 Patients with gMG receiving zilucoplan in the MAPs reflected a population with high clinical unmet need

		France (N=57)	Italy (N=38)
Age, years, mean (SD)		**56.5** (19.8)	**56.6** (14.4)
Male, n (%)		**28** (49.1)	**13** (34.2)
Weight, kg, mean (SD)		**77.7** (19.1)	**80.9** (22.8)
MG-ADL score, mean (SD) [n]		**7.3** (4.7) [45]	**8.7** (3.7) [38]
MGFA Class, n (%)	II	–	**12** (31.6)
	III	–	**23** (60.5)
	IV	–	**3** (7.9)
Garches score, mean (SD) [n]		**68.8** (17.2) [44]	–
Time since initial MG diagnosis	Mean (SD), years [n]	**8.7** (9.2) [56]	**10.7** (9.6) [38]
	<5 years, n (%)	**24** (42.1)	**11** (28.9)
	≥5 years, n (%)	**32** (56.1)	**27** (71.1)
	Missing, n (%)	**1** (1.8)	**0**
MG exacerbation in the prior 2 years, n/N (%)		–	**29/35** (82.9)
MG crisis in the prior 2 years, n/N (%)		**33/54** (61.1)	**4/37** (10.8)
MG-related hospitalisation in the prior 2 years, n/N (%)		**46/54** (85.2)	**18/36** (50.0)
Thymectomy, n/N (%)		**23/56** (41.1)	**20/38** (52.6)

– Indicates data not collected or missing.

Table 2 Patients received other treatments before entering the zilucoplan MAPs

		France (N=57)	Italy (N=38)
Long-term immunotherapy, n (%)	Corticosteroids	**43** (75.4)	**38** (100)
	Azathioprine	**24** (42.1)	**32** (84.2)
	Rituximab	**18** (31.6)	**0**
	MMF	**15** (26.3)	**9** (23.7)
	Ciclosporin	**2** (3.5)	**5** (13.2)
	MTX	**0**	**2** (5.3)
	Cyclophosphamide	**0**	**1** (2.6)
	Tacrolimus	**1** (1.8)	**0**
Rescue treatments, n (%)	Immunoglobulins	**41** (71.9)	**29** (76.3)
	PLEX	**27** (47.4)	**7** (18.4)
	Plasmapheresis	**0**	**9** (23.7)
Cholinesterase inhibitors, n (%)	Pyridostigmine	**42** (73.7)	**37** (97.4)
	Ambenonium chloride	**11** (19.3)	**0**
New targeted therapies, n (%)	Efgartigimod	**8** (14.0)	**7** (18.4)
	Ravulizumab	**6** (10.5)	**2** (5.3)
	Rozanolixizumab	**5** (8.8)	**1** (2.6)
	Eculizumab	**2** (3.5)	**2** (5.3)

Figure 2 MG-ADL score improved over follow-up for patients in (a) France and (b) Italy

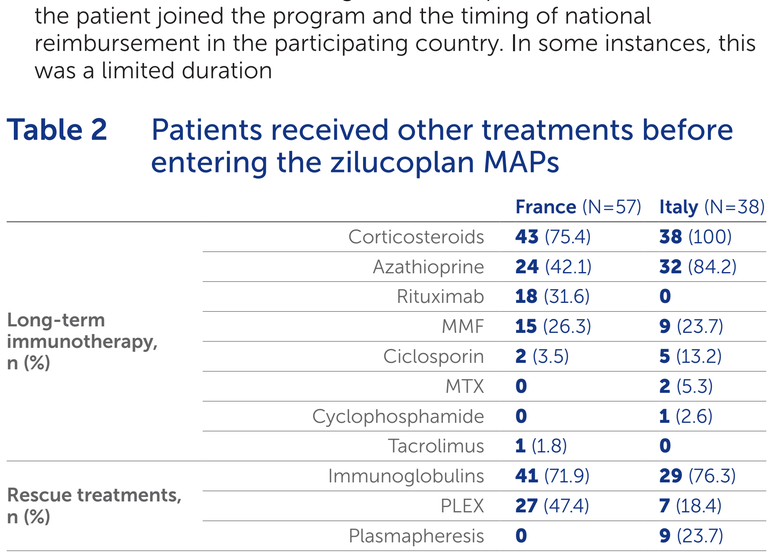
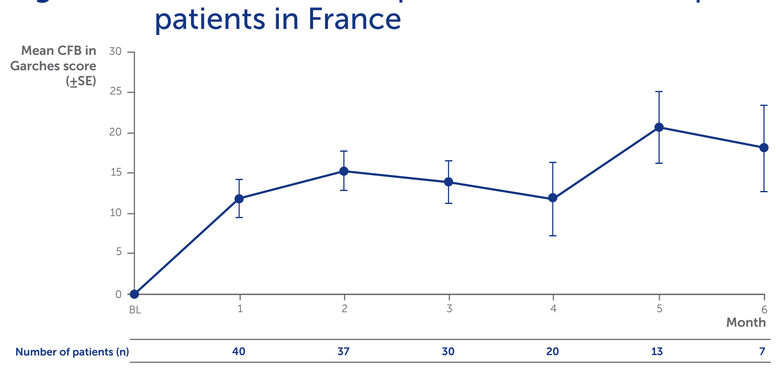


Figure 3 Garches score improved over follow-up for patients in France



Garches score data were not available for Italy.

Table 3 Corticosteroid use was reduced

		France (N=57)	Italy (N=38)
Corticosteroids, n (%)	Corticosteroid use, n (%)	**37** (64.9)	**29** (76.3)
	Dose reduced, n/N (%)*	**12/37** (32.4)	**16/29** (55.2)
	Dose increased, n/N (%)*	**3/37** (8.1)	**2/29** (6.9)
Immunosuppressants, n (%)	Immunosuppressant use, n (%)	**26** (45.6)	**17** (44.7)
	Dose reduced, n/N (%)†	**2/26** (7.7)	**0**
	Dose increased, n/N (%)	**0**	**0**
Rescue medication, n (%)‡	Immunoglobulins	**7** (12.3)	**2** (5.3)
	PLEX	**2** (3.5)	**0**
	Plasmapheresis	**0**	**0**

*The denominator is the number of patients receiving corticosteroids at baseline or during follow-up.
†The denominator is the number of patients receiving immunosuppressants at baseline or during follow-up.
‡During follow-up.

Table 4 The safety profile was consistent with the known safety profile of zilucoplan

	France (N=57)	Italy (N=64)
Any TEAE, n (%)*	**19** (33.3)	**12** (18.8)
MG	**5** (8.8)	**1** (1.6)
Drug ineffective	**3** (5.3)	**0**
Dehydration	**2** (3.5)	**0**
Diarrhoea	**2** (3.5)	**0**
Dysphagia	**2** (3.5)	**0**
Fatigue	**2** (3.5)	**0**
Gamma-glutamyl transferase increased	**2** (3.5)	**0**
Headache	**2** (3.5)	**0**
Pancreatic enzymes increased	**2** (3.5)	**1** (1.6)
Therapy interrupted	**2** (3.5)	**0**
Death, n (%)	**1** (1.8)†	**0**

Reporting on safety information was required for all patients in the MAP in addition to those who consented to RWE data collection.
*Individual TEAEs listed are those occurring in >1 patient in either France or Italy.
†Unrelated to zilucoplan.

Abbreviations: BL, baseline; C5, complement component 5; CFB, change from baseline; gMG, generalised myasthenia gravis; MAP, Managed Access Programme; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; MG-QoL15r, Myasthenia Gravis Quality of Life 15-item revised; MMF, mycophenolate mofetil; MTX, methotrexate; OLE, open-label extension; PLEX, plasma exchange; RWE, real-world evidence; SD, standard deviation; SE, standard error; TEAE, treatment-emergent adverse event.

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References: 1. Gilhus NE, et al. Nat Rev Dis Primers. 2019;5:30. 2. Law N, et al. Neurol Ther. 2021;10:1103–1125. 3. Howard JF Jr., et al. Lancet Neurol. 2023;22:395–406. 4. Howard JF Jr., et al. Long-term safety and efficacy of zilucoplan in generalized myasthenia gravis: 120-week interim analysis of RAISE-XT [poster]. AANEM 2024. Poster 192.

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