

Characterization of patients with generalized myasthenia gravis on zilucoplan treatment in European Managed Access Programmes

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Introduction

- MG is a rare, chronic, neuromuscular autoimmune disease characterized 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 authorization 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 hospitalization and crisis (**Table 1**)
- The majority of patients previously received corticosteroids and pyridostigmine before entering the MAP (**Table 2**)

Figure 1 Zilucoplan has been provided in MAPs across the world

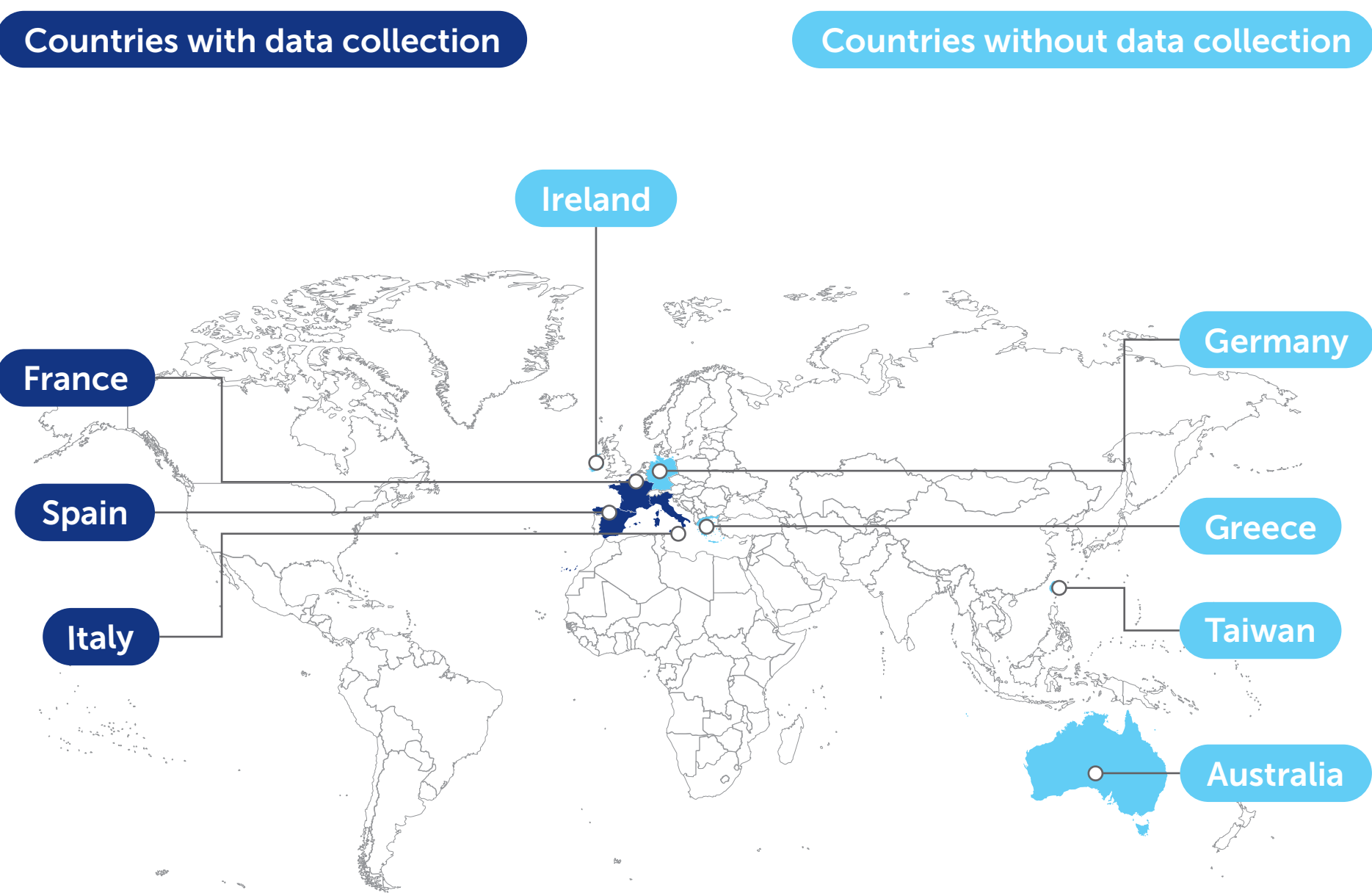


Table 1 Patients with gMG receiving zilucoplan in the MAPs reflected a population with high clinical unmet need

	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 Clinical Class, n (%)		
II –	–	12 (31.6)
III –	–	23 (60.5)
IV –	–	3 (7.9)
Garches score, mean (SD) [n]	68.8 (17.2) [44]	–
Mean (SD), years [n]	8.7 (9.2) [56]	10.7 (9.6) [38]
Time since initial MG diagnosis		
<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 hospitalization 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.

Abbreviations: BL, baseline; C5, complement component 5; CFB, change from baseline; gMG, generalized 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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Author disclosures: Michelle Mackechnie, Natasa Savic and Kerina Bonar are employees and shareholders of UCB. Paolo Emilio Alboini has received funding from Alexion Pharmaceuticals, Amgen, argenx and UCB. Pascal Cintas has received fees for participation on scientific boards and at scientific congresses sponsored by Sanofi Genzyme (now Sanofi). Sophie

- 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**)

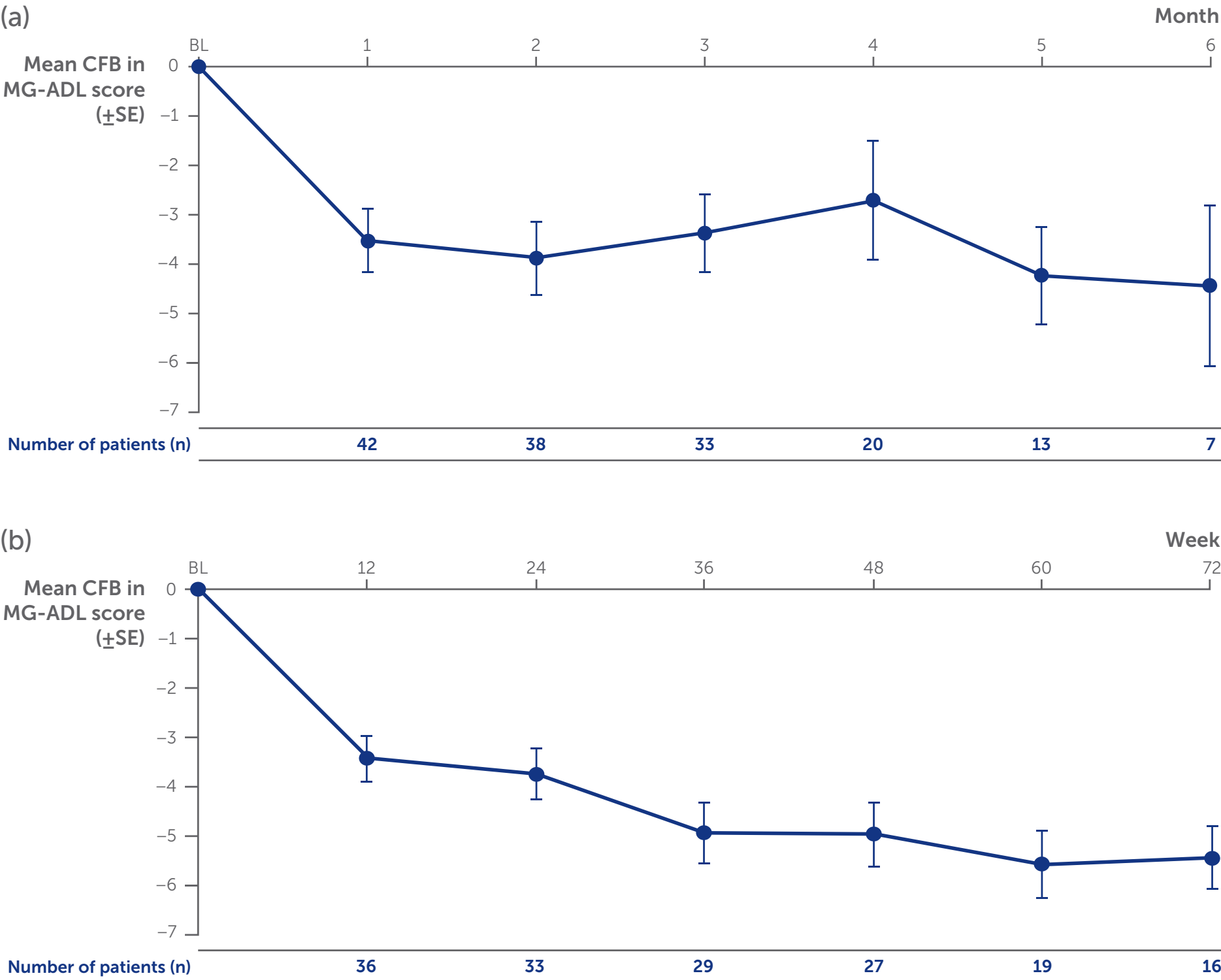
Limitations

- 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

Table 2 Patients received other treatments before entering the zilucoplan MAPs

	France (N=57)	Italy (N=38)
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
Immunoglobulins	41 (71.9)	29 (76.3)
PLEX	27 (47.4)	7 (18.4)
Plasmapheresis	0	9 (23.7)
Pyridostigmine	42 (73.7)	37 (97.4)
Ambenonium chloride	11 (19.3)	0
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



Demeret has served on scientific advisory boards for Alexion Pharmaceuticals, argenx, Johnson & Johnson and UCB. She has received consultant fees and research support from UCB. Vincenzo Di Stefano has received support for travel and meeting attendance from Alexion Pharmaceuticals, Alnylam, argenx and UCB. He is a member of the Italian Society of Clinical Neurophysiology (SINC), Italian Society of Myology (AIM), Italian Society of Neurology (SIN) and Italian Society of Peripheral Nervous System (IASNP). He has received speaker honoraria from Alexion Pharmaceuticals, Alnylam, argenx and UCB; he is a Sub-Investigator for clinical trials sponsored by Alexion Pharmaceuticals, Alnylam, argenx, Dianthus and Sanofi. Carmen Erra has received travel funding from CSL Behring and UCB. She has received speaker honoraria from Alexion Pharmaceuticals, argenx and UCB. Laura Fionda has received funding for travel and meeting attendance or advisory board participation from Alexion Pharmaceuticals, Amgen, argenx, Dianthus, Johnson & Johnson and UCB. Raffaele Iorio has received speaker honoraria and consulting fees from Alexion Pharmaceuticals, Amgen, argenx, Johnson & Johnson, Merck and UCB. Raul Juntas-Morales has nothing to disclose. Emmeline Lagrange has received funding from Alexion Pharmaceuticals, argenx and UCB. Michelangelo Maestri has received personal fees from Alexion

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

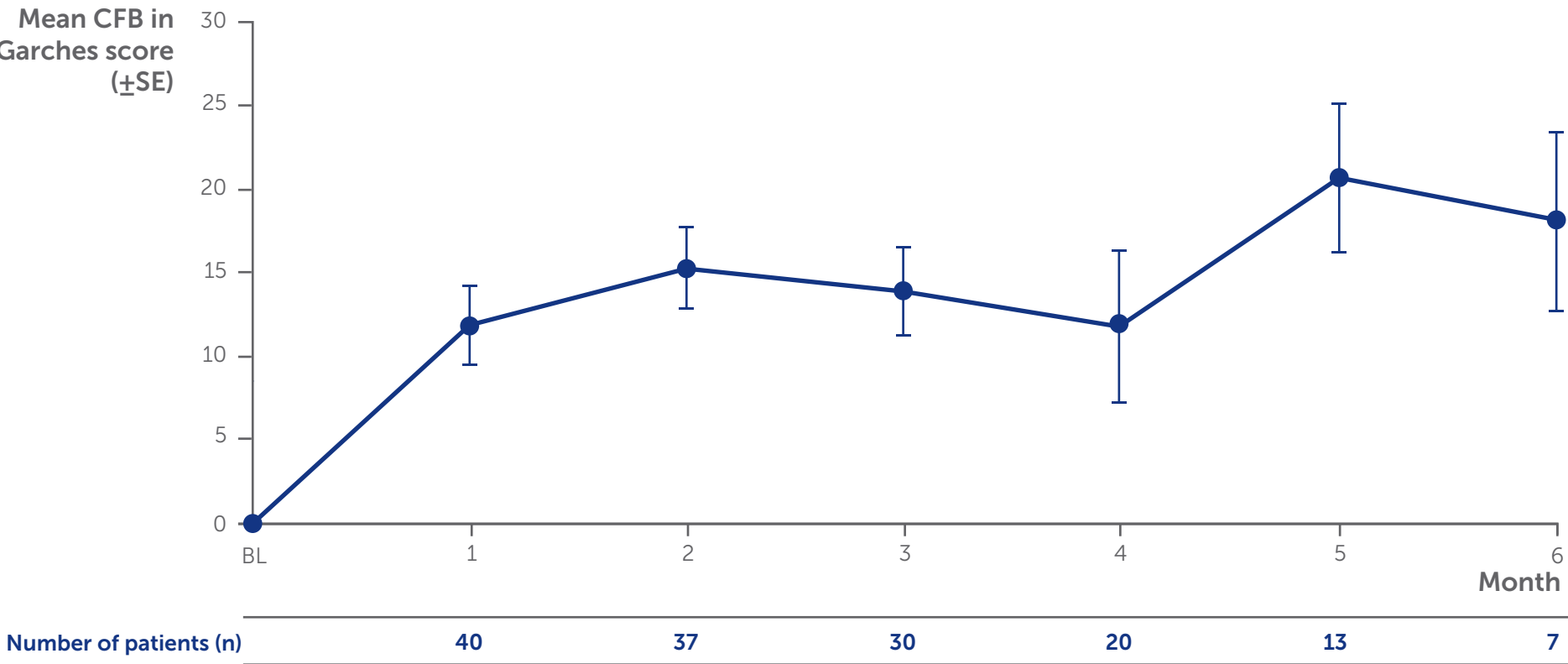


A third to a half of patients reduced their corticosteroid dose during follow-up after receiving zilucoplan



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

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)
Immunosuppressant use, n (%)	26 (45.6)	17 (44.7)
Immunosuppressants, n (%)		
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.

Pharmaceuticals, Amgen, argenx, Johnson & Johnson and UCB. Sabrina Sacconi has nothing to disclose. Fiammetta Vanoli has received funding for travel, congress attendance, speaking and advisory boards from Alexion Pharmaceuticals, argenx, Biogen and UCB. Claudia Vinciguerra has received funding for travel, meeting attendance, manuscript writing, educational events and speaker honoraria from Alexion Pharmaceuticals, argenx, Sanofi and UCB. Karin Annoni is a former employee of UCB. Grégory Chollet and Cristina Lopez are employees of UCB. Patrik Öhagen is a former contractor for UCB. Cassandra Slader is an employee and shareholder of UCB, and is a shareholder of Novartis.
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