Minimal Symptom Expression in Generalized Myasthenia Gravis Treated with Traditional Therapies: A Real-World Benchmark from MyRealWorld-MG

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KEY TAKEAWAYS

- In this real-world cohort of patients with gMG treated predominantly with traditional therapies, MSE was infrequent; these data highlight an unmet need and provide a benchmark before the broad uptake of targeted biologics
- Patients with MSE reported better HRQoL and mental health, and reduced societal burden approaching levels observed in the general population
- Patients with lower disease severity and comorbidity burden were more likely to have MSE, highlighting the value of intervention before disease burden accumulates
- Overall, these findings support MSE as a clinically meaningful, patient-centric treatment target and suggest that treatments that achieve MSE may provide broader benefits beyond symptom control

BACKGROUND

- gMG is a rare, immunoglobulin G-mediated, chronic, neuromuscular, autoimmune disease characterized by debilitating fatigable muscle weakness[1]
- MG-ADL is a disease-specific, four-domain, eight-item, patient-reported linear scale (range 0–24) used in clinical research and routine care to assess MG symptoms, functional status, and response to treatment[2]
- MSE, defined as an MG-ADL score of 0 or 1, is an emerging patient-centric treatment goal[3]
- Limited evidence exists on MSE prevalence, particularly in the real-world setting with traditional therapies, and on whether MSE corresponds to meaningful gains in broader HRQoL, work, and caregiver outcomes[2,4,5]

OBJECTIVES

- To assess the prevalence of MSE in patients with gMG treated with traditional therapies
- To examine the associations between MSE status and humanistic burden, work impact, and caregiver support needs in patients with gMG

METHODS

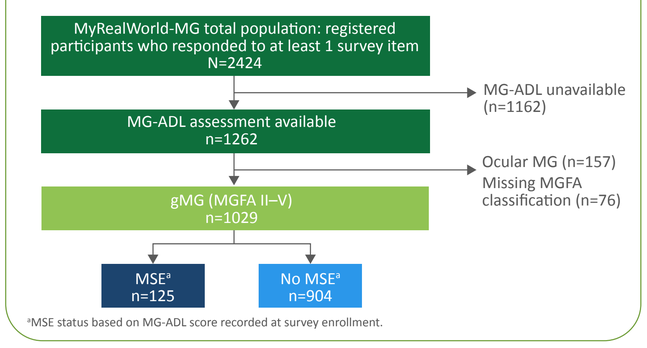
Study Design and Population Selection

- This cross-sectional analysis evaluated patient-reported data gathered at survey enrollment from adults aged ≥18 years participating in the MyRealWorld-MG observational study between December 2019 and May 2024[6] (**Figure 1**)
- Participants of MyRealWorld-MG were enrolled from Belgium, Canada, Denmark, France, Germany, Italy, Japan, Spain, the UK, and the USA
- The gMG cohort was stratified according to MSE status based on the MG-ADL score recorded at enrollment:
  - MSE: MG-ADL score of 0 or 1
  - No MSE: MG-ADL score of ≥2

Study Measures and Outcomes

- Outcomes included:
  - Treatments for MG received during routine care, either prior to or at the time of survey completion, including traditional therapies and targeted biologic therapy
    - Traditional therapies included anticholinesterase medications, immunoglobulins (intravenous and subcutaneous), corticosteroids, other immunosuppressive therapies (e.g., azathioprine, NSISTs, rituximab), plasma exchange, thymus surgery, supportive measures, lifestyle or complementary approaches, and other treatments
    - Advanced targeted biologic therapy at the time of survey development (2017) was limited to eculizumab or experimental treatments
  - PROs assessed using disease-specific (MG-ADL, MG-QoL15r) and generic (EQ-5D-5L, HADS-anxiety and HADS-depression) instruments and subscales
  - Societal burden, including work productivity, sick leave, and caregiver support needs
- PROs and societal outcomes were presented with values in the general population based on the POPUP digital survey[7]

FIGURE 1. Patient Flow Chart



[a]MSE status based on MG-ADL score recorded at survey enrollment.

Statistical Analyses

- Descriptive analyses were used to summarize demographic, clinical, and treatment characteristics by MSE status
- Multivariable logistic regression was used to identify factors associated with MSE, with covariates including country, sex, age, MGFA class, years since diagnosis, number of comorbidities, MG treatment, and year of survey completion
- Analyses were conducted using available cases; missing data were not imputed

RESULTS

Study Population

- Overall, 1029 patients with gMG were included in the analysis, of whom 125 (12.1%) had MSE at the time of survey completion (**Figure 1; Figure 2**)
- Most patients completed the survey between 2020 and 2022
- The largest proportion of patients were enrolled in Italy (30.1%), followed by the USA (28.0%)
- Around one-third of patients had four or more comorbidities (**Table 1**)
  - Patients in the MSE group had a lower overall comorbidity burden than those without MSE (mean [SD] 2.2 [1.4] vs 3.4 [2.3] comorbidities)

Treatment Usage Patterns

- The majority of patients received traditional therapies, most commonly anticholinesterase medications (88.0%), corticosteroids (68.5%), and NSISTs (41.8%) (**Table 2**)
- Few patients had received eculizumab at the time of the survey (5.2%)

Symptom Severity (MG-ADL)

- Mean (SD) MG-ADL score was 0.6 (0.5) in patients with MSE and 7.3 (3.6) in patients without MSE
- 74.1% of patients without MSE had moderate to severe symptoms (MG-ADL 5–9, MG-ADL ≥10, respectively) (**Figure 2**)

HRQoL, Mental Health, and Societal Outcomes

- Patients with MSE reported better HRQoL and mental health scores than those without MSE (p<0.001), with scores approaching the level of general population norms (**Figure 3**)
- Fewer patients with MSE reported being unable to work due to their disease, taking sick leave, or requiring caregiver support than those without MSE (**Figure 4**)

Factors Associated with MSE

- Higher MGFA class (III/IV) and comorbidity burden were independently associated with lower odds of having MSE (p<0.05 and p<0.001, respectively) (**Figure 5**)

FIGURE 2. Percentage of Overall Population (n=1029) by MG-ADL Symptom Category

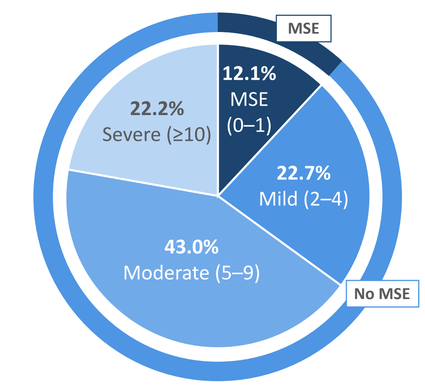


TABLE 1. Baseline Demographic and Clinical Characteristics

	Overall	MSE	No MSE
Sex	n=1015	n=124	n=891
Female, n (%)	730 (71.9)	75 (60.5)	655 (73.5)
Age, years	n=915	n=107	n=808
Mean (SD)	47.7 (14.1)	50.8 (14.2)	47.3 (14.1)
Current MGFA class, n (%)	n=1029	n=125	n=904
II	343 (33.3)	73 (58.4)	270 (29.9)
III	458 (44.5)	42 (33.6)	416 (46.0)
IV	211 (20.5)	9 (7.2)	202 (22.3)
V	17 (1.7)	1 (0.8)	16 (1.8)
Number of comorbidities, n (%)	n=656	n=53	n=603
0–3	411 (62.7)	47 (88.7)	364 (60.4)
4–5	143 (21.8)	5 (9.4)	138 (22.9)
≥6	102 (15.5)	1 (1.9)	101 (16.7)
Mean (SD)	3.3 (2.3)	2.2 (1.4)	3.4 (2.3)
Time since diagnosis, years, n (%)	n=912	n=107	n=805
<2	276 (30.3)	27 (25.2)	249 (30.9)
2–5	229 (25.1)	26 (24.3)	203 (25.2)
>5–10	159 (17.4)	21 (19.6)	138 (17.1)
>10	248 (27.2)	33 (30.8)	215 (26.7)
Median (Q1–Q3)	4 (1–11)	5 (1–16)	4 (1–10)

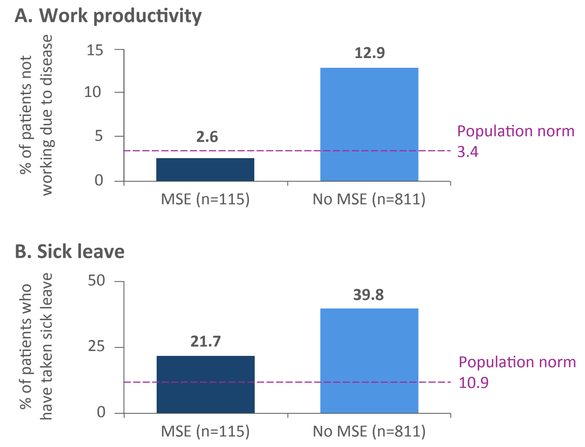
Percentages are calculated among patients with available information.

TABLE 2. Most Common Traditional and Targeted Therapies Received Prior to or at the Time of Survey Completion

	Overall	MSE	No MSE
Treatment information available, n	1014	125	889
Any routine MG treatment,[a] n (%)	917 (90.4)	108 (86.4)	809 (91.0)
Traditional therapies,[b,c] n (%)			
Anticholinesterase medication	807 (88.0)	92 (85.2)	715 (88.4)
Corticosteroids	628 (68.5)	78 (72.2)	550 (68.0)
NSISTs	383 (41.8)	38 (35.2)	345 (42.7)
IVIg	341 (37.2)	29 (26.9)	312 (38.6)
Azathioprine	336 (36.6)	42 (38.9)	294 (36.3)
Targeted therapies,[c,d] n (%)			
Eculizumab	48 (5.2)	1 (0.9)	47 (5.8)

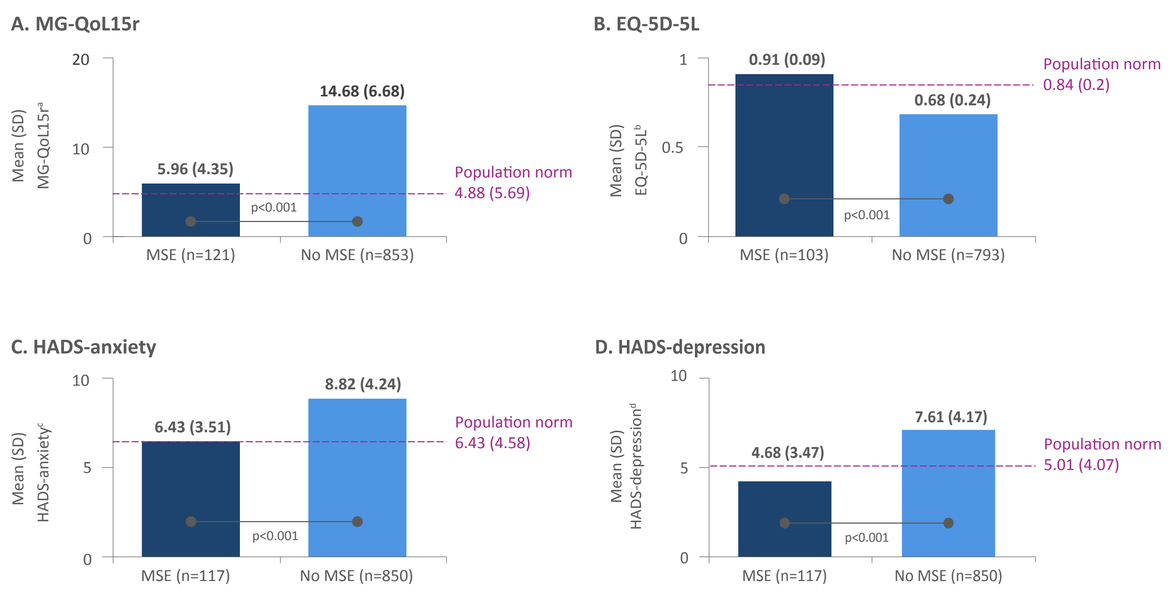
[a]Routine MG care was defined as any treatment not used to treat an exacerbation that was received prior to or at the time of the survey. [b]The five most frequently recorded traditional therapies are shown. Other treatments captured in the survey included: SCIg, 3.1%; other immunosuppressive therapies (e.g., rituximab, 8.9%); plasma exchange, 16.9%; thymus surgery, 29.8%; supportive measures, lifestyle, or complementary approaches; and other therapies. Categories are not mutually exclusive. [c]The denominator is the number of patients receiving routine treatment. [d]Eculizumab was the only advanced targeted therapy included in the questionnaire, per approved treatment options at the time of survey development (2017). A small number of patients reported the use of experimental treatments (n=3) or other targeted therapies under 'other treatments' (n<5).

FIGURE 4. Societal Burden by MSE Status



The dashed line corresponds to population norm values derived from 9000 participants in the multinational POPUP digital survey, a study designed to establish HRQoL representative of the general population.[7]

FIGURE 3. HRQoL (A and B) and Mental Health (C and D) Outcomes by MSE Status

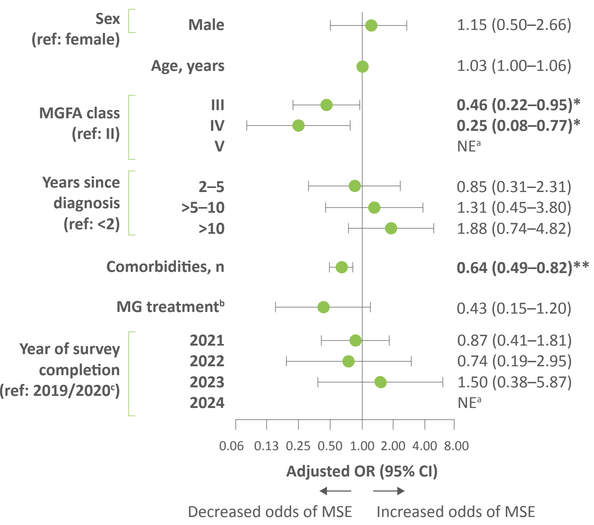


Statistical comparison of the MSE and no MSE cohorts was performed using independent sample T tests for continuous variables.

The dashed line corresponds to population norm values derived from 9000 participants in the multinational POPUP digital survey, a study designed to establish HRQoL representative of the general population.[7]

[a]MG-QoL15r scores range from 0–30; a higher score indicates poorer HRQoL.[6] [b]EQ-5D-5L utility index score of 1 indicates full health.[6] [c]HADS-anxiety subscale scores range from 0–21; a higher score indicates greater anxiety symptoms or psychological distress.[6] [d]HADS-depression subscale scores range from 0–21; a higher score indicates greater depression symptoms or psychological distress.[6]

FIGURE 5. Multivariable Logistic Model for Drivers of MSE



Only selected covariates are displayed for presentation purposes. Model adjusted for country; country-specific estimates not shown.

*p<0.05. **p<0.001. p-values are two-sided and were derived from Wald tests in the multivariable logistic regression model; based on 567 participants with complete data.

[a]Not estimable due to zero/few events in this category. [b]Any routine MG treatment (yes vs no). [c]Due to only one observation being available in December 2019, survey years 2019 and 2020 were combined for the analysis.

STUDY LIMITATIONS

- This was a cross-sectional analysis at a single time point (survey enrollment), which does not account for the fluctuating nature of MG
- The MyRealWorld-MG dataset relies on self-reported outcomes provided by patients via an online app-based survey, and is not clinically verified
- Cross-country heterogeneity and the broad range of severity within the no MSE group may have resulted in wide confidence intervals
- Most patients were treated with traditional therapies; however, a small proportion (~5%) had received targeted therapy with eculizumab, which may have influenced trends

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ABBREVIATIONS

CI, confidence interval; **EQ-5D-5L**, the EuroQol five-dimensional, five-level questionnaire; **gMg**, generalized myasthenia gravis; **HADS**, Hospital Anxiety and Depression Scale; **HRQoL**, health-related quality of life; **IVIg**, intravenous immunoglobulin; **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 questionnaire; **MSE**, minimal symptom expression; **NE**, not evaluable; **NSISTs**, non-steroidal immunosuppressive therapies; **OR**, odds ratio; **POPUP**, Population Norms Study; **PROs**, patient-reported outcomes; **Q**, quartile; **SCIg**, subcutaneous immunoglobulin; **SD**, standard deviation.

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