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# Effectiveness and safety of ravulizumab in generalized myasthenia gravis: updated analysis from a global registry

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## INTRODUCTION

- Anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG) is a rare, chronic autoimmune neuromuscular disease characterized by fatigable muscle weakness.[1-3]
- The complement component 5 inhibitor therapies (C5ITs) ravulizumab and eculizumab are approved treatments for AChR-Ab+ gMG.[4,5]
- The ongoing, global MG SPOTLIGHT Registry (NCT04202341) is a long-term, multicenter, observational registry and includes patients treated with ravulizumab, either alone or after transition from eculizumab.[6]
  - Registry data demonstrate ravulizumab is well tolerated and effective in clinical practice.[7]

## OBJECTIVE

- To assess long-term effectiveness and safety outcomes with ravulizumab among patients with gMG in routine clinical practice.

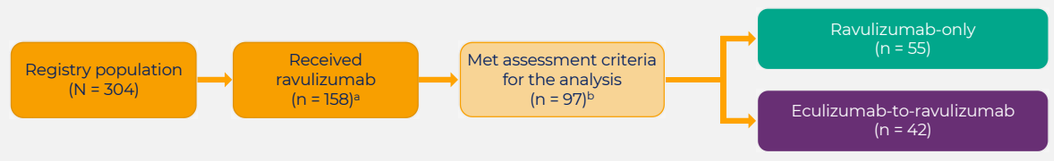
## CONCLUSIONS

- Mean Myasthenia Gravis Activities of Daily Living (MG-ADL) scores improved, and the proportion of patients with minimal symptom expression (MSE) and lower Myasthenia Gravis Foundation of America (MGFA) clinical classification increased during ravulizumab treatment compared with the period before C5IT initiation.
  - Among patients who transitioned from eculizumab to ravulizumab, the largest improvements were observed during eculizumab treatment, with improvements sustained during ravulizumab treatment.
- The safety profile of ravulizumab was consistent with prior analyses,[7,8] and no meningococcal infections were reported.
- These results demonstrate the sustained effectiveness and safety of ravulizumab in patients with gMG in routine clinical practice.

## METHODS

- Data were collected from patients enrolled in the MG SPOTLIGHT Registry from December 2019 through the data cutoff of October 2024.
- For this analysis, eligible patients were aged ≥ 18 years at enrollment and received ravulizumab, either alone (ravulizumab-only subgroup) or after transitioning from eculizumab (eculizumab-to-ravulizumab subgroup) (**Figure 1**).
  - Patients were required to have ≥ 1 MG-ADL assessment prior to C5IT initiation, and ≥ 1 MG-ADL assessment post-ravulizumab initiation; patients transitioned from eculizumab were included only if they had ≥ 1 additional MG-ADL assessment during eculizumab treatment.
- Outcomes assessed:
  - MG-ADL total scores
  - MGFA clinical classification
  - MSE, defined as MG-ADL total score ≤ 1
  - Safety

**Figure 1. Study population**




[a]Patients were excluded if they did not meet Registry inclusion criteria, had incorrect C5IT entry data, were missing treatment log records, or had received eculizumab only at the time of database lock. [b]Patients were required to have ≥ 1 MG-ADL assessment prior to C5IT initiation and ≥ 1 MG-ADL assessment post-ravulizumab initiation; patients who transitioned from eculizumab (eculizumab-to-ravulizumab subgroup) were included only if they had ≥ 1 additional MG-ADL assessment during eculizumab treatment.
C5IT, complement component 5 inhibitor therapy, MG-ADL, Myasthenia Gravis Activities of Daily Living.

## RESULTS AND INTERPRETATION

### Patient characteristics

- Of the 304 patients enrolled in the MG SPOTLIGHT Registry, 97 met inclusion criteria for this analysis (**Figure 1**): ravulizumab-only subgroup (n = 55) and eculizumab-to-ravulizumab subgroup (n = 42) (**Table 1**).
  - Most patients were male and White (86.6%).

**Table 1. Patient demographics and characteristics**

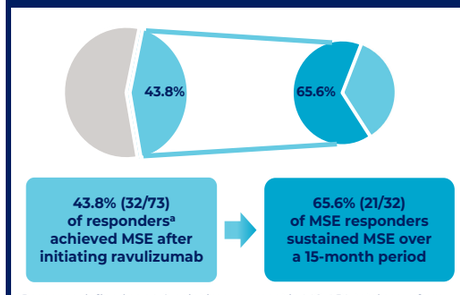
| Characteristic | All patients treated with ravulizumab (n = 97) | Ravulizumab-only (n = 55)[a] | Eculizumab-to-ravulizumab (n = 42) |
|---|---|---|---|
| **Sex, n (%)** | | | |
| Female | 36 (37.1) | 21 (38.2) | 15 (35.7) |
| Male | 61 (62.9) | 34 (61.8) | 27 (64.3) |
| **Ethnicity, n (%)** | | | |
| Hispanic or Latino | 4 (4.1) | 3 (5.5) | 1 (2.4) |
| Not Hispanic or Latino | 91 (93.8) | 52 (94.5) | 39 (92.9) |
| Unknown or not reported | 2 (2.1) | 0 | 2 (4.8) |
| **Country, n (%)** | | | |
| Canada | 5 (5.2) | 3 (5.5) | 2 (4.8) |
| United States | 92 (94.8) | 52 (94.5) | 40 (95.2) |
| **Age at MG diagnosis, mean (SD), y** | 59.3 (16.1)[b] | 59.1 (16.1)[c] | 59.5 (16.3) |
| **Ravulizumab treatment duration, median (range), y** | 1.2 (0.1-4.2) | 1.1 (0.1-4.2) | 1.3 (0.1-2.0) |

[a]One patient in this group subsequently transitioned from ravulizumab to eculizumab; only outcomes assessed before switching to eculizumab were considered for this analysis. [b]n = 95. [c]n = 53.
MG, myasthenia gravis.

### Effectiveness

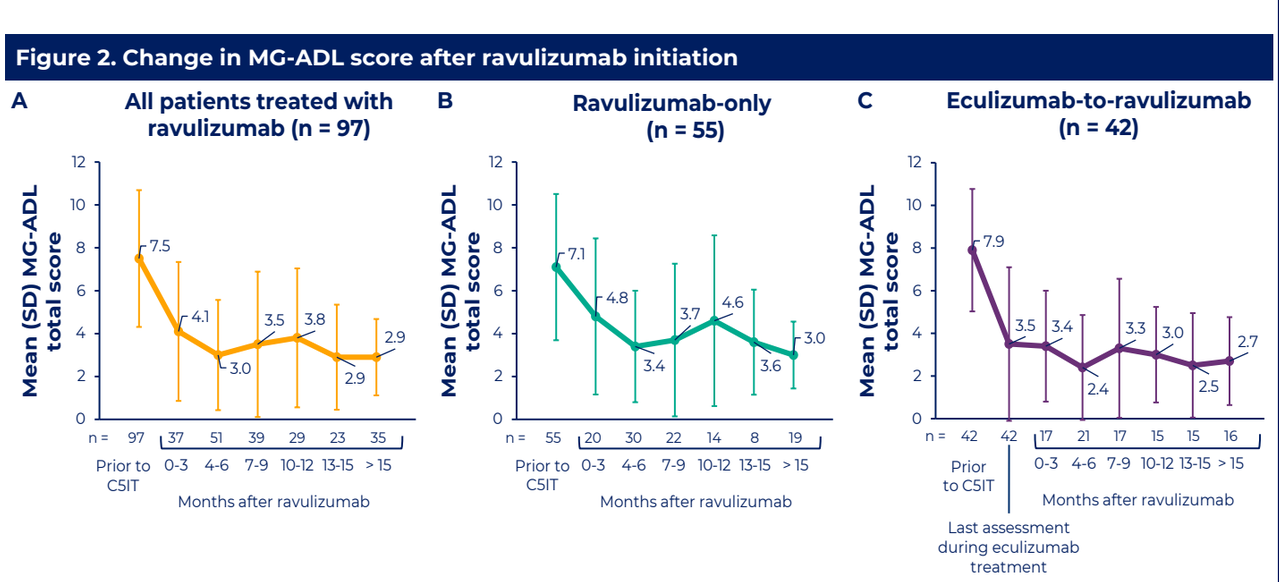
- Among these patients, mean MG-ADL scores improved from prior to C5IT during ravulizumab treatment (**Figure 2**).
  - Among the ravulizumab-only subgroup, improvements were observed at the first assessment after ravulizumab initiation and were sustained throughout treatment.
  - Among the eculizumab-to-ravulizumab subgroup, improvements observed during eculizumab treatment were sustained after switching to ravulizumab.
- Among all patients with a ≥ 2-point improvement in MG-ADL total score from baseline (n = 73), 32 (43.8%) experienced MSE at any point within 15 months after ravulizumab initiation; of these 32 patients with MSE, 21 (65.6%) had sustained MSE over the entire 15-month period (**Box 1**).
- After ravulizumab treatment, the proportion of patients with MGFA class 0-II increased (**Figure 3**).
  - Among the ravulizumab-only subgroup, the proportion of patients with MGFA class 0-II increased from prior to C5IT to the time of first assessment after ravulizumab initiation and generally increased over time.
  - Among the eculizumab-to-ravulizumab subgroup, improvements in MGFA classification were noted during eculizumab treatment and remained stable after transition to ravulizumab.

**Box 1. MSE with ravulizumab treatment**



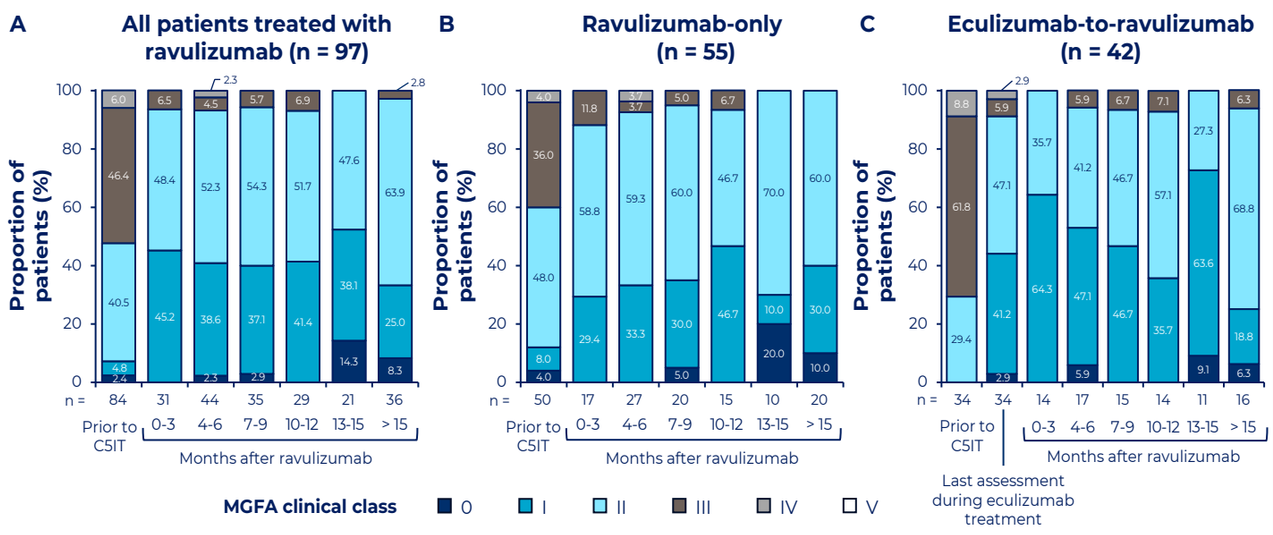

[a]Response defined as a ≥ 2-point improvement in MG-ADL total score from baseline.
MG-ADL, Myasthenia Gravis Activities of Daily Living; MSE, minimal symptom expression (MG-ADL total score ≤ 1).

**Figure 2. Change in MG-ADL score after ravulizumab initiation**




Data are shown for last assessment within each 3-month period.
C5IT, complement component 5 inhibitor therapy; MG-ADL, Myasthenia Gravis Activities of Daily Living.

**Figure 3. Change in MGFA clinical classification after ravulizumab initiation**




Data are shown for last assessment within each 3-month period. Percentages may not equal 100 due to rounding.
C5IT, complement component 5 inhibitor therapy; MGFA, Myasthenia Gravis Foundation of America.

### Safety

- No new safety signals were observed in patients treated with ravulizumab (**Table 2**).
  - Serious adverse events (SAEs) were experienced by 22/97 (22.7%) patients.
  - No SAEs were related to ravulizumab or eculizumab.
  - No meningococcal infections were reported.
  - Among the 4 deaths in the study population, none were related to ravulizumab or eculizumab.

### Study limitations

- This descriptive study had no adjustment for covariates.
- Due to the small sample size and the nature of real-world data collection, data do not represent longitudinal change in the same group of patients over time but rather reflect cross-sectional analyses at varied time points.

**Table 2. SAEs among all patients treated with ravulizumab (n = 97)**

| System organ class of reported SAE[a] | Events, n | Death, n |
|---|---|---|
| **Total SAE** | 46 | 4 |
| **Infections and infestations** | 11 | 0 |
| Coronavirus infections | 3 | 0 |
| Sepsis, bacteremia, viremia, and fungemia NEC | 2[b] | 0 |
| Urinary tract infections | 3 | 0 |
| **Cardiac disorders** | 10 | 4 |
| **Gastrointestinal disorders** | 2 | 0 |
| **Musculoskeletal and connective tissue disorders** | 4 | 0 |
| **Nervous system disorders** | 8 | 0 |
| **Respiratory, thoracic, and mediastinal disorders** | 4 | 0 |
| **Vascular disorders** | 1[c] | 0 |

[a]Data shown for system organ classes with ≥ 2 events. [b]Both events (sepsis and septic shock) occurred within the same patient. [c]Two cases had been reported as of the October 2024 data cutoff; however, one case was subsequently confirmed as a non-SAE by the site.
NEC, not elsewhere classified; SAE, serious adverse event.

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**1.** Conti-Fine BM, et al. J Clin Invest. 2006;116(11):2843-2854. **2.** Hehir MK, et al. Neurol Clin. 2018;36(2):253-260. **3.** San PP, et al. Front Neurol. 2023;14:1277596. **4.** Soliris (eculizumab). Prescribing information. Alexion Pharmaceuticals, Inc.; 2025. **5.** Ultomiris (ravulizumab-cwvz). Prescribing information. Alexion Pharmaceuticals, Inc.; 2024. **6.** NIH. Accessed Mar 24, 2025. https://clinicaltrials.gov/study/NCT04202341. **7.** Narayanaswami P, et al. Poster presented at: EAN; Jun 29–Jul 2, 2024; Helsinki, Finland. **8.** Meisel A, et al. J Neurol. 2023;270(8):3862-3875.

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**Poster**

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