# Patient characteristics associated with treatment preference for generalized myasthenia gravis (gMG): a multivariate analysis

Karen S. Yee,[1]* Christine Poulos,[2] Cooper Bussberg,[2] Kelley Myers[2]

[1]Alexion, AstraZeneca Rare Disease, Boston, MA, USA; [2]RTI Health Solutions, Research Triangle Park, NC, USA

*Presenting author

## INTRODUCTION

- gMG is a rare, chronic autoimmune condition that is characterized by fatigable skeletal muscle weakness that worsens after muscle use.[1,2]

- Approximately 85% of patients with gMG have autoantibodies against acetylcholine receptors (AChR).[3] Anti-AChR antibodies activate the complement system, which mediates the damage to the neuromuscular junction underlying anti-AChR antibody-positive (AChR-Ab+) gMG pathogenesis.[4]

- Therapeutic approaches differ among the available treatments for AChR-Ab+ gMG.[5] A clear understanding of patients' treatment priorities is needed to identify key unmet medical needs, aid in determining the value of new therapies, and inform clinical benefit–risk decision-making[6,7]; however, quantitative patient-centered data remain limited regarding treatment preferences in the United States.

## OBJECTIVE

- To identify characteristics of patients with gMG that were associated with a higher likelihood of choosing a ravulizumab-like profile over profiles similar to gMG therapies currently available.

## CONCLUSIONS

- Patients with gMG rated the ravulizumab-like profile as the most preferred treatment profile in each of the 3 scenarios described.
- Several characteristics were associated with a higher likelihood of selecting a ravulizumab-like profile, including not living with children, having a gMG diagnosis for < 3 years, having insurance other than Medicare, not having anxiety, and lack of experience with regular injections.
- These findings provide insight into which treatment attributes are considered important to patients with gMG and can help to inform shared decision-making when selecting gMG therapies.

## METHODS

- This web-based survey was conducted in adults who were located in the United States who self-reported a physician diagnosis of AChR-Ab+ gMG.
- Two object–case, best–worst scaling (BWS) exercises were used to evaluate treatment preferences.
  - The first BWS exercise assessed preferences across 5 different unlabeled treatment profiles similar to available gMG therapies: eculizumab, efgartigimod intravenous, ravulizumab, zilucoplan, and efgartigimod subcutaneous.
  - The second BWS exercise obtained preferences for the individual attributes used to define the treatment profiles: mode of administration, dosing frequency, consistent disease control, and required meningococcal vaccination.

- Profile scenarios were defined by mode of administration and dosing frequency only (Series 1), followed by the addition of consistent disease control and meningococcal vaccination requirements (Series 2 and 3).
- Self-reported characteristics of respondents who preferred a ravulizumab-like profile were evaluated via multivariate logistic regression with clinical and sociodemographic characteristics as variables.
- Estimated coefficients are reported as odds ratios, indicating the association between patient characteristic covariates and the likelihood of choosing a ravulizumab-like profile.

## RESULTS AND INTERPRETATION

### Patient characteristics

- A total of 153 respondents with AChR-Ab+ gMG completed the survey, with a mean (SD) age of 49 (14.9) years.
- The majority of respondents were female (76.5%), White (84.3%), and had a 4-year college degree or higher (54.2%; **Table 1**). The average time since gMG diagnosis was 9.2 (SD 8.5; range, 1-44) years, and the mean Myasthenia Gravis Activities of Daily Living total score was 8.0 (SD 3.9; range, 0-17).

### Table 1. Patient demographics and clinical characteristics

| Characteristic | Respondents (N = 153) |
|---|---|
| Age, mean (SD), years | 49.0 (14.9) |
| Gender identity, n (%) | |
| Female | 117 (76.5) |
| Male | 33 (21.6) |
| Other responses[a] | 3 (2.0) |
| Race,[b] n (%) | |
| Black or African American | 18 (11.8) |
| White | 129 (84.3) |
| Highest level of education, n (%) | |
| High school or equivalent | 16 (10.5) |
| Some college, no degree | 27 (17.6) |
| Technical school | 7 (4.6) |
| Associate's degree | 20 (13.1) |
| 4-year college degree or higher | 83 (54.2) |
| Employment status, n (%) | |
| Employed full time | 42 (27.5) |
| Employed part time | 14 (9.2) |
| Self-employed | 6 (3.9) |
| Homemaker | 3 (2.0) |
| Student | 3 (2.0) |
| Unemployed | 3 (2.0) |
| Retired | 27 (17.6) |
| Disabled/unable to work | 55 (35.9) |
| MG-ADL score, mean (SD) | 8.0 (3.9) |
| Time since gMG diagnosis, n (%) | |
| ≤ 3 years | 41 (26.8) |
| > 3 years | 112 (73.2) |
| Previous treatment experience, n (%) | |
| C5 inhibitors | 45 (29.4) |
| FcRn inhibitors | 37 (24.2) |
| Regular injections or infusions | 41 (26.8) |

[a]Other responses included "nonbinary" and "a gender identity not listed here." [b]Respondents were able to select more than one response. Only the 2 most common responses are listed, and the table does not show all response options selected by survey respondents, which also included the following: Alaska Native, American Indian, or Native American; Asian; Hispanic, Latin American, or Latinx; and a race or ethnicity not listed or prefer not to answer.
C5, complement component 5; FcRn, neonatal fragment crystallizable receptor; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living.

### Treatment profile ranking

- On average, respondents most preferred the ravulizumab-like profile across all 3 series of BWS questions (**Figure 1**).
  - When the treatment profiles were defined by mode and frequency of administration, the ravulizumab-like profile was most preferred by 34.6% of respondents compared with 10.5%-22.2% across the other 4 profiles.
  - With the addition of whether the treatment had consistent disease control, the ravulizumab-like profile remained the most preferred treatment profile.
  - In the third series, which had profiles defined by mode and frequency of administration, disease control, and meningococcal vaccination requirement, the ravulizumab-like profile remained the most preferred treatment profile (38.6% vs 5.2%-28.8%).
    ◦ Patients preferring the ravulizumab-like profile in series 3 were primarily female (80%) and aged < 65 years old (83%).

### Figure 1. Treatment profile preferences across series
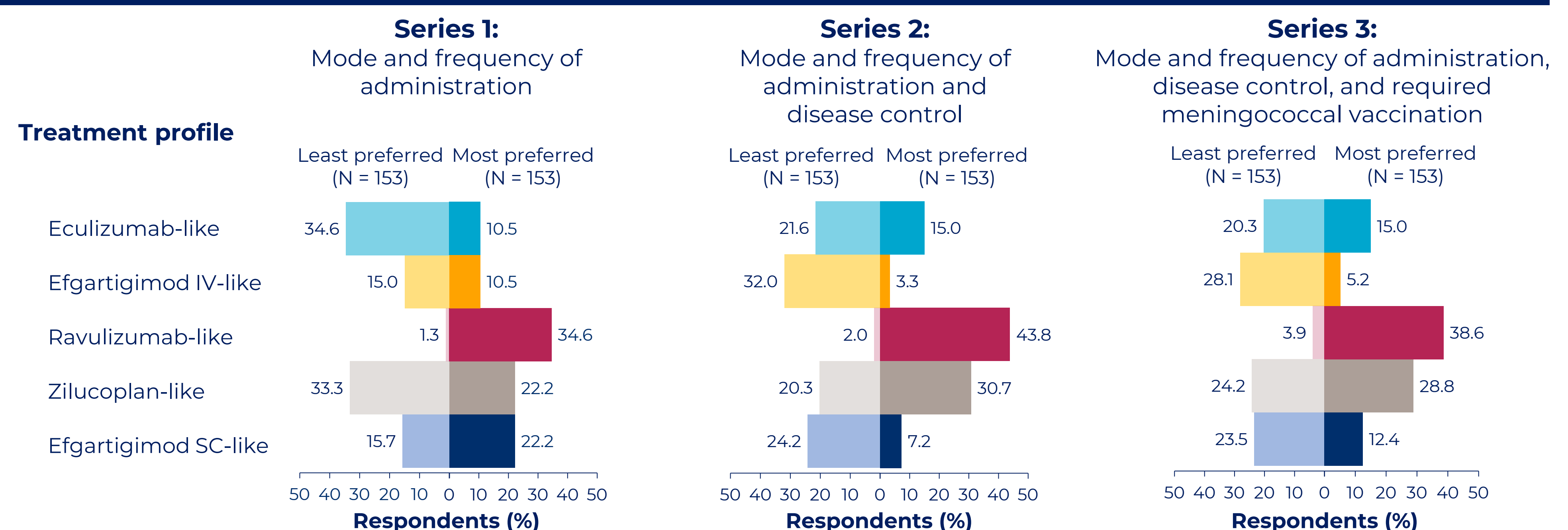


Percentages may not add up to 100% due to rounding.
IV, intravenous; SC, subcutaneous.

### Multivariate analysis

- Characteristics significantly associated with a higher likelihood of selecting a ravulizumab-like profile (**Figure 2**) were:
  - Not living with children
  - Having a gMG diagnosis for < 3 years
  - Having insurance other than Medicare
  - Not having anxiety
  - Lack of experience with regular injections

### Figure 2. Odds ratios of predictors for selecting a ravulizumab-like profile

| Condition | Odds ratio (95% CI) | P value | |
|---|---|---|---|
| Not living with children | 3.2 (1.3, 7.4) | < 0.05 | |
| gMG diagnosis for < 3 years | 3.0 (1.3, 6.8) | < 0.05 | |
| Insurance other than Medicare | 2.9 (1.3, 6.4) | < 0.05 | |
| Not having anxiety | 2.6 (1.2, 5.5) | < 0.05 | |
| Lack of experience with regular injections | 2.6 (1.1, 6.0) | < 0.05 | |

gMG, generalized myasthenia gravis.

**References**
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. Vanoli F, et al. Expert Opin Biol Ther. 2023;23(3):235-241. 4. San PP, et al. Front Neurol. 2023;14:1277596. 5. Narayanaswami P, et al. Neurology. 2021;96(3):114-122. 6. Jimenez-Moreno AC, et al. Patient. 2021;14(5):601-612. 7. Ho K-A, et al. Pharmaceut Med. 2024;38(1):55-62.

Please scan this quick response (QR) code with your smartphone camera or app to obtain a copy of this poster.
Copies of this poster obtained through this QR code are for personal use only and may not be reproduced without permission from the authors of this poster.

Poster

A portion of these data were previously presented at the 2024 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, March 3-6, 2024, Orlando, FL, USA

Poster presented at the 2024 International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Europe Congress, November 17-20, 2024, Barcelona, Spain

Corresponding author email address: karen.yee@alexion.com