Patient preferences for myasthenia gravis treatments: a discrete-choice experiment

AAN 2024, Denver, CO, USA; April 13–18, 2024

Carol Mansfield[1], Kerrie-Anne Ho[2], Anna Pierce[1], Caroline Vass[3], Xiaoying Liu[1], Babak Boroojerdi[4], Sandeep Kiri[2]

[1]RTI Health Solutions, Research Triangle Park, NC, United States; [2]UCB Pharma, Slough, United Kingdom; [3]RTI Health Solutions, Manchester, United Kingdom; [4]UCB Pharma, Monheim, Germany

Background and objectives

- Little is known about how people living with gMG feel about different modes of treatment administration or the importance of this in their treatment decisions
- This study used a discrete-choice experiment (DCE) to elicit the preferences of people living with gMG for selected treatment features
  - DCE is a survey-based method increasingly used in healthcare research to quantify preferences for treatments
  - In a DCE, respondents trade off different treatment features (attributes) when choosing their preferred alternative from a hypothetical choice set, indirectly revealing their preferences
- The preference information was used:
  - To explore the relative importance of each attribute in patients' choices for gMG treatments
  - To estimate the probability a patient would choose one hypothetical treatment profile over another

Design

- An online DCE survey was administered to adults living with gMG in the US, UK, and Germany who have or have experienced uncontrolled gMG (i.e., need(ed) frequent treatment changes or adjustments)
  - All participants provided informed consent
- Respondents chose between experimentally designed pairs of hypothetical treatments in 8 choice sets.
- Each treatment was defined by 6 attributes (**Table 1** and **Figure 1**)
  - Attributes were identified through literature reviews, reviews of labels of existing treatments, and consultation with patient advisors and medical experts
  - The survey was pretested with a sample of adults with gMG in the US (n = 10), UK (n = 5), and Germany (n = 5) using individual, semistructured "think-aloud" interviews to examine understanding of the survey prior to online administration
- The experimental design comprised 48 choice sets
  - The design was split into 6 blocks of 8 choice set questions. Respondents were randomly assigned to 1 of these blocks in which the choice sets were also randomized
- Descriptive statistics were used to characterize the study population in terms of key sociodemographic and economic variables
- Mixed logit model estimates were used to:
  - Calculate the conditional relative attribute importance (CRAI), which is the difference between preference weight of the most-preferred and least-preferred levels of each attribute
  - Predict the probability respondents would select one hypothetical treatment profile over another using clinically relevant combinations of attributes and calculating the difference in estimated expected utility

Results

- A total of 200 respondents (US, n=150; UK, n=25; Germany, n=25) completed the survey
  - Key demographic characteristics are presented in **Figure 2**
- Estimated preference weights for attributes were consistent with the natural ordering of the levels (better levels were preferred to worse levels) (**Figure 3**)
  - Self-administration at home was preferred to administration by a doctor or nurse in a healthcare facility.
  - Injections for up to 30 seconds were preferred to IV administrations
  - 1- or 2-weeks onset of action was preferred to 4 weeks, 8 weeks, or more
  - Lower risks of ISR were preferred.
  - Respondents were indifferent between 6 doses over 6 weeks and dosing once a week
- Respondents placed the most importance on changes in risk of ISR (from 40% to 0% [mild to moderate]) (**Figure 4**)
  - Changes in setting and administration mode were least important
- Model estimates suggest respondents are more likely to choose a treatment self-administered at home once daily through a prefilled syringe with 2 weeks until onset of action (65.2%) over a treatment administered once every 8 weeks through a 1- to 2-hour IV at a medical facility with 4 weeks until onset of action (34.8%) (**Figure 5**)

Table 1 Attribute levels for the discrete-choice experiment

Technical attribute label	Attribute levels
Administration setting	• Administered at home by you • Administered at your home by a doctor or nurse • Administered in a medical facility by a doctor or nurse
How you take the treatment and administration time	• Injection for up to 30 seconds • Infusion under the skin with a pump/device for up to 12 minutes • IV that takes 30 minutes • IV that takes 1-2 hours
How often you take the treatment	• Once a day • Once a week • 6 doses over 6 weeks (more cycles based on response) • Once every 8 weeks
How long on average until meaningful improvement in symptoms after starting treatment	• 1 week • 2 weeks • 4 weeks • 8 weeks or more
Annual risk of mild to moderate injection site reaction	• 0 out of 100 people (0%) • 3 out of 100 people (3%) • 10 out of 100 people (10%) • 40 out of 100 people (40%)
Annual risk of severe injection site reaction	• 0 out of 100 people (0%) • 1 out of 100 people (1%) • 7 out of 100 people (7%) • 10 out of 100 people (10%)

IV, intravenous.

Figure 1 Example discrete-choice experiment question

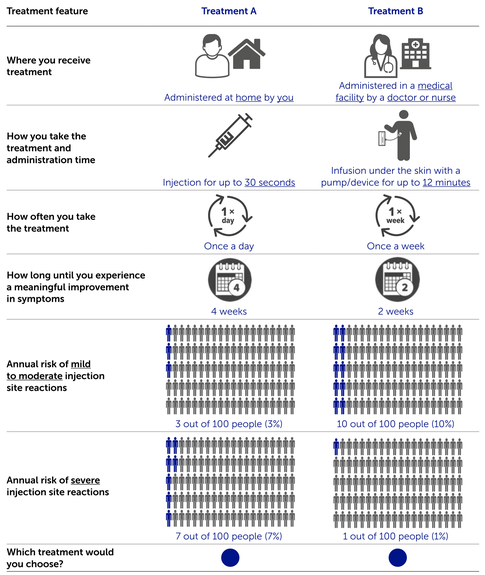
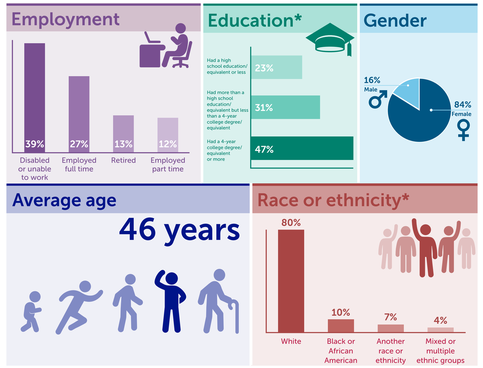
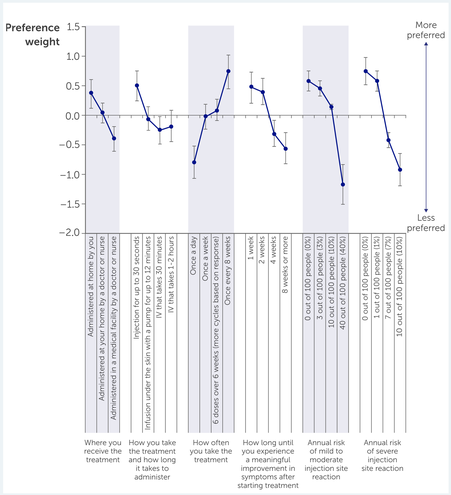


Figure 2 Participant demographics



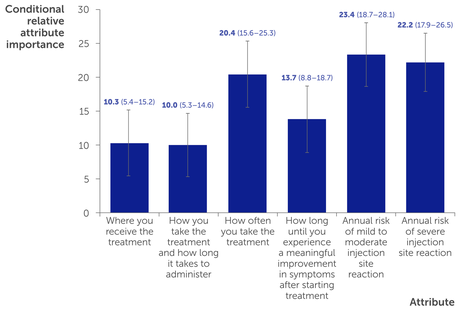
*Total may be greater than 100% due to rounding.

Figure 3 Preference weights estimated from random parameters logit model



The parameter estimates are the preference weights. Levels with higher preference weights are more preferred than the levels of the same attribute with lower preference weights. However, levels across different attributes are not comparable directly. Error bars denote 95% CI of point estimate.

Figure 4 Conditional relative importance (CRAI) of each attribute



CRAI shows the relative importance of each attribute as the percentage of importance across all attributes. It reflects the relative importance of each attribute within the range of levels of each attribute included in the study and relative to the other attributes included in the study. Error bars denote 95% CI of point estimates, which are also displayed as numbers in the figure.

Conclusions

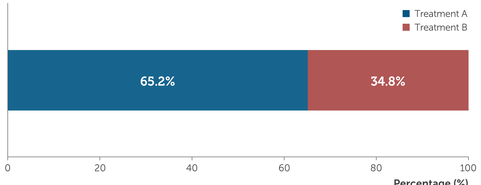
People living with gMG preferred gMG treatments with lower ISR risk and less frequent administration. They also preferred treatments with shorter time until onset of action, that were self-administered at home, that were a 30-second injection over an infusion pump or IV infusion, and that were taken once every 8 weeks over more frequent administrations

With the changing landscape in gMG treatments, these results demonstrate that people living with gMG are willing to trade off among treatment attributes, suggesting an important role for patient preferences in treatment selection

The finding of this research highlights the need for healthcare professionals and people living with gMG to share decision-making based on clinical judgment and individual preferences

Figure 5 Predicted probability that the average respondent would select each profile based on random parameters logit estimates

Attribute	Treatment A	Treatment B
Administration setting – where you take the treatment	Administered at home by you	Administered in medical facility by a doctor or nurse
How you take the treatment and administration time	Injection for up to 30 seconds	IV takes 1–2 hours
How often you take the treatment	Once a day	Once every 8 weeks
How long on average until meaningful improvement in symptoms after starting treatment	2 weeks	4 weeks
Annual risk of mild to moderate injection site reaction	3%	3%
Annual risk of severe injection site reaction	1%	1%



Contact information: Sandeep Kiri, Global Head, Health Economics and HTA Evidence, UCB Pharma Limited, 208 Bath Road, Slough, Berkshire SL1 3WE, Email: sandeep.kiri@ucb.com

Acknowledgments: This study was funded by UCB Pharma. The authors acknowledge Marilisa Valtazanou and Nadine Hammond of Ogilvy Health, London, UK, for editorial support, which was funded by UCB Pharma. The authors acknowledge Veronica Porkess, PhD, of UCB Pharma, Slough, UK, for publication coordination. The authors thank the patients and their caregivers, in addition to the investigators and their teams who contributed to this study.

Author disclosures: C. Mansfield, C. Vass, and X. Liu are employees of RTI Health Solutions, which received funding from UCB to conduct the study. Anna Pierce was an employee of RTI Health Solutions at the time the research was conducted.

Abbreviations: CI, confidence intervals; gMG, generalized myasthenia gravis; ISR, injection site reaction; IV, intravenous; UK, United Kingdom; US, United States.

References: 1. Hehir MK, Silvestri NJ. Generalized myasthenia gravis: classification, clinical presentation, natural history, and epidemiology. Neurol Clin. 2018 May 1;36(2):253-60. 2. Souto EB, Lima B, Campos JR, Martins-Gomes C, Souto SB, Silva AM. Myasthenia gravis: State of the art and new therapeutic strategies. J Neuroimmunol. 2019 Dec 15;337:577080. 3. Swait J, Louviere J. The role of the scale parameter in the estimation and comparison of multinomial logit models. J Market Res. 1993 Aug;30(3):305-14. doi:10.2307/3172883.

Please use this QR code to download a PDF of the poster.

Table 1 Attribute levels for the discrete-choice experiment

Technical attribute label	Attribute levels
Administration setting	• Administered at home by you • Administered at your home by a doctor or nurse • Administered in a medical facility by a doctor or nurse
How you take the treatment and administration time	• Injection for up to 30 seconds • Infusion under the skin with a pump/device for up to 12 minutes • IV that takes 30 minutes • IV that takes 1-2 hours
How often you take the treatment	• Once a day • Once a week • 6 doses over 6 weeks (more cycles based on response) • Once every 8 weeks
How long on average until meaningful improvement in symptoms after starting treatment	• 1 week • 2 weeks • 4 weeks • 8 weeks or more
Annual risk of mild to moderate injection site reaction	• 0 out of 100 people (0%) • 3 out of 100 people (3%) • 10 out of 100 people (10%) • 40 out of 100 people (40%)
Annual risk of severe injection site reaction	• 0 out of 100 people (0%) • 1 out of 100 people (1%) • 7 out of 100 people (7%) • 10 out of 100 people (10%)

IV, intravenous.

Figure 1 Example discrete-choice experiment question

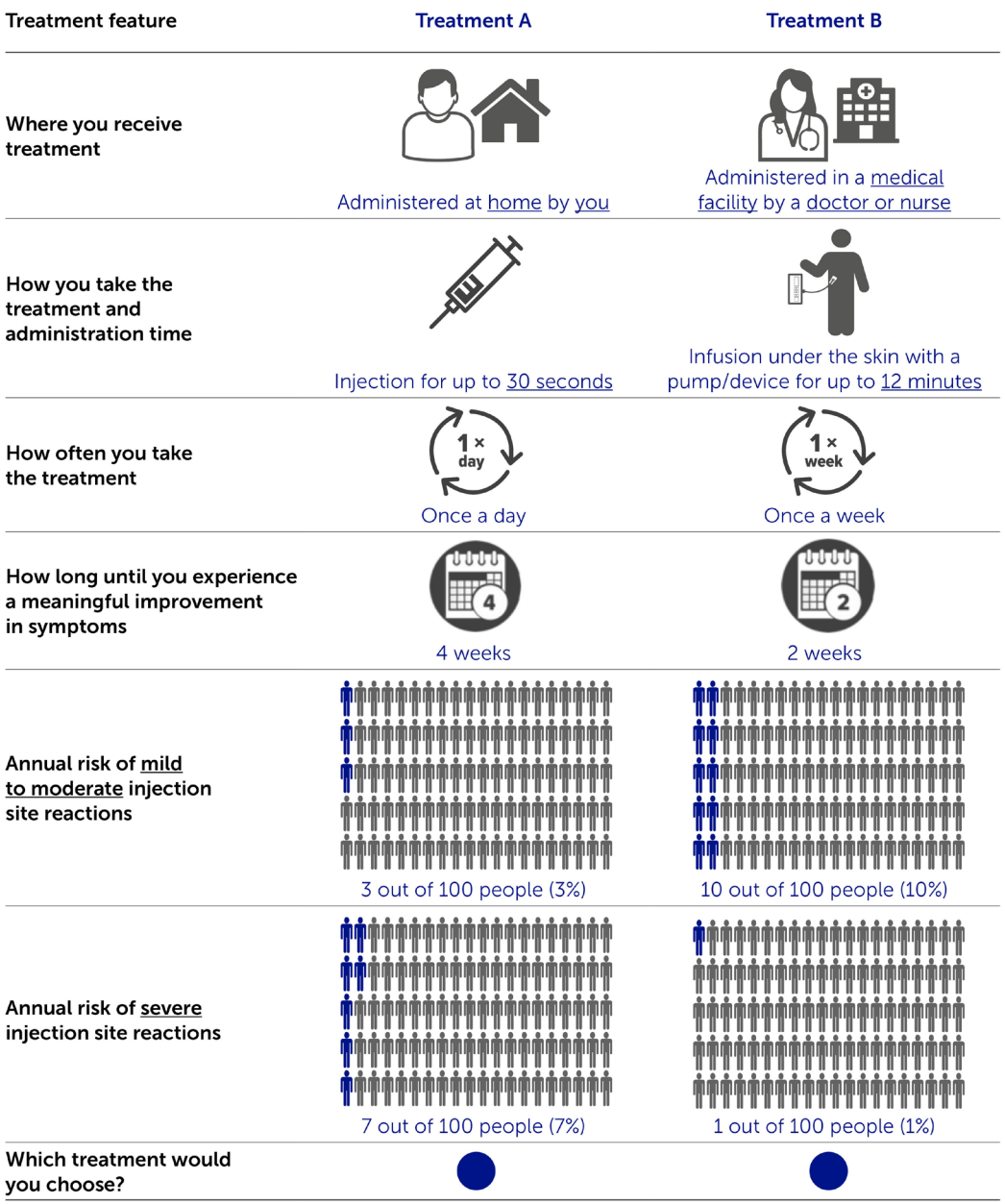
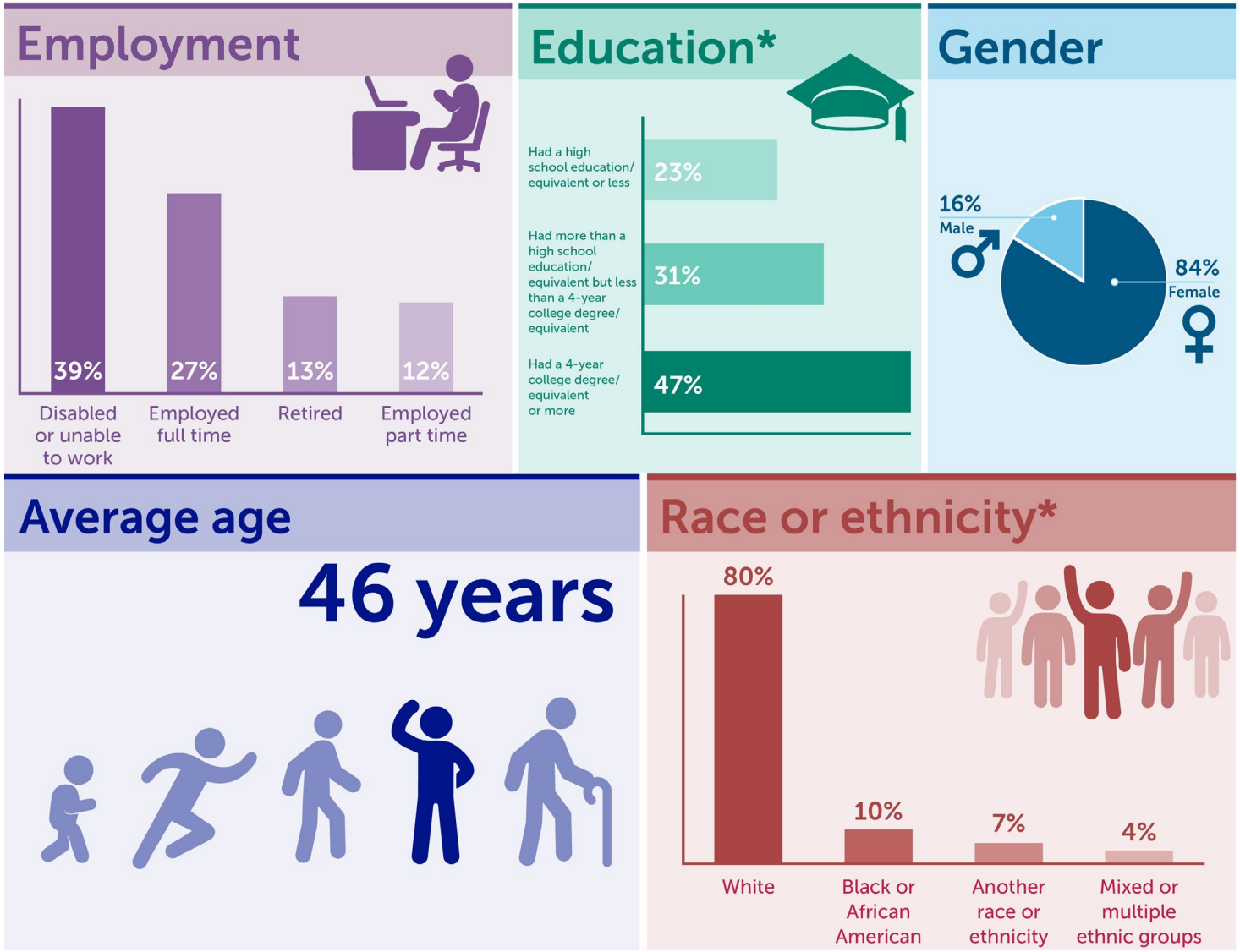
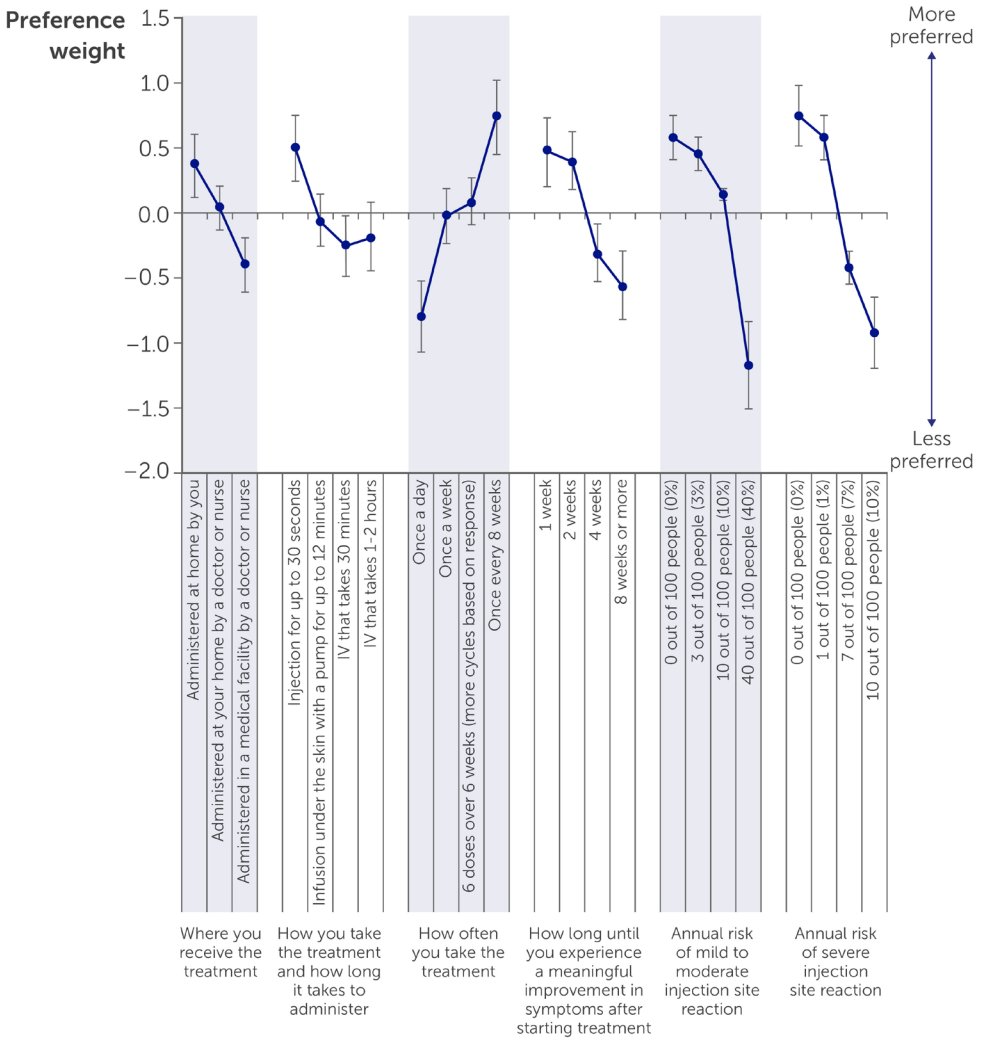


Figure 2 Participant demographics



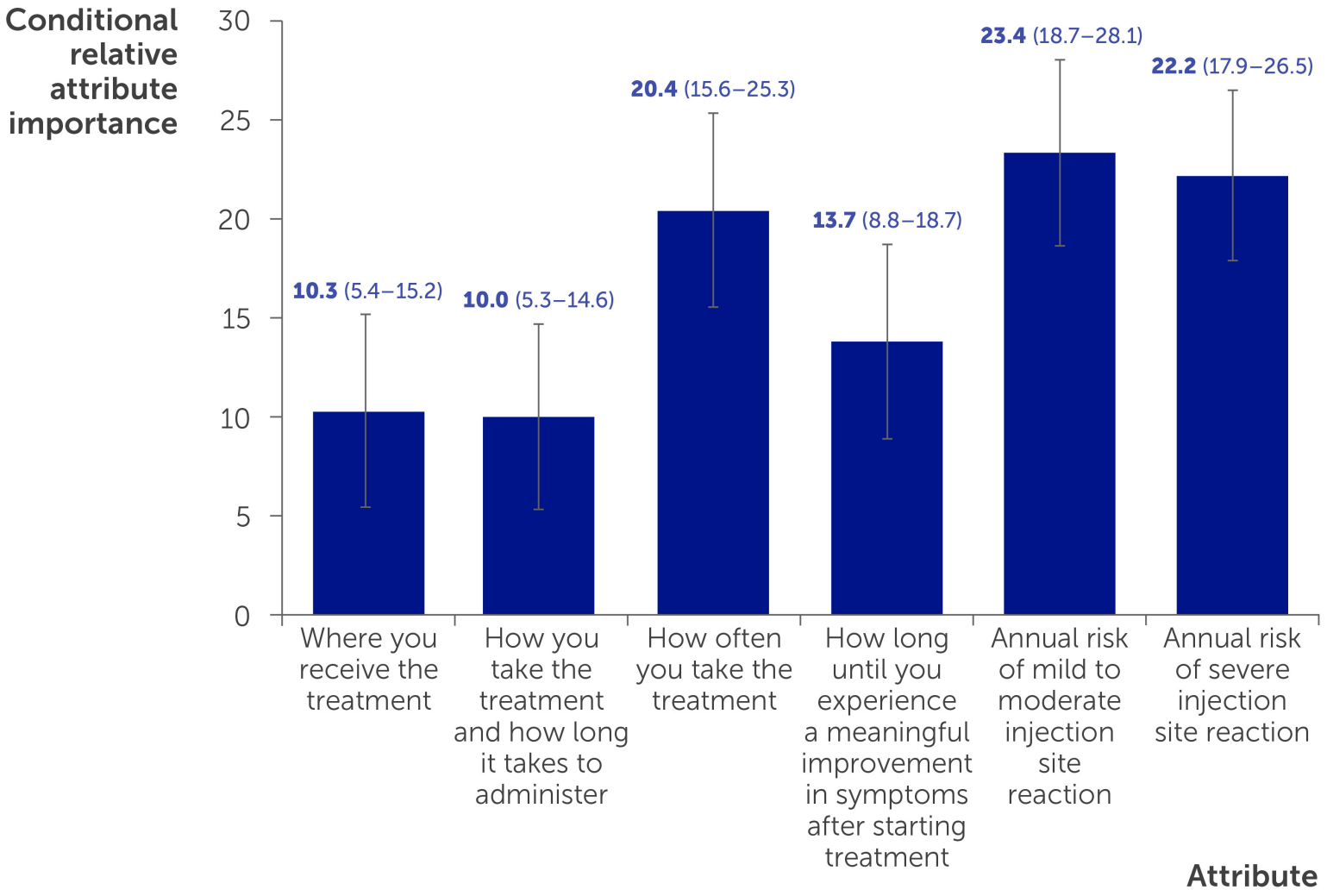
*Total may be greater than 100% due to rounding.

Figure 3 Preference weights estimated from random parameters logit model



The parameter estimates are the preference weights. Levels with bigger preference weights are more preferred than the levels of the same attribute with lower preference weights. However, levels across different attributes are not comparable directly. Error bars denote 95% CI of point estimate.
CI, confidence interval; IV, intravenous.

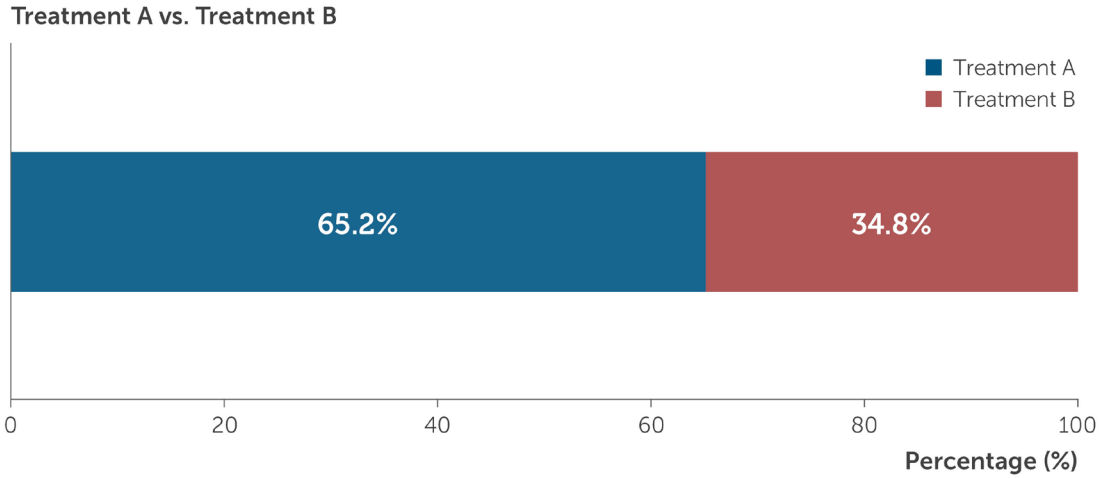
Figure 4 Conditional relative importance (CRAI) of each attribute



CRAI shows the relative importance of each attribute as the percentage of importance across all attributes. It reflects the relative importance of each attribute, given the range of levels of each attribute included in the study and relative to the other attributes included in the study. Error bars denote 95% CI of point estimates, which are also displayed as numbers in the figure.
CI, confidence interval; CRAI, conditional relative importance.

Figure 5 Predicted probability that the average respondent would select each profile based on random parameters logit estimates

Attribute	Treatment A	Treatment B
Administration setting – where you take the treatment	Administered at home by you	Administered in medical facility by a doctor or nurse
How you take the treatment and administration time	Injection for up to 30 seconds	IV takes 1–2 hours
How often you take the treatment	Once a day	Once every 8 weeks
How long on average until meaningful improvement in symptoms after starting treatment	2 weeks	4 weeks
Annual risk of mild to moderate injection site reaction	3%	3%
Annual risk of severe injection site reaction	1%	1%



IV, intravenous.

Summary and conclusions

People living with gMG preferred gMG treatments with lower ISR risk and less frequent administration. They also preferred treatments with shorter time until onset of action, that were self-administered at home, that were a 30-second injection over an infusion pump or IV infusion, and that were taken once every 8 weeks over more frequent administrations

With the changing landscape in gMG treatments, these results demonstrate that people living with gMG are willing to trade off among treatment attributes, suggesting an important role for patient preferences in treatment selection

The finding of this research highlights the need for healthcare professionals and people living with gMG to share decision-making based on clinical judgment and individual preferences

gMG, generalized myasthenia gravis; IV, intravenous; ISR, injection site reaction.