

Collecting Exploratory Patient Preference Data Using an Efficient Approach: An Example in Rare Diseases

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Background

- Several methods exist to collect patient preference information, including qualitative interviews, focus groups, and surveys, each of which provides a different perspective and has benefits and shortcomings.
- Stated preference elicitation techniques such as discrete choice experiments (DCEs) can be included in surveys as a robust way to collect information about health preferences to inform drug development and healthcare decision-making. Such studies are traditionally time and resource intensive, often taking over 12 months to complete.
- Employing less time- and resource-intensive exploratory approaches to elicit preference data can inform internal strategy, guide early decisions, and/or generate foundational evidence for larger studies more efficiently.
- Combining qualitative and quantitative methods can provide additional insights that each method alone will not provide.

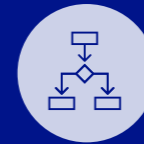
Objectives

- The objective of this study was to demonstrate a less time- and resource-intensive mixed-methods approach to generate exploratory preference data for the treatment of a rare disease, generalized myasthenia gravis (gMG).

Methods

- We conducted a study in the United States (US) (n = 12) and Germany (n = 6) that combined a brief, online patient preference survey with focus groups that expanded on the information collected in the survey. This study took 4 months to complete (see **Figure 1**).
- Participants first received preread materials personalized based on their responses to the screening questions. The materials included patient-friendly descriptions of the 6 treatment attributes, some of which were taken from previously conducted preference research, while others were newly identified. The preread materials also included a unique link to a brief online survey with a single risk threshold task. The threshold technique (TT) questions aimed to elicit patients' MAR of a single adverse event for the 2 treatment profiles presented.
- Following completion of the online survey, participants took part in qualitative focus groups.
 - Patients were asked open-ended questions about their treatment likes and dislikes and discussed reactions to the 6 attributes described in the preread document
 - Patients were asked to complete up to 3 TT tasks, one of which repeated the TT task from the online survey.
 - Qualitative research provides insight into patients' experiences and preferences captured in their own words. The focus group format allows the interviewer to follow up on interesting points that patients raise and understand not just the choice a patient makes but also why they made that choice.

Summary and Conclusions



- The study used a novel mixed-methods approach by employing both quantitative and qualitative methods to elicit preferences from focus groups of patients with a rare disease, gMG.
- The study combined a brief, online patient preference survey with focus groups that expanded on information collected in the survey.
 - We elicited qualitative and quantitative feedback from people living with gMG on attributes associated with potential gMG treatments.
 - TT exercises within focus group discussions yielded insights into the benefits and risks that are most important to people living with gMG, providing more detailed feedback on their treatment priorities than would have been achieved with a purely qualitative or quantitative approach.
 - The discussion in the focus groups did not appear to heavily influence patients' treatment choices in the TT exercise but may have influenced participants' thoughts about their MAR or clarified the intended definition of the attributes included in the TT exercise.
 - By leveraging previous preference research and using a pragmatic mixed-methods design, our study provided results quickly and efficiently.



- The findings in this study could be further explored in a full DCE study. Such a study would allow for:
 - Estimation of preferences for features of gMG treatments
 - Calculation of the importance of each treatment feature relative to the others included in the study
 - Exploration of whether preferences vary with individuals' demographic and clinical characteristics
 - Prediction of the probability that a patient would choose a treatment defined by a specific set of attributes



- Patient preference information is valuable for incorporating the patient perspective into the drug development process and medical decision-making.
- However, it is not always feasible to implement robust preference studies within the decision-making contexts and timeframes.
- Circumstances such as internal decision-making, early clinical development decisions, and difficult recruitment (e.g., for rare diseases) can make robust preference studies difficult or unnecessary. More pragmatic approaches, such as the mixed-methods approach applied here, may provide sufficient preference information to fit the study's research needs.

Figure 1. Study Design

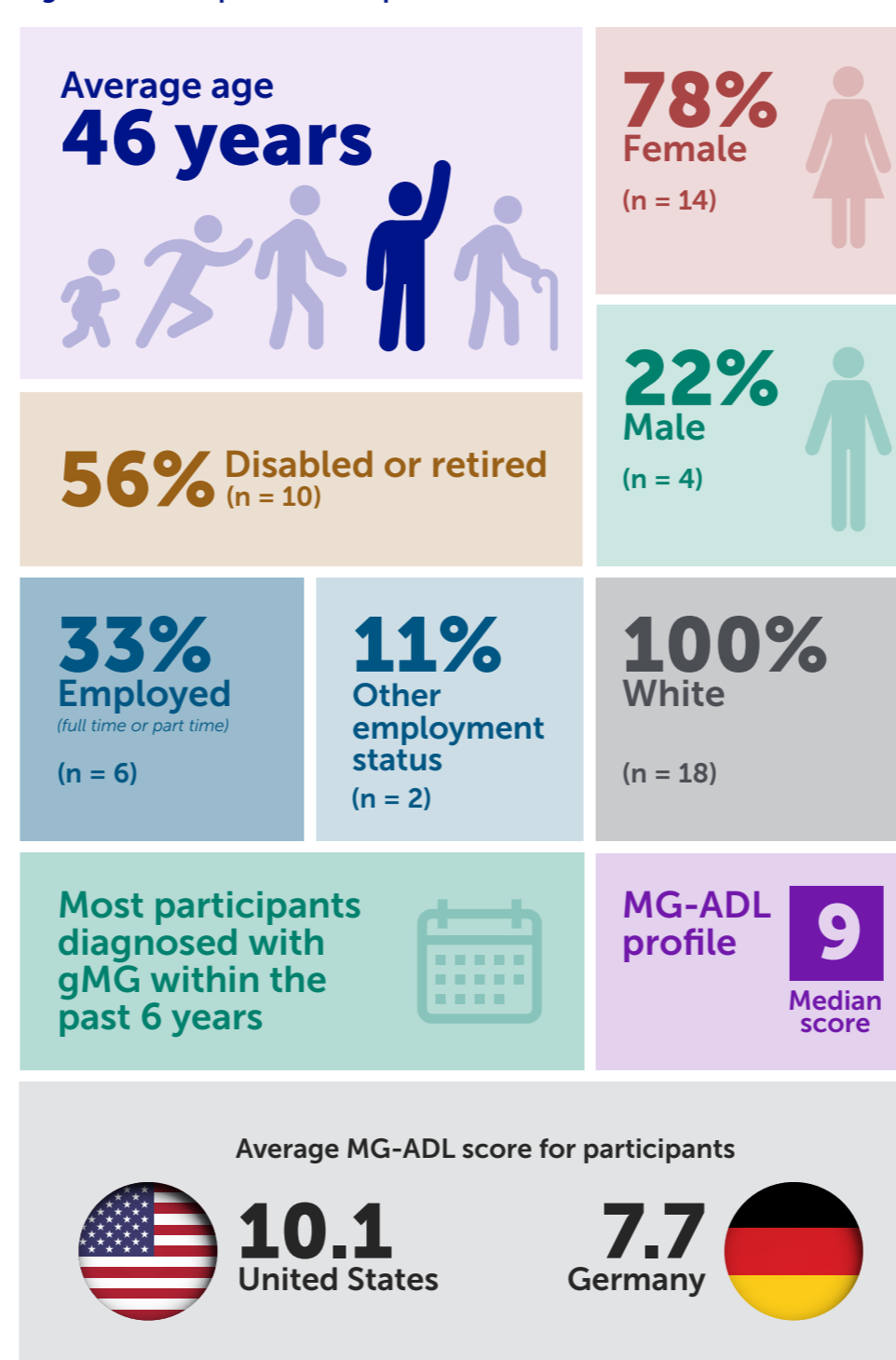


IRB = institutional review board; MAR = maximum acceptable risk; MG-ADL = Myasthenia Gravis–Activities of Daily Living.

Results

- The study elicited quantitative exploratory data on participants' (N = 18; **Figure 2**) risk tolerance, as well as qualitative data on treatment expectations and other important treatment attributes and trade-offs participants were willing to make.
- Focus group discussions provided insights into participants' choices in the TT tasks, confirmed that all the attributes were relevant, and helped clarify what was important about the attributes.
 - Participants expressed some concerns about gMG treatment and goals for gMG treatment beyond those reflected in the TT exercises, providing extra information that a survey alone would not provide.
 - Most participants in Germany, both male and female, spontaneously expressed concern regarding fertility when taking treatments for gMG.
 - Some US participants mentioned they would appreciate if there were more gMG treatment options developed for seronegative patients and patients with the newer antibodies.
- The attribute descriptions included in the preread were reviewed during the focus groups.
 - Patients provided insights into how they interpreted the attribute descriptions when they read the attribute descriptions on their own.
- The responses to the online TT questions matched the responses provided in the focus group in general.
 - Most participants (n = 15) chose the same hypothetical treatment for the first TT question in the patient focus group as they did in the preread survey.
 - The average MAR that participants stated during the focus groups was higher than the level stated in the preread survey for those participants who were asked to report their MAR in an open-ended question in the survey (i.e., participants who accepted the highest level of risk presented in the survey).
- The focus groups also allowed for the exploration of participants' thresholds for changes in 2 other attributes.
- A discussion about the TT questions identified attributes that were influencing participants' decisions and illuminated the importance of both the attribute that was subject to the TT questions and the other attributes that were kept constant.

Figure 2. Participants' Descriptive Statistics (N = 18)



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