Pharmaceutical Medicine
https://doi.org/10.1007/s40290-023-00509-4

ORIGINAL RESEARCH ARTICLE

Eliciting Exploratory Patient Preference Data: A Case Study in a Rare Disease

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Accepted: 27 November 2023

Abstract

Introduction Qualitative and quantitative methods provide different and complementary insights into patients' preferences for treatment.

Objective The aim of this study was to use a novel, mixed-methods approach employing qualitative and quantitative approaches to generate preliminary insights into patient preferences for the treatment of a rare disease—generalized myasthenia gravis (gMG).

Methods We conducted a mixed-methods study to collect exploratory qualitative and quantitative patient preference information and generate informative results within a condensed timeline (about 4 months). Recruitment was facilitated by an international health research firm. Study participants first reviewed a brief document describing six treatment attributes (to facilitate more efficient review of the material during the focus groups) and were then provided a link to complete an online quantitative survey with a single risk threshold task. They then participated in online focus groups, during which they discussed qualitative questions about their experience with gMG treatment and completed up to three quantitative threshold tasks, the first of which repeated the threshold task from the online survey.

Results The study elicited both quantitative data on 18 participants' risk tolerance and qualitative data on their treatment experience, additional treatment attributes of importance, the reasoning behind their preferences, and the trade-offs they were willing to make. Most participants ($n = 15$) chose the same hypothetical treatment in the first threshold task in the online survey and the focus groups. Focus group discussions provided insights into participants' choices in the threshold tasks, confirmed that all the attributes were relevant, and helped clarify what was important about the attributes.

Conclusions Patient preference information can be collected using a variety of approaches, both qualitative and quantitative, tailored to fit the research needs of a study. The novel mixed-methods approach employed in this study efficiently captured patient preference data that were informative for exploratory research, internal decision making, and future research.

1 Introduction

Patient preference data provide essential insight into patients' priorities for treatment. An understanding of how patients trade off benefits, risks, and other attributes of treatment is gaining importance in clinical and regulatory decision making[1–3]. While methods such as the discrete-choice experiment (DCE) yield robust preference estimates

Key Points

A pragmatic, exploratory approach employing both qualitative and quantitative methods may be useful in generating preliminary preference data on a short timeline.

Most participants selected the same hypothetical treatment in a short online survey and in the follow-up focus group, suggesting that participants' choice of treatment was not heavily influenced by the discussion in the focus group.

The methods used to generate patient preference data can be tailored to the information needs, the timeline, and the characteristics of the population of interest for a given study.

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Published online: 21 December 2023

to support decision making, such studies can be time-consuming, costly, and difficult to conduct in rare diseases because of recruitment challenges. Research teams may need to employ alternative exploratory approaches to elicit preference data; in turn, these data can generate foundational evidence for larger studies. Exploratory preference data are particularly useful to inform internal strategy where data are needed for time-critical decisions and the need for scientific rigor is lower, or to guide early decisions for a treatment in the early stages of drug development when there may be uncertainty about the benefit-risk profile.

Several methods exist to collect patient preference information on the trade-offs patients are willing to make among treatment attributes, including qualitative interviews, focus groups, and surveys[4]. Each method captures aspects of patients' experiences that complement each other and has benefits and shortcomings. Qualitative research, for instance, provides insight into patients' experiences and preferences as captured in their own words, and 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. Insights from qualitative research with patients may be especially useful in the early stages of product development to discern unmet needs and treatment goals[5]. Rigorously conducted formative qualitative research also lends validity to quantitative preference-elicitation[6]. Quantitative patient preference surveys use quantitative preference-elicitation methods (e.g., DCEs) to collect standardized responses that provide insight into the relative importance patients place on different treatment attributes, and quantify the trade-offs patients are willing to make among treatment attributes [6]. Combining qualitative and quantitative methods can provide additional insights that each method alone will not provide.

In this paper, we present a novel mixed-methods approach to elicit exploratory preference data by employing a short quantitative survey followed by patient focus groups to elicit both qualitative and quantitative data. We describe the methods used and present a case study where the approach was applied to patients with generalized myasthenia gravis (gMG), a rare, chronic, heterogeneous, and unpredictable autoimmune disease characterized by dysfunction and damage at the neuromuscular junction. gMG is a debilitating and potentially life-threatening disease resulting in fluctuating chronic muscle weakness (e.g., eyes, mouth, throat, neck, limbs) and fatigability that worsens with activity and usually improves with rest; symptoms and intensity can vary from day to day and from hour to hour [7]. The therapeutic landscape in MG is changing, with the availability of new targeted treatments in recent years (e.g., eculizumab, ravulizumab, efgartigimod, rozanolixizumab, and zilucoplan) [8, 9]. Research regarding patient preferences for MG treatment features is scarce, and the objective of this study was to generate preliminary insights to help better understand patients' unmet needs and preferences for the benefits and risks of gMG treatments and to inform product development strategies. The mixed-methods approach was selected to gather quantitative preference data in a setting that allowed qualitative exploration of the reasons behind the patients' benefit-risk preferences.

In a mixed-methods study designed to collect both qualitative and quantitative patient preference information, researchers need to decide the format of the qualitative data collection (e.g., focus group or individual interview) and the type of preference question to include (e.g., DCE, best-worst scaling, or threshold exercise) [10–12]. There are known benefits and challenges with each method of qualitative data collection and with the different types of quantitative preference-elicitation questions[13, 14]. The choice of methods should be based on the information needs, timeline, and characteristics of the target population (e.g., prevalence of the condition, disease symptoms that might make focus groups difficult). If the goal is to generate preliminary data on a short timeline, a focused research question is essential. In addition, the use of existing materials to create the preference questions will expedite the process (e.g., past studies, descriptions of attributes created for other reasons). In this study, we present an example from a recently conducted study, designed for efficiency and with a condensed timeline (approximately 4 months), and outline the choices of methods made for this example.

2 Methods

2.1 Approach

We conducted a study that consisted of two steps: (1) a brief, online patient preference survey that included a single risk threshold task; and (2) online patient focus groups, during which we repeated the threshold task from the first step and expanded on the survey findings with additional quantitative and qualitative questions (Fig. 1). We selected focus groups over individual qualitative interviews because we thought that focus groups could be completed in a shorter timeline than individual interviews. The focus groups were small, with a maximum of six participants, to allow each participant more time to talk and to cover more material. We also believed that smaller focus groups would work better with the Zoom web-conference platform. A total of five focus groups were held—three in the United States (US) and two in Germany.

For the online patient preference survey, we first created a short pre-read document that described, in patient-friendly US English for US participants and patient-friendly German for German participants, six attributes of hypothetical

gMG treatments—two benefit attributes evaluating symptom improvements, three risk attributes evaluating adverse effects of treatment, and an administration attribute. The attributes were derived from internal discussions among the study team and unpublished patient preference research on potential treatment attributes for gMG that was ongoing at the time of study design. Some attributes had been defined and cognitively piloted for potential use in other preference studies, and others were attributes that were newly defined based on the study objectives.

We included the pre-read document to allow participants to review the materials at their convenience, facilitating faster review of the material and lessening the time demand of the focus groups. Providing pre-read material had the added advantage that we could schedule 60-min focus groups (shorter than typical focus groups) in case participants did not have the energy for a longer focus group. The pre-read document also contained a link to a short online survey participants were required to complete prior to participating in a focus group. The online survey asked a set of threshold technique questions designed to elicit participants' tolerance for one of three risk attributes. We selected the threshold technique because the results provide a risk threshold for each participant that can be evaluated individually, unlike DCE or best-worst scaling methods, which produce sample-level estimates. The threshold technique also requires fewer questions, which shortened the length of the online survey. In each threshold technique question asked in both the pre-read survey and the patient focus groups, participants were asked to choose between two hypothetical treatment profiles with varying levels of each of the six treatment attributes. Depending on which treatment they chose, participants were asked a series of follow-up questions in which the levels for a given adverse event (AE) risk increased or decreased, depending on the initial choice. If the participant was willing to accept the highest level of risk shown in the question, they were asked

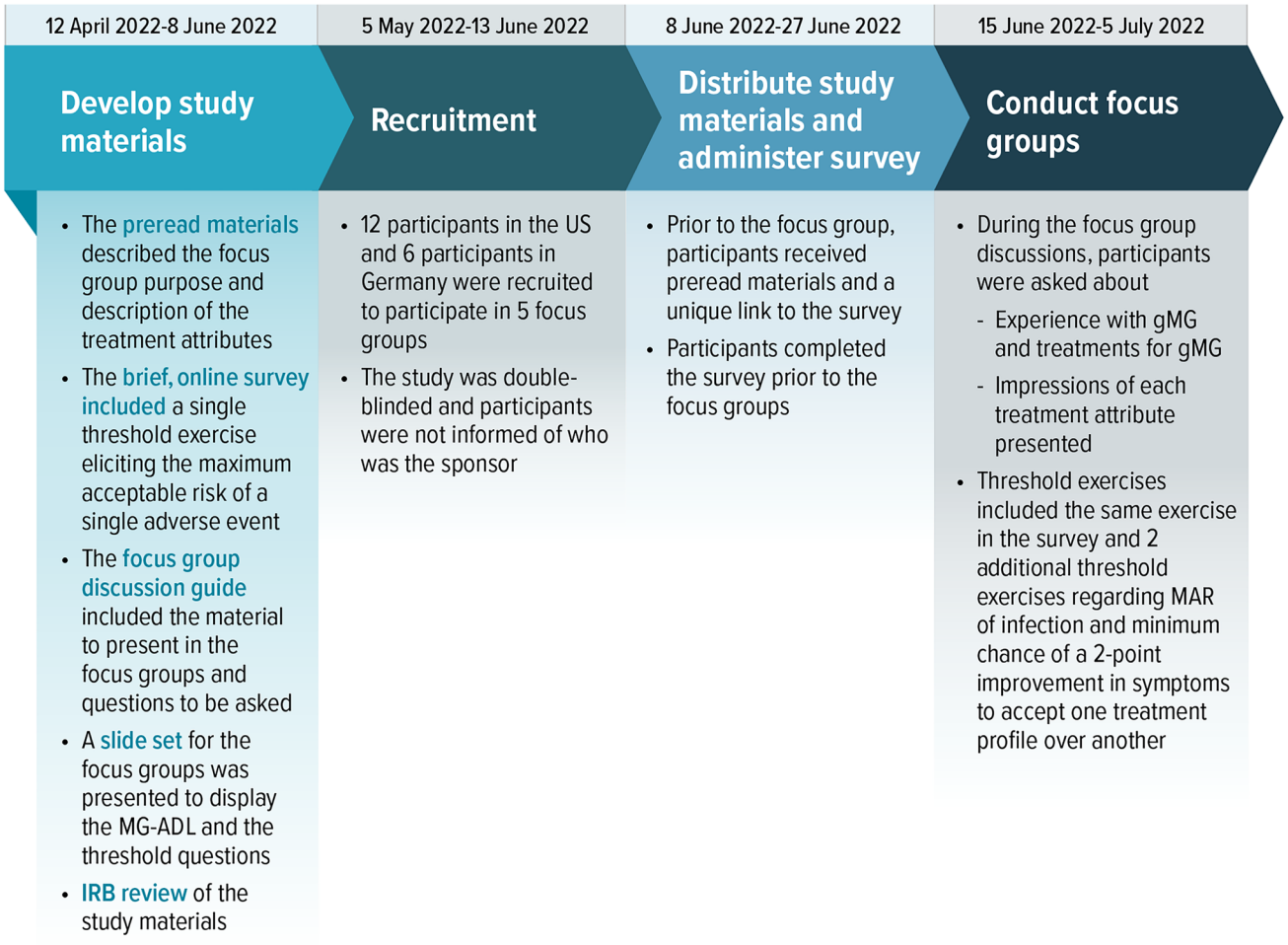

Fig. 1 Study design. *gMG* generalized myasthenia gravis, *IRB* Institutional Review Board, *MAR* maximum acceptable risk, *MG-ADL* Myasthenia Gravis-Activities of Daily Living, *US* United States

directly to report the maximum risk they would accept in an open-ended question. The participants' responses defined an interval within which their maximum acceptable threshold for a given AE risk or minimum chance of benefit to select one treatment over another could be calculated, and in some instances was stated directly.

The focus groups were conducted over Zoom to allow for presentation of slides during the discussion. Participants were asked open-ended questions about their experience with gMG treatments and what they liked or would change about their current treatment. After this discussion, to generate qualitative data on all attributes, the moderator reviewed the descriptions of the six treatment attributes included in the pre-read survey and discussed the attributes with participants to elicit qualitative insights into how participants interpreted those descriptions and their thoughts on the importance of the attributes. After reviewing the attributes, participants completed a series of up to three threshold technique exercises, time permitting; some focus groups were able to cover more material than others. Because we were interested in understanding participants' benefit-risk preferences, we chose to vary risk and benefit attributes in the threshold technique exercises. Two threshold technique exercises were focused on risk attributes to capture tolerance of AE risk, starting with the threshold technique question included in the pre-read survey. One threshold technique exercise was focused on a benefit attribute to capture the level of treatment benefits needed to accept worse levels of other attributes. Moderators followed up with probes about the reasons behind participants' choices in the threshold technique questions.

2.2 Sample

To be eligible to participate in the patient focus groups, participants had to meet the following criteria based on self-report: aged 18 years and older; currently live in the US or Germany; have a physician diagnosis of gMG; have a Myasthenia Gravis-Activities of Daily Living (MG-ADL) score ≥5 [14]; have uncontrolled gMG, either now or in the past; currently be receiving treatment for gMG; speak either US English or German; and be willing to be recorded.

Recruitment was facilitated by Global Perspectives, an international health research firm, via patient databases and support groups. Participants were first engaged to participate in the mixed-methods study. After they agreed to participate in the study, they were provided both the pre-read and access to the online survey. Participants received a modest honorarium for their time in completing the survey and participating in the focus group. The study protocol was reviewed on ethical grounds by the Institutional Review Board of RTI International and was deemed exempt from full review.

2.3 Analysis

No statistical analysis was conducted, as our approach was designed to provide preliminary insights into treatment preferences from a small sample on a relatively short timeline. The responses to the qualitative questions in the patient focus groups were summarized to identify themes or patterns in the way patients describe their experience with gMG as well as both current and past treatments for gMG. Feedback on the attributes and attribute descriptions was also summarized. Responses to the threshold technique questions regarding participants' maximum acceptable risk (MAR) for the AE included in both the pre-read survey and the focus groups were summarized and compared. Responses to the other threshold questions discussed in the focus groups were summarized. Finally, the discussion about why participants had selected the treatments and how they viewed the trade-offs in the threshold technique questions was summarized.

3 Results

The mixed-methods approach provided quantitative data on risk tolerance and benefit acceptance, with qualitative data on treatment priorities, individual interpretation of the treatment attributes, and rationales for the choices between the two hypothetical treatments. Table 1 presents the participants' characteristics and experiences with gMG treatment.

During the focus group discussions on the initial open-ended questions about treatment experience and symptom burden, participants expressed some concerns about gMG treatment and goals for gMG treatment beyond those reflected in the threshold exercises, providing extra information that a survey alone would not provide. For example, 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 more gMG treatment options were developed for seronegative patients (i.e., patients with no detectable auto-antibodies, for whom diagnosis and clinical management are typically more challenging) and patients with the newer antibodies. One US participant noted that it can be difficult to find a treatment covered by insurance because they are seronegative. Table 2 summarizes participants' descriptions of what aspects of gMG treatment they would choose to improve.

All participants completed the pre-read survey before the focus group discussions, as required. Most participants ($n = 15$) chose the same hypothetical treatment for the first threshold technique question in the patient focus group as they did in the pre-read survey. However, the average MAR participants stated during the focus groups was higher than the level stated in the pre-read 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).

Discussion in the focus groups provided insights into participants' choices in the threshold questions, including the influence of the three attributes that were not varied as part of the threshold questions. The discussion about each attribute confirmed that all the attributes were of interest to participants, and the comments about the choices in the threshold questions helped clarify what was important about the attributes. For example, many participants mentioned that the benefit of the treatment was the most important treatment attribute; they also mentioned the importance of onset of action. Some participants mentioned that mode of administration was important to them, but for most, other attributes were more important. A few participants identified additional information about the attributes that they would want to know in order to make a decision. The discussion also illuminated how participants had adjusted their lives to deal with both disease impacts and treatment AEs. For example, a few participants indicated that they rarely visit with other people because of their high risk of developing an infection, especially since the beginning of the coronavirus disease 2019 (COVID-19) pandemic. Finally, some participants indicated that they were more willing to try new treatments at the beginning of their diagnosis, but are less likely to try new treatments now that they have tried many treatments and lived with the disease.

4 Discussion

This study presents a novel mixed-methods approach to collecting information on patient preferences, simultaneously employing quantitative and qualitative methods to elicit preferences from focus groups of patients with a rare disease, i.e. gMG. Before the focus group discussions, participants were provided with a pre-read document describing the attributes of a potential gMG treatment to enable efficient review of the attributes during the discussion. Participants also completed a quantitative survey that included one threshold technique exercise to elicit the threshold for the risk of a treatment-related AE. Participants then took part in a focus group that gathered qualitative information on their experience with gMG and gMG treatments; they also completed and discussed up to three threshold technique exercises, time permitting. The pre-read survey provided quantitative data on risk thresholds obtained before the focus group discussion. The focus groups collected qualitative information on the participants' disease and treatment experience and uncovered additional treatment attributes of importance beyond those included in the survey, the reasoning behind participants' preferences and the trade-offs they were willing to make, and how their life circumstances impacted their choices. The focus groups were conducted on the Zoom platform and a slide set was used to present the MG-ADL and the threshold questions.

Participants' choice of treatment in the threshold technique exercise did not appear to be heavily influenced by the discussion in the patient focus groups, as almost all participants picked the same treatment in the first threshold question included in both the pre-read survey and the focus groups. The open-ended questions on tolerance for the AE risk included in the pre-read survey yielded higher MARs during the focus groups than those in the pre-read survey. The discussion in the focus groups may have influenced participants' thoughts about their MAR or clarified the intended definition of the attributes included in the threshold technique exercise for participants who may have interpreted the attributes differently during the pre-read survey. Taken together, the results from the qualitative and quantitative data reveal not only the treatment attributes that were most important to participants but also the drivers for these priorities as well.

Preference research can provide useful patient-centered insights across the research lifecycle. We elicited both qualitative and quantitative feedback from a rare disease population, using a combination of threshold exercises and open-ended questions. This mixed-methods study efficiently and cost effectively provided insights into patient preferences for the potential benefits and risks of emerging gMG therapies. The study included attributes that were either taken from previously conducted preference research or were newly identified. While DCEs generate rigorous evidence to inform decisions about available and emerging therapies, novel approaches such as ours can provide exploratory preference data to inform strategic decisions for sponsors and research teams. The approach also provides the basis for developing a more formal patient preference survey using the feedback provided about the treatment attributes and threshold questions during the qualitative discussions.

In our study, patient focus group discussions and threshold technique exercises yielded useful insights into patients' views of the benefits and risks of treatment, providing more detailed feedback on treatment priorities than would have been achieved with a purely qualitative or quantitative approach. The focus group discussions were lively and the participants were highly engaged, suggesting that they appreciated the opportunity to share their perspectives on gMG treatments. The results also illuminated patients' perspectives about the risks and benefits of treatments for the research team, allowing the study sponsor to understand patients' views of the safety and efficacy profile of emerging gMG treatments and complementing interpretation of the data from clinical development programs.

Table 1 Participant characteristics and experience with gMG treatment

Question	Participants [$N = 18$]	
Age, years		
Mean (SD)	46.1 (13.7)	
Median	46.5	
Min, max	26, 75	
Mean (SD)	46.1 (13.7)	
Sex [*n* (%)]		
Female	14 (77.8)	
Male	4 (22.2)	
Race or ethnicity [*n* (%)]		
White	18 (100)	
Employment status [*n* (%)][a]		
Employed full-time	5 (27.8)	
Employed part-time	1 (5.6)	
Student	1 (5.6)	
Unemployed	1 (5.6)	
Disabled	8 (44.4)	
Retired	5 (27.8)	
MG-ADL score		
Mean (SD)	9.3 (2.7)	
Median	9	
US ($n = 12$ [66.7%])		
Mean (SD)	10.1 (2.7)	
Median	10	
Min, max	6, 14	
Germany ($n = 6$ [33.3%])		
Mean (SD)	7.7 (2.0)	
Median	7.5	
Min, max	5, 11	
What treatments are you currently receiving for gMG? Please select all that apply[a]	US, $n = 12$ [*n* (%)]	Germany, $n = 6$ [*n* (%)]
Neostigmine (e.g., Prostigmin [US only: Bloxiverz])	6 (50.0)	1 (16.7)
Pyridostigmine (e.g., Mestinon [US only: Regonol; Germany only: Kalymin])	1 (8.3)	4 (66.7)
Plasmapheresis (plasma exchange [PLEX])	1 (8.3)	0 (0.0)
SCIg infusion (subcutaneous immunoglobulin)	1 (8.3)	0 (0.0)
IVIg infusion (intravenous immunoglobulin)	3 (25.0)	1 (16.7)
Azathioprine (e.g., [US only: Imuran, Azasan; Germany only: Aza Effect Pharma, Aza-Q, Azafalk, Azaqvida, Azathioprine, Imurek, Imurel])	1 (8.3)	1 (16.7)
Mycophenolate mofetil (e.g., CellCept)	1 (8.3)	0 (0.0)
Corticosteroids (e.g., prednisolone [Flo-Pred, Millipred, Orapred, Pediapred, Veripred])	4 (33.3)	4 (66.7)
Rituximab (e.g., Ruxience, Truxima [US only: Riabni, Rituxan; Germany only: Blitzima, MabThera, Rixathon])	0 (0.0)	1 (16.7)
Eculizumab (e.g., Soliris)	1 (8.3)	0 (0.0)
Efgartigimod (e.g., Vyvgart)[b]	2 (16.7)	0 (0.0)

gMG generalized myasthenia gravis, *max* maximum, *min* minimum, *MG-ADL* Myasthenia Gravis-Activities of Daily Living, *SD* standard deviation, *US* United States

[a]Participants were allowed to select more than one response to this question; therefore, the total number of responses may exceed the total number of participants who answered this question

[b]Choice was not listed in the screening survey but was spontaneously reported by participants during focus group discussions

Table 2 Desired improvement in treatment experience

What participants would improve about treatments	Comments
Shorter time until onset of efficacy	Many participants said they would like to know if the treatment was working within 1–2 months
Greater efficacy	Many participants indicated that they would prefer a treatment with stronger efficacy. Many participants also stated they would be willing to wait longer (e.g., 1–2 months, as opposed to 1–2 weeks) for treatment to work and tolerate more adverse events if the treatment provided stronger long-term efficacy
More convenient administration	Most participants preferred a treatment that could be administered at home—either self-administered or administered by a healthcare professional Most participants also preferred a subcutaneous injection to an intravenous infusion
Stability in efficacy	Some participants mentioned they would prefer a treatment that they received on a consistent basis instead of a treatment that made them wait to have the next cycle of treatment until they 'crashed' again. They would prefer treatments that stabilized their symptoms rather than receiving a large improvement that wears off over time
Accessibility	In the United States, insurance coverage was a big concern; a few participants mentioned they had to stop, or could not start, treatment because of insurance issues In Germany, access was not a major concern; one participant mentioned they had to hire a lawyer to get coverage for a treatment

By leveraging previous preference research and using a pragmatic mixed-methods design, our study provided results relatively quickly and efficiently—it was completed in approximately 4 months, rather than the year or longer needed to design a rigorous patient preference survey. We were also able to tailor the approach to the needs of the rare disease population, including using the pre-read materials and survey to shorten the time needed for the focus group. Such evidence can be used to inform drug development strategies in a timely manner or generate data for unanticipated questions and challenges. Where full DCEs may not be needed (e.g., to inform early internal decisions) or are challenging to conduct with a sufficient sample size (e.g., in rare diseases), mixed-methods approaches can provide exploratory evidence tailored to answer specific research questions.

The limitations of the pragmatic methods used in this study must be noted. A full DCE survey would include survey development work, including pretest interviews to test the survey instrument. The pre-read survey not only included some attributes that had been developed and tested in another setting but also described novel attributes, and the descriptions were not tested before administering the pre-read survey. The discussions about attributes in the patient focus groups provided feedback on the attribute descriptions and how they were interpreted, but this came after the pre-read survey was administered. Additional analysis of transcripts from the focus groups might also provide more insights, but the additional analysis would also add to the timeline and cost of the study. While the quantitative threshold questions provided data on the participants' risk tolerance, the small sample size limits the analysis that can be conducted and the generalizability of the results.

5 Conclusion

The novel mixed methods employed in this study allowed for a detailed view of patient preferences in an efficient way. This approach is useful for exploratory research and internal decision making, and can support future study design and product development. Furthermore, participants in this study expressed similar preferences in the pre-read survey and in the focus group discussions, supporting the view that surveys and qualitative research can function as complementary methods to elicit and explore a population's preferences. Patient preference information is a valuable source of data and can be collected using a variety of approaches tailored to fit the research needs, timeline, and budget of a study.

Acknowledgments Kimberly Moon of RTI Health Solutions provided overall project management for this study. Mark Price and Martina Sluga-O'Callaghan of RTI Health Solutions conducted the focus groups. Kate Lothman of RTI Health Solutions provided medical writing services under the authors' direction during development of this manuscript; these services were funded by UCB Pharma.

Declarations

Funding/support This study was funded by UCB Pharma.

Conflicts of Interest/Competing Interests This study was conducted under a research contract between UCB Pharma and RTI Health Solutions and was funded by UCB Pharma. Kerrie-Anne Ho, Meredin Stoltenberg, and Thais Tarancon are employees of UCB Pharma. Carol Mansfield and Anna Pierce are employees of RTI Health Solutions.

Role of the Funder/Sponsor Authors affiliated with the study sponsor, UCB Pharma, participated in the design and conduct of the study; analysis and interpretation of the data; preparation, review, and approval of the manuscript; and decision to submit the manuscript for publication.

Ethics Approval The RTI International Institutional Review Board reviewed the study protocol for this case study and confirmed it exempt from full review.

Consent to Participate All study participants provided informed consent.

Consent to Publish Not applicable.

Code Availability Not applicable.

Data Availability The datasets generated and/or analyzed during the current study are not publicly available.

Author Contributions Concept and design: K-AH, CM. Analysis and interpretation of data: K-AH, AP, MS, TT, CM. Critical revision of paper for important intellectual content: K-AH, AP, MS, TT, CM. Statistical analysis: AP, CM. Provision of study materials or patients: AP, CM. Obtaining funding: K-AH. Supervision: K-AH, CM. All authors have read and approved the final article.

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