

ARTICLE

Participant experiences with a new online modified-Delphi approach for engaging patients and caregivers in developing clinical guidelines

Courtney Armstrong MPH^a, Sean Grant DPhil^b, Kathi Kinnett MSN APRN^c, Brian Denger^d, Ann Martin MS CGC^e, Ian Coulter PhD^f, Marika Booth MS^g and Dmitry Khodyakov MA PhD^h

a Policy Analyst, RAND Health Care, RAND Corporation, San Francisco, CA, USA

b Assistant Professor, Department of Social & Behavioral Sciences, Indiana University Richard M. Fairbanks School of Public Health, Indianapolis, IN, USA; Adjunct Behavioral/Social Scientist, RAND Health Care, RAND Corporation, Santa Monica, CA, USA

c Senior Vice President, Clinical Care, Parent Project Muscular Dystrophy, Hackensack, NJ, USA

d Collaborative Programs Administrator, Parent Project Muscular Dystrophy, Hackensack, NJ, USA

e Director, The Duchenne Registry, Parent Project Muscular Dystrophy, Hackensack, NJ, USA

f Policy Researcher, Adjunct, RAND Health Care, RAND Corporation, Santa Monica, CA, USA

g Statistical Analyst, RAND Health Care, RAND Corporation, Santa Monica, CA, USA

h Senior Behavioral/Social Scientist, RAND Health Care, RAND Corporation, Santa Monica, CA, USA

Abstract

Background: Patient engagement in clinical practice guideline (CPG) development has increased significantly in recent years. However, only a few patients and caregivers join CPG development groups.

Objective: To describe participant experiences with a novel online, scalable approach for patient and caregiver engagement in CPG development.

Design: We developed and tested the RAND/PPMD Patient-Centeredness Method (RPM), a novel online modified-Delphi approach to patient engagement in CPG development that consists of an optional idea generation round and two rating rounds interspersed with an online discussion round.

Setting and Participants: Using the online ExpertLens™ system, we ran 2 concurrent panels of patients and caregivers of individuals with Duchenne Muscular Dystrophy (DMD).

Main Outcome Measures: We surveyed all 95 panel participants about their participation and satisfaction with the process. We also conducted telephone interviews with 25 participants.

Results: Participants expressed satisfaction with various ExpertLens™ features, noting that the system fostered lively interaction among them. Panelists also appreciated participating in an educational, interactive and convenient discussion forum that allowed them to share their opinions on the importance and acceptability of different recommendations. The RPM was viewed as empowering by patients and their caregivers who felt it would be useful for CPG developers.

Discussion and Conclusion: The results of our study show the overall participant satisfaction with a novel, scalable, online approach to engaging patients and caregivers in CPG development, which allows them to share their perspectives and lived experiences using a rigorous, systematic and iterative way that is similar to how clinicians provide their input.

Keywords

Duchenne Muscular Dystrophy, ExpertLens™, guideline development, lived experience of illness, Modified-Delphi method, online engagement, patient/caregiver perspectives, patient engagement, person-centered healthcare, RAND/PPMD Patient-Centeredness Method

Correspondence address

Dr. Dmitry Khodyakov, RAND Corporation, 1776 Main Street, Santa Monica, CA 90401-3208.

E-mail: Dmitry_Khodyakov@rand.org

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Introduction

Clinical practice guidelines (CPGs) are systematically developed to provide guidance on appropriate healthcare

options for various medical conditions [1]. A key component of the quality of these clinical recommendations is the composition of the guideline development group (GDG) [2-4]. GDGs, however, have historically struggled to engage patients in this process.

While clinicians contribute clinical expertise, patients and caregivers share their judgements, concerns, values and preferences on clinical processes based on their lived experience of a particular disease or condition [5]. Including patients and their caregivers throughout the stages of CPG development may help frame how patients view and think about clinical recommendations and may lead to the development of guidelines that are potentially more likely to be accepted by them [6]. Therefore, it is important that patients and their caregivers are involved in CPG development to ensure that they are patient-centered and to increase guideline adherence.

Increased interest in patient and caregiver engagement in the CPG development process has mobilized efforts to identify systematic methods for engaging these stakeholder groups [7,8]. However, due to limited budget and logistical constraints, the absence of patients in guideline development is common [1]. When engaged, only a few patient representatives are typically included in the CPG development process [9,10]. Additionally, patient representation primarily involves in-person engagement at guideline meetings [8], which can be particularly restrictive for patients with limited mobility. As a result, there have been calls to explore online methods as a promising way to include a wide array of patients and patient representatives in CPG development in a systematic and scalable way [11]. However, little is known about patients' and caregivers' online engagement experiences [11].

The objective of this study is to describe the experiences of patients and caregivers who tested the RAND/PPMD Patient-Centeredness Method (RPM) - a new online approach to patient engagement in CPG development [12]. Our study assesses patient and caregiver views on the usefulness of, their satisfaction with, and the challenges that they encountered while participating in the online engagement process that mirrors the approach clinicians used to develop the 2018 Duchenne Muscular Dystrophy (DMD) care considerations [13,14].

Method

Case Study

DMD is a progressively fatal neuromuscular disorder that affects approximately 1 in 5,000 boys [15]. The effects of this rare disease include muscle weakness, loss of ambulation and premature death by the mid-to-late second decade of life. In 2010, the US Centers for Disease Control and Prevention (CDC) developed DMD care considerations to help healthcare providers and Duchenne families better manage this rare disease [16,17]. Care considerations were revised in 2018 to include new evidence [13]. Clinical experts used a consensus method known as the RAND/UCLA Appropriateness Method (RAM), which involves reviewing existing evidence and rating the clinical appropriateness and necessity of different treatments or care management options [18].

Patients were not involved in the development of many sections of the 2018 DMD care considerations.

To address this gap, we developed the RPM - an online modified-Delphi approach for incorporating patient and caregiver input in the CPG development process. To test the RPM, we engaged individuals with DMD and their caregivers using ExpertLens™ - a previously-evaluated online modified-Delphi platform for expert elicitation and stakeholder engagement [19-21]. Participants were not required to travel to a centralized location, but rather shared their perspectives from the comfort of their home, which was particularly useful for DMD families for whom travel is challenging. While ExpertLens™ was not used to develop the CDC guidelines, we based our online approach on the modified-Delphi method and the RAM that clinicians used to develop the care considerations.

Study Participants

To identify individuals with DMD and their caregivers, we used the Parent Project Muscular Dystrophy's (PPMD) Duchenne Registry. In February 2017, we sent recruitment emails to 719 potential participants and asked them to express their interest in our study by answering a series of demographic questions. Out of 153 individuals interested in our study, we invited 122 individuals to participate. We excluded those who participated in our pilot study, parents and children providing the same email address and caregivers from the same household. Informed consent was provided to all participants prior to the start of the study and RAND's Human Subjects Protection Committee (HSPC) determined this study was exempt.

The RAND/PPMD Patient-Centeredness Method

The RPM is a three-round modified-Delphi process that can consist of 3 or 4 rounds [11] and that could be implemented using ExpertLens™. In Round 0, which is optional in the RPM, a separate, smaller sample of individuals with DMD and their caregivers provided input on the reasons for, barriers to, and facilitators of seeking care for various aspects of DMD.¹ In Round 1, individuals with DMD and their caregivers reviewed information about, rated and explained their perspectives on 19 care considerations. Specifically, they used 9-point Likert scales to rate the importance and acceptability of each care consideration for a typical DMD family. The RPM considers importance and acceptability of guideline recommendation to be key dimensions of patient-centeredness. In Round 2, participants saw bar charts that showed the distribution of group's responses, described how their Round 1 responses (presented as red dots on the charts) compared to those of other participants and stated whether the group reached agreement on the importance or acceptability of a given care consideration determined using the RAM's approach to measuring consensus [18].

¹ Round 0 participants were not included in the sample described in this article.

Table 1 Participant Characteristics

	Participated in at least one round (n=95)	Participated in Round 1 (n=88)	Participated in Round 2 (n=74)	Participated in Round 3 (n=56)
Participant Type				
Caregiver	71 (75%)	65 (74%)	58 (78%)	43 (77%)
Individual with DMD	24 (25%)	23 (26%)	16 (22%)	13 (23%)
Gender				
Female	59 (62%)	53 (60%)	47 (64%)	35 (63%)
Male	36 (38%)	35 (40%)	27 (36%)	21 (37%)
Hispanic/Latino/Spanish*				
Yes	4 (4%)	2 (2%)	3 (4%)	1 (2%)
No	90 (95%)	85 (97%)	70 (95%)	54 (96%)
Race				
White	86 (91%)	82 (93%)	67 (91%)	52 (93%)
Black/African American	1 (1%)	1 (1%)	1 (1%)	0 (0%)
Asian	4 (4%)	3 (3%)	4 (5%)	4 (7%)
Multi-race	1 (1%)	0 (0%)	1 (1%)	0 (0%)
Other	3 (3%)	2 (2%)	1 (1%)	0 (0%)
How far do you usually travel to receive neuromuscular care?*				
<50 miles	38 (40%)	37 (42%)	30 (41%)	25 (45%)
50-99 miles	22 (23%)	20 (23%)	16 (22%)	12 (21%)
100-249 miles	17 (18%)	13 (14%)	14 (19%)	8 (14%)
≥250 miles	15 (16%)	15 (17%)	13 (18%)	9 (16%)

Notes: * Not all participants provided responses to this question. There were no statistically significant differences between participants and non-participants in different rounds as measured by Fisher’s exact test [23].

Each question from Round 1 had its own chart and a summary of participant comments. In this round, participants also discussed Round 1 results using asynchronous and online discussion boards moderated by 3 trained professionals (a caregiver, a genetic counselor and a modified-Delphi expert). Participation in the discussion was partially anonymous: we used IDs that only revealed whether a participant was an individual with DMD or a caregiver/family member.

To encourage active engagement during discussion, participants received periodic discussion digests *via* email. Lastly, in Round 3, participants had the opportunity to revise their original ratings based on Round 2 feedback and discussion of Round 1 results. Additional information on study design [22] and patient-centeredness ratings [23] can be found elsewhere.

Assessment of Participant Experiences

To assess participant experiences with the RPM, we used a mixed-methods evaluation approach. We surveyed all Round 1 and Round 3 participants and conducted telephone interviews with a purposive sample of participants at the end of the modified-Delphi process. Participants used a 7-point Likert scale (1 = strongly disagree, 2 = disagree, 3 = slightly disagree, 4 = neutral, 5 = slightly agree, 6 = agree, 7 = strongly agree) to rate statements describing different aspects/features of the online modified-Delphi process (see Table 2). The majority of these statements are based on research on

computer-mediated communication and have been used in previous ExpertLens™ panels [20,21].

After the entire process finished, we selected a diverse sample of 5 patients and 20 caregivers for semi-structured telephone interviews to further share their experiences and thoughts about the RPM. We stratified our interview sample based on participant type (patient or caregiver), their level of engagement and participation in each round and their level of satisfaction with the online engagement process. The study team developed semi-structured interview questions that covered several topics about participants’ experiences: overall participation burden, advantages of the iterative Delphi process relative to a simple survey, perceived level of engagement in the discussion round, benefits and challenges of online discussion and overall usefulness of the RPM for patient/caregiver engagement in the process of CPG development. Interviews ranged from 30 to 45 minutes. Study participants received a \$50 Amazon.com gift card for successful completion of each study round, for a total of \$150 and an additional \$25 gift card for completing the interview.

Data Analysis

We conducted descriptive analyses of responses to satisfaction questions. As in previous studies of participant experiences with the ExpertLens™ process [20,21], we considered a mean of ≥5 on positively worded statements regarding different aspects/features of the online

Table 2 Survey Results about Participant Experiences with the Online Modified-Delphi Approach

Rating Statements	Total			Individuals with DMD			Caregivers			P value
	M	SD	N	M	SD	n	M	SD	n	
The topic of this study is important	6.49	0.64	54	6.67	0.65	12	6.44	0.64	42	0.282
The invitation text was clear	6.18	1.42	77	6.37	1.50	19	6.12	1.40	58	0.510
The login instructions were clear	6.44	1.07	77	6.82	0.39	19	6.31	1.18	58	0.008
The rating scales used in this study are clear	6.05	1.19	77	6.05	1.61	19	6.05	1.03	58	0.100
The charts in Round 2 were clear	5.81	1.25	52	6.33	0.78	12	5.65	1.33	40	0.098
The charts helped me understand how my responses compared to those of other participants	6.1	1.19	52	6.75	0.45	12	5.90	1.27	58	0.001
The ExpertLens™ system is easy to use	5.35	1.64	54	5.08	1.44	12	5.43	1.71	42	0.533
I would like to use ExpertLens™ in the future	5.34	1.58	54	5.00	1.60	12	5.44	1.58	42	0.403
Participation in this study was satisfying	5.61	1.27	54	5.83	1.19	12	5.55	1.29	42	0.500
The study will generate useful outcomes	6.04	0.97	54	6.08	1.16	12	6.02	0.92	42	0.853
The discussion was informative	5.92	1.16	52	5.75	1.14	12	5.97	1.18	40	0.564
I was comfortable sharing my views	6.33	0.79	52	6.42	0.90	12	6.30	0.76	40	0.656

Notes: Participants were asked to use 7-point Likert-type response scales, where, 1 = strongly disagree, 2 = disagree, 3 = slightly disagree, 4 = neutral, 5 = slightly agree, 6 = agree, 7 = strongly agree, to indicate the extent to which they agreed with each of the statements presented in this table. The sample sizes differ because participants were asked to rate different statements after different study rounds.

engagement process to be an indicator of a “positive” or “favorable” experience. We rounded the mean values to the nearest whole number. We also used t-tests to determine the existence of statistically significant differences between patient and caregiver experiences.

To qualitatively analyze the interview data, we developed a code book based on the interview guide and coded the telephone interview data using MaxQDA, a computer-assisted qualitative data analysis software [24]. CA (an experienced qualitative researcher) coded all qualitative data, which were then reviewed by DK (the principal investigator of the study who is an expert on qualitative and Delphi methodologies, stakeholder and patient engagement and the ExpertLens™ approach). CA and DK developed the code book together and discussed any disagreements about the way it was applied to the interview data until consensus was achieved. A similar process was used in previous studies [25]. Finally, we used an inductive thematic approach to qualitative data analysis to jointly identify emergent themes about participants’ experiences in the RPM, satisfaction with the data collection process, online engagement process and usefulness of the ExpertLens™ platform and the online engagement method.

Results

Out of 122 invited participants, 95 (78%) participated in at least one modified-Delphi round. Of these, 88 (93%) participated in Round 1; 74 (78%) participated in Round 2 and 56 (59%) participated in Round 3. Seventy-seven Round 1 participants (88%) and 54 Round 3 participants (96%) answered a series of questions regarding their satisfaction with the ExpertLens™ process.

The majority of study participants were caregivers (75%); most were female (62%) and white (91%). Forty percent of study participants reported living within a 50-mile radius from a clinic where individuals with DMD receive neuromuscular care. There were no statistically significant differences in demographic characteristics of those who participated and those who did not participate in different rounds based on the results of the Fisher’s exact test (see Table 1) [23].

The results section is structured thematically around 5 main themes describing participant experiences and perceived usefulness of our online approach to engaging patients and caregivers in CPG development that we developed based on the analysis of survey and interview data. We describe each of these themes below by using both survey and interview data and provide additional illustrative quotations in an online appendix (see Appendix A). To protect participant confidentiality, we used codes that only revealed whether they are individuals with DMD or caregiver, as well as their panel (A or B).

Perceived Importance of the Study and Comprehension of the Tasks

Study participants thought that the study topic was important, understood the reasons why it was conducted and felt that they understood how the data would be collected. To illustrate, those who completed a survey agreed that the study topic was important (M=6.49; SD=0.64, see Table 2). Our interviewees also reported that they fully understood the objective of the study. As caregiver A10 explained, the study purpose was to:

“get a better idea of the priorities of patients and caregivers and ... how we would respond to certain recommendations” (Caregiver A10).

From the perspective of an individual with DMD A01:

“the purpose of the study was to get both patient and family input on new proposed clinical guidelines with the goal of providing more efficient and better care for those with DMD” (Individual A01).

At first, however, some participants did not fully understand the purpose of the study. Nonetheless, even those initially confused understood the data collection process as they progressed through study rounds. According to caregiver B12:

“Initially, I didn’t really know what the point was, but as it went along, I kind of figured out what was going on” (Caregiver B12).

Despite the uniqueness and novelty of our data collection process, participants generally reported high levels of understanding the logistics of participation in our study. In their responses to survey questions, participants agreed that the invitation text ($M=6.18$, $SD=1.42$), login instructions ($M=6.44$, $SD=1.07$) and rating scales ($M=6.05$, $SD=1.19$) were clear. Individuals with DMD reported somewhat better understanding of login instructions than caregivers ($M=6.82$ vs. $M=6.31$, $p=0.008$, respectively).

In Round 2, participants saw Round 1 results presented in a chart format. Overall, they had positive attitudes towards the charts. Survey participants agreed that the charts were clear and easy to understand ($M=5.81$, $SD=1.25$) and that charts helped them understand how their responses compared to those of other participants ($M=6.1$, $SD=1.19$). It is worth noting that, compared to caregivers, patients were more likely to have a more favorable opinion about the usefulness of Round 2 charts in helping them understand the difference between their own and other participants’ responses ($M=5.9$, $SD=1.27$ and $M=6.75$, $SD=0.45$, $p=0.001$, respectively).

Most of our interviewees also expressed a positive opinion about the charts, suggesting that they help participants quickly understand what the group thinks. As caregiver B05 put it:

“The charts are beneficial just to have a visual look of where your answer falls and where the rest of the group went with their answer. It is important to have it. It allows for a glance into what other people were thinking” (Caregiver B06).

An individual with DMD B06 also agreed on the benefits of having charts:

“The charts are easy to understand because it’s a visual, so it’s easy to see. Seeing how my answers compared to others made me kind of want to look more in the discussions to see why” (Individual with DMD B06).

Nonetheless, a very small number of caregivers indicated that while they understood the charts, they:

“found it a little hard to find my original response” on the chart (Caregiver A04).

Benefits of Online Engagement Using ExpertLens™

Survey participants generally had positive online experiences: they reported that ExpertLens™ was easy to use ($M=5.35$; $SD=1.64$) and that they would like to use the platform again in the future ($M=5.34$; $SD=1.58$). Our interviewees discussed multiple benefits of using the online modified-Delphi platform for the purposes of soliciting patient and caregiver input, as compared to surveys and in-person meetings. Within the broad theme of benefits, participants emphasized 3 aspects of the ExpertLens™ process that they particularly enjoyed, including the interactive nature, anonymity and convenience.

Overall, participants enjoyed the interactive nature of the process that included feedback loops and opportunities to directly engage with and learn from other participants. The iterative nature of data collection fostered better participant engagement by increasing their willingness to share and discuss their opinions and experiences with others, resulting in the development of new and shared perspectives. According to caregiver A01, ExpertLens™ gave individuals:

“an opportunity to see where you are, in terms of other parents and patients, and what things they are discussing that you may not be thinking about” (Caregiver A01).

The ability to gain new insights from engaging with others in a similar predicament was highly regarded as a benefit by caregiver B02 who said:

“I really did appreciate what other people were writing about, and I learned a lot by just reading the other responses” (Caregiver B02).

Participants also appreciated the partial anonymity of Round 2 because they felt that it gave them a sense of comfort and security during the discussion. Participants commented on the usefulness of the labels that were used during the discussion round to denote comments made by different participants, and how these labels helped them understand different perspectives without divulging too much personal information:

“I like the ability to not put a face or a name on the person, just a number. And that way if there was major disagreement you could still speak your mind” (Caregiver B03).

While participants generally found the label to be sufficient and useful, some recommended adding a participant profile with the patient’s age, region and disease stage while still keeping participant’s name anonymous. As noted by individual with DMD A01, the use of the labels:

“reinforced, in my mind, that not only do I have to think about my perspective as a patient, but of the caregiver perspective, as well” (Individual with DMD A01).

Finally, compared to in-person meetings, participants found the online engagement process to be a very convenient way of participating in research, particularly given physical, geographic and time constraints Duchenne families face. To illustrate, individual with DMD A02 said:

“I think it’s just a more convenient way of doing it, considering that most people find it difficult to get out of the house. It was more convenient for me, instead of going somewhere” (Individual with DMD A02).

The convenience of an online approach was amplified by the asynchronous nature of the engagement process. Participants found the engagement process accommodating given that they could complete each round at their convenience rather than finishing all of it in one sitting. As individual with DMD B01 put it:

“[the engagement process] is set up nicely so that I can come in and do one of the sections at a time. I knew I could start one and get through maybe one of them and then come back at a later time to tackle each of the other ones, in turn” (Individual with DMD B01).

Satisfaction with the RPM

Survey participants agreed that their participation in this study that tested the RPM was satisfying ($M=5.61$; $SD=1.27$). By analyzing the interview data, we identified 4 factors that have contributed to participants’ overall high satisfaction of the engagement process.

First, the engagement of both caregivers and patients stimulated discussion of the importance of the patient perspective. Participants overwhelmingly agreed that the adult patients’ voice carried more weight than the caregiver/parent perspective when rating many recommendations, especially if caregivers were new to a Duchenne diagnosis, did not have much experience or knowledge of the disease progression, or even when a parent had a son who was too young to make decisions on their own. As caregiver B01 noted:

“It was definitely very helpful to hear what the patient is feeling rather than what I feel as a parent, which is pretty helpful because I want to do what’s important for my son even though he’s not old enough right now” (Caregiver B01).

Moreover, patients agreed that the parent/caregiver perspective can be limited when making certain clinical decisions:

“my parents absolutely want the best for me, it’s just they don’t necessarily exactly understand how I feel.” (Individual with DMD A03).

Second, the RPM offered patients a unique opportunity to serve as the experts and share their knowledge and experiences directly with caregivers:

“There’s a lot of things that [caregivers] aren’t being prepared for, so I was able to tell them that certain things may not be as easy as people might think” (Individual with DMD A02).

As pointed out by individual with DMD B04, the opinions and perspectives of caregivers, and especially patients, are not considered during the CPG development process. This emphasizes the need for a process to incorporate the patient voice in care decision-making and guideline development.

Third, participants enjoyed learning new information and new perspectives from other DMD families. As explained by caregiver B05, participants were able to gain more insight from others who have had a lot more experience with DMD:

“The hope is that you learned something from reading other people’s comments or at the least gives you a point of view into somebody else’s world of possibility. And that may not benefit you immediately, but at least in the future you can keep that in the back of your mind” (Caregiver B05).

Others reported gaining a better understanding of the disease phases based on their review of discussion comments and considered their participation a unique learning opportunity. This was particularly important for parents of younger boys who have yet to encounter a specific disease stage.

Finally, the Delphi-based RPM allowed participants to change their original responses, if they wished to do so, but did not require them to reach consensus. There were 2 broad perspectives on the topic of revising original responses. Some participants appreciated the ability to change their responses. To use the words of caregiver B06:

“I actually did like revising my answers. After reading everyone’s situations and different opinions, I learned more about [different topics], so I was able to tweak my answers because it made me feel differently” (Caregiver B06).

Many caregivers reported that their opinions and ratings changed after seeing patients’ ratings and comments. Caregiver B06 explained how thinking about height was not initially considered important:

“but for some, it was a big deal because they are non-ambulatory and that makes a big difference when your peers are all towering over you when you’re standing, as opposed to when you’re sitting. So those kinds of answers, I did change a little because it was quite clear a lot of people thought that was important” (Caregiver B06).

Others, however, reported that while their reasoning and understanding of issues broadened and they were:

“exposed to a new way of thinking, the [rating] answers never really changed that much” (Individual with DMD A02).

Some pointed out that they were already well-versed and knowledgeable about the disease and did not feel pressure to change their answers just to conform, which highlights the importance of not requiring participants to change answers or to reach consensus. Caregiver B07 stated that while the new perspective was appreciated, not much changed in Round 3:

“I liked the fact that we shared answers with others and we were able to go back, re-evaluate and see what others thought and then revisit your answer. So, although I pretty much stayed the same on my answer, I did reevaluate and assess it” (Caregiver B07).

Perceived Usefulness and Impact of the RPM

Survey participants agreed that this study would generate useful outcomes ($M=6.04$; $SD=0.97$). The RPM may be useful for engaging patients and their caregivers in CPG development because it facilitates sharing of opinions, perspectives and experiences. Our participants noted that because DMD is a rare disease, doctors may not see many patients with this condition, which may create variability and inconsistencies in recommendations and treatment options offered to patients. As such, the RPM created a forum for Duchenne families to share information, exchange perspectives, discuss experiences and debate recommendations, which is:

“super important, especially with muscular dystrophy, just because we really don’t know a ton about it,” (Caregiver A08).

By asking patients and caregivers to rate the importance and acceptability of care recommendations, the RPM provided a helpful format for soliciting the patient and caregiver perspectives during CPG development. As a caregiver A09 put it:

“It’s really important to engage patients and their caregivers. I believe that only they have the firsthand experience and they can share their important insight. Because they are dealing with everyday life, they are facing challenges and they are creative. They are so creative that they are finding solutions to how to overcome the challenges” (Caregiver A09).

The RPM also helped empower patients and caregivers by allowing them to apply some of the new information during the patient-clinician encounter. According to caregiver B10, participation in the online panel was useful during a recent provider visit because it:

“brought up questions that I didn’t even know to ask. It brought up topics that I wasn’t aware of. It empowered me. It gave me a little bit of a say in what’s going on. It made me feel better that the parents’ perspective is being taken and that they’re looking into these different areas, which is kind of cool” (Caregiver B10).

Participants also commented on the usefulness of the method for clinicians, suggesting that doctors could benefit from patient and caregiver input to support the current standards and recommendations. It was argued that until the Duchenne guidelines are universally followed by all the neuromuscular centers around the country, physicians should consider the patient and caregiver voice, which can be solicited using this engagement method:

“Once the guidelines are developed this way, they’re going to be pretty useful for clinicians. It will really help them understand what really matters most and is important for the community,” (Caregiver A01).

Challenges with the ExpertLens™ Process and Suggestions for Improving the RPM

Although participants were overall satisfied with the ExpertLens™ process, some offered several suggestions for improving the participant experience. Participants generally understood the purpose and benefits of answering the same questions twice. However, in Round 3, some suggested that showing not only their own Round 1 responses to the rating questions on the charts, but also their own rationale comments right below the charts might be helpful.² Doing so could help participants add to or modify their original rationale comments:

“It was difficult to determine what my initial rationale comment was. I think it would have been better if I could have somehow seen what all my answers were in that category [of rationale comments] in Round 1” (Caregiver B06).

Although participants agreed that the Round 2 discussion was informative ($M=5.92$; $SD=1.16$) and that they were comfortable sharing their views ($M=6.33$; $SD=0.79$), a few participants felt there could have been more clarity in the instructions about what to expect from the modified-Delphi process, especially from Round 2 discussions. Some individuals did not fully understand whether it was necessary to review all Round 2 comments related to a single rating question:

“The instructions weren’t exactly clear as to whether you have to respond to each one, just a few, or the ones you disliked or liked” (Caregiver B04).

They suggested to make the intent clearer in the instructions.

Participants also offered additional suggestions around the email digests and their layout. While several participants found the email digests to be useful, they felt that the digests could be improved by embedding web links directly into the email digest to instantly connect users to updated comments and/or responses.

Others commented on the organization and layout of the different rounds. As caregiver B11 put it:

² In Round 2, participants saw their rationale comments, but they were not listed right next to the charts.

“visually, I had to really focus to make sure I finished the page and that I clicked on each thing to make sure I could see all the responses” (Caregiver B11).

Caregiver B12 added:

“eventually I figured it out, but I would have liked it to be a little better organized so that you could see what people’s responses are and how the threads were organized” (Caregiver B12).

Finally, a few participants felt that there were ways to decrease participation burden without comprising the integrity and value of responses. Caregiver A10 suggested having each participant focus on just one topic:

“It would be interesting if you picked just one topic, like either weight or height. Then, on days one and two, you put your comments in. On days three and four, you read through [the comments], and then on days five and six, you responded” (Caregiver A10).

Discussion

Little rigorous empirical evidence exists on engaging patients and caregivers in CPG development and their attitudes and perceptions of engagement processes [26]. Our research not only developed and evaluated the RPM - a novel, online, scalable method that solicited feedback on the importance and acceptability of the CPG - but also assessed patients’ and caregivers’ attitudes towards and their perceptions of the method. Our findings contribute to a limited body of evidence on the usefulness of online methods that include patient and caregiver perspectives and user satisfaction [11]. Additionally, our research addresses the lack of evidence involving patients with rare diseases in CPG development [5].

We found that participants overwhelmingly reported positive experiences with the RPM, citing the convenience, anonymity and asynchronous nature of online engagement. This finding further supports evidence that online engagement methods may facilitate more openness from patients and their families [7], allowing them to be more comfortable to agree or dissent with others’ opinions. Participation convenience is critical for engaging patients with limited mobility and time constraints. Providing individuals with DMD and their caregivers an opportunity to share their insights in a manner that did not require travel or face-to-face engagement allowed for a more convenient way of sharing input and data gathering.

Moreover, our study participants also appreciated input provided by individuals with Duchenne and gave more weight to their perspectives due to their lived experiences of the disease. This demonstrates the importance of soliciting patient perspectives, particularly within the Duchenne community, where caregivers often make crucial treatment decisions on behalf of their young children. Additionally, the information and insight gathered from participating in such an engagement process could be immediately useful during medical visits.

Lastly, ExpertLens™ offered a platform for implementing the RPM that is not only useful for guideline development, but also for the patient and caregiver community engagement more broadly. Participants reported that the method encouraged learning and community-building through the interactive rounds. For individuals impacted by Duchenne and other rare diseases, the online modified-Delphi process can elevate the patient and caregiver voice in CPG development while simultaneously creating a sense of community among busy individuals who are located in different parts of the country or around the world.

We note several limitations to our study. Because this is a case study focused on only one test of the RPM, it may not be generalizable beyond our sample or limited to the Duchenne community. More research is needed to understand whether the specific nature of this rare condition allows participants to be more engaged than others. Second, not all invited study participants completed the study and we interviewed only a sub-sample of participants. Although our 78% study participation rate compares favorably with other online Delphi studies, our results are limited to the perspectives of those who completed the survey or the interview. Lastly, not all interview data were double coded. To increase the rigor of the analysis, the code books were developed jointly by the interviewers, the coder and the study PI. Moreover, the study PI reviewed all coding results and discussed any disagreement with the coders until consensus was reached.

Conclusion

Our online modified-Delphi engagement approach called the PRM and the ExpertLens™ platform offer a useful novel opportunity for patients and caregivers to participate in CPG development by providing their perspectives and lived experiences using a rigorous, systematic and iterative approach that is similar to how clinicians provide their input. Participants appreciated the ExpertLens™ features that fostered lively interaction and expressed their satisfaction with engaging in an educational, interactive and convenient forum that allowed them to share their opinions on the importance and acceptability of clinically appropriate and necessary care considerations. Lastly, the RPM could inform CPG development committees and could help determine the importance and acceptability of various recommendations, which are key components of the GRADE (Grading of Recommendations Assessment, Development and Evaluation) Evidence to Decision Framework [27].

We note several areas for future research. While the majority of participants comprehended the tasks for each round, some initially found the instructions confusing, which could be attributed to the uniqueness of the iterative and interactive nature of the modified-Delphi process. This finding is consistent with evidence showing that online methods for engaging patients may require additional initial training on how to complete the required tasks [28]. More research is needed to better understand how best to address

the challenges participants face when completing online engagement approaches. Furthermore, future studies should demonstrate the added value of engaging patients in CPG development using the RPM. One objective metric could be guideline adherence.

Acknowledgements and Conflicts of Interest

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Appendix

Interview Themes and Illustrative Quotations

Theme	Quotations
1. PERCEIVED IMPORTANCE OF THE STUDY AND COMPREHENSION OF THE TASKS	
<i>Participants Understood the Objective of the Study</i>	<ul style="list-style-type: none"> • “It felt to me that the purpose was to help adjust and set guidelines or recommendations for different treatment for Duchenne. Like they just wanted to get input from patients and clients, to better inform people about the disease” (Individual with DMD A2). • “I kind of thought the purpose was trying to find out what the patients and caregivers think is important, as far as the doctors go, what they’re concerned about” (Individual with DMD B12). • “Initially, I didn’t really know what the point was, but as it went along, I kind of figured out what was going on” (Caregiver B12).
<i>Positive Attitudes Towards the Charts</i>	<ul style="list-style-type: none"> • “I think the charts are beneficial just to have a visual look of where your answer falls and where the rest of the group went with their answer. I think it is important to have it. It allows for a glance into what other people were thinking” (Individual with DMD B05). • “The charts are easy to understand because it’s a visual, so it’s easy to see. Seeing how my answers compared to others made me kind of want to look more in the discussions to see why” (Individual with DMD B06). • “[The charts] made total sense and it was really nice to have so you could see what other people were thinking, as well, because there’s obviously no right or wrong answers” (Caregiver A42). • “The charts I think they’re beneficial to have a visual look of where your answer falls, where the rest of the group went with their answer; whether it was a wide, solid mark or just you standing alone on Number 4 or Number 3 scale. I think it is important to have it. It allows for a glance” (Caregiver B19). •
2. BENEFITS OF ONLINE ENGAGEMENT USING EXPERTLENS™	
<i>Interactive Nature of the Process</i>	<ul style="list-style-type: none"> • “I thought it was really good. I mean, there were some things that I didn’t think of from certain perspectives...like the patients were able to say - especially my son being 20 and having autism, he doesn’t have a lot of say simply because he’s just not capable of things that he may want to

	<p>do. So hearing it from some of the patients was very interesting for me and I learned some information. But I think overall, it was a good experience” (Caregiver B10).</p> <ul style="list-style-type: none"> • “I learned stuff that I didn’t really think about before and I learned about some treatment things that I didn’t really know about before” (Individual with DMD A14). • “ExpertLens™ gave individuals an opportunity to see where you are, in terms of other parents and patients, and what things they are discussing that you may not be thinking about” (Caregiver A1). • “I really did appreciate what other people were writing about, and I learned a lot by just reading the other responses” (Caregiver B2).
<p>Partial Anonymity of the ExpertLens™ process</p>	<ul style="list-style-type: none"> • “I like the ability to not put a face or a name on the person, just a number. And that way if there was major disagreement, you could still speak your mind” (Caregiver B3). • “It’s easier to be honest about your own experiences. Like you can just feel a little bit more freedom to express yourself maybe where you wouldn’t otherwise” (Caregiver A2). • “I also liked how it said whether it was a caregiver or a patient with DMD. So it kind of gave me a better understanding of how their opinion was. Because I know that adults and individuals with DMD, sometimes we see things more important than maybe what parents might and parents might think the opposite of certain things” (Individual with DMD B12). • “That was helpful to know that it was completely anonymous and I feel if you didn’t know that, it might change how I would respond to comments. It reinforced, in my mind, that not only do I have to think about my perspective as a patient but of the caregiver perspective, as well” (Individual with DMD A1).
<p>Convenient Way of Participating in Research</p>	<ul style="list-style-type: none"> • “You could log on at any time when it’s convenient for you and based on your own personal schedule and you could do it. So I mean, that makes it easy. I’m sure if you said, “Okay, this is the times we’re doing it,” a lot of people wouldn’t be able to do it at all during those times. So, I think just being able to get on when you can and know, okay, I have this amount of time to get it done, is good” (Caregiver B24). • “Yes, actually as I said, online discussion board is very convenient for those like I am a caregiver of DMD kid. So I am kind of busy every time, but I am interested in this kind of survey which is related to Duchenne Muscular Dystrophy. So, it is very beneficial for me to do this online survey as I can post my comments, I can participate in this survey at my own pace, at my own convenience of time” (Caregiver A2). • “I think it’s just a more convenient way of doing it, considering that most people find it difficult to get out of the house, I guess. It was more convenient for me, instead of going somewhere, I guess” (Individual with DMD A2). • “[ExpertLens™] is set up nicely so that I can come in and do one of the modules at a time. I knew I could start one and get through maybe one of them and then come back at a later time to tackle each of the other ones, in turn” (Individual with DMD B1).
<p>3. SATISFACTION WITH THE RPM</p>	
<p>Panels Included Both Caregivers and Individuals with DMD</p>	<ul style="list-style-type: none"> • “It was more the patients themselves, like the older patients. So just understanding from their perspective things they’ve been through or how they see and feel things is definitely more important. That’s not always what you get as a caregiver. You think about what you want for the person that you’re taking care of and so, yeah” (Caregiver A44). • “As caregivers, I felt like we were either on the same page or totally in different parameters, just kind of nowhere near each other. But when I was reading the actual patient - because then it makes me think, “Okay, well I’ve got to think about it from my sons’ perspective, if they were going through this, would they want to do this or not?” Instead of just assuming you have to do everything because I want you to do it versus quality of life for them and their input in making those decisions, which is equally as important if not more so” (Caregiver B24). • “I think it was helpful to get you out of just thinking about what’s valued by the patient, because...for certain parts of our lives, our families are heavily involved in care and making decisions based on recommendations. Or a recommendation that might be appropriate for one might not be appropriate for the other” (Individual with DMD A1).

	<ul style="list-style-type: none"> • “It was definitely very helpful to hear what the patient is feeling rather than what I feel as a parent, which is pretty helpful because I want to do what’s important for my son even though he’s not old enough right now” (Caregiver B1). • “...My parents absolutely want the best for me, it’s just they don’t necessarily exactly understand how I feel” (Individual with DMD A3).
<p><i>Individuals with DMD Serve as the Experts</i></p>	<ul style="list-style-type: none"> • “There’s a lot of things that [caregivers] aren’t being prepared for, so I thought I was able to tell them that certain things may not be as easy as people might think” (Individual with DMD A2). • “It was more the patients themselves, like the older patients. So just understanding from their perspective things they’ve been through or how they see and feel things is definitely more important” (Caregiver A44). • “The individual with Duchenne, I definitely weighed their opinions more than I weighed the caregivers. Because they’re the ones living through it. So they’re the ones - but especially as adults, my children are younger - so as adults I like to hear their perspective on the care that they received” (Caregiver B31).
<p><i>Learning New Information and New Perspectives</i></p>	<ul style="list-style-type: none"> • “The hope is that you learned something from reading other people’s comments or at the least gives you a point of view into somebody else’s world of possibility. And that may not benefit you immediately, but at least in the future you can keep that in the back of your mind” (Caregiver B5). • “Right definitely and I think having that ability to compare responses with other people, like you’re also learning a lot from different perspectives” (Caregiver A42). • “I learned stuff that I didn’t really think about before and I learned about some treatment things that I didn’t really know about before” (Individual with DMD A14). • “But it is good to hear from the families too and to really use the system just for the support or it’s just very educational, informative and it was easy-to-use” (Caregiver A29).
<p><i>Opportunity to Change Original Responses</i></p>	<ul style="list-style-type: none"> • “I found this study to be methodologically more robust, in the sense that it wasn’t just asking your responses once. There was opportunity for feedback from the other participants and then finally, the third phase, they ask you to repeat the scoring and providing the rationale for why one chose the score that they chose to give each of the individual items” (Individual with DMD A1). • “I actually did like revising my answers. After reading everyone’s situations and their different opinions, I learned more about [different topics], so I was able to tweak my answers because it made me feel differently” (Caregiver B6). • “... But for some, it was a big deal because they are non-ambulatory and that makes a big difference when your peers are all towering over you when you’re standing, as opposed to when you’re sitting. So those kinds of answers, I did change a little because it was quite clear a lot of people thought that was important” (Caregiver B6). • “[Although we] were exposed to a new way of thinking [as a result of participating in Round 2], the answers never really changed that much” (Individual with DMD A2). • “I liked the fact that we shared answers with others and we were able to go back, re-evaluate and see what others thought and then revisit your answer. So, although I pretty much stayed the same on my answer, I did reevaluate and assess it” (Caregiver B7).
<p>4. PERCEIVED USEFULNESS AND IMPACT OF THE RPM</p>	
<p><i>Forum for DMD Families</i></p>	<ul style="list-style-type: none"> • “[ExpertLens™ is] super important, especially with muscular dystrophy, just because we really don’t know a ton about it” (Caregiver A8). • “What made it easy for me, knowing that it’s the same kind of people those were responding. They’re going through the same thing what I’m going through. It’s not somebody...it wasn’t really somebody who hasn’t experienced what we are going through. So that makes it feel like a community. So the more closer community that you have, the more likely that you’re going to open up to talk about everything. So, ExpertLens™ what I felt, it gave a sense of a community” (Caregiver A21). • “It was good information and good to kind of know what other—because we’re all over the country and it’s a rare disease. So as much as we want to connect with each other, I think it’s

	<p>really hard, because we're trying to take care of our kids and work and do whatever we have to do. So it's hard sometimes to kind of get together" (Caregiver B24).</p> <ul style="list-style-type: none"> • "And in general, our Duchenne community is pretty accepting of all the parents. We talk anyway, so it's pretty good. We've been pretty supportive of each other anyway. So I feel like in general, we're in a supportive community. So our answers, regardless if our name is on them or not, are pretty much everyone's going to react the same for the most part. So, I just felt comfortable pretty much answering mostly anything with this disease" (Caregiver B44).
ExpertLens™ Provided a Format for Incorporating the Patient and Caregiver Voice	<ul style="list-style-type: none"> • "It's really important to engage patients and their caregiver. I believe that only they have the firsthand experience and they can share their important insight. Because they are dealing with everyday life, they are facing challenges and they are creative. They are so creative that they are finding solutions to how to overcome the challenges" (Caregiver A9). • "I think [ExpertLens™] is really, really important because a lot of time the perspective and opinions of the caregivers, and especially patients, are not taken into account" (Individual with DMD B6). • "I think [ExpertLens™] is really good because sometimes if the physicians and the medical team are not dealing with things every day, they don't quite understand what is more important or less important to the patients and sometimes I think they have unrealistic goals and even treatments sometimes. And I think it's good that they hear from the caregivers" (Caregiver B31).
Empowered Patients and Caregivers	<ul style="list-style-type: none"> • "It brought up questions that I didn't even know to ask. It brought up topics that I wasn't aware of. It empowered me. It gave me a little bit of a say in what's going on. It made me feel better that the parents' perspective is being taken and that they're looking into these different areas, which is kind of cool" (Caregiver B10). • "[ExpertLens™] was really helpful to me in my next appointment because of the age that my kids are at. So certain things like puberty and all of that that were discussed and a lot of it was, "Well okay it's really up to you guys" (Caregiver A42).
Doctors Could Benefit from Patient and Caregiver Input to Support Current Standards and Recommendations	<ul style="list-style-type: none"> • "Once the guidelines are developed this way, they're going to be pretty useful for clinicians. It will really help them understand what really matters most and is important for the community" (Caregiver A1). • "I know the doctors get involved in the guidelines, but...they don't see it day in and day out. I think it's very important for the caregivers and the patients to have output and input on what the guidelines should be" (Caregiver B44). • "Some doctors are not introduced to Duchenne with the information and some of them, like my son was introduced at the age of nine - not fully, but some. It's important for them because I feel that clinicians still have trouble understanding that not all patients that are diagnosed with Duchenne will follow the same paths and will have the same way of thinking. So it's good to have a multitude amount of information so that they can gauge the individual that they're treating with a more caring approach" (Caregiver B19). • "Even though doctors are experts in it, they still maybe don't understand exactly what people go through. So it's good to have somebody who's experienced to tell you what they've done" (Individual with DMD A4).
5. CHALLENGES WITH THE EXPERTLENS™ PROCESS AND SUGGESTIONS FOR IMPROVING THE RPM	
Show Not Only Round 1 Responses to Rating Questions, but also Rationale Comments	<ul style="list-style-type: none"> • "It was difficult to determine what my initial [rationale] response was. I think it would have been better if I could have somehow seen what all my answers were in that category [of rationale comments] in Round 1" (Caregiver B06). • "So sometimes I don't know if my answer was different because I was having difficulty finding what I originally said. Like it wouldn't come up right in the box like, "This is what you said and this is what you rated." I kind of had to scroll through the comments" (Caregiver B10). • "Most of it. The only thing I would have hoped was on there is that instead of going back to find my answer, that it would have your rating in your answer right there" (Individual with DMD A4)
Need More Clarity in the Instructions	<ul style="list-style-type: none"> • "The instructions weren't exactly clear as to whether you have to respond to each one, just a few, or the ones you disliked or liked" (Caregiver B04). • "I guess just separate it out a little bit more, just step-by-step instead of here's all the instructions and then you're kind of thrown into it with a whole lot to do, maybe one thing at a time" (Caregiver A44).

	<ul style="list-style-type: none"> • “And I think it can be simplified. Compared to the other ones, the way they're set up, I think [ExpertLens™] needs some work with the way it's orientated and explained to the people that are participating” (Caregiver B19).
<p><i>Improve Email Digests</i></p>	<ul style="list-style-type: none"> • “Visually, I had to really focus to make sure I finished the page and that I clicked on each thing to make sure I could see all the responses” (Caregiver B11). • “Eventually I figured it out, but I would have liked it to be a little better organized so that you could see what people’s responses are and how the threads were organized” (Caregiver B12). • “I like your reminders but the digests, I mean I wish I could have gotten it less often. If I would have gotten a daily reminder that said, “Hey, don’t forget if you’ve got time, come in, answer more, add to it,” but when other people answered, I guess it didn’t really impact me because I wasn’t—like I said, we were super busy. I wasn’t going to take extra time right that second, but I knew that later I was going to anyway. And every time I would log in, I would just go back over everything anyways” (Caregiver A42). • “The digests saying where there’s new comments and stuff, to me that wasn’t really helpful because it was hard to tell specifically where the new comments were and stuff. Like I feel like if it would be possible to make it more apparent where there’s new conversation taking place” (Individual with DMD A2).
<p><i>Decrease Participation Burden</i></p>	<ul style="list-style-type: none"> • “It would be interesting if for the survey, you picked just one topic like either weight or height. Then, on days one and two, you put your comments in. On days three and four, you read through [the comments], and then on days five and six, you responded. If you were just focused on one topic, you can think about it a little bit more and focus in on it” (Caregiver A10). • “It was exhausting the complexity of the question that followed with another question that seemed going back to the first question. So to me that’s what wore me out was just almost hearing the same question but you wanted to know from a different perspective and from this perspective which I think is just, again, how some of the questions were reworded and that was kind of exhaustive” (Caregiver B19). • “It was exhausting the complexity of the question that followed with another question that seemed going back to the first question. So to me that’s what wore me out was just almost hearing the same question but you wanted to know from a different perspective and from this perspective which I think is just, again, how some of the questions were reworded and that was kind of exhaustive” (Caregiver B3)