5

Prioritizing parental worry associated with Duchenne muscular dystrophy using best-worst scaling

CHAPTER 5.

Prioritizing Parental Worry Associated with Duchenne Muscular Dystrophy using Best-Worst Scaling

Peay HL[1,2], Hollin IL[3], Bridges JFP[3]

1. Parent Project Muscular Dystrophy
Hackensack, NJ, USA

2. Department of Clinical Genetics
Leiden University Medical Centre
Leiden, The Netherlands

3. Department of Health Policy and Management
Johns Hopkins Bloomberg School of Public Health
Baltimore, MD, USA

ABSTRACT

Duchenne muscular dystrophy (DMD) is a progressive, fatal pediatric disorder with significant burden on parents. Assessing disease impact can inform clinical interventions.

Best-worst scaling (BWS) was used to elicit parental priorities among 16 short-term, DMD-related worries identified through community engagement. Respondents viewed 16 subsets of worries, identified using a balanced, incomplete block design, and identified the most and least worrying items. Priorities were assessed using best-worst scores (spanning +1 to -1) representing the relative number of times items were endorsed as most and least worrying. Independent-sample t-tests compared prioritization of parents with ambulatory and non-ambulatory children.

Participants (n=119) most prioritized worries about weakness progression (BW score 0.64 $p<0.001$) and getting the right care over time (0.25, $p<0.001$). Compared to parents of non-ambulatory children, parents of ambulatory children more highly prioritized missing treatments (0.31 vs. 0.13, $p<0.001$) and being a good enough parent (0.06 vs. -0.08, $p=0.01$), and less prioritized child feeling like a burden (-0.24 vs. -0.07, $p<0.001$).

Interventions to reduce negative parental impact may be most effective in conjunction with care-related interventions for the child, regardless of disease stage. We demonstrate an accessible, clinically-relevant approach to prioritize disease impact using BWS, which offers an alternative to the use of traditional rating/ranking scales.

INTRODUCTION

Understanding patients' and caregivers' experience of disease impact has implications for clinical care provision, public health programs, and policy development. The associations of perceived disease impact with health and psychosocial outcomes are well-described (for example, see Baines and Wittkowski 2013; McAndrew et al. 2014). Quantifying patients'/caregivers' preferences is an important issue for clinicians (dosReis et al 2014; Black 2013) and models of patient-centered care (Haywood 2006) and shared decision making (Politi and Street 2011) mark an increasing focus on assessing patients'/caregivers' perspectives and using the resulting data to inform healthcare delivery and decision making. Measuring patients'/caregivers' views avoids clinician/researcher bias and encourages a focus on reducing

symptoms, minimizing disability, and improving quality of life (Black 2014). Special attention to disease impact is included in the Food and Drug Administration's Patient-Focused Drug Development Program (U.S. Food and Drug Administration), raising the visibility and importance of assessing impact of disease for clinical trial sponsors and disease community stakeholders. These concepts are familiar to genetic counselors, for whom exploring a clients' lived experience is defined as a Practice-Based Competency (Accreditation Counsel for Genetic Counseling 2013).

In research settings, disease impact is often assessed using qualitative approaches or standard quantitative measures of severity, burden, and quality of life. In the regulatory context, patient and caregiver testimonial is another common approach. Each approach comes with strengths and limitations. Qualitative approaches are excellent for obtaining a deep and nuanced understanding of disease impact and often generate hypotheses to be tested in a generalized population in subsequent studies, while quantitative research using validated measures allow generalizable data to be systematically collected and compared across populations (Creswell et al. 2011; Razafsha et al. 2012). In this study we employed a quantitative stated preferences method, best-worst scaling (BWS), to prioritize disease impact in Duchenne muscular dystrophy (DMD).

Duchenne muscular dystrophy (DMD) is a rare, life-threatening disorder with pediatric onset (Bushby et al. 2010). Affected individuals, primarily males, have progressive loss of functional muscle fibers that result in weakness, loss of ambulation that typically occurs in the teen years, and premature death in the 20s-30s (Bushby et al. 2010; Flanigan 2014). Though the use of corticosteroids and advances in respiratory support and cardiac care have substantially impacted the health of individuals with DMD (Bushby et al. 2010; Eagle et al. 2002; Flanigan 2014), patients and parents are still faced with significant burden related to disease progression, ongoing care demands, and financial impact (Boyer et al. 2006; Daoud et al. 2004; Hatzmann et al. 2008; Kenneson and Bobo 2010; Landfeldt et al. 2014; Pangalila et al. 2012; Reid and Renwick 2001).

The study aimed to document parents' prioritization of short-term, disease specific worries when caring for an individual with DMD, and to identify if worry prioritization varies based on the child's ambulation status (representing disease progression). In addition, we describe parents'

physical and mental health status. Duchenne worry can be conceptualized as an emotion-focused illness representation, as defined by Leventhal's Common-Sense Model of Health and Illness Self-Regulation (McAndrew et al. 2008). The model proposes individuals as active problem-solvers who, when faced with a threat such as DMD in their children, engage in a dynamic process of developing and refining cognitive and emotion-focused illness representations that influence coping efforts (McAndrew et al. 2008). The data presented here are part of a larger project that also evaluated the treatment preferences of parent/guardian caregivers (Hollin et al. 2014; Peay et al. 2014a). An overarching objective of the research program was to model a replicable, community-engaged approach to obtaining preference and priority data from a sample of parents and guardians.

METHODS

A central aspect of the research program was the community-engaged approach that involved stakeholders in development of the survey instrument and dissemination of findings (Peay et al. 2014a). The study was lead by Parent Project Muscular Dystrophy (PPMD), an advocacy organization focused on finding a cure for Duchenne muscular dystrophy. A disease-community oversight team comprising PPMD staff (a clinician, a scientist experienced in drug development, and two caregivers of individuals with DMD) collaborated with the research team to design and implement the study.

We employed BWS Case 1 to prioritize worries when caring for an individual with DMD. Worry is defined as thoughts and images that are negatively affect-laden and relatively uncontrollable (Borkovec et al. 1983). Worry is clinically meaningful in that it may be related to an increase in behaviors that the worrier believes will protect his or her health, or in this case, the health of the child (McCaul and Goetz 2008); for example, Magnan and colleagues (2009) describe benefits of non-pathological worry that include motivation for positive health behaviors by increasing the salience of a health threat and acting as a cue to action.

BWS is a stated preference method grounded in Random Utility Theory that is based on how people make choices of extremes from within a choice set (Louviere and Flynn 2010). BWS Case 1, also known as the object case or object scaling, is relatively new to healthcare research (Flynn 2010). It is used to assess the relative preferences for a series of related items that could

otherwise be evaluated using a rating scale (Flynn 2010). In designing a BWS study a detailed set of related items, called attributes, are typically developed through qualitative stakeholder engagement (Bridges et al. 2011). The attribute set can be developed to include items that are each expected to be highly relevant to the majority of the study population. Instead of presenting these items as a scale and asking respondents for level of endorsement, which would likely result in highly skewed data and poor discriminative ability, BWS Case 1 quantifies prioritization among items selected to be highly relevant. Advantages of BWS over rating scales and ranking exercises have been summarized by Erdem and Rigby (2013).

In a BWS survey instrument, attributes are presented in subsets that are chosen based on a balanced incomplete block design (Ross et al. 2014) to ensure equal probability of selection for each attribute. The respondent is asked to select the most relevant or favorable (the “best”) and the least relevant or favorable (the “worst”) attribute among each subset. The underlying assumption is that this choice represents the farthest difference between the degree of importance among any items on an underlying ranking of item importance (Louviere and Islam 2008). An example choice task is shown in Figure I.

Consistent with our overarching objective, BWS represents a pragmatic methodology that allows deep understanding of one component of illness impact while also allowing quantitative ranking and group comparison. Though BWS is typically utilized to identify preferences among fact-based or cognitive attributes, there are examples of BWS being used to prioritize control-based and worry-based attributes, for example related to food safety (Erdem and Rigby 2013), and to prioritize among quality of life attributes (Flynn et al. 2007) that have affective components. By asking participants to choose among extremes, BWS minimizes the chances of introducing false assumptions about decision making (Flynn et al. 2007). BWS requires relatively low sample sizes, which is important for studies of rare disorder populations.

INSTRUMENT DEVELOPMENT

In designing the survey we used standards outlined in the ISPOR checklist for conjoint analysis (Bridges et al. 2011) and specific guidance on the use of BWS (Flynn 2010; Louviere and Flynn 2010). As described by Johnson and colleagues (2009), development of the attributes should include careful consideration of the disorder symptoms and severity; the target population

should be involved in development of attributes through qualitative methods; and the resulting experimental attributes should then be pilot tested and refined. However, to achieve our overarching aim of a replicable, feasible model, we were unable to employ extremely complex, time and resource intensive development processes, such as that described by Grewal and colleagues (2006). Instead we employed a pragmatic community engaged approach with input from a wide range of stakeholders.

PPMD invited stakeholder informants to participate in the survey development. We solicited open-ended responses from 5 highly-engaged parent advocates about their most important, current DMD-related worries. The parents were chosen to represent caregivers of children and adults with DMD of different ages and disease stages. The worry narratives provided by the parent informants were compiled, redundant items were eliminated, and statements were grouped under themes. These statements were evaluated, refined, and reduced by the PPMD oversight team and the study team, drawing on the diverse personal, clinical, and research experience of the teams; the determinations were informed by a review of the literature. The next step, thematic analysis and additional item reduction and refinement, resulted in a list of 16 worry items grouped under 4 worry domains—the child's affect and emotion; medical concerns about the child; family and social worries; and parent well-being. Patient advocates and experts representing neurology, clinical genetics, biopharmaceutical companies, and social/behavioral science reviewed and revised the items, which were finalized once no further amendments were suggested. Though there are no published studies specific to DMD-related worries, the final worry domains and items are supported by published literature about DMD impact and burden (Boyer et al. 2006; Daoud et al. 2004; Hatzmann et al. 2008; Kenneson and Bobo 2010; Landfeldt et al. 2014; Pangalila et al. 2012; Reid and Renwick 2001).
The final worry items were randomized into 16 combinations of response sets, each comprising 6 worry attributes. For each of the 16 combinations, participants were asked: "In the past 7 days, choose which you have been most worried about and which you have been least worried about." An example choice task is shown in Figure I.

Figure I. Sample choice task

For each list of worries, please tell us which one you have been most worried about in the past 7 days, and which one you have been least worried about in the past 7 days. Even if you are really worried about all of them, or not too worried about any of them, please choose the most and least worrying item. In the past 7 days, choose which you have been most worried about and which you have been least worried about.

Most Worried	Worries	Least Worried
○	My child getting weaker	○
○	Managing my uncertainty about my child's future	○
○	Affording care my child needs within the family budget	○
○	Having time for myself	○
○	My child feeling happy	○
○	My child having good friends	○

Cognitive interviews with seven parents of individuals with DMD of varying ages and disease stages were used to assess comprehension, refine terminology, and explore the acceptability of the instrument; this is described in detail elsewhere (Peay et al. 2014a). During the interviews,

participants endorsed the face validity of the worry items (i.e., the items represented their significant worries) and consistently indicated more difficulty choosing an item of least worry than and item of most worry, suggesting successful item development. The worry statements and domains are shown in Table I.

Table I. Worry items and domains

Worry Item	Domain
My child getting weaker	**Medical concerns**
Getting the right care for my child over time	
My child missing out on new treatments	
Affording care my child needs within the family budget	
My child feeling happy	**Child affect/emotion**
My child having good friends	
My child not being able to express deep worries	
My child feeling like burden on the family	
Managing my uncertainty about my child's future	**Parent wellbeing**
Being a good enough parent for my child	
Me handling the emotional demands of Duchenne	
Having time for myself	
The wellbeing of my other children	**Family and social**
My child becoming independent from me over time	
Effect of Duchenne on my closest relationships	
Feeling isolated from other families	

In addition to the BWS tasks, the survey included participant demographics and health status, measured using the SF-12 (Ware et al. 1996). The SF-12 is scored into Physical and Mental

Health Composite Scores (PCS and MCS, respectively) that range from 0 to 100, where a zero score indicates the lowest level of health and 100 indicates the highest level of health. Norm-based scoring facilitates interpretation in that individual respondent sores below 45 and group mean score below 47 are below the average population range (Optum SF-12v2 Health Survey: Advantages of Norm-Based Scoring). We also obtained information about the health status and care information for the child with DMD, including age, number of affected children in the family, ambulation status, prior research experience, and whether the child has experienced a life-threatening emergency.

PARTICIPANTS

Participants were parents or guardians of at least one living child with DMD. They lived in the United States, were at least 18 years of age, and were able to complete an online survey in English. The affected child could be any age or at any stage of disease.

The survey was administered online using the Qualtrics survey system from January 17, 2013 to February 21, 2013. Recruitment occurred using newsletter notices, social media, word-of-mouth, and through emails from Parent Project Muscular Dystrophy and the DuchenneConnect self-report registry. The anonymous survey was determined to be exempt by the Western Institutional Review Board (# 1-756840-1).

DATA ANALYSIS

The dependent variable in BWS is the participants' judgment about the extremes (in this case, most and least worrying items) in each profile that is presented to them (Molassiotis et al. 2012). The simplest analytic technique focuses on the number of times an attribute was chosen as best and as worst over all of the choice tasks. The analytic output, which we call the relative best-minus-worst (BW) score, can be calculated by subtracting the number of times a feature was chosen as worst from the number of times it was chosen as best, then dividing by the total number of times it was available to be chosen (Flynn et al. 2007).

Such simple methods have demonstrated a very high level of correlation with more complicated regression-based techniques (Gallego et al. 2012; Louviere and Flynn 2010). Like all techniques to estimate ordinal, multinomial outcomes, scoring assumes equal spacing between things that were chosen as best (BW score=1) and those chosen as worst (BW score=-1). The BW score is

estimated as a mean across the sample. This allows us to report the standard errors for these means and conduct t-tests to determine whether the scores were significantly different than zero. Additional information about the relative best-minus-worst analysis has been described elsewhere (Peay et al. 2014a).

Next, we conducted a stratified analysis by calculating BW scores for parents/guardians with ambulatory children and those with non-ambulatory children. We used Spearman's rho to compare the correlation of the rank order of worry items between the two groups. Finally, we conducted t-tests on BW scores for each worry, hypothesizing no statistically-significant differences across the ambulatory and non-ambulatory groups.

RESULTS

One hundred and nineteen caregivers who self-identified as being a parent or guardian of an individual with DMD included in the analysis. When dichotomized into an "ambulatory" group, defined as those who could walk independently outdoors for at least short distances, and a "non-ambulatory" group, defined as those who could not walk outdoors without help, 64% of children were in the ambulatory group and 36% in the non-ambulatory group. Table II summarizes the characteristics of the sample. Participants were predominately Caucasian, married, biological mothers, and had one affected child. There were no significant differences between the sample characteristics collected from the ambulatory and non-ambulatory groups except for ages of parent participants (M= 40.5, SD=6.1 for parents of ambulatory children versus M= 49.1, SD 7.1 for parents of non-ambulatory children, $p<0.01$) and their children (M= 8.8, SD 3.5 for ambulatory children versus M= 18.0, SD 6.3 for non-ambulatory children, $p<0.01$).

Table II. Characteristics of participants and affected child(ren) by ambulation status

	Ambulatory (n=76)	Non-Ambulatory (n=43)	P-value
Participant characteristics			
Parent age in years	40.5 (SD=6.1)	49.1 (SD=7.1)	<0.01
Child age in years	8.8 (SD= 3.5)	18.0 (SD=6.3)	<0.01
Parent characteristics			
Relationship to child(ren)			
Biological mother	68.4%	65.1%	0.72
Biological father	26.3%	32.6%	0.47
Adoptive mother	5.3%	0.0%	0.13
Adoptive father	0.0%	2.3%	0.18
Marital status			
Married/long-term relationship	93.4%	83.7%	0.09
Divorced/Separated	6.6%	14.0%	0.19
Widowed	0.0%	2.3%	0.18
Race			
Caucasian	89.5%	95.3%	0.27
Education			
High school/GED	6.6%	0.0%	0.09
Some college	10.5%	20.9%	0.12
Technical school	3.9%	7.0%	0.47
Associated degree	5.3%	11.6%	0.21
Four-year college degree	43.4%	41.9%	0.87
Graduate/professional degree	28.9%	18.6%	0.22
Income			
<$25,000	3.9%	9.3%	0.24
$25,000-$50,000	6.6%	11.6%	0.34
$50,000-$75,000	22.4%	11.6%	0.15
$75,000-$100,000	18.4%	18.6%	0.98
>$100,000	47.4%	46.5%	0.93
Child characteristics			
Number of affected children			
One child	94.7%	88.4%	0.21
Two or more children	5.3%	11.6%	0.21
Research participation			
Clinical research	53.3%	65.1%	0.22
Clinical trial	29.3%	41.9%	0.17
Had life-threatening emergency			
Yes	14.5%	25.6%	0.14

Ambulatory= ability to walk independently outside for at least short distances. Note: In some cases, percents do not add to 100% because of missing values.

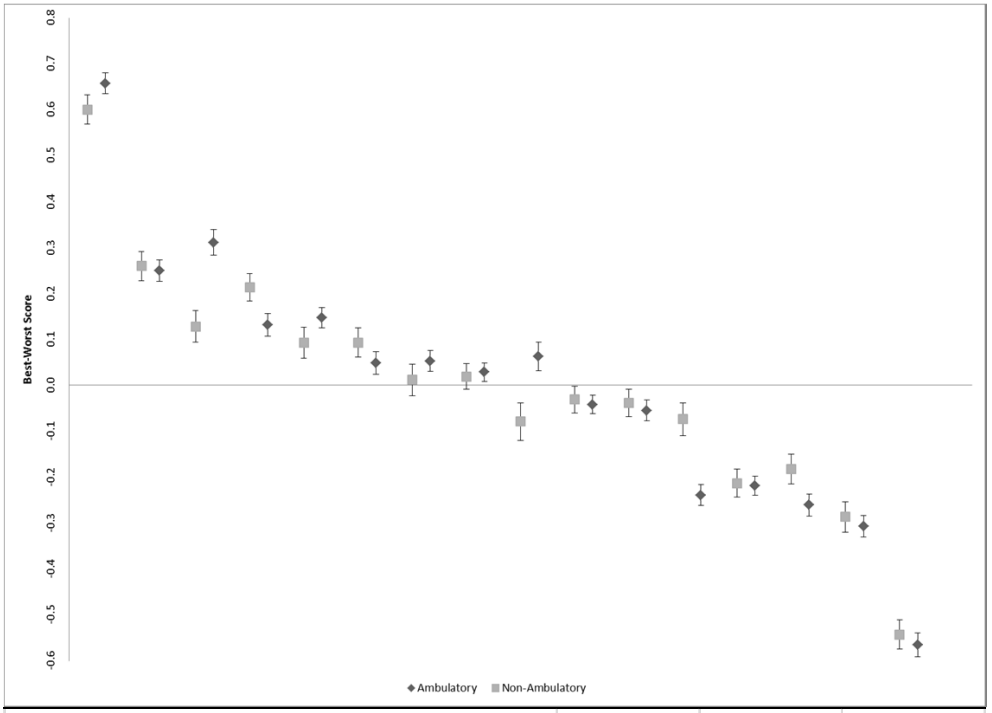
The SF-12 health status results indicate that our participants were physically healthier than the general population, with physical component scores above normative scores in 72% and at or below norm in 28%. However, 57% of participants had mental component scores (MCS) that were below the norm, 35% in the normative range, and 8% above the norm. 45 participants (40%) were identified to be at increased risk for depression compared to 20% in the normative group. The mean MCS for parents of non-ambulatory children (M=44.4, SD 11.9) was significantly higher than the mean score for parents of ambulatory children (M=39.8, SD=11.7) on independent-samples t-test, t (117)= 2.05, p=0.04, two tailed.

WORRY PRIORITIZATION

Figure II shows the worry prioritization task's best-worst scores. In the total group, worry about "my child getting weaker" was identified as most concerning (BW score 0.64, p<0.001). Respondents also prioritized "getting the right care for my child over time" (0.25, p<0.001) and "child missing out on new treatments" (0.25, p<0.001); each of the three most prioritized items were under the "medical concerns" domain. "My child feeling happy" was the most prioritized of the child affect/emotion domain (0.16, p<0.001). "Managing my uncertainty about my child's future" was the most prioritized of the parent wellbeing domain (0.13, p<0.001). In the family and social domain, which overall was not highly prioritized compared to the other domains, "the wellbeing of my other children" was the most prioritized (-0.04, p=.01). The least prioritized items were "Having time for myself" (-0.56, p<0.001) and "Feeling isolated from other families" (-0.30, p<0.001).

Priorities for parents of ambulatory and non-ambulatory children are also shown in Figure II. Parents of ambulatory children prioritized "missing out on new treatments" significantly more than parents of non-ambulatory children, t (118)= 3.34, p<0.001, two tailed. Parents of ambulatory children were also more likely to prioritize "being a good enough parent" than parents of non-ambulatory children t (118)= 2.50, p=0.01, two tailed. In contrast, parents of non-ambulatory children were more likely to prioritize "child feeling like a burden on the family" than parents of ambulatory children, t (118)= 3.50, p<0.001, two tailed. Finally, in comparing the rank ordering of worry items between groups, the correlation was high with a Spearman's rho of 0.90, p<0.001.

Figure II. Worry prioritization by ambulation status



Attribute	Complete sample Best-worst score	P-value	95% CI
My child getting weaker	0.637	0.000	(-0.67 , -0.60)
Getting the right care for my child over time	0.254	0.000	(-0.29 , -0.22)
My child missing out on new treatments	0.245	0.000	(-0.29 , -0.20)
My child feeling happy	0.161	0.000	(-0.20 , -0.12)
Managing my uncertainty about my child's future	0.127	0.000	(-0.16 , -0.09)
Afforiding care my child needs within the family bud	0.065	0.000	(-0.10 , -0.03)
My child having good friends	0.038	0.025	(-0.08 , 0.00)
My child not being able to express deep worries	0.025	0.061	(-0.06 , 0.01)
Being a good enough parent for my child	0.012	0.319	(-0.06 , 0.04)
The wellbeing of my other children	-0.038	0.013	(0.00 , 0.07)
Me handling the emotional demands of Duchenne	-0.049	0.003	(0.01 , 0.08)
My child feeling like a burden on the family	-0.179	0.000	(0.14 , 0.22)
Effect of Duchenne on my closest relationships	-0.217	0.000	(0.18 , 0.25)
My child becoming independent from me over time	-0.232	0.000	(0.20 , 0.27)

DISCUSSION

Assessing disease impact is necessary to understand the experience of patients and caregivers, improve care provision, and inform policy. In our study describing parental DMD-related worry, respondents ranked the child's disease management as of greatest immediate worry, followed by worry about the child's affect. The impacts of DMD on the family and parents' wellbeing were less prioritized. In interpreting the results it is important to keep in mind a primary strength of Best-Worst Scaling: it allows prioritization among items that may each be valued. Thus family and parent wellbeing may be highly important, but comparatively not as important as child disease management.

Parents of non-ambulatory children had higher SF-12 MCS scores than parents of ambulatory children, which suggests adaptation to the disorder over time. Overall, the worries prioritization was similar between groups. Though both groups prioritized child medical items, the higher prioritization of worry about "missing out on new treatments" by parents of ambulatory children may reflect increased optimism for new treatment opportunities juxtaposed with a perception of a limited window during which treatment may be most effective (Peay et al. 2014b). In contrast, parents of non-ambulatory children may be resigned to fewer treatment opportunities for their children (Murray 2014) and focus more worry on their children feeling like a burden on the family as their symptoms progress.

STUDY LIMITATIONS

There are several limitations to the study. First, the recruitment of caregivers through advocacy groups, while pragmatic and efficient, has a risk of selection bias. Second, we did not publish the preliminary, qualitative work where we identified the worry items and domains, though this has been done for other studies (for example, Ross et al., 2014) and is ideal. In addition, the domains were determined based on expert consensus and in future studies should be validated. Third, we conducted an aggregate analysis and pre-specified stratification by ambulation status, and important structures in preference heterogeneity may have been overlooked. Future research should consider a larger sample size to allow for additional stratification and segmentation analysis to adequately describe preference heterogeneity. Finally, future research should elicit DMD-related priorities from affected teenagers and adults, anticipating that DMD patients and caregivers may not agree on impact on quality of life (Usark et al. 2012).

PRACTICE IMPLICATIONS

We found high prioritization of worries about disease management in caregivers who manage their children's DMD and make treatment decisions. Taken together with the parents' SF-12 results, the data present a parent population exhibiting negative psychosocial impact that is likely related to caring for a child with DMD. For genetics providers and other health professionals, a primary goal in assessing illness representations is to identify outcomes that are potentially alterable and inform service provision (Hale et al. 2007). Regardless of the child's disease progression, the strong prioritization of worries about weakness progression suggests that interventions that aim to reduce negative psychosocial impact on parents may be most effective when provided in conjunction with care-related interventions for the affected child.

RESEARCH RECOMMENDATIONS

To our knowledge this study represents the first published use of BWS to prioritize among emotion-focused illness representations. Here we present a feasible, replicable community-engaged approach that demonstrates how BWS Case 1 provides an appealing alternative to quantitative rating scales. BWS Case 1 has been shown to outperform rating scales (Lee et al. 2007) and it has a particular strength in requiring participants to discriminate among items (Louvierre and Flynn 2010). It may be especially compelling for use in clinical settings because it allows estimation of preferences at an individual patient level (Louvierre and Flynn 2010), facilitating the development of clinical interventions. As we have shown, BWS benefits from a straightforward analytic approach without the need for specialized software and the results are intuitively understood. Thus BWS represents a rich, accessible analytic tool for clinicians and clinical researchers that can be used effectively across a wide range of clinical applications.

ACKNOWLEDGEMENTS

We appreciate the leadership and commitment of the Parent Project Muscular Dystrophy oversight committee: Pat Furlong, Brian Denger, Sharon Hesterlee, and Kathleen Kinnett. We are indebted to the stakeholder informants, parents who participated in the cognitive interviews, and caregivers who completed the survey. We acknowledge Aad Tibben's review and comments and Hadar Scharff's assistance with data cleaning and analysis of the SF-12 data.

CONFLICT OF INTEREST

This study was funded by Parent Project Muscular Dystrophy (PPMD). Holly Landrum Peay is an employee of PPMD and John FP Bridges was hired as a consultant by PPMD for this project. Ilene L. Hollin has no conflicts to disclose.

HUMAN STUDIES AND INFORMED CONSENT

All procedures followed were in accordance with the ethical standards of the responsible committee on human experimentation (institutional and national) and with the Helsinki Declaration of 1975, as revised in 2000 (5). Informed consent was obtained from all patients for being included in the study.

REFERENCES

Accreditation Counsel for Genetic Counseling. (2013). Practice-Based Competencies for Genetic Counselors. Retrieved from http://www.gceducation.org/Documents/ACGC%20Core%20Competencies%20Brochure_13-Web-Revised-FINAL.pdf. Accessed December 2014.

Baines, T. & Wittkowski, A. A systematic review of the literature exploring illness perceptions in mental health utilising the self-regulation model**.** *Journal of Clinical Psychology in Medical Settings, 20(3)*, 263-274.

Black, N. (2013). Patient reported outcome measures could help transform healthcare. *British Medical Journal, 346,* f167.
Borkovec, T.D., Robinson, E., Pruzinsky, T., & Dupree, J.A. (1983). Preliminary exploration of worry: Some characteristics and processes. *Behaviour Research and Therapy*, 23, 481-482.
Boyer, F., Drame, M., Morrone, I. & Novella, J.L. (2006). Factors relating to carer burden for families of persons with muscular dystrophy. *Journal of Rehabilitation Medicine, 38(5),* 309-315.
Bridges, J.F., Hauber, A.B., Marshall, D., Lloyd, A., Prosser, L.A., Regier, D.A., Johnson, F.R. & Mauskopf, J. (2011). Conjoint analysis applications in health--a checklist: a report of the ISPOR Good Research Practices for Conjoint Analysis Task Force. *Value in Health,14(4),* 403-413.
Bushby, K., Finkel, R., Birnkrant, D.J., Case, L.E., Clemens, P.R., Cripe, L., Kaul, A., at al. (2010). DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurology ,9(1),* 77-93.
Creswell, J.W., Klassen, A.C., Plano Clark, V.L. & Smith, K.C. for the Office of Behavioral and Social Sciences Research. (2011). Best practices for mixed methods research in the health sciences. National Institutes of Health. Retrieved from http://obssr.od.nih.gov/mixed_methods_research. Accessed December 2014.
Daoud, A., Dooley, J.M. & Gordon, K.E. (2004). Depression in parents of children with Duchenne muscular dystrophy. *Pediatric Neurology, 31(1),* 16-19.
dosReis, S., Ng, X., Frosch, E., Reeves, G., Cunningham, C. & Bridges, J. (2015) Using best-worst scaling to measure caregiver preferences for managing their child's ADHD: A pilot study. The Patient, Epub ahead of print, doi: 10.1007/s40271-014-0098-4.
Eagle, M., Baudouin, S.V., Chandler, C., Giddings, D.R., Bullock, R. & Bushby, K. (2002). Survival in Duchenne muscular dystrophy: improvements in life expectancy since 1967 and the impact of home nocturnal ventilation. *Neuromuscular Disorders,12(10),* 926-929.
Erdem, S. & Rigby, D. (2013). Investigating heterogeneity in the characterization of risks using best worst scaling. *Risk Analysis*, *33(9),* 1728-1748.
Flanigan, KM. (2014). Duchenne and Becker muscular dystrophies. *Neurologic Clin*ics *32(3),* 671-688.
Flynn, T. N., Louviere, J. J., Peters, T. J., & Coast, J. (2007). Best worst scaling: what it can do for health care research and how to do it. *Journal of Health Economics, 26(1),* 171-89.
Flynn, T.N. (2010). Valuing citizen and patient preferences in health: recent developments in three types of best-worst scaling. *Expert Review of Pharmacoeconomics & Outcomes*

Research, 10(3), 259-267.
Gallego, G., Bridges, J.F., Flynn, T., Blauvelt, B.M. & Niessen, L.W. (2012). Using best-worst scaling in horizon scanning for hepatocellular carcinoma technologies. *International Journal of Technology Assessment in Health Care, 28(3),* 339-346.
Grewal, I., Lewis, J., Flynn, T., Brown, J., Bond, J. & Coast, J. (2006). Developing attributes for a generic quality of life measure for older people: preferences or capabilities? *Social Science in Medicine, 62(8),* 1891-1901.
Johnson, F.R., Hauber, A.B., & Poulos, C.M. (2009). A brief introduction to the use of stated-choice methods to measure preferences for treatment benefits and risks. RTI Press publication No. RR-0009-0909. Research Triangle Park, NC: RTI International. Retrieved from http://www.rti.org/rtipress. Accessed December 2014.
Hale, E.D., Treharne, G.J. & Kitas, G.D. (2007). The Common-Sense Model of self-regulation of health and illness: how can we use it to understand and respond to our patients' needs? *Rheumatology, 46,* 904–906.
Hatzmann, J., Heymans, H.S., Ferrer-i-Carbonell, A., van Praag, B.M. & Grootenhuis, M.A. (2008). Hidden consequences of success in pediatrics: parental health-related quality of life—results from the Care Project. *Pediatrics, 122(5),* e1030-1038.
Haywood K.L. (2006). Patient-reported outcome I: measuring what matters in musculoskeletal care. *Musculoskeletal Care, 4(4),* 187-203.
Hollin, I.L., Peay, H.L. & Bridges, J.F. (2014). Caregiver Preferences for Emerging Duchenne Muscular Dystrophy Treatments: A Comparison of Best-Worst Scaling and Conjoint Analysis. *The Patient, Dec 19*. Epub ahead of print.
Kenneson, A. & Bobo, J.K. (2010). The effect of caregiving on women in families with Duchenne/Becker muscular dystrophy. *Health & Social Care in the Community, 18(5),* 520-528.
Landfeldt, E., Lindgren, P., Bell, C.F., Schmitt, C., Guglieri, M., Straub, V., Lochmüller, H. & Bushby, K. (2014). The burden of Duchenne muscular dystrophy: An international, cross-sectional study. *Neurology, 83(6),* 529-536.
Lee, J.A., Soutar, G. & Louviere, J.J. (2007). Measuring values using best worst scaling: the LOV example. *Psychology & Marketing, 24 (12),* 1043-1058.
Louviere, J.J. & Flynn, T.N. (2001). Using best- worst scaling choice experiments to measure public perceptions and preferences for healthcare reform in Australia. *The Patient, 3,* 275–283.
Louviere, J.J., & Islam T. (2008). A comparison of importance weights and willingness-to-pay measures derived from choice-based conjoint, constant sum scales and best–worst scaling.

Journal of Business Research, 61(9), 903-911.
Magnan, R.E., Köblitz, A.R., Zielke, D.J. & McCaul, K.D. (2009). The effects of warning smokers on perceived risk, worry, and motivation to quit. *Annals of Behavioral Medicine, 37(1),* 46-57.
McAndrew, L.M., Mora, P.A., Quigley, K.S., Leventhal, E.A. & Leventhal. H. (2014). Using the common sense model of self-regulation to understand the relationship between symptom reporting and trait negative affect. *International Journal of Behavioral Medicine, 21(6),* 989-994.
McAndrew, L.M., Musumeci-Szabo, T.J., Mora, P.A., Vileikyte, L., Burns, E., Halm, E.A., Leventhal, E.A. & Leventhal, H. (2008). Using the common sense model to design interventions for the prevention and management of chronic illness threats: from description to process. *British Journal of Health Psychology, 13(2),* 195**–**204.
McCaul, KD & Goetz, P.W. (2008). Worry. *Health Behavior Constructs: Theory, Measurement & Research,* National Cancer Institute. Retrieved from http://cancercontrol.cancer.gov/brp/constructs/worry/index.html. Accessed December 2014.
Molassiotis, A., Emsley, R., Ashcroft, D., Caress, A., Ellis, J., Wagland, R., Bailey, C.D., at al. (2012). Applying Best-Worst scaling methodology to establish delivery preferences of a symptom supportive care intervention in patients with lung cancer. *Lung Cancer, 77(1),* 199-204.
Murray, L. (2014). Left out in the cold: Barriers to clinical trial participation and the impact on parents of sons with Duchenne muscular dystrophy. Published Abstract, National Society of Genetic Counselors Annual Conference, Presented September 20, 2014.
Optum SF-12v2 Health Survey: Advantages of Norm-Based Scoring. (n.d.) Retrieved from https://www.optum.com/content/dam/optum/resources/Manual%20Excerpts/Norm-based%20Scoring%20(NBS).pdf. Accessed December 2014.
Pangalila, R.F., van den Bos, G.A., Stam, H.J., van Exel, N.J., Brouwer, W.B. & Roebroeck, M.E. (2012). Subjective caregiver burden of parents of adults with Duchenne muscular dystrophy. *Disability and Rehabilitation, 34(12),* 988-996.
Peay, H.L., Hollin, I., Fischer, R. & Bridges, J.F. (2014a). A community-engaged approach to quantifying caregiver preferences for the benefits and risks of emerging therapies for Duchenne muscular dystrophy. *Clinical Therapeutics, 36(5),* 624-637.
Peay, H.L., Tibben, A., Fisher, T., Brenna, E. & Biesecker, B.B. (2014b). Expectations and experiences of investigators and parents involved in a clinical trial for Duchenne/Becker muscular dystrophy. *Clinical Trials, 11(1),* 77-85.
Politi M.C. & Street R.L. (2011). The importance of communication in collaborative decision

making: facilitating shared mind and the management of uncertainty. *Journal of evaluation in clinical practice,17(4),* 579-584.

Razafsha, M., Behforuzi, H., Azari. H., Zhang, Z., Wang, K.K., Kobeissy, F.H. & Gold, M.S. (2010). Qualitative versus quantitative methods in psychiatric research. *Methods in Molecular Biology, 829*, 49-62.

Reid, D.T. & Renwick, R.M. (2001). Relating familial stress to the psychosocial adjustment of adolescents with Duchenne muscular dystrophy. *International Journal of Rehabilitation Research, 24(2),* 83-93.

Ross, M., Bridges, J., Ng, X., Wagner, L., Frosch, E., Reeves, G. & dosReis. S. (2015) A best-worst scaling experiment to prioritize caregiver concerns with attention deficit/hyperactivity disorder (ADHD) medicine for children, *Psychiatric Services*, Epub ahead of print, doi: 10.1176/appi.ps.201300525.

U.S. Food and Drug Administration (FDA). (n.d.) Enhancing benefit-risk assessment in regulatory decision-making. Retrieved from http://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm326192.htm. Accessed March 2014.

Uzark, K., King, E., Cripe, L., Spicer, R., Sage, J., Kinnett, K., Wong, B., et al. (2012). Health-related quality of life in children and adolescents With Duchenne muscular dystrophy. *Pediatrics,130,* 1–8.

Ware, J.E., Kosinski, M. & Keller, S.D. (1996). A 12 Item Short Form Health Survey: Construction of scales and preliminary tests of reliability and validity. *Medical Care, 34,* 220-233.