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The RAND/PPMD Patient-Centeredness Method: a novel online approach to engaging patients and their representatives in guideline development

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Abstract

Although clinical practice guidelines (CPGs) provide recommendations for how best to treat a typical patient with a given condition, patients and their representatives are not always engaged in CPG development. Despite the agreement that patient participation may improve the quality and utility of CPGs, there is no systematic, scalable method for engaging patients and their representatives, as well as no consensus on what exactly patients and their representatives should be asked to do during CPG development. To address these gaps, an interdisciplinary team of researchers, patient representatives, and clinicians developed the RAND/PPMD Patient-Centeredness Method (RPM) - a novel online approach to engaging patients and their representatives in CPG development. The RPM is an iterative approach that allows patients and their representatives to provide input by (1) generating ideas; (2) rating draft recommendations on two criteria (importance and acceptability); (3) explaining and discussing their ratings with other participants using online, asynchronous, anonymous, moderated discussion boards, and (4) revising their responses if needed. The RPM was designed to be consistent with the RAND/UCLA Appropriateness Method used by clinicians and researchers to develop CPG, while helping patients and their representative rate outcome importance and recommendation acceptability - two key components of the GRADE Evidence to Decision (EtD) framework. With slight modifications, the RPM has the potential to explore consensus among key stakeholders on other dimensions of the EtD, including feasibility, equity, and resource use.

Keywords

Care experience, clinical practice guidelines, Duchenne Muscular Dystrophy, patient-centeredness, patient participation/engagement, patient preferences, patient values, person-centered healthcare, RAND/PPMD Patient-Centeredness Method

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Introduction

Clinical practice guidelines (CPGs) provide recommendations for how best to treat a typical patient with a given condition [1] and therefore play an important role in ensuring the delivery of high quality healthcare to

patients [2,3]. The process of CPG development, however, does not always include patients or their representatives [4]. Failure to recognize the needs and preferences of individuals with expertise in the “lived experience” of a given medical condition can negatively affect guideline quality, usefulness, legitimacy, and adherence. Conversely, patient inclusion in CPG development can help ensure that

guidelines focus on topics, and outcomes important to patients, comprehensively identify risks and benefits of different recommendations, and address the feasibility and acceptability of care recommendations [5,6]. Research shows that patients and clinicians view the risk-benefit trade-off differently and patients and their families provide unique perspectives that may differ from factors that clinicians are trained to take into account [7,8]. Engaging all stakeholders with a legitimate interest in guideline development helps ensure that guidelines are created in a transparent, democratic manner and are acceptable to relevant stakeholder groups [9].

Patients and their representatives can be involved at different stages of the process [10], ranging from topic selection and reviewing and grading the strength of evidence to developing recommendations [11]. They can also be asked their views on living with their condition, accessing services, perceived benefits and harms of treatment options, or clinical outcomes of importance [12,13]. Asking patients to join the evidence review group or to submit evidence that would be considered for guideline development [12] could broaden the range of evidence that the guideline developers consider. Engaging patients in reviewing existing studies on patient preferences and soliciting input in designing data collection instruments can help identify areas where patients and caregivers feel guideline recommendations are most needed [14].

But with some exceptions, involving patients in GDGs typically means including only a few patient representatives rather than proactively engaging a wider group of patients, as budgetary and logistical constraints typically make broad outreach infeasible [15,16]. There is no consensus about how patients should participate in CPG development - for example, should they be active members of guideline groups, or should patient input and preferences be shared only with clinicians in guideline groups [17]? Moreover, there is little clarity about how guidelines should reflect patient-based evidence, or information generated by patients about different aspects of care, patient preferences, and care experiences [18,19].

Despite the realization that broad patient participation may improve the quality and utility of CPGs [20], there is no systematic, scalable method for engaging patients and no agreement on what exactly patients and their representatives should be asked to do during guideline development. Research is needed to develop and evaluate a scalable, non-burdensome, and culturally appropriate way to involve patients and their caregivers in developing CPGs. The method should be consistent with the approach clinicians use to develop consensus or evidence-based CPGs and useful for soliciting input from even hard-to-engage patient populations.

To address this challenge, a team of researchers from RAND and clinicians, caregivers, and patients from the Parent Project Muscular Dystrophy (PPMD) developed the RAND/PPMD Patient-Centeredness Method (RPM) and tested it using recently revised Duchenne Muscular Dystrophy (DMD) care considerations [21-23], or care guidelines, as they are commonly called in the Duchenne community. Clinical experts on DMD used a consensus-

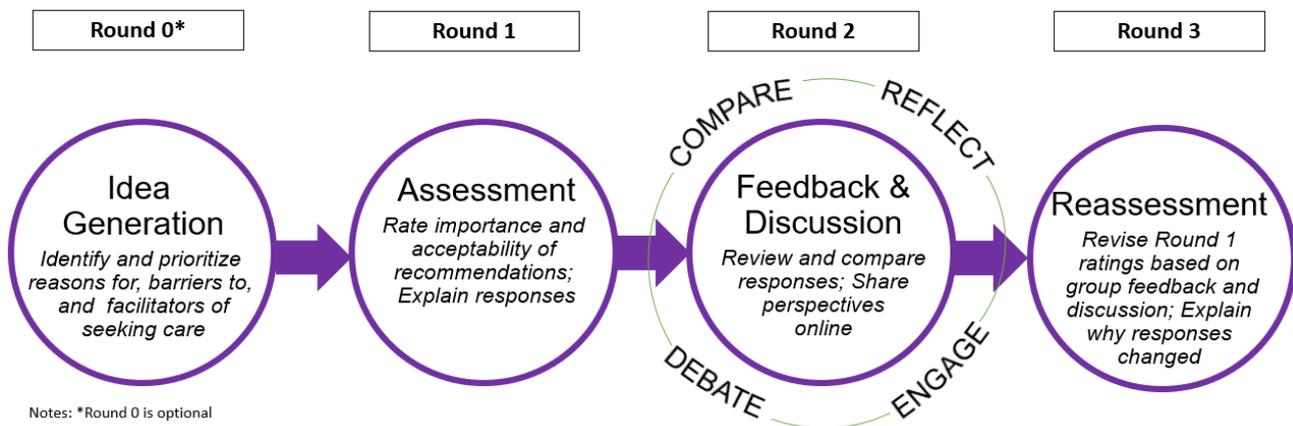
development method known as the RAND/UCLA Appropriateness Method (RAM) or modified-Delphi method [1], which involves reviewing existing evidence, rating the clinical appropriateness and necessity of different assessment and treatment options, discussing the rating results, and revising the original ratings if needed. The revised set of care considerations covers 11 domains of care. However, patients and caregivers were consulted in developing recommendations for only one domain. The lack of patient and caregiver participation in developing the 2018 DMD care considerations offered an opportunity to develop and test a new method for involving a large group of patients and caregivers in CPG development. We did so by engaging individuals with DMD and their caregivers; experts on guideline development, RAM, and DMD; as well as clinicians and genetic counselors working with Duchenne families [24].

Patient-centeredness of guideline recommendations

While clinicians and researchers have the most expertise in identifying clinically appropriate and necessary treatments, patients and caregivers are best positioned to judge the extent to which the guideline recommendations are patient-centered. According to the Institute of Medicine (IOM), patient-centered care is defined as “care that is respectful of, and responsive to, individual patient preferences, needs and values, and ensuring that patient values guide all clinical decisions” [25]. When asked about what makes care patient-centered, our study participants agreed with this definition and stated that patient-centered care is evidence-based care provided by clinicians who listen to, understand, and account for patients’ care needs, preferences, and values. They also felt that patient-centered care is team-based and requires inclusion of patients and caregivers as active decision-makers who work together with doctors on developing care plans. “Healthcare is patient-centered when educated patients and family present ideas, concerns, and beliefs to the practitioner, and the whole team comes together to create a plan of care”, said one caregiver.

From the perspective of clinicians, researchers, and guideline developers we interviewed, patients and caregivers can help ensure patient-centeredness of guideline recommendations in three ways. First, their input can help guideline developers determine what care outcomes should be considered, whether the outcomes included in the guidelines are important to patients, and whether the selected outcomes are likely to positively impact their quality of life. Because clinicians may not necessarily think about quality of life issues during the guideline development process, including patients can help make guidelines more relevant to their needs and preferences. Second, patients and caregivers can help clinicians and guideline developers better understand emotional and physical burdens related to getting care. Some treatment options may be psychologically taxing, while others physically challenging. For example, patients

Figure 1 The RAND/PPMD Patient-Centeredness Method (RPM)



taking glucocorticoids to delay loss of ambulation, preserve upper body function, and/or delay or prevent scoliosis, have to deal with such potential negative side effects of this treatment as weight gain, delayed puberty, short stature, bone loss and behavioral changes. Moreover, many bone health assessments, such as DEXA scans and X-rays, may be physically challenging to individuals with Duchenne because they require the ability to lay still. Finally, patients can provide important contextual information that can affect feasibility of, and likely adherence to, guideline recommendations. Patient and caregiver input can help ensure that the recommendations are realistic, focusing on such issues as convenience, accessibility, and costs.

Taken together, these findings suggest that one of the most useful ways patients and caregivers could contribute to guideline development is by providing their input on two key components of patient-centered care: (1) the importance of the outcomes that a particular care recommendation is trying to achieve for a typical patient and (2) the acceptability of the process of following the recommendation for a typical patient. We consider the importance and acceptability to be the patient equivalent or analogous to the appropriateness and necessity that experts are focusing on in the RAM.

The RAND/PPMD Patient-Centeredness Method

The RPM is a novel online modified-Delphi approach to patient engagement in CPG development that allows patients and their representatives to provide input on the patient-centeredness of draft guideline recommendations by participating in an optional idea generational round, two rating rounds, and an online discussion round. Ideally, patient and caregiver input should be solicited before the guidelines are finalized to ensure that recommendations are based on the systematic review of existing evidence and clinical expertise and are consistent with patients' care preferences and needs.

The RPM is based on the Delphi method [26], the RAM [1], and the GRADE Evidence to Decision Framework (EtD) [6,27]. The RPM borrows 3 main features from the Delphi method: an iterative approach to data collection, participant anonymity, and controlled feedback on how each participant's own responses compare to those of the group. The RPM borrows 3 main features from RAM: 9-point rating scales, the RAM approach to measuring consensus, and direct interaction among participants. Finally, the RPM adapts its operationalization of patient-centeredness from the GRADE EtD: namely the importance and acceptability of guideline recommendations as key areas for which patients have the most expertise.

Designed to solicit input from large and diverse groups, the RPM is best implemented online to ensure convenience and efficiency of data collection and analysis. An online data collection platform with survey, automated data analysis, and discussion functionalities, such as ExpertLens™ [28], is needed for the RPM implementation.

The RPM requires iterative data collection. Patients and caregivers provide their input by generating ideas, rating and commenting on draft recommendations, discussing preliminary rating results, and revising their responses based on the panel's preliminary ratings and group discussions. The RPM consists of the following 3-4 rounds (Figure 1).

In an optional **Round 0**, patients and caregivers answer a series of open-ended and close-ended questions and engage in an online discussion about reasons for, barriers to and facilitators of seeking care for a given medical problem. This round helps generate information about care preferences, needs, and values that may not be available in the published literature. Once these data are summarized, the results are shared with panelists in subsequent rounds to help them rate patient-centeredness.

In **Round 1**, participants rate and comment on draft recommendations already deemed clinically appropriate and necessary by experts. The recommendations should be presented in an easy-to-understand format and include a brief description of the clinical rationale, the process of

following the recommendation, and any additional relevant information, such as treatment burden and side effects.

Participants use 9-point Likert scales to rate patient-centeredness of each recommendation, operationalized as the importance and acceptability. The end points of each scale are labeled as 1: not very important/acceptable and 9: very important/acceptable. Importance is defined as the extent to which a recommendation is likely to be consistent with the preferences, needs, and values of a typical patient with a given condition. Acceptability is defined as the extent to which the process of following a given recommendation is likely to be consistent with available resources (e.g., time and finances) and with the ethical standards of a typical patient with a given condition.

To help participants comment about patients in general, it is important to provide them with a description of patient preferences and needs that is either based on the literature review or identified as part of Round 0. Such information may also include a description of common barriers and facilitators of seeking care for a given condition.

In addition to providing numeric responses, participants should explain the rationales behind their ratings by identifying the factors that affected their perspectives on importance and acceptability of each recommendation the most. Therefore, each rating question should be followed by a text box where participants could type their rationale comments.

In **Round 2**, participants review and discuss Round 1 results. Participants review charts showing the distribution of group responses, their own response, and the group median response. Rating data are analyzed to determine the existence of group consensus using the analytic approach described in the RAND/UCLA Appropriateness Method User's Manual [1]. To ensure that the results of these analyses are easy-to-understand, color-coding of group decisions and hover-overs are used to show if consensus has been reached and to provide explanations of different statistical terms.

In addition, rationale comments are summarized thematically to explain how and why patients and caregivers rated a recommendation. To ensure consistency between the analysis of rating data and rationale comments, summarizing qualitative data by rating tertiles is helpful. To illustrate, the analysis of comments from participants who rated a given recommendation as 7, 8, or 9 provides a summary of why a recommendation was deemed important or acceptable.

Participants also discuss Round 1 results using asynchronous, (partially) anonymous, and moderated discussion boards with a threaded structure. Asynchronous nature of discussions makes participation more convenient to participants and facilitates engagement across time zones. Using alpha-numeric participant IDs (i.e., patient 01 or caregiver 12) that reveal a participant's stakeholder group and assign a unique number helps ensure participant anonymity, while allowing for easy identification of all comments made by a given participant. Discussions are moderated to promote exchange of ideas and clarification of rationale comments.

In **Round 3**, participants are given an opportunity to review Round 2 discussion and modify their original

answers if they wish to do so. The wording of recommendations or any text that accompanies them could be modified based on Round 2 results. Such changes should be clearly identified. Participants are encouraged to explain why their responses changed using open-text boxes displayed after each rating question.

Discussion and Conclusions

The RPM offers a unique opportunity for guideline developers to engage large and diverse groups of patients, caregivers, and other relevant stakeholders in the process of developing guideline recommendations by soliciting their input on the patient-centeredness of draft recommendations using an online, modified-Delphi process. Designed to be consistent with the way clinicians and researchers participate in guideline development, the RPM helps collect information on outcome importance and treatment acceptability from the perspectives of patients and caregivers, which are two components of the EtD framework [6,27]. With slight modifications in rating criteria and the extension of the pool of invited participants, the RPM can be used to explore the existence of consensus among key stakeholders on other dimensions of the EtD, such as feasibility, equity, and resource use.

Our team evaluated participation experiences of the individuals with DMD and caregivers who helped us test the RPM. The results of satisfaction surveys and semi-structured interviews that are reported elsewhere in this issue of the *Journal* [29] suggest that participants had good experiences with the RPM, "citing the convenience, anonymity, and asynchronous nature of online engagement", commenting on the benefits of learning from the experiences of both patients and caregivers, and stressing the importance of "learning and community-building" that took place throughout the iterative process. Participants felt that the engagement process was not burdensome and appreciated the opportunity to engage from the comfort of their home.

Similarly, clinicians we engaged as part of developing the method thought that it would provide a useful approach for incorporating patient preferences and values before the guidelines are finalized and help educate clinicians on factors that might affect patient adherence to recommendations. Patient and caregiver input can draw attention to factors that may make them less likely to follow the recommendations and therefore encourage guideline developers and clinicians to think about implementation strategies to address concerns about patient-centeredness raised by the panelists [6].

Because of the novelty and the promise of scalable and convenient engagement, this method should be formally validated and tested in the context of other clinical conditions and compared to other ways of engaging patients in CPG development. Future research should also empirically identify the value added of patient engagement in CPG development to determine the extent to which it produces significantly better guidelines that have higher patient adherence and lead to better patient outcomes.

Acknowledgments and Conflicts of Interest

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References

[1] Fitch, K., Bernstein, S.J., Aguilar, M.D., Burnand, B., LaCalle, J.R., Lazaro, P. *et al.* (2001). The RAND/UCLA Appropriateness Method User's Manual. Santa Monica: RAND Corporation. Contract No.: MR-1269-DG-XII/RE.

[2] Lugtenberg, M., Burgers, J. & Westert, G. (2009). Effects of evidence-based clinical practice guidelines on quality of care: a systematic review. *BMJ Quality & Safety* 18 (5) 385-392.

[3] Smith, T.J. & Hillner, B.E. (2001). Ensuring quality cancer care by the use of clinical practice guidelines and critical pathways. *Journal of Clinical Oncology* 19 (11) 2886-2897.

[4] Wyatt, K.D., Stuart, L.M., Brito, J.P., Leon, B.C., Domecq, J.P., Prutsky, G.J., Eggington, J.S., Calvin, A.D., Shah, N.D., Murad, M.H. & Montori, V.M. (2014). Out of Context: Clinical Practice Guidelines and Patients With Multiple Chronic Conditions: A Systematic Review. *Medical Care* 52 (Supplement 3) S92-S100.

[5] Armstrong, M.J., Mullins, C.D., Gronseth, G.S. & Gagliardi, A.R. (2017). Recommendations for patient engagement in guideline development panels: a qualitative focus group study of guideline-naïve patients. *PLoS One* 12 (3) e0174329.

[6] Alonso-Coello, P., Oxman, A.D., Moberg, J., Brignardello-Petersen, R., Akl, E.A., Davoli, M., Treweek, S., Mustafa, R.A., Vandvik, P.O., Meerpohl, J., Guyatt, G.H., Schünemann, H.J. & GRADE Working Group. (2016). GRADE Evidence to Decision (EtD) frameworks: a systematic and transparent approach to making well informed healthcare choices. 2: Clinical practice guidelines. *British Medical Journal* 353, i2089.

[7] Fraenkel, L., Miller, A.S., Clayton, K., Crow-Hercher, R., Hazel, S. & Johnson, B. (2016). When patients write the guidelines: patient panel recommendations for the

treatment of rheumatoid arthritis. *Arthritis Care & Research* 68 (1) 26-35.

[8] Hunt, L.M. & Arar, N.H. (2001). An analytical framework for contrasting patient and provider views of the process of chronic disease management. *Medical Anthropology Quarterly* 15 (3) 347-367.

[9] Cluzeau, F., Wedzicha, J.A., Kelson, M., Corn, J., Kunz, R., Walsh, J., Schünemann, H.J. & ATS/ERS Ad Hoc Committee on Integrating and Coordinating Efforts in COPD Guideline Development. (2012). Stakeholder involvement: how to do it right: article 9 in integrating and coordinating efforts in COPD guideline development. An official ATS/ERS workshop report. *Proceedings of the American Thoracic Society* 9 (5) 269-273.

[10] Armstrong, M.J., Rueda, J-D., Gronseth, G.S. & Mullins, C.D. (2017). Framework for enhancing clinical practice guidelines through continuous patient engagement. *Health Expectations* 20 (1) 3-10.

[11] Boivin, A., Currie, K., Fervers, B., Gracia, J., James, M., Marshall, C., Sakala, C., Sanger, S., Strid, J., Thomas, V., van der Weijden, T., Grol, R., Burgers, J. & G-I-N PUBLIC. (2010). Patient and public involvement in clinical guidelines: international experiences and future perspectives. *Quality and Safety in Health Care* 19 (5) 1-4.

[12] Kelson, M. (2001). Patient involvement in clinical guideline development - where are we now? *Journal of Clinical Governance* 9 (4) 169-174.

[13] Graham, T., Alderson, P. & Stokes, T. (2015). Managing Conflicts of Interest in the UK National Institute for Health and Care Excellence (NICE) Clinical Guidelines Programme: Qualitative Study. *PLoS One* 10 (3) e0122313.

[14] Marriot, S. & Lelliot, P. (1994). Clinical practice guidelines and their development. London: Royal College of Psychiatrists.

[15] Institute of Medicine. (2011). Clinical practice guidelines we can trust. Washington, DC: National Academies Press.

[16] World Health Organization. (2012). WHO Handbook for Guideline Development. Geneva, Switzerland: World Health Organization.

[17] van de Bovenkamp, H.M. & Trappenburg, M.J. (2009). Reconsidering patient participation in guideline development. *Health Care Analysis* 17 (3) 198-216.

[18] Staniszewska, S., Boardman, F., Gunn, L., Roberts, J., Clay, D., Seers, K., Brett, J., Avital, L., Bullock, I. & O'Flynn, N. (2014). The Warwick Patient Experiences Framework: patient-based evidence in clinical guidelines. *International Journal for Quality in Health Care* 26 (2) 151-157.

[19] Staniszewska, S., Crowe, S., Badenoch, D., Edwards, C., Savage, J. & Norman, W. (2010). The PRIME project: developing a patient evidence-base. *Health Expectations* 13 (3) 312-322.

[20] Armstrong, M.J., Mullins, C.D., Gronseth, G.S. & Gagliardi, A.R. (2018). Impact of patient involvement on clinical practice guideline development: a parallel group study. *Implementation Science* 13 (1) 55.

[21] Birnkrant, D.J., Bushby, K., Bann, C.M., Apkon, S.D., Blackwell, A., Brumbaugh, D. *et al.* (2018). Diagnosis and management of Duchenne muscular dystrophy, part 1:

diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *The Lancet Neurology* 17 (3) 251-267.

[22] Birnkrant, D.J., Bushby, K., Bann, C.M., Alman, B.A., Apkon, S.D., Blackwell, A. *et al.* (2018). Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *The Lancet Neurology* 17 (4) 347-361.

[23] Birnkrant, D.J., Bushby, K., Bann, C.M., Apkon, S.D., Blackwell, A., Colvin, M.K. *et al.* (2018). Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *The Lancet Neurology* 17 (5) 445-455.

[24] Khodyakov, D., Kinnett, K., Grant, S., Lucas, A., Martin, A., Denger, B., Peay, H., Coulter, I. & Fink, A. (2017). Engaging patients and caregivers managing rare diseases to improve the methods of clinical guideline development: A research protocol. *JMIR Research Protocols* 6 (4) e57.

[25] Committee on Quality of Health Care in America. (2001). *Crossing the Quality Chasm: A New Health System for the 21st Century*. Washington, DC: National Academy Press.

[26] van Teijlingen, E., Pitchforth, E., Bishop, C. & Russell, E. (2006). Delphi method and nominal group technique in family planning and reproductive health research. *Journal of Family Planning and Reproductive Health Care* 32 (4) 249-252.

[27] Alonso-Coello, P., Schünemann, H.J., Moberg, J., Brignardello-Petersen, R., Akl, E.A., Davoli, M., Treweek, S., Mustafa, R.A., Rada, G., Rosenbaum, S., Morelli, A., Guyatt, G.H., Oxman, A.D. & GRADE Working Group. (2016). GRADE Evidence to Decision (EtD) frameworks: a systematic and transparent approach to making well informed healthcare choices. 1: Introduction. *British Medical Journal* 353, i2016.

[28] Dalal, S.R., Khodyakov, D., Srinivasan, R., Straus, S.G. & Adams, J. (2011). ExpertLens: A system for eliciting opinions from a large pool of non-collocated experts with diverse knowledge. *Technological Forecasting & Social Change* 78 (8) 1426-1444.

[29] Armstrong, C., Grant, S., Kinnett, K., Denger, B., Martin, A., Coulter, I., Booth, M., & Khodyakov, D. (2019). Participant experiences with a new online modified-Delphi approach for engaging patients and caregivers in developing clinical guidelines. *European Journal for Person Centered Healthcare*. 7 (3) 476-489.