COMMENTARY AND ANALYSIS

Engaging patients and their caregivers/representatives in person-centered clinical guidelines development. A commentary on Khodyakov et al. (2019) EJPCH 7 (3) 470-475 & Armstrong et al. (2019) EJPCH 7 (3) 476-489

Andrew Miles BMedSci MSc MPhil PhD DSc (hc)a and Jonathan Elliott Asbridge Kt DSc (hc) DHSc (hc) DSc (hc)b

a Professor of Person Centred Health and Social Care & Co-Director, European Institute for Person Centred Health and Social Care, University of West London; Senior Vice President/Secretary General, European Society for Person Centered Healthcare/Editor-in-Chief, European Journal for Person Centered Healthcare & Honorary Professor of Person Centred Care, Centre for Public Engagement, St. George’s University Hospital Campus, University of London, UK
b President & Chairman of Council, European Society for Person Centered Healthcare; Chief Clinical Officer, Healthcare At Home/Deputy Chairman, Oxford Healthcare NHS Trust, England, UK; Chairman, Editorial Board, European Journal for Person Centered Healthcare and Visiting Senior Clinical Professor, European Institute for Person Centred Health and Social Care, University of West London, UK

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Correspondence addresses
Professor Andrew Miles (andrew.miles@pchealthcare.org.uk) & Professor Sir Jonathan Elliott Asbridge (president@pchealthcare.org.uk)

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Introduction

Few readers will be unfamiliar with the celebrated definition of clinical practice guidelines (CPGs), promulgated by the Institute of Medicine in 1990, as “systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances” [1]. A subsequent publication from the same source, similarly celebrated, and with recommendations for CPG development and use, appeared soon afterwards in 1992 [2]. Since that time, the CPG literature has followed an exponential and seemingly inexorable trajectory upwards, vividly demonstrating, during its course, the vast number of complexities associated with guideline construction and use [3-13].

Most recently, the shift in medicine’s focus away from single diagnosis acute conditions, to the management of the co- and multi-morbid, socially complex chronic illnesses [14], has raised important questions in terms of the clinical applicability of the vast majority of current guidelines in the present era of person-centered care (PCC). In this context, practice variability and divergence from general guidelines is far more likely to prove indicative of carefully tailored, personalised care, than it is of ‘physician style’ or ‘eccentricity’ [15-23]. Indeed, as Miles and Asbridge [24] point out, “… many practice variations are readily explained by case-specific contraindications, patient risk factors, patient preferences and patient choices, and are thus clinically justified by individual patient circumstances” [25-27]. CPG development going forward therefore needs to take stock of the limitations of previous methodological approaches to development, and to modify such approaches with reference to the new environment in order to ensure that all new guidelines are fit for purpose as part of individual, person-focussed management of complexity. This will require a level of multi-stakeholder involvement in CPG development as never before, with overwhelming importance placed on patient and public involvement/engagement (PPI/E), so that the full range of patient needs can be taken firmly - and operationally - into account [14,16,24,28].
Patient and Public Involvement in Modern Healthcare Systems and CPG Development Initiatives

Patient and public involvement (PPI) is now internationally recognised as an important component of guideline development and its progress appears inexorable. Price and her colleagues [29-33], for example, describe how PPI contributes to salient, pragmatic study design, recruiting benefits, interview quality and a person-centered policy setting, and how PPI can address cultural challenges, destigmatize mental health, reduce age bias and decrease disease stereotypes. Here, a key factor, indeed the general prerequisite of success, is the building of community through relationship [29-33].

Despite the major enthusiasm for PPI, a relative paucity of studies has examined precisely how PPI can meaningfully influence the guideline development process as a whole. Feedback, to date, appears to have been derived from guideline developers’ reflections on PPI experiences and analyses of participant feedback, which cannot easily be regarded as appropriately inclusive, and for sure there are many obstacles to the operationalisation of PPI. Indeed, a wide range of investigators have documented a multiplicity of barriers which include the methods through which to identify and recruit patients, precisely how to elicit their views and experiences, how to support and train patients in making their particular contributions, how to help patients understand the nature of what they are being requested to do, how to ensure the representativeness of the recruited patient participants, how to enable a requisite degree of health literacy and an adequate understanding of the relevant medical terminology and, not least, how to ensure that patients do not feel isolated or marginalised in any way. While initially it was not typically the case, the modern emphasis on PPI means that funding for PPI is now more widely available, and success stories are being increasingly documented [29-49].

The RAND/PPMD Patient-Centeredness Method and its evaluation

We turn now to two recently published papers within the Journal. Both articles report research led by the RAND Corporation, USA. In the first, Khodyakov and his colleagues [50] begin with a straightforward recognition that clinical practice guideline development by no means always includes patients or their representatives as part of its methodology, and that a failure to include a phenomenological understanding of patients’ needs, values and preferences can negatively affect guideline quality, usefulness, legitimacy, and adherence. Conversely, as the authors importantly point out, patient inclusion in CPG development is associated with a proper focus on the topics and outcomes that are important to patients and their families, with the attendant ability to identify the varying risks and benefits of different management strategies and recommendations, therefore enabling their feasibility and acceptability to be taken fully into account. The authors additionally recognize that patients, their families, and clinicians, assess treatment risk-benefit trade-offs significantly differently, so that patient involvement alongside all of the other stakeholders involved with practice guideline development ensures that the resulting CPGs have been created in a transparent, indeed ‘democratic’ manner. These are powerful arguments which demonstrate not a relative, but rather an absolute necessity for patient and public involvement/engagement in guidelines development that is entirely in accordance with person-centered thinking.

In order to put all such concepts and principles into operational action, Khodyakov and associates [50] describe their RAND/PPMD Patient-Centeredness Method (RPM), a novel online modified-Delphi approach to patient engagement in practice guideline development that enables patients and their representatives to contribute directly to the patient-centeredness of draft CPG recommendations. The method itself represents, and in the authors’ own words, 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 process. Through their innovative methodology, Khodyakov and co-workers [50] have ensured that the overall design of their approach enables an efficient collection of data on the acceptability of treatment strategies and recommendations and the desired clinical and care outcomes of patients and caregivers, and with a high degree of consistency with the process most familiar to researchers and clinicians, while impressively determining the degree of consensus on indices such as feasibility, equity and the use of resources, among the stakeholders as a whole.

The specific clinical condition studied by Khodyakov et al. was Duchenne muscular dystrophy (DMD). DMD is a rare disease of the musculature but which nevertheless represents one of the common genetic disorders, affecting circa 1 in 3,500 male infants globally. Diagnostically, it normatively becomes apparent early in infancy, usually between the ages of three to six years. DMD is principally characterized by muscle weakness and atrophy around the pelvic skeleton, with subsequent involvement of the rotator cuff. The disease is progressive, with atrophy then occurring in the trunk and forearms, followed by subsequent atrophic change in additional other muscles. The majority of patients become wheelchair users before or during their teenage years. Life threatening complications typically include cardiomyopathy and respiratory insufficiency. The extent of distress and existential challenge is highly considerable, requiring, if not mandating, a fully person-centered approach to management and general assistance [cf.51-56].

Khodyakov and colleagues [50] were careful to evaluate the personal experiences of their study participants with DMD and their carers, an approach entirely in accordance with the basic principles of person-centered care, and we examine those gratifying results
later, below. Suffice it is to note at this juncture in the Commentary that the satisfaction surveys and semi-structured interviews that were employed by the authors positively indicate that the participants reported “good experiences” with the RPM and, as part of these, cited the “convenience, anonymity, and asynchronous nature of online engagement”, additionally commenting on the “benefits of learning from the experiences of both patients and caregivers” and, very encouragingly, we thought, emphasizing “the importance of ‘learning and community-building’ that was observed as having taken place during the entirety of the iterative process. A ‘pragmatic’ result was, in our view, the documentation that participants, far from experiencing the process as burdensome, on the contrary valued the opportunity afforded to them to engage fully in the study from the security, as it were, of their own homes.

While the study by Khodyakov and associates [50] is in many ways preliminary, it amply demonstrates such significant promise for patient and caregiver involvement in CPG development, that it needs to progress, now, to a rigorous evaluation not only in DMD, which has demonstrated a clear ‘proof of principle’, but also to the wide range of other long-term, chronic, socially complex conditions that afflict our Society, and which are equally in need of urgent, person-centered resolution [14,16,24].

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

We turn now to the accompanying paper from the RAND Corporation. In this article, Armstrong and her associates [57], with Khodyakov et al. [50], agree with what we write above, that “little rigorous empirical evidence exists on engaging patients and caregivers in CPG development and their attitudes and perceptions of engagement processes”. A truism, for sure. But the significance to the reader is surely that these authors’ research has not simply developed (and evaluated) the methodology and research described by Khodyakov et al. [50], but also that it has directly analyzed the views of both patients and caregiver attitudes specifically in terms of their understanding of the methodological approach that the investigators employed. This is a significant exercise that is not only of immediate relevance to rare diseases, but to all of the long-term, co- and multi-morbid, social complex conditions that afflict our Society, and which are equally in need of urgent, person-centered resolution [14,16,24].

Armstrong and colleagues [57] document results that add very significantly to the corpus of knowledge that illustrates the importance of online methodology designed to guarantee the inclusion of patient and caregiver perspectives, ensure patient satisfaction in CPG development and, in addition, how rare diseases can be included in this overall formula. These researchers report how they found that study participants “overwhelmingly reported positive experiences with the RPM, citing the convenience, anonymity and asynchronous nature of online engagement” as previously noted by Khodyakov et al. [50]. This observation is surely of considerable importance to all PPI/E researchers, given that it lends additional credence to the suggestion, previously advanced, that online engagement methods may facilitate more openness and frank cooperation from patients and their families [57].

Of clear importance is the nature and function of ExpertLens™ which, as Armstrong and colleagues [57] describe, affords an effective platform to navigate the implementation of the innovative RPM that clearly guides not simply the basic guideline development implementation process, but also the patient/caregiver community engagement which is of considerable importance going forward. It is more than gratifying that the reported method “encouraged learning and community-building through the interactive rounds”. We read, with great interest, that the DMD patients and their families successfully ‘elevated’ the so called ‘profile’ of this challenging disease, and that the raising of awareness of DMD is generating a major re-think of how we respond to the distress that DMD causes. Such ‘awareness’, and the generation of ‘higher profile’, is immediately cross-applicable to the other chronic illnesses and should continue to be pursued [14,16,24].

Conclusion

Patient involvement/engagement in health and social care is an evolving concept, and there is a not insignificant amount of work to do to make such contributions normative in practice, and not exceptional within experimental settings. Happily, this is the direction of travel. The degree of patient participation, and the level of power or authority gained by patients and their families through a dynamic involvement and environment which has hitherto been largely unfamiliar to them, is increasing, but there are many methodological questions which remain to be addressed. For example, the impact of patient engagement, not only in terms of added value, but also in terms of cost and potential drawbacks, is poorly understood, and is in need of clarification. Additionally, there is an essential lack of a generally agreed consensus on what precisely constitutes a method or tool to demonstrate impact and through which outcomes of patient engagement can be meaningfully measured. Moreover, different stakeholder groups advance differing expectations and objectives for the CPG development exercise, and strategies for conflict avoidance are one priority among the others. The papers by Khodyakov et al. [50] and Armstrong et al. [57] are contributions to the literature of considerable importance, and we congratulate the authors on their vision and leadership.

Conflicts of Interest

The authors declare no conflicts of interest.
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