

## 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

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Caregivers, chronic illness, clinical practice guidelines (CPGs), Duchenne Muscular Dystrophy (DMD), ExpertLens™, guideline development, lived experience of illness, patient-centeredness, patient/caregiver perspectives, patient engagement, patient preferences, patient values, person-centered healthcare, RAND/PPMD Patient-Centeredness Method, treatment adherence

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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-centeredness 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 [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.

## References

- [1] Field, M.J. & Lohr, K.N. (1990). Clinical practice guidelines: directions for a new program. Washington DC: Institute of Medicine, National Academy Press.
- [2] Field, M.J. & Lohr, K.N. (1992). Guidelines for clinical practice: from development to use. Washington DC: Institute of Medicine, National Academy Press.
- [3] Shaneyfelt, T.M., Mayo-Smith, M.F. & Rothwangl, J. (1999). Are guidelines following guidelines? The methodological quality of clinical practice guidelines in the peer-reviewed medical literature. *Journal of the American Medical Association* 281 (20) 1900-1905.
- [4] AGREE Collaboration. (2003). Development and validation of an international appraisal instrument for assessing the quality of clinical practice guidelines: the AGREE project. *Quality and Safety in Health Care* 12 (1) 18-23.
- [5] Watine, J., Friedberg, B., Nagy E., Onody, R., Oosterhuis, W., Bunting, P.S., Charet, J.C. & Horvathet, A.R. (2006). Conflict between guideline methodologic quality and recommendation validity: a potential problem for practitioners. *Clinical Chemistry* 52 (1) 65-72.
- [6] Vigna-Taglianti, F., Vineis, P., Liberati, A. & Faggiano, F. (2006). Quality of systematic reviews used in guidelines for oncology practice. *Annals of Oncology* 17 (4) 691-701.
- [7] Nuckols, T.K., Lim, Y.W., Wynn, B.O., Mattke, S., MacLean, C.H., Harber, P., Brook, R.H., Wallace, P., Garland, R. H. & Aschet, S. (2008). Rigorous development does not ensure that guidelines are acceptable to a panel of knowledgeable providers. *Journal of General Internal Medicine* 23 (1) 37-44.
- [8] Hinchey, P.R., Myers, J.B., Lewis, R., De Maio, V.J., Reyer, E., Licatase, D., Zalin, J., Snyder, G. & Capital County Research Consortium. (2010). Improved out-of-hospital cardiac arrest survival after the sequential implementation of 2005 AHA guidelines for compressions, ventilations, and induced hypothermia: the Wake County experience. *Annals of Emergency Medicine* 56 (4) 348-357.
- [9] Brouwers, M., Kho, M.E., Browman, G.P., Burgers, J.S., Cluzeau, F., Feder, G., Fervers, B., Graham, I.D., Grimshaw, J., Hanna, S.E., Littlejohns, P., Makarski, J., Zitzelsberger, L. & AGREE Next Steps Consortium. (2010). AGREE II: advancing guideline development, reporting and evaluation in healthcare. *Canadian Medical Association Journal* 182 (18) E839-E842.
- [10] Committee on Standards for Developing Trustworthy Clinical Practice Guidelines. (2011). In: Clinical Practice Guidelines We Can Trust. Graham, R., Mancher, M., Miller, W D., Wolman, D., Greenfield, S. & Steinberg, E. (Eds.). Washington, DC: The National Academies Press.
- [11] Eccles, M.P., Grimshaw, J.M., Shekelle, P., Schünemann, H.J. & Woolf, S. (2012). Developing clinical practice guidelines: target audiences, identifying topics for guidelines, guideline group composition and functioning and conflicts of interest. *Implementation Science* 7, 60.
- [12] Gagliardi A.R., Alhabib, S. & Members of Guidelines International Network Implementation Working Group. (2015). Trends in guideline implementation: a scoping systematic review. *Implementation Science* 10, 54.
- [13] Chan, W.V., Pearson, T.A., Bennett, G.C., Cushman, W.C., Gaziano, T.A., Gorman, P.N., Handler, J., Krumholz, H. M., Kushner, R.F., MacKenzie, T.D., Sacco, R.L., Smith, S.C., Stevens, V.J., Wells, B.L., Castillo, G., Heil, S.K.R., Stephens, J. & Jacobson Vann, J.C. (2017). ACC/AHA Special Report: Clinical Practice Guideline Implementation Strategies: A Summary of Systematic Reviews by the NHLBI Implementation Science Work Group: A Report of the American College of Cardiology/American Heart Association Task Force on Clinical Practice Guidelines. *Circulation* 135 (9) e122-e137.
- [14] Miles, A. & Asbridge, J.E. (2016). The chronic illness problem. The person-centered solution. *European Journal for Person Centered Healthcare* 4 (1) 1-5.
- [15] Cabana, M.D., Rand, C.S., Powe, N.R., Wu, A.W., Wilson, M.H., Abboud, P.A. & Rubin, H.R. (1999). Why don't physicians follow clinical practice guidelines? A framework for improvement. *Journal of the American Medical Association* 282 (15) 1458-1465.
- [16] Miles, A., Asbridge, J.E. & Caballero, F. (2015). Towards a person-centered medical education: challenges and imperatives. *Educación Médica* 16 (1) 25-33.
- [17] Wennberg, J.E. (2002). Unwarranted variations in healthcare delivery: implications for academic medical centres. *British Medical Journal* 325 (7370) 961-964.
- [18] Wennberg, J. (2011). Time to tackle unwarranted variations in practice. *British Medical Journal* 342, 687-690.
- [19] Mercuri, M. & Gafni, A. (2011). Medical practice variations: what the literature tells us (or does not) about what are warranted and unwarranted variations. *Journal of Evaluation in Clinical Practice* 17 (4) 671-677.
- [20] Mercuri, M., Sherbino, J., Sedran, R.J., Frank, J.R., Gafni, A. & Norman, G. (2015). When Guidelines Don't Guide: The Effect of Patient Context on Management Decisions Based on Clinical Practice Guidelines. *Academic Medicine* 90 (2) 191-196.
- [21] Anjum, R.L. (2016). Evidence Based or Person Centered? An Ontological Debate. *European Journal for Person Centered Healthcare* 4 (2) 421-429.
- [22] Mercuri, M. & Gafni, A. (2018). Reflecting on Evidence Based Medicine, Person Centered Medicine and Small Area Variations: how contemporary frameworks for medicine address (or not) the needs of the individual patient. *European Journal for Person Centered Healthcare* 6 (3) 454-461.
- [23] Bogerd, M.J.L., Slottje, P., Schellevis, F.G., Giebels, A., Rijken, M., van Hout, H.P.J. & Reinders, M.E. (2019). From protocolized to person-centered chronic care in general practice: study protocol of an action-based research project (COPILOT). *Primary Health Care Research & Development* 20, e134.
- [24] Miles, A. & Asbridge, J.E. (2019). The NHS Long Term Plan (2019) - is it person-centered? *European Journal for Person Centered Healthcare* 7 (1) 1-11.
- [25] Arts, D.L., Voncken, A.G., Medlock, S., Abu-Hanna, A. & van Weert, H.C. (2016). Reasons for intentional

- guideline non-adherence: a systematic review. *International Journal of Medical Informatics* 89, 55-62
- [26] Corallo, A.N., Croxford, R., Goodman, D.C., Bryan, E.L., Srivastava, D. & Stukel, T.A. (2018). A systematic review of medical practice variation in OECD countries. *Health Policy* 114 (1) 5-14.
- [27] Cook, D.A., Pencille, J., Dupras, D.M., Linderbaum, J.A., Pankratz, V.S. & Wilkinson, J.M. (2018). Practice variation and practice guidelines: Attitudes of generalist and specialist physicians, nurse practitioners, and physician assistants. *PLoS One* 13 (1) e0191943.
- [28] Miles, A. & Asbridge, J.E. (2014). On the need for transformational leadership in the delivery of person-centered clinical practice within 21st Century healthcare systems. *European Journal for Person Centered Healthcare* 2 (3) 261-264.
- [29] Price A. (2014). Patients doing research for themselves. *BMJ Blogs*. Available at: <https://blogs.bmj.com/bmj/2014/10/17/amy-price-patients-doing-research-for-themselves/>
- [30] Price, A., Albarqouni, L., Kirkpatrick, J., Clarke, M., Liew, S.M., Roberts, N. & Burls, A. (2017). Patient and public involvement in the design of clinical trials: An overview of systematic reviews. *Journal of Evaluation in Clinical Practice* 24 (1) 240-253.
- [31] Price, A. (2016). Public led online trials and participatory action research: Why do we need them? *European Journal for Person Centered Healthcare* 4 (2) 340-345.
- [32] Price, A. (2017). Developing tools for practice that support patient choice. *BMJ Blogs*. Available at: <https://blogs.bmj.com/bmj/2017/05/24/amy-price-developing-tools-for-practice-that-support-patient-choice/>
- [33] Price, A., Gupta, U., Srivastava, U. & Chu, L. (2019). Co-Production, Co-Education and Person-Centered Healthcare Practice. *European Journal for Person Centered Healthcare* 7 (2) 219-222.
- [34] National Institute for Clinical Excellence. (2004). A report on a study to evaluate patient/carer membership of the first NICE Guideline Development Groups. London: National Institute for Clinical Excellence. Available at: <https://www.nice.org.uk/media/default/About/NICE-Communities/Public-involvement/Public-involvement-programme/PIU-GDG-evaluation-report-2004-1.pdf>.
- [35] Graham K. (2006). Patient/carer involvement in clinical guidelines: the SIGN experience. Presented at the 2006 conference of the Spanish HTA Group (Seville, Spain) 2006. Available at: <http://www.fgcasal.org/aeets/ficheros/aeets-vii/Graham.pdf>.
- [36] Légaré, F., Boivin, A., van der Weijden, T., Pakenham, C., Burgers, J., Légaré J, St-Jacques, S. & Gagnon, S. (2011). Patient and public involvement in clinical practice guidelines: a knowledge synthesis of existing programs. *Medical Decision Making* 31 (6) E45-E74.
- [37] Cowl, J., Tyrrell, H., Sakala, C., Gracia, J. & Huang N. (2012). G-I-N Public Toolkit: Patient and Public Involvement in Guidelines. How to conduct public and targeted consultation. pp. 17-27. Available at: <https://g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015>.
- [38] Tong, A., Lopez-Vargas, P., Howell, M., Phoon, R., Johnson, D., Campbell, D., Walker, R.G. & Craig, J.C. (2012). Consumer involvement in topic and outcome selection in the development of clinical practice guidelines. *Health Expectations* 15 (4) 410-423.
- [39] Diaz Del Campo, P, Gracia, J., Blasco, J.A. & Andradas, E. (2011). A strategy for patient involvement in clinical practice guidelines: methodological approaches. *BMJ Quality and Safety* 20 (9) 779-784.
- [40] Carman, K.L., Dardess, P., Maurer, M., Sofaer, S., Adams, K., Bechtel, C. & Sweeney, J. (2013). Patient and family engagement: a framework for understanding the elements and developing interventions and policies. *Health Affairs (Millwood)* 32 (2) 223-231.
- [41] Frank, L., Forsythe, L., Ellis, L., Schrandt, S., Sheridan, S., Gerson, J., Konopka, K. & Daugherty, S. (2015). Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute. *Quality of Life Research* 24 (5) 1033-1041.
- [42] Shippee, N.D., Domecq Garces, J.P., Prutsky Lopez, G.J., Wang, Z., Elraiyah, T.A., Nabhan, M., Brito, J.P., Boehmer, K., Hasan, R., Firwana, B., Erwin, P. J., Montori, V.M. & Murad, M.H. (2015). Patient and service user engagement in research: a systematic review and synthesized framework. *Health Expectations* 18 (5) 1151-1166.
- [43] G-I-N PUBLIC Toolkit: Patient and Public Involvement in Guidelines. (2015). Available at: <https://g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015>.
- [44] Coon, J.T., Gwernan-Jones, R., Moore, D., Richardson, M., Shotton, C., Pritchard, W., Morris, C., Stein, K. & Ford, T. (2016). End-user involvement in a systematic review of quantitative and qualitative research of non-pharmacological interventions for attention deficit hyperactivity disorder delivered in school settings: reflections on the impacts and challenges. *Health Expectations* 19 (5) 1084-1097.
- [45] den Breejen, E.M., Hermens, R.P., Galama, W.H., Willemsen, W.N., Kremer, J.A. & Nelen, W.L. (2016). Added value of involving patients in the first step of multidisciplinary guideline development: a qualitative interview study among infertile patients. *International Journal of Quality in Health Care* 28 (3) 299-305.
- [46] van der Ham, A.J., van Erp, N. & Broerse, J.E. (2016). Monitoring and evaluation of patient involvement in clinical practice guideline development: lessons from the Multidisciplinary Guideline for Employment and Severe Mental Illness, the Netherlands. *Health Expectations* 19 (2) 471-482.
- [47] 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.
- [48] 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.

[49] Brouwers, M.C., Vukmirovic, M., Spithoff, K., & Makarski, J. (2017). Understanding optimal approaches to patient and caregiver engagement in the development of cancer practice guidelines: a mixed methods study. *BMC Health Services Research* 17, 186.

[50] Khodyakov, D., Denger, B., Grant, S., Kinnett, K., Armstrong, C., Martin, A., Peay, H., Coulter, I. & Hazlewood, G. (2019). The RAND/PPMD Patient-Centeredness Method: a novel online approach to engaging patients and their representatives in guideline development. *European Journal for Person Centered Healthcare* 7 (3) 470-475.

[51] Acsadi G. (2003). Duchenne Muscular Dystrophy. *NORD Guide to Rare Disorders*. Philadelphia, PA: Lippincott Williams & Wilkins.

[52] Boland, B.J., Silbert, P.L., Groover, R.V., Wollan, P.C. & Silverstein, M.D. (1996). Skeletal, cardiac, and smooth muscle failure in Duchenne muscular dystrophy. *Pediatric Neurology* 14 (1) 7-12

[53] Dubowitz, V. (1997). The muscular dystrophies—clarity or chaos? *New England Journal of Medicine* 336 (9) 650-651.

[54] Birnkrant, D.J., Bushby, K., Bann, C.M., Apkon, S.D., Blackwell, A., Brumbaugh, D., Case, L.E., Clemens, P.R., Hadjiyannakis, S., Pandya, S., Street, N., Tomezsko, J., Wagner, K.R., Ward, L.M., Weber, D.R. & DMD Care Considerations Working Group. (2018). Diagnosis and management of Duchenne muscular dystrophy. Part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *Lancet Neurology* 17 (3) 251-267 [Erratum: *Lancet Neurology* 17 (6) 495].

[55] Birnkrant, D.J., Bushby, K., Bann, C.M., Alman, B.A., Apkon, S.D., Blackwell, A., Case, L.E., Cripe, L., Hadjiyannakis, S., Olson, A.K., Sheehan, D.W., Bolen, J., Weber, D.R., Ward, L.M. & DMD Care Considerations Working Group. (2018). Diagnosis and management of Duchenne muscular dystrophy. Part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurology* 17 (4) 347-361.

[56] Birnkrant, D.J., Bushby, K., Bann, C.M., Apkon, S.D., Blackwell, A., Colvin, M.K., Cripe, L., Herron, A.R., Kennedy, A., Kinnett, K., Naprawa, J., Noritz, G., Poysky, J., Street, N., Trout, C.J., Weber, D.R., Ward, L.M. & DMD Care Considerations Working Group. (2018). Diagnosis and management of Duchenne muscular dystrophy. Part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *Lancet Neurology* 2018; 17 (5) 445-455.

[57] 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.