
Article:
de Wit, M, Cooper, C, Reginster, JY et al. (1 more author) (2019) Practical guidance for patient-centred health research. The Lancet, 393. pp. 1095-1096. ISSN 0140-6736

https://doi.org/10.1016/S0140-6736(19)30034-0

THELANCET-D-18-01328

Article type: Letter (word limit: 750 words, 10 refs, 1 panel)

Leading initiatives to provide practical guidance for engaging patients in health research, treatment guidelines and regulatory processes

Findings of an Expert Group of the World Health Organization (WHO) and European Society for Clinical and Economic Aspects of Osteoporosis, Osteoarthritis and Musculoskeletal Diseases (ESCEO)

To support the development, approval and reimbursement of medical interventions that best meet patients' needs, there is increasing emphasis on patient-centred research through the engagement of patients in identifying unmet needs,[1] the design and conduct of clinical studies[2] and subsequent regulatory assessments[3] and post-marketing vigilance. However, despite the many ongoing pilots, there is currently little evidence-based practical guidance on how effective patient engagement may be facilitated. To this end, an expert group, representing a wide range of stakeholders and disciplines, was convened by the European Society for Clinical and Economic Aspects of Osteoporosis, Osteoarthritis and Musculoskeletal Diseases (ESCEO) and the World Health Organization (WHO). The group generated a set of practical recommendations for patient engagement in drug development, clinical research and regulatory decisions (see Panel). These principles are based on lessons learned within longitudinal research initiatives such as Outcome Measures in Rheumatology (OMERACT)[2,4] and active patient engagement in regulatory processes by the European Medicines Agency (EMA) and the US Food and Drug Administration (FDA).[5,6] The nine principles outlined here were developed after an extensive dialogue among the expert participants and an iterative consensus process, and form a starting point from which tailoring of the approach to suit different chronic diseases and other healthcare context needs may be undertaken.

Engaging with patients helps to bridge the gap between health research, policy and patient-centred practice, increases transparency, and will lead to more meaningful outcomes. Patient engagement should be initiated in a stepwise approach through which all parties can learn together and identify the format that works best for all involved. At all stages of engagement, provide support, define roles, manage expectations and give feedback, to ensure that engagement is mutually beneficial. In this way, ultimately everyone can benefit from knowledge sharing. Overarching principles for engaging patients include the recognition that the perspective of patients is pivotal, that earlier involvement of patients is always better and that involvement at all stages is necessary. Patients should be offered the possibility to consult each other on experience-based views, furthermore, to ensure proper representation, inviting at least two patient research partners (PRPs) is recommended.[7] Lastly, acknowledgement of input and feedback to patients is essential, and integrated knowledge translation is desirable.[8]

Patient engagement is an evolving concept. We acknowledge that there are different levels of patient engagement, all equally valuable and complementary, and that the degree of patient participation and level of power or authority should not be mandatory but may be tailor-made to suit the individual research purpose. The

research agenda for future refinement of the process will include the development of new methodologies to assess the impact of patient engagement on both research process and outcomes and novel ways to enhance the significance of existing methods of engagement. The impact of patient engagement, in terms of added value, but also cost and potential downsides is currently poorly understood. Reasons for this include a lack of consensus on a validated methodology or tool to demonstrate impact, and a lack of consensus on important outcomes of patient engagement; people and stakeholder groups have different expectations and objectives regarding patient engagement, and thus require different methodologies and outcomes for evaluation. Another challenge is that we, as an expert group, all agree that principal investigators and stakeholders should invest in support, information, education and feedback to patient experts; however, there is a growing awareness that it does not make sense to train patients in the medicine development cycle without simultaneously preparing researchers for their role and the task of engaging patients in that process. Thus, there is a need to explore both the benefits and downsides of educating patient experts as well as exploring the needs of researchers for guidance, coaching and training.[9] Ultimately, we hope that adoption of our best practice principles and other initiatives will pave the way for increased patient engagement that is optimised to meet the needs and expectations of all stakeholders, including researchers, clinicians, regulatory bodies, and patients, with clear, measurable outcomes.

Maarten de Wit PhD[a], Professor Cyrus Cooper FMedSci[b], Professor Jean-Yves Reginster MD[c]

[a] Patient Research Partner, Amsterdam University Medical Centre, Dept. Medical Humanities, Amsterdam Public Health (APH), Amsterdam, The Netherlands

[b] MRC Lifecourse Epidemiology Unit, University of Southampton, Southampton General Hospital, Southampton, UK; and NIHR Musculoskeletal Biomedical Research Unit, University of Oxford, Oxford, UK; and WHO Collaborating Centre for Public Health Aspects of Musculoskeletal Health and Aging, Liège, Belgium

[c] Department of Public Health, Epidemiology and Health Economics, University of Liège, CHU Sart Tilman B23, 4000 Liège, Belgium; Chair for Biomarkers of Chronic Diseases, Biochemistry Department, College of Science, King Saud University, Riyadh, Kingdom of Saudi Arabia; and WHO Collaborating Centre for Public Health Aspects of Musculoskeletal Health and Aging, Liège, Belgium

Author for correspondence: jyreginster@uliege.be

[Panel]

WHO-ESCEO Best practice principles for engaging patients in health research, treatment guidelines and regulatory processes

1. The perspective of patients is pivotal in health research, treatment guidelines and the decision-making process of medicine authorisation.
2. Capturing patients' perspectives requires multiple forms of engagement that are complementary and need to be tailored to suit different chronic diseases and contexts.
3. Transparency for all stakeholders about patients' roles in the process facilitates participation and manages expectations from all angles.
4. Broad representativeness of patients' perspectives in demography, geography, disease severity and numbers must be ensured.
5. Involvement of at least two patient experts throughout the research, assessment and deliberation processes ensures preservation of the patient perspective and increases the validity of the outcomes.
6. Providing adequate information, support and feedback to patient representatives is key to effective engagement.
7. Teaching researchers the expertise and skills to support public engagement should always be considered.
8. Fruitful participation always requires additional resources to be allocated to the process, with extra effort in time, money and energy.
9. Continuous monitoring and measuring of interactions are vital to refine procedures according to feedback received.

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Acknowledgements

An expert group meeting was organized by the European Society for Clinical and Economic Aspects of Osteoporosis, Osteoarthritis and Musculoskeletal Diseases (ESCEO) and the World Health Organization (WHO), and held in Geneva, Switzerland, on June 9, 2017, and funded by the ESCEO, a Belgian not-for-profit organisation. The expert group comprised a global representation of clinicians, outcome researchers, social scientists, epidemiologists, health technology assessment and regulatory experts, pharmacists and patient representatives.

WHO-ESCEO Working Group members: Maarten de Wit PhD, Cyrus Cooper FMedSci, Peter Tugwell MD, Nathalie Bere MSc, John Kirwan MD, Philip G. Conaghan MBBS, Charlotte Roberts MD, Isabelle Aujoulat PhD, Nasser Al-Daghri PhD, Islene Araujo de Carvalho MD, Mary Barker PhD, Nicola Bedlington BA Hons, Maria Luisa Brandi MD, Olivier Bruyère PhD, Nansa Burlet, MD, MPH, Philippe Halbout PhD, Mickaël Hiligsmann PhD, Famida Jiwa MHSc, John A. Kanis MD, Andrea Laslop MD, Wendy Lawrence PhD, Daniel Pinto PhD, Concepción Prieto Yerro PhD, Véronique Rabenda MSc, René Rizzoli MD, Marieke Scholte-Voshaar MSc, Mila Vlaskovska MD, Jean-Yves Reginster MD

Declaration of interests

C. Cooper reports personal fees from Alliance for Better Bone Health, Amgen, Eli Lilly, GSK, Medtronic, Merck, Novartis, Pfizer, Roche, Servier, Takeda and UCB, outside of the submitted work.

M. de Wit has received fees for lectures or consultancy through Stichting Tools from Abbvie, BMS, Celgene, Eli Lilly, Janssen-Cilag, Novartis, Pfizer and Roche, outside of the submitted work.

J.Y. Reginster reports personal fees and other from Servier, IBSA, Genevrier, UCB, Asahi, Radius Health, Meda, Pierre Fabre, grants from Merck Sharp & Dohme (MSD), Amgen, Lilly, Servier, Pfizer, Danone, Meda, Cniel, IBSA, Genevrier, personal fees from MSD, IBSA, Genevrier, Servier, Danone, Pharmevo, Cniel, Meda, and the Dairy Research Council (DRC), outside of the submitted work.