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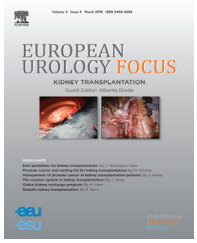

Guidelines

The Key Role of Patient Involvement in the Development of Core Outcome Sets in Prostate Cancer

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Abstract

Patients are the stewards of their own care and hence their voice is important when designing and implementing research. Patients should be involved not only as participants in research that impacts their care, as the recipients of that care and any associated harms, but also as research collaborators in prioritising important questions from the patient perspective and designing the research and the ways in which is it most appropriate to involve patients. The PIONEER Consortium, an international multistakeholder collaboration lead by the European Association of Urology, has developed a core outcome set (COS) for localised and metastatic prostate cancer relevant to all stakeholders in particular patients. Throughout the work of PIONEER, patient representatives were involved as collaborators in setting the research agenda, and a wider group of patients was involved as participants in developing COSs, for instance in consensus meetings on choosing important outcomes and appropriate definitions. This publication showcases the process for COS development and highlights the most important recommendations to ultimately inform future research projects co-created between patients and other stakeholders.

Patient summary: An important step in involving patients in the selection of outcomes for clinical trials, clinical audits, and real-world evidence is the development of a core outcome set (COS) that is relevant to all stakeholders. This report highlights the patient participation throughout our PIONEER COS development.

Take Home Message: An important step in involving patients in the selection of outcomes for clinical trials, clinical audits, and real-world evidence is to develop a core outcome set (COS) that is relevant to all stakeholders. As part of the work of the PIONEER Consortium, we aim to highlight the patient participation throughout our PIONEER COS development.

1. Introduction

With the shift in health care management towards patient-centred care and shared decision-making, it has become increasingly important to conduct research with patients as participants in which their voice is central and to involve them in setting the research agenda to ensure that it is relevant. An important step in involving patients in the selection of outcomes for clinical trials, clinical audits, and real-world evidence (RWE) is the development of a core outcome set (COS) that is relevant to all stakeholders. A COS is a minimum set of outcomes that should be measured and reported in clinical trials [1] and it is beneficial for stakeholders if this is also applicable to RWE. A COS recommends what outcomes are most important to stakeholders and how they should be defined and measured. Historically, outcomes for clinical trials have not considered patient opinions when choosing which outcomes to report on. As research findings inform the development of guidelines and regulatory decisions, it is problematic that the choice of outcomes reported on does not centrally involve patients because they are the ones living with the disease and have first-hand knowledge of its impact on their

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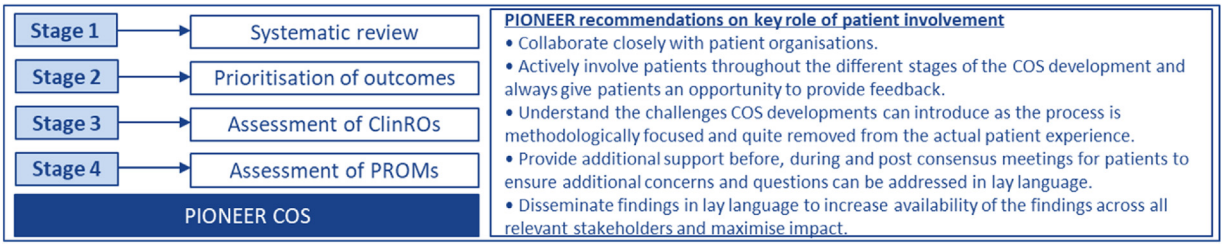

Fig. 1 – PIONEER COS development process.
COS = core outcome set; ClinROs = clinician-reported outcomes; PROMs = patient-reported outcome measures.

life. Therefore, it is important to understand what outcomes matter to patients [1].

Patient focus has been considered in the development and execution of all milestones across PIONEER, an international collaboration led by the European Association of Urology (EAU) that aims to use big data technologies to answer prioritised questions in prostate cancer (PCa) to improve guideline development and clinical practice [2]. Patients have been involved in identifying the most important research questions in the field of PCa, prioritising the most important outcomes, and in choosing the most appropriate definitions of those outcomes; patient advocates are also participating in every board and scientific meeting and are a vital decision body throughout the process.

Guidance on how to involve patients from an early stage in COS development and setting the research agenda is available [1,3–5]. One aim of the PIONEER Consortium is to update and standardise the terminology of currently available outcome sets for PCa [6–8]. Here, we highlight the patient participation throughout our PIONEER COS development. We describe how we involved patients in the different stages of COS development (Fig. 1). Following a systematic review of PCa outcomes (stage 1), we sought a consensus on outcomes to include (stage 2), followed by a specific assessment of how to quantify these outcomes (stage 3A for clinician-reported outcomes and stage 3B for patient-reported outcomes) in different stages. We followed the Core Outcome Set-Standards for Development recommendations [9]. Methodological details for this COS development will be published elsewhere.

1.1. Stage 1: systematic review

We performed four systematic reviews across PCa stages to identify what outcomes have been reported in effectiveness research for the different stages of PCa. Patient representatives attended the annual PIONEER General Assembly at which the systematic review plans (year 1) and results (year 2) were presented and they had an opportunity to provide feedback during the assembly and via email afterwards.

1.2. Stage 2: prioritisation of outcomes (consensus and interviews)

We held two consensus meetings with participants from relevant stakeholder groups (patients, health care professionals, and researchers). They were asked to vote on the preferred terminology and newly identified outcomes identified in the systematic review outlined in stage 1. The three attending patient participants were briefed before the meeting to ensure the research goal was clearly communicated. Additional support after the meeting was offered to clarify any outstanding questions and ensure that the patients' voice was captured correctly. Moreover, we conducted another study in which we interviewed patients to obtain a deeper understanding of what outcomes are important to them to supplement the list of outcomes identified in the systematic reviews in stage 1. In developing the interview schedule for the semi-structured interview study, we first conducted a patient group discussion with four patients to check that the questions were understandable from their perspective and to check that other important questions were not missed.

1.3. Stage 3A: clinician-reported outcomes (ClinROs)

We held two consensus meetings (one for localised PCa and one for metastatic PCa) to vote on the preferred measurement definitions of the outcomes identified in stages 1 and 2. The participants were patients, health care professionals, and researchers. They were invited to discuss and vote on their preferred definition and terminology. Discussions in subgroups were conducted before voting and each group included a patient advocate. As these discussions could be rather technical at times, we assigned a health care professional "buddy" in each group with the task of ensuring that everything was also described in lay language.

1.4. Stage 3B: patient-reported outcome measures (PROMs)

Following an assessment of the psychometric properties and feasibility of PROMs available for PCa, we reported our findings to the PIONEER General Assembly and invited patients to provide us with feedback. The overall feedback was positive and enabled us to continue with the assessment.

2. Recommendations

In the PIONEER project, we have actively engaged with patients from initiation of the project to ensure that our research is meaningful to men with PCa. We have included patient representatives in the project steering group to help in designing the research processes and ensure that they are relevant, and as participants to ensure that their voices are heard. As described, to develop the COS, one of the main early PIONEER outputs, we actively sought patients'

opinions on our research plans through discussion groups and feedback sessions, and included them as participants in developing the COS through interview studies and consensus processes. Moreover, to ensure that patients understood the technicalities of the methodologies being applied, we ensured that a researcher or health care professional in the PIONEER team always took responsibility for describing the findings and issues in lay language. The PIONEER Consortium would therefore like to make the following recommendations for patient engagement in COS development:

- Collaborate closely with patient organisations such as the European Cancer Patient Coalition in setting the research agenda.
- Actively involve patients throughout the different stages of COS development and always give patients an opportunity to provide feedback (eg, PIONEER General Assembly).
- Understand the challenges that COS development can introduce, as the process is methodologically focused and quite removed from the actual patient experience (ie, additional interviews with patients can enable researchers to bridge the gap between the abstract process and the patient perspective).
- Provide patients with additional support before, during, and after consensus meetings to ensure that additional concerns and questions can be addressed in lay language and that all patients can actively engage.
- Disseminate findings in lay language to increase the availability of the findings across all relevant stakeholders and maximise their impact.

In conclusion, the PIONEER Consortium would like to highlight that it is key for methodologically intensive studies such as COS development to keep patient experiences of disease, treatment, and care as a central focus. Ultimately, the COS will have a better chance of being implemented and impacting research and ultimately clinical care if we ensure that the patients' voice and experience are appropriately captured throughout and we as a research group communicate this important message in COS dissemination.

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Declaration of Competing Interest
The authors report no declarations of interest.

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