Standards for COS development

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Background

Despite extensive searching and consultation, we have not identified any guidelines regarding best practice for the development of core outcome sets or for choosing key outcomes to include in research into the effectiveness of health care. However, there appears to be consistency in that the first step in the process is typically to agree about 'what' to measure, with decisions about 'how' and 'when' to measure these outcomes usually later in the process. This document relates to standards for developing consensus about 'what' to measure. Work Package 4 will produce guidance on finding (deliverable 4.1) and selecting (deliverable 4.3) instruments for measuring the outcomes in the core set (i.e. the 'how' and 'when').

We have identified issues to be considered in the development of COS [1] and describe those here together with additional ones identified since this earlier publication. Reviewing the approaches to development taken to date against these recommendations in the 198 published COS (deliverable 1.1), we also highlight two key areas of improvement where we have been able to ascertain information from the 52 ongoing studies that have been registered with COMET.

(i) Scope

The specific area of health or healthcare of interest should be described, in terms of the health condition, target population and which interventions the COS is to be relevant to; to focus the development of the COS and to help potential users to decide on its relevance to them. These cover the first three elements of the PICO (Population, Intervention, Comparator, Outcomes) structure for a clinical trial.

Area for improvement: The systematic review identified that 58% of published studies did not specify whether the COS was intended for all interventions or a particular intervention type.

Ongoing studies: This has reduced to 15%.

(ii) Establishing an evidence gap

(a) Does a COS already exist?

One of the difficulties for people wishing to develop a COS, and for those wishing to improve the methods for COS development, is how to identify existing (and ongoing) studies to develop COS. As part of the COMET Initiative, a searchable database has been developed, which is a unique resources dedicated to COS. Researchers should check for existing or ongoing work before embarking on a new project, thus minimizing unnecessary duplication of effort.

(b) Is a COS needed?
A review of previous trials [2] or systematic reviews in the area can provide evidence of need for a COS. Systematic reviewers are starting to use the outcome matrix recommended by the ORBIT project [3] to display the outcomes reported in the eligible studies. This matrix may demonstrate inconsistency of outcomes measured to date in addition to potential outcome reporting bias.

(iii) Protocol
There are potential sources of bias in the COS development process, and preparing a protocol in advance may help to reduce these biases, improve transparency and share methods with others. We recommend that a protocol is developed prior to the start of the study, and made publically available [4].

(iv) Registration of the COS
One of the aims of the COMET Initiative is to provide a means of identifying existing, ongoing and planned COS studies. COS developers should be encouraged to register their project in a free to access, unrestricted public repository, such as the COMET database, which is the only such repository we are aware of.

(v) Stakeholder involvement

Key stakeholders may include health service users, health care practitioners, trialists, regulators, industry representatives, policy makers, researchers and the public. Decisions regarding the stakeholder groups to be involved, how they are to be identified and approached, and the target number and proportion from each group will be dependent upon the particular scope of the COS as well as upon existing knowledge and practical feasibility considerations. For example, a COS for an intervention that is optional, e.g. breast reconstruction, is likely to have predominantly patients as the key stakeholders. These decisions should be documented and explained in the study protocol.

Consideration should be given to the representativeness of the sample of stakeholders and the ability of people across the different groups to engage with the chosen consensus method (including online activities and face-to-face meetings).

Consideration should be given to potential conflicts of interest within the group developing the COS (for example, the developers of measurement instruments in the area of interest or those whose work is focused on a specific outcome).

Area for improvement: The systematic review identified that 16% of the published COS reported that there was input from patients in the development process. Examples exist where patients have identified an outcome important to them as a group that might not have been considered if the core outcome set was developed by practitioners on their own [5,6].

Ongoing studies: This has increased to 88%.

(vi) Consensus methods

(a) Identifying existing knowledge about outcomes

It is necessary to decide what information about possible outcomes should be given to stakeholder participants before a consensus exercise begins. A review of previous trials [2] or systematic reviews in the area can identify a potential list of outcomes. A review of studies other than clinical trials (for example, observational research into harms) may also identify additional outcomes, such as rare endpoints, that would be worthy of consideration for inclusion in the COS. The identification of candidate Patient Reported Outcome (PRO) domains from existing Patient Reported Outcome Measures (PROMs) should form part of this review [7]. This should be combined with outcomes deemed to be important to health service users if such work has been previously undertaken. This initial list may be supplemented by undertaking interviews with key stakeholders or obtaining input from an advisory group whose membership reflects the key stakeholders.

Criteria for determining inclusion of items to be considered in the initial round of the consensus exercise may be needed if this scoping work leads to a long list.

(b) Eliciting views about important outcomes

Considerations concerning the choice of method include the need to build a consensus with methodological rigor, and to adopt strategies to ensure that a diverse range of opinions are heard.

If consensus participants are shown a list of potential outcomes, we recommend that in general they should be given the opportunity to propose the inclusion of additional items, especially as the literature may not include outcomes associated with the most recent treatments available or the most pressing current concerns for stakeholders.

If it is felt that the sharing of a list of outcomes at the outset of the consensus process may bias responses, open questioning may be preferred. Techniques to do this include administering questionnaires, focus groups and in-depth interviews. However, this may lead to stakeholders not considering areas previously deemed important, and subsequent questions to prompt consideration of specific outcomes may be warranted.

Methods used in previous studies to elicit opinions and to develop consensus about important outcomes include expert panel meetings and Delphi surveys. A single heterogeneous consensus panel comprising the various stakeholders may be deemed appropriate for particular areas of health care whereas separate panels for

different stakeholder groups followed by work to integrate the multiple perspectives may be more appropriate for others.

(c) Face to face meeting

We recommend that representatives of key stakeholder groups are brought together to discuss the evidence produced from the review of existing knowledge and elicitation of opinion.

(d) Determining the COS

Consideration should be given in advance to the criteria that will be used to determine when consensus has been achieved. Specification of the definition in the study protocol should reduce the risk that the people leading the process will define consensus post-hoc in a way that would bias the conclusions toward their own beliefs.

It is important to ensure that views from all key stakeholder groups are considered when making the final decision regarding the COS, and that the process for reaching that decision is reported transparently.

Researchers should consider the potential impact of the following methodological decisions on the final results: group composition, questioning technique, the information participants receive to inform their answers, whether or not responses are anonymous, how the group participants interacted with or influenced each other, the medium of the interaction, attrition bias, analysis which can miss or overstate the importance of certain outcomes, and the way in which consensus is reached.

(vii) Achieve global consensus

To compare and contrast all research in a topic area, a COS must be applicable and adopted across relevant settings and disciplines, including internationally where appropriate. However, for practical and resource reasons, stakeholders from a limited number of geographical areas may have been involved in the development of a COS. Consideration should be given to the generalisability of the results and the need for any further research involving additional stakeholders if the COS is to be used in settings other than the one in which it was developed.

Reporting

COS developers should provide a clear and transparent report of the methods they used. Reporting standards for a Delphi survey component of a COS study have been proposed previously by some authors of this document [8]. We are developing a guideline for reporting COS studies more generally.

Methodology research

Our systematic review revealed wide variation in the methods used to develop COS and work is needed to compare these different methods in order to minimise bias,

maximise efficiency and uptake, and reduce waste in this important area of research for the development of COS. In order to improve the methods, we recommend that COS developers take the opportunity of nesting methodology research studies, whenever possible, to resolve uncertainties and strengthen the evidence base.

References

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