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MASTERCLASS

Core outcome sets for research and clinical practice

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Abstract
Background: This masterclass introduces the topic of core outcome sets (COSs), describing rationale and methods for developing a COS, and providing some examples that are relevant for clinical research and practice.
Method: A COS is a minimum consensus-based set of outcomes that should be measured and reported in all clinical trials for a specific health condition and/or intervention. Issues surrounding outcome assessment, such as selective reporting and inconsistency across studies, can be addressed by the development of a COS. As suggested by key initiatives in this field (i.e. OMERACT and COMET), the development requires achieving consensus on: (1) core outcome domains and (2) core outcome measurement instruments. Different methods can be used to reach consensus, including literature systematic reviews to inform the process, qualitative research with clinicians and patients, group discussions (e.g. nominal group technique), and structured surveys (e.g. Delphi technique). Various stakeholders should be involved in the process, with particular attention to patients.

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Results and conclusions: Several COSs have been developed for musculoskeletal conditions including a longstanding one for low back pain, IMMPACT recommendations on outcomes for chronic pain, and OMERACT COSs for hip, knee and hand osteoarthritis. There is a lack of COSs for neurological, geriatric, cardio-respiratory and pediatric conditions, therefore, future research could determine the value of developing COSs for these conditions.

Background

The efficacy or effectiveness of health interventions is ordinarily assessed in randomized clinical trials which compare the outcome of the health intervention under study with a control group such as placebo treatment (for efficacy trials), or alternatives such as usual care or no treatment (for effectiveness trials).[1] Since outcomes are supposed to reflect beneficial and adverse effects of the interventions, they need to be appropriate and assessed with validated instruments to make the comparison meaningful (i.e., clinical trials are only as credible as the precision of their methodology, including the appropriateness and quality of their endpoints).[2] Notwithstanding these considerations, there are numerous issues surrounding experimental design, data analyses, and interpretation of results as well as the adequacy of outcomes assessment in clinical trials. There has been a long history of publications regarding the methodology of clinical trials and data analytic strategies and interpretation. Recently greater attention has been given to the composition of the end-points that should be considered as outcomes in clinical trials, ensuring that they are of importance to patients, and encouraging use of the same measures across studies.

Currently, outcomes are often not measured and reported consistently across clinical trials for the same health condition and/or intervention.[3] This hampers the ability to compare and pool results from different trials, and reduces the statistical power and precision of meta-analyses.[4] As an example, a systematic review including 171 clinical trials investigating physical therapy interventions for shoulder pain found that overall there was a large diversity of outcomes assessed across trials, and that only three were assessed by a large majority of trials.[5] An additional problem is selective outcome reporting bias where authors report only outcomes for which there are favorable results.[6] This may bias the interpretation of the results of clinical trials and systematic reviews.[7] For instance, two large Cochrane reviews on the effectiveness of rehabilitation interventions for low back pain (LBP) found that the large majority of trials presented an unclear or high risk of outcome selective reporting.[8,9] Outcomes of trials also need to be relevant to patients, whose views should be incorporated when considering outcome choices.[10,11]

The development of a core outcome set (COS) has been suggested as a way of addressing these issues and aim to reduce outcome irrelevance, inconsistency and selective reporting.[12]

What is a core outcome set?

A COS is an agreed, standardized and minimum set of outcomes that should be measured and reported in all clinical trials for a specific health condition.[13] This core set should be consensually agreed to by all the relevant stakeholders (e.g., health care professionals, researchers, policy makers, people who fund health services and research, industry representatives, patients, and the public).[14] A COS does not mandate which outcome(s) should be designated as primary outcome(s) in a trial, and does not preclude measurement of additional outcomes if relevant to a specific trial. Decisions about primary and secondary outcomes should be specific to the research question of interest and possibly the treatment being evaluated. For example, the most relevant primary outcome for a trial evaluating an analgesic would be a measure of pain, whereas for a rehabilitation trial it might be physical functioning. In general, the primary outcome of a trial will often coincide with one of the COS outcomes as these putatively represent the most relevant outcomes.[13]

Evidence-based synthesis and meta-analyses could be substantially improved if there was greater outcomes consistency, as more trial results could be included in pooled analyses and this would result in more precise estimates of the effectiveness of interventions.[4] The uptake of COSs in systematic reviews is gradually increasing,[15] and a survey of Cochrane editors showed strong support for use of COSs in Cochrane reviews and agreement that reliability of the reviews could benefit.[16] One way to implement COSs in Cochrane reviews is to include their outcomes in Summary of Findings tables which were developed by the Grading of Recommendations Assessment, Development and Evaluation (GRADE) initiative to provide a summary of the overall quality of the evidence, an estimate of the treatment effects and the uncertainty around those estimates.[17] These tables were developed to provide an abbreviated overview of the reviews' results for clinicians, consumers and others.[18]

Visionary work in this research field has been conducted by the Outcome Measures in Rheumatology (OMERACT) initiative which has been developing COS for rheumatologic conditions for 25 years.[2,14] In more recent times, the Core Outcome Measures in Effectiveness Trials (COMET) initiative was created, with the goals of raising awareness around COSs and of supporting methodological research around this topic.[19] OMERACT and the COMET initiatives both agree that the development of a COS requires consensus to be reached through a stepwise approach, firstly on 'what' to measure (i.e. the core outcome domains that should be measured), and, secondly on 'how' to measure (i.e. the core outcome

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