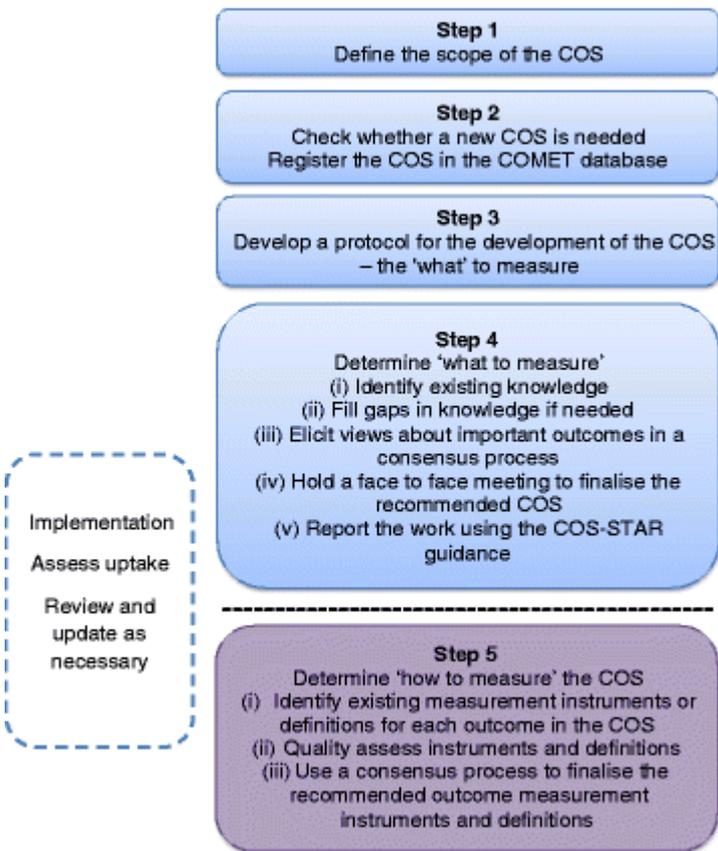


Orphan Drug Development Guidebook

Building Block E131

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	Core Outcome Measures in Effectiveness Trials (COMET) Initiative
References	http://www.comet-initiative.org/
Description	<p>The selection of appropriate outcomes is crucial when designing clinical trials in order to compare the effects of different interventions directly. For the findings to influence policy and practice, the outcomes need to be relevant and important to key stakeholders including patients and the public, health care professionals and others making decisions about health care. It is now widely acknowledged that insufficient attention has been paid to the choice of outcomes measured in clinical trials. Researchers are increasingly addressing this issue through the development and use of a core outcome set, an agreed standardised collection of outcomes which should be measured and reported, as a minimum, in all trials for a specific clinical area.</p> <p>The COMET (Core Outcome Measures in Effectiveness Trials) Initiative brings together people interested in the development and application of agreed standardised sets of outcomes, known as ‘core outcome sets’ (COS). These sets represent the minimum that should be measured and reported in all clinical trials of a specific condition, and are also suitable for use in clinical audit or research other than randomised trials.</p> <p>There is an expectation that the core outcomes will be collected and reported, making it easier for the results of trials to be compared, contrasted and combined as appropriate; while researchers continue to explore other outcomes as well.</p>

	<p>The figure below illustrates the core outcome set (COS) development process.</p> 
Category	Development practices Building Block
Geographical scope	European Union
Availability	<p>Database is publicly available for applicants developing medicines for rare and non-rare diseases. Contact person for outcome set is listed.</p> <p>The COMET Handbook: version 1.0 is available at this link: https://trialsjournal.biomedcentral.com/articles/10.1186/s13063-017-1978-4</p>
Scope of use	The COMET Initiative has developed a database and a development handbook of all studies relevant to the development of core outcome sets for use in clinical trials.
Stakeholder	http://www.comet-initiative.org/cosuptake

ders	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 number from each group will be dependent upon the particular scope of the COS as well as upon existing knowledge, the methods of COS development to be used, and practical feasibility considerations. For example, a COS for an intervention that aims to improve body image, e.g. breast reconstruction following mastectomy, is likely to have predominantly patients as the key stakeholders.
Enablers / Requirements	None
Output	Agreed 'core outcome sets' (COS) to be measured in the clinical trials.
Best time to apply and time window	While designing of the clinical trials.
Expert tips	None