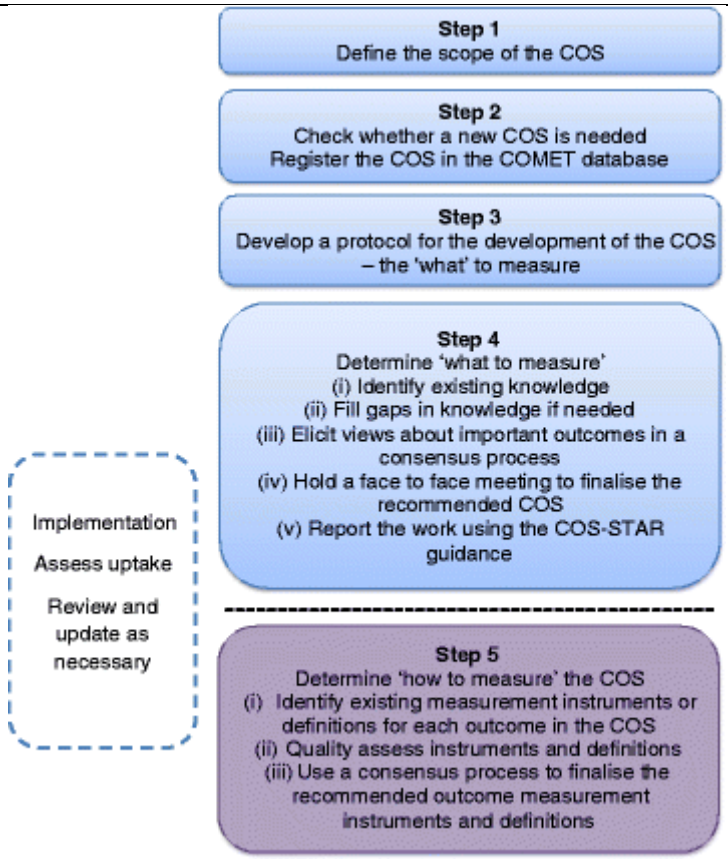
Orphan Drug Development Guidebook

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	COMET Initiative
References	http://www.comet-initiative.org/
Description	The COMET 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, but COS are also suitable for use in routine care, clinical audit and research other than randomised trials. The existence or use of a core outcome set does not imply that outcomes in a particular study should be restricted to those in the relevant core outcome set. Rather, there is an expectation that the core outcomes will be collected and reported, making it easier for the results of studies to be compared, contrasted and combined as appropriate; while researchers continue to explore other outcomes as well. The figure below illustrates the core outcome set (COS) development process.

	
Category	Development practices Building Block
Geographical scope	European Union
Availability	The COMET Initiative has developed a database of all studies relevant to the development of core outcome sets for use in clinical trials. .https://www.comet-initiative.org/studies/. Database is publicly available for applicants developing medicines for rare and non-rare diseases The COMET Handbook: version 1.0 is available at this link: https://trialsjournal.biomedcentral.com/articles/10.1186/s13063-017-1978-4
Scope of use	COMET aims to collate and stimulate relevant resources, both applied and methodological, to facilitate exchange of ideas and information, and to foster methodological research in this area. Specific objectives are to: 1. Raise awareness of current problems with outcomes in clinical trials

	2. Encourage COS development and uptake 3. Promote Patient and Public Involvement (PPI) in COS development 4. Provide resources to facilitate these aims 5. Avoid unnecessary duplication of effort 6. Encourage evidence-based COS development
Stakeholders	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. Guideline developers – NICE, CMTP, GIN Industry – EFPIA Journal editors – CROWN Patients and the public – PoPPIE Regulators – EMA , FDA Systematic reviewers – Cochrane Trial funders – NIHR, ARUK, AMRC, HRB Ireland, Horizon 2020 Trialists – SPIRITS guidelines
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 (any phase).
Expert tips	None