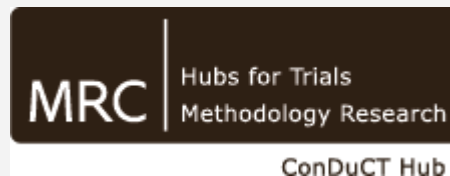


Selection and development of PROMs

Dr Sara Brookes & Dr Kerry Avery



Session overview

- Types of outcome
- Selecting outcomes (PRECIS tool)
- Core Outcome Sets
- Why measure PROs
- Types of PROMs
- Issues around selecting PROMs
- Developing PROMs - psychometric testing

What is an outcome

- An event that is either present or absent after participants receive an intervention or exposure which can be measured and compared

Types of outcome

observer dependent

health
economic

clinical

patient-reported

objective

patient-centred

observer independent

patient-based

subjective

Types of outcome measures

WHO ICF	Clinician-reported	Patient-reported
Body function/ structure	National Institutes of Health Stroke Scale	General Health Questionnaire - 28
	Mini-Mental State Examination	Beck Depression Inventory
	Clock Drawing Test	Geriatric Depression Scale
Activities	Barthel Index	Frenchay Activities Index
	Modified Rankin Scale	
	Nine-hole Peg-Test	
Participation		Nottingham Health Profile
		Stroke Impact Scale
		Stroke-Adapted Sickness Impact Profile

Selecting outcomes

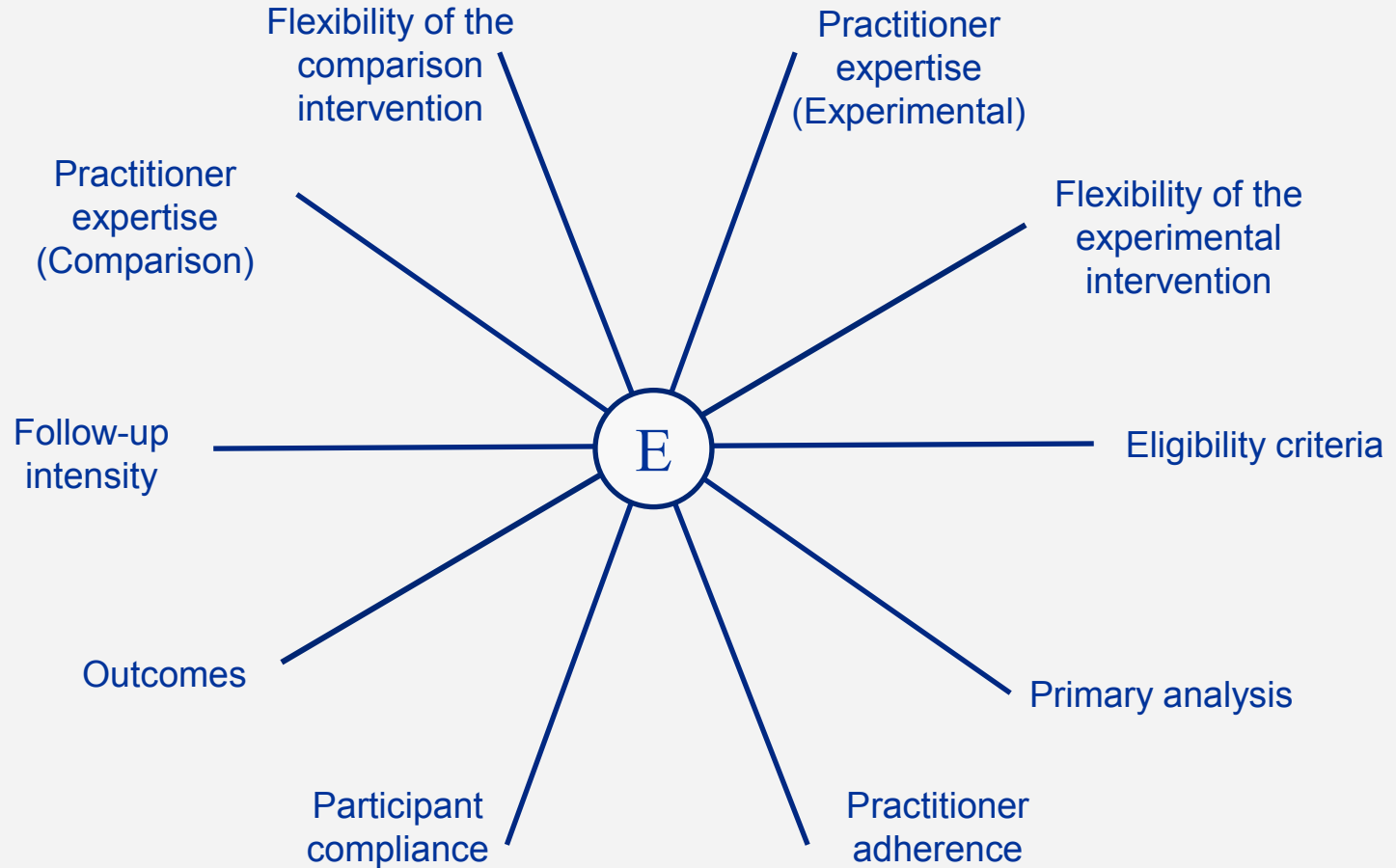
Trial design decisions need to be consistent with the trial's stated purpose

- PRECIS: a pragmatic-explanatory continuum indicator summary (Thorpe. J Clin Epidemiol 2009 62:464-475)
- Graphical summary to place trial on pragmatic-explanatory continuum
- To assess degree with which design is aligned with trial's stated purpose
- Still under development

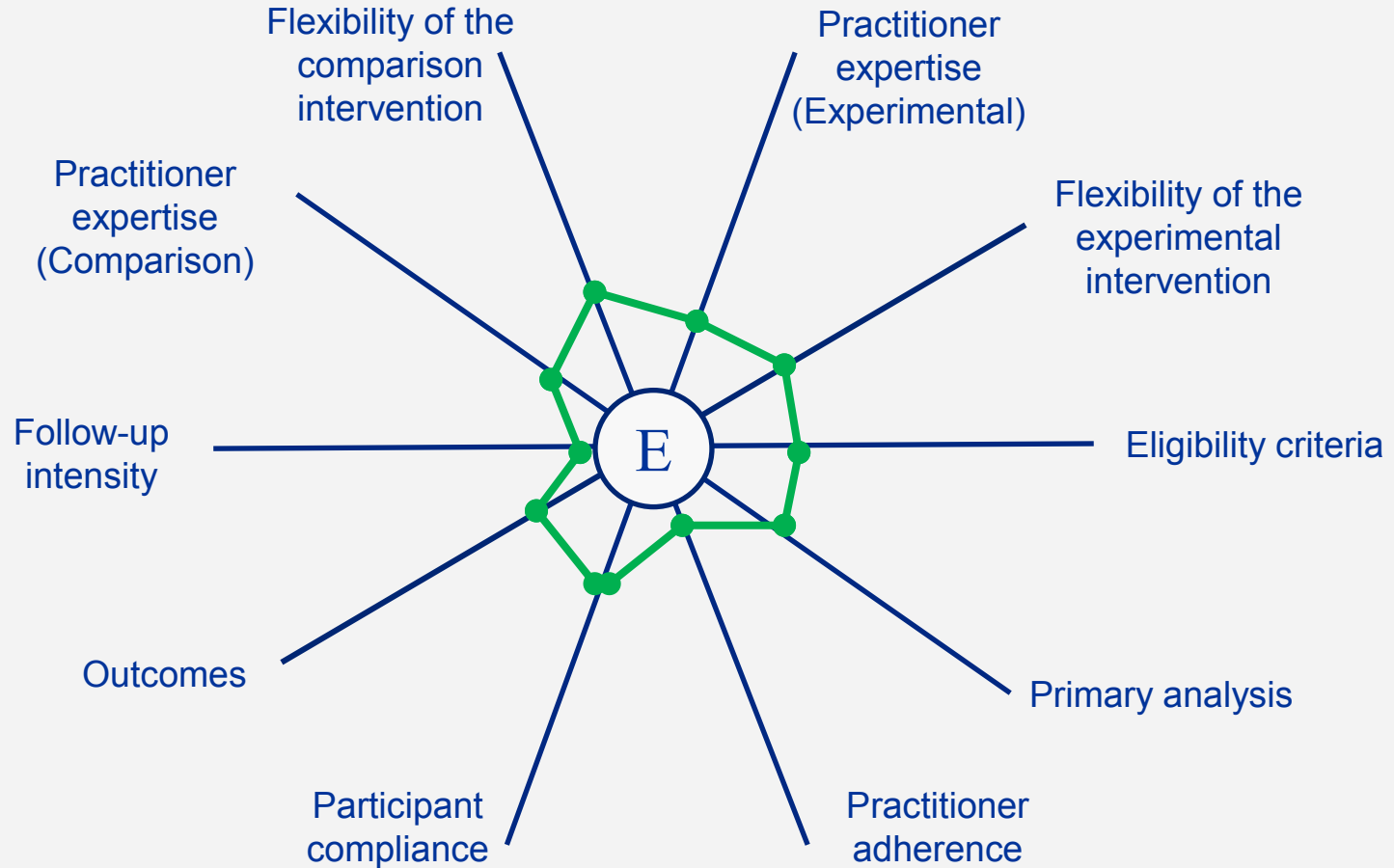
Selecting outcomes

Explanatory	Pragmatic
Central outcome adjudication	No central outcome adjudication
Additional training/measurement	Assessed under usual circumstances
Short-term	Long-term
Intensive follow-up	No additional follow-up
Important outcome to clinicians	Meaningful outcome to patients
Outcomes most believed to be consequence of intervention	More uncertainty

PRECIS tool

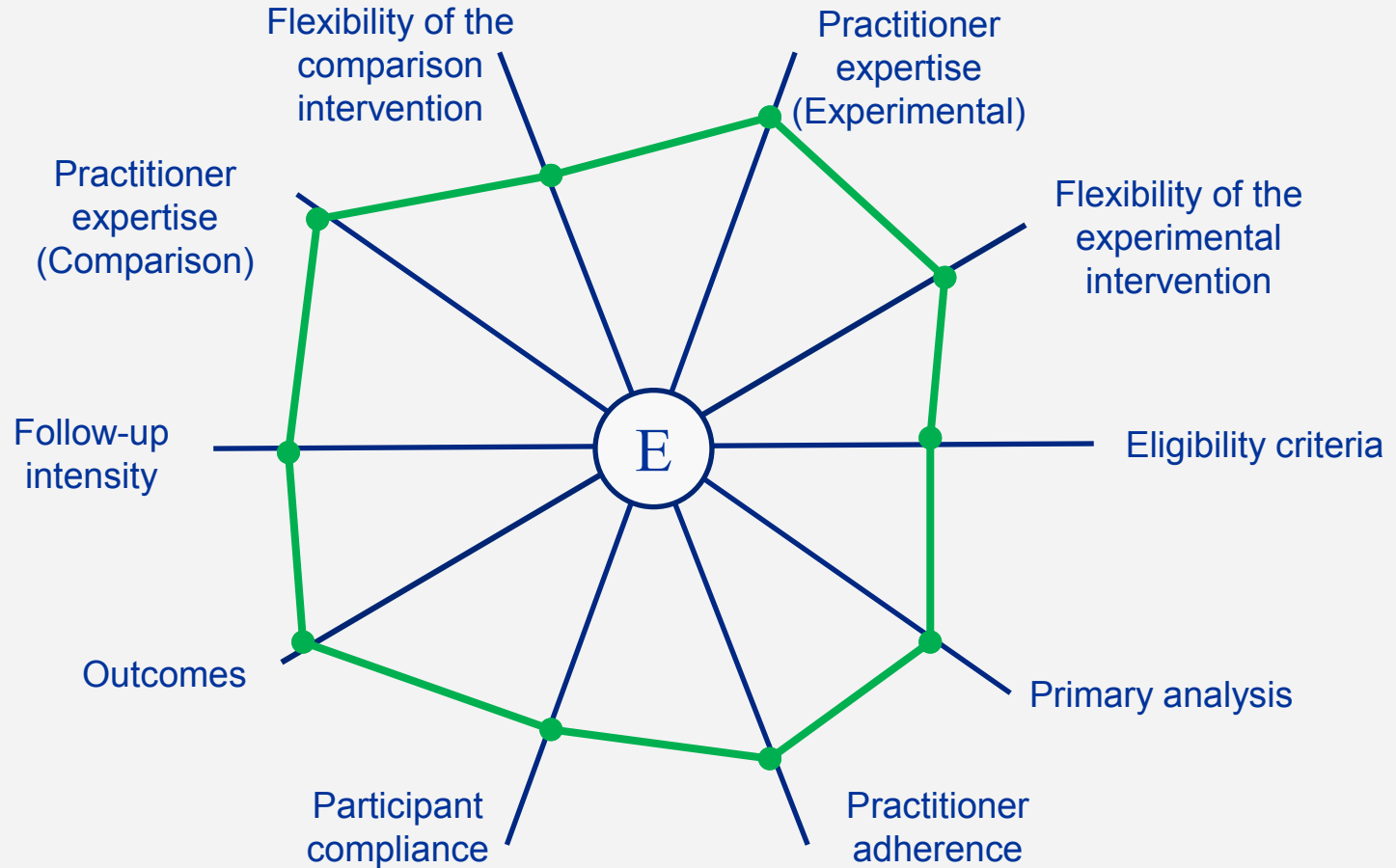


PRECIS tool



NASCET: carotid endarterectomy + best medical therapy vs. best medical therapy

PRECIS tool



Continued intervention from a stroke nurse after discharge from hospital

Core Outcome Sets (COS)

- An agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical trials in specific areas of health or health care.

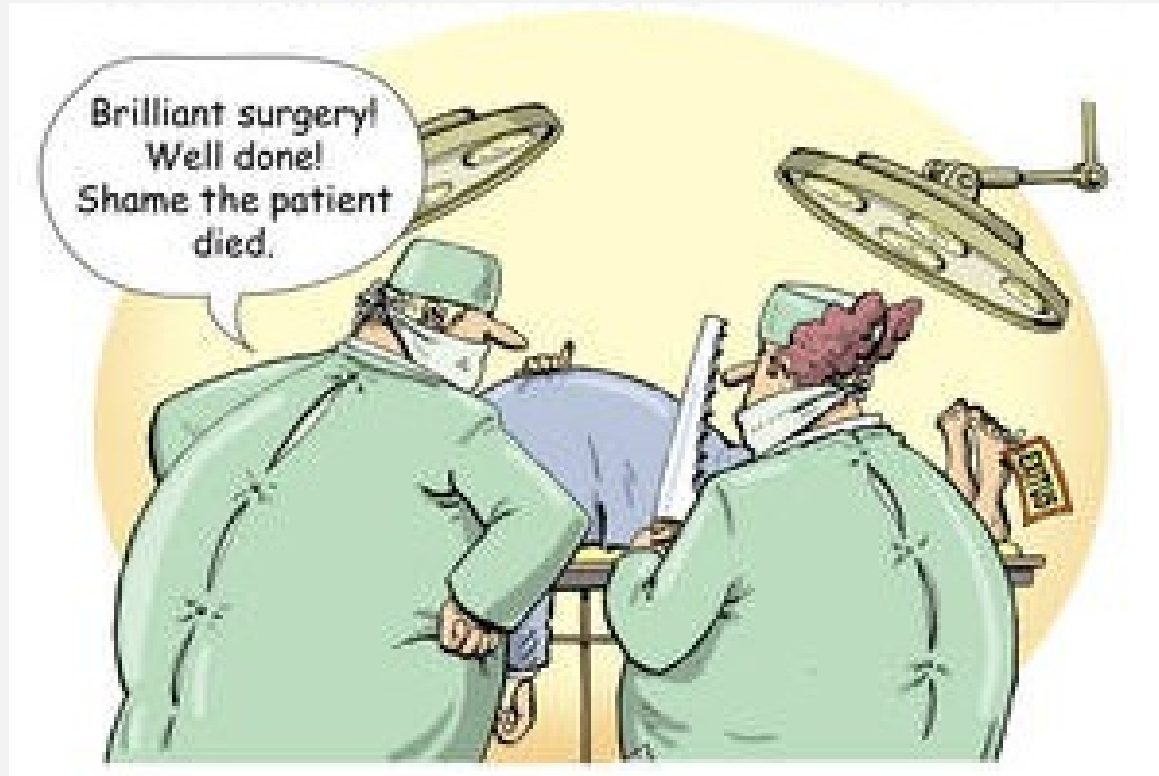
Core Outcome Sets



Core Outcome Measures in Effectiveness
Trials

www.comet-initiative.org

Why measure PROs



© Governance International

Why measure PROs

- Patients interested in different things
- Patients views differ from observers
- Capture patients overall experience of disease



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Types of PROMs

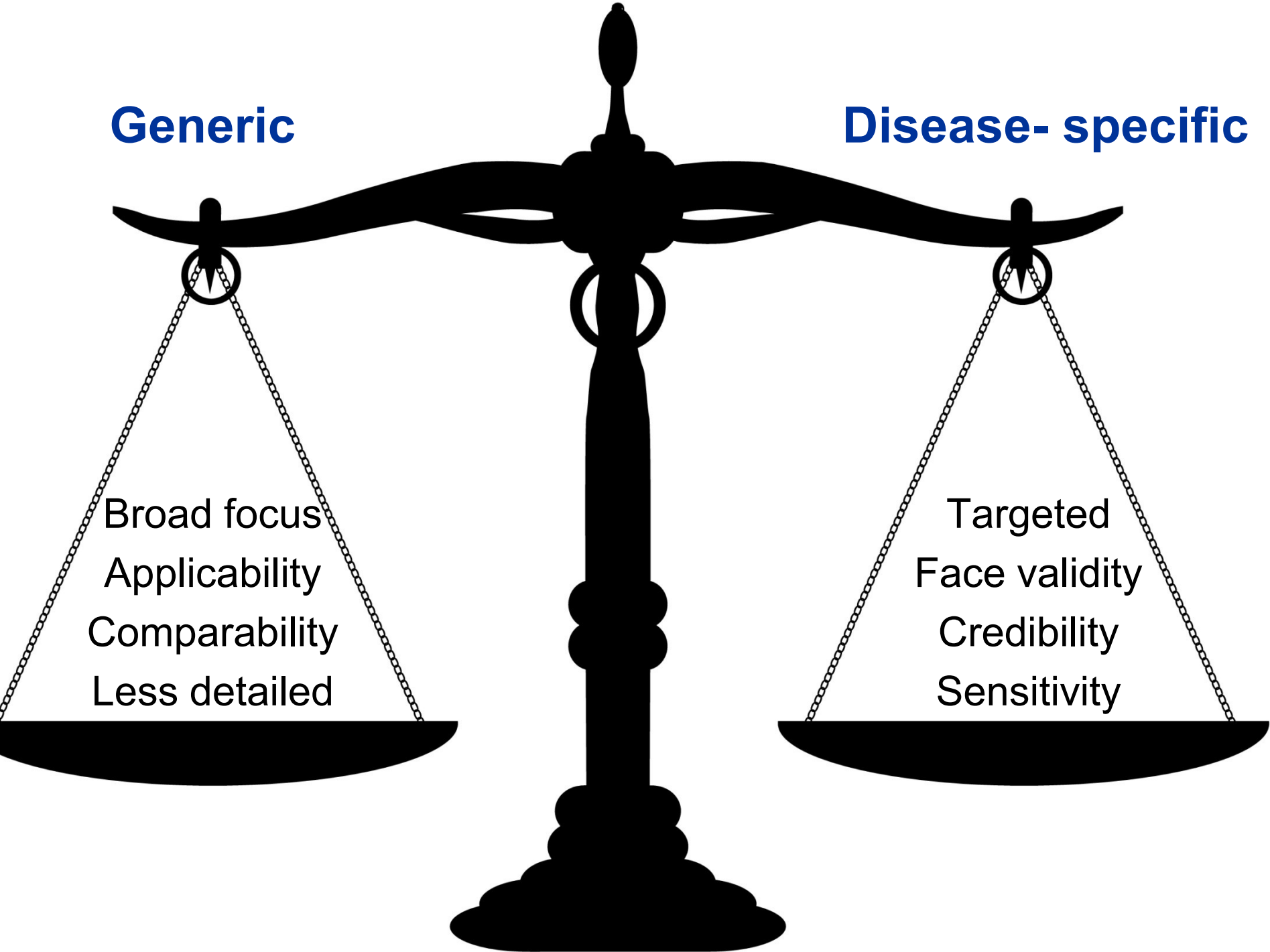


Types of PROMs

Scope	Domains	Purpose	Example PROM	Example item
Generic	Multi-dimensional	Measure broad aspects of health	SF-36	<i>In general, would you say your health is...</i>
Disease-specific	Multi-dimensional	Address complex and unique areas of function impaired in specific diseases	SS-QOL	<i>Did you have trouble finding the word you wanted to say?</i>

Generic

Disease-specific



Selecting PROMs

Advantages of using existing PROMs

- ✓ Cheaper, easier, quicker
- ✓ Facilitates comparison of data across studies
- ✓ Maximises consensus within a field
- ✓ Avoids repetition



Guidance for PROM selection



Selection: issues to consider

- Trial objective(s)
- Scope
- Study population
- Psychometric properties
- Scoring system
- Practical issues



Avery KNL & Blazeby JM. Quality of life assessment in surgical oncology trials. World J Surg (2006);30.

Fitzpatrick R et al. Executive summary: evaluating patient-based outcome measures for use in clinical trials. HTA Programme 14, 1998.

Selection: resources

- Expert opinion
- Published research, trials, audit, etc.
- Online databases...

e.g. www.PROQOLID.org

Home > Quick Search Engine

Search criteria

[Advanced search](#)

Total documents found: 7

Abbrev.	Full name	Author(s)	Pathology(s)
NEWSQOL	Newcastle Stroke-specific Quality of Life measure	Buck Deborah Ford Gary A Jacoby Ann et al.	Cardiovascular diseases Nervous system diseases
NHSS	National Institutes of Health Stroke Scale	NINDS Investigators	Cardiovascular diseases Nervous system diseases
SAQOL-39	Stroke and Aphasia Quality of Life Scale - 39 item version	Hilari Katerina	Cardiovascular diseases Nervous system diseases Pathological conditions signs and symptoms
SIS	Stroke Impact Scale & Stroke Toolbox	Duncan Pamela W Lai Sue M Studenski Stephanie	Cardiovascular diseases Nervous system diseases
SS-QOL	Stroke-Specific Quality Of Life measure	Williams Linda S	Cardiovascular diseases Nervous system diseases
CNS	Canadian Neurological Scale	Cote Robert	Cardiovascular diseases Nervous system diseases
mRS-SI	Structured Interview for the Modified Rankin Scale	Hareendran Asha Wilson Lindsay	Cardiovascular diseases Nervous system diseases

Developing PROMs



Validity

Does the PROM measure what it claims to?

Face: do the items appear to be measuring the qualities they claim to measure? Do the questions make sense?

Content: do the components of the PROM cover *all* aspects of the attribute to be measured?

Criterion: does the PROM correlate with the 'gold standard' measure?

Construct: relationship between PROM and hypotheses surrounding construct(s) it measures

Reliability

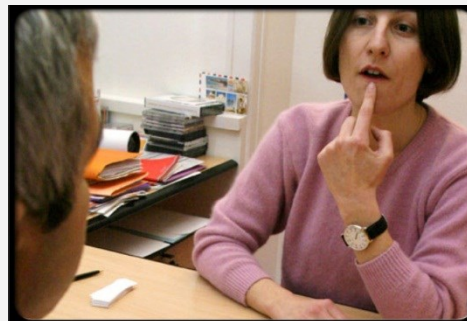
Does the PROM behave consistently?

- Internal consistency
 - ❖ Cronbach's alpha
- Test-retest reliability
 - ❖ Interval between testing
 - ❖ Practice effects
- Inter-observer reliability
 - ❖ Consistency between different observers
- Intra-observer reliability
 - ❖ Consistency within the same observer/responder

Sensitivity to change

Is the PROM responsive to changing circumstances (e.g. treatment)?

- Big changes should be detectable – if not your tool is not working well
- Little sensitivity to change means that you will not be able to detect outcomes



Developing the final version

■ Finalising content

- ❖ Various techniques – factor analysis, principal component analysis, multi-trait scaling, item response theory
- ❖ Confirming structure, identifying domains
- ❖ Item reduction – removal of redundant items

■ Scoring

- ❖ Factor analysis, multi-trait scaling
- ❖ Relationship between items
- ❖ Expert judgement
- ❖ Validation of scoring system



Thank you

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