



# Patient-reported outcomes and PROMs

**KEMTA Masterclass**  
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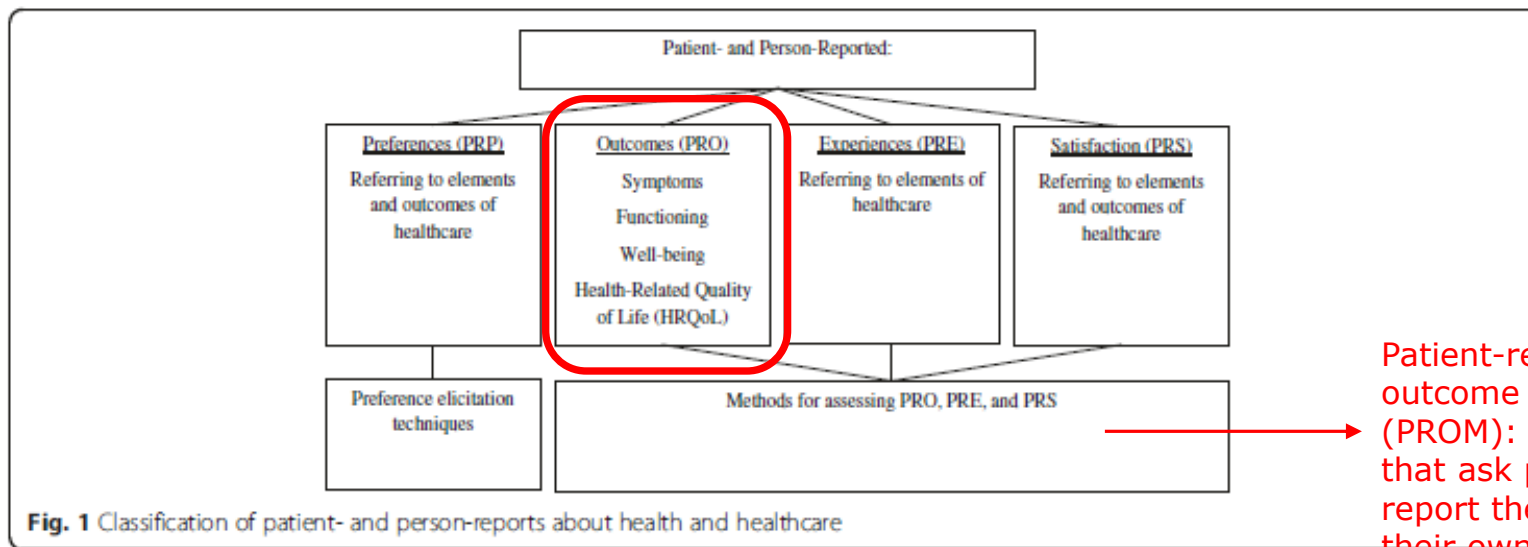
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# Content

- Patient-reported outcomes
  - What are patient-reported outcomes?
  - Why measure them?
- Selecting PROs
  - Conceptualisations of health and QoL
  - Core Outcome Sets
- Selecting PROMs
  - Type of PROMs
  - Validity testing

# Patient-reported outcomes

- PRO is frequently used as an umbrella term for health outcomes that are directly and subjectively reported by patients.
- Classification Klose et al (2016). Klose K, Kreimeier S, Tangermann U, Aumann I, Damm K; RHO Group. Patient- and person-reports on healthcare: preferences, outcomes, experiences, and satisfaction - an essay. *Health Econ Rev.* 2016;6(1):18. doi:10.1186/s13561-016-0094-6



Patient-reported outcome measure (PROM): tools (surveys) that ask patients to report their views on their own health

# Why measure PRO?

## Support clinical care & shared decision-making

- To characterize what the patient has **experienced** as the result of medical care: supplements to physiological or biological measures of health status.
- Enhance connection/communication between patient and doctor & set treatment goals



# Evidence?

Evidence that use of PROMs in clinical practice can:

- improve communication between health care personnel and patients
- facilitate shared medical decision-making
- enable personalized care
- improve patient satisfaction
- possibly improve HRQoL

**\*Yang, L.Y., et al., Patient-reported outcome use in oncology: a systematic review of the impact on patient-clinician communication. Support Care Cancer, 2018. 26(1): p. 41-60.**

**\*Graupner, C., et al., Patient outcomes, patient experiences and process indicators associated with the routine use of patient-reported outcome measures (PROMs) in cancer care: a systematic review. Support Care Cancer, 2020.**

**\*Kotronoulas, G., et al., What is the value of the routine use of patient-reported outcome measures toward improvement of patient outcomes, processes of care, and health service outcomes in cancer care? A systematic review of controlled trials. J Clin Oncol, 2014. 32(14): p. 1480-501.**

**\*Ishaque, S., et al., A systematic review of randomised controlled trials evaluating the use of patient-reported outcome measures (PROMs). Qual Life Res, 2019. 28(3): p. 567-592.**

# Why measure PRO?

## Effectiveness research

- Did the treatment really improve outcomes, or just change a biomarker that doesn't translate into a subjective improvement for the patient?
  - Avoid measuring irrelevant outcomes that will not change practice
- PRO data on trajectory of symptoms and HRQL throughout treatment
  - Improving HRQoL may be the most important outcome in many diseases with a chronic course and without cure
- PRO as a sensitive measure of treatment-related toxicities
- Complimenting AE reporting by clinicians
  - underreporting for fatigue, anxiety, pain
- Self-reported AE associated with treatment discontinuation
- Comparison of patient self-report to objective assessments

# Why measure PRO?

## To learn & improve quality

- Person-centered care: focus on outcomes that matter to patients: clinical outcomes AND patient-reported outcomes (e.g. QoL, pain) → quality
  - Within MUMC (and other UMCs) measure a generic set of patient relevant outcomes (e.g. fatigue, pain, emotional wellbeing)
- PRO data aggregated → internal and external quality assessment

# Why measure PRO?

## Reimbursement decisions

- A required part of the evidence used in the appraisal of, and decision-making about, health technologies (e.g. National Health Care Institute, FDA, NICE appraisals).

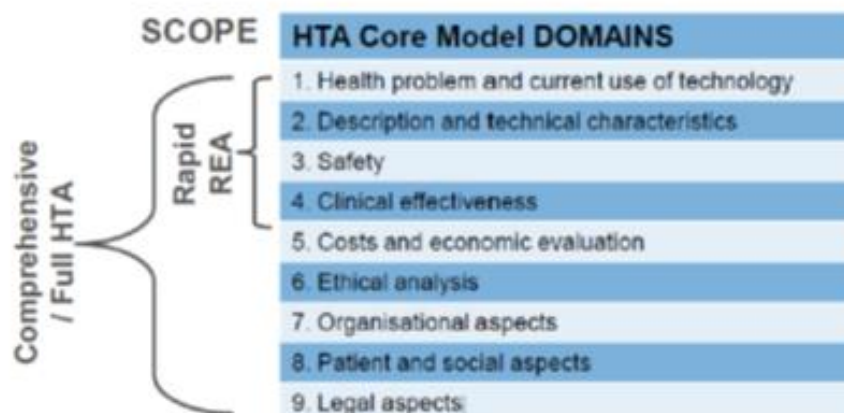


Figure 1. HTA Core Model domains as developed by the EUnetHTA JA

# Selecting outcomes

- What is health and what is relevant for my patients?
- Core Outcome Sets
  - Standardize outcome (measures) to enable comparison and learning!
- Literature review (e.g. previous clinical studies, qualitative studies)
- Involve patients and their perspective

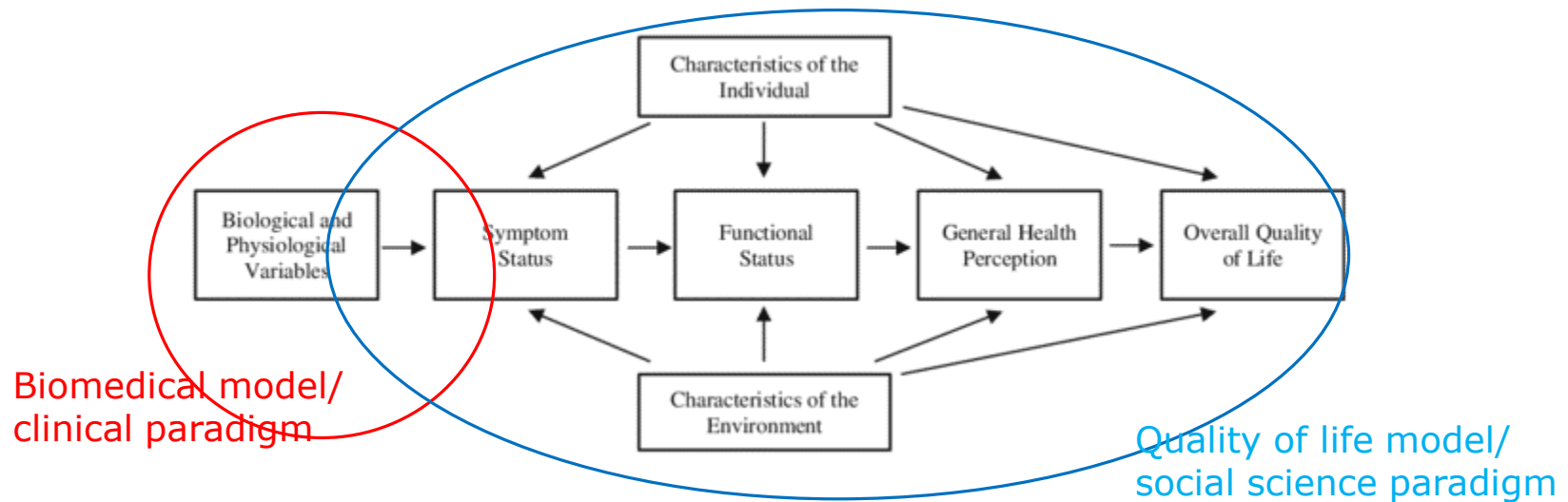


# Theories of health

- **Boorse:** Health as the absence of disease (Boorse 1977, 2014)
- **WHO's definition of health:** 'A state of complete physical, mental and social well-being, and not merely the absence of disease and infirmity'
- **Positive health:** Health as the ability to adapt and self-manage in the face of social, physical and emotional challenges (Huber, 2011)
- **The capability approach** is an alternative framework for conceptualising wellbeing for public policy that defines wellbeing in terms of what an individual can 'do' and 'be' in their life (Amartya Sen, 1983).

# Selecting health outcomes

- **Wilson & Cleary (1995)** Wilson IB, Cleary PD. Linking clinical variables with health-related quality of life. A conceptual model of patient outcomes. JAMA. 1995;273:59–65.
  - Taxonomy of patient outcomes
  - Categorizes patient outcomes according to underlying health concepts



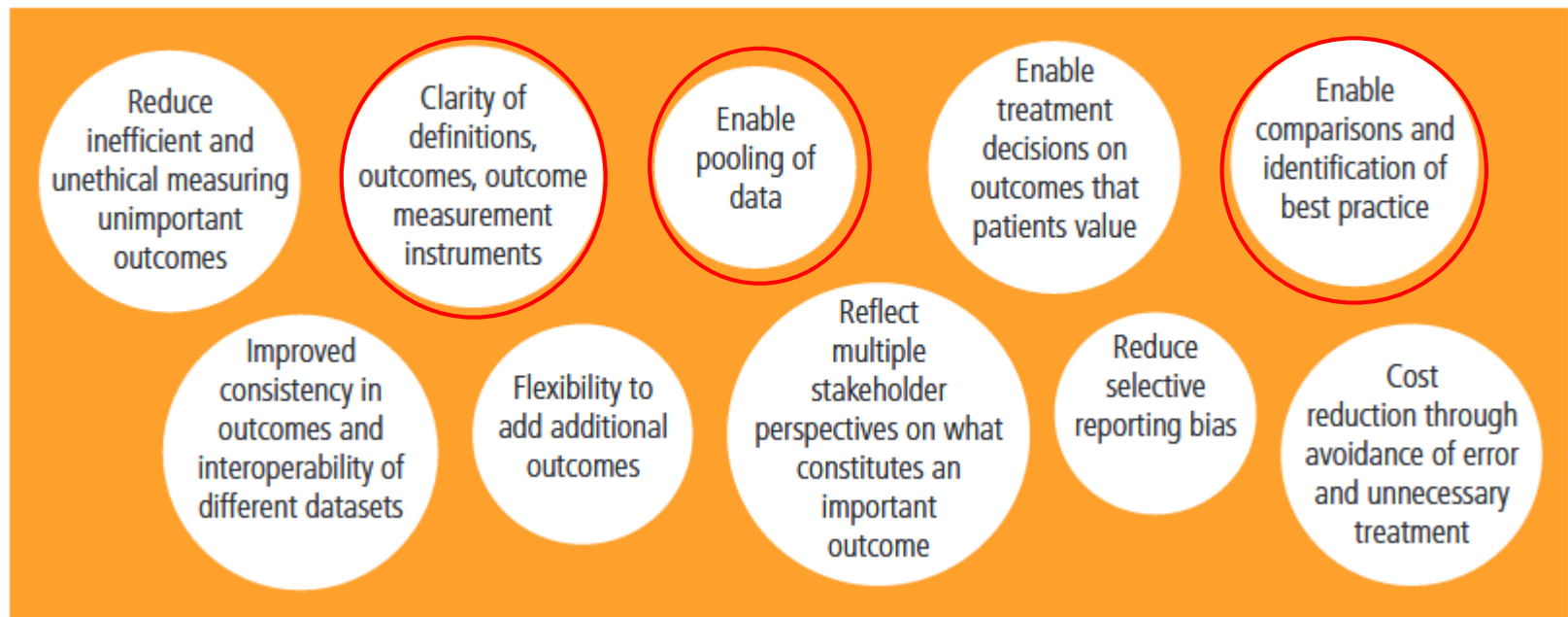
# What are Core Outcome Sets?

“an agreed standardised set of outcomes... [that] 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”.

<https://youtu.be/AlLc2yNopII>

# Potential benefits of a COS

FIGURE 1 Potential benefits of developing and using COS



Innovative Medicines Initiative (IMI) Big data for better outcomes (BD4BO) (2018). *A Practical Toolkit for the identification, selection and measurement of outcomes including in realworld settings*. Available at: <http://bd4bo.eu/index.php/toolkit>



International **C**onsortium for **H**ealth **O**utcomes  
**M**easurement: <https://www.ichom.org/>



Core **O**utcome **M**easures in **E**ffectiveness **T**rials:  
[www.comet-initiative.org](http://www.comet-initiative.org)



**C**onsensus-based **S**tandards for the selection of  
health **M**easurement **I**nstruments:  
<https://www.cosmin.nl/>

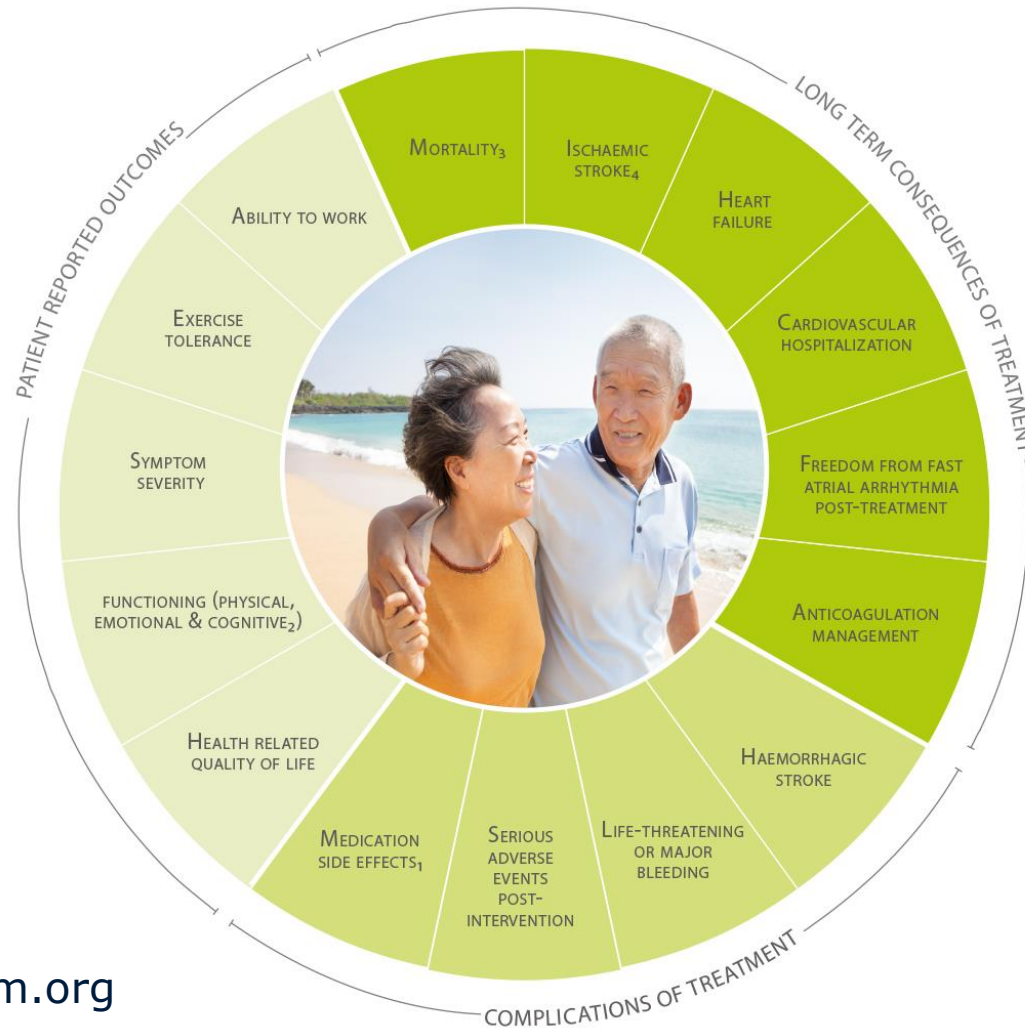


Cochrane Skin **C**ore **O**utcome **S**et **I**nitiative:  
<http://cs-cousin.org/>



**O**utcome **M**easures in **R**heumatology:  
<https://omeract.org/>

# ICHOM Standard Set –Atrial Fibrillation



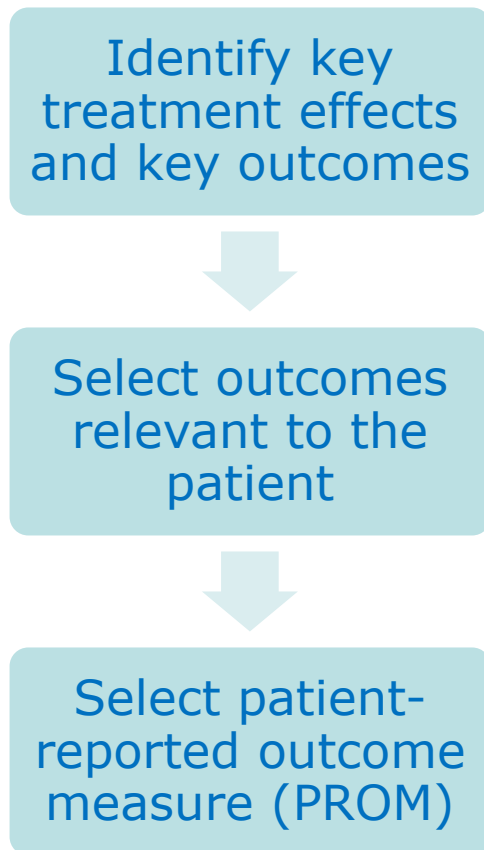
Source: [www.ichom.org](http://www.ichom.org)

# No Core Outcome Set?

- Literature review
- Talk to patients!
  - Conduct a qualitative study
  - Include patient representatives

Patient-based evidence and patient involvement!

# Selecting a PROM



Systematic reviews of PROMs in your study population

<https://www.cosmin.nl/tools/database-systematic-reviews/>

Literature review

<https://www.cosmin.nl/finding-right-tool/available-outcome-measurement-instrument/>

Other sources (e.g. online databases  
[www.meetinstrumentenindezorg.nl](http://www.meetinstrumentenindezorg.nl))

Quality assessment

PROMIS database

# Types of PROMs

- **Generic:** physical, mental and social aspects of health

- Comparison between diseases
- Population norms may be available
- Sensitive to multiple aspects of treatment
- Insensitive to small changes

Examples: SF-36, WHO-QoL, EQ-5D

- **Condition/disease or function specific**

- Sensitive to small and relevant changes
- Support clinical decision making
- Measure of efficacy or effectiveness

Examples: Atrial Fibrillation Quality of Life (AFQoL), Asthma (AQLQ), Knee / Hip (Oxford Knee Score), High Blood pressure (Bulpitt), cancer (EORTC)

How to choose? Is the objective to gain an overall understanding of the patient's health status, or is the goal to gain a more detailed view of the patient? Frequently, both generic and condition-specific PROMs are used together to get the full picture.

# Quality assessment of PROMs

## Measurement properties (evidence in target population?)

We use the ISOQOL minimum standards to guide our PROM research

|   |                                |    |                                               |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |
|---|--------------------------------|----|-----------------------------------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| 1 | Conceptual & measurement model | 1a | Conceptual and measurement model              | <ul style="list-style-type: none"> <li>The rationale for and description of the concept and the populations that a measure is intended to assess and the relationship between them</li> </ul>                                                                                                                                                                                                                                                                                                                     |
|   |                                | 1b | Target population                             |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |
| 2 | Reliability                    | 2a | Test - retest reliability (= reproducibility) | <ul style="list-style-type: none"> <li><b>Reliability:</b> degree to which the PROM is free from random error</li> <li><b>Test-retest reliability (reproducibility)</b> Stability of scores over time when no change is expected in the concept of interest</li> <li><b>Internal consistency</b> Extent to which the items comprising a scale measure the same concept. The intercorrelation of items that contribute to a score.</li> </ul>                                                                      |
|   |                                | 2b | Internal consistency                          |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |
| 3 | Validity                       | 3a | Content validity                              | <ul style="list-style-type: none"> <li><b>Validity:</b> degree to which the instrument measures what it purports to measure</li> <li><b>Content-validity</b> appropriateness and comprehensiveness of the items and domains</li> <li><b>Construct validity</b> hypotheses concerning logical relationships that should exist with measures of related concepts or scores produced in similar (or diverse) patient group</li> <li><b>Responsiveness</b> instrument's ability to detect change over time</li> </ul> |
|   |                                | 3b | Construct validity                            |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |
|   |                                | 3c | Responsiveness                                |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |
| 4 | Interpretability               |    | Interpretability                              | <ul style="list-style-type: none"> <li>The degree to which one can assign easily understood meaning to an instrument's quantitative scores.</li> </ul>                                                                                                                                                                                                                                                                                                                                                            |
| 5 | Translations                   |    | Translations                                  | <ul style="list-style-type: none"> <li>Cultural and linguistic adaptation and validation of the instrument</li> </ul>                                                                                                                                                                                                                                                                                                                                                                                             |
| 6 | Assessment burden              | 6a | Patient burden                                | <ul style="list-style-type: none"> <li>The resources needed to administer the instrument (time, effort, and other demands)</li> </ul>                                                                                                                                                                                                                                                                                                                                                                             |
|   |                                | 6b | Clinician & admin.burden                      |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                   |

Source: Reeve BB, Wyrwich KW, Wu AW, Velikova G, Terwee CB, Snyder CF, Schwartz C, Revicki DA, Moinpour CM, McLeod LD, Lyons JC, Lenderking WR, Hinds PS, Hays RD, Greenhalgh J, Gershon R, Feeny D, Fayers PM, Cella D, Brundage M, Ahmed S, Aaronson NK, Butt Z. ISOQOL recommends minimum standards for patient-reported outcome measures used in patient-centered outcomes and comparative effectiveness research. Qual Life Res. 2013 Oct;22(8):1889-905.

# Translation of PROMs

# Critique.... What do you think?

- Feasibility of measurement of PROs?
  - No time...
  - No money...
  - No ICT support...
- Too subjective...
  - Purposefully so!
- Benefits of use in daily clinical practice uncertain
- Valid ways to measure? Which PROM to select?
  - Thousands of PROMs... The quality of these instruments, in terms of their reliability and validity, varies considerably

# Take home messages

- What is health?
- What outcomes are relevant *for your patient (population)?*
- Identify valid PROMs – check carefully



## Relevant sources

- <https://training.cochrane.org/handbook/current/chapter-18>
- <https://www.ichom.org/>
- [www.comet-initiative.org](http://www.comet-initiative.org)
- <https://www.cosmin.nl/>
- <https://www.zorginzicht.nl/ondersteuning/prom-cyclus/over-de-prom-cyclus>