An overview of patient-reported outcome measures

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Patient-reported outcomes, an overview

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Conflict of Interest

Speaking fees from MSD, Gilead, ViiV

Expert Board Gilead


GREAT NEWS, YOU ARE "UNDETECTABLE", YOU'RE RESPONDING WELL TO THE ANTIRETROVIRAL THERAPY THAT WE PRESCRIBED.
BUT THIS DIAGNOSIS HAS AFFECTED MY WHOLE LIFE.
I AM HAVING TROUBLE SLEEPING ...
I FEEL TIRED, I KEEP GETTING THESE HEADACHES...

What is the matter with you ?

versus

What matters to you ?

Patient-reported outcome measures (PROMs) are tools which help us determine what matters to individuals in care.

Patient-reported outcomes (PRO)

« any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else »

- Food and Drug Administration (FDA)

US Food and Drug Administration. Guidance for industry: patient-reported outcome measures: use in medical product development to support labeling claims. Federal Register. 2009;74(235):65132–65133.

Patient-reported outcome measure (PROM)

- Standardized questionnaires or instruments designed to assess **patient-reported outcomes**
- Completed by the patient
- Provide valuable information about the patient's experience of their condition *(e.g. symptoms, functioning, quality of life, and other aspects of their health)*
- Used to
  - improve patient-physician communication
  - monitor patient outcomes
  - guide clinical decision-making
  - enhance patient-centered research and care

Devlin NJ, Appleby J. Getting the most out of PROMs: Putting health outcomes at the heart of NHS decision-making. The King's Fund; 2010.

An origins story...

1920 : Assess symptoms in tuberculosis patients (St. George's Respiratory Questionnaire and the Tuberculosis Symptoms Index)

1950 : RAND-36 Health Survey, a precursor to the widely used Short Form-36 (SF-36) questionnaire, was developed.

1980/90 : PROMs explosion !

2000 : PROMs used in clinical trials and healthcare quality improvement initiatives.

Development of guidelines for the selection, administration, and interpretation of PROMs in clinical research and practice.

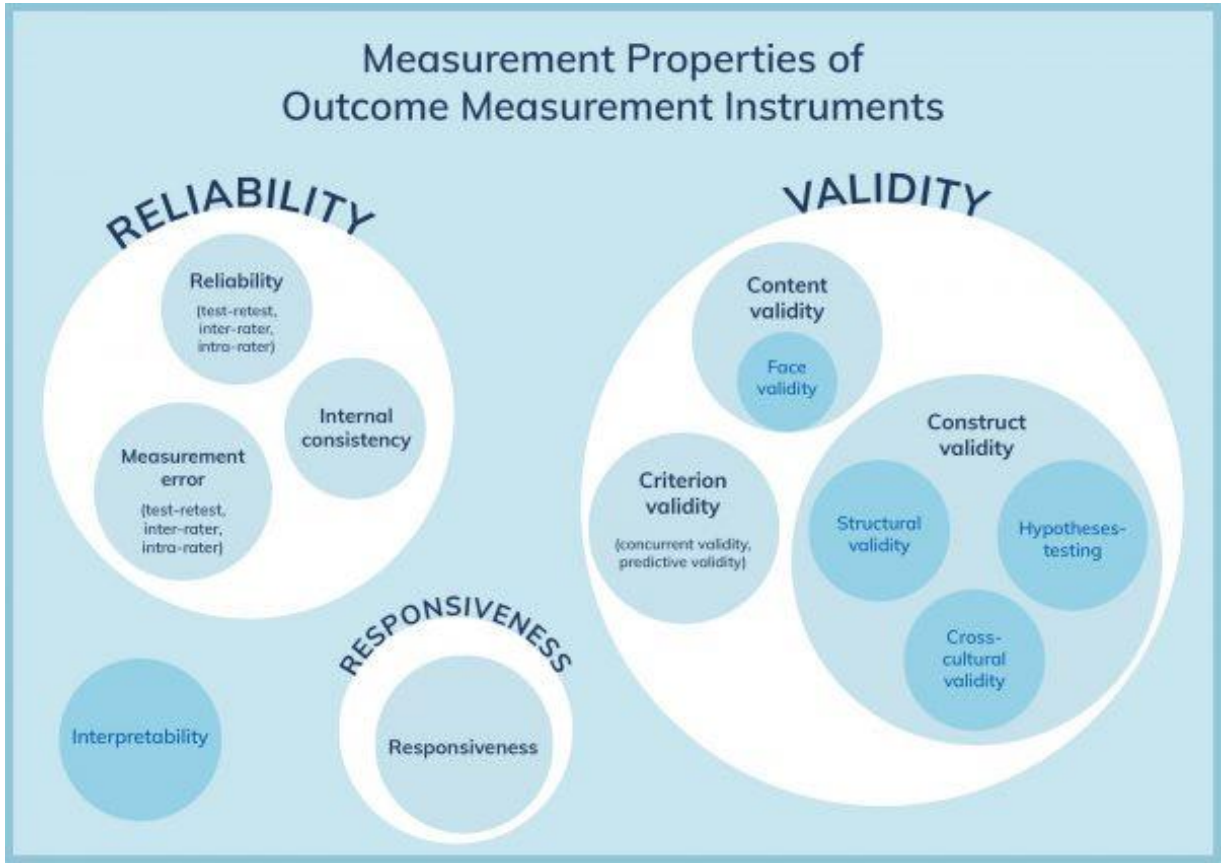
Types of PROMs & applications

- Generic versus (disease) specific ?
  - broad aspects of quality of life, health status versus unique concerns specific to a disease/condition
  - applicable to ***all*** populations versus to a specific disease/condition, sensitive to changes
  - specificity, depth
  - not mutually-exclusive

- Applications : clinical trial, clinical practice, quality improvement

What makes a good PROM ?

- Relevance
- Reliability
- Validity
- Responsiveness
- Interpretability
- Feasibility / Acceptability
- Equitable/ Inclusive



Mokkink, L. B., C. B. Terwee, D. L. Patrick, J. Alonso, P. W. Stratford, D. L. Knol, L. M. Bouter and H. C. de Vet (2010). "The COSMIN checklist for assessing the methodological quality of studies on measurement properties of health status measurement instruments: an international Delphi study." Qual Life Res **19**(4): 539-549.

PROMs for HIV ?

- **Good news**, we have options !
  - 117 HIV-specific PROMs identified *Engler, Lessard et al. 2017*
  - 9 generic and 7 HIV-specific **health-related quality** of life PROMs *Cooper et al., 2017*
  - Newer instruments reflecting needs and priorities of people with HIV in the treat-all era (e.g. Positive Voices (UK)) *Harding et al. 2021*
  - Conducive environment : instruments, advances in measurement, technology, stakeholder motivation

- **Bad News**
  - Absence of a “gold standard” in terms of PROMs for quality of life
  - Choice of the scale is a function of specific research question and end use
  - Poor practices (selection, reporting, etc.)
  - Relevance ?

Final thoughts

- Why were they developed ?
- When were they developed ?
- What is clinically meaningful change in people living with HIV who are maintained on treatment ?
  - Should we be thinking about “clinically meaningful change” differently now that HIV is a chronic condition ?
- How do we validate new instruments in populations where fewer an fewer people are expected to experience advanced disease (AIDS) yet still consider them “disease-specific” ?
- Do we still need HIV-specific instruments ?

Thank you for your attention