PATIENT-REPORTED OUTCOME MEASURES (PROM) IN CLINICAL RESEARCH

Professor Pernilla Lagergren
Department of Surgery and Cancer

OUTLINE

- PROMs – What are they and why should we use them
- How to choose our PROMs – What to look for and what to avoid
- Analysing and interpreting PROM data
DEFINITION PROMS

PRO/PROMs – an umbrella term that covers a whole range of potential types of measurement but is used specifically to refer to self-reports by the patient.

“A measurement based on a report that comes directly from the patient about the status of a patient’s health condition without amendment or interpretation of the patient’s response by a clinician or anyone else”

(FDA, 2009)

DIFFERENT PRO

Symptoms
Functioning
Health-related quality of life (HRQOL)/Quality of life (QOL)
Health behaviours
Psychosocial outcomes
Satisfaction with care/information (=PREM)

Self-assessment providing patient’s perspective
PROMs – What are they and why should we use them

WHY USE PROMS?

Clinical outcomes not always related to how the patient feels
• "Good" medical outcomes ≠ "good" patient outcomes

Autonomy reasons
• Informed patients
  • Expectations in a short and long term perspective

Facilitate patient-clinician communication

Enhance patient care/outcomes?
• Rehabilitation program and guidance of follow-up

Requirement for RCTs and Quality registers
Screening tool? Prognostic tool?

PROMs – What are they and why should we use them

REASONS NOT TO USE PROMS?

Burden of the patient

Time consuming for clinicians (and patients?)
• If used in the clinics one should evaluate the results directly
HOW TO MEASURE

Interviews
Questionnaires
  • Paper format
  • Computer based
Diaries
Applications

PROMS – What are they and why should we use them

MEASURES: QUESTIONNAIRES

Study specific questionnaire
  • Own developed questionnaire for a particular study
Generic
Disease-specific
Aspect-specific
HOW TO CHOOSE PROMS

How do we choose the right measure for our study aim?
What are you investigating, why, and when?

WHO, WHERE AND WHEN?

Patient clientele – palliative patients?
Setting of care
• Out-patient vs in-patient care
• Short-term stay vs long-term stay

Timing of assessments
• Frequency - one, few or many?
• When?
• Baseline – when is that?

Ability to self-report?
• Proxies?

Number of measures?
• Combination of questionnaires?
Does it measure what we aim to measure? Does it fit with the aim and study design?

Look into what it is measuring and not measuring

Validity
Reliability
Sensitivity
Responsiveness

WHAT ARE THE RIGHT PROPERTIES? CONT.

Validity
Does it measure what it’s supposed to measure?
Absence of systematic errors
• Measurement errors
Reliability

Measure of consistency
- External — stability across time
- Internal — internal consistency
- 'Absence' of random error (chance)

High reliability does NOT guarantee high validity
- Measure exact but maybe the wrong thing

Sensitivity

Detect differences between patients with different status?
- Between patient groups

Responsiveness

Detect changes in patient status over time
WHAT ARE THE RIGHT PROPERTIES? CONT.

Other considerations

Acceptability
- Will people fill it in?

Missing items/cases
- Difficult/Sensitive questions? Plan for handling of missing

Feasibility
- Cost and time consuming
- Ease of scoring and interpretation of scoring
- Supporting documentation (manuals, norm reference scoring etc)

Mode of administration
- In person: Paper questionnaire / face-to-face interview
- Not in person: Postal survey / internet survey

Pilot your PROM!

CHOOSING THE “WRONG” PROM?

Measure the “wrong” outcome
Measure the intended outcome poorly
Misrepresent disease and treatment impact
Mislead clinical practice
Fail to capture the patient’s perspective
Biased results
Etc…
RISK TO CONSIDER WHEN ANALYSING PROM

Multiple testing
- Many scales and items → many analyses
- Risk of chance findings (type I errors)

Reduce the risk
- Predefined hypotheses
- Clinical relevant level before testing for statistical significance
- Lower the statistical significance level

INTERPRETATION OF RESULTS

High scores = good functions or more symptoms

Difficulties
- Is baseline good?
- What does a score of 60 mean?
- How to facilitate the interpretation?
- Linearity
- Floor and ceiling effects
- "Response shift"

When is it clinically relevant?
- Have you lacked appetite?
  - Not at all 1 2 3 4
- Have you vomited?
  - Not at all 1 2 3 4
**INTERPRETATION OF RESULTS CONT.**

Ways to facilitate the interpretation:
- Compare
  - Between groups
  - Reference population
  - Over time
- Clinical significance (Over time or between groups)
  - Small 5-10 scores
  - Moderate 10-20 scores
  - Large >20 scores
- Categorical ('not at all' – 'a little' – 'quite a bit' – 'very much')

Statistical significance vs clinical significance/relevance

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**IN SUMMARY**

- PROM important in addition to other measures
- PROM must be fit for purpose
- Know what you want to measure
- Of sound psychometric properties
- Choose PROM using an evidence-based approach
- Don’t assume the PROM is flawless
- Statistical significance does not equal clinical significance
- Take care in your interpretation of results
THANK YOU!

E-mail: p.lagergren@imperial.ac.uk
Instagram: @professorlagergren
Twitter: @ProfLagergren

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Chair of the group: Pernilla Lagergren
Contact with the group: hpag@imperial.ac.uk