Overview

1. Importance of patient perspective
2. Definitions
3. Measurement of PROs
4. Class exercise: complete & score the SF-36
5. Selecting a PRO for your study

Back in the Day

“Listen to the patient: He is telling you the diagnosis”

- William Osler
Today
Convergence of PRO + CER + EHR

Categories of Patient Outcomes

Problem Solving in Public Health - June 2008
Some Questions Cannot Be Answered Without Asking the Patient

- The main objective of much of health care is improving how patient feels and functions
  - Reduction in pain (hip replacement)
  - Improved functioning (cataract extraction)
- Patient is best judge
- Patient best observer of some events and health outcomes (complications)

Conventional Clinical Measures

- 1. Mortality
- 2. Disease or treatment complications
- 3. Pathology
- 4. Physiologic or lab abnormalities
- 5. Deformity
- 6. Signs and symptoms

Outcomes from Different Perspectives

- Clinical Perspective
- Patient Perspective
  - Subjective health status
  - Quality of life
  - Satisfaction
- Societal Perspective
  - Utilization
  - Cost
Definitions (Apologies…) Imprecise

- Many terms used interchangeably
  - Health
  - (Subjective) health status
  - Functional status
  - Quality of life
  - Health-related quality of life

What is a PRO?

- “Patient-reported outcomes represent the patient’s report of a health condition and its treatment” (Acquadro et al. Value in Health 2003;6:522-531)
- “Any report coming directly from patients (i.e., study subjects) about a health condition and its treatment” (FDA Draft PRO Guidance)
Health-related Quality of Life

- ...encompasses several aspects of health that are directly experienced by the person including physical functioning, social and role functioning, mental health, general health perceptions.

Relationship of Pathophysiology to Subjective Health and QOL

Pathophysiology → Symptoms → Physical/Mental Health → QOL

Wilson & Cleary, JAMA

How do you Measure HRQOL?
A Domain is the specific feeling, function, or perception you want to measure.

Do you feel sad?
**Measuring HRQOL**

- No standard scale, need to specify what we want to measure
- Assemble several indicators which approximate the concept
- Create scale scores by combining responses to questions

Do you feel sad?

Do you feel worthless?

Do you wake up in the middle of the night?
How Do You Measure HRQOL?

- An infinite number of indicators would fully represent the concept
- To be practical, assemble several indicators which approximate the concept
- Create scale scores by combining responses to questions
Response Categories

- Adjective rating response scale
- Ordered responses
- Assigned numerical values

Questions

- Reports and ratings
- Single barrelled (no “and”s)
- Positive and negatively worded
- Redundant
- Range of ability

Check one answer

- All of the Time
- Most of the Time
- A Good Bit of the Time
- Some of the Time
- A Little of the Time
- None of the Time
Content and administration
- Self-administered, also phone or interview
- Standardized (different) 3-6 point response scales
- Time frame last 4 weeks

Language
- Plain language
- 8th grade reading level
- Short questions
- English

Scoring
- Response are assigned numerical values
- Scores summed for each dimension
- 8 Dimensions scored on 0-100 scale
- 2 Summary Score
  - Physical Component Score
  - Mental Component Score
Scoring Example: MHI-5

9b. Have you been a very nervous person?
9c. Have you felt so down in the dumps that nothing could cheer you up?
9d. Have you felt calm and peaceful?
9f. Have you felt downhearted and blue?
9h. Have you been a happy person?

Mental Health Scores in General US Population (n=2459)

SF-36 MH Score

SF-36 Scales Measure Physical and Mental Components of Health
**T-Scores for the SF-36**

- Mean = 50
- SD = 10

**PROMIS Domain Framework**

- Physical Health
  - Symptoms
  - Function

- Self-Reported Health
  - Physical Health
  - Social Health
- Relationships
  - Function

**PROMIS® Profile Short Forms (v1)**

- (29-43-57 items)

- Mental
  - Anxiety: 29
  - Depression: 28

- Physical
  - Fatigue: 96
  - Pain Intensity: 41
  - Sleep Disturbance: 27
  - Physical Function: 124

- Social
  - Satisfaction with Roles: 14

(+ pain intensity)
PROMIS Measures

- Adult Health Measures
  - More than 1,000 individual items (questions)
  - 51 distinct item banks or scales
  - 20 more coming soon
  - 20 languages
- Pediatric Health Measures
  - More than 150 items (questions)
  - 18 distinct banks or scales
  - 22 more on the way
  - 8 languages

Generic vs Disease-Specific

- Generic Measures
  - Can be used across populations
  - Generally better-tested
- Disease (Treatment, Population, Study) Specific Measures
  - Theoretically more sensitive to difference or changes

Q:

“Should I use the SF-36 or the SIP in my study of liver transplant patients?”
A:

- What is your research question?
- Who are the patients you are studying?
- What do you anticipate will happen?

Selection of a Health Status Measure

- Appropriateness: of the measure to the question or issue of concern
- Evidence in relevant populations of:
  - Reliability, Validity, Responsiveness
  - Practical considerations

Appropriateness:

- Of the measure to the question or issue of concern
- Correspondence between the content of the measure and goals of the study
  - Always examine the questionnaire itself
    - Do scales go into sufficient depth?
- Range in study sample vs the instrument
- Level of aggregation of scores
Evidence in relevant populations

- Reliability
- Validity
  - Face validity
  - Content
  - Construct
  - Responsiveness
- Pilot test?

Practical considerations:

- Mode of administration
- Time to administer
- Language
- Respondent burden
- Availability of supporting materials

Epic Systems Corporation MyChart

- Secure tethered website
- PRO implementation integrated in 2012 release
- Provides
  - MOS SF20, RAND36
  - PHQ2, PHQ9
  - PROMIS static adult, peds self-report/proxy short forms
- Complete integration with EpicCare EHR
- Clinician can specify timing and intervals
- Additional PRO can be built by trained programmers
- Reports developed within Clarity/data warehouse tools
Conclusion

- PRO important predictors and outcomes in clinical research studies
- Key concepts to measure include health related quality of life, symptoms, satisfaction, adherence
- Measurement relies on questions and scales as indicators of latent constructs of interest
- SF-36 as example
- Selection based on research questions, evidence of usefulness in your population, practical considerations

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REFERENCES

