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

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)
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 2003:5:522-531)
- "Any report coming directly from patients (i.e., study subjects) about a health condition and its treatment" (FDA Draft PRO Guidance)

Categories of Patient Outcomes

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

PROs

HRQoL
Utility
Symptoms

Satisfaction (Patient Experience)
Behavior
Utilization
The Six “D’s” of Outcomes Research

- Death
- Disease
- Disability
- Discomfort
- Dissatisfaction
- Dollars

World Health Organization definition of Health

"a state of complete physical, mental, and social well-being, and not merely the absence of disease and infirmity".

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

Wilson & Cleary, JAMA

How do you Measure HRQOL?
Do you feel sad?

Do you wake up in the middle of the night?

Do you feel worthless?

Do you wake up in the middle of the night?
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

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

Medical Outcomes Study Conceptual Framework

<table>
<thead>
<tr>
<th>Structure of Care</th>
<th>Process of Care</th>
<th>Outcome of Care</th>
</tr>
</thead>
<tbody>
<tr>
<td>System Characteristics</td>
<td>Provider Technical Style</td>
<td>Clinical End Points</td>
</tr>
<tr>
<td>Provider Characteristics</td>
<td>Providers' Interactional Style</td>
<td>Functional Status</td>
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<tr>
<td>Patient Characteristics</td>
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<td>General Well-Being</td>
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<td></td>
<td></td>
<td>Satisfaction with Care</td>
</tr>
</tbody>
</table>
Language

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

Questions

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

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

Content and administration

- Self-administered, also phone or interview
- Standardized (different) 3-6 point response scales
- Time frame last 4 weeks

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?

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

Scaling and scoring -

• Sum of item scores
• Recode and reverse
• Linear conversion to 0-100 scale
Item Scoring

- Items 9b, 9f, 9c - use precoded values
- Items 9d and 9h - require recoding prior to computation of the scale score

- All of the Time: 1 = 6
- Most of the Time: 2 = 5
- A Good Bit of the Time: 3 = 4
- Some of the Time: 4 = 3
- A Little of the Time: 5 = 2
- None of the Time: 6 = 1

Scale Scoring

- Recode and reverse items
- Compute sum of recoded items scores
- 5 items, 6 response categories: Lowest possible score 5, highest 30 (range 25)
- Transformation of raw summated score:
  \[(\text{Raw scale score} - \text{Lowest possible score}) / \text{possible score range} \times 100\]
  e.g. Mental health score of 21
  \[(21 - 5)/25 \times 100 = 64\]

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

[Graph showing distribution of SF-36 MH scores]
T-Scores for the SF-36

- Mean = 50
- SD = 10

PROMIS Measures – PROMIS 29
**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

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**How Do You Select What PRO to Use?**

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**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

"Measure twice, cut once."
Measure Once, Cut Twice

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

EpicCare PRO + Clinical Flowchart
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

