Overview

1. Importance of patient perspective
2. Definitions
3. Key concepts to measure as predictors and outcomes in clinical registries
4. Measurement of PROs
5. Class exercise: complete & score the SF-36
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)
Types of Information Captured in Data Used for CER

<table>
<thead>
<tr>
<th></th>
<th>Research Data</th>
<th>Clinical Care Data</th>
<th>Administrative Data</th>
</tr>
</thead>
<tbody>
<tr>
<td>Utilization</td>
<td>-</td>
<td>+</td>
<td>++</td>
</tr>
<tr>
<td>Clinical detail</td>
<td>++</td>
<td>+++</td>
<td>+</td>
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<tr>
<td>Patient perspective</td>
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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

- **Clinician-Reported**
  - For example, Global impressions, Observations & tests of function

- **Physiological**
  - For example, FEV1, HbA1c, Tumor size

- **Caregiver-Reported**
  - For example, Dependency, Functional status

- **Patient-Reported**
  - Global Impression, Functional status, Well-being, Symptoms, HRQL, Satisfaction with TX, Treatment adherence, Utility/preference-based measures

Source: Acquadro et al. Value in Health 2003;5:522-531

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  Satisfaction
Utility  Behavior
Symptoms  Utilization

The Six “D’s” of Outcomes Research

• Death
• Disease
• Disability
• Discomfort
• Dissatisfaction
• Dollars
World Health Organization definition of Health (1948)

• "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

- Pathophysiology
- Symptoms
- Physical/Mental Health
- QOL

Wilson & Cleary, JAMA

### Patient-based Screening Tools

- Goal is classification
- Performance criteria = sensitivity, specificity, positive predictive value, negative predictive value
- Mostly dichotomous
- May not be useful as outcome measures
  - E.g., have you ever attempted suicide
How do you Measure HRQOL?
Do you feel sad?

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

Do you feel sad?

Do you wake up in the middle of the night?

Do you feel worthless?
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>
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<tbody>
<tr>
<td>System Characteristics</td>
<td>Provider Technical Style</td>
<td>Clinical End Points</td>
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<tr>
<td>Provider Characteristics</td>
<td>Providers’ Interactional Style</td>
<td>Functional Status</td>
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<tr>
<td>Patient Characteristics</td>
<td></td>
<td>General Well-Being</td>
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<td></td>
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<td>Satisfaction with Care</td>
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</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:
  \[
  \frac{(\text{Raw scale score} - \text{Lowest possible score})}{\text{possible score range}} \times 100
  \]
e.g. Mental health score of 21
  \[
  \frac{(21 - 5)}{25} \times 100 = 64
  \]

Mental Health Scores in General US Population (n=2459)
T-Scores for the SF-36

- Mean = 50
- SD = 10
Generic vs Disease-Specific

- Generic Measures
  - Can be used across populations
  - Generally better-tested
- Disease (Treatment, Population, Study) Specific Measures
  - At least 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

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

- William Osler