Health-Related Quality of Life (HRQOL) - 2014

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Introduction to the Principles and Practice of Clinical Research
National Institutes of Health - Warren G. Magnuson Clinical Center Building 10
Lipsett Amphitheater, Bethesda, MD, December 1, 2013

Quality and Outcomes

"The best measure of quality is not how well or how frequently a medical service is given, but how closely the result approaches the fundamental objectives of prolonging life, relieving distress, restoring function, and preventing disability."

Lembcke, 1952


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Quality of Life (QoL)

- Community
- Education
- Family Life
- Friendships
  - Health
  - Housing
- Marriage
- Nation
- Neighborhood
- Self
- Standard of Living
- Work

Source: Campbell, 1981

World Health Organization Definition of Health

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

WHO, 1948

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Health is Measured in Terms of:

- Bodily structure & function
- Specific symptoms
- What you do/are able to do – functioning
- How you feel – subjective ill- and well-being (+ and -)
- What you say it is – personal evaluation

Continuum of Disease-specific and Generic Health Measures - Arthritis

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There is More to the Continuum

Predictive Validity:
HRQoL is One of the Best Predictors

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Summary of Content/Concepts for Widely-Used Generic Health Surveys

<table>
<thead>
<tr>
<th>Concepts and Characteristics</th>
<th>Utility</th>
</tr>
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<tbody>
<tr>
<td></td>
<td>SIP</td>
</tr>
<tr>
<td>Physical functioning</td>
<td></td>
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<tr>
<td>Social functioning</td>
<td></td>
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<tr>
<td>Role functioning</td>
<td></td>
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<tr>
<td>Psychological distress</td>
<td></td>
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<tr>
<td>Health perception (gen')</td>
<td></td>
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<tr>
<td>Pain</td>
<td></td>
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<tr>
<td>Vitality</td>
<td></td>
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<tr>
<td>Psychological well-being</td>
<td></td>
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<tr>
<td>Sleep</td>
<td></td>
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<tr>
<td>Cognitive functioning</td>
<td></td>
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<tr>
<td>Quality of life</td>
<td></td>
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<tr>
<td>Reported health transition</td>
<td></td>
</tr>
</tbody>
</table>

SIP = Sickness Impact Profile (1976)  
HIE = Health Insurance Experiment surveys (1979)  
NHP = Nottingham Health Profile (1980)  
QLI = Quality of Life Index (1981)  
COOP = Dartmouth Function Charts (1987)  
DUKE = Duke Health Profile (1990)  
MOS FWBP = MOS Functioning & Well-Being Profile (1992)  
MOS SF-36 = MOS 36-Item Short-Form Health Survey (1992)  
QWB = Quality of Well-Being Scale (1973)  
EUROQOL = European Quality of Life Index (1990)  
HUI = Health Utility Index (1998)  
SF-6D= SF-36 Utility Index (2002)

Source: Adapted from Ware, 1995

PROMIS Website  
www.nihpromis.org

PROMIS References


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Medical Outcomes Study
36-Item Health Survey (SF-36)

<table>
<thead>
<tr>
<th>Items</th>
<th>Scales</th>
<th>Summary Measures</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Activities of Daily Living (ADL)</td>
<td>Physical Functioning</td>
<td>Physical</td>
</tr>
<tr>
<td>2. Role-Physical (RP)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>3. Social Functioning (SF)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>4. Mental Health (MH)</td>
<td></td>
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</tr>
</tbody>
</table>

* Significant correlation with other summary measure.

Utility Index
(Brazier et al., 2002)
(Lam, Brazier, McGhee 2008)

Generic Health Profiles:
Before & After Medication

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Next Step: Integrate and Standardize Disease-specific and Generic Measures

Example: Osteoarthritis

Comparison of Content of Generic And Disease-Specific Measures

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Content Areas*
QOL Disease Impact Scale (QDIS)

1. Global ("activity" and "QOL")
2. Physical Functioning
3. Mobility
4. General Health
5. Fatigue
6. Cognitive
7. Emotional
8. Sleep
9. Role Functioning
10. Social Functioning

*Note: Bank of 49 items reduced to 25 items with attribution to specific condition

Anatomy of a Survey Item:
QDIS Changes Attribution from Health to Specific Disease

During the past 4 weeks, how often did your arthritis limit your ability to do your everyday activities?

- Very often
- Often
- Sometimes
- Rarely
- Never

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Better Measures Are Being Constructed

- Standardized Metrics
- Adaptive survey administrations
- Norm-based scoring
- Internet and mobile data collection

Improving the Physical Function "Ruler"

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We Need the Health Equivalent of a Two-Sided Tape Measure

and Public-Private Partnerships Meeting the Needs of Research and Business

Short-Form Surveys and Ceiling Effects

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Some Thermometers Focus on a Very Narrow Range

Cooking Thermometer

Temperature

Example: Cross-Calibrating Celsius and Fahrenheit

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Solution: Adaptive Survey Methods

Adaptive Assessments of Disease Impact
Match Questions to Each Patient’s Level

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First Question
Noisy Score Estimate (+/- 15)

Score estimate, 1st response = 62 +/- 15

Severe
Moderate
Mild

Higher is worse.
Mean = 50
SD = 10

Second Question: Standard Error
Reduced by One Third

Score estimate, 2 responses = 64 +/- 10

Severe
Moderate
Mild

Higher is worse.
Mean = 50
SD = 10

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Third Question: Standard Error
Cut in Half

Score estimate, 3 responses = 63
+/- 7

Severe
Moderate
Mild

Higher is worse.
Mean = 50
SD = 10

Fourth Question: Standard Error
Cut by Two Thirds

Score estimate, 4 responses = 62
+/- 5

Severe
Moderate
Mild

Higher is worse.
Mean = 50
SD = 10

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Practical Implications of CAT in Health Assessment

We have the potential to substantially advance the field of health status assessment by constructing and calibrating questionnaires based on item response theory (IRT) and administering them using computerized adaptive methods. This approach could substantially increase the accuracy and user-friendliness of health status reports. It would be useful to patients and those who treat them. It can be applied in a wide range of patients and in a wide range of health care settings. It can be used in the assessment of health status in patients with chronic conditions such as headache, diabetes, and heart disease. It can also be used in the assessment of health status in patients with acute conditions such as surgery, trauma, and stroke.

A Promising Solution in 1999: CAT-Based Health Assessment


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Performance of 5-item CAT Scores Confirmed in NIH-Sponsored Studies

- Mental Health: \( r = 0.98 \), \( N = 2,753 \)
- Headache Disability: \( r = 0.94 \), \( N = 1,016 \)
- Pediatric Disability: \( r = 0.96 \), \( N = 263 \)
- Chronic Kidney Disease: \( r = 0.95 \), \( N = 1,846 \)
- Diabetes Impact: \( r = 0.93 \), \( N = 100 \)
- Post Acute Rehabilitation: \( r = 0.95 \), \( N = 485 \)

What are the Advantages of Dynamic Assessments?

- More accurate risk screening
- Reliable enough to monitor individual outcomes
- Brevity of a short form – 90% reduction in respondent burden
- Elimination of “ceiling” & “floor” effects
- Can be administered using various data collection technologies
- Markedly reduced data collection costs
- Monitor data quality in real time

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Matching Methods to Applications

Population Surveys
- Single-Item
- Noisy Individual Classification

Group-Level Studies
- Multi-Item Scale

Patient-Level Assessments
- "Item Bank" (CAT Dynamic)
- Very Accurate Individual Classification

Most Functionally Impaired

Adaptive Survey Logic (ASLX®) Flow Chart

Survey Content And User Interface (UI) -> Generic Health Assessments -> QDIS Assessments (QOLIX®CCC) -> Follow-up Assessment Modules

- Estimate Score & CI from Screen:
  - NEG: ASLIX® Screen
  - POS: Supplemental Domains Assessment (CAT/Static)*

- Condition Present?
  - NO: Next Condition
  - YES: Supplemental Disease Impact Assessment (CAT/Static)*

Repeat Cycle for Each Generic Domain

Repeat Cycle for Each Chronic Condition

Abbreviations: QOLIX®DIS, Chronic condition checklist QOLIX®CCC, Diagnosis (Dx), Electronic data capture (EDC), User Interface (UI). Follow-up assessment modules vary across applications.

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HRQoL Validation Strategies

Clinical Causes
- Diagnosis
- Disease severity
- Clinical endpoint
- Treatment

HR-QOL

Gold Standard

Economic & Social Consequences
- Work productivity
- Costs of care
- Mortality
- Self-evaluated health

Other Measures & Methods

Interpreting HRQoL Scores

MOS Physical Component Summary (PCS)
(Mean = 50, SD = 10)

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Interpreting HRQoL Scores

- Content of questionnaire items
- Statistically significant change
- Important reduction in disease burden
- Reduction in subsequent expenditures
- Substantial increase in work productivity

MOS Physical Component Summary (PCS)
(Mean = 50, SD = 10)

Are Generic HRQoL Measures Responsive?

- X-ray, Disease Progression
- SF-36 agreed with primary endpoint (across Rx):
  - 219 of 253 RCTs
  - 86.6%

Clinical Markers
Specific Symptoms
Impact of Disease-specific Problems
Generic Health
In general, would you say your health is:
- Excellent
- Very good
- Good
- Fair
- Poor

Internet Sampling and Data Collection


Health Outcomes Research
Using Handhelds – Mobile Metrics

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Final Comment

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