Measurement and valuation of treatment effectiveness: clinical, HRQOL and preference

Parts 3 and 4

Measuring Patient Outcomes

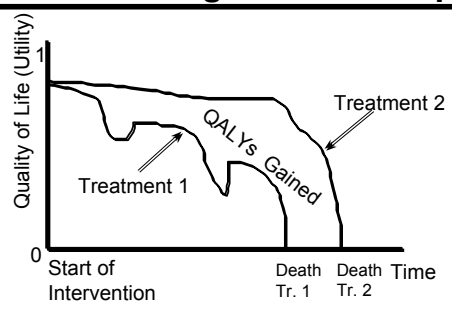
Sean D. Sullivan, PhD

What is Health-Related Quality of Life (HRQL)?

- Subjective - patient focused
- Measurement of individual life quality from a health/medical perspective
- Focus on maintaining a level of functioning that
  - allows pursuing of life goals
  - promotes a sense of well-being

Source: Kaplan RL 1989.

Quality-Adjusted Life Years Gained Using a New Therapy



Why Measure HRQL?

- "Objective" clinical measurement often unsatisfactory:
  - Of limited interest to patients
  - Correlate poorly with ability to function and sense of well-being
  - Patients with same clinical indices often have dramatically different emotional/functional responses

Changing Attitudes in Medicine

Early 20th Century

- Patient histories are unreliable
- Disease defined by collection of "objective" physical findings + lab values
- Rise of "objective" diagnostic tests (e.g., CT, MRI)

Late 20th Century

- Technology-driven ethical issues force patients back into discourse
- Civil rights movement
- Economic pressures: what type of endpoint is worth paying for?

Uses of HRQL

- Research
  - Patient-centered “outcomes” as valid endpoints
- Clinical care
  - Outcomes Vs. intermediate endpoints
- Medical Administration
  - Measure of quality of care
  - Predicting expenditure patterns
  - Used for reimbursement decisions

Considering Outcomes Appropriate for CEA

- Potential differences between groups
- Side effects of the intervention
- Outcomes of interest to consumers, patients, families, clinicians, society as a whole
- Capturing quality and quantity of life

Dimensions of HRQL

- Overall health perception
- Social functioning
  - Social relations
  - Usual social role
  - Intimacy/sexual function
  - Communication/speech
- Physical functioning
  - Mobility and self-care
  - Physical activity

Dimensions of HRQL (con't)

- Psychological functioning
  - Cognitive function
  - Emotional function
  - Mood/feelings
- Impairment
  - Sensory function/loss
  - Symptoms/impairments

Case Example

- A 42 year old woman is diagnosed with breast cancer, stage II. After a lumpectomy and lymph node dissection, she begins chemotherapy. During treatment she is tired, has frequent nausea/vomiting, and her hair falls out. Her thinking is also affected in a way that is difficult for her to describe. On the other hand she is hopeful and feels that she is "fighting" her cancer.

Non-preference Vs. Preference-based HRQL measures

- Non-Preference Based
  - subjects state which description most closely fits their current health
  - responses for each dimension not weighted
- Preference Based
  - subjects state which description most closely fits their current health
  - dimensions weighted according to their relative importance (e.g.. social > cognitive functioning)
  - Summary 0 to 1 score is tabulated

Types of HRQL Instruments

- Generic
  - Applicable to many conditions
- Disease-specific
  - Designed specifically for a particular condition

Taxonomy of HRQOL Measures

Generic

- Preference based
  - Summary scores
- Nonpreference based
  - Summary scores
  - No summary scores

Disease Specific

- Preference based
  - Summary scores
- Nonpreference based
  - Summary scores
  - No summary scores

Types of Generic Instruments

- Health profiles (Non-preference based)
  - SF-36
  - Sickness Impact Profile
  - Nottingham Health Questionnaire
- Utility measures (Preference based)
  - standard gamble
  - time-tradeoff
  - quality of well being

Generic Instruments

- **Advantages**
  - Allow comparisons across populations and diseases
  - Applicable in a wide number of settings
- **Disadvantages**
  - May not be sensitive to small changes in health status
  - Less likely to decipher why HRQL has changed

Disease Specific Instruments

- St. Georges Respiratory Questionnaire
- Minnesota Living With Heart Failure Questionnaire
- Seattle Angina Questionnaire
- Arthritis Self-Efficacy Questionnaire

Disease-Specific Instruments

- **Advantages**
  - Can be quite sensitive to changes in the condition being studied
- **Disadvantage**
  - Do not allow cross-population (disease) comparisons

Methods of Administration

- Interviewer-administered
  - advantages
  - disadvantages
- Self-administered
  - advantages
  - disadvantages

Methods for obtaining preference weights

- Direct measures
  - Utility approaches
    - Std. Gamble
    - Time tradeoff
  - Psychophysical approaches
    - Category scaling
    - Visual analogue scale
- Indirect measures
  - Multiattribute measures

Utilities vs. Values*

- Utilities = preferences under uncertainty
  - Std. Gamble
- Values = preferences under certainty
  - TTO
  - VAS

*See Torrance GW, Journal of Health Economics, 1986

Weighted health classification systems (Multiattribute Utility Measures)

- Affected individuals rate their health across several dimensions
- Weights for each domain derived from the community
  - Std. Gamble
  - Time Tradeoff

Multiattribute generic health classification systems

- Health Utilities Index
- EuroQol 5D (EQ-5D)
- Quality of Well Being Scale

Issues with Multiattribute Questionnaires

- Are the weights representative?
  - From what population were they derived?
- HRQOL survey instruments differ in the concepts and domains that they address
  - e.g.,
    - QWB has no questions related to psychological function
    - HUI has no questions related to social role and sexual function
  - Different instrument = different utility score!

Selected preference weights from Beaver Dam Health Outcomes study

	TTO Scores		QWB Scores	
	With condition	Without Condition	With Condition	Without Condition
Asthma	0.71	0.87	0.68	0.73
Arthritis	0.82	0.90	0.69	0.75
Angina	0.79	0.87	0.66	0.73
Stroke	0.90	0.86	0.68	0.73
Severe back pain	0.79	0.88	0.67	0.74
Migraine	0.82	0.86	0.70	0.73
Myocardial infarction	0.73	0.86	0.64	0.73
Diabetes (insulin)	0.63	0.87	0.66	0.73
Depression	0.70	0.87	0.65	0.73
Hiatal hernia	0.85	0.86	0.70	0.73

Desirable Properties of HRQL Instruments

- Validity
- Reliability
- Responsiveness
- Interpretability
- Feasibility

Validity

- Does the instrument measure what it is supposed to measure? 3 aspects:
  - (1) Construct Validity
    - Theoretical basis is sound
  - (2) Content Validity
    - Do the questions cover the areas of interest?
  - (3) Criterion Validity
    - Is there a “gold standard”?
    - Relation to other outcomes (physiologic scores, death, hospitalizations)

Reliability

- Internal Consistency
  - Degree to which instrument is free from random error
- Reproducibility
  - Gives the same result on repeated testing of same patients with constant health state
- High “signal to noise ratio”

Responsiveness

- Able to detect changes in health status that are meaningful to respondents
- No *ceiling* effect
- No *floor* effect

Interpretability

- Do the results tell whether the individual is doing well or not?
  - do they indicate *how* well or poorly?
- Does the magnitude of difference in scores have meaning?
  - e.g., trivial, small, moderate, or great

Feasibility

- Ease of administration (Respondent burden)
  - self-administered preferred
  - completed quickly
  - inexpensive per survey
- Well-accepted by respondents

Nonpreference based HRQOL

SF-36

Short Form-36 (SF-36)

- Designed as a general health status survey instrument
- First version a 20 item questionnaire for Medical Outcomes Study circa 1985
- Derived from health status questions originally written 30-40 yrs. ago
- Attempts to encompass all health concepts that are important to individuals

SF-36

- Multiple methods of administration:
  - self
  - telephone
  - interviewer
- Translated into many languages
  - probably most widely used HRQL instrument
- About 10 minutes for self-administered version

SF-36

- 8 subscales (domains):
  - physical functioning
  - role limitations d/t physical problems
  - social functioning
  - bodily pain
  - general mental health
  - role limitations d/t emotional problems
  - vitality
  - general health perceptions

SF-36

- Two composite scores
  - Physical
  - Mental

Preference Based HRQOL

Health Utilities Index

Health Utilities Index

- Generic multiattribute preference-based health state classification system
- Developed by Torrance and colleagues
  - McMaster University
- Three versions
  - HUI I (1982)
  - HUI II (1992)
  - HUI III (1995)

HUI Mark III

- 8 Attributes
  - Vision, hearing, speech, ambulation, dexterity, emotion, cognition, pain
  - 5-6 levels of response per attribute
- 15 Questions
- Preference weighting using two methods
  - VAS
  - Std Gamble
- Modes of administration
  - face to face, telephone, self-administered

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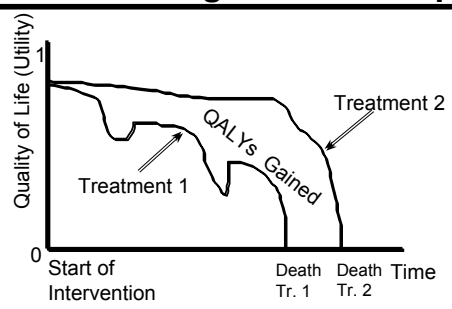

Whose Preferences Should be Used in CEA?

Subject	*Pro*	*Con*
Society	-Societal decisions the core of CEA -Less incentive to "game" responses	-Difficulty conceptualizing a health state -May not distinguish between "close" health states
Affected individual	-Knowledge of health state	-Incentive for "gaming" -Some persons unable to responsd (e.g., dementia)
Surrogate (e.g., physician)	-Some knowledge of health state -Gaming less likely	-May not represent society

Obtaining utilities from persons with disease vs. persons in the community

- Persons with the condition tend to adapt
  - Rate their QOL higher than an unaffected individual would rate the same condition
- Result:
  - Affected individual's weights will "undervalue" the benefit of an intervention
  - Community weights will result in more resources going to treatment/prevention than weights from those with the condition

The Ideal Utility Measurement System (US Panel on Cost-Effectiveness):

- Derived from a theory-based method using empirical data
- Weights available from community-based sample of the US population
- Low burden of administration
- Ability to furnish weights for health states as well as for illnesses and conditions

Case Example

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