### **Health-Related Quality of Life Measures**

(HS249T: Decision Analysis and Cost-Effectiveness Analysis)

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911 Broxton Avenue, 2<sup>nd</sup> Floor Conference Room May 11 & 16, 2011, 9-11:00 am

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### Health-Related Quality of Life is ...

### What you can do.

- Functioning
  - Self-care
  - Role
  - Social

### How you feel about your life.

- Well-being
  - Emotional well-being
  - Pain
  - Energy

# HRQOL is Multi-Dimensional



# HRQOL is Not

Quality of environment Type of housing Level of income Social Support



# Types of HRQOL Measures



- Profile: Generic vs. Targeted

# In general, how would you rate your health?

Poor Fair Good Very Good Excellent

## SF-36 Generic Profile Measure

- Physical functioning (10 items)
- Role limitations/physical (4 items)
- Role limitations/emotional (3 items)
- Social functioning (2 items)
- Emotional well-being (5 items)
- Energy/fatigue (4 items)
- Pain (2 items)
- General health perceptions (5 items)

The following items are about activities you might do during a typical day. Does your health now limit you in these activities? If so, how much?

- 1. Yes, limited a lot -----> 0
- 2. Yes, limited a little ----> 50
- 3. No, not limited at all -->100
- 1. Vigorous activities, such as running, lifting heavy objects, participating in strenuous sports
- 2. Moderate activities, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf
- 3. Lifting or carrying groceries
- 4. Climbing several flights of stairs
- 5. Climbing one flight of stairs
- 6. Bending, kneeling, or stooping
- 7. Walking more than a mile
- 8. Walking several blocks
- 9. Walking one block
- 10. Bathing or dressing yourself

## MHI-5

How much of the time during the past 4 weeks:

- Have you been very nervous?
- Have you felt so down in the dumps that nothing could cheer you up?
- Have you felt calm and peaceful?
- Have you felt down-hearted and depressed?
- Have you been happy?

# Targeted HRQOL Measures

- Designed to be relevant to particular group.
- Sensitive to small, but clinically-important changes.
- More familiar and actionable for clinicians.
- Enhance respondent cooperation.

# Kidney-Disease Targeted Items

During the last 30 days, to what extent were you bothered by cramps during dialysis?

Not at all bothered Somewhat bothered Moderately bothered Very much bothered Extremely bothered

# Scoring HRQOL Scales

- Average or sum all items in the same scale.
- Transform average or sum to
  - 0 (worse) to 100 (best) possible range
  - z-score (mean = 0, SD = 1)
  - T-score (mean = 50, SD = 10)

# Formula for Transforming Scores

$$Y = target mean + (target SD * Zx)$$

$$Z_{X} = \frac{(X - \overline{X})}{SD_{x}}$$

### SF-36 "Physical Health" Scale



### SF-36 "Mental Health" Scales



# SF-36 PCS and MCS Factor Scoring Coefficients

(PF\_Z \* .42402) + (RP\_Z \* .35119) + (BP\_Z \* .31754) + (GH\_Z \* .24954) + (EF\_Z \* .02877) + (SF\_Z \* -.00753) + (RE\_Z \* -.19206) + (EW\_Z \* -.22069) MCS z =

(PF\_Z \* -.22999) + (RP\_Z \* -.12329) + (BP\_Z \* -.09731) + (GH\_Z \* -.01571) + (EF\_Z \* .23534) + (SF\_Z \* .26876) + (RE Z \* .43407) + (EW Z \* .48581)

### **T-score** Transformation

 $PCS = (PCS_z*10) + 50$ MCS = (MCS\_z\*10) + 50

### Weights

Summary scores for SF-36 derived from uncorrelated (orthogonal) two factor (physical and mental health) solution, producing negative weights in scoring.

$$PCS-z = (PF-z^*.42) + (RP-z^*.35) + (BP-z^*.32) + (GH-z^*.25) + (EN-z^*.03) + (SF-z^*-.01) + (RE-z^*-.19) + (MH-z^*-.22)$$
$$MCS-z = (PF-z^*-.23) + (RP-z^*-.12) + (BP-z^*-.10) + (GH-z^*-.02) + (EN-z^*.24) + (SF-z^*.27) + (RE-z^*.43) + (MH-z^*.48)$$

Physical Functioning and Emotional Well-Being at Baseline for 54 Patients at UCLA-Center for East West Medicine



MS = multiple sclerois; ESRD = end-stage renal disease; GERD = gastroesophageal reflux disease.

# Effect Sizes for Changes in SF-36 Scores

Effect Size



Energy = Energy/Fatigue; EWB = Emotional Well-being; Gen H=General Health; MCS =Mental Component Summary; Pain = Bodily Pain; PCS = Physical Component Summary; PFI = Physical Functioning; Role-E = Role-Emotional; Role-P = Role-Physical; Social = Social Functioning

### Significant Improvement in all but 1 of SF-36 Scales (Change is in T-score metric)

|       | Change | t-test | prob.   |
|-------|--------|--------|---------|
| PF-10 | 1.7    | 2.38   | .0208   |
| RP-4  | 4.1    | 3.81   | .0004   |
| BP-2  | 3.6    | 2.59   | .0125   |
| GH-5  | 2.4    | 2.86   | .0061   |
| EN-4  | 5.1    | 4.33   | .0001   |
| SF-2  | 4.7    | 3.51   | .0009   |
| RE-3  | 1.5    | 0.96   | .3400 ← |
| EWB-5 | 4.3    | 3.20   | .0023   |
| PCS   | 2.8    | 3.23   | .0021   |
| MCS   | 3.9    | 2.82   | .0067   |

## Defining a Responder: Reliable Change Index (RCI)



 $SEM = SD_{hl} \times \sqrt{1 - r_{xx}}$ 

*Note:*  $SD_{bl}$  = standard deviation at baseline  $r_{xx}$  = reliability

### Amount of Change in Observed Score Needed for Significant Individual Change

| Scale | RCI  | Effect size | Cronbach' s<br>alpha |
|-------|------|-------------|----------------------|
| PF-10 | 8.4  | 0.67        | 0.94                 |
| RP-4  | 8.4  | 0.72        | 0.93                 |
| BP-2  | 10.4 | 1.01        | 0.87                 |
| GH-5  | 13.0 | 1.13        | 0.83                 |
| EN-4  | 12.8 | 1.33        | 0.77                 |
| SF-2  | 13.8 | 1.07        | 0.85                 |
| RE-3  | 9.7  | 0.71        | 0.94                 |
| EWB-5 | 13.4 | 1.26        | 0.79                 |
| PCS   | 7.1  | 0.62        | 0.94                 |
| MCS   | 9.7  | 0.73        | 0.93                 |

## 7-31% of People in Sample Improve Significantly

|       | % Improving | % Declining | Difference |
|-------|-------------|-------------|------------|
| PF-10 | 13%         | 2%          | + 11%      |
| RP-4  | 31%         | 2%          | + 29%      |
| BP-2  | 22%         | 7%          | + 15%      |
| GH-5  | 7%          | 0%          | + 7%       |
| EN-4  | 9%          | 2%          | + 7%       |
| SF-2  | 17%         | 4%          | + 13%      |
| RE-3  | 15%         | 15%         | 0%         |
| EWB-5 | 19%         | 4%          | + 15%      |
| PCS   | 24%         | 7%          | + 17%      |
| MCS   | 22%         | 11%         | + 11%      |

### Computerized Adaptive Testing (CAT)

- Select questions based on a person's response to previously administered questions.
- Iteratively estimate a person's standing on a domain (e.g., fatigue, depressive symptoms)
- Administer most informative items
- Desired level of precision can be obtained using the minimal possible number of questions.







•Are you able to get in and out of bed?
•Are you able to stand without losing your balance for 1 minute?
•Are you able to walk from one room to another?
•Are you able to walk a block on flat ground?
•Are you able to run or jog for two miles?
•Are you able to run five miles?



# Reliability and SEM

- For z-scores (mean = 0 and SD = 1):
  - Reliability = 1 SEM<sup>2</sup>
    - = 0.91 (when SEM = 0.30)
    - = 0.90 (when SEM = 0.32)
- With 0.90 reliability
  - 95% Confidence Interval
    - z-score: -0.62 → 0.62
- T-score = (z-score \* 10) + 50
  - T-score: 44 → 56

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# CAT assessments can achieve higher precision than fixed forms

measurement precision (standard error)



# PROMIS Banks (454 items)

### http://www.assessmentcenter.net/ac1/

- Physical Function [124]
- Emotional Distress [86]
  - Depression (28)
  - Anxiety (29)
  - Anger (29)
- Pain [80]
  - Behavior (39)
  - Impact (41)
- Fatigue [95]
- Satisfaction with Participation in Discretionary Social Activities (12)
- Satisfaction with Participation in Social Roles (14)
- Sleep Disturbance (27)
- Wake Disturbance (16)





$$\underline{M} = 50, \, \underline{SD} = 10$$
  
T = (z \* 10) + 50



### Ultimate Use of HRQOL Measures--Helping to Ensure Access to Cost-Effective Care

Cost ↓

Effectiveness ↑

### Is New Treatment (X) Better Than Standard Care (O)?



### Is Medicine Related to Worse HRQOL?

| Person | Medicati<br>Use H                 | on<br>RQOL (0-100)   |
|--------|-----------------------------------|--|
|        | 1                                 | Nodead   |
|        | 2                                 | Nodead   |
|        | 3 No 50                           | *******  |
|        | 4 No 75                           |  |
|        | 5 No 100                          |  |
|        | 6 Yes0                            | and the second s |
|        | 7 Yes25                           |  |
|        | 8 Yes50                           |  |
|        | 9 Yes75                           |  |
|        | 10 Yes100                         |  |
| Group  | n                                 | HRQOL  |
|        | No Medicine375<br>Yes Medicine550 |  |

## Direct Preference Measures

- Underlying attributes unknown
   Rating Scale
   Standard gamble
  - ≻Time tradeoff

# Rating Scale

### Overall, how would you rate your <u>current health</u>? (Circle One Number)







Downloaded from mdm.sagepub.com at UCLA on May 16, 2011

Alternative 1: Certainty of living in given health state y Alternative 2: Probability of living in full health (x) or immediate death (z) Time Trade-off approach:



Alternative 1: intermediate health state x, for time t, followed by death. Alternative 2: full health for time s where s < t, followed by death.

Time t is given and the individual is asked to state s. The preference score is then worked out as s/t.

×

### **Utility Assessments**

An important issue in medical decision making is how to measure people's preferences for health states in a way that will facilitate comparisons of health states. The most important measure of preference is the "utility" of the health state to the individual who will experience it, which is a value from 0 (representing death) to 1 (perfect health and well-being).

This page allows you to assess the utility for a health state using three techniques: rating scale, standard gamble, and time tradeoff.

Enter the health state that you'd like to assess the utility of: amputation of your left hand at the wrist

Select the assessment method to use:

Rating scale

Standard Gamble

Time Tradeoff

Let's do it!

## Indirect Preference Measures

- Attributes know and used to estimate societal preferences
  - ➢Quality of Well-Being (QWB) Scale
  - ≻EQ-5D
  - ≻HUI2 and HUI3
  - ≻SF-6D

### Quality of Well-Being (QWB) Scale

- Summarize HRQOL in QALYs
  - Mobility (MOB)
  - Physical activity (PAC)
  - Social activity (SAC)
  - Symptom/problem complexes (SPC)



• Well-Being Formula: w = 1 + MOB + PAC + SAC + SPC

### Quality of Well-Being Weighting Procedure

Each page in this booklet tells how an imaginary person is affected by a health problem on one day of his or her life. I want you to look at each health situation and rate it on a ladder with steps numbered from zero to ten.

The information on each page tells 1) the person's age group, 2) whether the person could drive or use public transportation, 3) how well the person could walk, 4) how well the person could perform the activities usual for his or her age, and 5) what symptom or problem was bothering the person.

Adult (18-65) Drove car or used public transportation without help (MOB) Walked without physical problems (PAC) Limited in amount or kind of work, school, or housework (SAC) Problem with being overweight or underweight (SYM)



### Your own health state today

By placing a tick in one box in each group below, please indicate which statement best describes your own health state today.

Do not tick more than one box in each group.

### Mobility

I have no problems in walking about I have some problems in walking about I am confined to bed

### Self-Care

I have no problems with self-care I have some problems washing and dressing myself I am unable to wash or dress myself

Usual Activities (eg. work, study, housework, family or leisure activities) I have no problems with performing my usual activities I have some problems with performing my usual activities I am unable to perform my usual activities

### Pain/Discomfort

I have no pain or discomfort I have moderate pain or discomfort I have extreme pain or discomfort

### Anxiety/Depression

I am not anxious or depressed I am moderately anxious or depressed I am extremely anxious or depressed

### Quality of Life for Individual Over Time



### http://www.ukmi.nhs.uk/Research/pharma\_res.asp



### Correlations Among Indirect Measures

|       | EQ-5D | HUI2 | HUI3 | QWB-SA | SF-6D |
|-------|-------|------|------|--------|-------|
| EQ-5D | 1.00  |      |      |        |       |
| HUI2  | 0.71  | 1.00 |      |        |       |
| HUI3  | 0.68  | 0.89 | 1.00 |        |       |
| QWB   | 0.64  | 0.66 | 0.66 | 1.00   |       |
| SF-6D | 0.70  | 0.71 | 0.69 | 0.65   | 1.00  |

Fryback, D. G. et al., (2007). US Norms for Six Generic Health-Related Qualityof-Life Indexes from the National Health Measurement Study. Medical Care, 45, 1162-1170.

### Change in Indirect Preference Measures Over Time

|        | Cataract (1 mon. – B) | Heart F (6 mons. – B) |
|--------|-----------------------|-----------------------|
| HUI3   | 0.05                  | 0.02                  |
| HUI2   | 0.03                  | 0.00                  |
| QWB-SA | 0.02                  | 0.03                  |
| EQ-5D  | 0.02                  | 0.00                  |
| SF-6D  | 0.00                  | 0.01                  |

Kaplan, R. M. et al. (2011). Five preference-based indexes in cataract and heart failure patients were not equally responsive to change. J Clinical Epidemiology, 64, 497-506.

