Health-Related Quality of Life Assessment as an Indicator of Quality of Care (HPM 216)

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Key Problems With US Health Care System (ACE)

- Access to care
 - ~ 50 million people without health insurance
- Costs of care
 - Expenditures ~ \$ 2.7 Trillion
- Effectiveness (quality) of care
 Not all care delivered is beneficial

Access to Care (In the last 12 months ...)

- When you phoned this provider's office to get an appointment for care you needed right away, how often did you get an appointment as soon as you needed?
- When you made an appointment for a check-up or routine care with this provider, how often did you get an appointment as soon as you needed?
- When you phoned this provider's office during regular office hours, how often did you get an answer to your medical questions that same day?
- When you phoned this provider's office after regular office hours, how often did you get an answer to your medical question as soon as you needed?
- How often did you see this provider within 15 minutes of your appointment time?

Cost-Effective Care

Cost ↓

Effectiveness ↑

How Do We Know If Care Is Effective?

- Effective care maximizes probability of desired health outcomes
- Outcomes are markers of whether or not care is effective

What Are Health Outcomes?

- Traditional clinical endpoints
 - Death, disease occurrence, other adverse events
 - Clinical measures/biological indicators
 - Blood pressure
 - Blood hemoglobin level
 - Symptoms (e.g. fever)
- Health-Related Quality of Life

Health-Related Quality of Life is:

How the person FEELs (well-being)

- Emotional well-being
- Pain
- Energy

What the person can DO (functioning)

- Self-care
- Role
- Social

HRQOL is Not

Quality of environment Type of housing Level of income Social Support



Patient-Reported Measures (PRMs)

- Patient-reported outcomes (PROs)
 - HRQOL
 - Satisfaction with care
- Mediators
 - Health behaviors (adherence)
- Patient evaluations of care

 Reports about care (e.g., communication)
- Needs assessment (preferences for care)

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)

Weights

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

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

SF-12 Scale

- Items by Scale
 - General health (1)
 - Physical functioning (3b, 3d)
 - Role-Physical (4b, 4c)
 - Role-Emotional (5b, 5c)
 - Bodily pain (8)
 - Emotional well-being (9d, 9f)
 - Energy/fatigue (9e)
 - Social functioning (10)

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.

Vision-targeted items (Paz et al.)

- Color vision
 - Match colors of clothes
- Distance vision
 - See street signs
- Near vision
 - See things close up during day
- Ocular symptoms

 Redness in eyes
- Psycho/social
 - Frustrated or upset
- Role performance
 - Limited in how long can do work or activities

Kidney-Disease Targeted Item

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 Profile 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)

HRQOL for HIV Compared to other Chronic Illnesses and General Population



Hays et al. (2000), <u>American Journal of Medicine</u>

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



Is Medicine Related to Worse HRQOL?

Person	Medicatio Use HF	tion IRQOL (0-100)	
	1	Nodead	
	2	Nodead	
	3 No 50		
	4 No 75		
	5 No 100	· · · · · · · · · · · · · · · · · · ·	
	6 Yes0 i		
	7 Yes25		
	8 Yes50		
	9 Yes75	A	
	10 Yes100		
Group	n	HRQOL	
	No Medicine375 Yes Medicine550		

http://www.ukmi.nhs.uk/Research/pharma_res.asp



Health state 424421

- Your health limits you <u>a lot</u> in moderate activities (such as moving a table, pushing a vacuum cleaner, bowling or playing golf)
- You are <u>limited in the kind of work or other</u> <u>activities</u> as a result of your physical health
- Your health limits your social activities (like visiting friends, relatives etc.) most of the time.
- You have pain that interferes with your normal work (both outside the home and housework) <u>moderately</u>
- You feel tense or downhearted and low <u>a little</u> of the time.
- You have a lot of energy <u>all of the time</u>

Health state 424421 (0.59)

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Variability

- Responses fall in each response category
- Distribution approximates bell-shaped "normal" curve (68.2%, 95.4%, and 99.6%)



Reliability

<u>Reliability</u> is the degree to which the same score is obtained for thing being measured (person, plant or whatever) when that thing hasn' t changed.

- Ratio of signal to noise

Reliability (0-1) >=0.70 (group comparisons) >=0.90 (individual assessment) >SEM = SD (1- reliability)^{1/2} ➢ 95% CI = true score +/- 1.96 x SEM \succ if z-score = 0, then CI: -.62 to +.62 so width of CI is 1.24 z-score units when reliability = 0.90z-scores (mean = 0 and SD = 1):

- Reliability = $1 SE^2$
- So reliability = 0.90 when SE = 0.32

T-scores (mean = 50 and SD = 10): T = 50 + (z * 10)

- Reliability = $1 (SE/10)^2$
- So reliability = 0.90 when SE = 3.2

I was grouchy [1st question]

- Never	[39]
- Rarely	[48]
- Sometimes	[56]
- Often	[64]

- Always [72]

Theta = 56.1 SE = 5.7 (rel. = 0.68)

I felt like I was ready to explode

[2nd question]

- Never
- Rarely
- Sometimes
- Often
- Always

Theta = 51.9 SE = 4.8 (rel. = 0.77)

- I felt angry [3rd question]
 - Never
 - Rarely
 - Sometimes
 - Often
 - Always

Theta = 50.5 SE = 3.9 (rel. = 0.85)

I felt angrier than I thought I should [4th question]

- Never
- Rarely
- Sometimes
- Often
- Always

Theta = 48.8 SE = 3.6 (rel. = 0.87)

- I felt annoyed [5th question]
 - Never
 - Rarely
 - Sometimes
 - Often
 - Always

Theta = 50.1 SE = 3.2 (rel. = 0.90)

I made myself angry about something just by thinking about it. [6th question]

- Never
- Rarely
- Sometimes
- Often
- Always

Theta = 50.2 SE = 2.8 (rel = 0.92)

Theta, SEM, and 95% CI

>56 and 6 (reliability = .68) W = 22 >52 and 5 (reliability = .77) W = 19 >50 and 4 (reliability = .85) W = 15 >49 and 4 (reliability = .87) W = 14 >50 and 3 (reliability = .90) W = 12 >50 and <3 (reliability = .92) W = 11

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 To be Statistically Significant



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

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



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

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

Effect Size

(Follow-up – Baseline)/ SD_{baseline}

Cohen's Rule of Thumb:

- \checkmark ES = 0.20 Small
- \checkmark ES = 0.50 Medium
- \checkmark ES = 0.80 Large

Effect Sizes for Changes in SF-36 Scores



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

Amount of Change Needed for Significant Individual Change



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

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%	

"Implementing patient-reported outcomes assessment in clinical practice: a review of the options and considerations"

Snyder, C.F., Aaronson, N. K., et al. <u>Quality</u> of Life Research, 21, 1305-1314.

- HRQOL has rarely been collected in a standardized fashion in routine clinical practice.
- Increased interest in using PROs for individual patient management.
- Research shows that use of PROs:
 - Improves patient-clinician communication
 - May improve outcomes

Reliability and Validity



by Experiment-Resources.com

Content Validity

- Does the measure adequately represent the domain?
 - Do items operationalize concept?
 - Do items cover all aspects of concept?
 - Does scale name represent item content?
- Face validity is extent to which measure "appears" to reflect what it is intended to
 - E.g., by expert judges or by patient focus groups

Evaluating Construct Validity

Scale	Age	Obesity	ESRD	Nursing Home Resident
Physical Functioning	Medium (-).	Small (-)	Large (-)	Large (-)
Depressive Symptoms	?	Small (+)	?	Small (+)

Cohen effect size rules of thumb (d = 0.2, 0.5, and 0.8): Small correlation = 0.100 Medium correlation = 0.243 Large correlation = 0.371 $\underline{r} = \underline{d} / [(\underline{d}^2 + 4)^{.5}] = \underline{0.8} / [(0.8^2 + 4)^{.5}] = 0.8 / [(0.64 + 4)^{.5}] = 0.8 / [(4.64)^{.5}] = 0.8 / 2.154 = \underline{0.371}$ (Beware r's of 0.10, 0.30 and 0.50 are often cited as small, medium, and large.) Responsiveness to Change and Minimally Important Difference (MID)

- HRQOL measures should be responsive to interventions that changes HRQOL
- Need external indicators of change (Anchors)
 - mean change in HRQOL scores among people who have changed ("minimal" change for MID).

Self-Report Indicator of Change

• Overall has there been any change in your asthma since the beginning of the study?

Much improved Moderately improved Minimally improved No change Minimally worse Moderately worse Much worse

Raw Score Change on PROMIS Physical Functioning (T-score) by Change on Anchor

	Lot Better	Little Better	Same	Little Worse	Lot Worse
	(n = 21)	(n = 35)	(n = 252)	(n = 113)	(n = 30)
Wave 3 – Wave 1	1.94 ^a	<u>1.63</u> ^{a,b}	0.27 ^b	<u>-1.68</u> c	-3.20 ^d
Wave 3 – Wave 2	3.26 ^a	<u>1.96</u> ^{a,b}	0.43 ^{b,c}	<u>-0.82</u> c	-3.16 ^d

Wave 3 is 12 months after wave 1. Wave 2 is 6 months after wave 1.

Better = got a lot better or a little better on anchor. Worse = got a lot worse or a little worse on anchor.

DIF (2-parameter model)



Person Fit

- Large negative Z_L values indicate misfit.
 - one person who responded to 14 of the PROMIS physical functioning items had a $Z_L = -3.13$
 - For 13 items the person could do the activity (including running 5 miles) without any difficulty.
 - But this person reported a little difficulty being out of bed for most of the day.

Final Thoughts



"Your doctor can only do so much. The rest is up to you. Stop getting older."