Patient-Centered Outcomes of Health Care

CTSI Training Module 3C
Comparative Effectiveness Research
January 23, 2014
8:30am – 12:30pm
CHS 17-187

Ron D.Hays, Ph.D.

Introduction to Patient-Reported Outcomes

8:30-9:30am

U.S. Health Care Issues

- **A**ccess to care
  - ~ 50 million people without health insurance
- **C**osts of care
  - Expenditures ~ $ 2.7 Trillion
- **E**ffectiveness (quality) of care

How Do We Know If Care Is Effective?

- Effective care maximizes probability of desired health outcomes
  - Health outcome measures indicate whether care is effective

Cost ↓

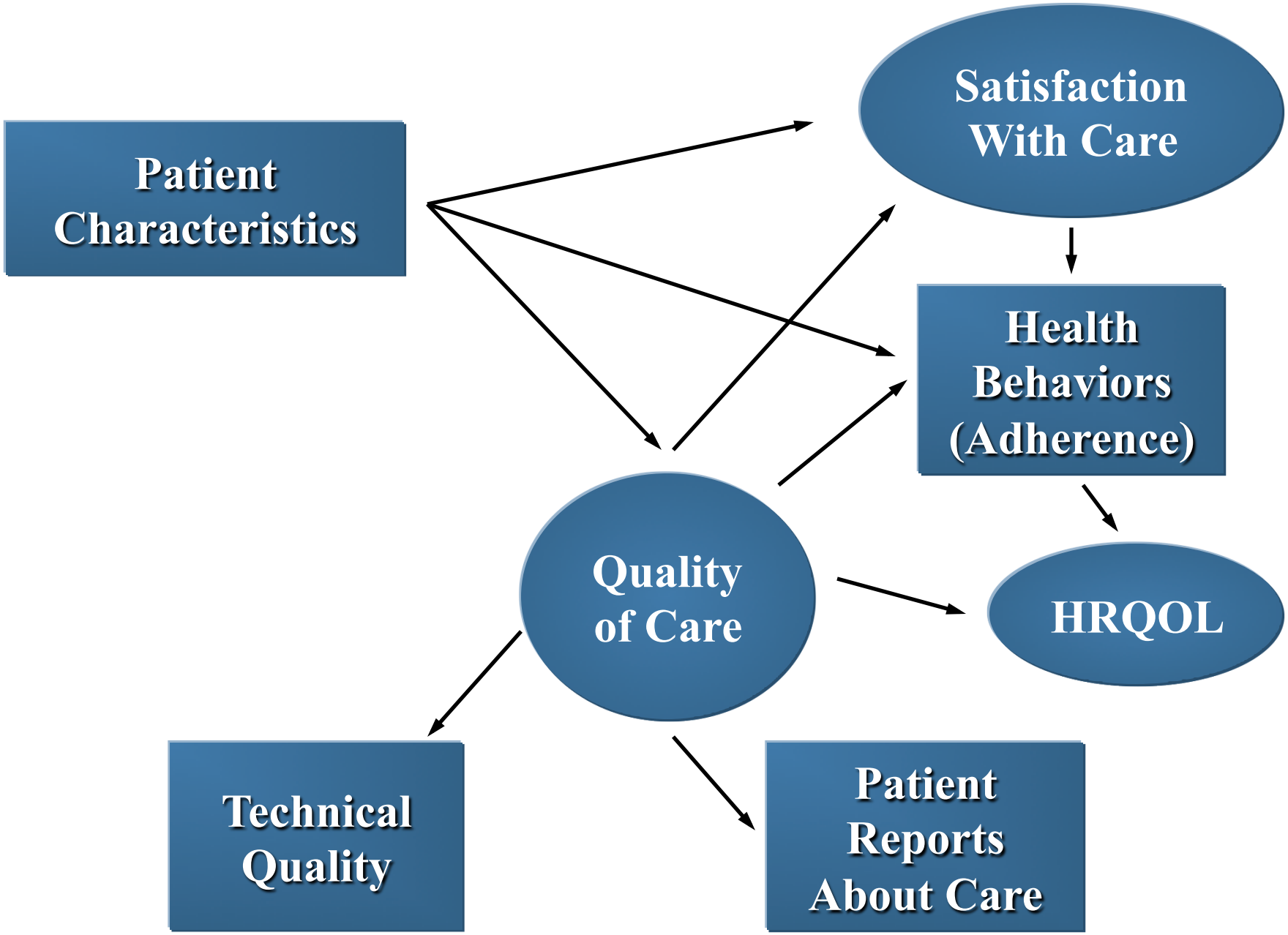
Effectiveness ↑

Health Outcomes Measures

- Traditional clinical endpoints
  - Survival
  - Clinical/biological indicators
    - Rheumatoid factor
    - Blood pressure
    - Hematocrit
- Patient-Reported Outcomes

Patient-Reported Measures (PRMs)

- Mediators
  - Health behaviors (adherence)
- Health Care Process
  - Reports about care (e.g., communication)
- Outcomes (PROs)
  - Patient satisfaction with care
  - Health-Related Quality of Life (HRQOL)


Satisfaction With Care
Patient Characteristics
Health Behaviors (Adherence)
Quality of Care
HRQOL
Technical Quality
Patient Reports About Care

Health-Related Quality of Life (HRQOL)

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

Types of HRQOL Measures

- Targeted vs. Generic

- Profile vs. Preference-based

Targeted Item

"I'm afraid that your irritable bowel syndrome has progressed. You now have furious and vindictive bowel syndrome."

During the last 4 weeks, how often were you angry about your irritable bowel syndrome?

None of the time
A little of the time
Some of the time
Most of the time
All of the time

Burden of Kidney Disease (Targeted Scale)

- My kidney disease interferes too much with my life.
- Too much of my time is spent dealing with my kidney disease.
- I feel frustrated with my kidney disease.
- I feel like a burden on my family.

Generic Item

In general, how would you rate your health?

Excellent
Very Good
Good
Fair
Poor

Does your health now limit you in walking more than a mile?

(If so, how much?)

Yes, limited a lot

Yes, limited a little

No, not limited at all

How much of the time during the past 4 weeks have you been happy?

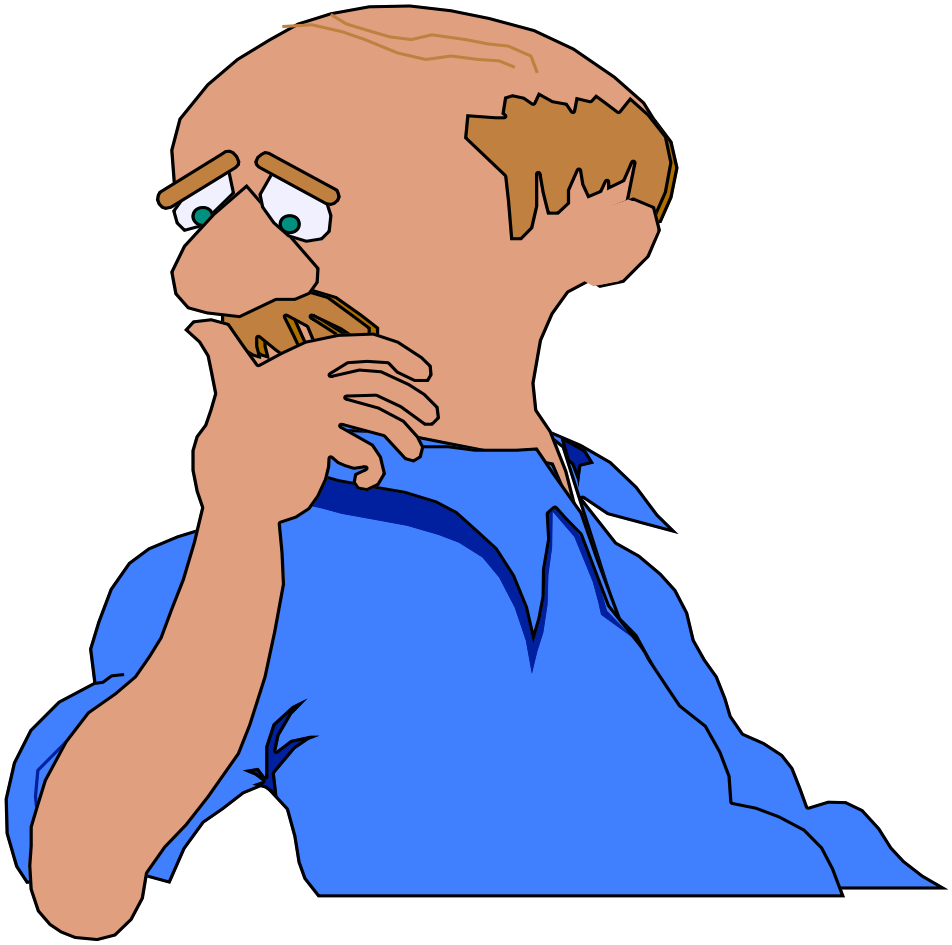
None of the time

A little of the time

Some of the time

Most of the time

All of the time



Generic Profile (SF-36)

- 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)

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)

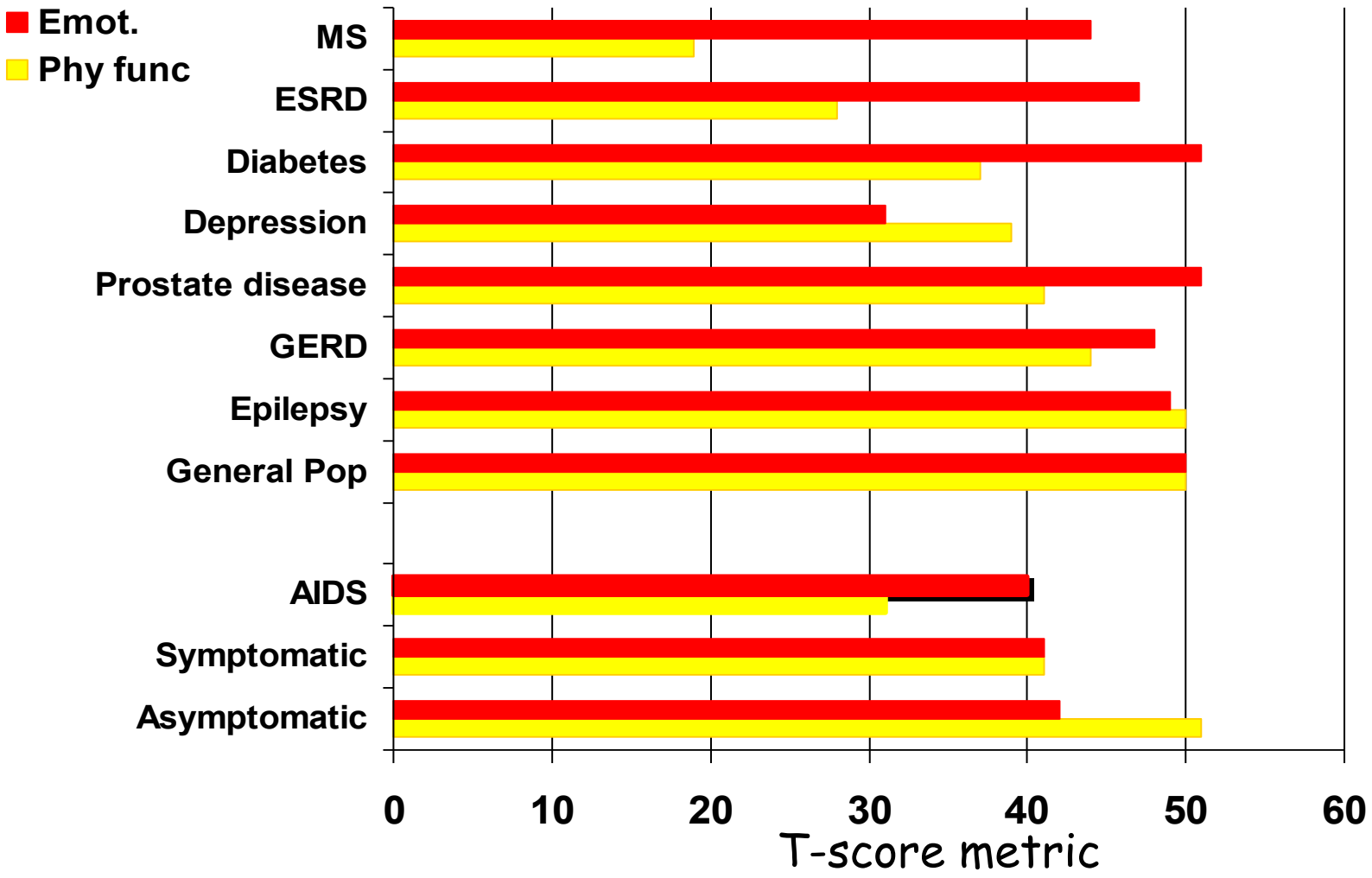
Linear Transformations

$$X = \frac{\text{(original score - minimum) *100}}{\text{(maximum - minimum)}}$$

$$Y = \text{target mean + (target SD * Zx)}$$

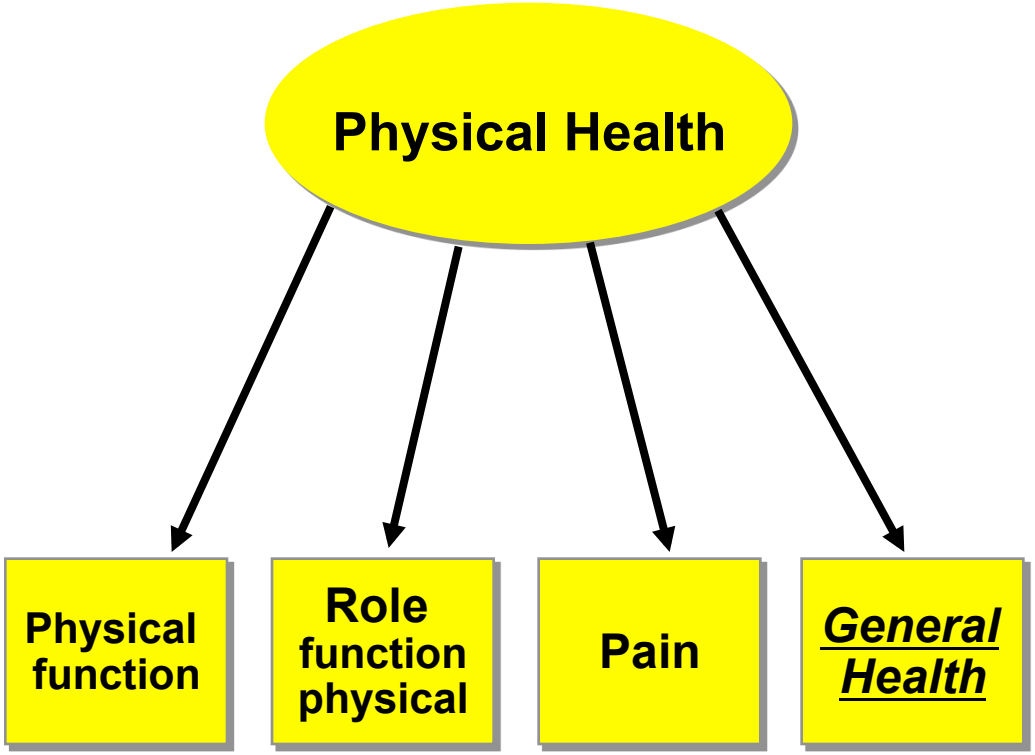
$$Z_X = \frac{(X - \bar{X})}{SD_x}$$

HRQOL in HIV Compared to other Chronic Illnesses and General Population

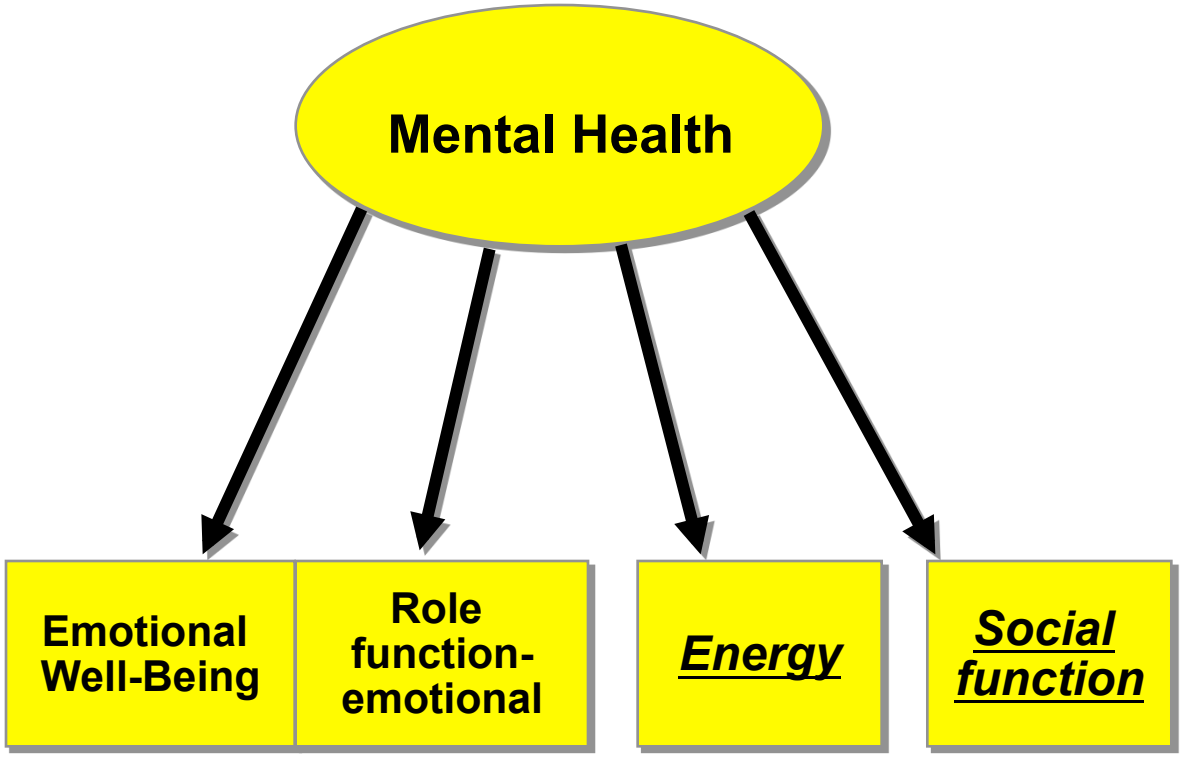


Hays et al. (2000), American Journal of Medicine

Physical Health



Mental Health



SF-36 PCS and MCS

PCS_z = (PF_Z * 0.42) + (RP_Z * 0.35) +
(BP_Z * 0.32) + (GH_Z * 0.25) +
(EF_Z * 0.03) + (SF_Z * -.01) +
(RE_Z * -.19) + (EW_Z * -.22)

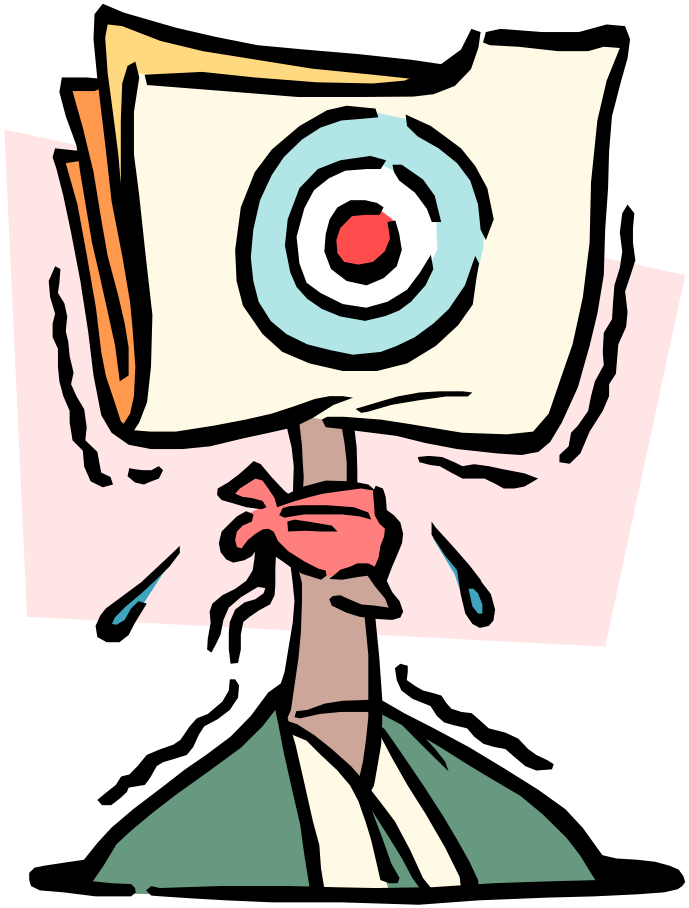
MCS_z = (PF_Z * -.23) + (RP_Z * -.12) +
(BP_Z * -.10) + (GH_Z * -.02) +
(EF_Z * 0.24) + (SF_Z * 0.27) +
(RE_Z * 0.43) + (EW_Z * 0.49)

PCS = (PCS_z*10) + 50

MCS = (MCS_z*10) + 50

536 Primary Care Patients Initiating Antidepressant Tx

- 3-month improvements in physical functioning, role—physical, pain, and general health perceptions ranging from 0.28 to 0.49 SDs.
  - Trivial < 0.20 SD
  - Small = 0.20 SD
  - Medium = 0.50 SD
  - Large = 0.80 SD
- Yet SF-36 PCS did <u>not</u> improve.



- *Simon et al. (<u>Med Care</u>, 1998)*

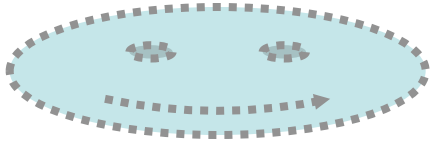
n = 194 with Multiple Sclerosis

- Lower scores than general population on
  - Emotional well-being (↓ 0.3 SD)
  - Role—emotional (↓ 0.7 SD)
  - Energy (↓1.0 SD)
  - Social functioning (↓1.0 SD)

- Yet SF-36 MCS was only 0.2 SD lower.

Nortvedt et al. (Med Care, 2000)

Farivar et al. alternative weights

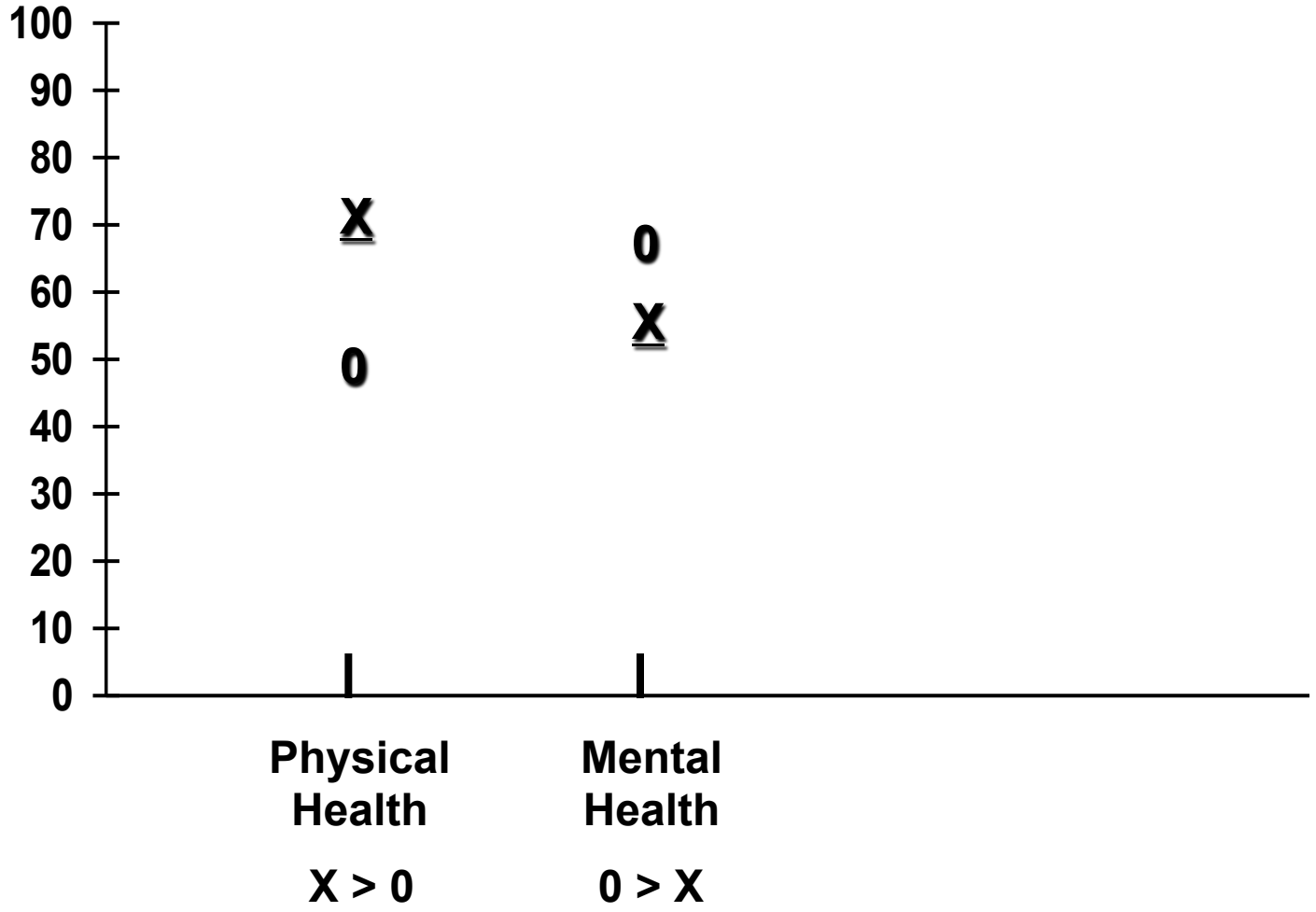


PCS_z = (PF_z * .20) + (RP_z * .31) + (BP_z * .23) +
(GH_z * .20) + (EF_z * .13) + (SF_z * .11) +
(RE_z * .03) + (EW_z * -.03)

MCS_z = (PF_z * -.02) + (RP_z * .03) + (BP_z * .04) +
(GH_z * .10) + (EF_z * .29) + (SF_z * .14) +
(RE_z * .20) + (EW_z * .35)

•**Farivar, S. S., Cunningham, W. E., & Hays, R. D. (2007). Correlated physical and mental health summary scores for the SF-36 and SF-12 health survey, V. 1. Health and Quality of Life Outcomes, 5: 54. [PMCID: PMC2065865]**

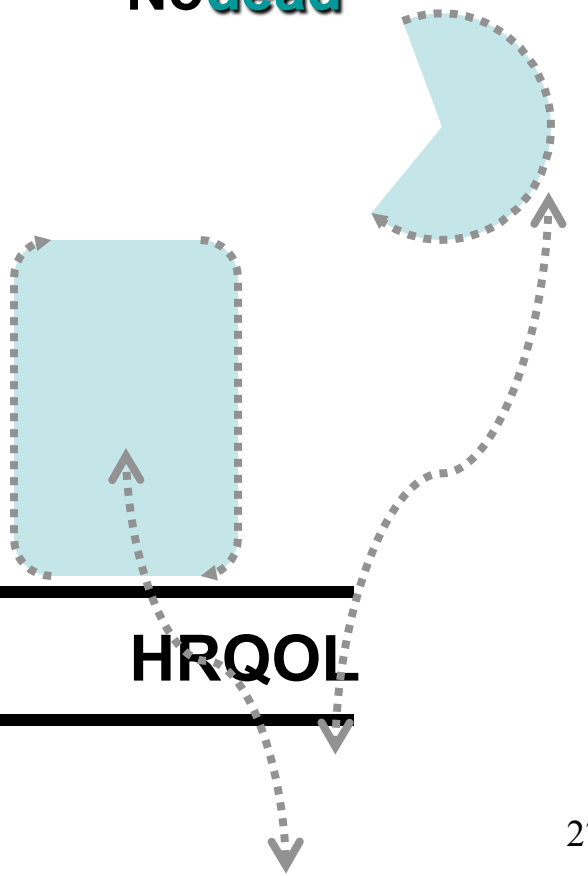
Is New Treatment (X) Better Than Standard Care (O)?



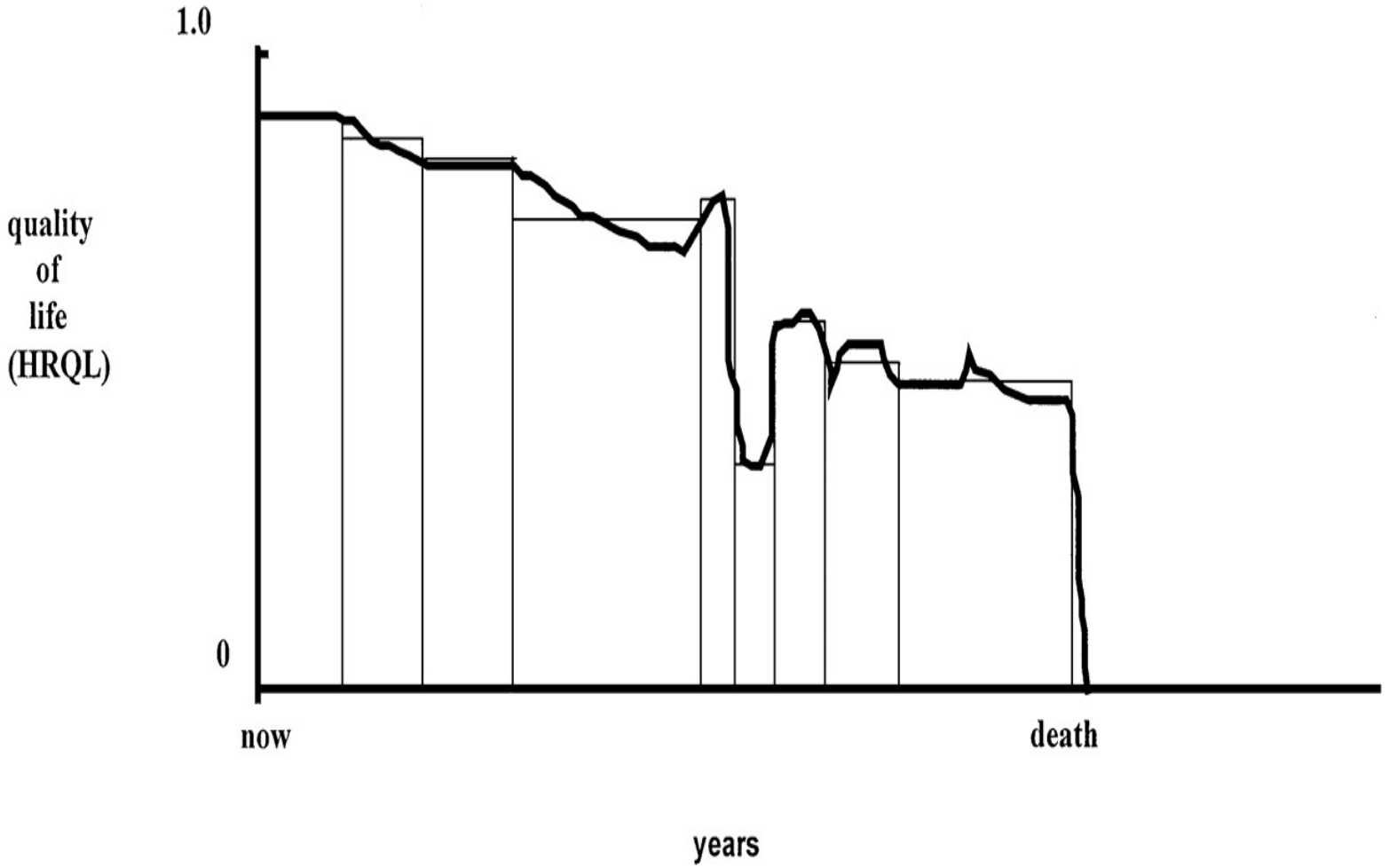
Is Medicine Related to Worse HRQOL?

Person	Medication Use	HRQOL (0-100)
	1	No dead
	2	No dead
	3 No	50
	4 No	75
	5 No	100
	6 Yes	0
	7 Yes	25
	8 Yes	50
	9 Yes	75
	10 Yes	100

Group	n	HRQOL
No Medicine	3	75
Yes Medicine	5	50



Quality of Life for Individual Over Time



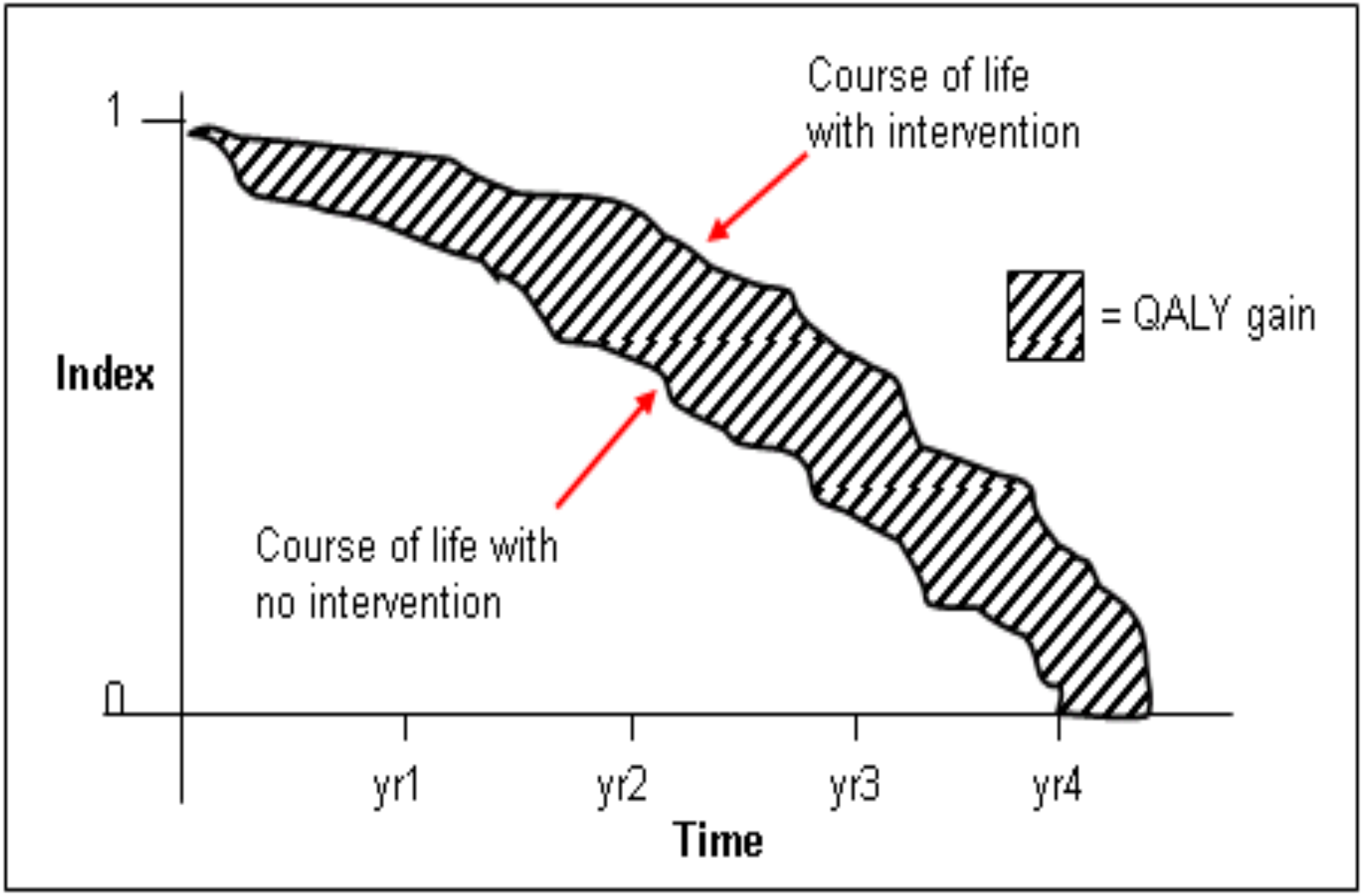
Ultimate Use of HRQOL Measures--Helping to Ensure Access to Cost-Effective Care

Cost ↓

— — — — — — — — —

Effectiveness (“Utility”) ↑

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



"QALYs: The Basics"

Milton Weinstein, George Torrance, Alistair McGuire

(Value in Health, 2009, vol. 12 Supplement 1)

- What is value?
  - Preference or desirability of health states
- How are QALYs used?
  - Societal resource allocation
  - Personal decisions such as decision about whether to have a treatment
  - Societal or program audit
    - Evaluate programs in terms of health of the population.

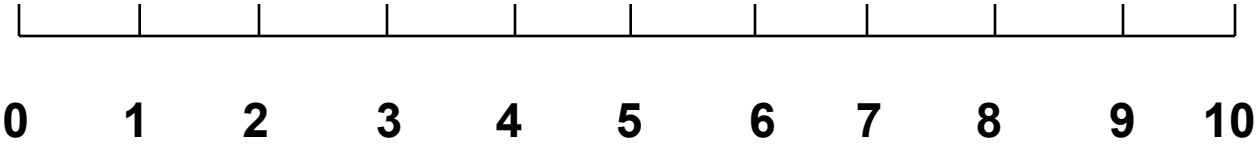
Direct Preference Measures

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

Rating Scale

Overall, how would you rate your <u>current health</u>?

(Circle One Number)



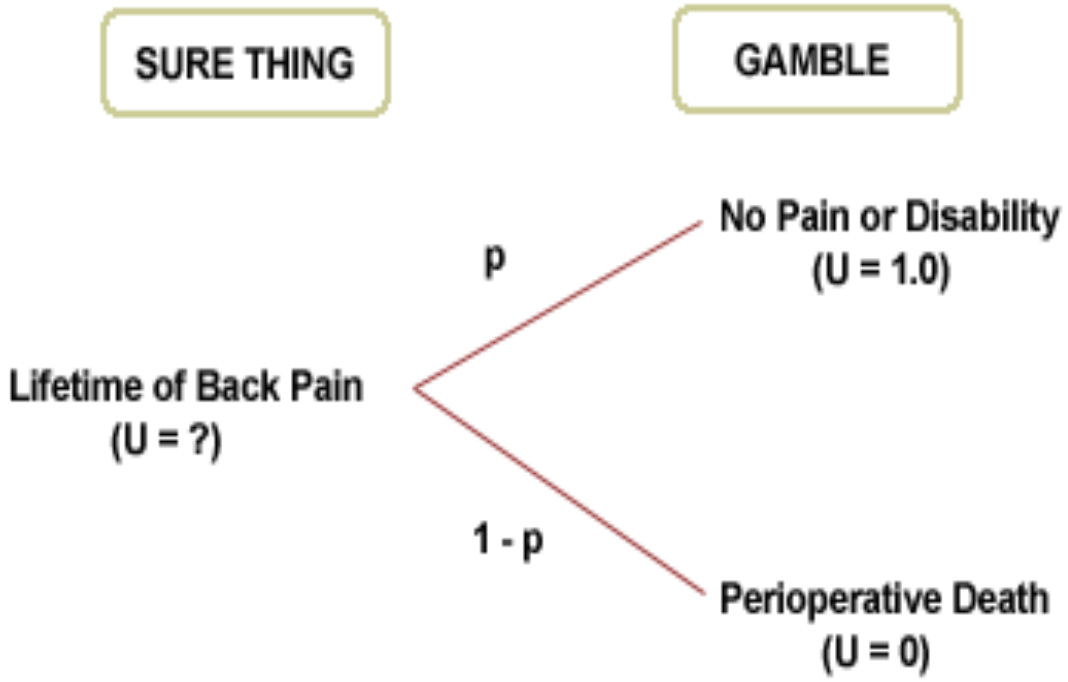
0 1 2 3 4 5 6 7 8 9 10

Worst possible health (as bad or worse than being dead)

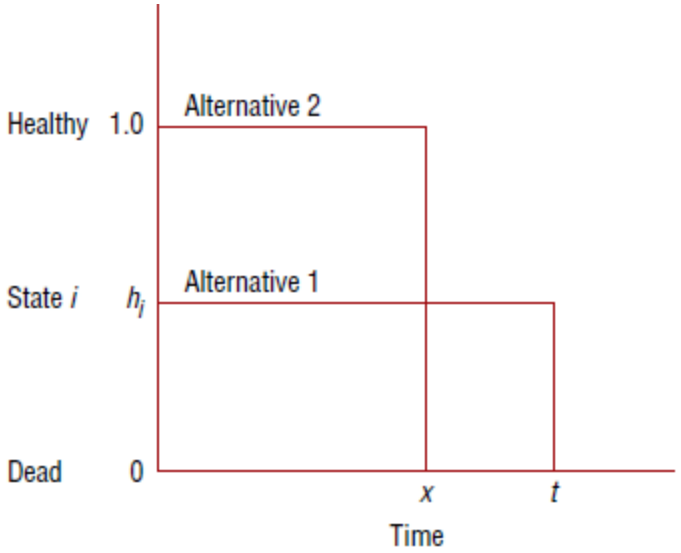
Half-way between worst and best

Best possible health

Standard Gamble

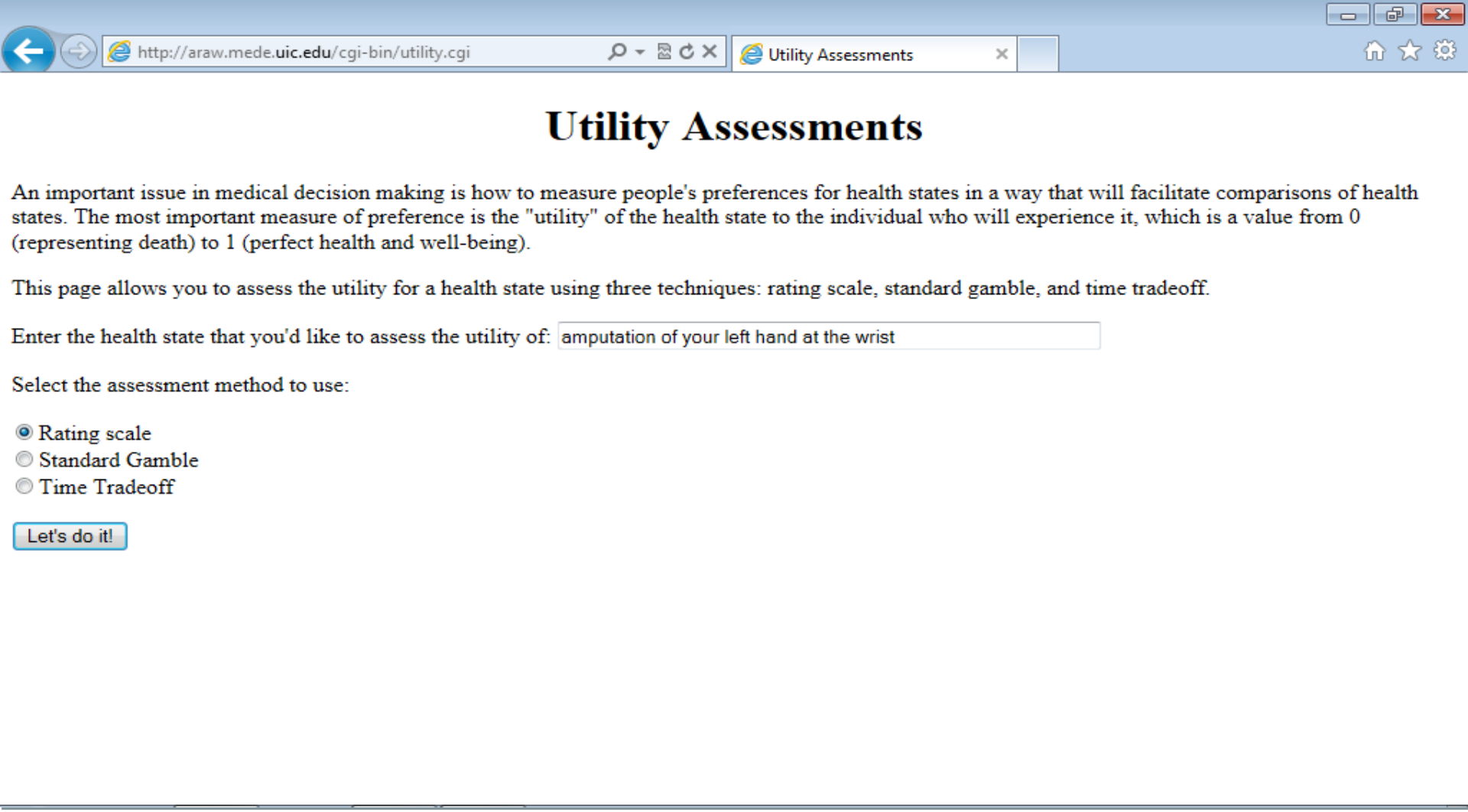


Time Tradeoff



Source: DiPiro JT, Talbert RL, Yee GC, Matzke GR, Wells BG, Posey LM: *Pharmacotherapy: A Pathophysiologic Approach, 8th Edition*: www.accesspharmacy.com

Alternative 1 is current health for time "t" (given), followed by death.
Alternative 2 is full health for time "x" (elicited), followed by death.
x/t = preference for current health



http://araw.mede.uic.edu/cgi-bin/utility.cgi

SG>TTO>RS

- $SG = TTO^{a}$
- $SG = RS^{b}$

Where a and b are less than 1

Indirect Preference Measures

- Attributes know
- Based on “societal preferences” a single score is assigned
  - 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)

EQ-5D (243 states, 3 levels each)

- Mobility
- Self-care
- Usual activities
- Pain/discomfort
- Anxiety/depression

http://www.euroqol.org/

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 ☐

SF-6D

Brazier et al. (1998, 2002)

— 6-dimensional classification (collapsed role scales, dropped general health)

— Uses 11 SF-36 items (8 SF-12 and 3 additional physical functioning items)

--- 18,000 possible states

-— 249 states rated by sample of 836 from UK general population

http://www.shef.ac.uk/scharr/sections/heds/mvh/sf-6d

Health state 424421 (0.59)

- Your health limits you a lot in moderate activities (such as moving a table, pushing a vacuum cleaner, bowling or playing golf)
- You are limited in the kind of work or other activities 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) moderately
- You feel tense or downhearted and low a little of the time.
- You have a lot of energy all of the time

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 Quality-of-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

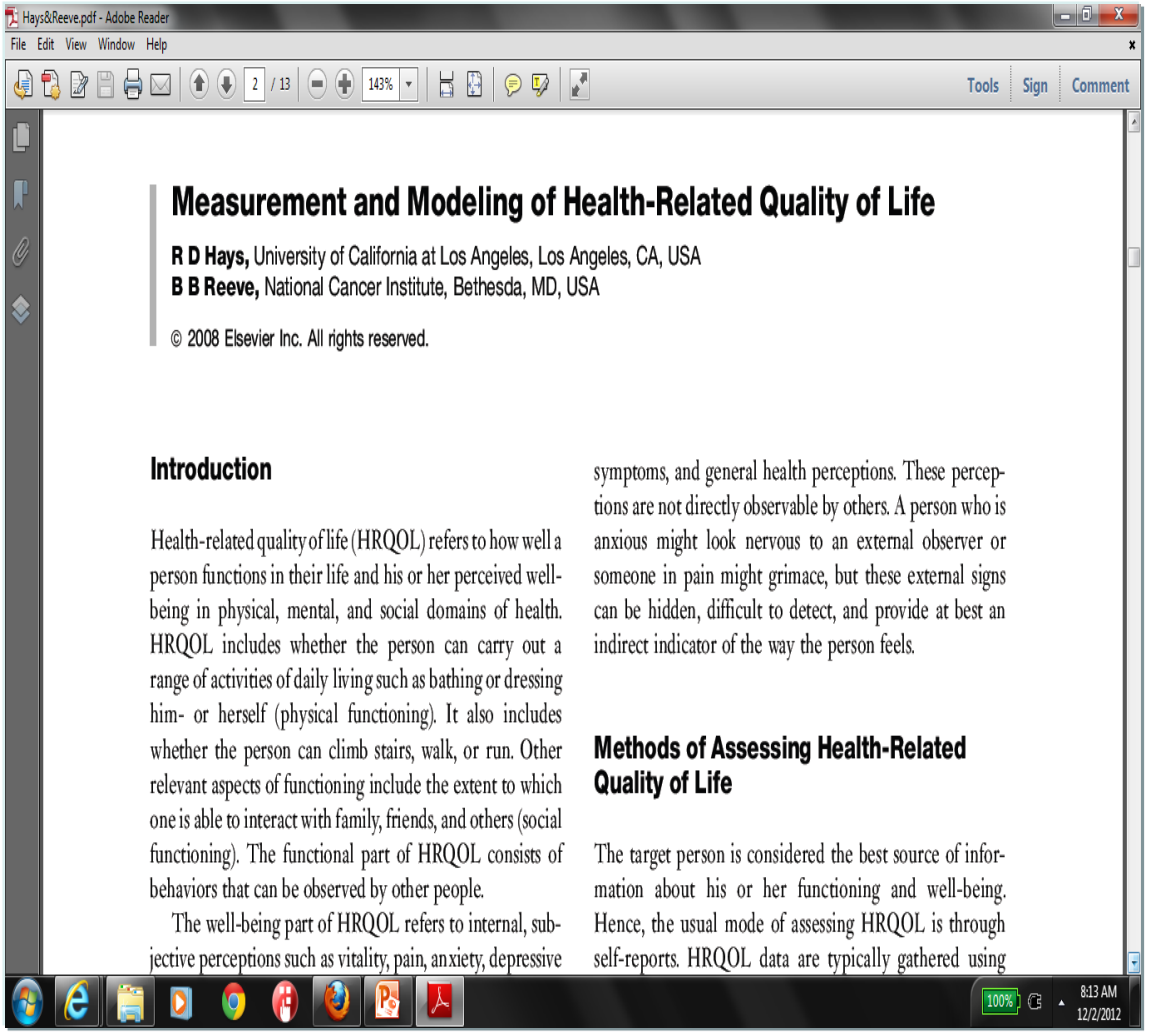
Kaplan, R. M. et al. (2011). Five preference-based indexes in cataract and heart failure patients were not equally responsive to change. J Clinical Epidemiology, 64, 497-506.

ICC for change was 0.16 for cataract and 0.07 for heart failure.
Feeny, D. et al. (2011). Agreement about identifying patients who change over time: Cautionary results in cataract and heart failure patients. Medical Decision Making, 32 (2), 273-286.

Break #1

Development and Evaluation of Patient-reported Outcomes

9:45-10:45am



End goal is measure that is "Psychometrically Sound"

- Same people get same scores
- Different people get different scores and differ in the way you expect
- Measure works the same way for different groups (age, gender, race/ethnicity)
- Measure is practical

Measurement Steps

- Review literature
- Focus groups
  - Define constructs and draft items
- Pretest (cognitive interviews)
  - Revise items
- Field test
  - Analyze and finalize items

Focus Groups

- Discuss feelings, attitudes, perceptions
- Learn
  - Vocabulary and thinking patterns
- Conversational meeting
  - Moderator and 6-12 people
  - Questions posed
  - Group synergy
  - Economical

Pretesting

"Cut and try, see how it looks and sounds, see how people react to it, and then cut again, and try again" *Converse & Presser (1986, p. 78)*

Identify problems with

- Comprehension of items (stem/response options)
- Retrieval of information
- Skip patterns
- Response burden

Cognitive Interviews

- “Think aloud”
- Intermittent probes
- Retrospective recall

Flesch-Kincaid Grade Level

FK GL = **0.39** * (n of words/n of sentences) + **11.8** * (n of syllables/n of words) – 15.59

- Driven by sentence length and syllables per word
- U.S. school grade level (e.g., 8.0 implies that 8th grader can understand the document).
- Possible minimum = -3.4
  - Green eggs and ham averages 5.7 words per sentence and 1 syllable per word
  - (FK GL = -1.3)

Listed below are a few statements about your relationships with others. How much is each statement TRUE or FALSE for you

	Definitely True	Mostly True	Don't Know	Mostly False	Definitely False
1. I am always courteous even to people who are disagreeable.	1	2	3	4	5
2. There have been occasions when I took advantage of someone.	1	2	3	4	5
3. I sometimes try to get even rather than forgive and forget.	1	2	3	4	5
4. I sometimes feel resentful when I don't get my way.	1	2	3	4	5
5. No matter who I'm talking to, I'm always a good listener.	1	2	3	4	5

Scoring Multi-Item 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)

Listed below are a few statements about your relationships with others. How much is each statement TRUE or FALSE for you

	Definitely True	Mostly True	Don't Know	Mostly False	Definitely False
1. I am always courteous even to people who are disagreeable.	100	75	50	25	0
2. There have been occasions when I took advantage of someone.	0	25	50	75	100
3. I sometimes try to get even rather than forgive and forget.	0	25	50	75	100
4. I sometimes feel resentful when I don't get my way.	0	25	50	75	100
5. No matter who I'm talking to, I'm always a good listener.	100	75	50	25	0

Create T-score

z-score = (score – 36)/31

T-score = (10 * z-score) + 50

z-score = (100- 36)/31 = 2.06

T-score = 71

Reliability

- Extent to which measure yields similar result when the thing being measured hasn't changed
- Ranges from 0-1

Reliability and Intraclass Correlation

Model	Reliability	Intraclass Correlation
One-way	$\frac{MS_{BMS} - MS_{WMS}}{MS_{BMS}}$	$\frac{MS_{BMS} - MS_{WMS}}{MS_{BMS} + (k-1)MS_{WMS}}$
Two-way fixed	$\frac{MS_{BMS} - MS_{EMS}}{MS_{BMS}}$	$\frac{MS_{BMS} - MS_{EMS}}{MS_{BMS} + (k-1)MS_{EMS}}$
Two-way random	$\frac{N(MS_{BMS} - MS_{EMS})}{NMS_{BMS} + MS_{JMS} - MS_{EMS}}$	$\frac{MS_{BMS} - MS_{EMS}}{MS_{BMS} + (k-1)MS_{EMS} + k(MS_{JMS} - MS_{EMS})/N}$

BMS = Between Ratee Mean Square **N = n of ratees**
WMS = Within Mean Square **k = n of items or raters**
JMS = Item or Rater Mean Square
EMS = Ratee x Item (Rater) Mean Square

01 34
02 45
03 33
04 21
05 54
06 22

Two-Way Fixed Effects (Cronbach's Alpha)

Source	df	SS	MS
Respondents (BMS)	5	15.67	3.13
Items (JMS)	1	0.00	0.00
Resp. x Items (EMS)	5	2.00	0.40
Total	11	17.67	

$$\text{Alpha} = \frac{3.13 - 0.40}{3.13} = \frac{2.93}{3.13} = \boxed{0.87}$$

ICC = 0.77

Reliability Minimum Standards

- 0.70 or above (for group comparisons)
- 0.90 or higher (for individual assessment)
  - SEM = SD (1- reliability)$^{1/2}$
  - 95% CI = true score +/- 1.96 x SEM
    - if true z-score = 0, then CI: -.62 to +.62
      - Width of CI is 1.24 z-score units

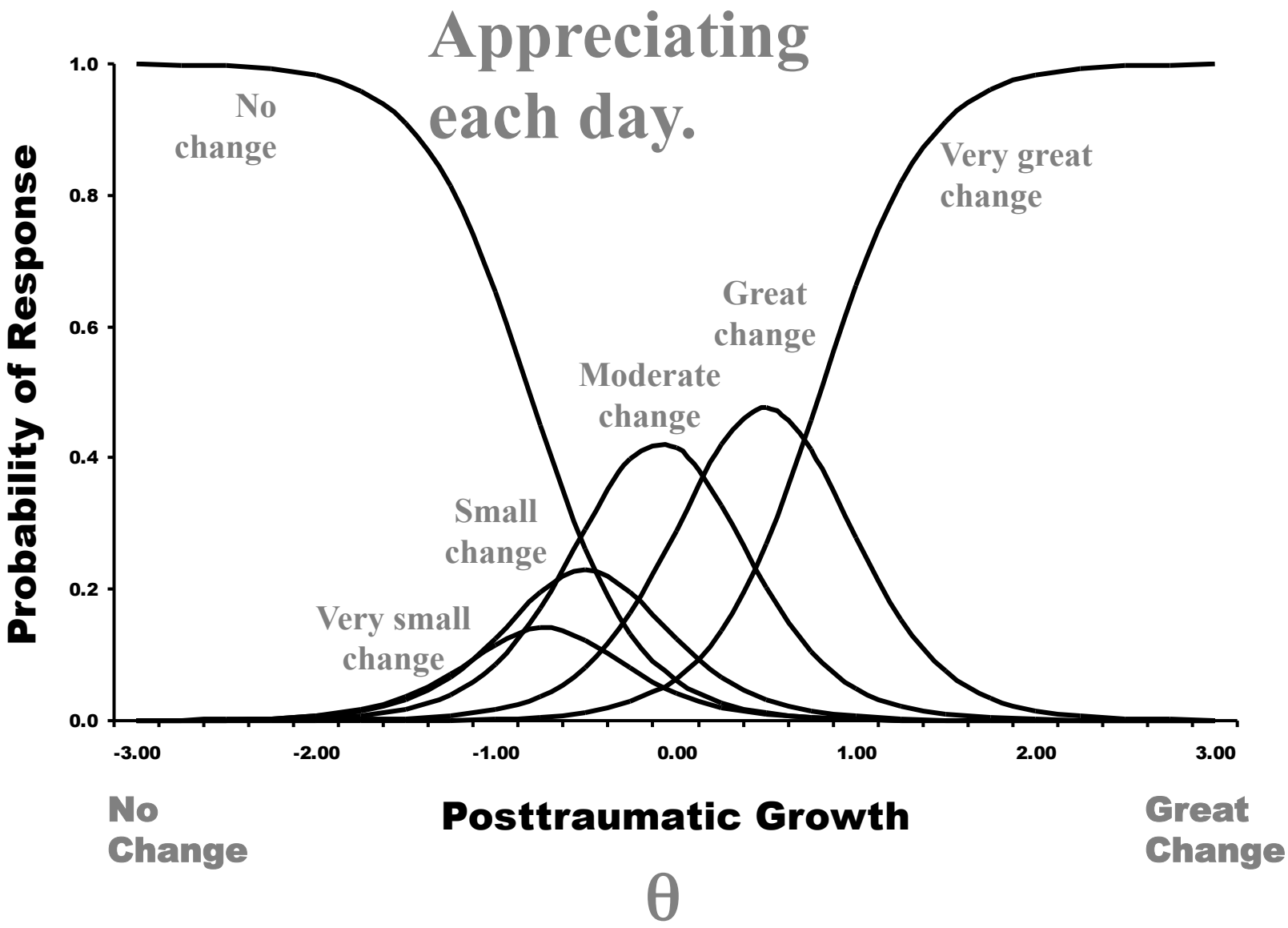
Range of reliability estimates

0.80-0.90 for blood pressure

0.70-0.90 for multi-item self-report scales

Hahn, E. A., Cella, D., et al. (2007). Precision of health-related quality-of-life data compared with other clinical measures. Mayo Clin Proceedings, 82 (10), 1244-1254.

Category Response Curves



Item-scale correlation matrix

	Depress	Anxiety	Anger
Item #1	0.80*	0.20	0.20
Item #2	0.80*	0.20	0.20
Item #3	0.80*	0.20	0.20
Item #4	0.20	0.80*	0.20
Item #5	0.20	0.80*	0.20
Item #6	0.20	0.80*	0.20
Item #7	0.20	0.20	0.80*
Item #8	0.20	0.20	0.80*
Item #9	0.20	0.20	0.80*

*Item-scale correlation, corrected for overlap.

Item-scale correlation matrix

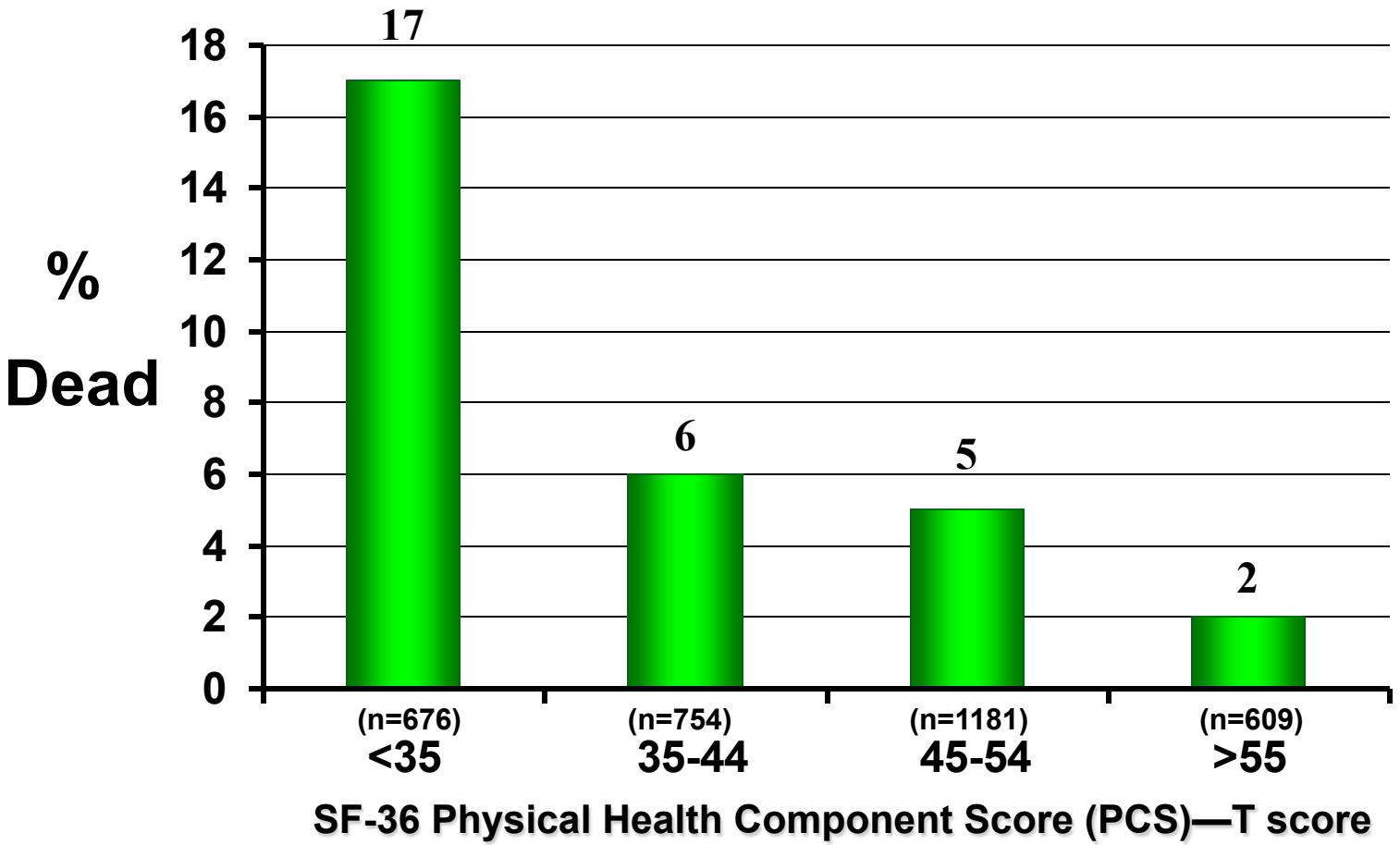
	Depress	Anxiety	Anger
Item #1	0.50*	0.50	0.50
Item #2	0.50*	0.50	0.50
Item #3	0.50*	0.50	0.50
Item #4	0.50	0.50*	0.50
Item #5	0.50	0.50*	0.50
Item #6	0.50	0.50*	0.50
Item #7	0.50	0.50	0.50*
Item #8	0.50	0.50	0.50*
Item #9	0.50	0.50	0.50*

*Item-scale correlation, corrected for overlap.

Validity

- Content validity
  - Patients and/or experts judge the items to be representing the intended concept adequately
- Construct validity
  - Extent to which associations with other variables are consistent with prior hypotheses

Self-Reports of Physical Health Predict Five-Year Mortality



Ware et al. (1994). SF-36 Physical and Mental Health Summary Scales: A User's Manual.

Mortality Prediction with a Single General Self-Rated Health Question

DeSalvo, K. B., Bloser, N., Reynolds, K., He, J., & Muntner, P. (2005). Mortality prediction with a single general self-rated health question: A meta-analysis. JGIM, 20, 267-275.

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
$r = d / [(d^2 + 4)^{.5}] = 0.8 / [(0.8^2 + 4)^{.5}] = 0.8 / [(0.64 + 4)^{.5}] = 0.8 / [(4.64)^{.5}] = 0.8 / 2.154 = 0.371$

Beware: r's of 0.10, 0.30 and 0.50 are often cited as small, medium, and large.

Responsiveness to Change

- Valid measures should be responsive to interventions that change the thing being measured.

- Compare change on measure to change indicated on external indicator of change ("anchor")

Listed below are a few statements about your relationships with others. How much is each statement TRUE or FALSE for you?

- I am always courteous even to people who are disagreeable.
- There have been occasions when I took advantage of someone.
- I sometimes try to get even rather than forgive and forget.
- I sometimes feel resentful when I don't get my way.
- No matter who I'm talking to, I'm always a good listener.

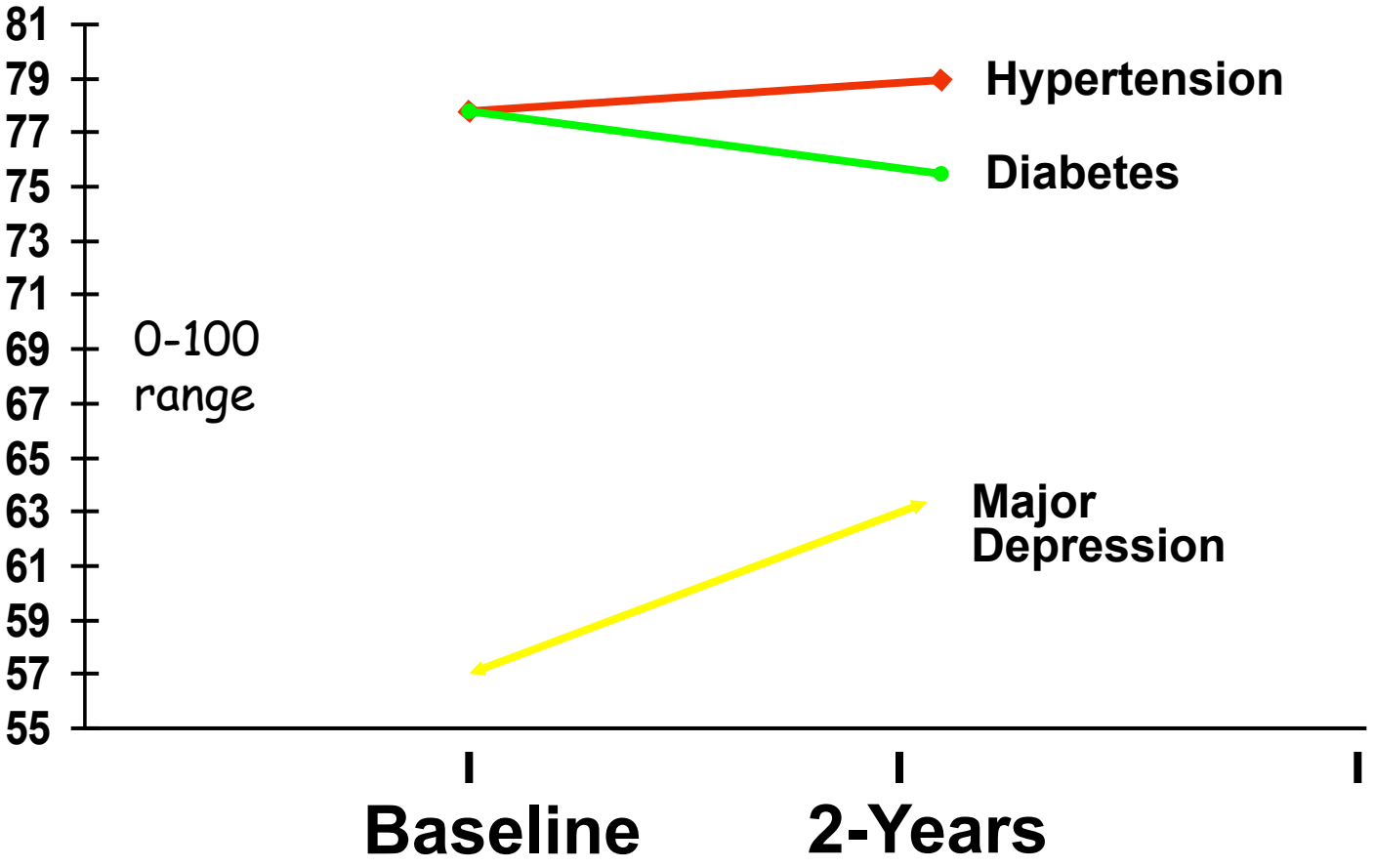
Definitely True/Mostly True/Don't Know/Mostly False/Definitely False

Break #2

Use of Patient-Reported Outcome Measures in Research

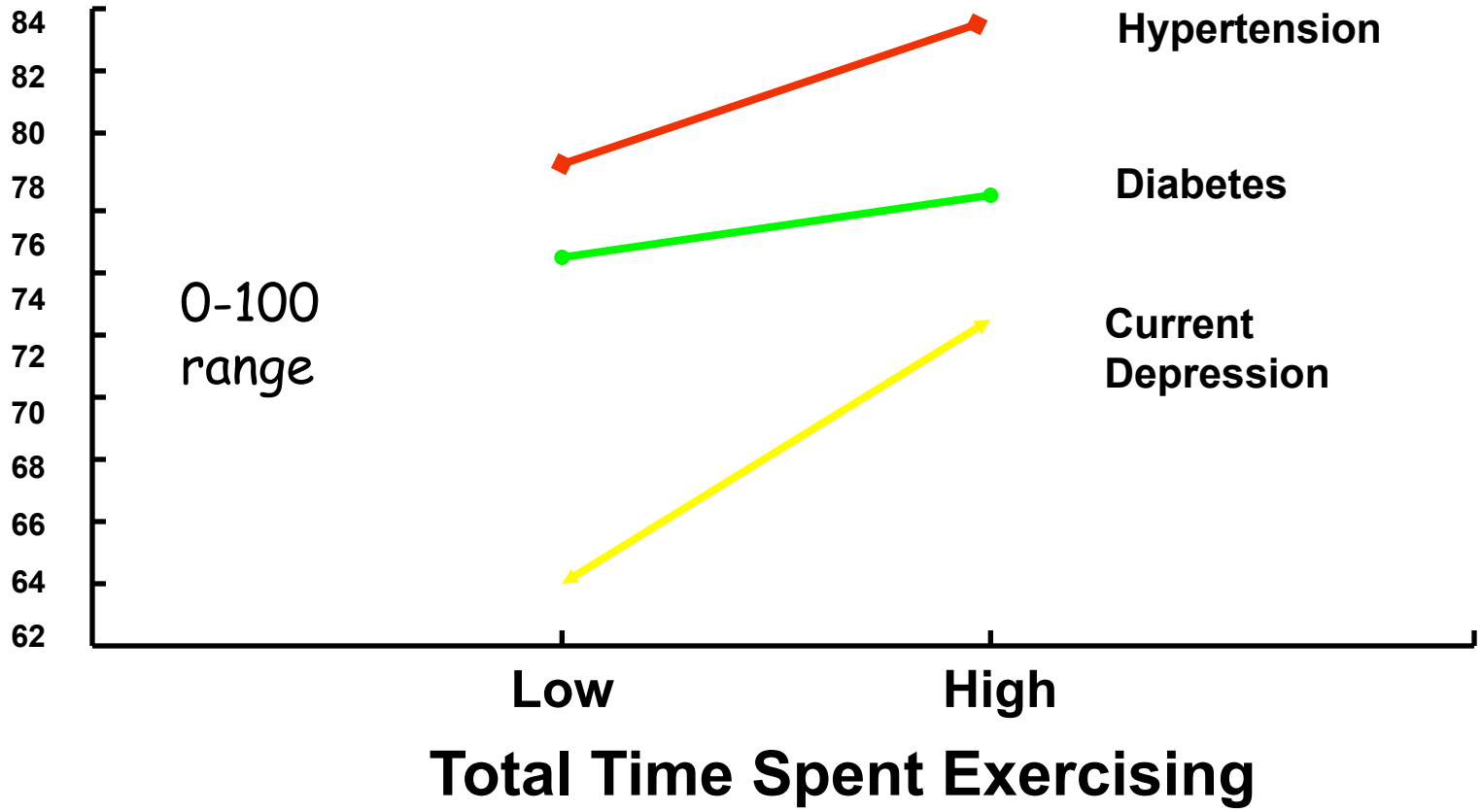
11:00-11:50am

Course of Emotional Well-being Over 2-years for Patients in the MOS General Medical Sector



Hays, R.D., Wells, K.B., Sherbourne, C.D., Rogers, W., & Spritzer, K. (1995). Functioning and well-being outcomes of patients with depression compared to chronic medical illnesses. Archives of General Psychiatry, 52, 11-19.

Physical Functioning in Relation to Time Spent Exercising 2-years Before



Stewart, A.L., Hays, R.D., Wells, K.B., Rogers, W.H., Spritzer, K.L., & Greenfield, S. (1994). Long-term functioning and well-being outcomes associated with physical activity and exercise in patients with chronic conditions in the Medical Outcomes Study. Journal of Clinical Epidemiology, 47, 719-730.

Specific Aims

Among Medicare managed care beneficiaries …

- 1) Do the associations of different types of cancer and (non-cancer) chronic conditions with health-related quality of life vary among Medicare managed care beneficiaries?

- 2) Do the associations between cancer and health-related quality of life vary by stage of disease?

Hays, R. D., Reeve, B. B., Smith, A. W., & Clauser, S. B. (2013, epub). Associations of cancer and other chronic medical conditions with SF-6D preference-based scores in Medicare beneficiaries. Quality of Life Research.

- Surveillance, Epidemiology and End Results (SEER) program of cancer registries that collect standardized clinical and demographic information for persons with newly diagnosed (incident) cancer in specific geographical areas

- Began in 1973 and covers ~ 26% of U.S. pop.
  - http://seer.cancer.gov/registries/list.html
  - California, Connecticut, Hawaii, Iowa, Kentucky, Louisiana, New Mexico, New Jersey, Utah
  - Atlanta, Detroit, rural Georgia, Seattle-Puget Sound metropolitan areas

- Medicare Health Outcomes Survey (MHOS)
  - 95-item survey administered to 1,000 randomly selected beneficiaries (including institutionalized and disabled) in Medicare managed care plans
  - Baseline and follow-up survey (2 years later).
  - 63-72% response rates for baseline surveys
  - MHOS respondents matched using identifiers to SEER-Medicare file for 4 cohorts (1998 to 2003).
- http://outcomes.cancer.gov/surveys/seer-mhos/

Sample (n = 126,366)

- 55% female
- 79% non-Hispanic white, 7% Hispanic, 5% Black, 5% Asian
- 60% married
- 58% high school graduate or less
- 51% < $30,000 income

Dependent Variable = SF-6D

- SF-36 health survey, version 1

- 11 of 36 questions representing 6 of 8 domains
  - –Physical functioning
  - –Role limitations
  - –Social function
  - –Pain
  - –Emotional well-being
  - –Energy/fatigue

- Standard gamble elicitation of preferences from a population sample in the UK.

- Scores for those alive range from 0.30 to 1.00 (dead = 0.00).

10 Cancer Conditions (n = 22,740; 18%)

- Prostate cancer (n = 5,593; 4%)
- Female breast Cancer (n = 4,311; 3%)
- Colorectal cancer (n = 3,012; 2%)
- Non-small cell lung cancer (n = 1,792; 1%)

- Bladder cancer (n = 1,299; 1%)
- Melanoma (n = 1,135; 1%)
- Endometrial cancer (n = 902; 1%)
- Non-Hodgkin's lymphoma (n = 668; 1%)
- Kidney cancer (n = 488; 0.4%)

- Other cancer (n = 3,540; 3%)

Note: Those with more than one cancer diagnosis are excluded.

Historic Stage of Disease (time of diagnosis)

- Localized
  - 2045 breast, 2652 prostate, 1481 colorectal, 466 lung
- Distant (metastatic)
  - 26 breast, 61 prostate, 48 colorectal, 47 lung
- Unstaged
  - 347 breast, 633 prostate, 203 colorectal, 65 lung

13 Non-cancer Conditions (mean number = 2.44)

• Hypertension	n = 66,968	(53%)
• Arthritis of the hip	n = 44,524	(35%)
• Arthritis of the hand	n = 40,402	(32%)
• Sciatica	n = 26,878	(21%)
• Other heart disease	n = 25,455	(20%)
• Diabetes	n = 20,089	(16%)
• Angina/coronary artery disease	n = 18,017	(14%)
• Chronic obstructive pulmonary disease	n = 15,445	(12%)
• Depressed in the last year	n = 14,815	(12%)
• Myocardial infarction/heart attack	n = 11,982	(9%)
• Stroke	n = 9,479	(8%)
• Congestive heart failure	n = 7,893	(6%)
• Inflammatory bowel disease	n = 5,882	(5%)

Has a doctor ever told you that you had: …
In the past year, have you felt depressed or sad much of the time?

Demographic & Administration Variables

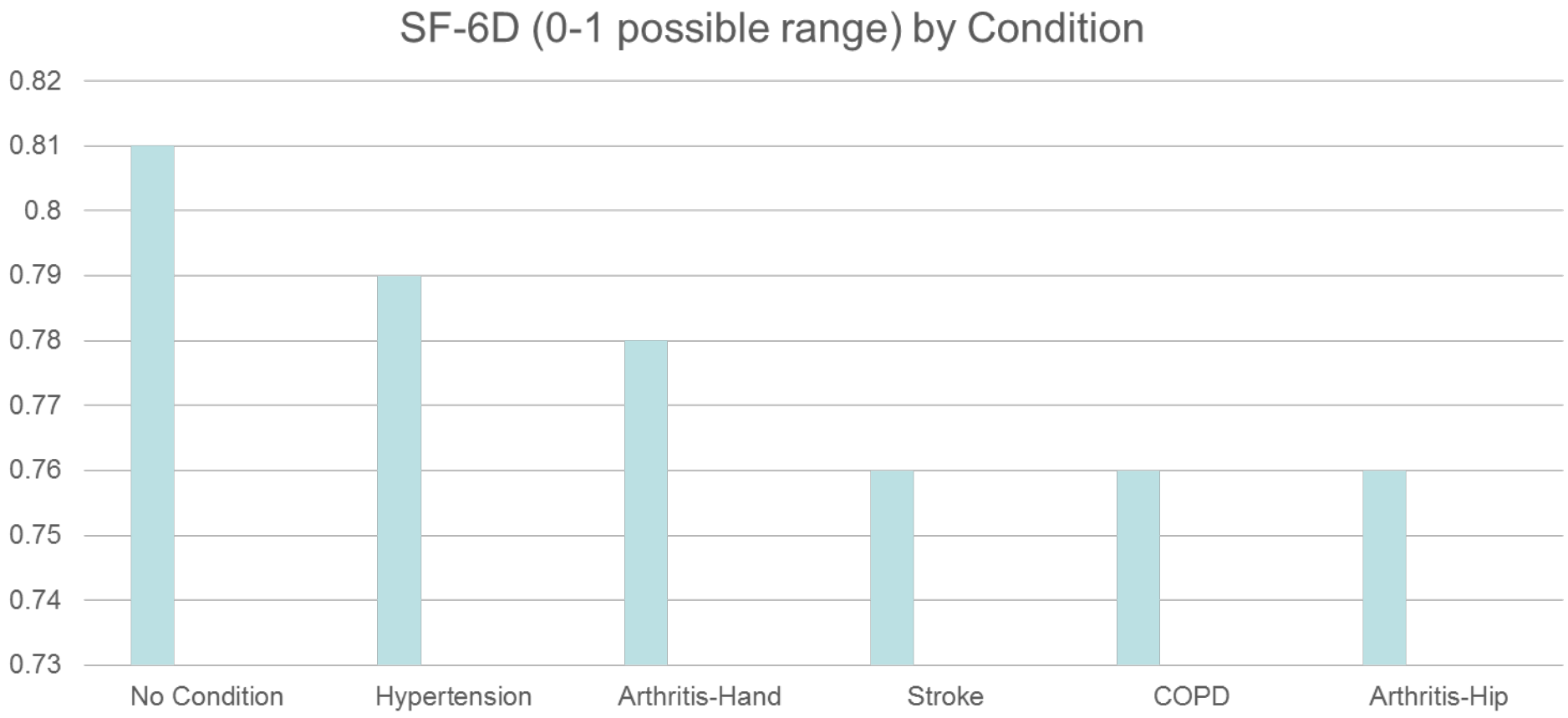
- Age (continuous)
- Education (8th grade or less; some high school; high school graduate; some college; 4 year college grad; > 4 year college)
- Gender (male; female)
- Income (<10k, 10-19999, 20-29999, 30-39999, 40-49999, 50-79999, 80k and above, don't know or missing)
- Race/ethnicity (Hispanic, non-Hispanic white, black, Asian, American Indian, other race, missing)
- Marital status (married, widowed, divorced/separated/never married)

- Proxy completed survey (11%)
- Mode of administration (88% mail vs. 12% phone)

Results

- Adjusted R-squared of 39% for 43 dfs
- Intercept = 0.81
  - No chronic condition, average education and age, divorced/separated/never married, white, don't know/missing income, phone mode)
  - SD = 0.14
- Only 2 of 23 conditions had non-significant associations (melanoma, endometrial cancer)

HRQOL in SEER-Medicare Health Outcomes Study (n = 126,366)



Controlling for age, gender, race/ethnicity, education, income, and marital status.

Distant stage of cancer associated with 0.05-0.10 lower SF-6D Score

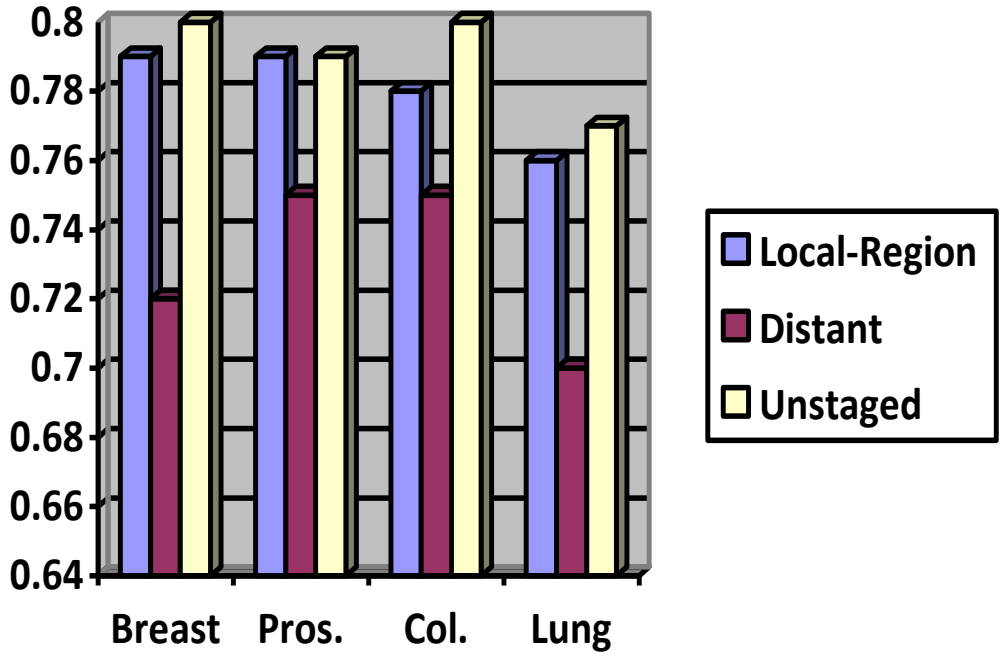
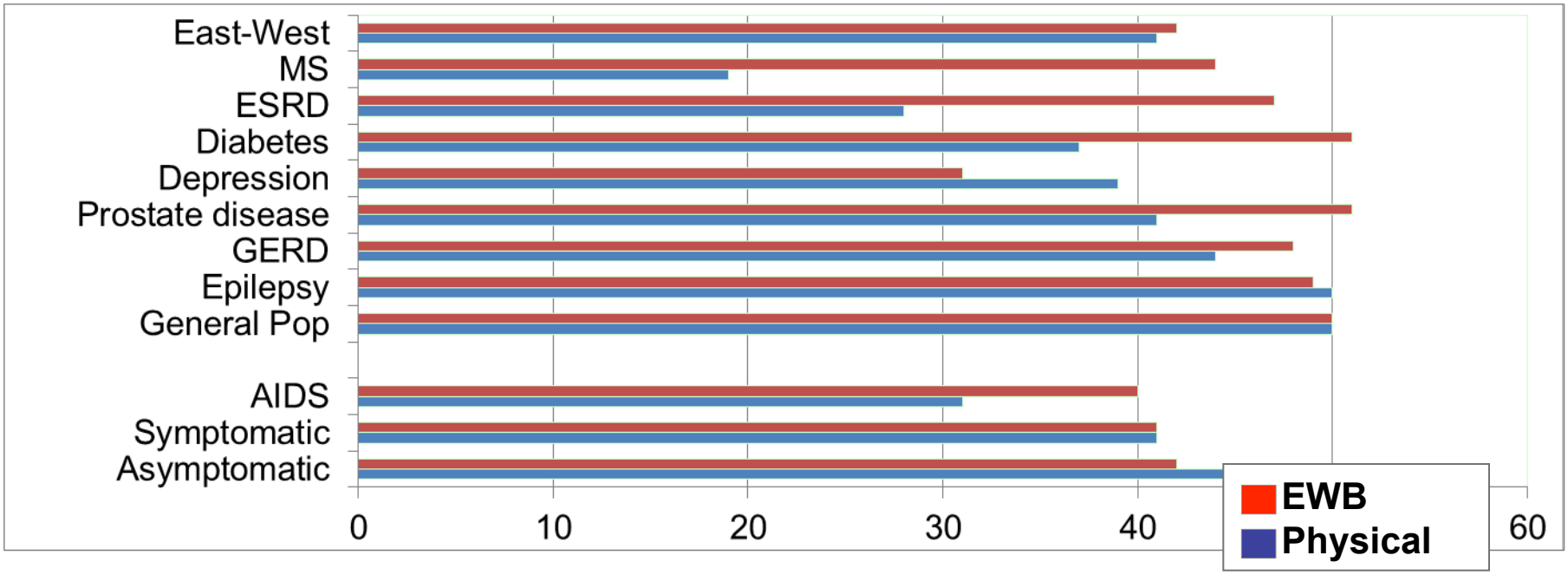


Figure 1. Distant Stage of Disease Associated with Worse SF-6D Scores (Sample sizes for local/regional, distant, and unstaged: Breast (2045,26, 347); Prostate (2652, 61 and 633), Colorectal (1481, 48 and 203), and Lung (466, 47 and 65).

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.

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

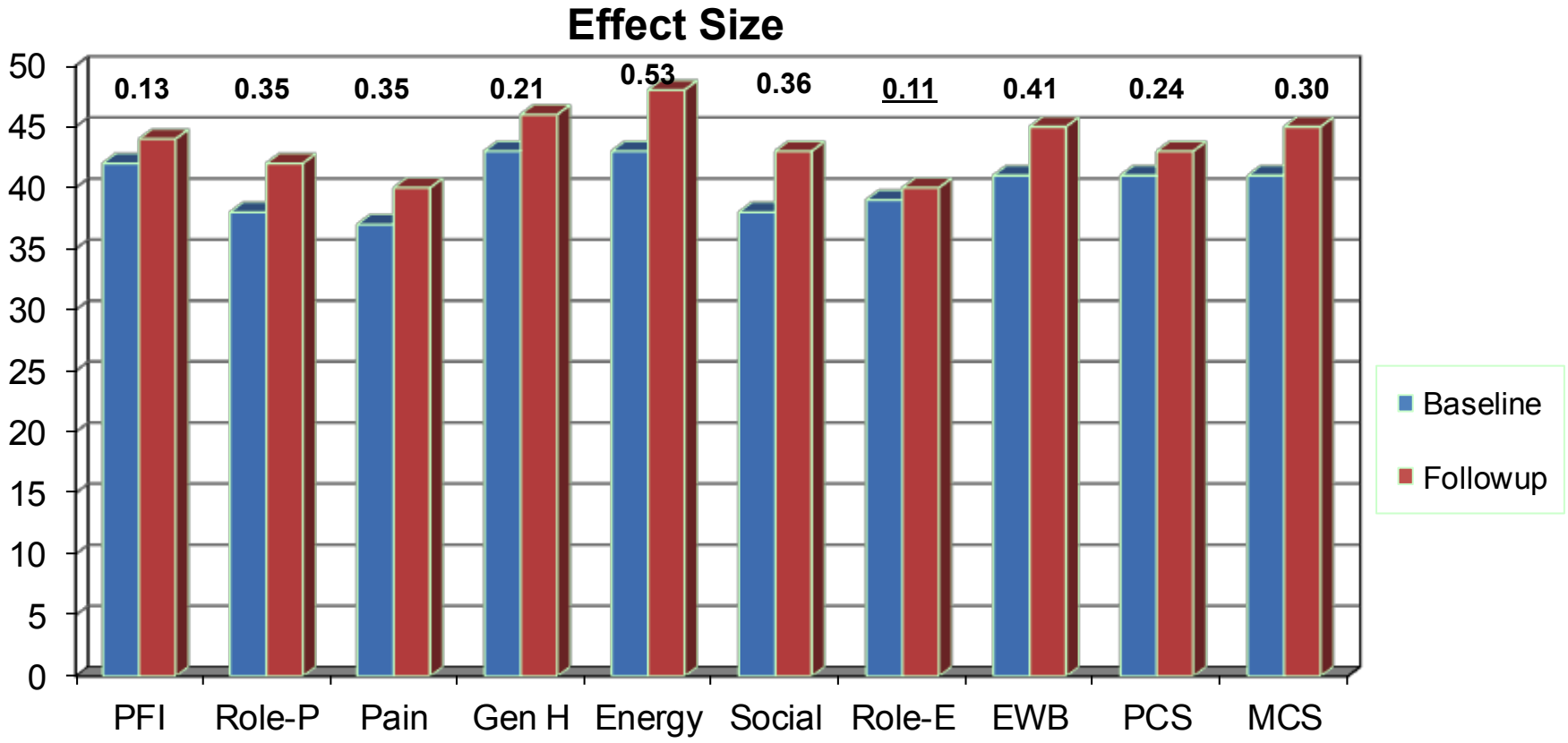
Effect Size

(Follow-up – Baseline)/ $SD_{baseline}$

Cohen's Rule of Thumb:

- ✓ ES = 0.20 Small
- ✓ ES = 0.50 Medium
- ✓ 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.

Break #3

Use of Patient-Reported Outcome Measures in Clinical Practice

12:00-12:30 pm

Defining a Responder: Reliable Change Index (RCI)

$$\frac{X_2 - X_1}{(\sqrt{2})(SEM)}$$

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

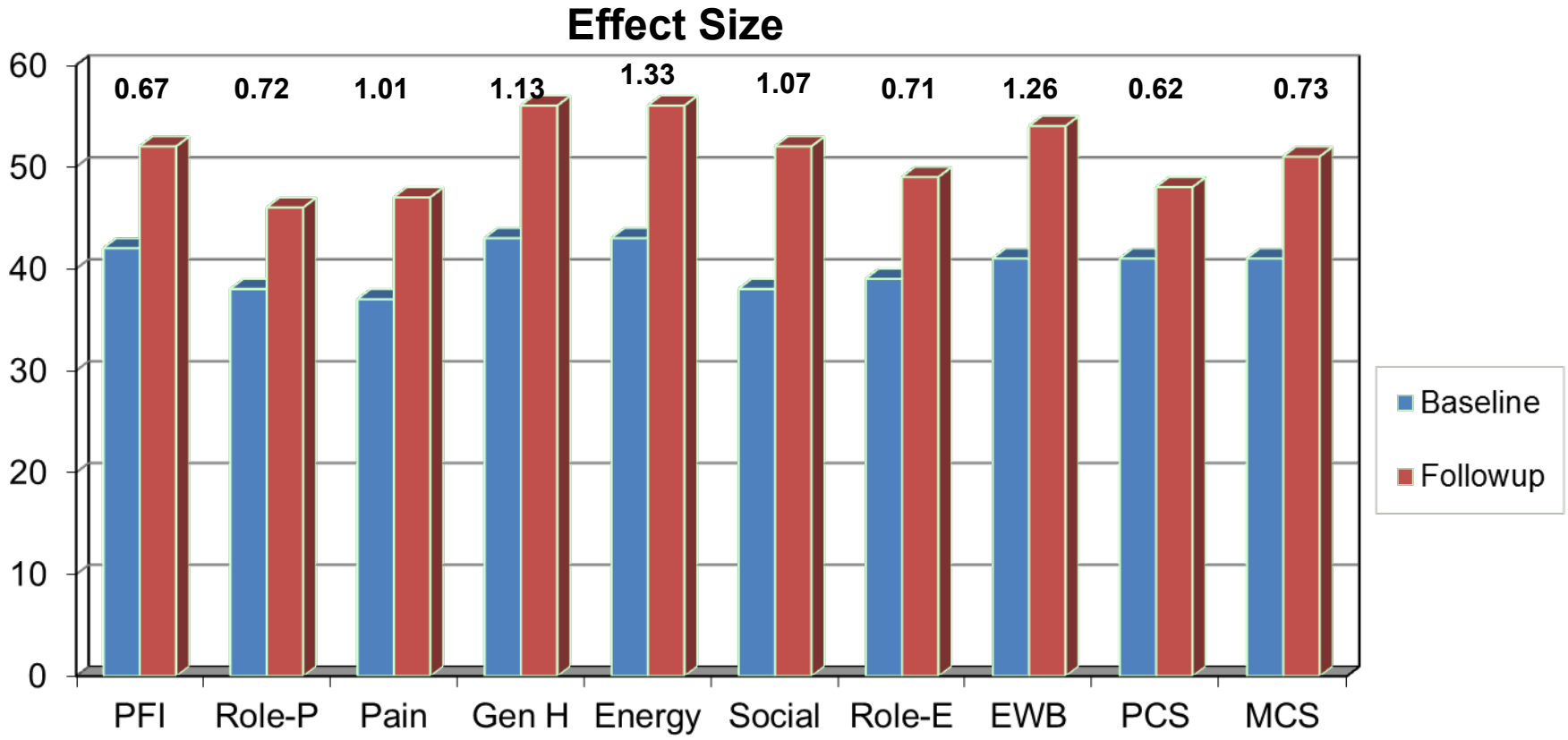
Note: SD_{bl} **= standard deviation at baseline**
r_{xx} **= reliability**

Amount of Change in Observed Score Needed To be Statistically Significant

$$\frac{}{(\sqrt{2})\,(SD_{bl})\sqrt{(1 - r_{xx})}\,(1.96)}$$

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

Amount of Change Needed for Significant Individual Change

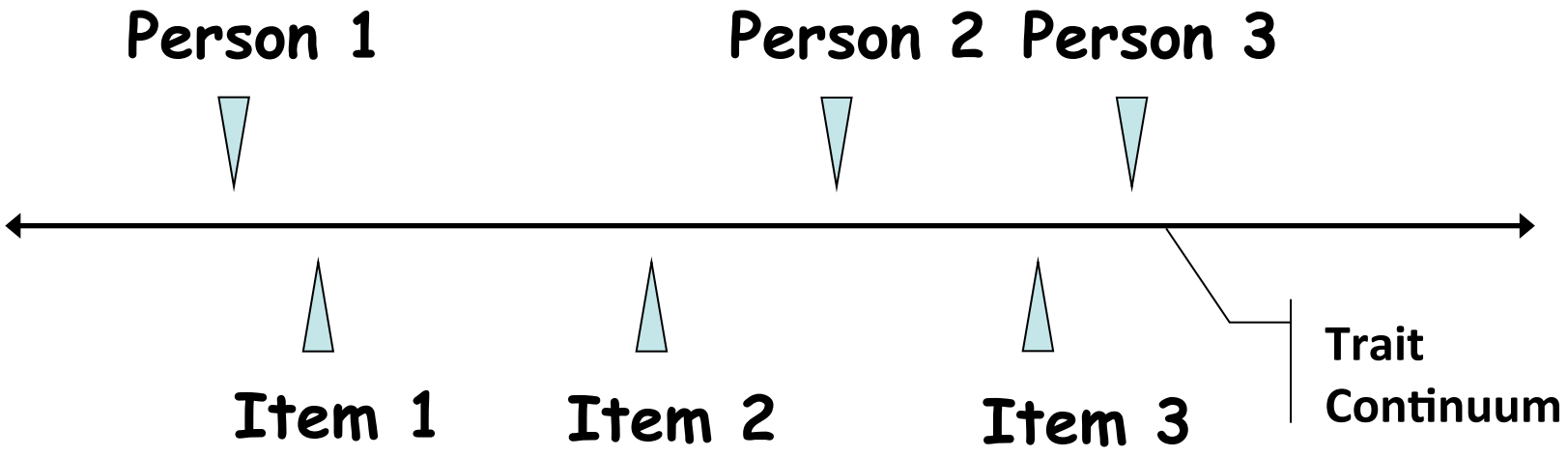


PFI = Physical Functioning; Role-P = Role-Physical; Pain = Bodily Pain; Gen H=General Health; Energy = Energy/Fatigue; Social = Social Functioning; Role-E = Role-Emotional; EWB = Emotional Well-being; PCS = Physical Component Summary; MCS =Mental Component Summary.

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%

Item Responses and Trait Levels



www.nihpromis.org

Computer Adaptive Testing (CAT)

Reliability Target for Use of Measures with Individuals

- Reliability ranges from 0-1
  - 0.90 or above is goal
  - SEM = SD (1- reliability)$^{1/2}$
  - 95% CI = true score +/- 1.96 x SEM
    - if true z-score = 0, then CI: -.62 to +.62
      - Width of CI is 1.24 z-score units

- Reliability = 0.90 when SE = 3.2
  - T-scores (mean = 50, SD = 10)
  - Reliability = 1 – (SE/10)^2

T = 50 + (z * 10)

Reliability and SEM

- For z-scores (mean = 0 and SD = 1):
  - Reliability = $1 - SE^2$
  - So reliability = **0.90** when SE = 0.32

- For T-scores (mean = 50 and SD = 10):
  - Reliability = $1 - (SE/10)^2$
  - So reliability = **0.90** when SE = 3.2

In the past 7 days …

I was grouchy [1st question]

- Never [39]
- Rarely [48]
- Sometimes [56]
- Often [64]
- Always [72]

Estimated Anger = 56.1

SE = 5.7 (rel. = 0.68)

In the past 7 days …

I felt like I was ready to explode

[2nd question]

- Never
- Rarely
- Sometimes
- Often
- Always

Estimated Anger = 51.9

SE = 4.8 (rel. = 0.77)

In the past 7 days …

I felt angry [3rd question]

- Never
- Rarely
- Sometimes
- Often
- Always

Estimated Anger = 50.5

SE = 3.9 (rel. = 0.85)

In the past 7 days …

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

- Never
- Rarely
- Sometimes
- Often
- Always

Estimated Anger = 48.8

SE = 3.6 (rel. = 0.87)

In the past 7 days …

I felt annoyed [5th question]

- Never
- Rarely
- Sometimes
- Often
- Always

Estimated Anger = 50.1

SE = 3.2 (rel. = 0.90)

In the past 7 days …

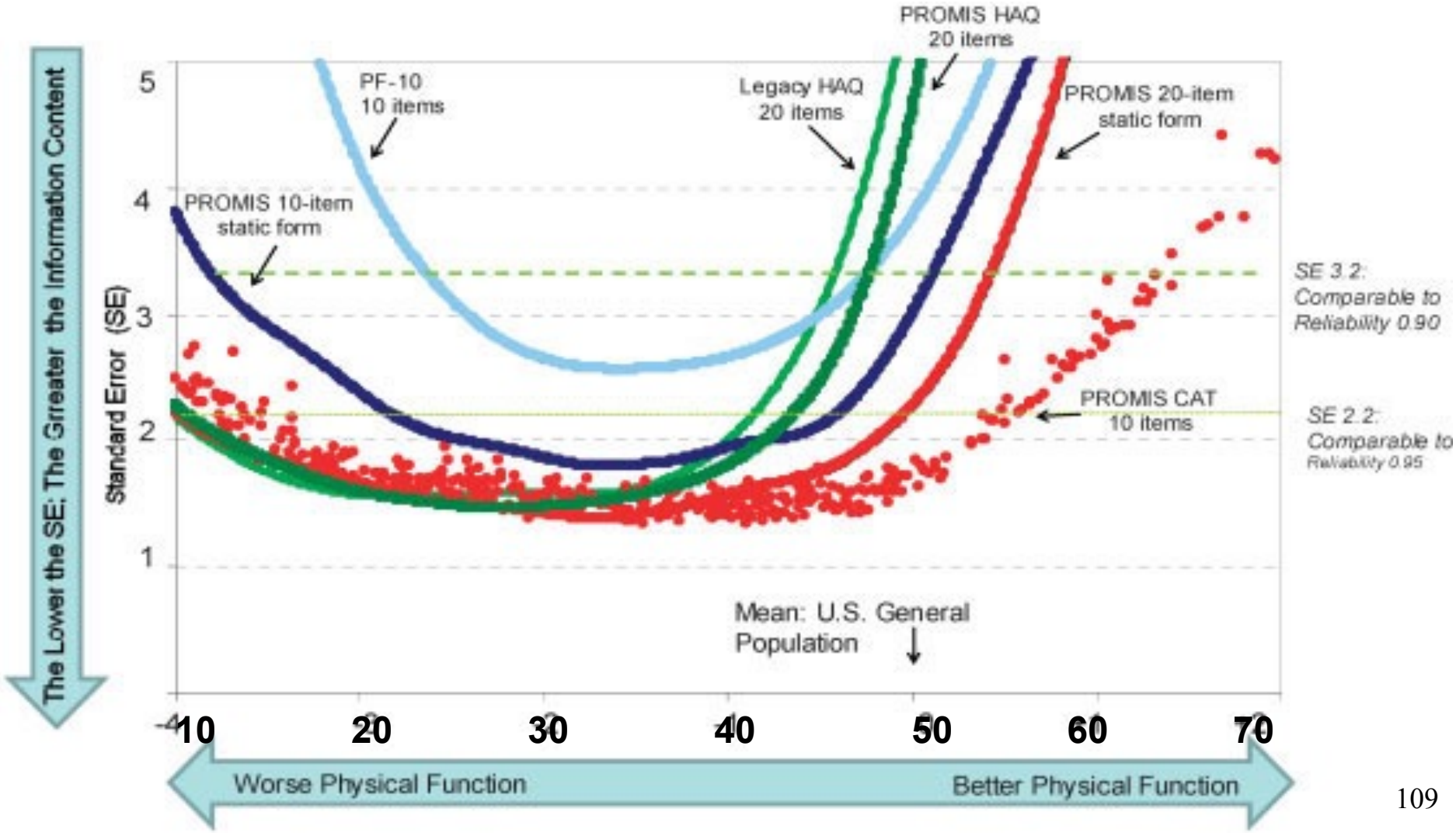
I made myself angry about something just by thinking about it. [6th question]

- Never
- Rarely
- Sometimes
- Often
- Always

Estimated Anger = 50.2

SE = 2.8 (rel = 0.92)

PROMIS Physical Functioning vs. "Legacy" Measures



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

- Snyder, C.F., Aaronson, N. K., et al. Quality of Life Research, 21, 1305-1314, 2012.

  - 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

Thank you

drhays@ucla.edu (310-794-2294). Powerpoint file available for downloading at: http://gim.med.ucla.edu/FacultyPages/Hays/

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