

Comprehensive Approach to Measuring Health Outcomes

Ron D. Hays, Ph.D. (drhays@ucla.edu)
UCLA GIM & HSR

October 23, 2006 (3:15-4:45 pm)

MacDonald Research Building (MRL)

Room 2740

How do you know how the patient is doing?

Temperature

Respiration

Pulse

Weight

Blood pressure



Also, by talking to her or him about ...

Symptoms

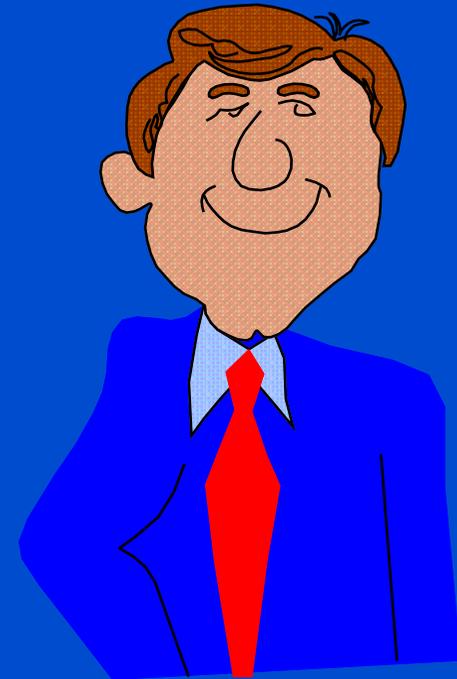
- Have you had a fever in the last 7 days?

No

Yes

What they are able to do

And how they feel about their life



First RCT of Treatment for Newly Diagnosed Prostate Cancer (NEJM, 2002)

Radical prostatectomy vs. watchful waiting

- Trend to reduction in all-cause mortality

(18% versus 15%; RR 0.83, 0.57 to 1.2, $p = 0.31$)

Impact on Symptoms

Urinary obstruction (weak stream)

- 44% waiting, 28% prostatectomy +

Urinary leakage

- 49% prostatectomy - vs. 21% waiting

Sexual dysfunction

- 80% prostatectomy - vs. 45% waiting

Woman's Health Initiative (NEJM, 2003)

**Effects of hormone therapy on HRQOL (n = 16,608;
1,511 with HRQOL data)**

Postmenopausal women 50-79 randomized to either:

Placebo or estrogen plus progestin

No difference on general health, energy, emotional well-being, and sexual satisfaction.

Significant but small difference favoring treatment for physical functioning, pain, and sleep disturbance

Archives of Internal Medicine (2006)

RAND appropriateness method applied to 784 undergoing total hip replacement and 792 undergoing total knee replacement.

Appropriate candidates for *total hip joint replacement* demonstrated greater improvement than those judged inappropriate in terms of:

**physical functioning (34 vs. 20) , role–physical (35 vs. 9),
bodily pain (33 vs. 6), and social function (27 vs. 7).**

Appropriate candidates for *total knee replacement* had greater improvement in social functioning than those deemed inappropriate candidates (20 vs. 8).

Health-Related Quality of Life (HRQOL) is:

What the person can DO (functioning)

- Self-care
- Role
- Social

How the person FEELS (well-being)

- Emotional well-being
- Pain
- Energy

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?

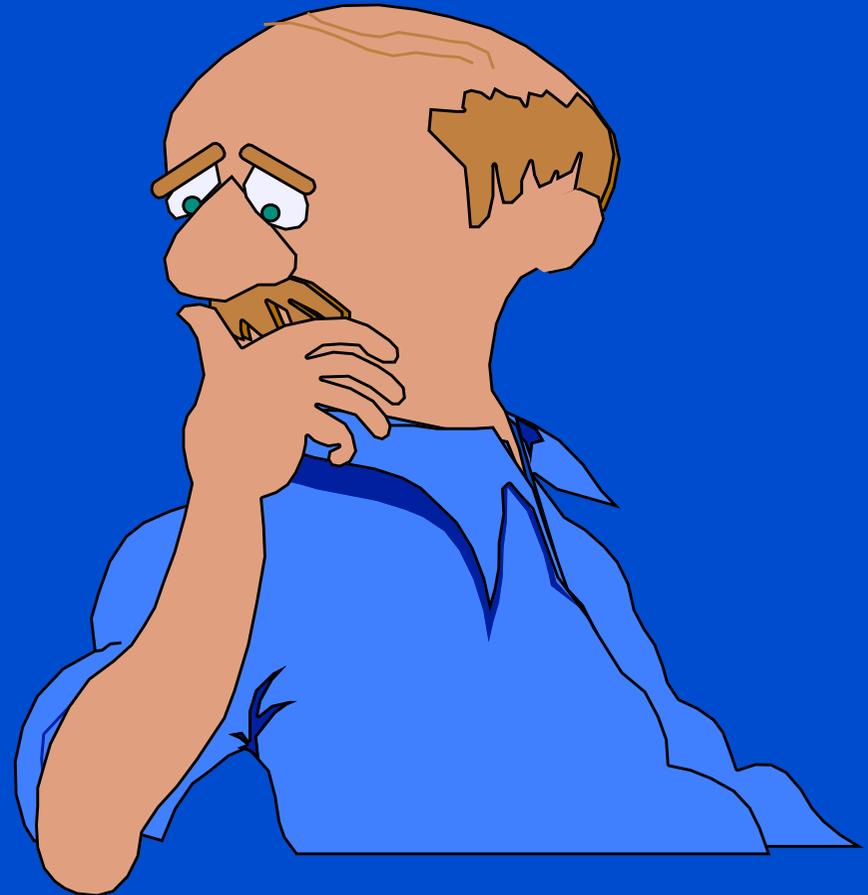
None of the time

A little of the time

Some of the time

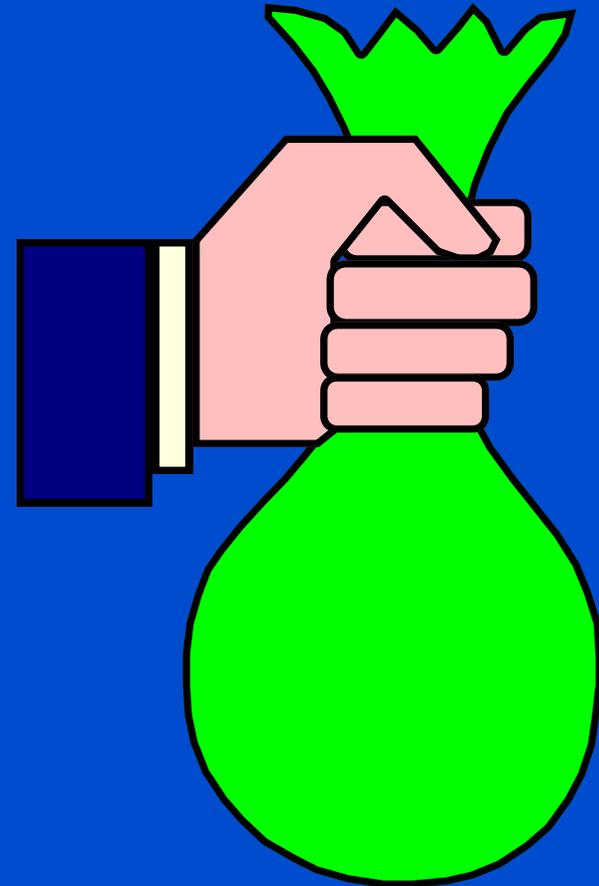
Most of the time

All of the time



HRQOL is Not

- Quality of environment
- Type of housing
- Level of income
- Social Support



Reliability estimates

Reliability—extent to which you get the same score on repeated assessments

0.80-0.90 for blood pressure

0.70-0.90 for multi-item self-report scales

Are self-reports about HRQOL valid?

Validity—score represents what you are trying to measure rather than something else

In general, how would you rate your health?

Excellent

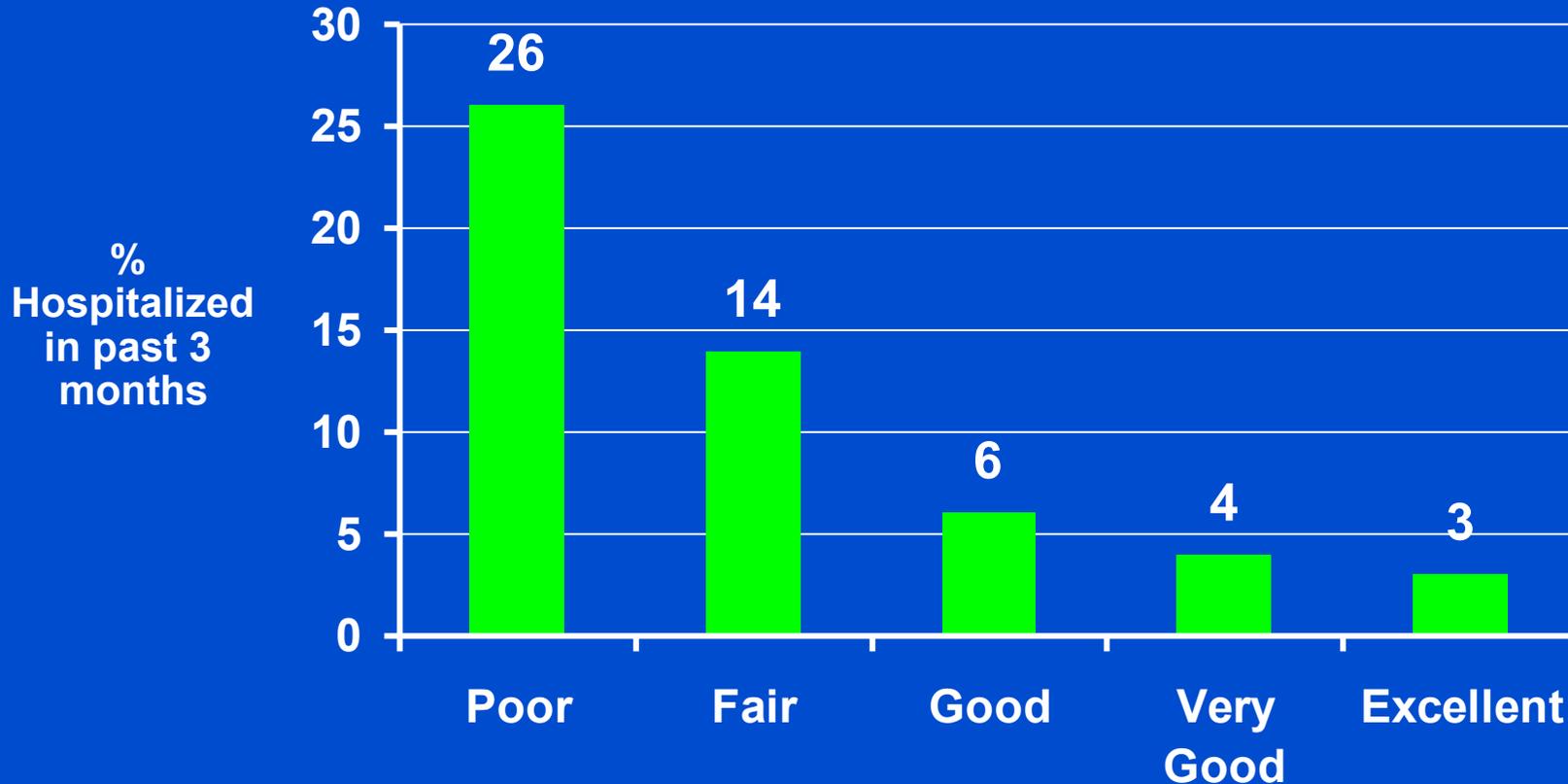
Very Good

Good

Fair

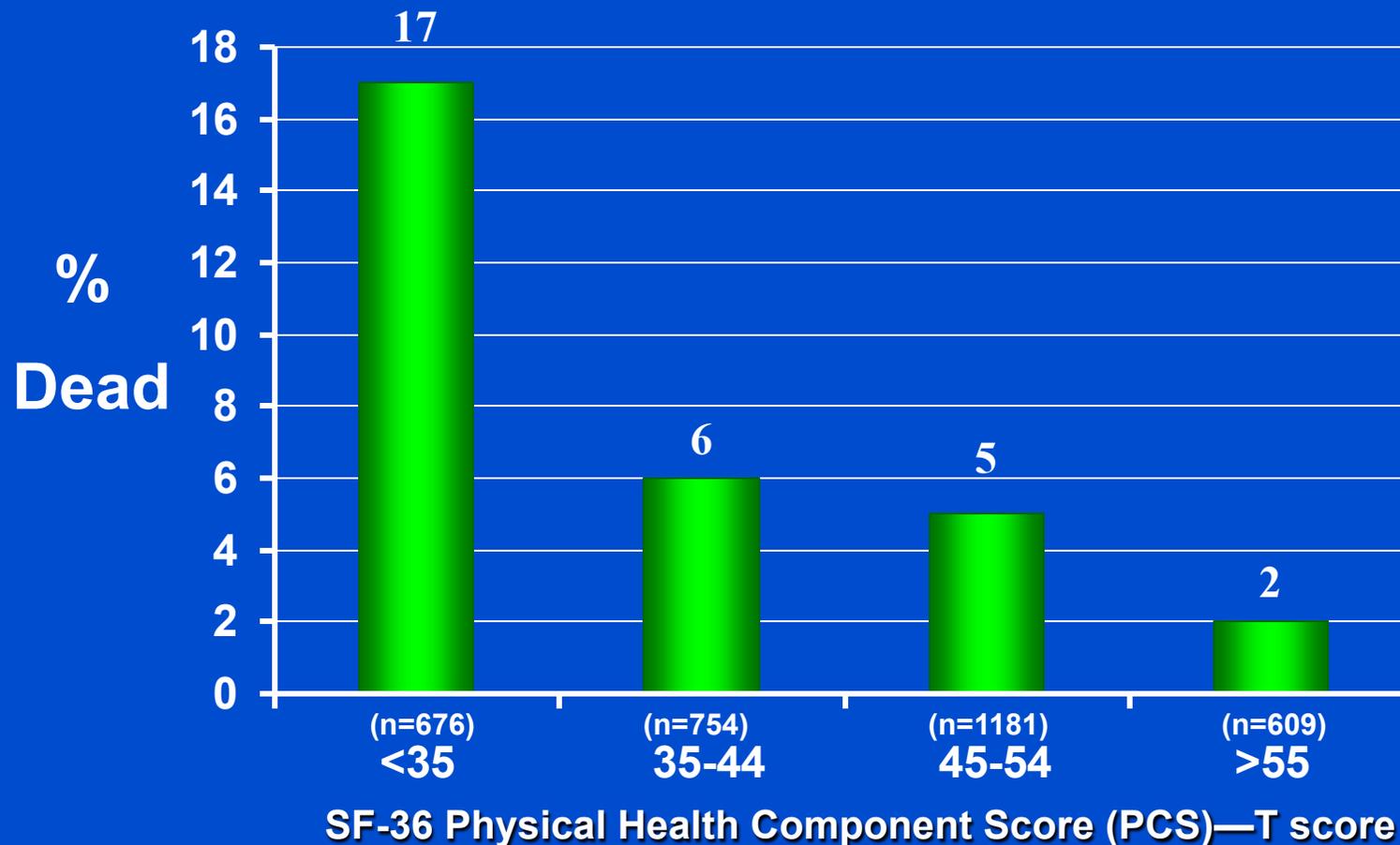
Poor

Hospitalized Patients Report Worse General Health (n = 20,158)



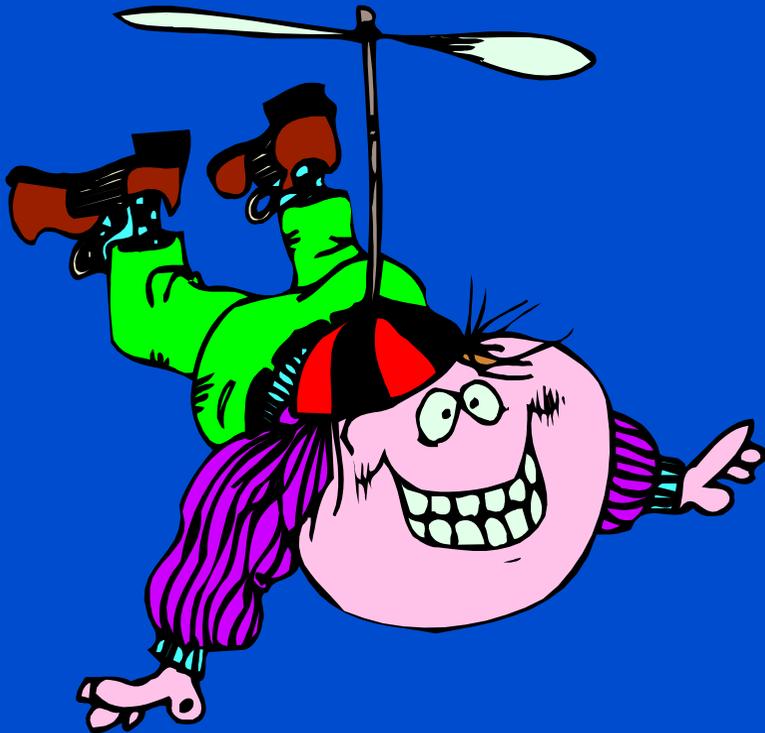
Kravitz, R. et al. (1992). Differences in the mix of patients among medical specialties and systems of care: Results from the Medical Outcomes Study. *JAMA*, *267*, 1617-1623.

Self-Reports of Physical Health Predictive of Five-Year Mortality Rates



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

Types of HRQOL Measures



Generic Profile

Targeted Profile

Preference Measure

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)

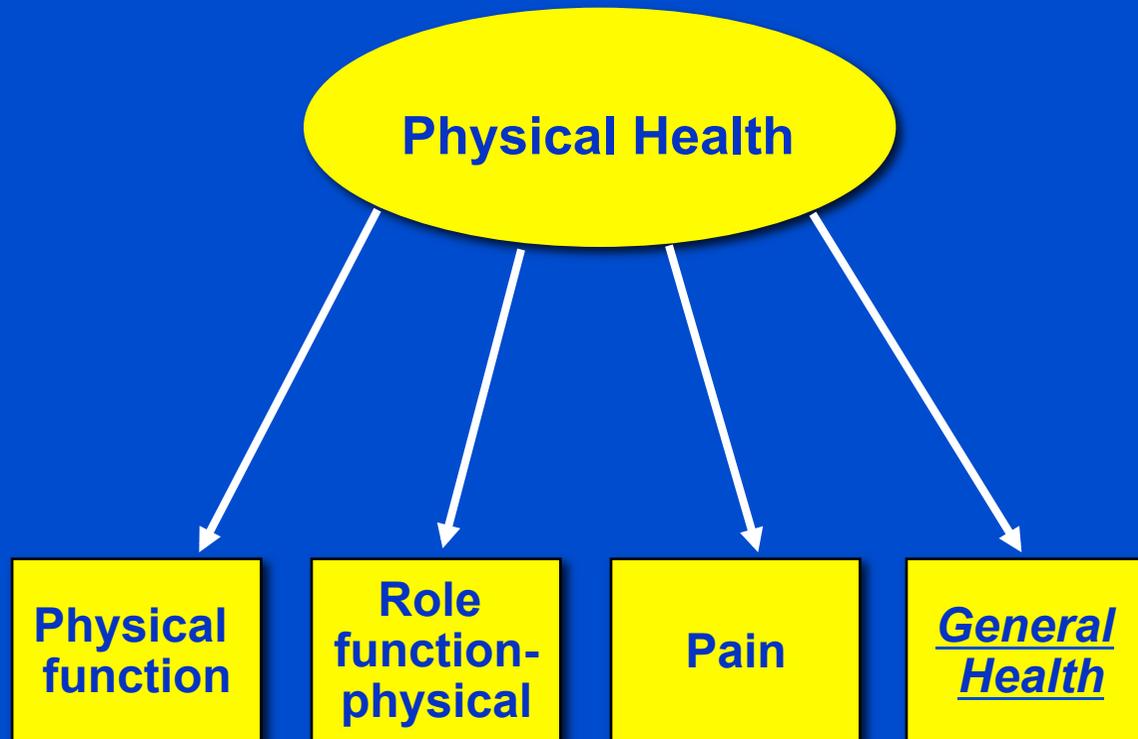
Scoring Generic HRQOL Scales

Average or sum all items in the same scale.

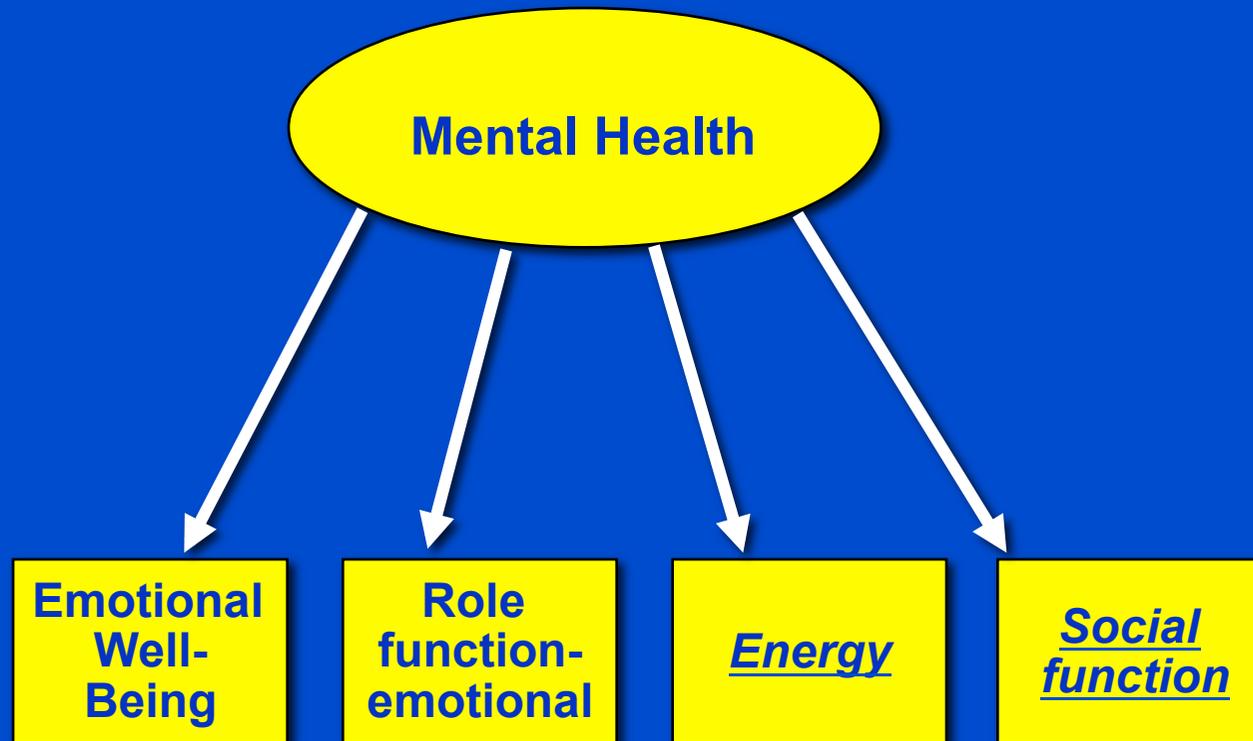
Transform average or sum to

- 0 (worse) to 100 (best) possible range
- z-score (mean = 0, SD = 1)
 - T-score (mean = 50, SD = 10)

Physical Health



Mental Health



Example Uses of Generic HRQOL Measures

Cross-Sectional

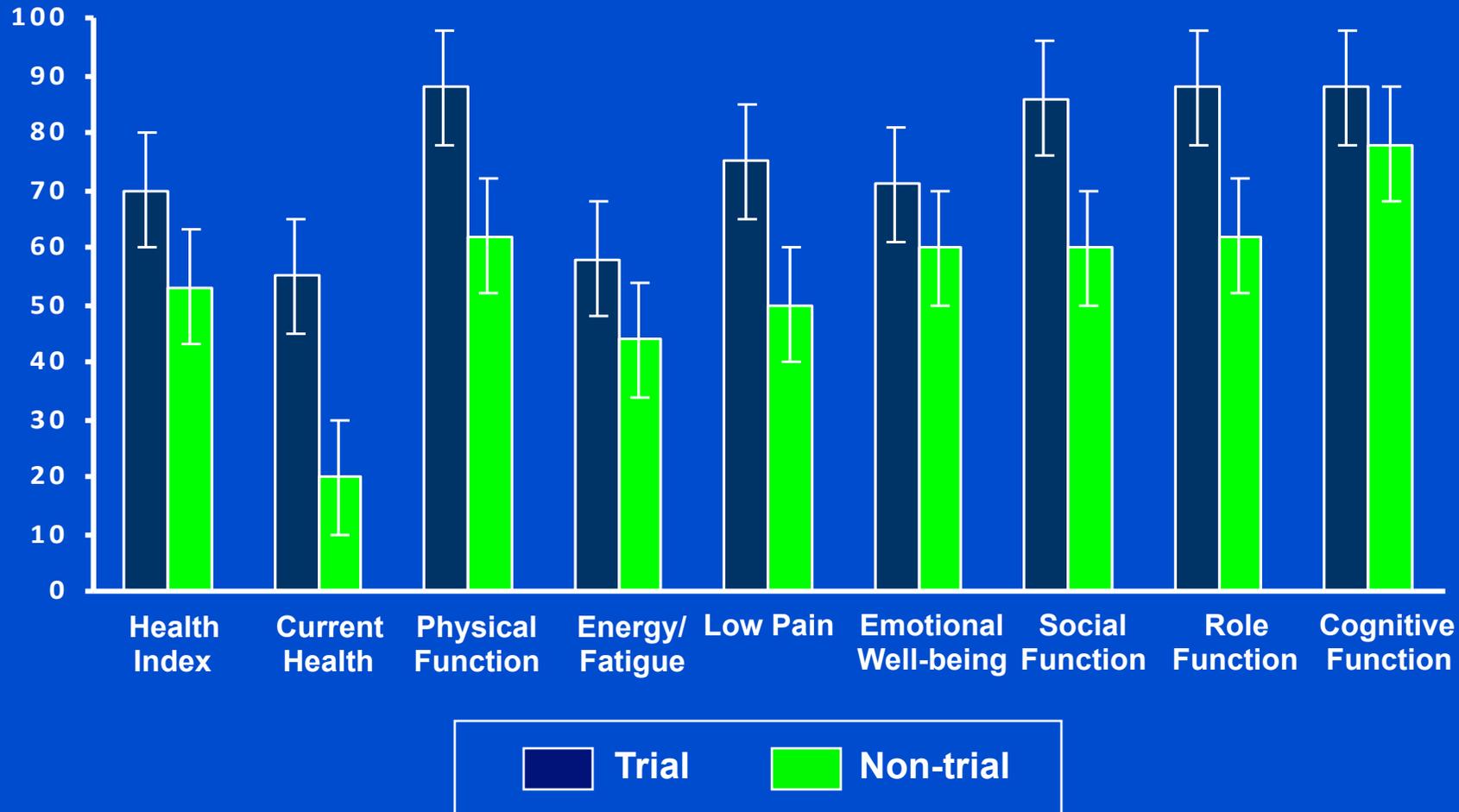
- Comparison of Same Disease in Different Samples
- Profiles of Different Diseases

Longitudinal

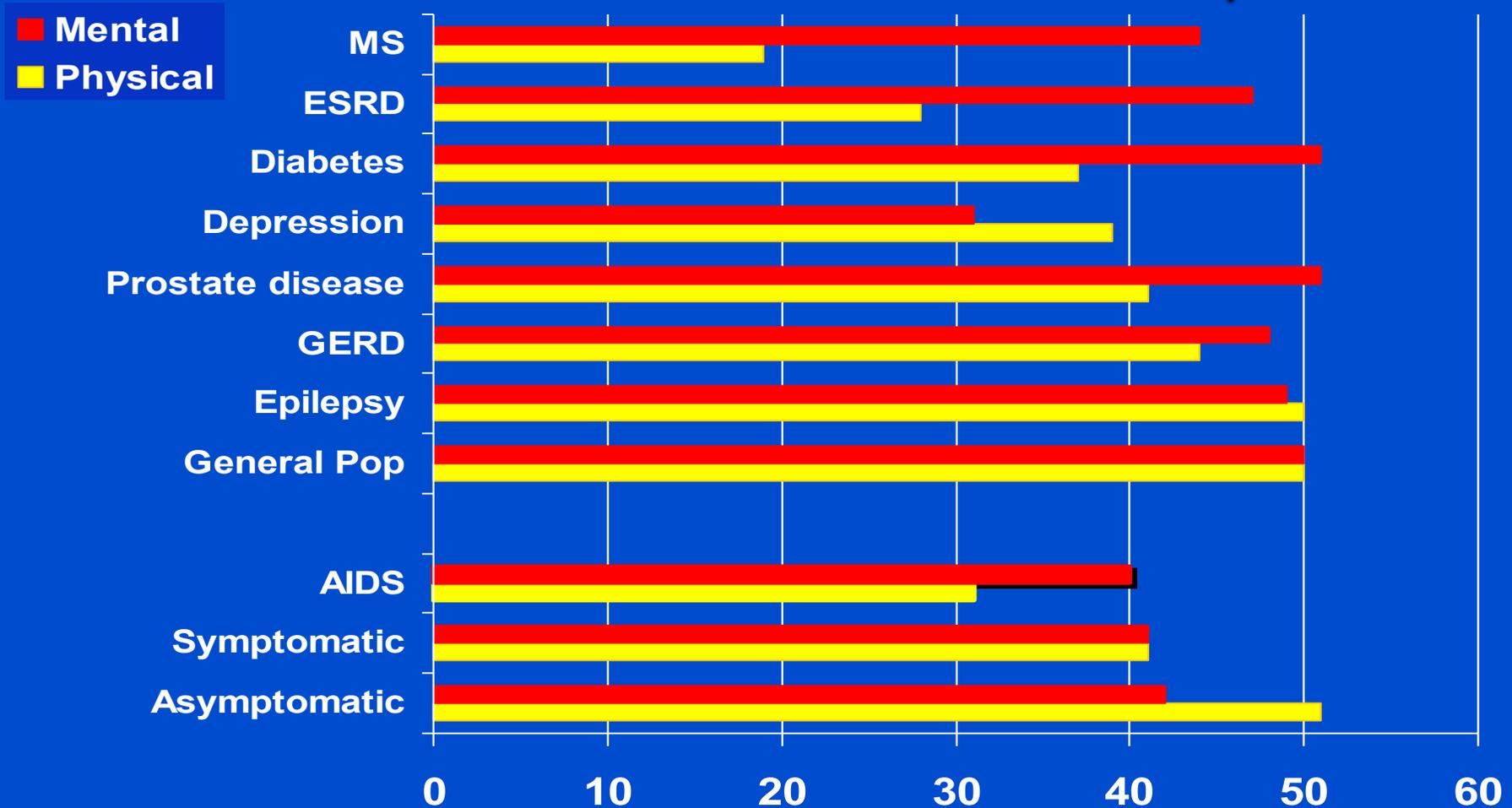
- Profiles of Different Disease
- Identifying Antecedents/Causes of HRQOL

HRQOL of Patients in ACTG versus Public Hospital Samples

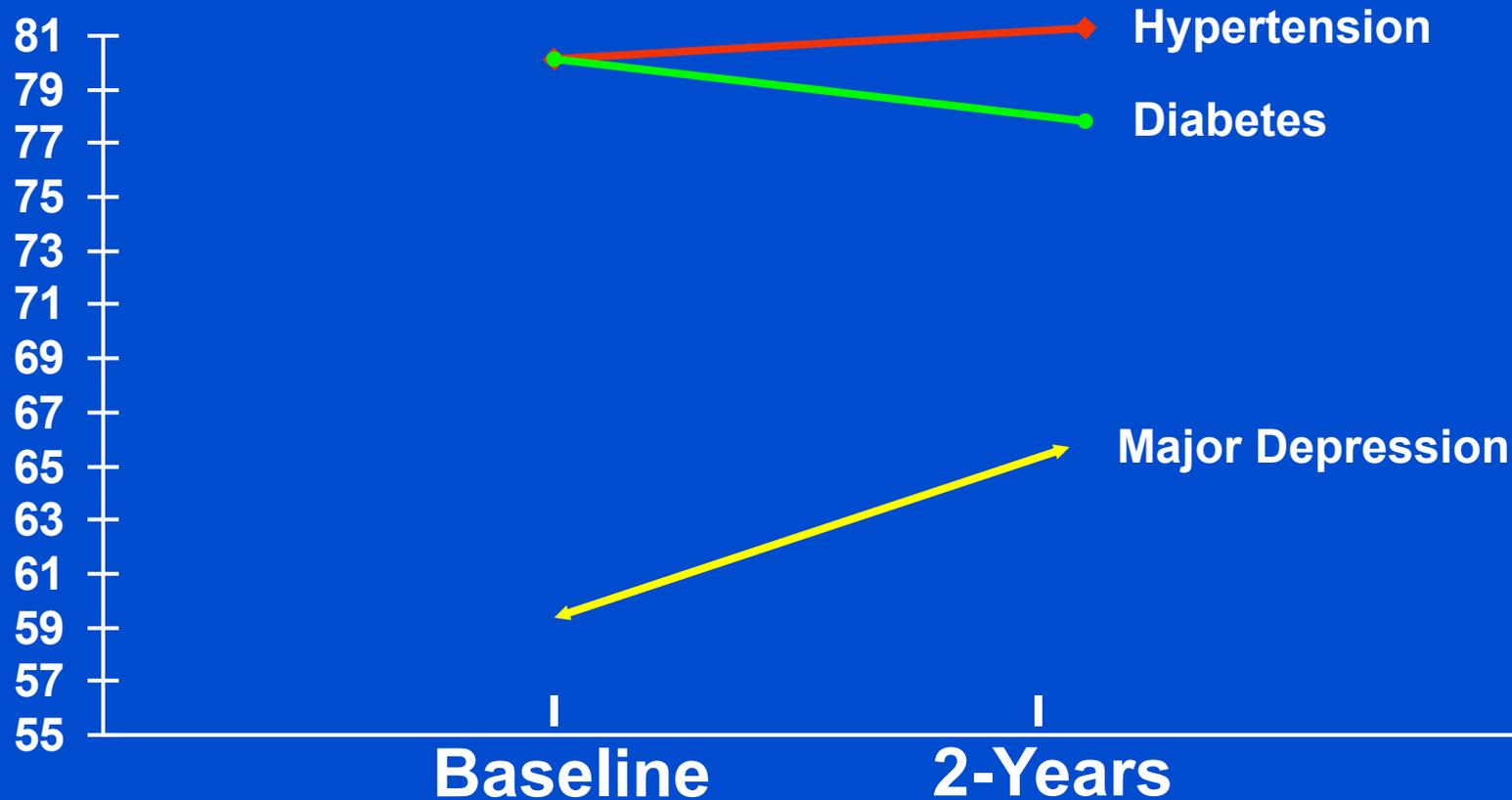
Adjusted Scale Scores (Cunningham et al., 1995)



HRQOL for HIV Compared to other Chronic Illnesses and General Population

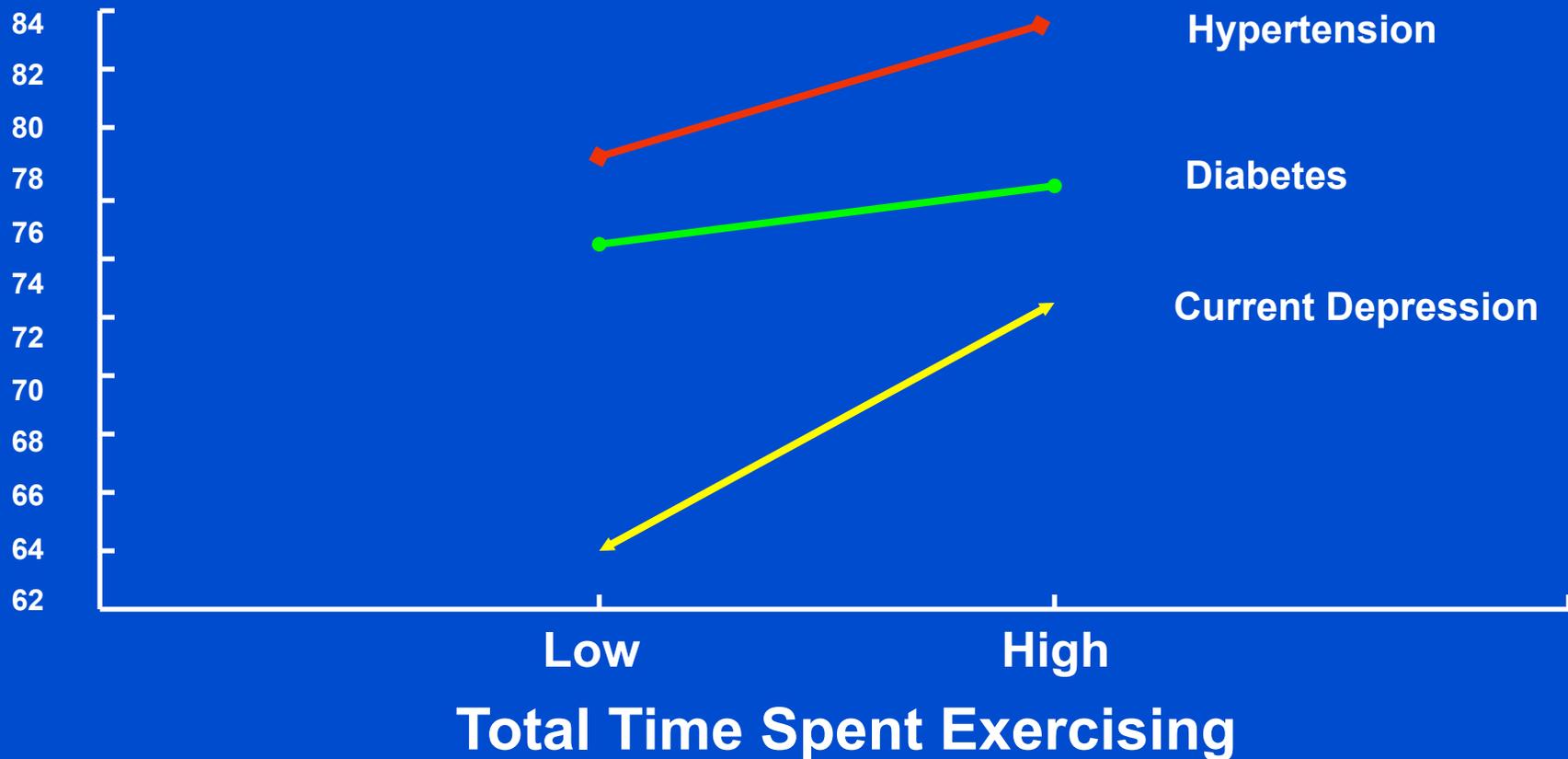


Course of Emotional Well-being Over 2-years for Patients in the MOS General Medical Sector



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.

Targeted HRQOL Measures

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

Kidney-Disease Targeted Items

During the last 30 days, to what extent were you bothered by each of the following?

- Cramps during dialysis
- Washed out or drained

Not at all bothered

Somewhat bothered

Moderately bothered

Very much bothered

Extremely bothered

IBS-Targeted Item

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

HRQOL in Men Treated for Localized Prostate Cancer

Cross-sectional study of managed care pop.

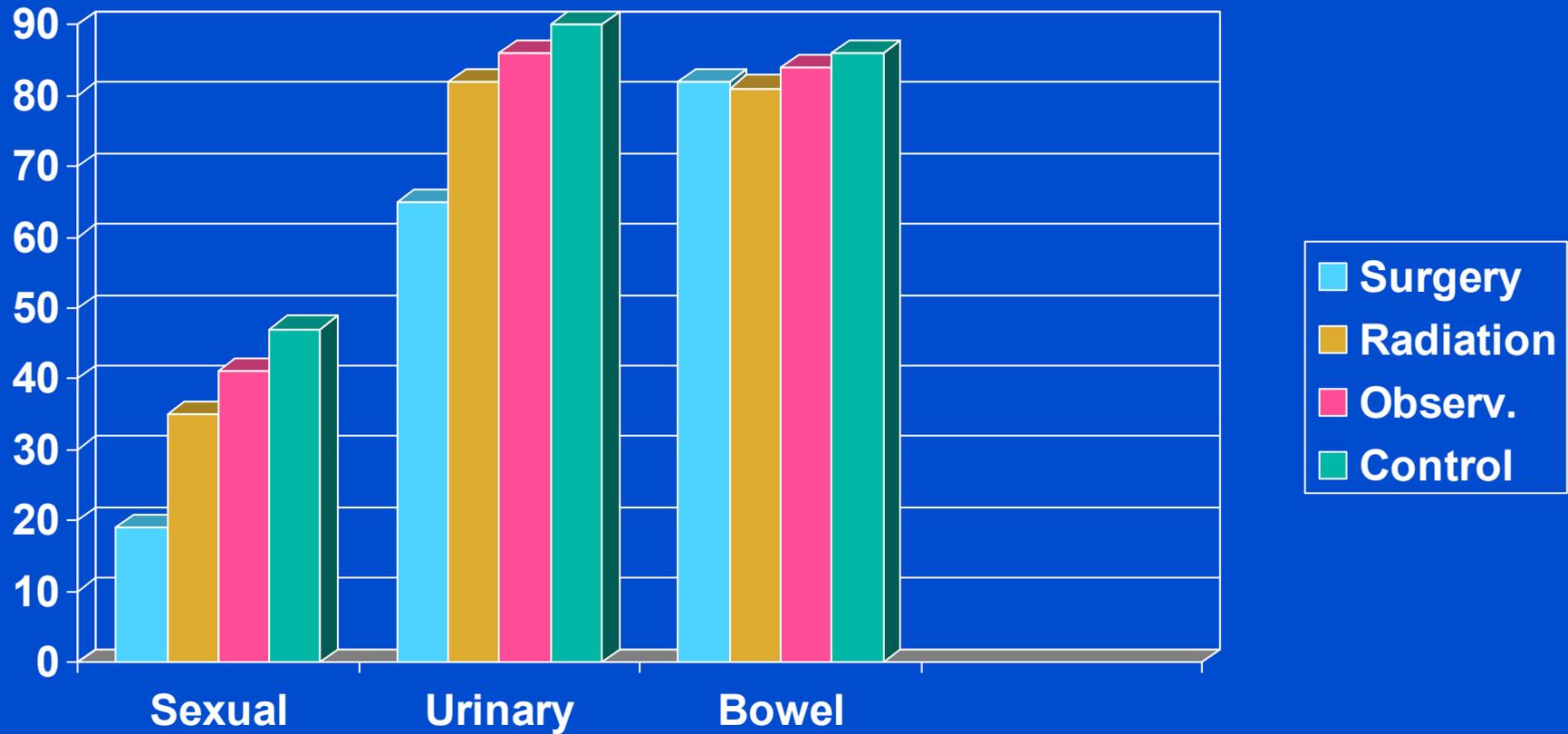
214 men with prostate cancer

- 98 radical prostatectomy
- 56 primary pelvic irradiation
- 60 observation alone

273 age/zip matched pts. without cancer

Litwin et al. (1995, JAMA)

Sexual, Urinary and Bowel Function



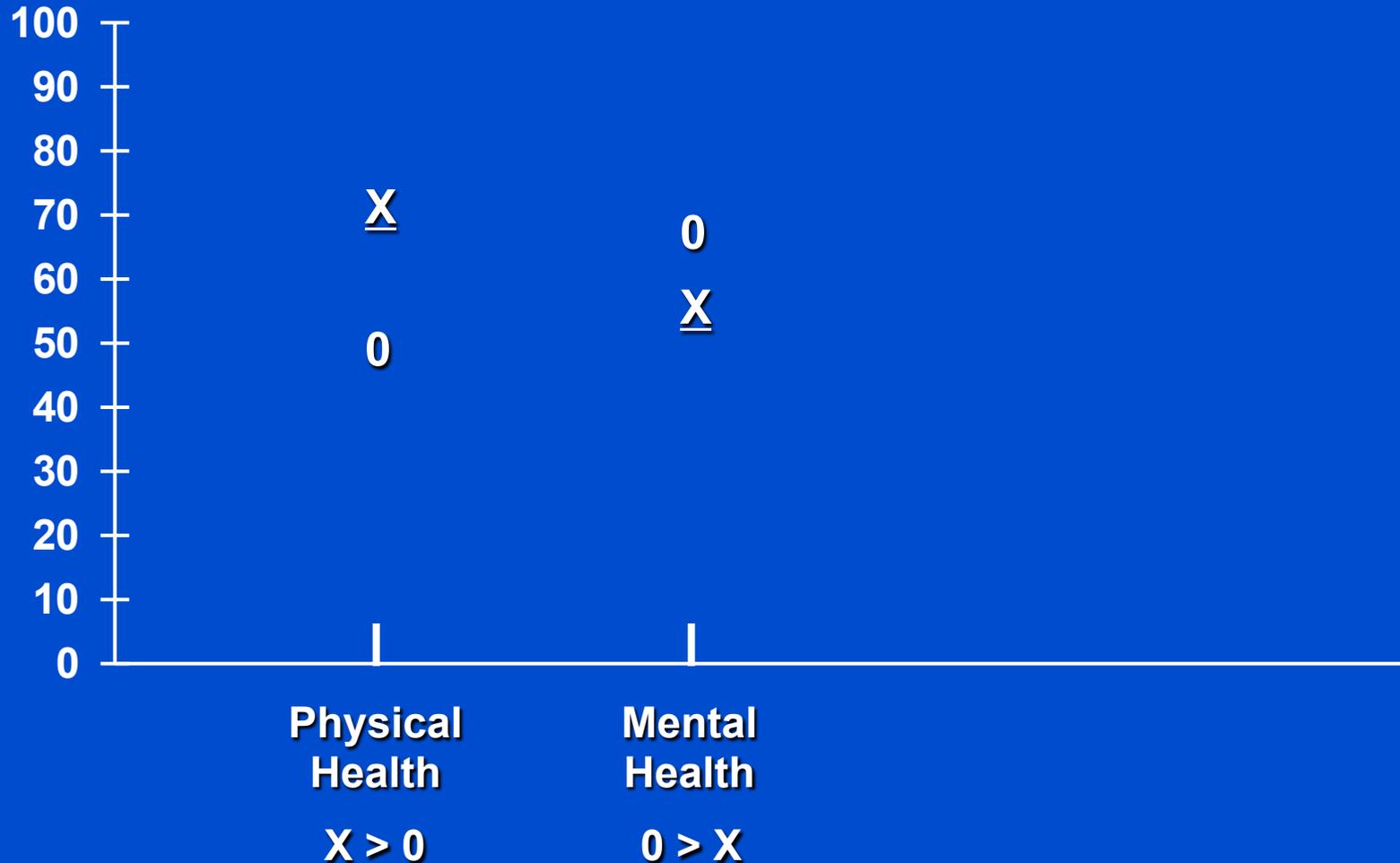
HRQOL Measures Helpful in Ensuring Access to Cost-Effective Care

Cost



Effectiveness

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

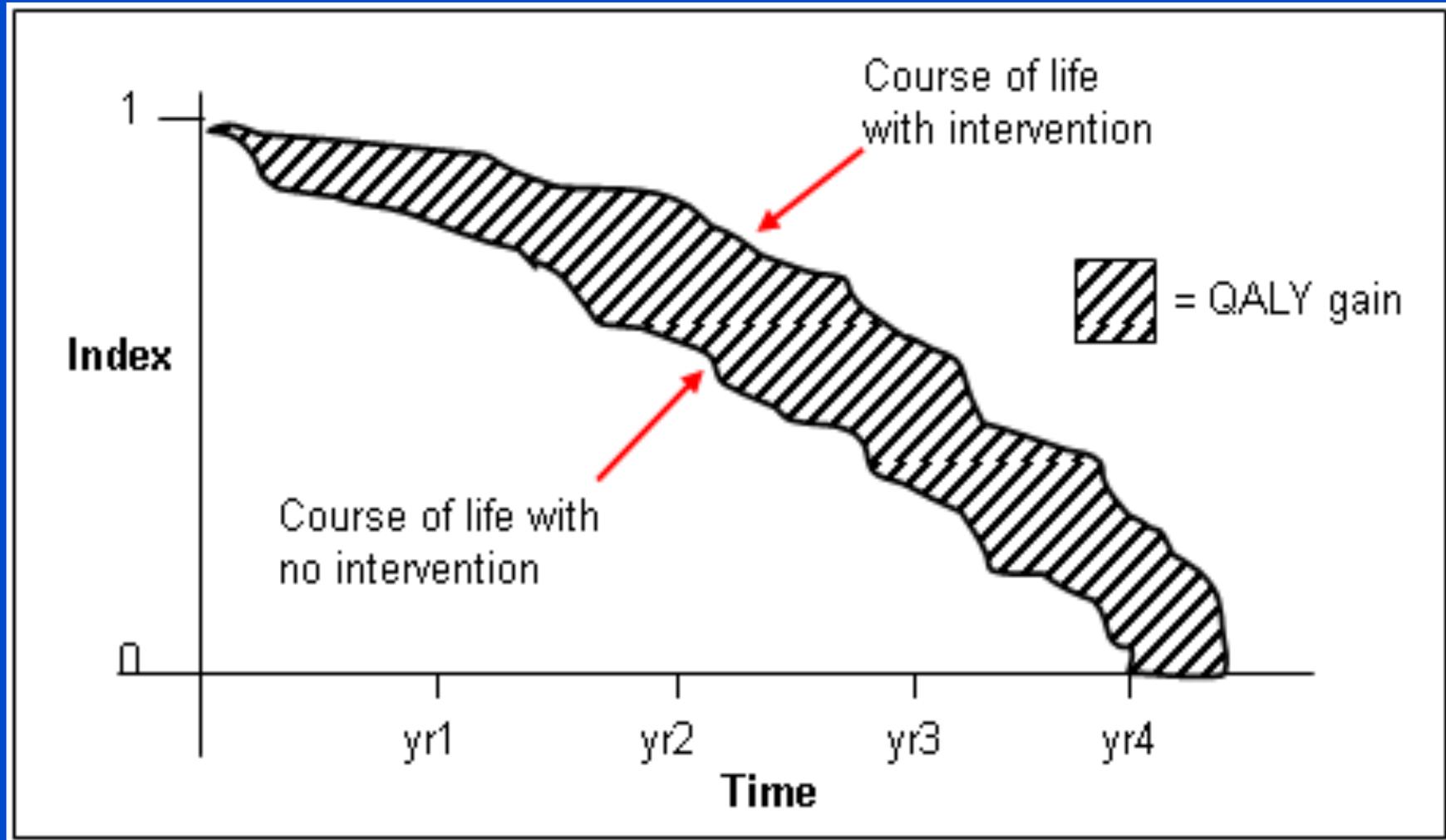


Do a Survival Analysis?

Marathoner and person in coma = 1.0



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



Health State 111111

Health state 111111

Your health does not limit you in **vigorous activities** (e.g. running, lifting heavy objects, participating in strenuous sports).

You have no problems with your work or other regular daily activities as a result of your **physical health or any emotional problems**.

Your health limits your **social activities** (like visiting friends or close relatives) a little or none of the time

You have no pain

You feel **tense or downhearted and low** a little or none of the time.

You have a lot of **energy** all of the time

Brazier et al. 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

Hot HRQOL Issues

Use of HRQOL in clinical practice

Item response theory and computerized adaptive testing

Minimally important differences (MID)

Use of HRQOL in labeling of drugs (FDA)

Questions?



For further information

<http://gim.med.ucla.edu/FacultyPages/Hays/>

<http://www.rand.org/health/surveys.html>

<http://www.chime.ucla.edu/measurement/measurement.htm>

<http://www.proqolid.org/>

www.sf-36.com

Quality of Life Research

An International Journal of Quality of Life Aspects of Treatment, Care and Rehabilitation - Official Journal of the International Society of Quality of Life Research

Editor-in-Chief: R. Hays

ISSN: 0962-9343 (print version)

ISSN: 1573-2649 (electronic version)

Journal no. 11136

Springer Netherlands



**Assessing Quality of Life in Clinical
Trials: Methods and Practice, 2nd Edition**

Peter Fayers and Ron Hays, Eds.

**Oxford University Press, 2005
464 pages**