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Session 4

General Introduction to Health-related Quality of life

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Outline

- ➊ **What is Health-related Quality of Life?**
- ➋ **How is it measured?**
- ➌ **Why is it important?**



Definitions

✓ Quality of Life

The term « Quality of Life », represents a broad range of dimensions of human experience ranging from those associated with the necessities of life such as food and shelter to those associated with achieving sense of fulfillment and personal happiness

(D. Patrick – P. Erickson)



Definitions

✓ Patient-reported Outcomes

The patient's report of a health condition and its treatment

Acquadro C, Berzon R, Dubois D, Kline Leidy N, Marquis P, Revicki D, Rothman M; PRO Harmonization Group. Incorporating the Patient's Perspective into Drug Development and Communication: An Ad Hoc Task Force Report of the Patient-Reported Outcomes (PRO) Harmonization Group Meeting at the Food and Drug Administration, February 16, 2001. Value in Health 2003; 6 (5): 22-31



Patient Outcomes Assessment Sources and Examples

Clinician - Reported

For example,
Global impressions
Observation & tests
of function

Physiological

For example,
FEV₁
HbA1c
Tumor size

Caregiver - Reported

For example,
Dependency

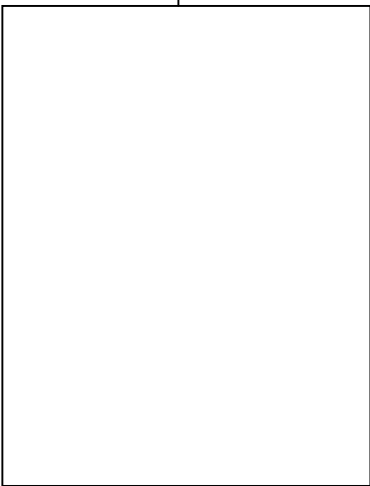
Patient - Reported

For example,
Functional status
Symptoms
HRQL

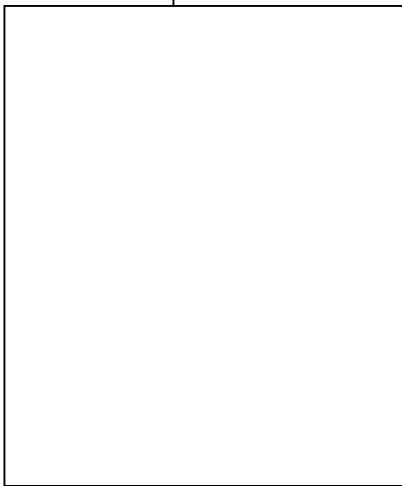


Patient Outcomes Assessment Sources and Examples

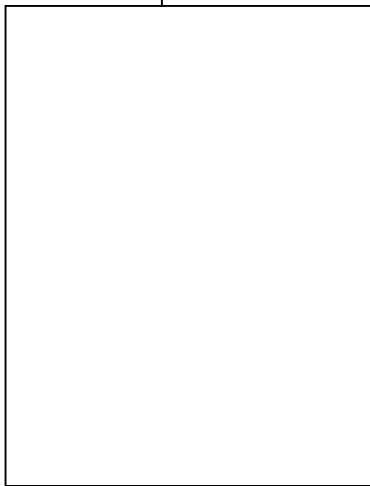
Clinician - Reported



Physiological



Caregiver - Reported



Patient - Reported

- Global Impression
- Functional status
- Well-being
- Symptoms
- HRQOL
- Satisfaction with TX
- Treatment adherence



Definitions

✓ Health-Related Quality of Life

One Type of PRO

Represents the patient's evaluation of the impact of a health condition and its treatment on relevant aspects of life



Health-related Quality of Life

Essential Elements

- ✓ Represents the patient's *evaluation* of the impact of a health condition and its treatment on relevant aspects of life
 - The evaluative component can be measured by
 - *severity*
 - *bothersomeness*
 - *importance or*
 - *satisfaction*



Health-related Quality of Life

Essential Elements

- ✓ Represents the patient's evaluation of the impact of a health condition and its treatment on *relevant aspects* of life
 - ▣ The relevant aspects of life are measured as domains, e.g.,
 - *physical*
 - *psychological*
 - *social*
 - *symptoms*



Health-related Quality of Life

Essential Elements

- ✓ Represents the patient's evaluation of the impact of a health condition and its treatment on *relevant aspects* of life
 - ▣ Domains are selected to be relevant to patients and their significant others, e.g.,
 - *focus groups*
 - *cognitive interviews*
 - *literature reviews*



What is a HRQL Instrument?

- ✓ A HRQL instrument is comprised of one or more self-reported items that reflect some underlying concept
- ✓ Items must provide good coverage of the concept
 - Comprehensiveness of coverage is a qualitative determination, but instrument should:
 - *Include* items that are relevant and important to the patient
 - *Exclude* items that are not relevant to the patient
 - *Not* be overly weighted toward less important concepts

*From: Patient Reported Outcomes Harmonization Meeting
Food and Drug Administration, Rockville, Maryland, March 1, 2002*



Taxonomy of Measures

❖ Descriptive questionnaires

- * Generic: a few

 - Duke, HAQ, MOS SF-36, NHP, SIP, WHOQOL

- * Specific: a lot

 - AQLQ, CLAUS, FACT, MLHF, ONYCHO, PC-QoL, SAQ, UQOL, etc...

❖ Preference-based measures

- * Generic:

 - Standard Gamble / Time Trade Off / Global Rating

 - Quality of Well-Being Scale

 - Multi-attribute system: *Health Utility Index – EuroQol*

- * Specific (*Multi-attribute Systems*)



Taxonomy of Measures

Advantages

Generic

- * Comprehensiveness
- * Comparison across interventions and conditions
- * Established psychometric properties
- * Some provide single number

Specific

- * Tailored to disease, condition, population
- * May be more acceptable to respondents
- * May be more responsive

Disadvantages

Specific

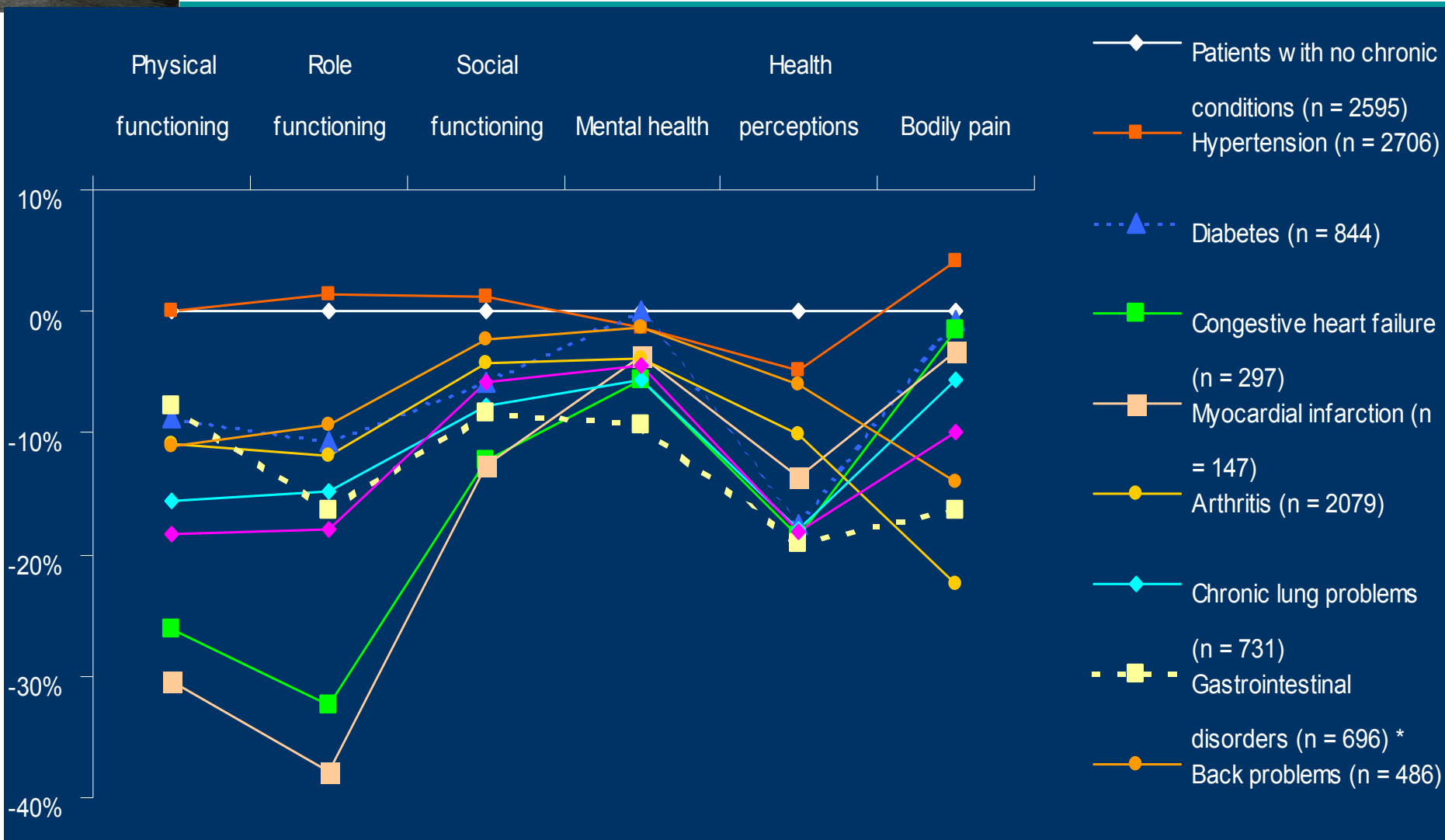
- * Comparisons across conditions and interventions may not be possible
- * Psychometric properties not established for some measures

Generic

- * May not have focus of interest
- * Some are lengthy
- * May be not responsive enough



Taxonomy of Measures





Life Cycle of a HRQL Instrument

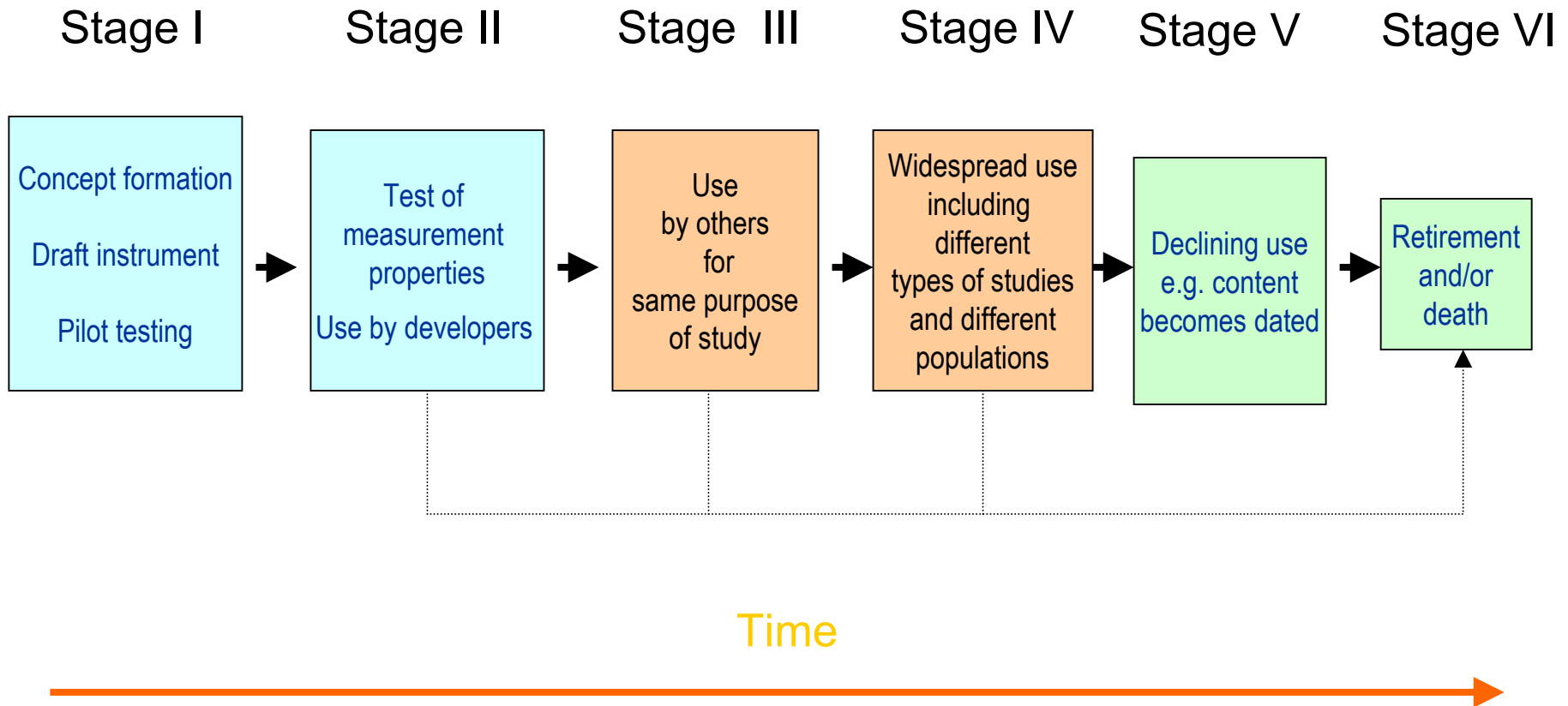


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Why is HRQL (and other PROs) Important?

The Patient's Perspective is ...

- ✓ A **unique** indicator of the impact of disease
- ✓ **Essential** for evaluating treatment efficacy
- ✓ **Useful** for interpreting clinical outcomes
- ✓ A **key** element in treatment **decision-making**



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Why is HRQL (and other PROs) Important?

PROs May be the Sole Indicator of Disease Activity

■ Patient-reported symptoms

rhinitis, migraine, functional gastrointestinal disorders, menopause, pain

■ Patient-reported functioning

sexual dysfunction, insomnia



Why is HRQL (and other PROs) Important?

PROs is Key Indicator of Disease Activity

■ Patient-reported symptoms

COPD, asthma, psoriasis, atopic dermatitis, benign prostatic hypertrophy

■ Patient-reported functioning

Parkinson's disease, MS, arthritis, cancer, stroke, traumatic brain injury



Why is HRQL (and other PROs) Important?

PROs Supplement Traditional Indicators

- PROs and traditional markers of disease activity are related, but not perfectly correlated
 - daily functioning & FEV-1
 - $r = 0.10$ to 0.30
 - shared variance = 9%
- Thus, the two indicators are complementary



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Why is HRQL (and other PROs) Important?

PROs are essential endpoints in clinical trials when...

- ❖ the patient's self-report is the primary or sole indicator of disease activity
- ❖ the treatment has a small impact on survival
- ❖ it is important to ensure that treatments do not adversely affect patients' lives
- ❖ the study is an equivalence trial where the drug under study has PRO benefits



Why is HRQL (and other PROs) Important?

PROs Provide Supplemental Information

- In advanced breast cancer,
 - continuous chemotherapy led to better HRQL than intermittent chemotherapy (Coates et al., 1987)
 - standard-dose chemotherapy led to better HRQL than half-dose chemotherapy (Tannock et al., 1988)
- In soft tissue sarcoma of the limbs,
 - amputation of the affected limb led to better HRQL than limb-sparing surgery plus adjuvant therapy (Sugarbaker et al., 1982)



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Why is HRQL (and other PROs) Important?

Interpreting Clinical Outcomes in Terms of Patient Benefit

- Improvement in FEV-1
 - Effect on daily activity?
- Reduction in seizure frequency
 - Effect on social functioning & well-being?



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Why is HRQL (and other PROs) Important?

PROs in Clinical-Decision Making

● Treatment Guidelines

- Oncology - ASCO

- Respiratory Disease

- Practice Guidelines - COPD, Asthma

- Gastrointestinal Disease - IBS

- Practice Guidelines - GERD

- Cardiovascular Disease

- Vascular Surgeons; Atrial Fibrillation

- Urology - BPH, urinary incontinence

- HIV- DHHS Panel Guidelines



Sources of Information

Source

- Literature search
(Medline, Embase, etc)
- Published reviews
- QOLID
- Ongoing research
- Network of experts

Information

- ⇒ Beware false HRQL studies
(8000 references)
- ⇒ Review of major disease
areas and key generic
instruments
- ⇒ Existing published
questionnaires (around 600)
- ⇒ Questionnaires under
development
- ⇒ Existing questionnaires not yet
published