

Patient Reported Outcomes (PROs) Tools for Measurement of Health Related Quality of Life

David Victorson, Ph.D.

Assistant Professor

Department of Medical Social Sciences

Northwestern University Feinberg School of Medicine

D-victorson@northwestern.edu

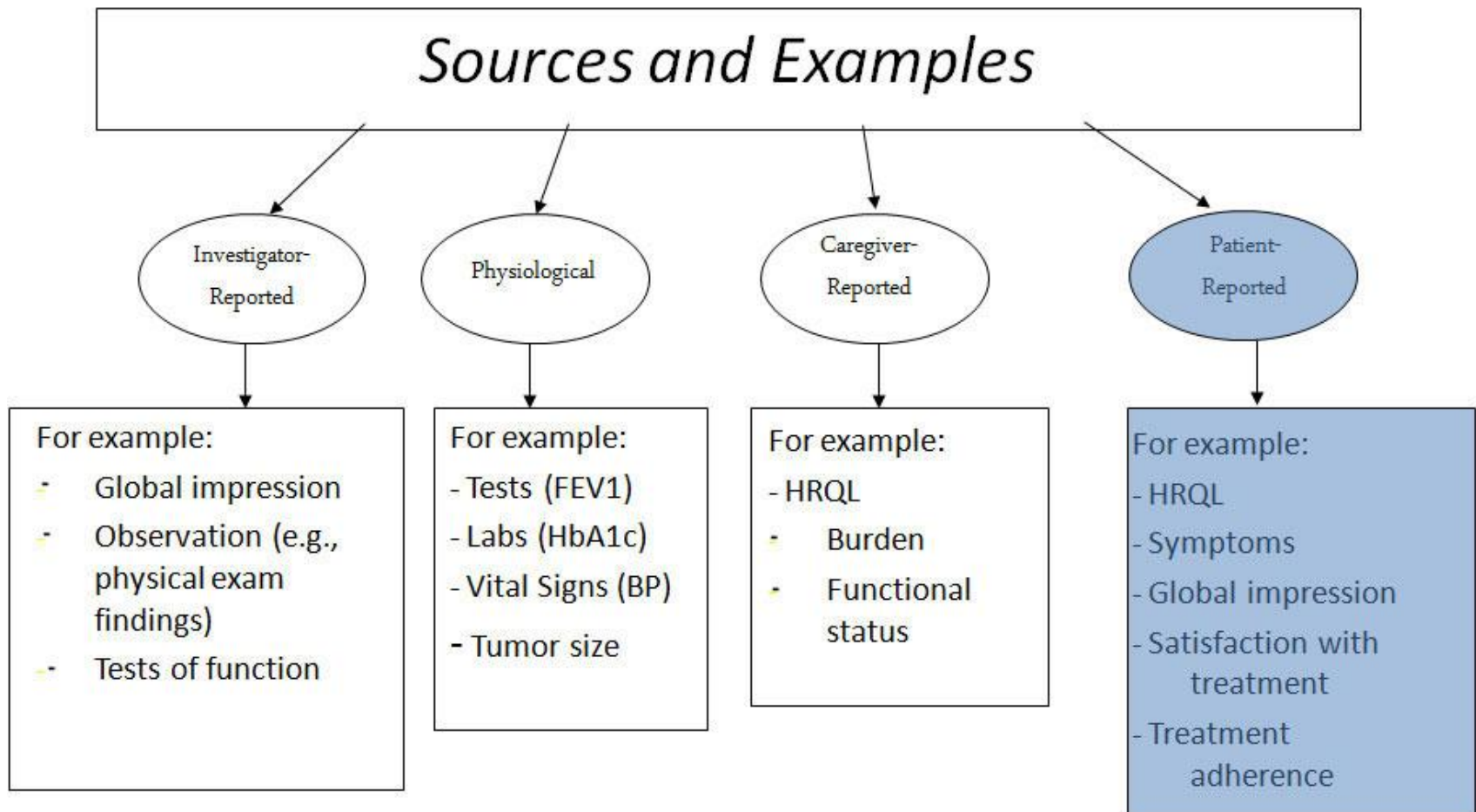


Overview

- What are PROs?
- Why PROs are Important?
- Some Challenges to PROs
- Best Practices for Creating PROs
- PRO Conceptual Models and Frameworks



Patient Outcomes Assessment



What are Patient-Reported Outcomes (PROs)?

- A **patient-reported outcome (PRO)** is the measurement of any aspect of a patient's health status that comes directly from the patient (i.e., without the interpretation of the patient's responses by a physician or anyone else).



What are Patient-Reported Outcomes (PROs)?

- Administration of PROs may be via paper and pencil forms, digital capture (internet, handheld device), or interviewer-administered questionnaires.
- The most commonly used PRO questionnaires assess one of the following:
 - Symptoms (impairments)
 - Functioning (disability)
 - Wellness (health)
 - Health-related quality of life (HRQL)
- **Generic** questionnaires are designed to be used in any population and are meant to cover a broad aspect of the concept being measured (e.g SF-36, PROMIS Global Health).
- **Targeted**, or disease-specific questionnaires are designed to assess those concerns of most importance for a given population.



Why PROs are Important?

- Unique perspective on treatment effectiveness.
 - Physiological assessments often do not reflect how a patient functions or feels (e.g., FEV1)
- May be more reliable than informal interview
- Some treatment effects are known only to the patient
 - patient's symptoms (e.g., fatigue, depression)
 - how well the patient feels
 - how well the patient functions
 - how the patient perceives care/treatment



PROs are Important Endpoints

- PROs are used in clinical trials to
 - Describe patients & disease severity
 - Trial eligibility
 - Treatment effects
 - Converge with other outcomes
 - Risk/Benefit Evaluation



Some Challenges for PROs

- Development Limitations
- Unreliable/Unresponsive
- Poor validity
- Sensitivity/Specificity
- Meaningfulness of Scores
- Single vs. Multiple Item?
- Best recall period?
- Response options?
- Mode of administration?



Best Practices for Creating PROs



Consulting the Literature

- Before you set out to develop a new tool, first check what's available
- If nothing exists or what's available could be improved upon,
 - Conduct a thorough review of the literature to identify signs, symptoms issues related to your area of interest, including available PRO measures



Consulting Experts

- Open-ended survey or interview to known experts in a particular area to inquire about:
 - PROs typically used and why
 - Most important and frequent symptoms, impact on HRQL due to the condition
 - Issues that are most challenging for patients based on experts' clinical experience
- Rate/rank existing PRO items for relevance, clarity, ease of understanding, etc.



Consulting Target Audience (e.g., Patients)

- **Focus Groups**

- Well-established exploratory qualitative research approach to elicit and direct discussion on topics related to one's experience with a given phenomenon.
- Draw upon the collective experience, wisdom and group dynamics of individual members coming together around a common goal
- Efficient and economical approach that can yield rich data and serve as a starting point for more in-depth follow up work.



Consulting Target Audience (e.g., Patients)

- **Individual Interviews**

- Allow for greater and more in depth exploration of concepts and issues.
- Afford an opportunity to probe and explore sensitive topics that may have been moderated or withheld in focus groups because of the social influences or stigma.
- Corroborate previous exploratory findings from other sources.



Identifying Core Concepts



Triangulation of
Data Sources



Identification of Core Concepts

- Examine data sources systematically
 - Theoretically-based qualitative analysis approach
 - Constantly comparing with one another
 - Multi-level Coding & Theme creation
- Inter-rater agreement
- Data saturation
- Begin to organize concepts & identify which ones are of interest to be measured



Conceptual Model

- Theoretical representation, which defines the concepts of interest, their interfaces and possible determinants.
- Visual representation of the complex interrelationships among variables and can assist in the selection of target endpoints.
- Generally the basis for a new patient reported outcome measure.



Modified Wilson & Cleary HRQL Model for Dyspnea and Functional Limitations

Concepts of Dyspnea and Functional Limitations

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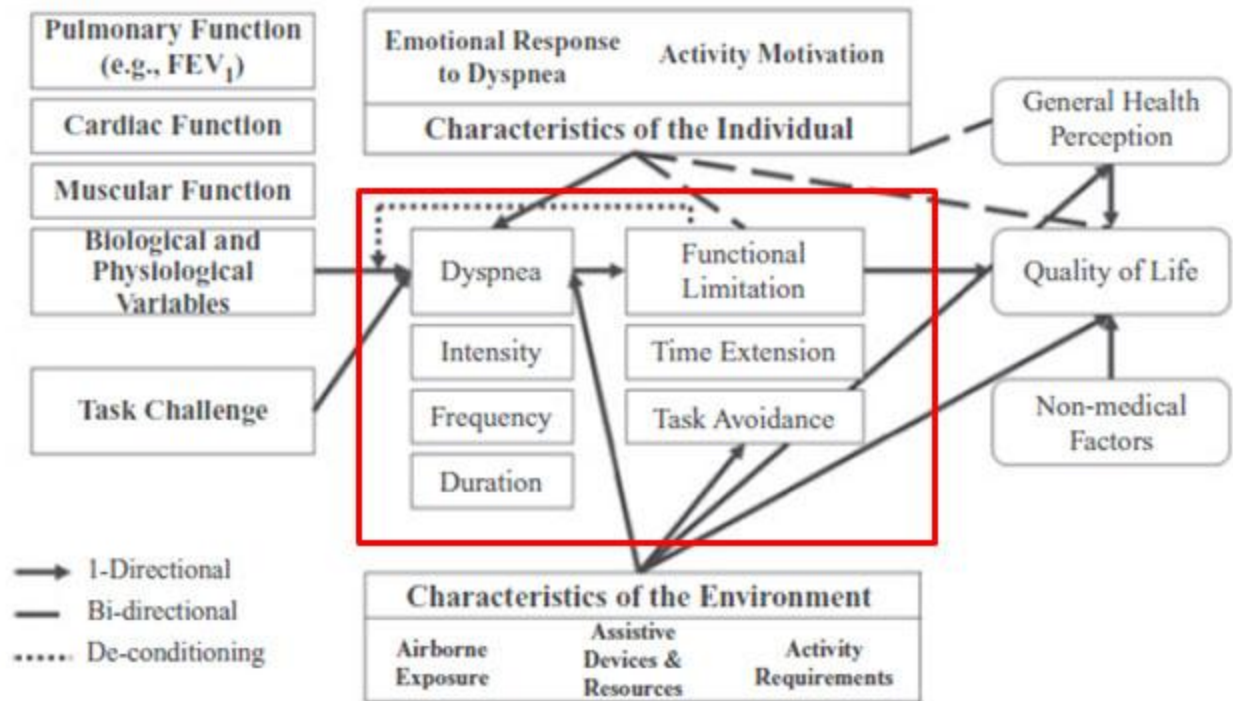


Figure 2 Dyspnea-specific conceptual model for chronic obstructive pulmonary disease.

Wilson I.B. and Cleary P.D. (1995) Linking Clinical Variables with Health-related Quality of Life. JAMA 273(1), 59-65.

Victorson, D, Anton, S, Hamilton, A, Yount, S, Cella, D (2009). A Conceptual Model of the Experience of Dyspnea and Functional Limitations in Chronic Obstructive Pulmonary Disease. Value in Health, 12(6), 1018-25.



Writing Items

- 3 Elements of an Item

1. Context

2. Stem

3. Response

Please indicate how true each statement has been for you during the past 7 days.

I have nausea.

0 Not at all

1 A little bit

2 Somewhat

3 Quite a bit

4 Very much



Item Writing Guidelines:
Context is Relevant to Concept

- Choose an item context that is relevant to the concept being measured (e.g., past 24 hours, past 7 days, past 2 weeks, past month)
- This can be identified from the literature and/or explored during focus groups or individual interviews



Item Writing Guidelines:
Use Simple, Universal Language

- Write clear, unambiguous items
- Use language that is simple and appropriate for the target population
- Avoid colloquialisms and activities that might not be familiar across different age groups, ethnicities, cultures etc.



Item Writing Guidelines:
Make Items Specific

- Ask about specific versus general
 - I enjoy sports.
 - I enjoy watching college football.
- Avoid double-barreled questions
 - Do you approve or disapprove of abortion in cases of incest or threats to the mother's health?



Item Writing Guidelines:

Pay Attention to Phrasing

- Avoid negatively phrased items
- Write items that require little cognitive processing.
- Try to keep grammar to simple past or present tense
 - I don't have symptoms of nausea (Strongly Disagree, Slightly Disagree, Disagree, etc.)
 - I have symptoms of nausea



Item Writing Guidelines:

Key Response Options to Type of Item

- **Opinions**
 - Very important → Not at all important
 - Strongly agree → Strongly disagree
- **Knowledge**
 - True/false
 - Very familiar → Not at all familiar
- **Frequency of Events or Behaviors**
 - Times per day, per week, per month etc.
 - Always/sometimes/never
- **Ratings**
 - Excellent → poor
 - Better/worse/about the same



Expert Item Review I

- Expert Review
 - Review items and rate each candidate item according to relevance and prevalence.
 - Items deemed as irrelevant or low-relevant set aside for further review.
 - Document all decisions on item exclusion, inclusion, or revision to provide an historical account of all items.



Patient Item Review I

– Purpose of Cognitive Interviews

- To ensure that item content, response scales and instructions are understood as intended
 - Comprehension of the question
 - Processes used to retrieve relevant information from memory
 - Decision processes, such as motivation and social desirability
 - Response processes

– Conduct of Cognitive Interviews

- Paraphrase the item in their own words
- Define terminology used in the items
- Describe any lack of clarity or confusion about the appropriateness of their answer
- Describe how confident they are about their ability to provide an accurate answer to the item
- Describe how they arrived at their answer to items that request a numerical or rating response.



Item Changes & Modifications

- Team members review items and modify as needed



Expert & Patient Item Review II

- Second review by experts and patients of modified items

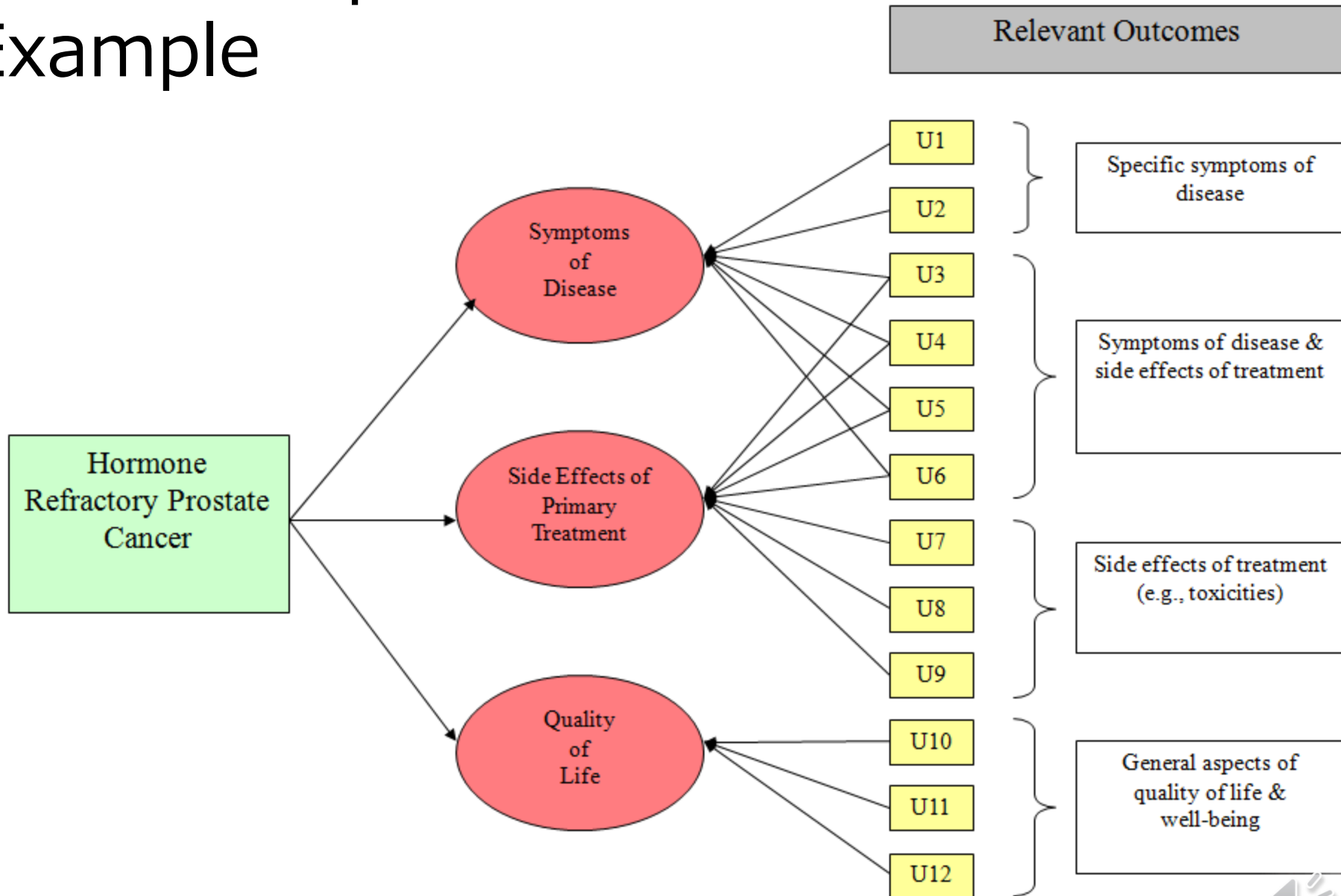


Conceptual Framework

- Illustrates the anticipated associations between the items within a PRO tool and their respective domains.
- Clearly identifies the concepts important to patients and how they should be measured and represents the goals of treatment as “concepts” important in a specific disease and treatment context with a clear description of treatment benefit.



PRO Conceptual Framework Example



In Sum

- What PROs are
- Why PROs are Important
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Thank you

