

Patient-Reported Outcomes in Clinical Research

Kevin P. Weinfurt, PhD

Introduction to the Principles and Practice of Clinical Research

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Overview

1. Why measure patient-reported health status?
2. Different types of PROs
3. Development and evaluation of PROs
4. “New” methods using Item Response Theory
5. Interpreting scores on PRO measures

Why measure patient- reported health status?

How do we assess benefit?

Copies of virus in blood Tumor size

Blood pressure Peak VO2

For endpoints to inform decisions, they must matter to patients, clinicians, and payers

Peak V02

?

$r = .53$

Overactive Bladder Syndrome

Pad weight to measure leakage volume

What is a meaningful reduction in volume?

Treatment Benefit

Patient-Reported Outcome (PRO)

A measurement based on a report that comes directly from the patient (i.e., study subject) about the status of a patient's health condition

without amendment or interpretation of the patient's response by a clinician or anyone else

PROMIS® Fatigue Measure

Different Types of PROs

1. Dilution of effects of biological interventions
2. Correlation between successive boxes decreases

Development and Evaluation of PRO Measures

Determine what PRO concept we want to measure and why

Collect qualitative data to understand meaning of the PRO concept

Write items you think will measure the concept

Test items for understanding (cognitive interviews)

Administer items to a large sample of people

Use psychometric (statistical) analyses to see how well items are working and develop

Evaluate the reliability and validity of the measure

Types of Validity

Content

Face

Construct

Convergent/discriminant

Known groups Predictive

Responsiveness

Convergent Validity: PROMIS Depression Domain

If I have not changed, I should get the same score . . .

“New” Methods Using Item Response Theory

Chart

Traditional Off-the-Shelf PRO Measure

Everyone must complete the same items.

All items are necessary to obtain a score.

Score might not be on the same metric as other measures of the same thing.

An item bank is a large collection of items measuring a single domain

Any and all items can be used to provide a score for that domain.

Dynamic, not fixed.

Physical Functioning Item Bank

Using a Traditional Off-the-Shelf PRO Measure

Using a PRO Item Bank

Item Bank

Item Bank

Fatigue Item Bank

Different Sites, Different Measures

Chart

Examples of PRO Resources Based on Item Banks

Adult and pediatric item banks measuring domains relevant across chronic diseases

Freely available www.nihpromis.org

Multidimensional set of brief measures assessing cognitive, emotional, motor and sensory function from ages 3-85

Freely available www.nihtoolbox.org

Core set of instruments for use in chronic neurological conditions (supplemental set for specific diseases, patient subgroups)

Freely available www.neuroqol.org

Differential Item Functioning

In the past 7 days, did you cry?

Differential Item Functioning

Item Response Theory

Item Response Theory

Item Response Theory

Differential Item Functioning

In the past 7 days, did you cry?

Interpreting Scores on PRO Measures

Exercise vs Usual care

Review

1. Why measure patient-reported health status?
2. Different types of PROs
3. Development and evaluation of PROs
4. “New” methods using IRT
5. Interpreting scores on PRO measures

Questions