# Selecting a Patient-Reported Outcome Measure:
## Minimum Standards from the International Society for Quality of Life Research

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*Funded by the Patient-Centered Outcomes Research Institute and Genentech*

PROTEUS
Patient-Reported Outcomes Tools: Engaging Users and Stakeholders

# The PROTEUS Consortium

## Patient-Reported Outcome Tools: Engaging Users & Stakeholders

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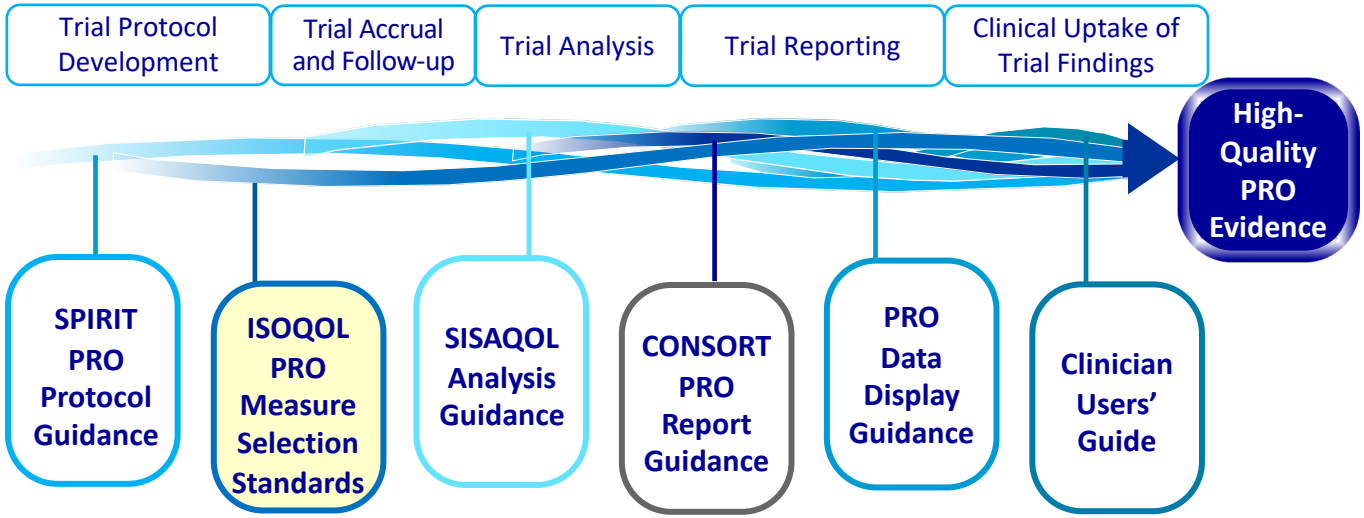
TheProteusConsortium.org

# Overview of Presentations

Introduction to PROs and PROTEUS

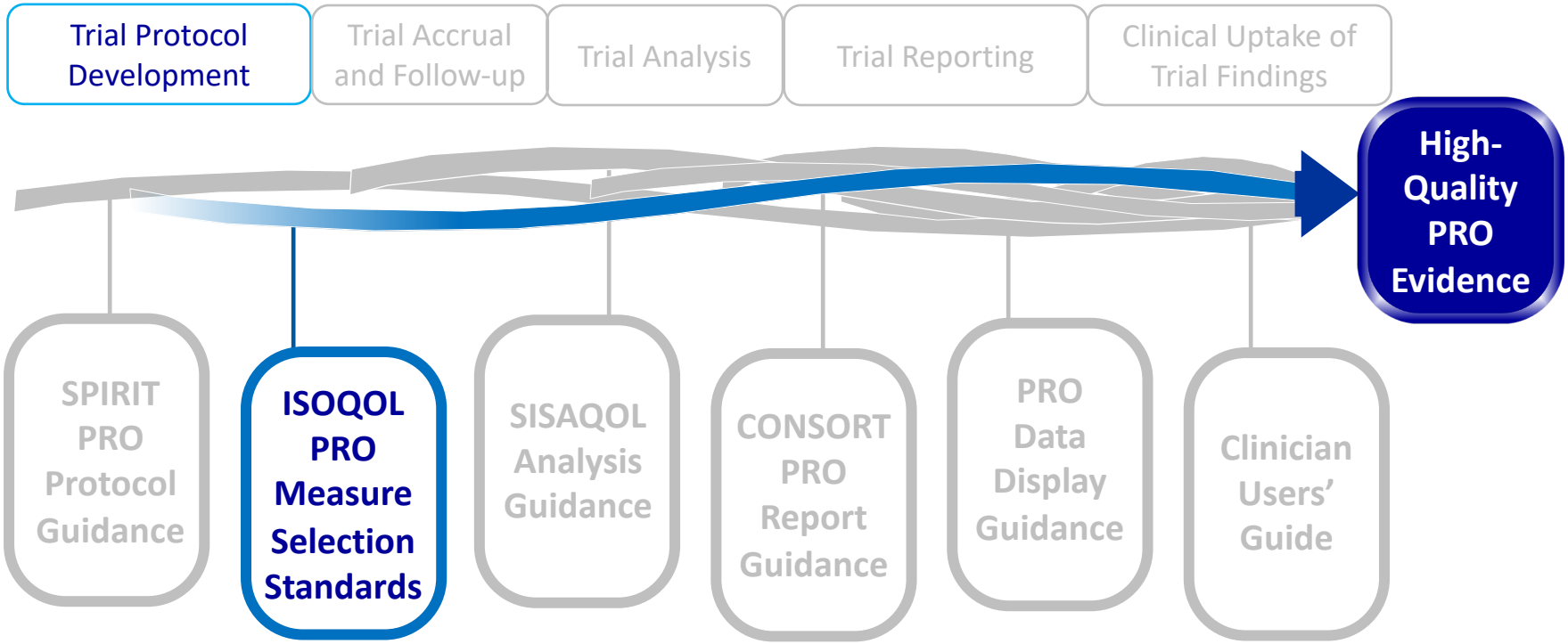
Introduction to the PROTEUS Tools




Overview of Tool Recommendations

How to Apply the Tools

# Minimum Standards for PRO Measures

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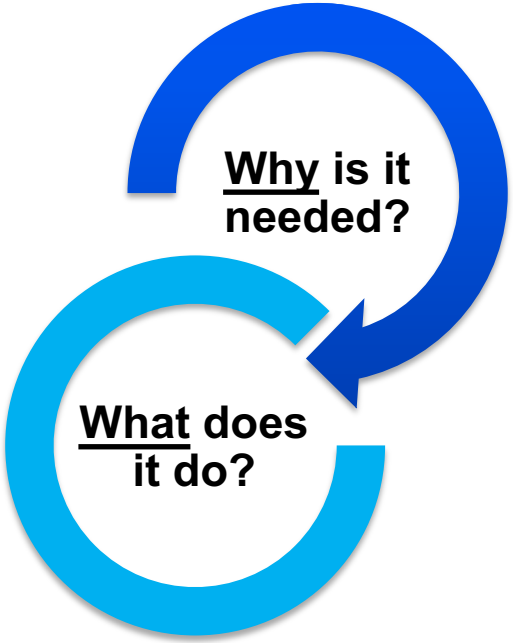
# Measuring PROs Effectively




PROs must be measured in a valid, standardized way using appropriate tools and methods to ensure valid conclusions

Provides guidance for selecting PRO measures for use in patient-centered and comparative effectiveness research

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# PRO Measure Selection Standards

Qual Life Res (2013) 22:1889–1905
DOI 10.1007/s11136-012-0344-y

## ISOQOL recommends minimum standards for patient-reported outcome measures used in patient-centered outcomes and comparative effectiveness research

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*Reeve et al, Qual Life Res 2013, 22, 1889-1905*

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ISOQOL
International Society for Quality of Life Research

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# Why We Need PRO Measure Selection Standards

- An essential aspect of patient-centered outcomes research (PCOR) and comparative effectiveness research (CER) is integration of patient perspectives and experiences about their health with clinical and biological data to evaluate the safety and effectiveness of interventions
- Clinical trials are one kind PCOR/CER; the ISOQOL standards address PCOR/CER more broadly, but we will refer to clinical trials in this presentation

# Why We Need PRO Measure Selection Standards

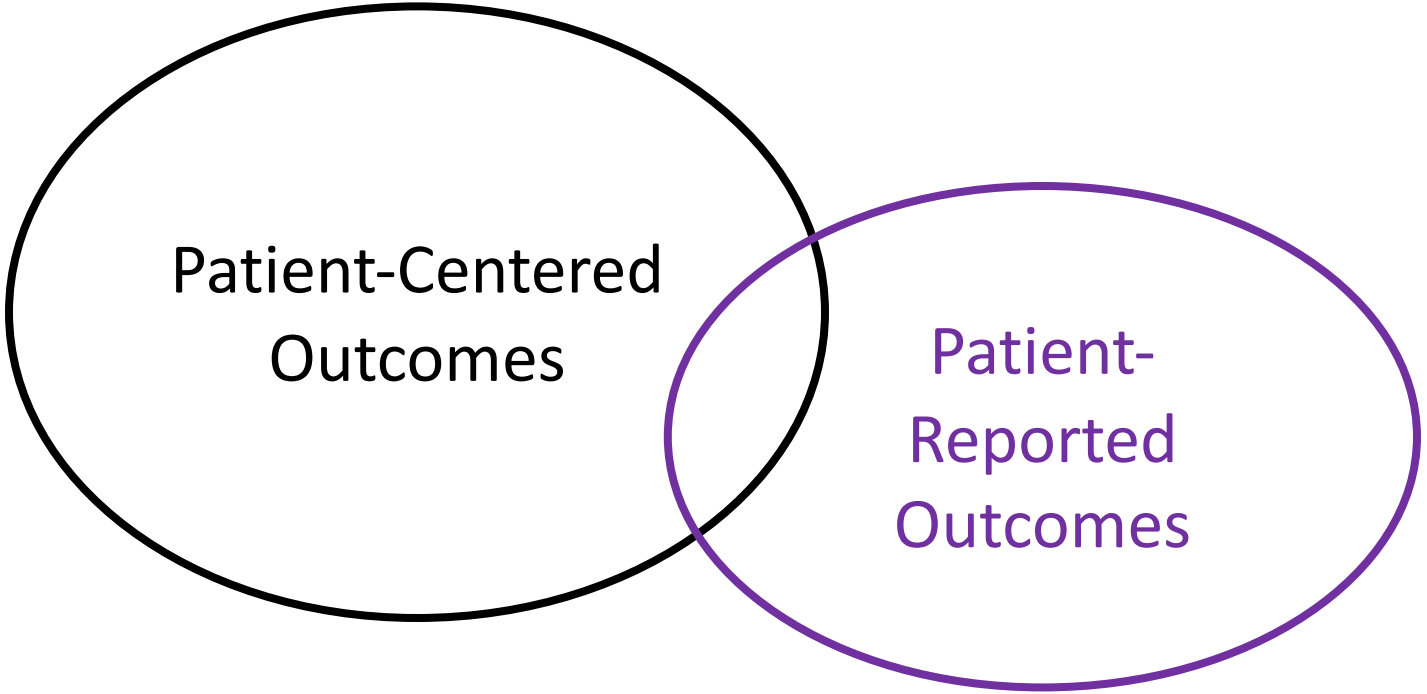
- Widely accepted that patient's report is the best source of information about what they are experiencing
- Challenge for PCOR and CER is how to best capture patient-reported data to inform decision making in healthcare delivery, research, and policy settings
- To draw valid research conclusions regarding patient-centered outcomes, PROs should be measured in a standardized way using appropriate methods

# Why We Need PRO Measure Selection Standards

- A PRO is the measurement of any aspect of a patient's health that comes directly from them without interpretation by another[1]
- PROs can be symptoms (e.g. pain, anxiety, nausea, fatigue), aspects of functioning (e.g. role, physical, emotional, social) and multidimensional constructs (e.g. health-related quality of life)
- A PRO measure is the questionnaire, index, checklist, instrument, or tool[2], along with the algorithm used to score patient responses into summary scores for analysis and reporting

1. *https://www.fda.gov/media/77832/download*
2. *Mayo 2015 ISOQOL Dictionary of Quality of Life and Health Outcomes Measurement (p. 85). Kindle Edition*

# Why We Need PRO Measure Selection Standards

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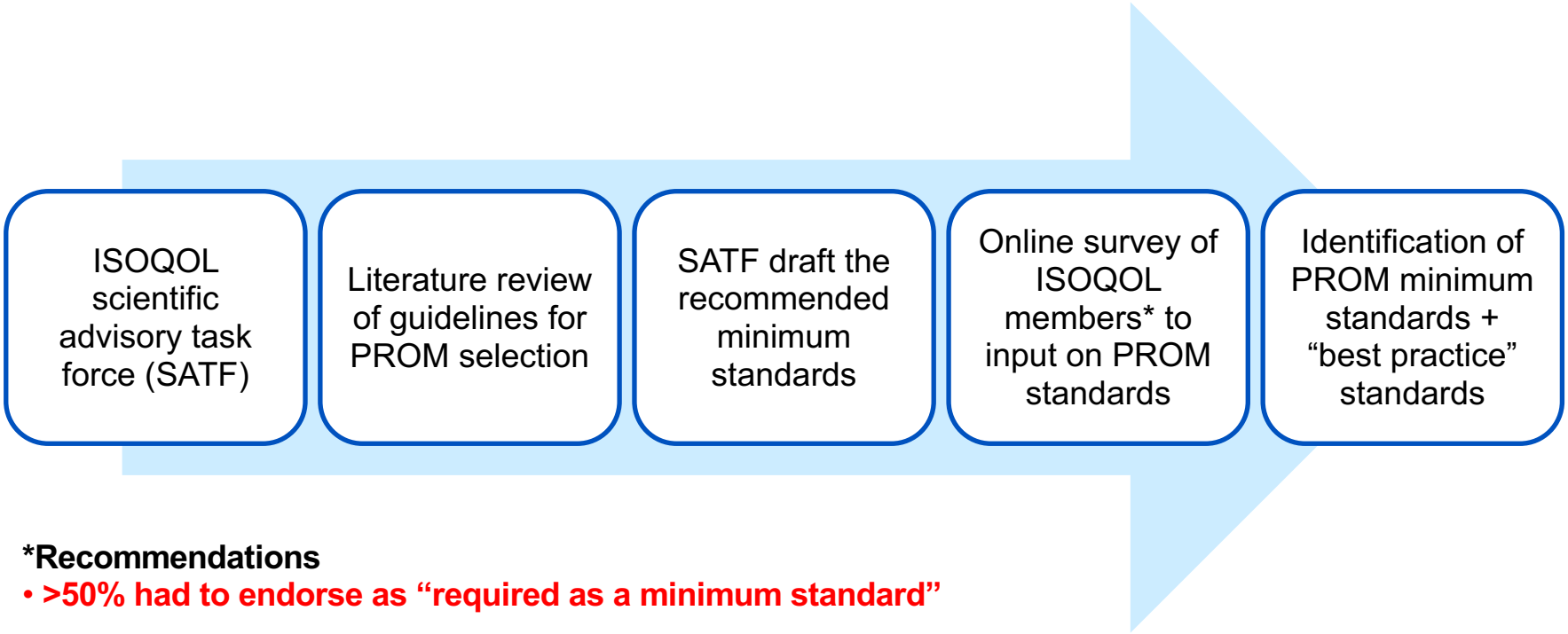
# Objective of Resource

- To develop PRO measure minimum standards for the design and selection of a PRO measure for use in PCOR and CER
- The standards represent the minimum criteria required for a PRO measure to be judged suitable for a PCOR or CER study
- These standards are intended to promote the appropriate use of PRO measures in PCOR and CER, which in turn can improve the effectiveness and efficiency of healthcare delivery

# Methods for Resource Development




***Recommendations**

- **>50% had to endorse as "required as a minimum standard"**

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# Overview of PRO Measure Selection Standards

- Conceptual and measurement model
  - Concepts included and how organized into measurement model
  - Intended population
- Reliability
- Validity
  - Content
  - Construct
  - Responsiveness
- Interpretability of scores
- Translations
- Patient and investigator burden

# Conceptual and Measurement Model

A PRO measure should have documentation:

1) defining and describing the concept(s) included and the intended population(s) for use; and

2) how the concept(s) are organized into a measurement model, including evidence for the dimensionality of the measure, how items relate to each measured concept, and the relationship among concepts included in the PRO measure.

# Reliability

The reliability of a PRO measure should preferably be at or above 0.70 for group level comparisons, but may be lower if appropriately justified.

Reliability for a multi-item unidimensional scale can be estimated using a variety of methods including internal consistency reliability, test-retest reliability, or item response theory. Each method should be justified.

# Content Validity

A PRO measure should have evidence supporting its content validity, including evidence that patients and experts consider the content of the PRO measure relevant and comprehensive for the concept, population, and aim of the measurement application.

This includes documentation of:

1) qualitative and/or quantitative methods used to solicit and confirm attributes (i.e., concepts measured by the items) of the PRO relevant to the measurement application;
2) the characteristics of participants included in the evaluation (e.g., race/ethnicity, culture, age, gender, socio-economic status, literacy level) with an emphasis on similarities or differences with respect to the target population; and
3) justification for the recall period for the measurement application.

# Construct Validity

A PRO measure should have evidence supporting its construct validity, including documentation of empirical findings that support predefined hypotheses on the expected associations among measures similar or dissimilar to the measured PRO.

# Responsiveness

A PRO measure for use in longitudinal research study should have evidence of responsiveness, including empirical evidence of changes in scores consistent with predefined hypotheses regarding changes in the measured PRO in the target population for the research application.

# Interpretability of Scores

A PRO measure should have documentation to support interpretation of scores, including what low and high scores represent for the measured concept.

# Translation of the PRO Measure

A PRO measure translated to one or more languages should have documentation of the methods used to translate and evaluate the PRO measure in each language. Studies should at least include evidence from qualitative methods (e.g., cognitive testing) to evaluate the translations.

# Patient and Investigator Burden

A PRO measure must not be overly burdensome for patients or investigators:

- length of the PRO measure should be considered in the context of other PRO measures included in the assessment,
- frequency of PRO data collection,
- literacy demand of the items in the PRO measure should usually be at a 6th grade education level or lower (i.e., 12 year old or lower); however, it should be appropriately justified for the context of the proposed application.

# Recap

- Minimum standards for PRO measure selection for use in PCOR and CER include:
  - Conceptual and measurement model
  - Reliability
  - Validity (Content and Construct)
  - Responsiveness
  - Interpretability of scores
  - Translations
  - Patient and Investigator Burden
- Minimum standards should be demonstrated in same/similar populations as the target study population and in similar context when possible
- If a PRO measure does not meet these criteria, it is considered NOT suitable for a PCOR or CER study

# Further Reading

Reeve BB, Wyrwich KW, Wu AW, et al. (2013) ISOQOL recommends minimum standards for patient-reported outcome measures used in patient-centered outcomes and comparative effectiveness research. *Qual Life Res*; 22:1889–1905.

*Crossnohere NL, Brundage M, Calvert MJ, King M, Reeve BB, Thorner E, Wu AW, Snyder C. (2020) International guidance on the selection of patient-reported outcome measures in clinical trials: A review. *Qual Life Res*. 14 Sept [Epub ahead of print].

** This paper reviews alternative guidance documents regarding selecting a PRO measure and compares the recommendations to the ISOQOL Minimum Standards.*