

PATIENT REPORTED OUTCOMES 101

RAPID Paclitaxel Pathways



Jessica P Simons, MD MPH,
on behalf of the Patient Preference Science Working Group

PATIENT REPORTED OUTCOMES

PROs or PROMs - any report of the status of a patient's health condition that *comes directly from the patient*, without interpretation of the patient's response by a clinician or anyone else

Structured versus unstructured

Examples:

- Health-related quality of life (HRQOL)
- Symptoms
- Function
- Satisfaction with care or symptoms
- Adherence to prescribed medications or other therapy
- Perceived value of treatment

1. FDA Guidance for Industry. Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. 2009. Available at: <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims>. Accessed 12.03.19
2. Calvert M, Blazeby J, Altman DG, et al. Reporting of patient-reported outcomes in randomized trials: the CONSORT PRO extension. JAMA 2013;309:814–822. PMID: 23443445.
3. Patient Reported Outcomes. NIH Collaboratory. Living Textbook of Pragmatic Clinical Trials. Available at: <https://rethinkingclinicaltrials.org/resources/patient-reported-outcomes-3/>. Accessed 12.03.19



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Typically assess the effect of illness on patient perceptions in multiple domains of quality of life:

- Physical functioning
- Mental functioning
- Emotional functioning
- Social functioning

1. FDA Guidance for Industry. Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. 2009. Available at: <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims>. Accessed 12.03.19
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PATIENT REPORTED OUTCOMES

There are general health-related and disease-specific QoL instruments

Disease-specific instruments are designed for PAD and IC, but not for CLTI

The relative performance of PAD-specific instruments for IC and for CLTI has not been extensively studied.

Typical format is questions with Likert-scale response choices

Visual analog scores are also used in some instruments

Output is typically a numeric score



PSYCHOMETRIC PROPERTIES

Validity: degree to which an instrument measures what is intended to measure

Responsiveness: degree to which an instrument detects meaningful change over time

Reliability: degree to which measures are reproducible and consistent over time in patients with a stable condition

Acceptability: degree to which the instrument is acceptable to the patient

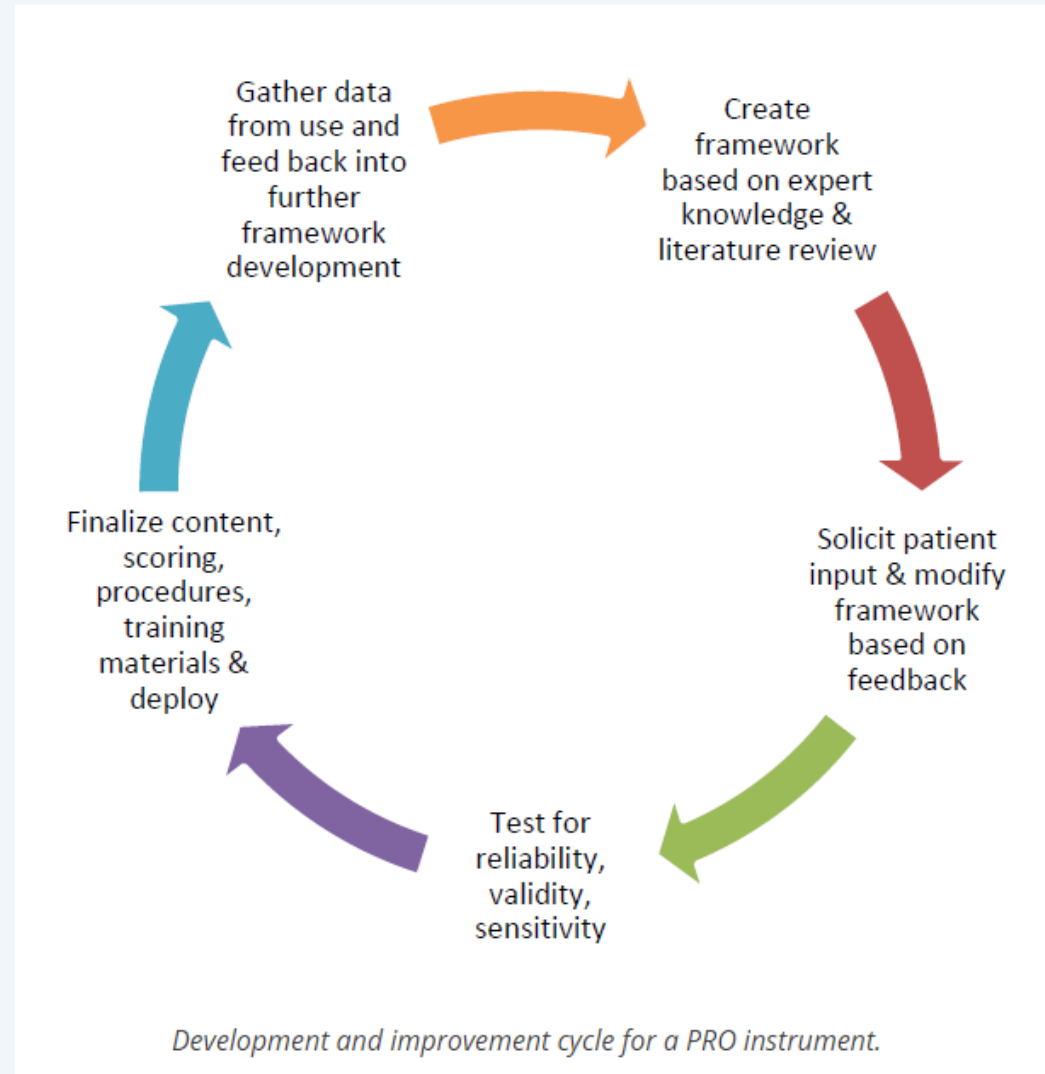
These should be studied prior to general use of an instrument.
The quality of an instrument is judged according to the evidence of these properties.



DEVELOPMENT OF PRO

Rigorous methodology

Multi-step process



ADDITIONAL CONSIDERATIONS

Additional more specific criteria also exist (e.g. floor-ceiling effects, internal consistency, criterion validity)

The assessments are contingent upon the instruments being applied in exactly the manner they were studied

- How administered, what language, etc

Since the output is numeric:

- Often needs to be measured repeatedly, with assessments of *change* over time
- Additional study is helpful for interpreting results (e.g., minimal clinically significant difference)

Missingness is a major limitation of QoL research – “missingness bias” – those who are not completing the surveys are different somehow from those who are.



HOW COULD WE USE PROS IN PAD?

APPLICATION	EXAMPLES
Clinical research	Objective evidence of comparative effectiveness, magnitude of benefit of therapy, natural history of disease, durability
Regulatory	Typically as a secondary endpoint; verification of benefit for stated device indication (IC), cost-effectiveness analysis/QALYs
Registries	Benchmarking, appropriate use criteria, payer mandates
Patient counseling	Expectation setting, demonstration of improvement/decline



So, if there are so many reasons to collect PROs, and so many potentially interested parties, then why aren't we already doing it?



Framework To Guide The Collection And Use Of Patient-Reported Outcome Measures In The Learning Healthcare System

Patricia Franklin, MD, MBA, MPH, Kate Chenok, MBA, [...], and Erin Holve, PhD

EGEMS (Wash DC). 2017; 5(1): 17.

Published online 2017 Sep 4. doi: [10.5334/egems.227](https://doi.org/10.5334/egems.227)



Collection and use of PROMs in the Learning Health Care System



FINDINGS FROM FRANKLIN ET AL.

Barriers to implementing routine PRO collection

- Altered clinic workflow
- Limited web-based tools to support real-time scoring and trending across time and clinical settings
- Limited visualization tools for diverse stakeholder use

Suggest a framework for implementation with six key steps.

Report completion rates of >80% in several registries.

Franklin PD et al. *Framework to guide the collection and use of PROs in the learning healthcare system.* EGEMS (Wash DC). 2017; 5(1): 17. Published online 2017 Sep 4. doi: [10.5334/egems.227](https://doi.org/10.5334/egems.227)



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1. Limited EHR interoperability across health care systems is a significant barrier to longitudinal PROM use.

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3. To engage patients and maximize value in their care, PROM tools must assure real-time scored, consistent measures are trended over time and available to clinicians at the time of treatment decisions.
4. Research to define the full potential for PROMs to inform clinical decisions—both for individual patient care and national best practices—is critical before patients, clinicians, health care leaders, and researchers view PROMs as an integral component of the learning health system

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IN SUMMARY

Most commonly used and well studied PRO is health-related QoL.

Several tools exist for PAD; they have variable pros and cons, depending on the intended application.

There is no question that they are valuable to a wide range of stakeholders, for a broad variety of applications.

The larger challenge is implementing routine ***and repeated*** data collection.

But there are experts in this field to whom we can turn for valuable lessons learned.

