

Patients Reported Outcome : Homeworks, Tips & Tricks for the next decade

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V-ITALy 2023

25/05/2023

PRO/PROM : What are we speaking about ?

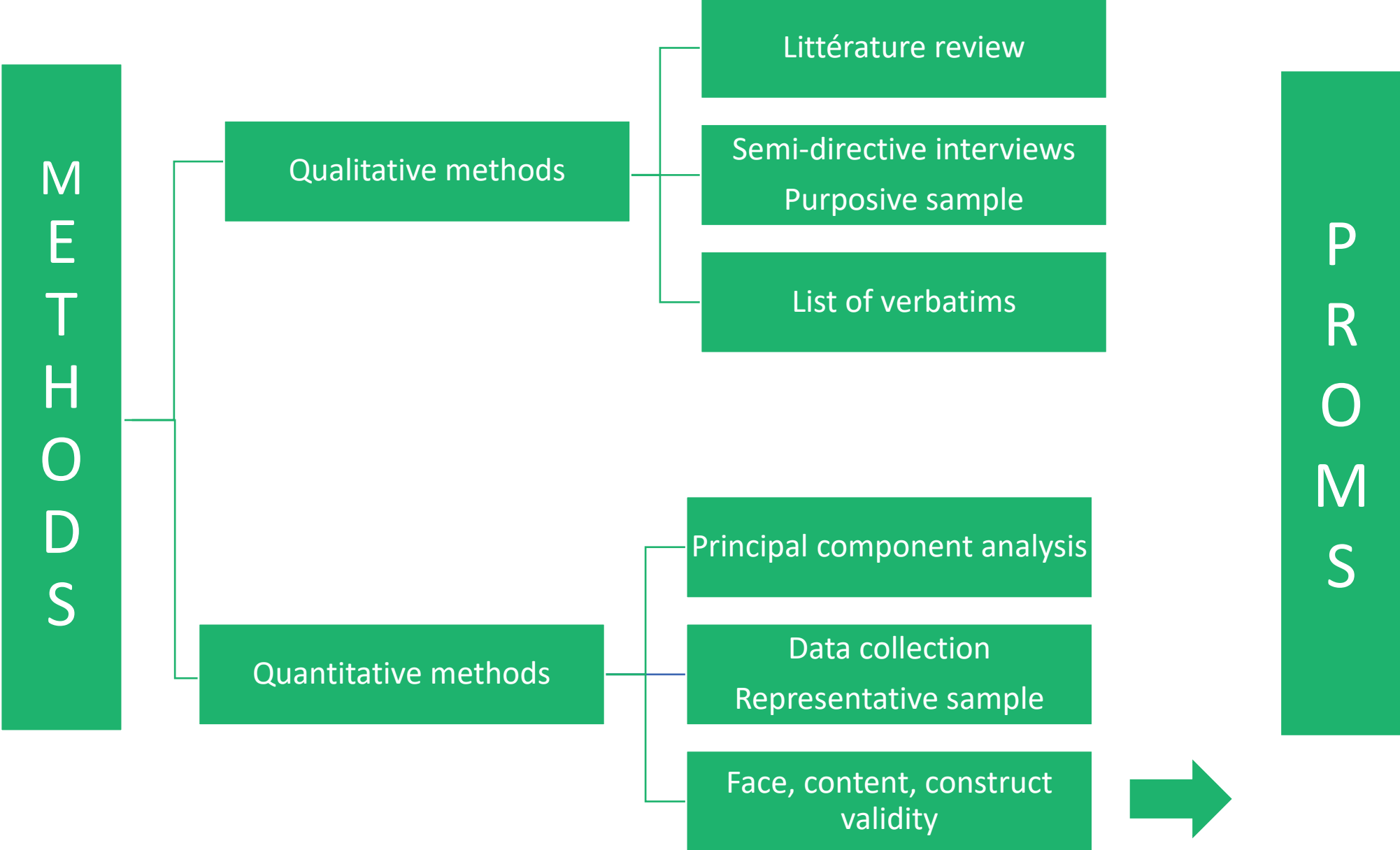
PRO	PROM
Patient-Reported Outcomes	Patient-Reported Outcomes Measures
<p>Outcomes in health status reported directly by the patient, without interpretation by a physician, in several areas:</p> <ul style="list-style-type: none">- Symptoms- Functional capacity- Well-being- Health-related quality of life	<p>Tools for measuring outcomes in health status reported by the patient:</p> <p>Generic questionnaires: <i>SF-36, EQ-5D</i></p> <p>Specific questionnaire: <i>Chronic Venous Insufciency Questionnaire (CIVIQ-14) , Upper Limb Lymphedema Quality of Life Questionnaire (ULL-27), Cystic Fibrosis Questionnaire (CFQ)</i></p>

- CMS MMS Hub, 'Patient Reported Outcome Measures', 2022
- Minvielle, Étienne, Aude Fourcade, and Marie Ferrua, 'Des enquêtes de satisfaction aux patient-reported outcomes', *Risque et qualité*, 16.4 (2019)
- Roussel, Christophe, Olivier Chassany, Isabelle Durand-Zaleski, Anne Josseran, Lise Alter, Pascal Auquier, and others, 'Place of Patient-Reported Outcomes & Experiences Measurements (PROMS/PREMS) in the Assessment and Pricing of Health Technologies in France', *Therapies*, 77.1 (2022), 103–15
- Cella, David, Elizabeth Hahn, Sally Jensen, Zeeshan Butt, Cindy Nowinski, Nan Rothrock, and others, *Patient-Reported Outcomes In Performance Measurement (RTI Press, 2015)* <<https://doi.org/10.3768/rtipress.2015.bk.0014.1509>>

Why Patient-Reported Outcomes should be implemented ?

- Patients are the best judges of the impact of their treatment on their pain, function, symptoms and quality of life
- PROMs are a valuable support for patient-centred care
- Systematic collection of PRO data informs efforts to improve quality and safety

Quantitative and qualitative approaches are complementary



PROMs : psychometric properties

Characteristics	Statistical Tests	Validity thresholds
Reliability	Intraclass correlation Coefficient (ICC) across stable patient at D0-D28	>0,80
	Cronbach ∞ coefficients	>0,70
Construct validity : <i>Within scale analysis</i>	Multi-trait, Multi-items analysis Item scale correlation coefficient at D0	>0,40
Construct validity <i>Know groups differences</i>	Analysis of variance (Kruskal wallis)	P<0,05
Construct validity <i>Convergence analysis</i>	Spearman correlation coefficient at D0 with homologous dimensions of validated quality of life scales	>0,40
Reponsiveness	Effect size	>0,40

Guidelines to construct PROMs

1. Formulate hypotheses: what is the expected and relevant difference between the groups (within the context of randomization or external comparison)?
2. Define what is a responder based on a minimum relevant change in order to turn mean scores into percentages (several responder definitions in a sensitivity analysis are recommended by the FDA)
3. Prioritize PROMs among the primary/secondary end-points
4. Integrate PROMs into the statistical analysis plan with alpha risk control (example: integration of a PROM into sequential hierarchical analysis).

Implementing PROMs for what purpose ?

PROMS are being used to evaluate health care effectiveness at 3 levels:

- **Micro** : used during the clinical consultation and in multidisciplinary team discussions is expected to contribute to shared clinical decision-making and patient-centred care
- **Meso** : used to compare treatment alternatives
- **Macro** : aggregated results to assess the performance of health care organisations (PRO-PM)

Moving from PROM to PRO based Performance Measures



- **PRO-based performance measure (PRO-PM):** A performance measure that is based on PROM data aggregated for an accountable healthcare entity (e.g., percentage of patients in an accountable care organization whose depression score as measured by the PHQ-9 improved).

- **Performance measure :**
 1. The item or instrument that measures the health concept of interest
 2. The calculation of the performance score
 3. The target population and inclusion and exclusions criteria
 4. The risk adjustment methodology

ULL-27:

Quality of life among breast cancer patients with lymphedema: a systematic review of patient-reported outcome instruments and outcomes

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review, two lymphedema-specific PRO instruments were identified [31, 48]. Based on assessment using the COSMIN criteria, only the ULL-27 can be recommended without hesitation.

CIVIQ-14:

The impact of lower limb chronic venous disease on quality of life: patient and physician perspectives

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CFQ:

Development and Validation of the Cystic Fibrosis Questionnaire in the United States*

A Health-Related Quality-of-Life Measure for Cystic Fibrosis

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