Prevalence and factors associated with patient-reported outcomes in pragmatic randomized controlled trials

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Background

- Patient-reported outcomes (PROs) are subjective measures of health that come directly from patients, without interpretation by clinicians or anyone else
- PROs are patient-centred and therefore well suited to pragmatic trials, but their use and reporting in pragmatic trials has not been described

Objectives

Among health-focused pragmatic RCTs, to determine:

1. The prevalence and types of PROs used.
2. Factors associated with the use of PROs as primary/co-primary outcomes.
3. How sample sizes and target differences were determined for trials with PROs as primary/co-primary outcomes.

Methods

Search

- An electronic search filter was developed and applied to MEDLINE to identify primary reports of health-focused pragmatic RCTs published 2014-2019 and registered at ClinicalTrials.gov

Extraction

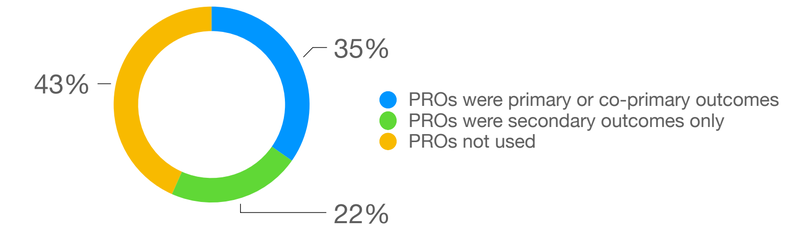
- Trial descriptors were downloaded from ClinicalTrials.gov and extracted manually

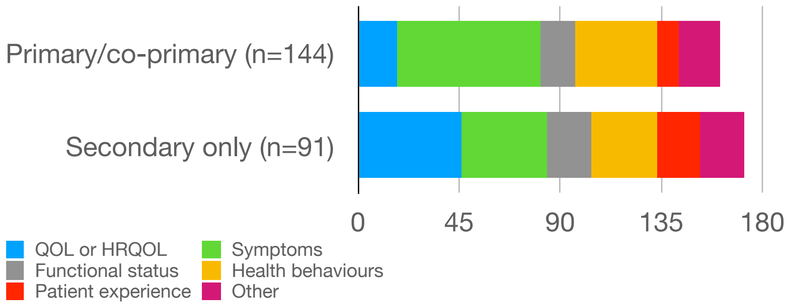
Analysis

- Descriptive statistics were used to summarize trial characteristics
- Chi-squared, Wilcoxon rank sum, and Cochran-Armitage trend tests were used to compare characteristics of trials with and without PROs as primary outcomes

Results

415 trials met inclusion criteria:



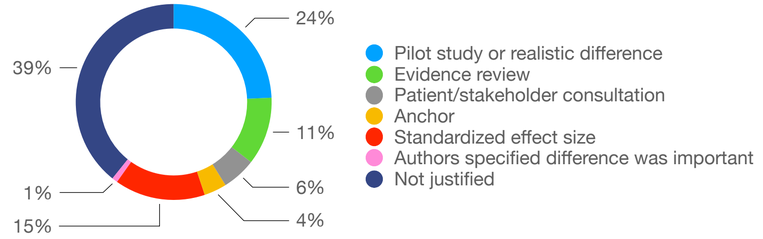


Note: multiple selections per trial were possible. QOL = Quality of Life. HRQOL = Health-Related Quality of Life.

Factors associated with use of PROs as primary outcomes

Higher prevalence	Lower prevalence	Not associated
Conducted in Europe vs. elsewhere	Published in higher impact journals	Patient/stakeholder engagement
Primary purpose was treatment vs. prevention, health services research, other	Conducted in low- or middle-income countries vs. elsewhere	Clinical setting vs. non-clinical
Dietary or behavioural interventions vs. clinical, other	Paediatric or older adult participants vs. all ages	Year of publication
Individually randomized vs. cluster	Industry funded vs. government, university, foundation, other	Government, university or foundation funded vs. industry, other

Methods used to determine target difference



Discussion & Implications

- PROs were infrequently used as primary or co-primary outcomes in pragmatic trials
- Patient and stakeholder engagement was rare, especially in determining target differences for sample size calculations
- Research funding bodies, institutions and scientific journals can support the use of PROs and patient engagement in pragmatic trials by establishing policies, providing methodological support, or creating incentives