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Primary Outcomes Reporting in Trials (PORTal): a systematic review of inadequate reporting in pediatric randomized controlled trials

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Abstract

Objective: Conduct a systematic review of pediatric randomized controlled trials (RCTs) published in high-impact journals to assess the reporting of primary outcomes and the psychometric properties of their measures.

Study Design and Setting: Systematic review with screening and simultaneous data extraction conducted by two independent reviewers. Electronic searches of six general medicine and four pediatric journals were conducted in MEDLINE, EMBASE, and the Cochrane Central Register of Controlled Trials (CENTRAL) databases. RCTs of a single phase/step in a single publication, published in English between 2000 and 2010 with participants less than 21 years of age were included.

Results: A random sample of 20% (n = 445) of 2,229 initial references was screened and 206 (46%) met inclusion criteria. Half (48.5%) of included studies reported a singular primary outcome, 27% did not identify any primary outcome, and 24% identified multiple primary outcomes (range 2–20). Twenty-one trials used an instrument to measure their primary outcome, but only 7 (33%) reported its psychometric properties.

Conclusion: Pediatric trials published in top medical journals have inadequate reporting of their primary outcomes and the psychometric properties of their outcome measures. Whether the issue is one of poor reporting and/or poor validation will be further investigated.

Keywords: Pediatric; Outcome measure; Primary outcome; Reporting; Systematic review; Randomized controlled trial

1. Introduction

Randomized controlled trials (RCTs) represent the gold standard for evidence about treatment effectiveness for health care providers, researchers, policy makers, and other decision makers. RCTs are preferentially included in knowledge synthesis efforts such as systematic reviews and meta-analyses, which inform decision makers at every level. Many RCTs are published annually in high-impact journals; however, there is growing concern with regards to the reporting of outcomes and consequently the reporting of the measurement properties of the outcome measures, namely their validity and reliability [1–4]. As clinical trials are ''only as credible as their outcomes'' [5], a lack of reporting and validation implies that tremendous expense, effort, and resources may not be used optimally.

Conflict of interest: All authors have completed the ICMJE uniform disclosure form at www.icmje.org/coi_disclosure.pdf and declare no support from any organization for the submitted work; no financial relationships with any organizations that might have an interest in the submitted work in the previous 3 years; no other relationships or activities that could appear to have influenced the submitted work.

Transparency declaration: The lead author affirms that the article is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted; and that any discrepancies from the study as planned (and, if relevant, registered) have been explained.

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What is new?

Key findings

- Pediatric trials, across disciplines published in high-impact journals, have inadequate reporting of both their primary outcomes and the measurement properties of their outcome measures.

What this adds to what was known?

- Preliminary investigation of clinical trials in select populations has identified poor reporting of primary outcomes.
- Reporting guidelines represent a "minimum set" and do not yet address measurement properties of primary outcome measures.
- As trials are only as valid as their primary outcome measures, adequate reporting of measurement properties of primary outcomes is essential.

What is the implication and what should change now?

- Trialists, journal editors, and reporting guideline developers should improve reporting of primary outcome measures and their measurement properties.

An outcome is a measurable variable that should be clearly stated by the authors, and an outcome measure is the tool used for measuring the outcome (scales, questionnaires, instruments, or scoring systems—we describe these collectively using the term "outcome measure") [1]. The measurement properties of an outcome measure, that is, validity, reliability, and responsiveness provide information regarding the measure's intended purpose, its performance and accuracy, and its ability to detect a true change. When selecting which outcome measures to use in any given study or when evaluating the use of a particular measure, the measurement properties are often compared. Inadequacies related to primary outcome reporting and their consequent impediment on the conduct of knowledge synthesis efforts have been discussed in light of selective outcomes reporting, wherein only a selected subset of analyses or outcomes is reported based on the results they yield [6].

The issue of selective outcomes reporting is secondary to a larger issue of trials that fail to identify any primary outcome at all. The inadequate reporting of outcomes in the pediatric population has been identified while investigating outcomes selection within a specified clinical area. In systematic reviews of RCTs within pediatric subspecialties, authors consistently fail to report identifiable primary outcomes [1,4,7].

Although it is recognized that the "prespecification of a single primary outcome based on biologic credibility, clinical importance, and potential responsiveness to the intervention" is the best approach, the reader is more often "offered a shopping list of end points" [4]. Along with the poor reporting of primary outcomes, the validation of outcome measures is also poorly reported or missing altogether. Few studies report that a validated instrument was used or provide evidence of formal evaluation against some sort of reference standard, and those that do, fail to provide citations to support the reported measurement properties [1,3].

A variety of initiatives [5,8–10] have been developed along with systematic reviews [6,7] that address some of the issues of inadequate reporting and validation. To assess the magnitude of this problem across pediatric disciplines, we conducted a systematic review of a random sample of pediatric RCTs published in 10 high-impact journals between 2000 and 2010. Our primary interest was assessing outcome measures since these have been identified as in need of further study. As such, the main aim or primary outcome of this systematic review was to examine primary outcome reporting including (1) how many RCTs reported a primary outcome, (2) the number of primary outcomes reported, (3) how many RCTs reported the measurement properties of the instruments used, and (4) the relevant citations provided for the measurement properties reported. A secondary outcome was to examine other key pediatric trial metrics and their reporting, such as information about the population (participant ages, condition(s) under study, sample size, and calculation), intervention and control group(s).

2. Methods

2.1. Search strategy

With the help of an experienced health research librarian, electronic searches in MEDLINE, EMBASE, and the Cochrane Central Register of Controlled Trials (CENTRAL) databases were undertaken. We selected 10 journals by impact factor (six general medicine journals and four pediatric journals), all of which include pediatric trials in their publications. All searches used the respective journals name: *New England Journal of Medicine*, *Journal of the American Medical Association*, *Lancet*, *Annals of Internal Medicine*, *British Medical Journal*, *Plos Medicine, Journal of the American Academy of Child and Adolescent Psychiatry*, *Pediatrics*, *Journal of Pediatrics*, and *Archives of Pediatrics & Adolescent Medicine*. Searches were limited by publication type (RCTs), publication year (2000–2010), respective pediatric filters, and the English language. The full Medline search strategy is available in Appendix 1 at www.jclinepi.com.

2.2. Study selection

We included studies that (1) were RCTs, that is, studies that randomly allocated participants to interventions, and included parallel, crossover, factorial or N-of-1 designs,