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ORIGINAL ARTICLE

A principal component analysis is conducted for a case series quality appraisal checklist

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Abstract

Objective: Because of a lack of a control group, a case-series study is considered one of the weaker study designs from which to obtain evidence on treatment effectiveness. Under certain circumstances, however, this is the only available evidence to inform health-care decisions. This study's intent was to develop and validate a quality appraisal checklist specifically for case-series studies.

Study design and setting: A modified Delphi process was used to develop the checklist, which was then used by multiple researchers to appraise a random sample of 105 case-series studies. A principal component analysis of these appraisals was conducted to further refine the checklist.

Results: The modified Delphi process resulted in a 20-criterion checklist. The principal component analysis of the appraisals for the 105 case-series studies revealed two components. The first component (10 criteria) indicated the extent to which a case series presented traditional features of a statistical hypothesis-testing paradigm. The second component (seven criteria) indicated whether detailed descriptions of the subjects' characteristics that might feature in the experimental design were present, particularly in judgments about the likelihood of confounding.

Conclusion: This quality appraisal checklist may be useful in assessing case-series studies, but further validation of the checklist is required.

Keywords: Case series; Quality appraisal; Delphi; Validation; Research evidence; Treatment intervention

1. Introduction

Assessment of the safety and efficacy of a treatment intervention requires research evidence from clinical studies. Well-developed methodologies for scrutinizing flaws in design, conduct, analyses, and reporting exist for judging the potential for bias in randomized control trials (RCTs) [1]. In recent years, observational studies (including case-series studies) have been increasingly included in health technology assessments (HTAs) and systematic reviews, but the methodology specifically for assessing case series is less developed [2].

A case-series study is an uncontrolled study that describes a single group of patients who receives a particular intervention and is followed (either prospectively or retrospectively) to observe their outcomes; such a study does not allow for a direct, statistical comparison to a control sample [3,4]. For this reason, a case-series study occupies a low position in the hierarchy of evidence and is considered one of the weaker study designs from which to obtain research evidence on treatment effectiveness [5]. Nevertheless, there are some circumstances where evidence from case-series studies is considered important, necessary, and generally accepted by clinicians [6] and is used in HTAs and systematic reviews for assessing treatment interventions [5]. For example, when examining long-term outcomes, particularly harms of treatment interventions, case-series studies often comprise a large part of the evidence base [7]. Sometimes, case-series studies are the only source of research evidence available to inform decisions about the implementation of new technologies [8–10]. For example, many surgical procedures, therapeutic devices, or rapidly developing interventions are used for conditions where randomization of patients is problematic,

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What is new?

Key findings:

- A principal component analysis of a 20-criterion quality appraisal checklist developed specifically for case series studies revealed two components, with the first component (10 criteria) being related to hypothesis-testing paradigm and the second component (7 criteria) being related to detailed descriptions of subject/intervention characteristics.
- On the basis of the preliminary principal component analysis, no criterion can be eliminated from the 20-criterion checklist.

What this adds to what was known?

- Although a number of quality appraisal checklists for case-series studies were found in the literature, there is a lack of information about whether these tools are formally validated.
- The preliminary validation process serves as a starting point for future large-scale studies in this area.

What is the implication and what should change now?

- We invite health technology assessment researchers/systematic reviewers to use this checklist in assessing evidence from case-series studies and provide feedback to help further refine the checklist.

unethical, or impossible [4,11]. In some cases, the treatment intervention of interest is the last resort for the condition; thus, even a nonrandomized comparison (such as in a cohort study) would not be possible.

Because case-series studies are prone to various types of biases in relation to selection, performance, detection, attrition, and reporting, the derived results are generally ranked as low quality [12]. Assessing the robustness of the evidence of uncontrolled studies remains challenging [10]. In contrast to the availability of well-developed quality appraisal tools for RCTs (eg, the Cochrane Collaboration risk of bias tool [1]) or for non-RCTs (eg, A Cochrane Risk Of Bias Assessment Tool for Non-Randomized Studies of Interventions [13]) in which two patient groups are compared, few quality appraisal tools exist for case-series studies that have only one group of patients.

Although some criteria in nonspecific checklists can be common between case-series studies and other types of studies, using nonspecific checklists for case-series studies represents a redundant practice in the field of HTA and systematic reviews, as these checklists usually include many criteria not relevant to case-series studies (eg, criteria related to the control group). Some quality appraisal tools have been developed specifically for case series [14] or have been used for assessing case-series studies [15,16]. However, no widely accepted and validated quality appraisal tool exists for assessing the methodologic quality of case-series studies [2,17]. Furthermore, there is no consensus about which criteria should be included for this purpose [12]. As quality appraisal tools should be rigorously developed, evidence based, valid, reliable, and easy to use [2], there has been an increased interest in developing such a quality appraisal checklist.

The objective of this study was to validate a quality appraisal checklist for case-series studies that was developed through a modified Delphi process.

2. Methods

The development and validation of the checklist consisted of three separate steps: the Delphi process, the pilot exercise, and the validation process. As detailed descriptions of the first two steps are available in a previous report [18], this article focuses mainly on the validation process, with only a brief summary of the first two steps for context.

2.1. The Delphi process

The Delphi process, a structured communication process characterized by purposive sampling of experts in the field of interest, panelist anonymity, multiple iterations, and feedback of statistical aggregation of group responses [19,20], was used for final consensus of the criteria.

A group of seven experienced HTA researchers (five from the Institute of Health Economics [IHE], Canada, one from ASERNIP-S, Australia, and one from the AETS, Spain), participated in the modified four-round e-mail–based Delphi process to develop the quality appraisal checklist. Each panelist received an initial list of 30 criteria (Table 1) compiled from a literature search [12,21–24] and were asked to rate the relevance and importance of each criterion based on a five-point Likert scale that ranged from 1 (very important) to 5 (not important at all). A criterion was included in or excluded from the final checklist if at least five of the seven panelists (ie, 70% agreement among panelists, a cutoff decided a priori) judged that criterion either very important (rank 1) or not important at all (rank 5). The panelists also had the opportunity to suggest new criteria and how to improve clarity of the final checklist.

Each panelist received a personalized summary of his/her own rating results (blind to other individuals' rating to ensure anonymity) and the aggregated group ratings (results and distribution of ratings from all panelists), along with any newly suggested criteria. The panelists' responses were analyzed by a biostatistician (D.S.), who was not part of the Delphi panel and was also blinded as to the identities of the panelists.