SA109

Quality Assessment Tools for Single-arm Studies: A Rapid Review

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Background

- Quality assessment (QA) is a vital aspect of conducting a thorough systematic literature review (SLR), as the validity of the conclusions of the review depends on the reliability of the included literature.
- There are several quality assessment tools that are industry-standard for the appraisal of various study designs, yet there is currently no quality assessment tool developed specifically for single-arm studies (including single-arm trials, open-label extensions, or prospective interventional studies with historical control arms).
- While these trials are valuable for generating preliminary evidence, they are typically followed by more rigorous study designs, such as randomised controlled trials; however, in rare disease areas or new interventions, single-arm studies may be the main source of evidence.
- In this case, it is important to appraise the quality of the evidence before using it in further analyses and decision-making.
- The current options available for reviewers to assess single-arm studies are imperfect, because using a tool not developed for the evaluation of single-arm study designs will mean excluding sections regarding comparator groups, and does not fully consider the potential biases associated with single-arm study designs.

Objective

- In June 2024, a rapid review was conducted to identify which existing QA tools are utilised in SLRs to assess risk of bias in single-arm studies.

Methods

- Searches were conducted on June 17th 2024 in Embase®, Medline®, and the Cochrane Database of Systematic Reviews.
- The search strategies incorporated terms including “single-arm”, ”open-label extension”, ”risk of bias”, ”quality assessment”, and “systematic literature review”.
- Results from the electronic database searches were downloaded into Covidence®, which was used to manage citation screening of titles and abstracts, and to complete the review of full papers. Eligibility screening and data collection were performed by a single reviewer.

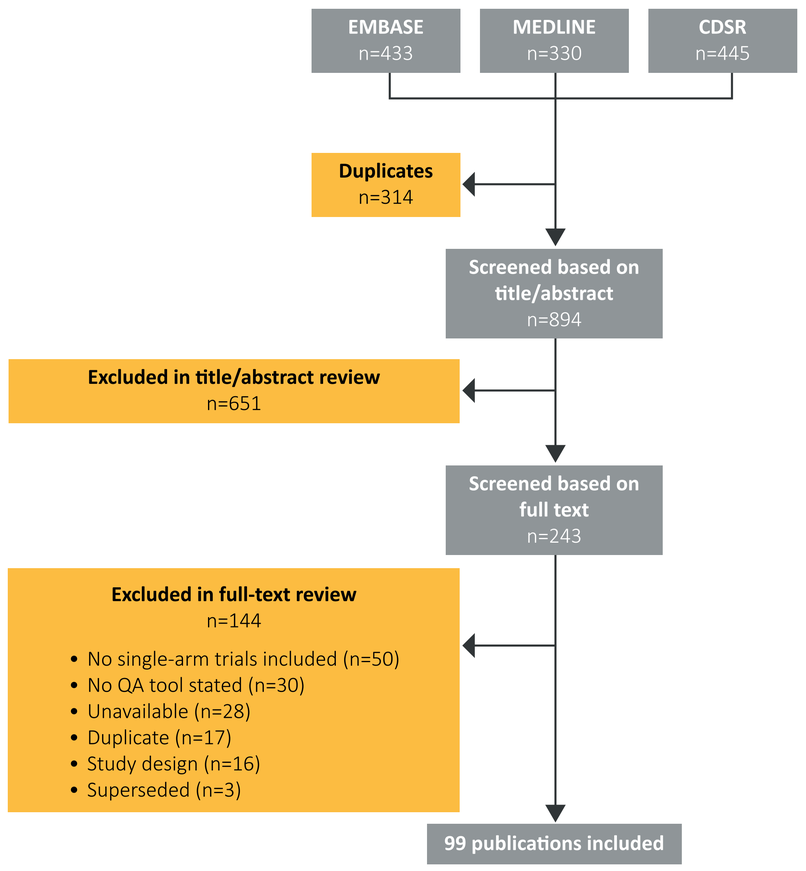
Results

- In total, 99 relevant publications were identified for inclusion in this rapid review.
- The flow of publications through the review is depicted in the PRISMA flow diagram in Figure 1.
- Most of the included SLRs (88%) were published in the last 5 years (2019–2024).
- A breakdown of the QA tools used in each of the included SLRs can be found in Figure 2.
- ROBINS-I[1] was the most common QA tool (28%), followed by MINORS[2] (11%), the Newcastle-Ottawa scale[3] (9%), and RoB 1.0[4] (9%). Descriptions of these tools are presented in Table 1.
- Of these four tools, three are designed to primarily assess comparative studies. The MINORS checklist features items that are specific to non-comparative studies, however it doesn’t address the fundamental limitations of single-arm study designs.
- The authors in 16% of the included SLRs did not consider the current QA tools adequate for the appraisal of single-arm studies and instead used a bespoke or adapted tool (8%) or did not conduct a quality assessment of the single-arm studies (8%). In the latter studies, authors noted an inherent high risk of bias due to the lack of a comparator arm.
- The remaining SLRs featured one of fourteen other QA tools, including the NIH Quality Assessment Tool[5] (6%), JBI Critical Appraisal Tools[6] (4%), and the Downs and Black checklist[7] (4%).

Conclusion

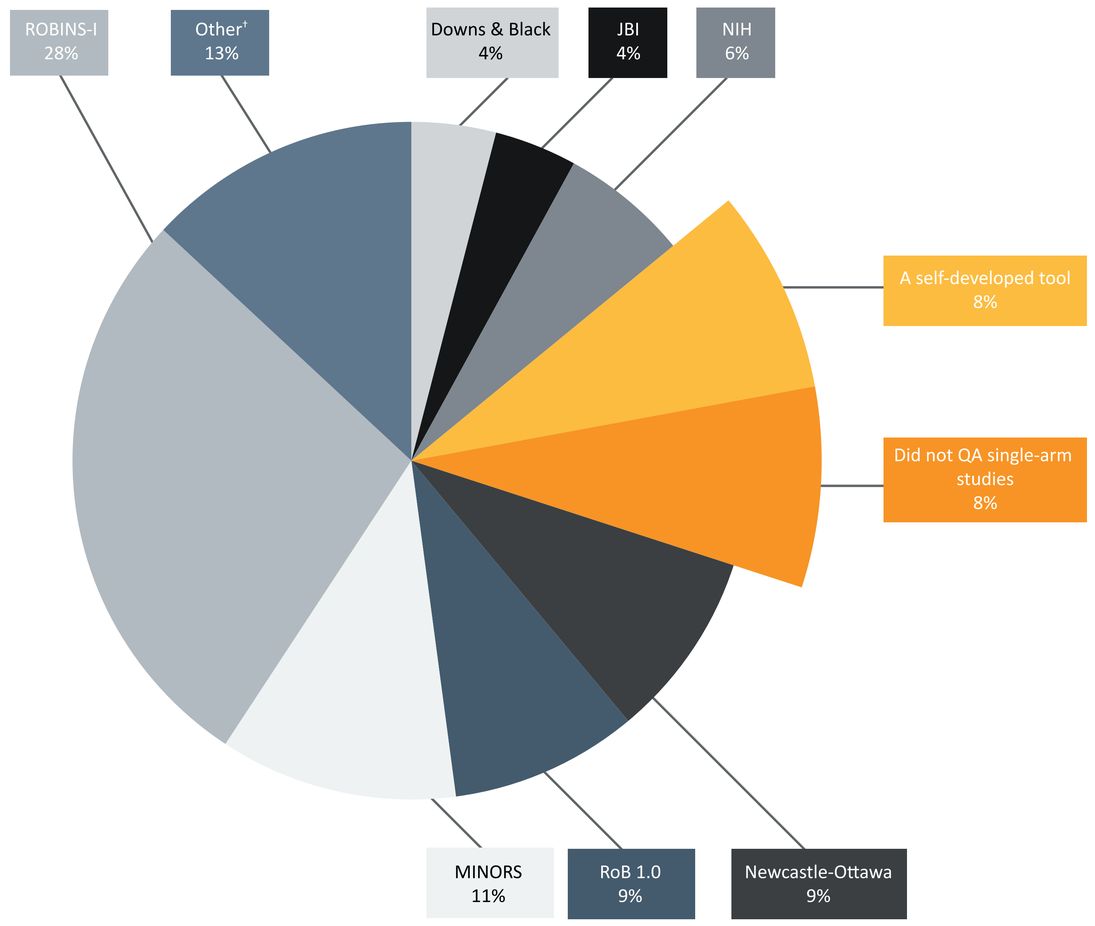
- This rapid review found that current practice for quality assessing single-arm studies is to use a pre-existing QA tool, a bespoke/adapted tool, or not to conduct QA at all.
- While the currently available tools provide reasonably structured approaches for quality assessment, they are insufficiently nuanced to adequately appraise the unique study design of single-arm studies.
- Recently, there has been an increased requirement for the quality assessment of all included publications from health technology assessment (HTA) agencies in submissions, including single-arm studies.
- Most SLRs identified in this rapid review were published in the last 5 years (2019–2024). This trend in recent years highlights the expanding role of single-arm studies in clinical research and decision-making.
- With the growing number of such studies being published, the need for a robust and consistent evaluation framework is becoming ever more critical for ensuring accuracy and reliability in HTA submissions.
- Therefore, there is an increasing demand for an industry-standard quality assessment tool specifically designed to consider the unique challenges and characteristics of single-arm studies.

Figure 1: PRISMA flow diagram



Abbreviations: CDSR, Cochrane Database of Systematic Reviews; QA, quality assessment.

Figure 2: Summary of QA tools used in identified studies



†Includes Cochrane EPOC, CRD 2001, Evidence Project Group tool, Jadad scale, PEDro, QualSyst, and reviewer opinion (1% each); a disease-specific tool, EPHPP, and those for which the QA tool used was unclear (2% each).
Abbreviations: CRD, Centre for Reviews and Dissemination; EPHPP, Effective Public Health Practice Project; EPOC, Effective Practice and Organisation of Care; JBI, Joanna Briggs Institute; MINORS, methodological index for non-randomised studies; NIH, National Institutes of Health; PEDro, Physiotherapy Evidence Database; QA, quality assessment; RoB, Risk of Bias; ROBINS-I, Risk of Bias In Non-randomised Studies of Interventions.

Table 1: Common risk of bias and quality assessment tools

Quality assessment tool	Intended study designs	Tool description
ROBINS-I[1]	Non-randomised comparative studies	Evaluates risk of bias in estimates of the comparative effectiveness of interventions from studies that did not randomise patients to arms. Recommended by Cochrane for non-randomised studies[8].
MINORS[2]	Non-randomised surgical studies	12 items designed to assess methodological quality of surgical studies but can be applied to other study types. The first 8 items are specific to non-comparative studies.
Newcastle-Ottawa Scale[3]	Non-randomised studies, particularly observational and cohort studies	Assesses quality of selection, comparability, exposure, and outcome of study participants. Recommended by Cochrane in previous editions of the manual[8].
RoB 1.0[4]	RCTs	Assesses risk of bias in 7 sections pertaining to allocation concealment, blinding, and reporting. Recommended by Cochrane in previous editions of the manual[8].

Abbreviations: JBI, Joanna Briggs Institute; MINORS, Methodological index for non-randomized studies; RCT, randomised controlled trial; RoB, Risk of Bias tool; ROBINS-I, Risk Of Bias In Non-randomised Studies of Interventions.

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