



BMJ Open International guidelines for self-report and proxy completion of paediatric health-related quality of life measures: a protocol for a systematic review

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To cite: Mpundu-Kaambwa C, Chen G, Dalziel K, *et al.* International guidelines for self-report and proxy completion of paediatric health-related quality of life measures: a protocol for a systematic review. *BMJ Open* 2021;**11**:e052049. doi:10.1136/bmjopen-2021-052049

► Prepublication history and additional supplemental material for this paper are available online. To view these files, please visit the journal online (<http://dx.doi.org/10.1136/bmjopen-2021-052049>).

Received 04 April 2021

Accepted 18 October 2021



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ABSTRACT

Introduction Measures of health-related quality of life (HRQoL), accompanied by the values (or utilities) required to estimate quality-adjusted life-years, are crucial for determining health benefits within economic evaluation and health technology assessment. Several generic and condition-specific measures or instruments of HRQoL, accompanied by values, currently exist for application with child populations. However, there is a lack of a structured summary of guidelines and recommendations for applying these measures in practice. This protocol describes a systematic review of guidelines and recommendations for child and proxy completion of child-specific measures of HRQoL. The aims of the review are to (1) identify and summarise published guidelines and recommendations for existing child-specific measures of HRQoL, (2) determine whether the identified guidelines and recommendations differ by instrument and child characteristics, (3) identify current gaps in these guidelines and recommendations and (4) identify best practices for child self and proxy assessment in paediatric HRQoL measurement for economic evaluation and health technology assessment.

Methods and analysis The review will identify, collate and synthesise published guidelines and recommendations for existing child-specific utility measures of HRQoL. Electronic databases to be searched include the Cochrane Library, Medline, Scopus, Web of Science, EconLit, PsycINFO, CINAHL, Embase and Informat. The search will be extended to websites of (1) international organisations for health technology assessment, (2) regulation, health economics and HRQoL outcomes research and (3) instrument developers. Three reviewers will independently screen titles and abstracts against the inclusion criteria. A narrative synthesis will describe the key features of the guidelines identified.

Ethics and dissemination Ethical approval is not required as the proposed systematic review will not use primary data. A paper of the systematic review will be submitted to a peer-reviewed journal for publication.

PROSPERO registration number CRD42020207160.

INTRODUCTION

Outcome measures used in assessing health-related quality of life (HRQoL) are a

Strengths and limitations of this study

- An extensive literature search of existing guidelines and recommendations for completing both self and proxy reports of child-specific preference-accompanied measures will be conducted.
- A comprehensive summary of both similarities and differences of existing guidelines for completion of generic and condition-specific measures of paediatric health-related quality of life (HRQoL) with utility values' in paediatric populations will be performed.
- Latest best practice guidance for self-report and proxy completion in paediatric HRQoL measurement will be provided.
- An anticipated limitation of this review is that though language translation services will be used to interpret the guidelines published in non-English languages to English, there is a small possibility that some of the translations may not be accurate.

ubiquitous source of information for paediatric clinical trials, child health research and population surveys. There has been an increasing interest in assessing HRQoL in child populations and how it is impacted by disease and treatments in recent years. This has given rise to the growing use of child self-report and proxy reporting of HRQoL using generic and condition-specific measures or instruments in paediatric clinical trials and health services research. HRQoL is a complex, multidimensional concept, which includes physical, emotional and social functioning associated with a person's health state.¹ The WHO defines QoL as 'an individual's perception of their position in life in the context of the culture and value system in which they live, and in relation to their goals, expectations, standards and concerns'.² QoL is a comprehensive multidimensional concept which refers to the impact of all

features of a person's life on their overall well-being, including HRQoL.

The terms 'QoL' and 'HRQoL' are often used interchangeably despite their reflecting concepts, which, while overlapping, are not strictly interchangeable.^{3,4} To ensure broad coverage of all the articles relevant to this study, we will include both terms in the search strategy as we expect some articles to be indexed under QoL even though they refer to HRQoL. However, in the text of this paper, we will use HRQoL from this point onwards. Validated instruments providing standardised subjective evaluations of HRQoL in children are available using self-reports and proxy reports.^{5,6} Self-report refers to the assessment of an individual's QoL, depending on their subjective feeling. On the other hand, a proxy report refers to an assessment provided by a respondent about another individual's QoL. In children, proxy assessments are generally completed by a parent/guardian, caregiver, teacher or health professional. Instruments can be distinguished into condition-specific (eg, the Cerebral Palsy QoL questionnaire) and those that are generally applicable or generic (eg, the Paediatric Quality of Life Inventory (PedsQL)) HRQoL instruments.

HRQoL measures can also be distinguished according to their ability to generate values (or utilities) required to estimate QALYs, thereby facilitating the use of HRQoL data in economic evaluation. Some measures are accompanied by value sets that facilitate estimation of QALYs, whereas others are not and apply a simple summary scoring system whereby responses to individual items or dimensions are typically aggregated to produce unweighted total scores.⁷ HRQoL measures that generate utility estimates assess individual items or dimensions according to the relative value that society places on living in a particular health state (usually referred to as 'societal preferences'). These utilities are reflected in scoring algorithms pertaining to the respective HRQoL measures, which are typically premised on preference weights developed from the stated preferences of large general population samples. Valuation methods such as the standard gamble, time trade-off (TTO) and more recently, discrete choice experiments have been applied for this purpose. They are anchored to the zero (denoting being dead) to one (representing full health) QALY scale. When applying child-specific measures of HRQoL, for example, in economic evaluations alongside randomised controlled trials or other prospective study designs, individual (child self-report and proxy) responses to these measures are typically ascertained at various time points throughout the study duration. Individual responses are then converted to utilities by applying a preference-based value algorithm pertaining to the HRQoL measure.⁸⁻¹⁰

In the assessment of HRQoL, there is a general consensus that an individual's self-assessment of their own HRQoL is usually more reliable and accurate than proxy assessment, and therefore self-report should be used wherever possible.⁸ Several child-specific measures of HRQoL accompanied by values are currently available

that are suitable for application in economic evaluation and health technology assessment, including the EQ-5D-Y, the Child Health Utility 9 Dimension (CHU9D) and the Health Utilities Index (HUI). Some instruments have demonstrated applicability in children as young as 6-7 years of age.¹¹ However, one of the main challenges of measuring HRQoL in child populations is that proxy assessment may be necessary. This is especially the case for very young children and older children with severe health problems and developmental delays who may be cognitively unable to assess their own HRQoL and complete the necessary measurement tasks.^{12,13}

Evidence from the psychometric literature indicates that in the early stages of childhood, children's ability to self-report their own HRQoL may be hampered by their relative lack of development (compared with children in older age groups). Some studies have reported that children may not always be capable of self-reporting their HRQoL for various reasons, including cognitive and communication competencies.^{14,15} A disadvantage to using proxy reports (particularly those from parents) is that relative to young people's self-reports, these reports tend to underestimate the more subjective facets of children's functioning compared with child self-reports.^{16,17} This potentially has implications for the findings of economic evaluations based on proxy versus self-reported HRQoL.^{18,19}

Validated measures of paediatric HRQoL, including both generic and condition-specific, are advantageous over ad hoc measures due to the assessment of psychometric performance prior to their widespread application, including validity, reliability, specificity and sensitivity.²⁰

There are several HRQoL instruments accompanied by values that are available for use in populations of children. These instruments differ in many ways, including the target population (eg, child vs adult), descriptive systems, different empirical approaches for developing preference weights, recall period, whether they are self or proxy reported, etc. Due to these differences, it has been observed that 'there is no unifying institutional policy or strategy that stipulates the collection, reporting and use of patient-reported outcomes (PROs).'²¹ Many bodies such as the Organisation for Economic Co-operation and Development champion the need to develop guidelines for the use of such instruments that will ensure a standardised approach to enable fair comparisons of data internationally.²² Establishing and understanding these guidelines helps determine (1) the appropriate target populations, (2) the correct method of data collection, (3) their adequacy in measuring the outcomes required and (4) how to summarise and interpret responses to the instruments correctly. Our research seeks to collate such guidelines.

According to WHO, guidelines are defined as 'documents that contain recommendations for clinical practice or public health policy.'²³ Recommendations provide methodically established information to assist with policy-makers, healthcare providers and patients' decisions.

Several validated generic utility measures now exist for use with children and proxy respondents, accompanied by guidelines for both self-report and proxy completion of these paediatric HRQoL measures.^{24–28} Developers of specific instruments have provided such guidelines in terms of when proxy instead of self-respondents should complete these measures.^{29–32} Additionally, professional agencies such as the International Society for Pharmacoeconomics and Outcomes Research (ISPOR)³³ and decision-making entities such as the US Food and Drug Administration (FDA)³⁴ have also published good practice recommendations specific to paediatric PRO measures. However, these guidelines have not been systematically reviewed, compared and summarised. Therefore, this systematic review aims to (1) identify and retrieve international evidence on guidelines and recommendations for child and proxy completion of paediatric preference-accompanied measures and (2) to systematically appraise and synthesise the findings of the search to inform practice, policy and further research.

The specific aims of this systematic review are to (1) identify and systematically summarise published guidelines and recommendations for self, and proxy completion of child-specific preference-accompanied measures, (2) determine whether these guidelines differ by instrument, age of child and child characteristics, including educational level and intellectual/cognitive abilities, (3) describe similarities and differences of these guidelines and (4) Identify current gaps in guidelines and recommendations for self and proxy completion of child-specific measures of HRQoL accompanied by values. If a guideline reports on self-report completion of a measure but not proxy completion and vice versa, we will only report guidelines for the available assessment, as data analysis, synthesis, critical appraisal and reporting may differ for guidelines for self-report vs proxy measures.

Generic measures accompanied by values may not always be considered appropriate for determining health benefits in specific populations/health conditions for economic evaluation. In these instances, condition-specific measures may be more sensitive or responsive to various health states within a health condition may be applied. Therefore, both generic and condition-specific measures accompanied by values that are available for use in child and adolescent populations will be included in this review.

METHODS

Design and registration

This systematic review will be conducted in accordance with the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) checklist.^{35 36} The protocol follows the PRISMA-Protocols (PRISMA-P) guidelines.^{37 38} The PRISMA-P checklist is presented in online supplemental appendix 1. The systematic review publication will report any revisions to the protocol. The structure and content of this review's inclusion and exclusion criteria will be guided by the Patient/Population, Intervention, Comparison and Outcomes mnemonic developed by the Joanna Briggs Institute³⁹ (table 1).

Inclusion criteria

This study will focus on guidelines and recommendations for completing both self and proxy reports of child-specific measures or instruments of HRQoL, which are accompanied by utility weights (values). For the purposes of this study, guidelines are defined as any instructions or prescriptions for completing these instruments within child (0–18 years) populations. As such, peer publications and grey literature that report guidelines for completing these measures within child (0–18 years) populations and in which a child or a proxy respondent could complete

Table 1 PICO criteria for including studies

Criteria	Inclusion	Exclusion
Population	Children ages 0–18 years	
Intervention	Guidelines and recommendations for completing both self and proxy reports of child specific preference-accompanied measures or instruments	<ol style="list-style-type: none"> 1. Publications that report third party content, that is, reproduce other authors' guidelines or recommendations rather than original guidelines or recommendations. 2. Publications that report guidelines for completing HRQoL measures which are not suitable for economic evaluation or health technology assessment because they are not accompanied by preference weights (values)
Outcome	HRQoL	
Comparison	Self-report versus proxy assessment	
Date	Cut-off date limit: from date of database inception to July 2021	
Language	Publications written in all languages will be included	

HRQoL, health-related quality of life; PICO, Population, Intervention, Comparison and Outcomes.

will be included. Only guidelines and recommendations published by (1) international organisations for health technology assessment, regulation (of medicines and medical devices), health economics and HRQoL outcomes research, (2) clinicians, clinical organisations and other entities involved in quality and safety initiatives and (3) instrument developers will be included. In this study, instruments developers refer to developers of the measures or instruments.

Publications that report guidelines for completing HRQoL measures in languages other than English will be included in this review. Publications in non-English languages identified during the search will be translated into English using (1) Google Translate,⁴⁰ a web-based translation tool and (2) a professional language translation service, for example, Elsevier publishing company translation services. Using two different methods to translate the guidelines from languages other than English to English will ensure the translation accuracy of journal articles, reports, websites and other literature and subsequently guidelines. Translation of publications and guidelines from other languages into English will be conducted at the identification stage, (ie, the title and abstract review), full-text article assessment and data extraction stages of the review process.

Exclusion criteria

1. Publications that report third party content, that is, reproduce other authors' guidelines or recommendations rather than original ones.
2. Publications that report guidelines for completing HRQoL measures which are not suitable for economic evaluation or health technology assessment because they are not accompanied by preference weights (values).

Information sources

An extensive search of the literature will be conducted with a medical subject librarian in the following electronic databases: The Cochrane Library (including the Cochrane CENTRAL, EED and HTA), Medical Literature Analysis and Retrieval System Online (MEDLINE) (including in-process and other non-indexed citations via Ovid interface), Scopus (via Elsevier interface), Web of Science Core Collection, EconLit (via Ovid interface), PsycINFO (via Ovid interface), Cumulative Index of Nursing and Allied Health Literature (via EBSCO-host), Excerpta Medical dataBASE (EMBASE) and Informit (via Informit interface). The primary electronic search strategy was designed for MEDLINE and adapted as appropriate for each of the nine electronic databases. All searches (both initial and updated searches) will be conducted between May and July 2021.

The websites of available HRQoL measures suitable for children who meet the inclusion criteria will also be searched for additional guidelines. Where necessary, the review team will contact developers of the included child-specific measures for clarification and further information.

A review by Chen and Ratcliffe identified several generic measures accompanied by preferences that have been used in child populations,⁴¹ including the CHU9D,²⁸ the HUI Mark 2 (HUI2),⁴² the HUI Mark 3 (HUI3),⁴³ the Assessment of QoL-6D Adolescent,⁴⁴ the Youth version of the EQ-5D (EQ-5D-Y),^{32,45} the 16-dimensional measure of HRQoL (16D),⁴⁶ the 17-dimensional measure of HRQoL (17D),⁴⁷ the Adolescent Health Utility Measure,⁴⁸ the Quality of Well-Being Scale.^{49–51} The measures mentioned above will be included in the review and any other we identify in the review. Two recent reviews^{52,53} have identified several existing condition-specific measures accompanied by preferences that can be used in child populations, including the Pediatric Asthma Health Outcome Measure,⁵⁴ the Paediatric Atopic Dermatitis QoL Measure,⁵⁵ and the Aberrant Behaviour Checklist Utility Index.⁵⁶ As there are currently only a limited number of condition-specific measures accompanied by preferences available for application in paediatric populations, these will be included in the review. Additionally, a recently developed cerebral palsy-specific measure that can be used in both child and adult populations will also be included.⁵⁷ Another measure that will be considered in the review is the PedsQL.⁵⁸ Although preference weights are not currently available for it, we are aware that these are presently being developed.⁵⁹

We will consult the websites of leading international organisations for HTA (National Institute for Health and Care Excellence (NICE), Pharmaceutical Benefits Advisory Committee (PBAC) regulation (FDA), health economics and outcomes research (ISPOR, WHO, International Society for Quality of Life Research (ISOQOL), National Institutes of Health (NIH), National Institute for Health Research (NIHR) for additional guidelines in relation to child and proxy completion of utility measures of HRQoL used in child populations. Specific website pages will be searched, including resources (good practice and health technology assessment documents), reports, publications, journal articles, research papers, newsletters and other publications. Despite the limits in searching on Google regarding geographical biases, language and lack of replicability, a list of target terms and words will be run in Google searches to discover grey literature in the form of web-published guidelines from official bodies. A data extraction template has been designed to extract data from web-based searches.

Search strategy

Electronic databases

A search strategy was developed with a medical subject librarian. Combinations of keywords, Medical Subject Headings, text words and other terms relevant to the review question were selected and adapted for each electronic database to enhance the search sensitivity and specificity.

The search will comprise of the following key terms: 'guidelines', 'self-report', 'proxy-report', 'child', 'adolescent', 'quality of life', 'utility', 'value'; 'preference-based

instruments' and 'preference-based measures'. The terms will be followed by appropriate truncation symbols, such as (* or \$). Boolean operators such as (AND/OR) and proximity searching will be used to further refinement. The search strategy is presented in online supplemental appendix 2. Forward (inspecting articles to ascertain if key articles have been cited) and backward (inspecting reference lists) citation checking will be conducted on all full texts to retrieve all relevant literature. A citation search will extend to include Google Scholar, and all papers identified will be cross-checked against the papers identified in the formal search.

Instrument developers and international organisation websites

A series of the search terms (in several combinations) will be entered into the Google search engine. Keyword search terms including 'guidelines', 'self-report', 'proxy-report', 'child', 'adolescent', 'QoL', 'utility', 'value'; 'preference-based instruments' and 'preference-based measures' will be used.

Data management

All search results from the selected electronic databases, including references, bibliographies and citations, will be retrieved in Endnote V.X9.3 (2020) reference management software and transferred to Covidence (www.covidence.org), an online screening and data extraction software for systematic reviews. Using Covidence, the review team will check for and remove all confirmed duplicate references. After that, all records will be independently screened for eligibility using the inclusion criteria.

Screening and data extraction

A structured three-stage approach will be used to screen articles obtained from electronic databases. First, all articles identified from the database search will be screened in triplicate by a team of three reviewers. Each reviewer will screen titles and abstracts to determine the eligibility of the articles. Second, following the initial screening, the full text will be retrieved for all references that meet the selection criteria, and these will be further screened against the eligibility criteria. Third, for articles that all three reviewers agree on, data will be extracted and entered into a table format. All the review stages (identification, screening and data extraction) will be cross-examined among the three reviewers. Discrepancies will be resolved by discussion among all the study reviewers. A PRISMA four-phase flow diagram will depict the flow of information through the different stages of the systematic review. The diagram will display information on the number of records identified from the literature searches, which will be based on inclusion criteria and the number of studies included and excluded and the justifications for exclusion. The selected guidelines will be assessed for quality.

The authors developed a data extraction template based on the keywords and search terms for each of the

three information sources: electronic databases, websites of available preference accompanied measures suitable for children, and websites of leading international organisations. A test screening of five articles from databases and five websites of instrument developers and international organisations was conducted by three independent authors to ensure that the data extraction template was adequate. The template showed good agreement between the raters.

Data from all information sources will be extracted based on a data extraction template developed for this purpose. The main outcomes of this study are to identify, appraise, compare and summarise all available guidelines for self and proxy report of paediatric HRQoL instruments. A table of the identified guidelines will be presented along with the summary findings from each of the three information sources. Guidelines for self or proxy completion of generic instruments from instrument developers via journal articles will be summarised and discussed in terms of (1) parameters for child self-report (eg, mode of administration and age of the child); (2) relevance of the content of the utility instruments for children of different ages or social backgrounds; (3) proxy type (parent/caregiver, teacher, clinician etc.); (4) equity considerations; (5) mode of administration (paper and pencil, computer-based, eg, tablets, laptops/desktop); (6) ease of administration; (7) descriptive system: Dimensions and item response level of the measures and recall period; (8) utility instrument scores reported; (9) details of valuation methods and scoring algorithm; and (10) details of how to obtain measure/instrument of interest.

Guidelines from instrument developers websites will be summarised and discussed in terms of the same items as the guidelines from journal articles. Additionally, we will collect data on how to obtain a value set for scoring algorithm, target users of the instrument and is the measure available at no cost versus if a fee is required to access it.

Guidelines from leading international organisations for HTA (NICE, PBAC), regulation (FDA), health economics and HRQoL outcomes research (ISPOR, WHO, ISOQOL, NIH, NIHR) will be synthesised according to the type of organisation, type of document (journal article), target group, purpose and guidance and recommendations.

Data synthesis

A summary of the included studies and measures will be presented following the best practice recommendations from the Cochrane Collaboration. The main aspects of the included studies, guideline descriptions and contexts in which they are applied, and information about measures will be summarised in a table.⁶⁰ All of the guidelines and recommendations of identified generic and condition-specific measures of HRQoL used in child populations will be assessed. Comparisons and disparities between these will be described.



Patient and public involvement

Funding bodies and the broader research community currently expect that researchers actively involve patients and the public in their research, including systematic reviews. Patient and public involvement in research has been proposed to add value to the synthesised research evidence, therefore addressing barriers to uptake evidence into practice.⁶¹

This paper describes a protocol for a systematic review nested within a broader research programme entitled ‘Quality of Life in Kids: Key evidence for strengthening decision making in Australia (QUOKKA)’. QUOKKA is informed and guided by input from both a decision makers’ panel, on which there is consumer representation and a consumer advisory group comprising parents and guardians of children with various chronic health conditions. The decision-makers’ panel consists of 15 members (6 females and 9 males), while the consumer advisory group comprises seven members (all female).⁶²

The decision-makers panel and consumer advisory group will be consulted at the literature search stage and interpretation and dissemination of results. We will try to keep the duration required for patient representative’s involvement to a minimum as most of them work voluntarily and care for children with various chronic health conditions.

DISCUSSION

To the authors’ knowledge, this will be the first systematic review to identify and synthesise all available guidelines and recommendations for self and proxy completion of generic and condition-specific paediatric HRQoL. Several electronic databases will be extensively searched from inception to date of search. Websites of available utility measures and those of leading organisations in the measurement and valuation of child HRQoL will also be consulted. The review findings will inform future research directions for informing policy and practice in relation to self and proxy completion of generic and condition-specific paediatric HRQoL measures accompanied by values. Some of the policy directions will help establish an understanding of, among many things: (1) the appropriate target populations, (2) the correct method of data collection, (3) their adequacy in measuring the outcomes required and (4) how to summarise and interpret responses to the measures correctly. The review will provide a comprehensive summary of the similarities and differences between existing guidelines for completing generic and condition-specific measures of HRQoL accompanied by values in paediatric populations.

A key strength is that reporting guidelines for both self and proxy-reported outcomes will provide a comprehensive assessment of existing paediatric measures accompanied by values, information that is currently lacking. Another strength is the comprehensive search strategy with keywords essential in measuring and valuing child HRQoL. The public and patient involvement through the

Decision Makers’ Panel and Consumer Advisory Group at several vital stages (literature search, particularly identifying grey literature and interpretation and dissemination of results will increase the comprehensiveness of the results).

A potential limitation of this review is that though language translation services will be used to interpret the guidelines published in non-English languages to English, there is a slight possibility that some of the translations may not be accurate. However, the bias will be reduced as we will use more than one method (a web-based translation tool and a professional language translation service) to translate the guidelines from languages other than English to English. We believe our conclusions will still be sufficiently accurate to provide a reliable position.

Acknowledgements We would like to acknowledge Shannon Brown, Flinders University, for providing helpful advice on strategies for the literature search. We would also like to thank the QUOKKA study investigators (Rosalie Viney, Deborah Street, Richard Norman, Brendan Mulhern and Harriet Hiscock) and research fellows (Tessa Peasgood and Cate Bailey) for their guidance in developing this review protocol.

Collaborators On behalf of the Quality Of Life in Kids: Key evidence to strengthen decisions in Australia (QUOKKA) project team.

Contributors JR, KD and GC conceptualised this study. CM-K wrote the first draft. JR, KD, GC and ND provided feedback on the first draft and agreed on the final draft. All authors reviewed and approved the final amendments. JR and CM-K will act as a guarantor of the review.

Funding This work was funded by the Department of Health, Australian Government, National Health and Medical Research Council. Grant number MRF1200816. Project title ‘quality of life in kids: key evidence for strengthening decision making in Australia (QUOKKA)’. The project aims to improve how quality of life is measured and valued within paediatric populations. The funder was not involved in developing this protocol.

Competing interests ND is a member of the EuroQol Group. All other authors declare no conflict of interest.

Patient consent for publication Not applicable.

Provenance and peer review Not commissioned; externally peer reviewed.

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