

# BMJ Open Health state utility values among children and adolescents with disabilities: protocol for a systematic review

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## ABSTRACT

**Introduction** Increasingly, assessment of healthcare technologies and interventions requires the assessment of both costs and utilities. Health state utility values (HSUVs) are measured using a range of generic and condition-specific measures. While reviews have identified that generic measures of HSUVs may lack validity in adults with conditions that result in physical disability, there is little information available on the methods used to obtain HSUVs in children and adolescents with disabilities. The objectives of this systematic review are to describe the methods used to obtain HSUVs, including mode of administration and psychometric properties, and provide summary statistics for HSUVs among children and adolescents with disabilities.

**Methods and analysis** The following databases will be searched from inception for English-language studies of any design: PubMed, PsychInfo, Medline, Scopus, CINAHL Plus, Econlit and EMBASE databases. Two reviewers will independently screen titles, abstracts and full text articles for studies reporting HSUVs and/or data on the psychometric properties of preference-based measures for children and adolescents with disabilities aged up to 19 years. Two reviewers will independently extract data items including descriptors of the study methods and sample, instruments used to capture HSUVs, summary statistics for HSUVs and items relating to the quality of reporting. A descriptive summary of results from included studies and summary statistics for HSUVs will be presented. If sufficient data is identified, we will pool summary statistics for HSUVs according to the method used to obtain the HSUV using a random effects model. In addition, we will explore the determinants of the HSUVs using a meta-regression.

**Ethics and dissemination** Ethical approval will not be required as no original data will be collected as part of this review. The completed review will be submitted for publication in a peer-reviewed journal and presentation at conferences.

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## BACKGROUND

Economic evaluations are increasingly used to inform the evidence base in decisions regarding the adoption of healthcare interventions. A cost-utility analysis (CUA), which

## Strengths and limitations of this study

- A strength of this review is that it will include studies of children and adolescents with a range of disabilities. Where possible results will be presented according to disability type.
- A limitation of this review is that publications that are not peer reviewed such as unpublished theses and conference presentations will not be included.
- This review will also only include reports written in English and therefore could exclude relevant information.
- It may not be possible to report summary statistics for health state utility values (HSUVs) if the methods used to obtain HSUVs are found to be inappropriate for use in this population.

describes the relationship between costs and health benefits (using quality of life), is commonly used in the appraisal of interventions and technologies. The strength of the CUA is the fact that costs are compared with quality-adjusted life years (QALYs), a measure that incorporates both the quantity and quality of life. By using QALYs, various disparate outcomes can be combined into a single composite summary outcome which allows broad comparisons across different disease areas in the health sector. When calculating QALYs, quantity is captured using life years while quality is captured using health state utility values (HSUVs). To enhance the transparency and hence reliability of evidence from CUA, it is key that the process of collecting data for the CUA is robust, transparent and systematic.<sup>1 2</sup> HSUVs are ranked on a scale anchored at 1 (full health) and 0 (a health state of equivalent value to being dead). HSUVs can be elicited using methods such as the Time Trade Off (TTO) methods or the Standard Gamble. Often generic measures, such as the EuroQol five dimensions questionnaire

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(EQ-5D), are recommended to be used in economic evaluations because they allow comparison across healthcare interventions and sectors.<sup>2</sup>

Disability is 'a difficulty in functioning at the body, person, or societal levels, in one or more life domains, as experienced by an individual with a health condition in interaction with contextual factors'.<sup>3</sup> Disability refers to the negative aspects of the interaction between individuals with a health condition (such as cerebral palsy, cardiovascular disease, depression) and personal and environmental factors (such as negative attitudes, inaccessible transportation and public buildings, and limited social supports).<sup>4</sup> Although the prevalence of disability varies between countries and within countries, depending on how disability is conceptualised and assessed,<sup>4</sup> the global prevalence among adults is approximately 15.6%.<sup>4</sup> Approximately, 5% of children aged 0–14 years worldwide experience a moderate or severe disability.<sup>4</sup> Research into the health needs, health outcomes, and effectiveness of interventions, particularly rehabilitation interventions, is a priority for improving healthcare among people with disabilities.<sup>4</sup> Encompassed in this is the need to evaluate the cost-effectiveness of healthcare interventions.

Condition-specific measures of quality of life are often used when assessing the effectiveness of interventions in children and adults with disabilities.<sup>5–7</sup> However, traditionally, generic preference-based measures such as the EQ-5D have been employed in CUA analyses and not condition-specific measures.<sup>2</sup> The validity of applying these generic measures, which have been developed in the general population, to groups with specific conditions has been debated.<sup>8–10</sup>

Several reviews have identified that generic measures of HSUVs may lack validity in adults with conditions resulting in physical disability.<sup>10–13</sup> There is a lack of information, however, regarding the utility and psychometric properties of measures used to obtain HSUVs in children and adolescents with disabilities. Understanding how HSUVs are obtained in children and adolescents with disabilities and the psychometric properties of these measures in this population is important for interpreting the findings of CUA of health interventions.

## OBJECTIVES

This review will address the following objectives:

1. Describe the methods used to obtain HSUVs from children and adolescents with disabilities.
2. Describe how these methods are administered to children and adolescents with disabilities.
3. Describe the psychometric properties of the methods used to obtain HSUVs from children and adolescents with disabilities.
4. Report summary statistics for HSUVs among children and adolescents with disabilities obtained from each method identified.

## METHODS

The methods used for this systematic review will be in line with available recommendations on reviews of HSUVs.<sup>14–18</sup> In addition, a scoping review was conducted to inform the methods of this review. Reporting of the review will adhere to recommendations of the preferred reporting items for systematic reviews and meta-analyses (PRISMA) statement.<sup>19</sup> The protocol will be registered with the International Prospective Register of Systematic Reviews following peer review.

### Study eligibility

#### Study designs

We will include: (1) studies reporting HSUVs among children and adolescents with disabilities derived from both direct (such as Standard Gamble, TTO, visual analogue scale) and indirect methods (such as EuroQol EQ-5D and all its variants, Child Health Utility 9D (CHU-9D), Assessment of Quality of Life 6D, Paediatric Asthma Health Outcome Measure, Health Utilities Index Mark 2, Quality of Well-Being Scale and 16D-questionnaire) and (2) studies reporting the utility and/or psychometric properties of measures used to obtain HSUVs from children and adolescents with disabilities.

There will be no restriction on the type of studies to be included. Examples of types of studies to be included in the review are:

- Studies that compare existing or new measures of HSUVs.
- Studies that validate measures of HSUVs.
- Randomised clinical trials that incorporate cost utility assessments.

### Participants

The review will include studies reporting HSUVs for children and adolescents with disabilities aged 0–19 years. We will include studies reporting HSUVs for the general population if they report HSUVs for children and adolescents with disabilities separately. Studies of children and adolescents aged 0–19 years with the following broad forms of disabilities will be included: (1) intellectual impairment (eg, learning difficulties) or intellectual disability, (2) physical impairment (eg, mobility impairment, poliomyelitis), (3) developmental disability (eg, autism spectrum disorder or cerebral palsy), (4) sensory impairments (eg, visual impairment, speech impairment) and (5) multiple impairment—at least two of the above.

Where studies include children, adolescents and adults, for example participants aged 5–20 years, or adolescents and adults, for example participants aged 15–20 years, we will extract data on children and adolescents separately if possible. If this is not possible, we will include the study in the descriptive analysis but not in the quantitative analysis. Where studies include adults with disabilities, we will interpret the results of these studies in the context of the proportion of children and/or adolescents included in the study.

## Language

We will include articles reported in the English languages only.

## Exclusion criteria

We will exclude studies reporting HSUVs in adults with disabilities only or in children or adults without disabilities. We will also exclude reviews, commentaries, unpublished theses, conference abstracts and any unobtainable texts.

## Search strategy

A systematic search will be conducted to capture HSUVs used in children and adolescents with disabilities. The following databases will be searched from inception: PubMed, PsychInfo, Medline, Scopus, CINAHL Plus, Econlit and EMBASE. We will also search for studies on the EQ-5D, Health Utilities Inc, National Health Service Economic Evaluation Database and Health Technology Assessment websites. Reference lists of key papers will be reviewed for additional references.

## Search terms

The development of the search terms was informed by a scoping review of the literature.<sup>20–26</sup> Specific search terms include different variants of child and adolescent terms (infant, newborn, child and adolescent), health utility terms (EQ-5D, TTO, Standard Gamble) and disability. A sample search strategy is provided in the online Supplementary appendix 1.

## Data management

Literature search results will be managed using Mendeley reference management software.

## Study selection

Two reviewers will review the titles and abstracts for inclusion. If a study appears to meet the inclusion criteria or if there is any doubt regarding the inclusion of the study the full text of the article will be retrieved. Full text articles will be reviewed independently by both reviewers and disagreements resolved through discussions with a third reviewer. Reasons for exclusions will be documented for all full text articles. The PRISMA flow diagram<sup>19</sup> will be used to summarise the number of articles identified, retrieved, screened, assessed, included and excluded as well as the reasons for exclusions.

## Data extraction

Data extraction will be conducted independently by two reviewers. Disagreements between the two reviewers will be resolved primarily through discussions and where necessary a third reviewer will be involved. If HSUVs are not available from the study report, we will contact the authors. A period of 2 months from the request date will be allowed for the author to respond.

## Data items

Details of the data extraction items are provided in the online Supplementary appendix 2. The data

extraction form has been piloted by two reviewers (LK and JR). Data extraction items include a description of the study methods, sample and results as well as items relating to the quality of reporting. As this review will identify patient-reported outcome measures used to obtain HSUVs in children and adolescents with disabilities, the International Society for Quality of Life Research (ISOQOL) minimum standards for patient-reported outcome measures<sup>27</sup> was used to inform data extraction items. These items include information on the reliability, validity and burden of the patient-reported outcome measure, which will aid identification of suitable outcome measures as well as highlight improvements in reporting and additional research that are required in the field. Further, the Checklist for REporting VALuaTion StudiEs (CREATE) checklist<sup>28</sup> was consulted when developing the data extraction items. As the objectives of this review are broad, all items on the CREATE checklist may not be applicable to all included studies and therefore the checklist as a whole will not be used. Instead, items from the CREATE checklist informed the data to be extracted such as a description of the attributes of the instrument, the sampling method, the response rate and reasons for excluding any respondents or observations. The items extracted will include:

- ▶ A description of the study background, aims and methods: funding sources, conflicts of interest, statement of ethical approval, aim of study, study design, duration of study, duration of participation, study setting, method of recruitment, sample size, sampling method, number randomised to each group and description of intervention and comparator (if study is a trial).
- ▶ A description of the participant (and respondent, if different) characteristics: age, sex, race, socioeconomic status, diagnosis, type of disability, years in present disability, disability severity, other medical conditions and years in this condition. As there is not a standard method of categorising severity across all disability types, the method used to categorise severity will be extracted from each article.
- ▶ A description of the methods used to obtain HSUVs: instrument (eg, CHU-9D) or direct elicitation technique (eg, TTO) used, mode of administration (eg, telephone, face-to-face), data source (eg, self-reported or proxy), relationship between participant and respondent if administered by proxy, time points measured and reported and length of time to complete the instrument or administer the method to elicit HSUVs.
- ▶ A description of the psychometric properties of the instrument in children and adolescents with disabilities: a statement of validity, reliability and responsiveness (if longitudinal study); methods used to determine psychometric properties and statement of differences between participants and target population if study is examining psychometric properties of the instrument



- A description of the results of the study: response rates, reasons for missing data, reasons for exclusion of respondents or observations and summary statistics for HSUVs.

## Data synthesis

### Descriptive analyses

A summary of the results from the included studies will be presented according to disability type (intellectual impairment or disability, physical impairment, developmental disability, sensory impairments, multiple impairments) and instrument used to obtain HSUVs if a sufficient number of studies for each type is identified. In addition, all identified methods of obtaining HSUVs and the relevant attributes of their associated studies will be tabulated. We will also report the number of studies where data extraction items were not available in order to provide an overview of the quality of reporting by study authors.

### Quantitative analyses

If we identify sufficient data of sufficient quality for a meta-analysis, we will pool summary statistics for HSUVs according to the method used to obtain the HSUV using a random effects model. We will only include data obtained using methods that are identified as having adequate validity and reliability among children and adolescents with disabilities. Heterogeneity between the findings of the reviewed studies will be assessed using the  $I^2$  statistic. In addition, we will explore the determinants of the HSUVs, including disability type, using a meta-regression. Statistical analyses will be performed using STATA software V.14.

## Ethics and dissemination

Ethical approval will not be required as no original data will be collected as part of this review. If a quantitative analysis is conducted, it will rely entirely on data extracted from published studies. The completed review will be submitted for publication in a peer-reviewed journal and presented at conferences.

## DISCUSSION

The principal objectives of the planned systematic review are to evaluate how HSUVs are obtained in children and adolescents with disabilities, report the psychometric properties of measures used to obtain HSUVs among children and adolescents with disabilities and if appropriate, provide summary statistics for HSUVs in this population. We will collate this data to identify areas that require further research regarding the measurement of HSUVs in children and adolescents with disabilities.

Previous literature suggests that adult-specific methods of obtaining HSUVs are used in studies of children,<sup>20 29 30</sup> even though the utility weights obtained from these methods have not been adapted to incorporate the possibly different child and adolescent preferences.<sup>29 30</sup>

These issues may be compounded by potential problems with administering generic measures to people with disabilities<sup>10–13</sup> resulting in inaccurate CUA among children and adolescents with disabilities and poorly informed decisions regarding the adoption of new interventions in this population.

The intended audience of this review therefore goes beyond health economists to guideline developers, policymakers, clinicians and researchers. In summarising the instruments that have been validated for use in this population group, we will also highlight those that have not yet been validated. We hope that the identification of these knowledge gaps will encourage and direct future instruments validation work.

While it is anticipated that this review will provide useful information on measures used to obtain HSUVs in children and adolescents with disabilities, there are a number of limitations with the proposed review. First, as reports that are not peer reviewed and are not written in English will be excluded from the review, relevant information may not be included. This may also reduce the generalisability of the findings to specific populations of children and adolescents. Further, it may not be possible to report summary statistics for HSUVs if the methods used to obtain HSUVs are found not to have adequate validity and reliability among children and adolescents with disabilities. It may also not be possible to determine if disability type predicts HSUVs if insufficient data for each disability type is identified.

**Contributors** LK, NA and JMR developed the idea for the review. LK wrote the first draft. NA and JMR revised the protocol. JMR will act as guarantor of the review.

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**Competing interests** None declared.

**Patient consent** Not required.

**Provenance and peer review** Not commissioned; externally peer reviewed.

**Data sharing statement** No original data will be generated from this review.

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