



Valuing health in children – where are we now, and what further work is needed?

Koonal Shah, PHMR, UK

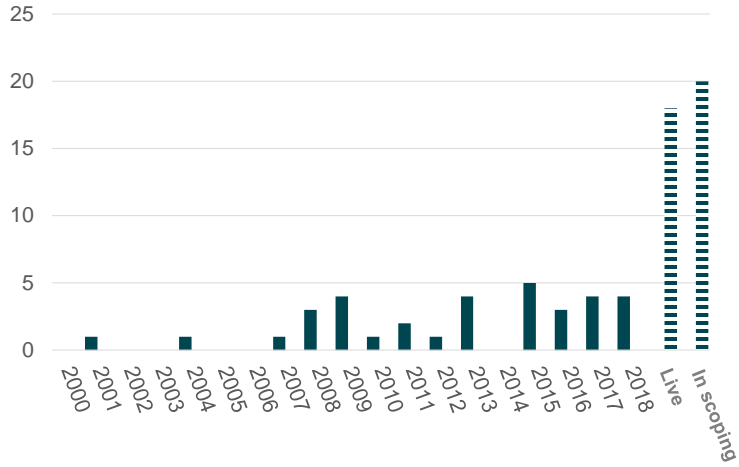
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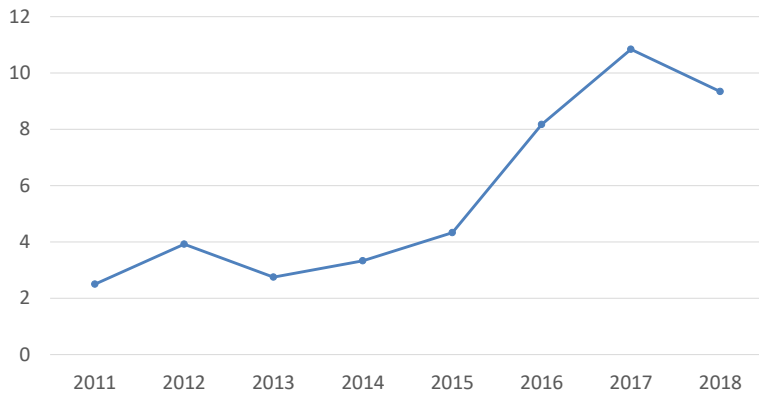
The issue

- Increasing research and HTA interest in measuring and valuing the health of children and adolescents
- To facilitate the evaluation of health technologies for younger patients, utility values are needed for preference-based measures that reflect the value of health in children and adolescents
- However, valuing health in children is challenging
- To date, HTA agencies have not provided specific guidance
- Literature is thin, but there is a growing interest in child health valuation issues

NICE evaluations that include children



EQ-5D-Y licence requests (average per month)



Valuation challenges

- Normative issues (*whose preferences* should we elicit?)
- Perspective issues (*whose health* should we elicit the preferences for?)
- Methods issues (*how* do we elicit the preferences, and on what basis do we make this choice?)
- Consistency issues (what are the implications if methods/values for children differ from those for adults?)

Which approaches have been used to date?

Instrument	Country	Sample	Perspective	Elicitation method
AHUM	UK	Adults	Self	Time trade-off
AQoL-6D	Australia, Fiji, New Zealand, Tonga	Adolescents	Self	Time trade-off
CHU-9D	Australia	Adolescents	Self	BWS (time trade-off anchor)
CHU-9D	China	Adolescents	Self	BWS (time trade-off anchor)
CHU-9D	Netherlands	Adults	Self	Discrete choice experiment
CHU-9D	UK	Adults	Self	Standard gamble
EQ-5D-Y	Australia	Adults	Child	Discrete choice experiment
EQ-5D-Y	UK	Adults, adolescents	Child, self	Discrete choice experiment
EQ-5D-Y	UK	Adults	Child, self	Rating scale, time trade-off, discrete choice experiment, personal utility function
EQ-5D-Y	USA	Adults	Child	Discrete choice experiment
HUI2	Canada	Adults – parents	Child	Standard gamble / rating scale
HUI2	UK	Adults	Child	Standard gamble / rating scale
HUI3	Canada	Adults	Self	Standard gamble / rating scale
QWB	USA	Adults	Self	Rating scale
16D	Finland	Adolescents	Self	Rating scale
17D	Finland	Adults	Child	Rating scale



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Panellists

- **Donna Rowen**, Senior Research Fellow, University of Sheffield, UK
- **Elly Stolk**, Scientific Team Leader, EuroQol Research Foundation, Netherlands
- **Rosie Lovett**, Senior Scientific Advisor, National Institute for Health and Care Excellence, UK



Valuation methods of preference-based measures of health for economic evaluation in child and adolescent populations: A cautionary tale

Donna Rowen
University of Sheffield



Is the valuation of child and adolescent health important?

- Currently there is limited guidance from international agencies
- This is important for the measures that are used and how they have been valued
- E.g. Recent review identified 31 HTAs submitted to NICE where the licensed indication for the technology included people under 18 years (Hill et al, 2019)
- Of these 7 used a child and adolescent-specific preference-based measure, all also used another method
- Not aware of any evidence base demonstrating that adult preference-based measures (such as EQ-5D) appropriately and accurately capture the health and quality of life of children and adolescents
- Research has suggested that adult value sets are inappropriate for use with child health states (in the context of EQ-5D-Y) (Kind et al, 2015; Kreimeier et al, 2018)





Why is the valuation of child and adolescent health different?

- In general adult measures are valued by general population adults imagining hypothetical health states for themselves
 - Tax-payers
 - Democracy
 - Veil of ignorance
- Child measures – should it be adults or children?

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Whose preferences?

- Normative question
- Practical difficulties
- Consistency and comparability with utility values for adult health states

Adults

- Potential consistency of methods to adult health states – but not comparability of values
- Cognitive understanding but may not understand the health states

Adolescents

- Potential inconsistency of methods to adult health states – potential incomparability of values
- Ability to understand, imagine and choose
- Issue of 'dead'

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Issues for concern when eliciting values from adults

- Which perspective?
- **Own health**
 - Veil of ignorance
 - Yet for some dimensions this is not appropriate e.g. schoolwork, daily routine
- **10 year old child**
 - Which child may matter (and which age)
 - Trade-off between length of life and quality of life may differ
 - Participants' views about children and child health
 - Potentially views about answering questions for an 'other'



Which perspective? A cautionary tale

- Adults valuing health states from the perspective of a 10 year old child
- Research using visual analogue scale (VAS) found that values elicited using an adult own health perspective were higher than values elicited considering the perspective of a child (Kind et al, 2015)
 - i.e. **lower** utility when health state is valued for a child
- Research using time trade-off (TTO) found the opposite (Kreimeier et al, 2018)
 - i.e. **higher** utility when health state is valued for a child
- Research using TTO, DCE, VAS, and PUF also found the same (higher utility when valued for a child) (Ramos-Goni et al)





Perspective of a 10 year old child



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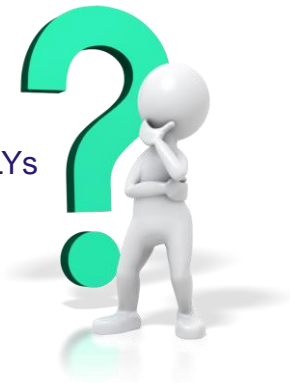


Perspective of a 10 year old child

Do the preferences only reflect the quality of life of the health state?



Use to generate QALYs



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Perspective of a 10 year old child

Do the preferences only reflect the quality of life of the health state?

Do the preferences reflect how bad (or good) it is for a child to experience ill health?

Use to generate QALYs



Should this be outside the generation of QALYs?

QALY weighting?

Deliberative process?

Where are we going?

- Need better understanding of how the following impact on the way members of the adult general population value states for children and adolescents:
 - Perspective
 - Elicitation technique
 - Wider views around child and adult health
- Qualitative research is needed (EuroQol funded project examining this), quantitative research alone cannot answer this question
- Need better understanding of how to frame tasks for adolescents including mention of dead, and issues around anchoring onto 1-0 full health-dead scale
- “Safe” option may be to use adult values for own health
 - But “safe” does not necessarily mean it is the best option



The EuroQol Group's initial experiences with valuing child health

Elly Stolk

EuroQol Group



Introduction

- In 2009, the EQ-5D-Y to measure child health was presented
- The next few years were used to address key questions:
 - Are child specific value sets needed?
 - What valuation methods will work?
- Based on the results in 2018, a preliminary valuation protocol was delineated
- In 2019, two countries launched a valuation study
- Today we reflect on the results

Are child specific value sets needed?

- Kreimeier et al (2019) compared values for EQ-5D and EQ-5D-Y, using the cTTO method to obtain values. Key findings:
- People attach a different value to the health states of both descriptive systems, even when they apply to the same person (the 'wording' matters).
- If one set of descriptors was framed to concern children and the other adults, that increased the differences (the 'person' matters)
- So **yes**, separate value sets for children are needed

What methods will work?

- Kreimeier found high values for EQ-5D-Y states compared to values for adults. An earlier VAS study (no dead anchor) suggested lower values for Y
- Ramos Goni et al investigated the difference in health state values for children and adults across a wider set of methods (TTO, DCE, VAS, and PUF).
- They found that **all** methods produced higher values for children than for adults, when measured on the 0 (dead) – 1 (full health) scale
- Given the consistent results across methods, EuroQol opted to value child health also by TTO, to promote comparability with adult value sets

Key difference EQ-5D and EQ-5D-Y valuation protocols

	EQ5D adults	EQ5DY children
Who values health?	Adults	Adults
For whom?	For themselves	For a hypothetical child (10 years of age)
What method?	TTO	TTO

- Self-report valuation for adults; proxy-report valuation for children

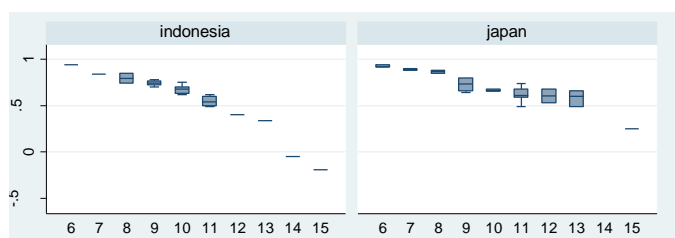
EQ-5D-Y valuation protocol

- Restricted to 3L version of EQ-5D-Y
- Combine DCE and TTO responses to make studies less complex and costly
 - **TTO**: values for at least 10 states from at least 150 people;
 - **DCE**: 150 pairs presented to a sample of n=1000
- Regress DCE onto TTO to inform the value set
 - Following Rowen et al, MDM 2015 Apr;35(3):328-40.

Valuation studies EQ-5D-Y 2019

- Data collection has been completed in two countries: Japan and Indonesia:
- Japan: n=1000, each participant received 15 DCE pairs (10 blocks) and 6 TTO tasks (5 blocks)
- Indonesia: n=1000 participants received 15 DCE pairs (10 blocks) and n=200 completed 12 or 13 TTO tasks (2 blocks).

TTO values by level sum score



- Values have good face validity, but the EQ-5D-Y values are high compared to EQ-5D 3L and 5L for adults, e.g.
- Worst state EQ-5D-Y versus EQ-5D-5L in Japan: 0.20 versus -0.025
- Worst state EQ-5D-Y versus EQ-5D-5L in Indonesia: -0.185 versus -0.865

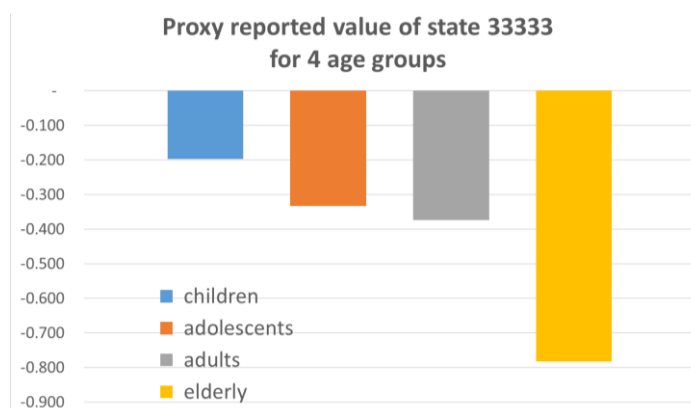
Poll

- Please consider: TTO values for Y states are higher than values for corresponding states in adults. For example: $EQ-5D-Y_{22222} > EQ-5D_{22222}$
- What can we conclude?
 - A. Adults in state 22222 have better quality of life than children in the same state
 - B. Children in state 22222 have a better quality of life than adults in the same state
 - C. Quality of life of adults and children cannot be compared in this way

Why are TTO values different? Two plausible explanations:

- Problems affect QOL of a child less
 - Children are surrounded by people who are keen to make life work for the child, regardless of health. Independence is not expected.
 - QOL child > QOL adult is plausible
- Or, years are valued differently
 - We establish QOL weights by measuring how many years are traded off. People may need to pass a higher severity threshold to make the same time tradeoff for children.
 - Values are calibrated differently. Equal value \neq equal QOL ($0.7 \neq 0.7$)

Same phenomena will affect proxy-reported values for elderly (*preliminary results from China, dr. Pei Wang*)



Conclusions

- The proxy reported values for EQ-5D-Y are valid, and may be used for the intended use of computing QALYs
- QOL weights obtained for child and adult health at the scale required for QALY computation may not be directly comparable
- Can we study how TTO values are calibrated to restore comparability?
- Major challenge remain for the HTA research community.

The case for clearer methods guidance



Children aren't little adults



Parents want children's health-related QoL to be reflected adequately



Clearer methods guidance should lead to more consistent submissions



Patient and public involvement policy: 'guidance and standards...covering children and young people...informed and influenced by their views and experiences'



Wider review includes related issues: carer quality of life and rare diseases

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Challenge 1: which instrument(s), completed by whom?

•Limited evidence on psychometrics

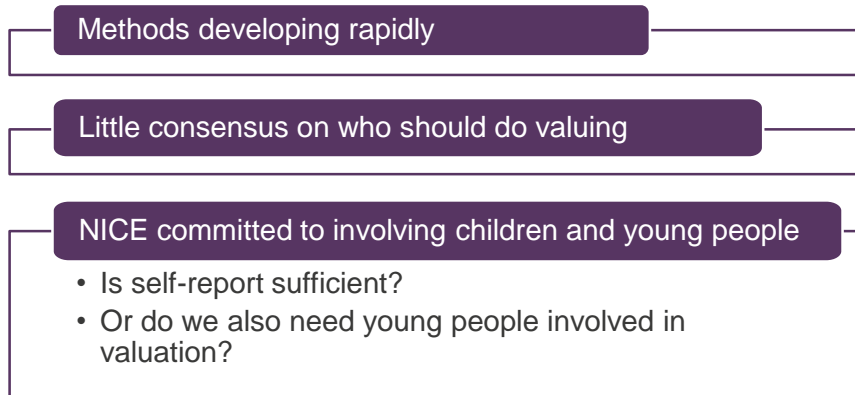
- Most evidence for EQ-5D-Y and CHU9D
- Very few head-to-head comparisons
- Wide variation in methods, difficult to synthesise and compare each measure

Self or proxy?

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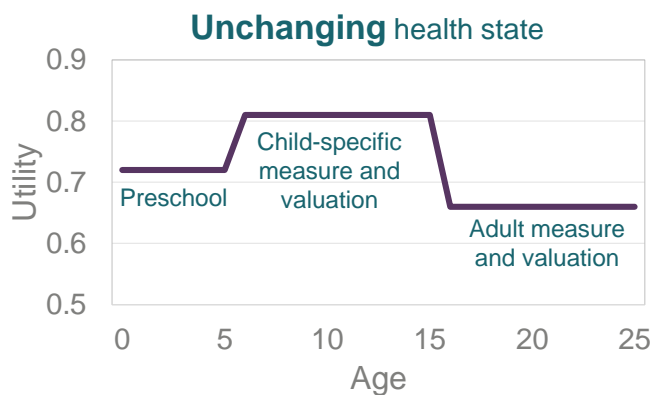
Challenge 2: valuation



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Challenge 3: consistency within models

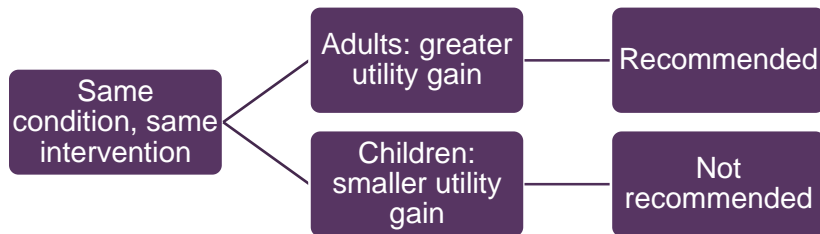


- Pass the Amanda Adler test?

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Challenge 4: consistency in decision making



- If different recommendations arise:
 - Are they justified?
 - Are the reasons understood and supported by public?

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Research priorities



Explore why adult valuations depend on perspective



Psychometrics



Engagement with patients, the public, industry, other HTA agencies, regulators

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Proposed ISPOR taskforce

- We are planning to propose an ISPOR taskforce to provide emerging good practice recommendations around the valuation of health and quality of life in children and adolescents to generate QALYs
- If you would be interested in participating as a reviewer, please contact:
 - Donna Rowen: d.rowen@sheffield.ac.uk
 - or Koonal Shah: koonalshah@phmr.com

Polling instructions

- Go to the ISPOR Europe app
- Navigate to **Menu**
- Then to **Live Polling**
- This will take you to an online list of sessions
- Scroll down to **IP23**
- When each poll is live, choose your preferred option
- Don't forget to press 'Submit'!
- You can also submit and vote for questions using the **Social Q&A** feature

The screenshot displays the ISPOR Europe app interface. At the top, a navigation bar includes icons for Schedule, My Schedule, My Favorites, Notifications, Search, Now, and Menu. The Menu icon is circled in red. Below the navigation bar, a sidebar menu lists various options: Sponsors, Favorites, Schedule, Contacts, Notifications, Technical Support, and User Gateway. The 'Live Polling' option is highlighted with a red box. Below the sidebar, instructions are provided: 'Click + to add an item to 'My Schedule'.' and 'Click ★ to add/remove an item to 'My Favorites'.'. On the right side, a list of sessions is shown, with the session 'IP23: Valuing Health In Children – Where Are We Now, And What Further Work Is Needed?' highlighted with a red box. The session details include the time '2:00pm – 3:00pm'.

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Whose preferences should form the basis for the values for a child health outcomes instrument?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Suppose a given health state H has been valued using time trade-off both for adults and for children, and the child value exceeds the adult value. What can we conclude?

Live Content Slide

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Poll: Should NICE work towards broad or specific methods guidance?

Polling question 1 (backup)

Whose preferences should form the basis for the values for a child health outcomes instrument?

- A. Preferences of adults
- B. Preferences of children
- C. Preferences of both adults and children

Polling question 2 (backup)

Suppose a given health state H_i has been valued using time trade-off both for adults and for children, and the child value exceeds the adult value.

What can we conclude?

- A. Adults in H_i have better quality of life than children in H_i
- B. Children in H_i have a better quality of life than adults in H_i
- C. The quality of life of adults and children cannot be compared in this way

Polling question 3 (backup)

Should NICE **work towards** broad or specific methods guidance?

- That is:
 - Broad guidance on **desirable characteristics** of descriptive system(s) and valuation studies; or
 - Specific guidance on **preferred descriptive system(s) and value set(s)**
- A. Broad guidance
- B. Specific guidance

Suggested questions for discussion

- How important is it that the methods for valuing health in children are consistent with those used to value health in adults?
- Is it possible to reach consensus on the normative issues? Which stakeholders should be involved in the process? What method should be used to achieve consensus?
- Should further work be undertaken to understand the preferences of children and adolescents?
- What research would help HTA agencies to make methodological recommendations?
- How can we better understand how quality of life weights obtained on the full health-dead scale are calibrated differently for adults and children?