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

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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

Source: NICE (Rosie Lovett)

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

Source: EuroQol Office (Gerben Bakker)
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?

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

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’
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

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

Use to generate QALYs
**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
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

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<thead>
<tr>
<th></th>
<th>EQ5D adults</th>
<th>EQ5DY children</th>
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</thead>
<tbody>
<tr>
<td>Who values health?</td>
<td>Adults</td>
<td>Adults</td>
</tr>
<tr>
<td>For whom?</td>
<td>For themselves</td>
<td>For a hypothetical child (10 years of age)</td>
</tr>
<tr>
<td>What method?</td>
<td>TTO</td>
<td>TTO</td>
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- 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
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 ≠ equal QOL (0.7 ≠ 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.
Valuing health in children: a NICE perspective

Rosie Lovett, Senior scientific adviser
ISPOR Copenhagen, November 2019

Most paediatric evaluations use adult EQ-5D

- Half of evaluations only use adult EQ-5D questionnaire and valuation set
- The rest:

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

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?
Challenge 2: valuation

- Methods developing rapidly
- Little consensus on who should do valuing
- NICE committed to involving children and young people
  - Is self-report sufficient?
  - Or do we also need young people involved in valuation?

Challenge 3: consistency within models

Unchanging health state

- Pass the Amanda Adler test?

NICE
Challenge 4: consistency in decision making

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

Research priorities

- Explore why adult valuations depend on perspective
- Psychometrics
- Engagement with patients, the public, industry, other HTA agencies, regulators
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.
Poll: Whose preferences should form the basis for the values for a child health outcomes instrument?

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?
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?