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OPEN MEETING

ISPOR Good Practice Task Force Recommendations: *Valuing HRQoL of Children & Adolescents in Economic Evaluation (Pediatric Utilities)*

ISPOR Europe 2023

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Discussants

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Pediatric Utilities:

An introduction to the Taskforce

Professor Donna Rowen, University of Sheffield,
United Kingdom

Current practice in use of pediatric utilities in CEA

PBAC: “Submissions involved inconsistent approaches, use of adult measures and weights, and substantial gaps in evidence”
[\(Bailey et al 2021\)](#)

NICE: “...most used generic HRQoL measures designed for adults. Measures were usually completed by adult patients or clinical experts. Committees frequently commented on limitations in the HRQoL data”
[\(Lamb et al 2021\)](#)

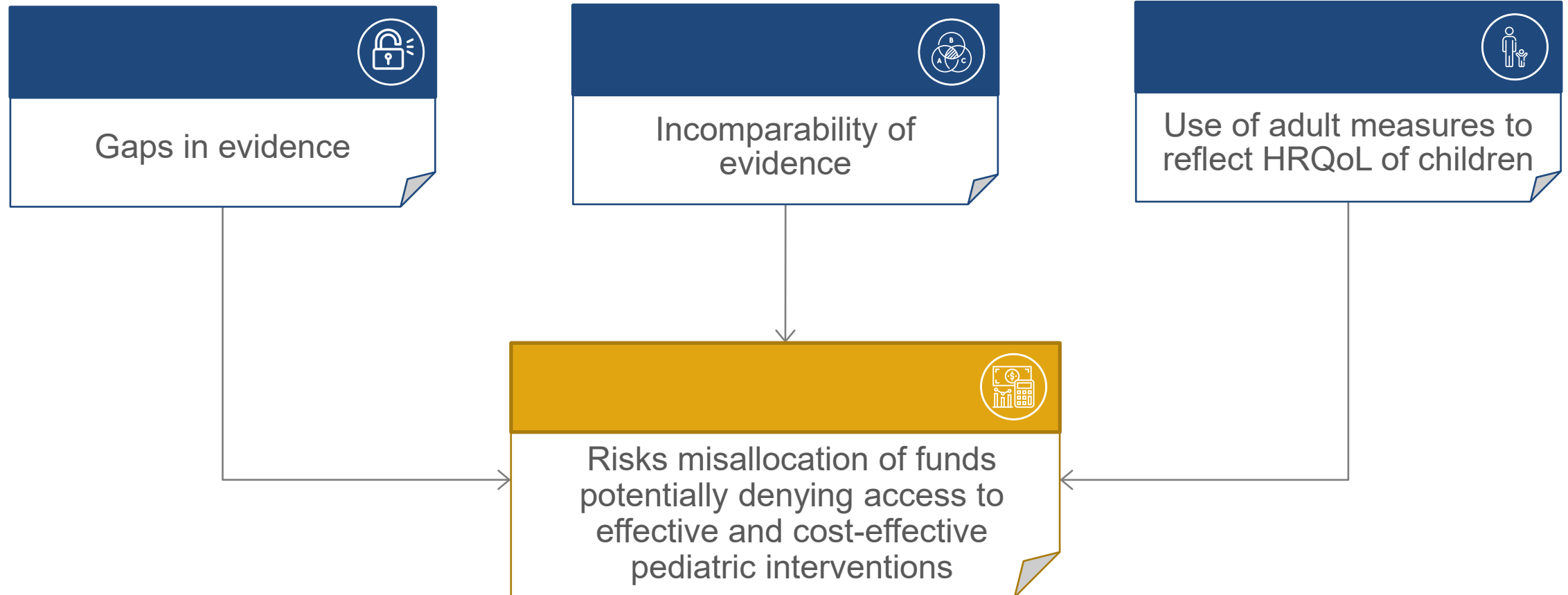
25% of assessments used child and adolescent population–specific measures [\(Hill et al, 2020\)](#)

“...we found considerable gaps and weaknesses in the current evidence base for utilities used in economic evaluations of pediatric vaccines.”
[\(Herdman et al 2016\)](#)

“...considerable gaps in the way (pediatric) valuation weights are used and reported in CEA” [Neppelenbroek et al 2023\)](#)

Growing awareness by HTA bodies – but currently no HTA body provides clear guidance on child HRQoL utilities [\(Devlin, Rowen, Lovett et al 2022\)](#)

Why is this important?



Why pediatric utilities?

- Historically neglected area of research
- For use in health technology assessment, health state utilities are required
- No common methods
- Value judgements underlying the choice of methodology (no “right” answer)

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<https://doi.org/10.1007/s40273-021-01128-0>

SYSTEMATIC REVIEW



Systematic Review of Conceptual, Age, Measurement and Valuation Considerations for Generic Multidimensional Childhood Patient-Reported Outcome Measures

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<https://doi.org/10.1007/s40273-019-00873-7>

REVIEW ARTICLE



Review of Valuation Methods of Preference-Based Measures of Health for Economic Evaluation in Child and Adolescent Populations: Where are We Now and Where are We Going?

Donna Rowen¹ · Oliver Rivero-Arias² · Nancy Devlin³ · Julie Ratcliffe⁴

Published online: 6 January 2020
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Valuing HRQoL of Children & Adolescents in Economic Evaluation (Pediatric Utilities)

Task Force

► Special Interest Groups

▼ Task Forces

Performance Outcome (PerfO) Assessments

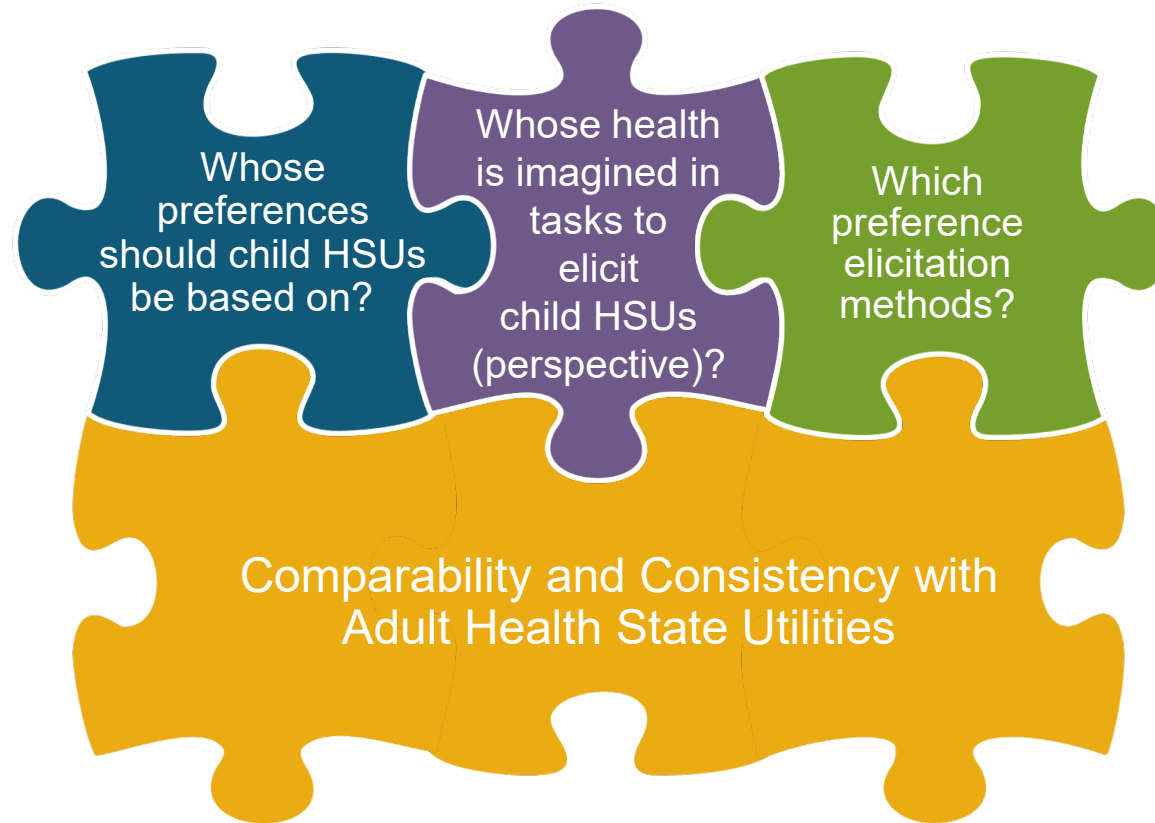
Structured Expert Elicitation for Healthcare Decision Making

Objective

To develop emerging good practice recommendations for the valuation of health related quality of life (HRQoL) in children and adolescents for the generation of quality adjusted life years (QALYs) for use in economic models.

Task force report structure

Introduction and Aims



Selecting the overall approach to generating child HSUs

Recommendations

Pediatric utilities task force members

Donna Rowen, PhD, (Co-Chair), Professor of Health Economics, University of Sheffield, Sheffield, UK

Nancy Devlin, PhD, (Co-Chair), Professor in Health Economics, University of Melbourne, Melbourne, Australia

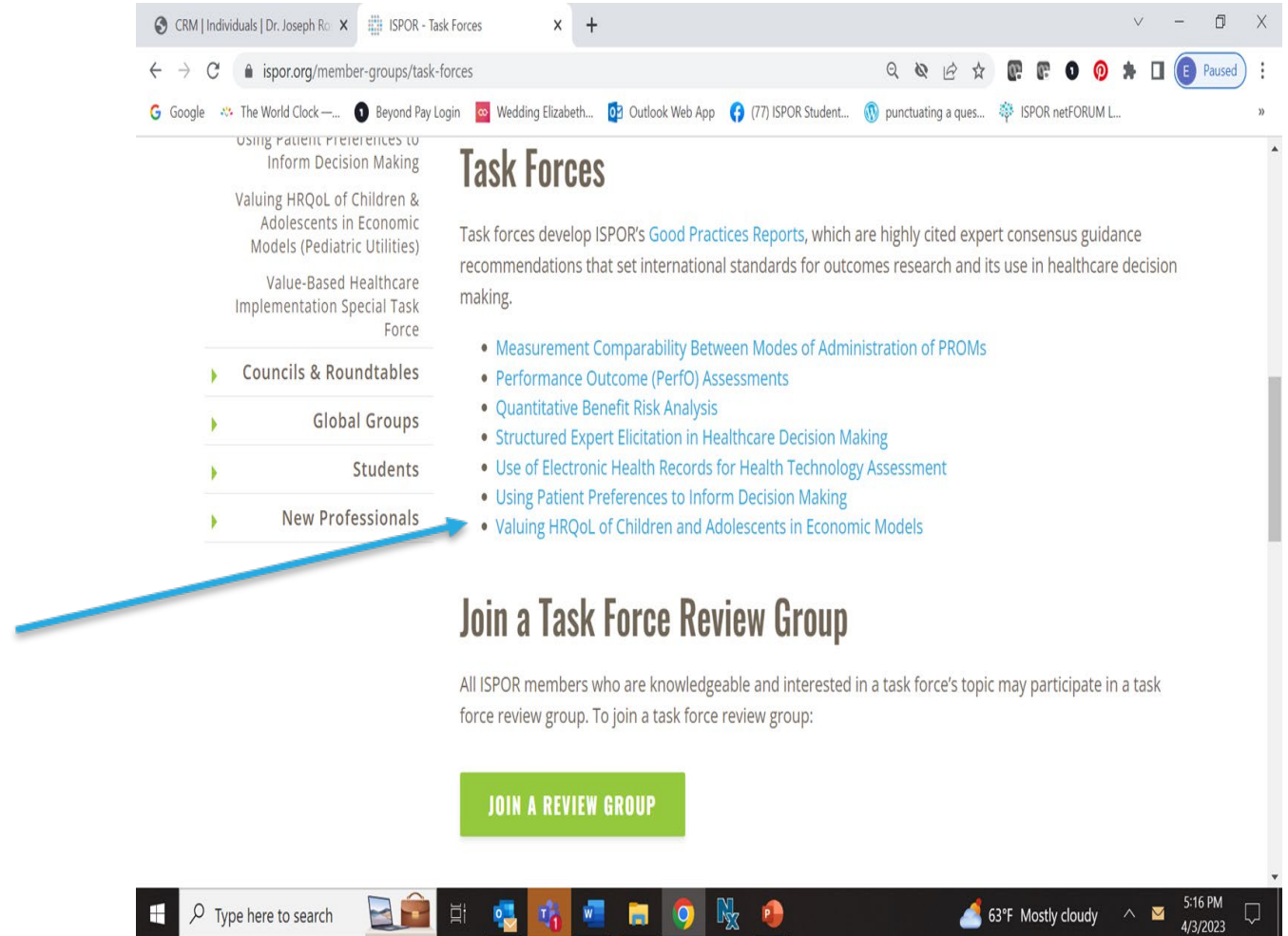
- **Fleur Chandler, MSc**, Head of Market Access UK and Ireland, Sanofi, Reading, England, UK; Patient Advisory Board Lead, Duchenne UK
- **Kim Dalziel, PhD**, Associate Professor and Head of the Health Economics Group, University of Melbourne, Melbourne, Australia
- **Salah Ghabri, PhD**, Senior Scientific Referent in Health Economics, Department of Medical Evaluation, Haute Autorite de Sante (HAS), Paris, France
- **Ernest Law, PhD**, Senior Manager, Global Health Economics and Outcomes, Pfizer, New York, NY, USA
- **Louis Matza, PhD**, Senior Research Leader, Patient-Centred Research, Evidera, Bethesda, MD, USA
- **Lisa Prosser, PhD**, Professor & Director, Susan B. Meister Child Health Evaluation & Research Center, Dept of Pediatrics, Professor, Dept of Health Management & Policy School of Public Health, University of Michigan Office of Research, Ann Arbor, MI, USA
- **Oliver Rivero-Arias, PhD**, Associate Professor of Health Economics, University of Oxford, Oxford, England, UK
- **Koonal Shah, PhD**, Associate Director, National Institute for Health and Care Excellence (NICE), London, England, UK
- **Elly Stolk, PhD**, Scientific Director, EuroQol Research Foundation; Professor in Health economics, Erasmus University Rotterdam
- **Jonathon (Jo) Wolff, PhD**, Professor, University of Oxford, Oxford, England, UK

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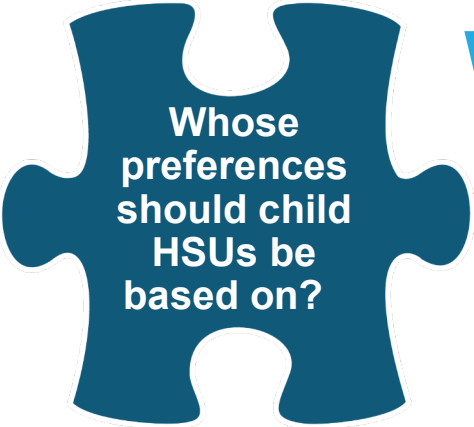
Likely timeline for review round: Q1 2024



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Whose preferences should pediatric HRQoL utilities reflect?

Elly Stolk, Professor at Erasmus University Rotterdam, NL, and Scientific Director, EuroQol Research Foundation, Rotterdam, NL



Whose
preferences
should child
HSUs be
based on?

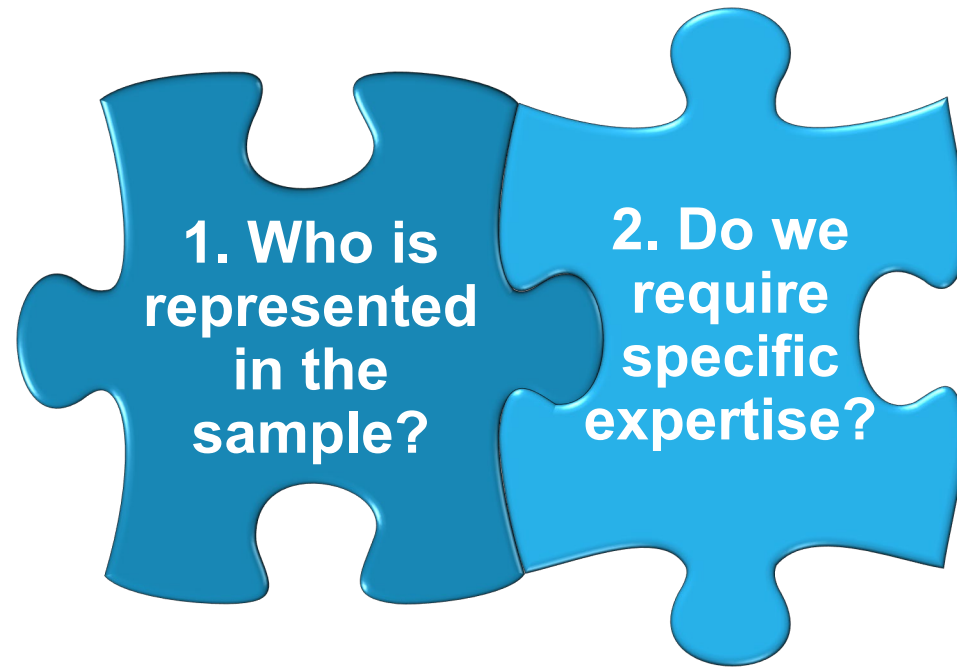
Whose preferences?

- General public preferences are in most jurisdictions considered more appropriate than patient values. Reasons include ([Versteegh & Brouwer, 2016](#)):
 - Resource allocation of scarce resources affects the entire population
 - Insurance perspective
 - Inclusiveness and representation
- So, we already know whose values count in valuing child health? No!
- These normative/theoretical considerations work in the same direction for adults but are conflicting when children are concerned requiring to follow one and downplay other ones, but which one? Furthermore, empirical issues can be raised



What sample frame options could be considered?

- The options can be defined by the answer to two questions:



First question: who is represented in the sample?

Represented	General public	Patients valuing their own health
Children	A	D
Adolescents	B	E
Adults	C	F

Preferred option depends on **normative** reasoning

1. General public should decide as taxpayers/group who decides in democracy → C
2. General public should decide to ensure inclusiveness and representation → ABC
3. Patient preferences should count → DEF

Second question: do we require specific expertise?

Represented	No expertise required	Experienced the childhood condition	Knowledge of childhood condition		Aware of children's wants and needs
	General public	Patients	Selected parents/Carers	Healthcare professionals	Parents/Carers
Children	A	D	N/A	N/A	N/A
Adolescents	B	E	N/A	N/A	N/A
Adults	C	F	G	H	I

Preferred option depends on **normative and empirical reasoning**

1. No experience required → general public
2. Lived experience with condition → patients
3. Able to understand the disease and/or how it impacts a child → groups chosen for their knowledge

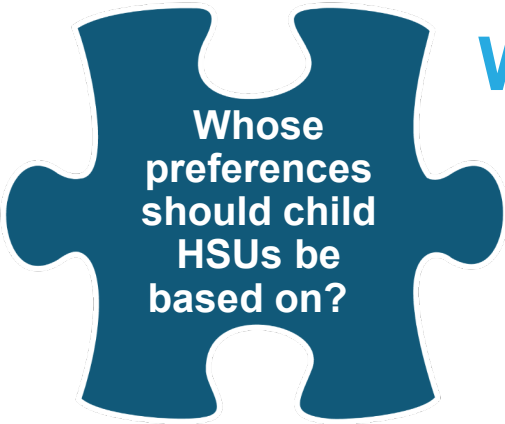
Total of 9 possible frames

1. Who is represented in the sample?

2. Do we require specific expertise?

	No expertise required	Experienced the childhood condition	Knowledge of childhood condition		Aware of children's wants and needs
Represented	General public	Patients	Selected parents/Carers	Healthcare professionals	Parents/Carers
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Adolescents	B	E	N/A	N/A	N/A
Adults	C	F	G	H	I

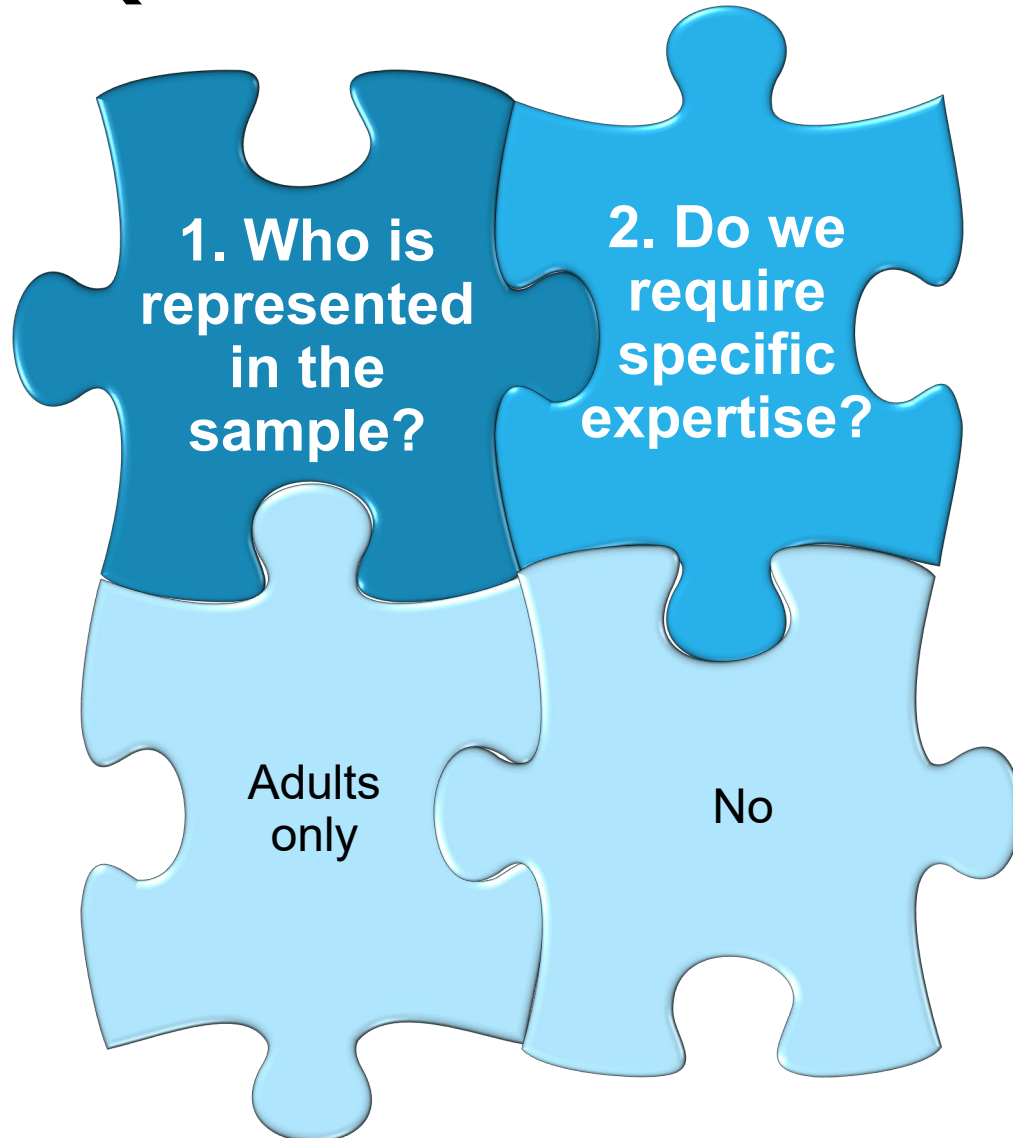
- Decisions driven by a mix of normative and empirical concerns



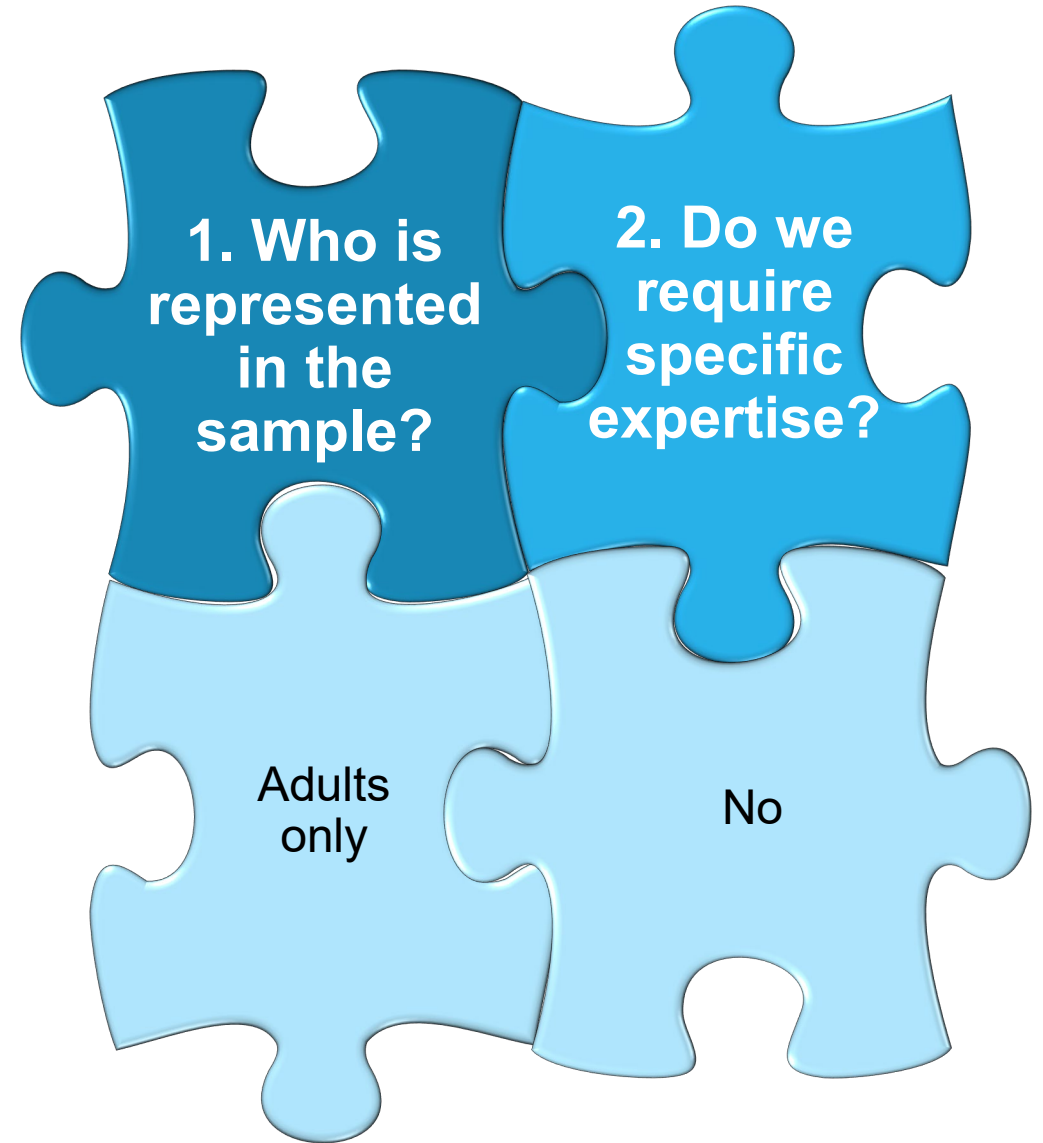
Whose preferences should child HSUs be based on?

- A recent review ([Bailey et al 2022](#)) found 77 studies assessing child HSUs, and sample frames varied:
 - Parent of child with condition (27%)
 - Adults from general public (23%)
 - Adolescents from general public (23%)
 - Adolescents with condition (14%)
- 33% used more than one population group, promoting learning about how much choices matter empirically

EQ-5D-Y-3L values



CHU-9D



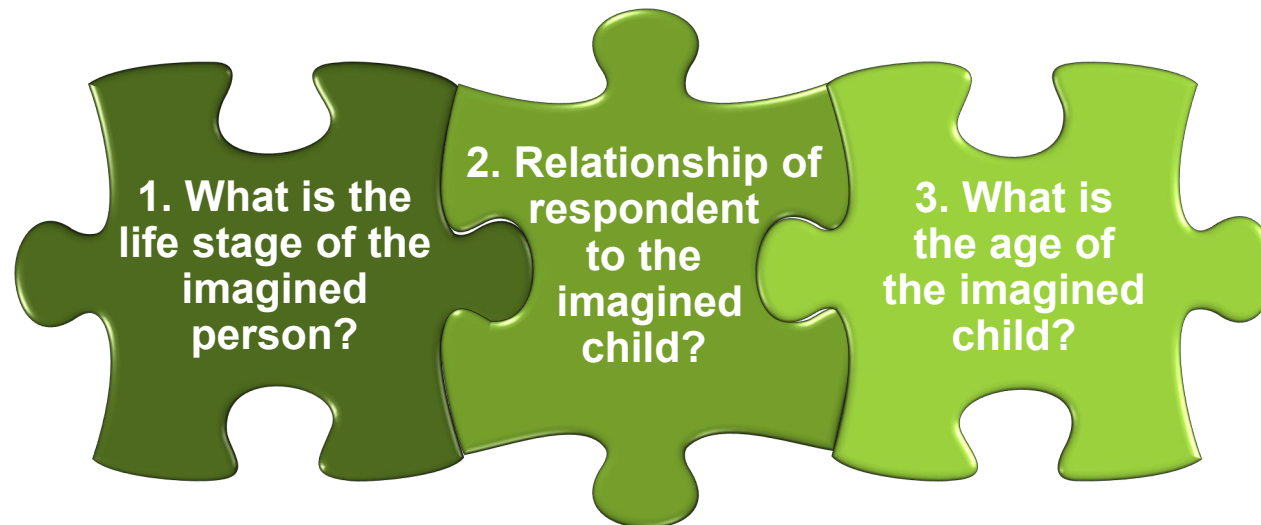
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Whose health is imagined when valuing pediatric health states?

Dr. Louis Matza, Evidera, United States

Whose health is imagined when valuing pediatric health states?

- **There are a range of options.**
 - This should be considered for all pediatric health states (e.g., EQ-5D-Y health states, health state vignettes, or health states derived from a condition-specific PRO).
- **Three questions to consider:** The answer to each question can have an impact on the resulting utility value.





1. Life stage

First question: What is the life stage of the imagined person?

- Adult
 - With pediatric health states, it may be possible for adult respondents to imagine living in the health state at their current age.
 - Limitation: The impact of disease and treatment may differ between children and adults, and this framing would not capture the specific impact in children.
- Child
 - Usually the preferred approach so that the resulting utilities are truly relevant in models of treatment for children.
- Child growing into adulthood
 - A valuation task can be structured so that respondents consider a child growing into adulthood during the time in the imagined health state.
 - For example, if you're using a time trade-off valuation with a 10-year time horizon: A 15-year-old at the beginning of the time horizon would be 25 by the end.



Second question: What is the relationship of the respondent to the imagined person described in the health state?

- If the respondent is told that health states represent a child, relationship to the child may be specified.
- Two types of frames:
 - “Self” frame
 - Respondents can be asked to consider the health state for themselves
 - Adults can imagine themselves at their current age or as a child
 - Children can theoretically be asked to imagine themselves living in the health state, but this raises cognitive and ethical challenges
 - “Other” frame
 - Consider the health state for another person
 - A variety of “other” frames are possible



Possibilities for the “other” frame

- Respondents can be told to imagine **their own child** living in the health state
 - May be challenging for people who are not parents.
 - Imagining one’s own child could result in different valuations than imagining a different child.
 - It can be difficult to avoid the influence of family/caregiver spillover effects on health state preferences, especially when valuing health for very young children.
- A **different known child** (e.g., “a child you know”)
- A **non-specified child**
 - e.g., “an 8-year-old child” without further specification
 - This approach leads to substantial heterogeneity in who is imagined (e.g., my own child, my niece, my neighbor, myself as a child, a child with specific demographics, no particular child...)

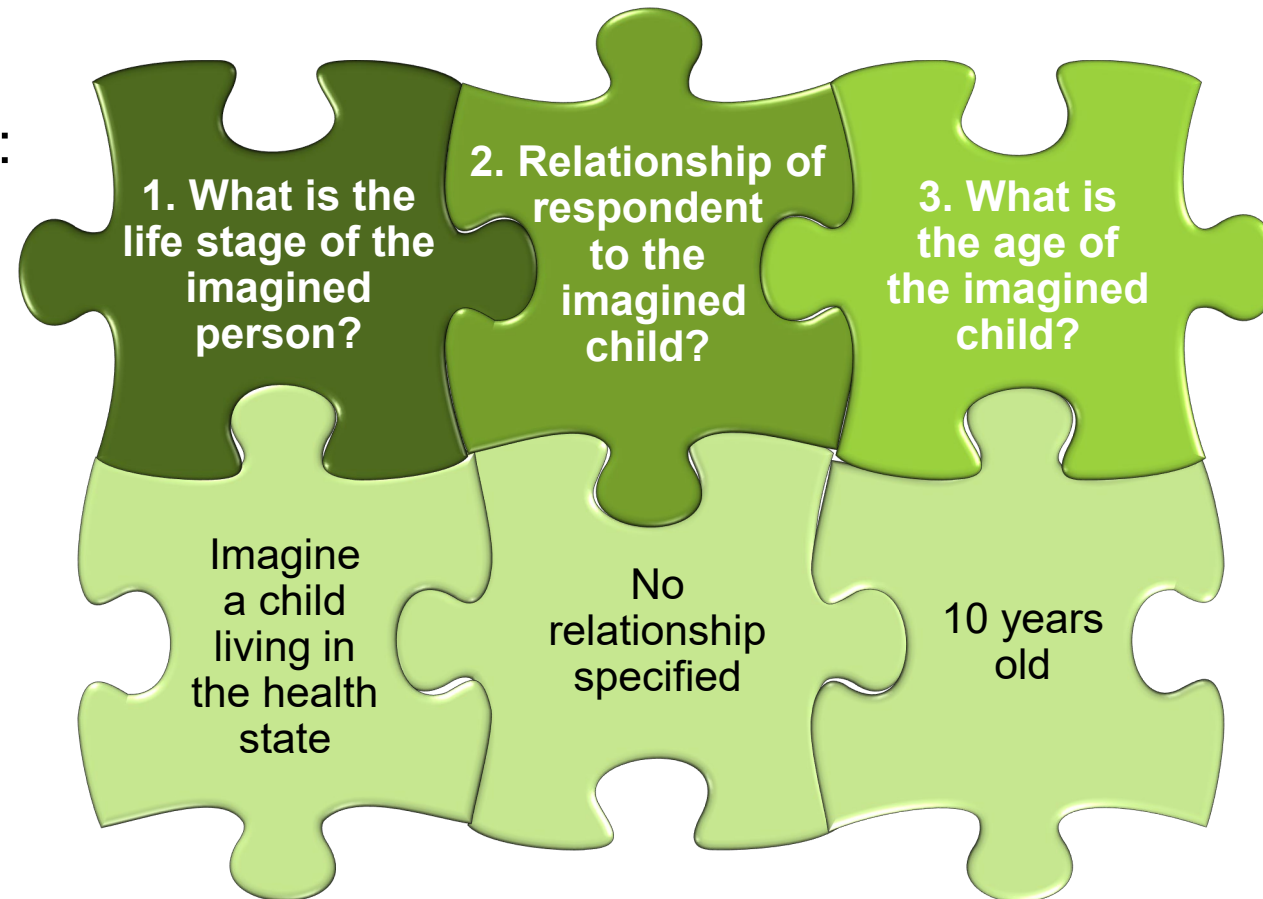


Third question: What is the age of the imagined child?

- If the respondent is told to imagine a child living in the health states, the age may be specified.
- The age can have an impact on the resulting utility.
- There are several ways this can be done.
 - Specific age (e.g., an 8-year-old child)
 - Age range (e.g., a child of ages 8-12)
 - Distribution across a range of specified ages
 - Several specific ages, each randomly assigned to a subset of respondents
 - Unspecified age
 - Health states describe a “child” without specifying the age
 - This could introduce unnecessary heterogeneity as each respondent makes their own assumptions about the child’s age

Combining the puzzle pieces: A recent study valuing EQ-5D-Y health states

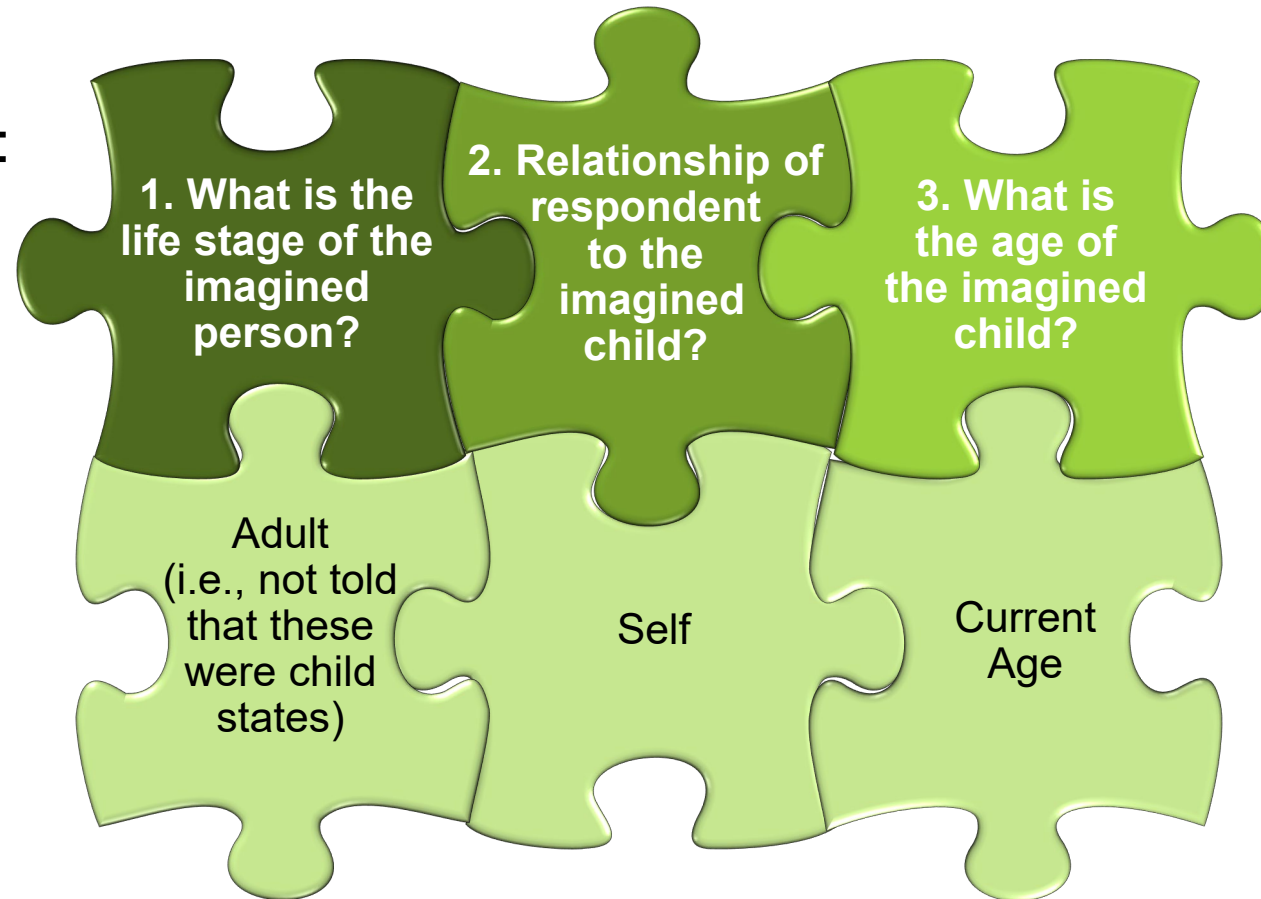
Three questions:



Three answers:

Combining the puzzle pieces: A study valuing CHU9D health states

Three questions:



Three answers:

Final thoughts on whose health is imagined

- These decisions should be considered carefully for pediatric utility assessments because the answers can have an impact on the resulting utilities.
- Selection of the framing will depend on a range of factors such as modeling goals and the pediatric population represented in the health states.
- Further research is needed to better understand how respondents, researchers, and policy makers are considering and applying these frames.

4

How to recommend ‘good practice’ when value judgements are unavoidable?

Professor Nancy Devlin, University of Melbourne, Australia






Positive vs. Normative

- As Elly and Louis have shown, researchers face many choices in selecting methods for eliciting pediatric utilities
 - Whose preferences?
 - Whose perspective?
 - What age of child?
 - What duration of state?
 - What stated preference methods?
- Empirical evidence can tell us *what difference* these methods choices makes on the properties and characteristics of pediatric utilities
 - In some cases, methods may produce ‘better’ results e.g., in terms of data quality
- In many cases, choosing between these sets of methods will rely on value judgements
- **What are the implications of this for good practice recommendations? How have we addressed this in our Task Force?**

Choosing between value judgements


- How to proceed when ‘determining good practice’ = ‘choosing between value judgements’?
- e.g., whose stated preferences should be sought in eliciting pediatric utilities will (partly) depend on **the principles and social values of the decision makers** using that evidence.

 Why ask children?	 vs.	Why ask adults? 
Consumer sovereignty		Tax payers
Patient-centered care		Consistency e.g., with adult HRQoL utilities
'Nothing about me without me'		To avoid ethical risk to children

Abbreviation: HRQoL = health-related quality of life

Normative considerations sitting underneath these choices

- Discussion of methods choices re: pediatric utilities often assume that we *want* age-specific utilities for pediatric HRQoL instruments
- But that itself is a value judgement
- An alternative is to use a single, age-invariant set of utilities across HRQoL instruments
- Current practice re: pediatric utilities varies widely ([Bailey et al 2022](#)), which decision makers may not be aware of
- **There is no right or wrong answer, but which is chosen will have an important effect on utilities** ([Devlin et al 2023](#))

<u>Age-specific</u> values for child HRQoL	 vs.	<u>Age-invariant</u> values for child HRQoL
EXAMPLE: EQ-5D-Y-3L (asks adults their views about a 10 year old child)		EXAMPLE: HuPS (response mapping to HUI3 and use HUI3 utilities) (Furlong et al 2023)
Preferences may be specific to age-specific HRQoL descriptions		Avoids artefactual differences in utilities between age groups, even where HRQoL unchanged
Children’s preferences are relevant		Provide a consistent basis for weighing up QALYs gained and foregone across different ages

Who should make these value judgements?

- At present, these methods choices are made by **researchers**.
- Methods choices vary widely (by pediatric HRQoL instrument; by study); reporting is not always adequate.
 - The rationale for the choices is not always clear
 - The implications for the resulting utilities is not always clear
 - The implications for QALY estimates is not always clear
- End users (e.g., decision makers) may or may not be aware of these limitations when interpreting evidence on pediatric HRQoL presented to them.
- Currently, no HTA body has a clear position on these methods issues ([Devlin, Rowen, Lovett 2021](#)).
- This makes it extremely challenging for those developing pediatric HSUs and for those undertaking economic evaluations in pediatrics.

Pediatric utilities task force recommendations

We recommend that:

1. Decision makers carefully consider the methods choices and take a clear stance on these that are communicated (e.g., via HTA methods guidance) to researchers and to those submitting evidence on pediatric HRQoL.
2. Studies reporting pediatric HSUs need to ensure that all methods choices involving value judgements are transparent and clearly justified to end users.
3. Studies reporting pediatric HSUs ensure that users are aware of the likely effect of those methods choices on the characteristics and properties of the utilities produced, and the implications for estimates of QALYs.
4. The inclusion of children in studies eliciting HSUs needs to be carefully considered in terms of both potential benefits and harms.



Thank you!

Questions or comments can be sent to the Taskforce co-chairs

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d.rowen@sheffield.ac.uk

Questions for discussion

- Are the proposed Task Force recommendations appropriate?