PHMR

Are child QALYs equivalent to adult QALYs?

Issue panel 5, Virtual ISPOR 2021

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Conflicts of interest

- Koonal Shah's employer, PHMR Ltd, receives income from a variety of sources, including pharmaceutical companies and not-for-profit organisations.
- No specific funding was received for Koonal Shah's presentation or involvement in this issue panel.

- Koonal Shah and Nancy Devlin are members of the EuroQol Group. The EuroQol Research Foundation is the copyright holder of the EQ-5D family of instruments, including the EQ-5D-Y.

The issue

- Increasing interest in measuring and valuing the health of children and adolescents has led to research seeking to generate utility values for younger populations
- However, valuing health in children is challenging
- Valuation protocols are emerging but there lacks consensus on best practice methods
- Recent research suggests that child health state values tend to exceed values for corresponding adult health states

The issue (2)

- Recent research suggests that child health state values tend to exceed values for corresponding adult health states
- Implications for the evaluation of technologies that cover both children and adults, and for trials and models in which patients transition from childhood to adulthood
- It also raises questions about social value
  - Does the QALY = QALY mantra hold?
  - Should age-specific preferences be reflected in value sets?
  - Or should QALY weights be introduced?
  - What role should society's preferences play?
  - Is it even meaningful to compare child QALYs and adult QALYs?

Valuation challenges

- Sampling 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?)

Panellists

Alan Lamb

Science Research and Policy Programme, National Institute for Health and Care Excellence, UK

Nancy Devlin

Health Economics Unit, School of Population and Global Health, University of Melbourne, Australia

Vivian Reckers-Droog

Erasmus School of Health Policy & Management, Erasmus University Rotterdam, The Netherlands

Are child QALYs equivalent to adult QALYs?

An HTA perspective

Virtual ISPOR 2021

NICE National Institute for Health and Care Excellence

Declarations

Alan Lamb is an employee of NICE. Views expressed are his own and do not necessarily reflect the views of NICE.

NICE

2

Wider view

Perspectives from a range of HTA agencies

1

Methods update

Proposed recommendations for measuring and valuing children's quality of life

NICE

3

What next?

What information might HTA agencies need to inform methods?

Valuation of child health states

Reflections from joint ISPOR/NICE international HTA roundtable

NICE

Some initial consensus . . .

Almost all survey respondents (n=11, 92%) recommended that children's utilities should be based on stated preference data relevant to their own country

. . . But no clear recommendations on:

- Whether preferences should come from adults or children
- Whether value sets should cover all children or be specific to a particular age
- What methods should be used

HTA/ISPOR roundtable. Lovett et al. 2021 (in submission)

NICE methods update:

Choice of measure and valuation approach

NICE

The case for change

"Changes to the methods are proposed to improve the quality of evidence for health-related quality of life [...] Substantial research is needed to explore the appropriateness of different measures of health-related quality of life in children and young people, and to identify appropriate valuation methods."

Proposed recommendations

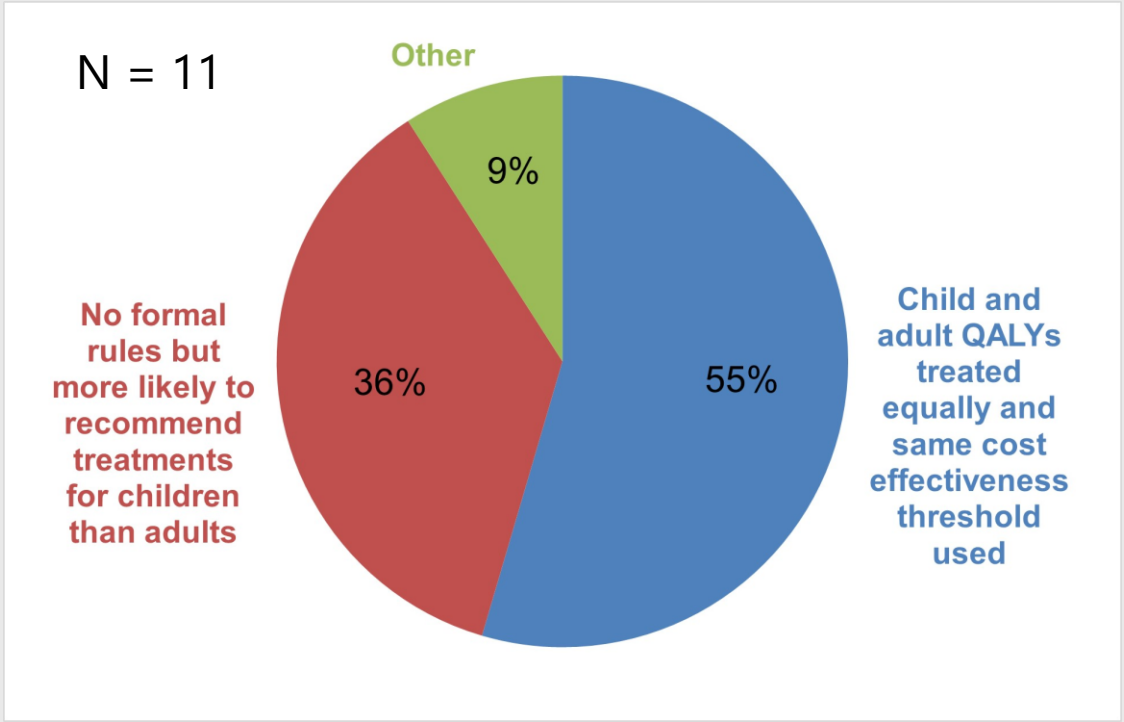
- Children's quality of life should be measured using a generic measure that has been shown to have good psychometric properties in the relevant age range(s)
- No specific measure recommended – the quality and availability of value set(s) is one consideration when choosing a measure

Do child QALYs = adult QALYs?

Reflections from joint ISPOR/NICE international HTA roundtable

NICE

Answer varies across HTA agencies



HTA/ISPOR roundtable. Lovett et al. 2021 (in submission)

Various factors influence approach

Normative issues underpinning priority setting, equalities legislation, consequences of methodological choices

NICE methods update:

NICE does not propose introducing a modifier based on age

NICE

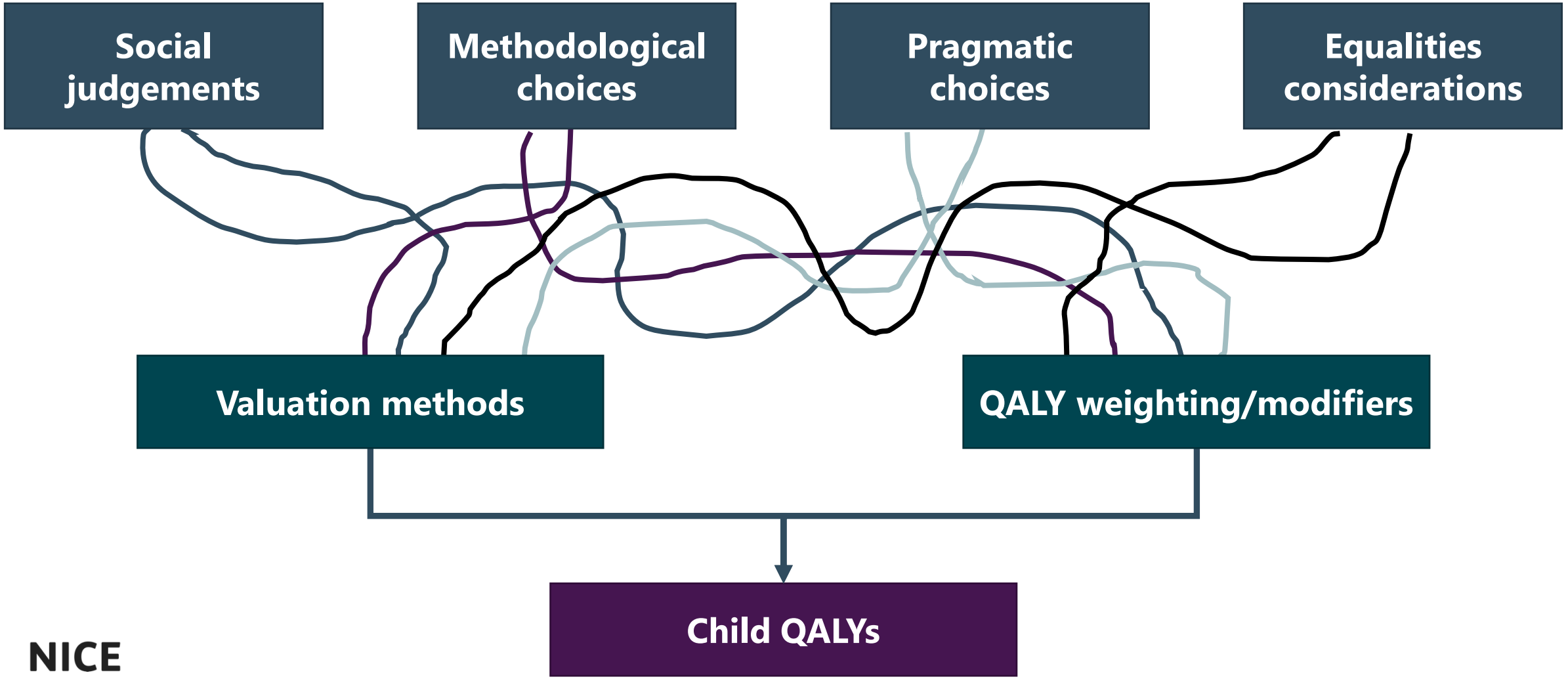
The case for change (or no change)

- Some evidence suggests that the UK population considers different age groups differently – "the evidence is not sufficient [...] further research would be valuable."
- Conclusions of NICE Citizens Council on age (2003) do not support giving additional weight based on the age of the target population

Need to consider potential impact of other proposed modifiers

Case for change identified for including a modifier based on 'severity.' Depending on if/how this is done such a modifier could put a different weight on QALYs depending on age, all other things being equal.

Putting it all together



NICE

What do HTA bodies need to understand?

Societal preferences

- Does the public favour prioritising resources towards children?
- Who does the public think should be involved in valuation studies?

Implications for decision-making

- For the available approaches to valuation and QALY weighting:
  - How do they affect QALYs? How would any changes impact on results of cost-effectiveness analyses?
  - Do these implications align with societal preferences?
  - Are we double counting?

Implementation

- Is the approach fair, transparent and understandable?
- Does the approach comply with equalities legislation?
- How do we encourage collection of data?

NICE

NICE National Institute for Health and Care Excellence

Thank you for listening.

THE UNIVERSITY OF MELBOURNE

Utilities for pediatric HRQoL: challenges for HTA

Prof. Nancy Devlin
University of Melbourne
& Senior Fellow, Office of Health Economics

QUOKKA Research Program

Issues Panel: Are child QALYs equivalent to adult QALYs? ISPOR May 2021

https://www.ohe.org/news/do-child-qalys-adult-qalys-five-reasons-why-they-might-not

What is most relevant to measure (in terms of HRQoL) is different
Limits to self-report and proxy completion
*How to derive utilities for child HRQoL?
How to combine HRQoL and length of life in children
Are child QALYs ‘worth’ the same as adult QALYs

QUOKKA Research Program

1. Why is this issue important?

The overall aim of those managing resource allocation: how to spend the health system budget, across all competing uses of it (existing and new technologies) in a way that improves patients' health overall to the greatest extent possible.

- 'special considerations' may apply to decisions about children – but budgets for child health care **are not generally ring-fenced.**
- A new cost-increasing technology can only be funded by 'giving up' something else: *health gains to other patients.*
- **Decisions to spend more on a new technology to improve health in adults potentially has an opportunity cost in child health, and vice versa.**
- It is crucial to ensure that we are **measuring health outcomes in a commensurate way across both adults and children**, so outcomes can be weighed up 'like for like'.

Are we measuring QALYs for adults and children using the same scales?

Comparability with respect to

- What is being valued: how we *measure* self reported HRQoL – what, how?
- How we value self reported health – the 'utilities' – who, what, how?
- Combining with length of life to estimate QALYs – are all QALYs of equal value?

2. Pediatric HRQoL utilities for HTA: current state of play

- Methods used to value adults' HRQoL do not work well in valuing paediatric HRQoL
- Given problems in both measurement and valuation, there has been low uptake of these tools in evidence used to inform healthcare decisions.
- **The resulting gap in evidence risks significant misallocation of funds**, potentially denying access to effective and cost-effective pediatric interventions.
- Poor and inconsistent practice in assigning utilities to child HRQoL states.
  - Review of PBAC public summary documents (Bailey et al 2021)
  - Systematic review of use of HRQoL in infectious disease (Herdman et al 2016)
- Growing awareness by HTA bodies – but a lack of clear guidance (Lovett et al 2021)
- **An ISPOR emerging good practices taskforce is planned.**

3. Uncertainty about how to value child HRQoL

"Child HRQoL is measured in different ways (using age-appropriate PROs) so we have to create corresponding utilities"

But:

- **Whose preferences** are relevant? Adult general public? Children/adolescents (where feasible)?
- **If adults – what perspective?** (own health; self as child; own child; hypothetical child?)
- **What age child?**
- **What methods**? **What duration** of states to be valued?

- How to resolve these – who decides? (especially where value judgements involved).
- Is a 'one size fits all' international protocol for valuing child PROs realistic, given different views and requirements of HTA bodies?
- Observed differences in characteristics of utilities for child HRQoL: length of scale; importance of domains
- **Protocols for valuing child HRQoL could end up looking quite different than those used for adult HRQoL.**

Example: Valuing EQ-5D-Y – protocol & methods

Protocol feature	DCE side	C-TTO side
Target population	Adult general population	
Perspective	Taxpayers	
Framing	Considering your views about a 10-year-old child What do you prefer?	
Design	10 blocks/15 pairs each	1 block of 10 health states
Sample size	1000	200
Interview environment	Online	Face to face
Interview	Introduction	
	Demographics	
	15 DCE forced pair comparisons	10 TTO tasks
	Feedback questions	Feedback questions
Expected interview duration	~30 min	~15 min

c-TTO composite time-trade-off, *DCE* discrete choice experiment, *TTO* time-trade-off

PharmacoEconomics (2020) 38:653–663
https://doi.org/10.1007/s40273-020-00909-3

PRACTICAL APPLICATION

International Valuation Protocol for the EQ-5D-Y-3L

Juan M. Ramos-Goñi[1,2] · Mark Oppe[1,2] · Elly Stolk[2] · Koonal Shah[3,4] · Simone Kreimeier[5] · Oliver Rivero-Arias[6] · Nancy Devlin[3,7]

Published online: 16 April 2020

What methods?

- DCE & TTO (the latter only to anchor at 0)

Whose preferences?

- Adult general public

What perspective?

- 'Imagine a hypothetical child'

What age(s) child to be considered?

- 10 year old child

What duration?

- 10 years

- *Different* choices about these things are likely to lead to *different* values.
- Choices unavoidably entail normative aspects i.e., cannot be based just on best practice, but require value judgements

4. What's special about children - & where should that be reflected in HTA?

What is the shape of society's welfare function in *QALYs* by age (children through to adults)?
In the two components of the QALY:

- ***life expectancy/life years* by age**. Society may have high marginal utility (MU) in life years in children.
- ***HRQoL* by age.** Society might value some HRQoL domains differently in children than adults.

This is important, because it has implications for HRQoL utilities:

- MU of life years is very high in children, and may dominate the MU in HRQoL
- Manifests as **unwillingness to trade off years of life (in TTO) or risk of death (in SG)**
- results in: **higher values for poor health (cf. adults); fewer negative values; a shorter overall value scale**

So, what is the role of QALY weights?

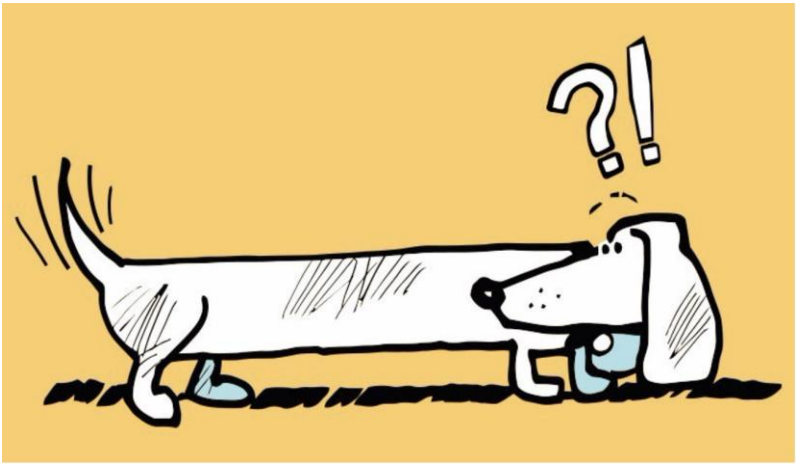
- 'correct' for effect of unwillingness to trade on child utilities?
- Create an exchange rate between adult and child HRQoL QALYs, acknowledging potential for different social welfare in both length and HRQoL?

Confounded by underlying differences in HRQoL utility – the 'special' character of children's health is arising in multiple parts of the process – lacks transparency

5. What do decision makers need? Initial thoughts

Having child-specific utilities may have some important uses, but in HTA it compromises

- **Comparability of QALYs and ICERs between adults vs children**
- **Ability to compare child QALYs and ICERs against existing thresholds**
- **Ability to assign special priority to child QALYs - special considerations already exerting an affect on utilities**



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6. What does this imply for research to value HRQoL in children?

Age neutrality in HRQoL utilities is desirable for applications/decision contexts (such as HTA) where comparability important.

This could be achieved in a number of ways e.g.:

- Minimising differences between the methods applied to valuation of adult and child HRQoL e.g., CHU9D asked adults to value states as if experiencing them themselves
- Mapping from child HRQoL instruments to adult HRQoL instruments – we plan to explore this in EQ-5D-Y and EQ-5D-5L in age groups where both are appropriate for use (and in younger age groups, using proxy)
- Developing tasks to empirically identify the underlying latent 'willingness to trade' governing the values yielded in trade off tasks

Our ongoing work* is considering the pros and cons of these and other options

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*Devlin N, Pan T, Sculpher M, Jit M, Stolk E, Rowen D, Norman R (2021)
Using utilities for pediatric HRQoL in cost effectiveness analysis – challenges and potential solutions.

HTA committees may consider child QALY gains to be special, and to warrant special priority, and this can be taken into account via deliberation or QALY weights.

But this makes it all the more important that we make the effort to disentangle these special considerations from the HRQoL utilities used to estimate QALYs and ICERs

Acknowledgments:

This presentation draws on the following paper, which is a work in progress:

Devlin N, Pan T, Sculpher M, Jit M, Stolk E, Rowen D, Norman R (2021)

Using utilities for pediatric HRQoL in cost effectiveness analysis – challenges and potential solutions.

Funded by the EuroQol Research Foundation. Opinions expressed are those of the authors and are not necessarily those of the EuroQol Group.

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Thanks for listening!

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Poll question 1

Is it appropriate to have separate value sets for adults and for children, or should there be a single value set that is applicable across all ages?

- There should be separate value sets for adults and for children
- There should be a single value set that is applicable across all age groups

Erasmus School of
Health Policy
& Management

Empirical evidence

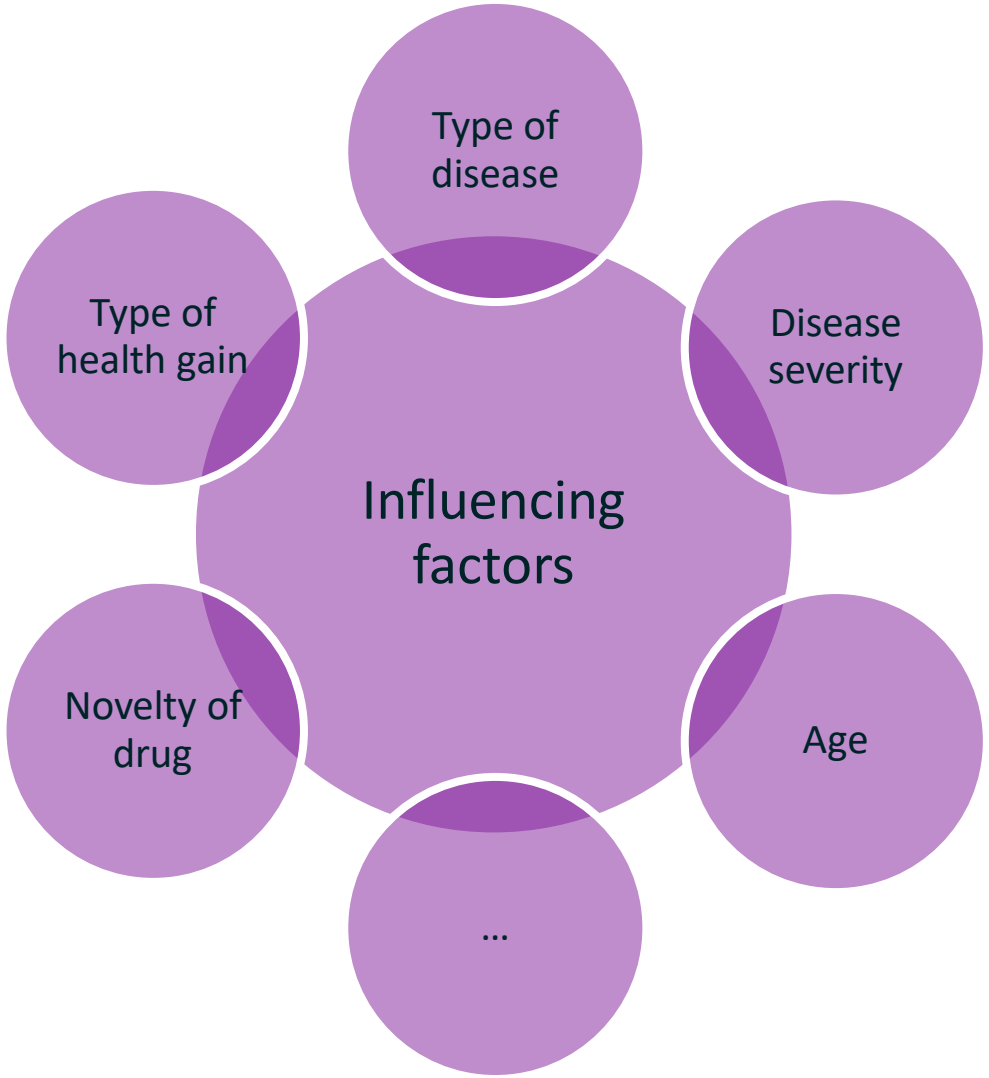
on age-related preferences in society

Vivian Reckers-Droog
Assistant Professor of Equity Considerations
Department of Health Economics

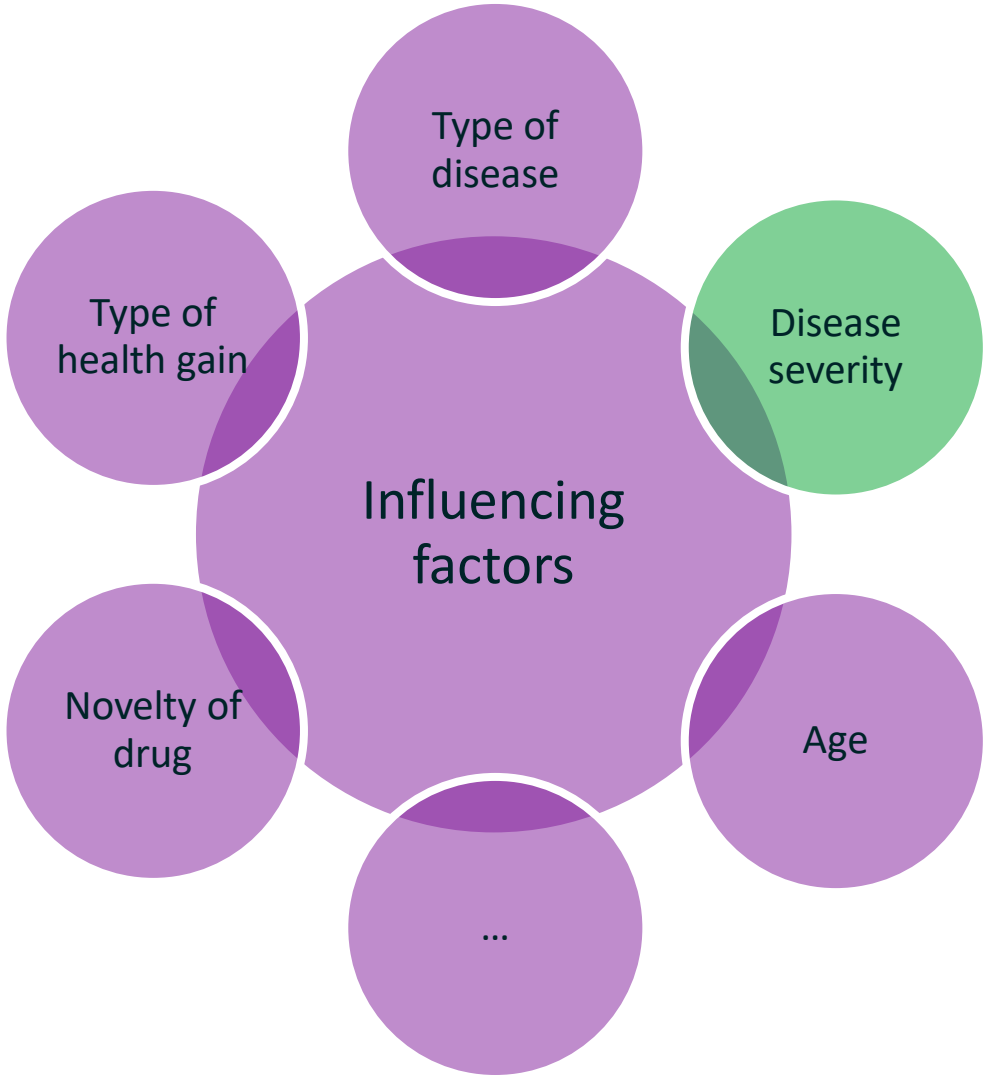
ISPOR, May 2021

Erasmus University Rotterdam

QALY ≠ QALY


Type of disease
Disease severity
Age
…
Novelty of drug
Type of health gain
Influencing factors

QALY ≠ QALY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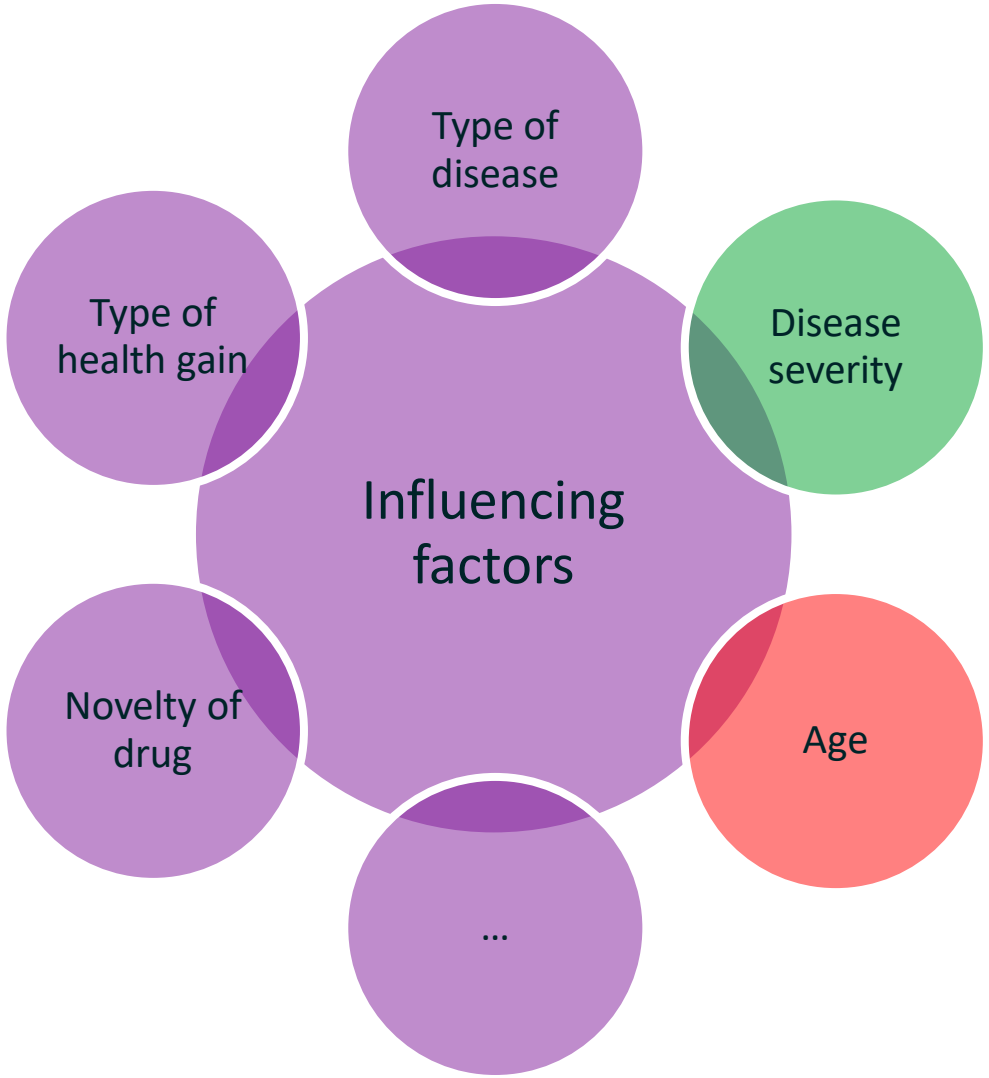
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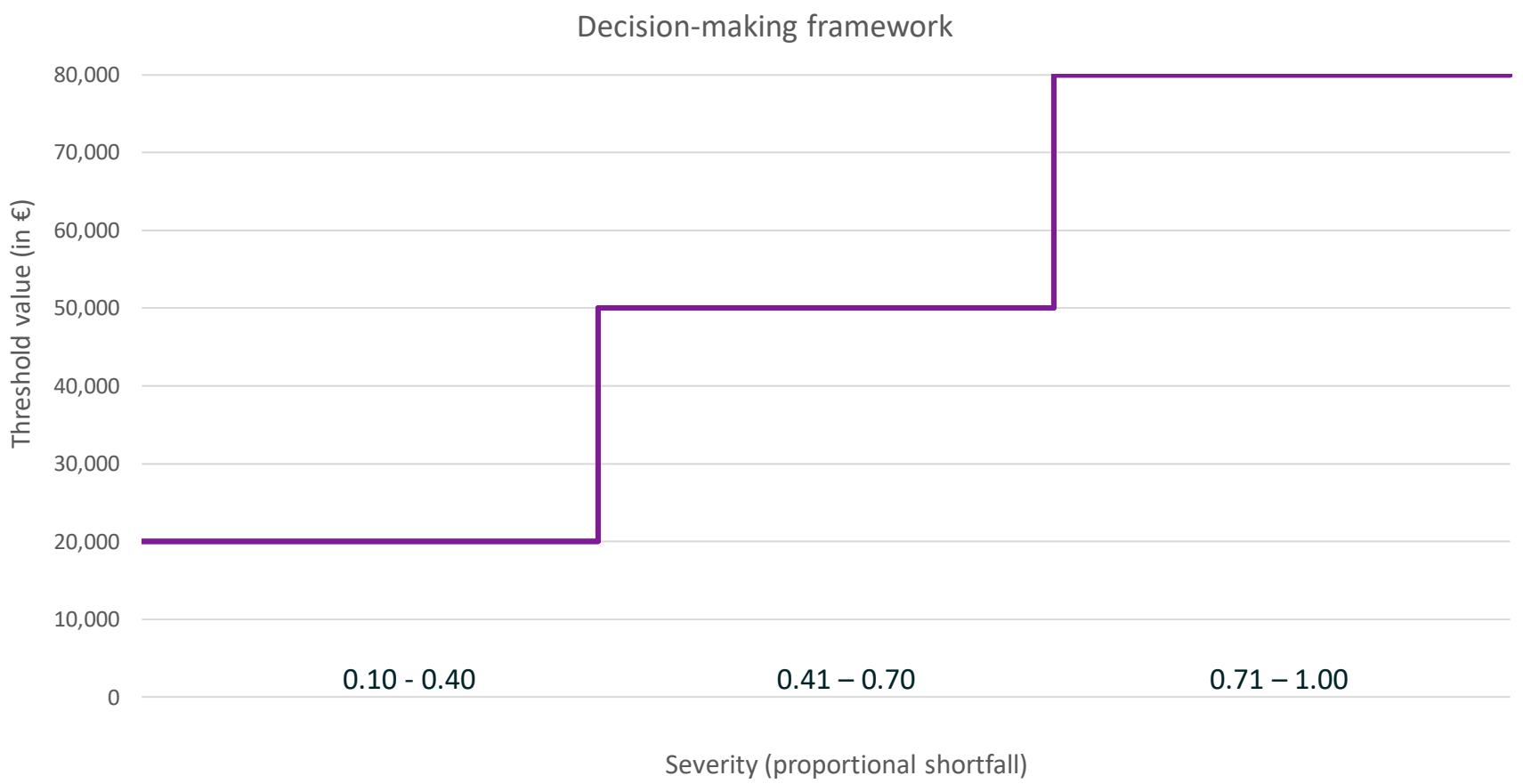


Why proportional shortfall?

- Combines severity of illness and fair innings approaches
- Avoids discrimination on the basis of age

- Proportional shortfall may implicitly prioritize *older* patients

- Absolute shortfall may implicitly prioritize *younger* patients

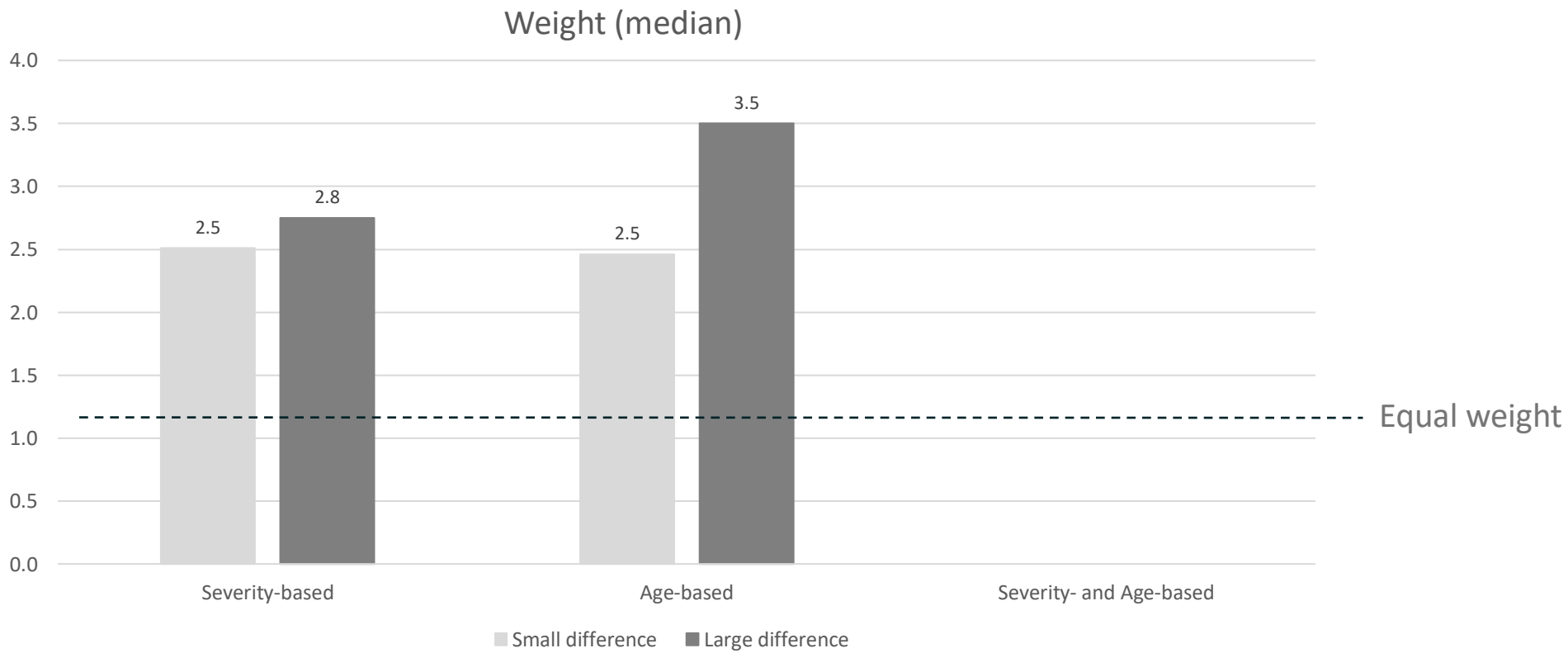
- End-of-life premium may implicitly prioritize *older* patients

- End-of-life premium may implicitly prioritize *older* patients

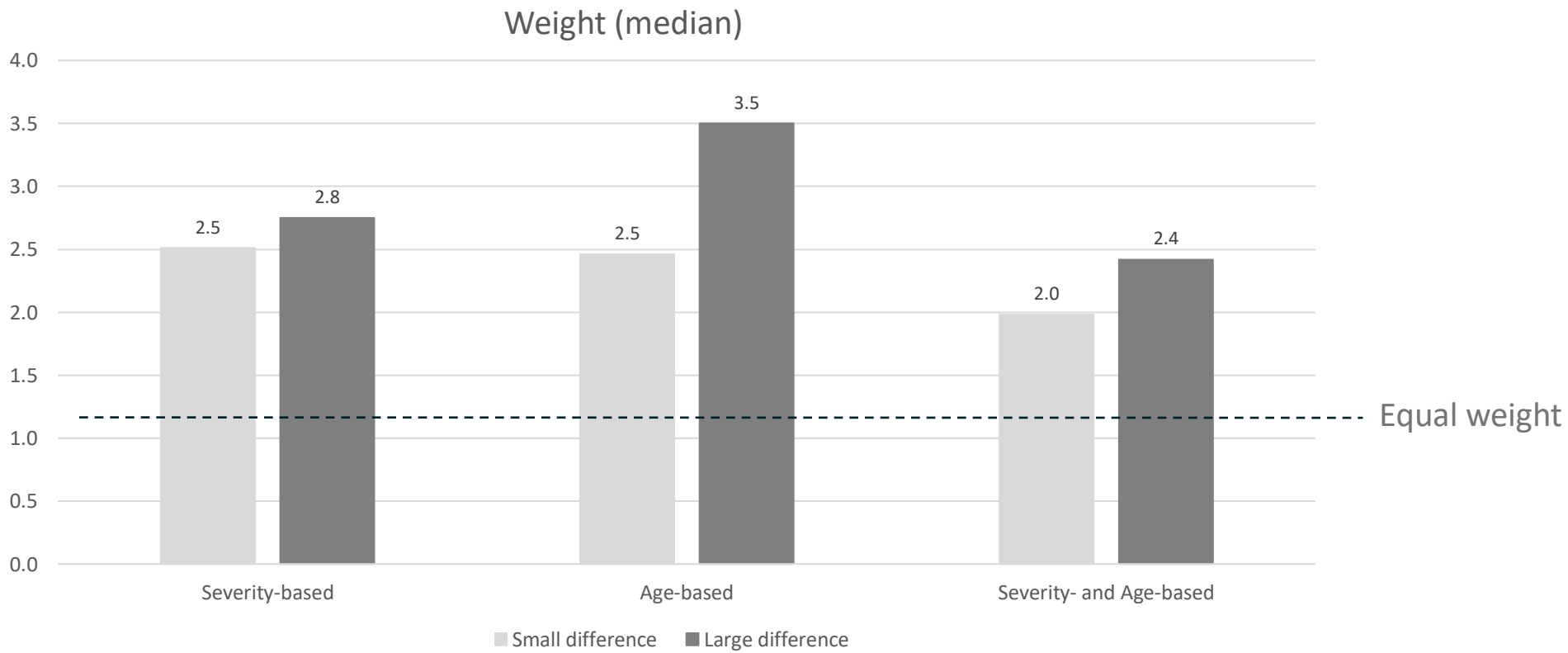
Preferences for severity and age

Results from two studies:

- Person trade-off approach
- Contingent-valuation approach



VT Reckers-Droog, NJA van Exel, WBF Brouwer. (2019). Equity Weights for Priority Setting in Healthcare: Severity, Age, or Both? *Value in Health*, *22*(12), 1441-1449.



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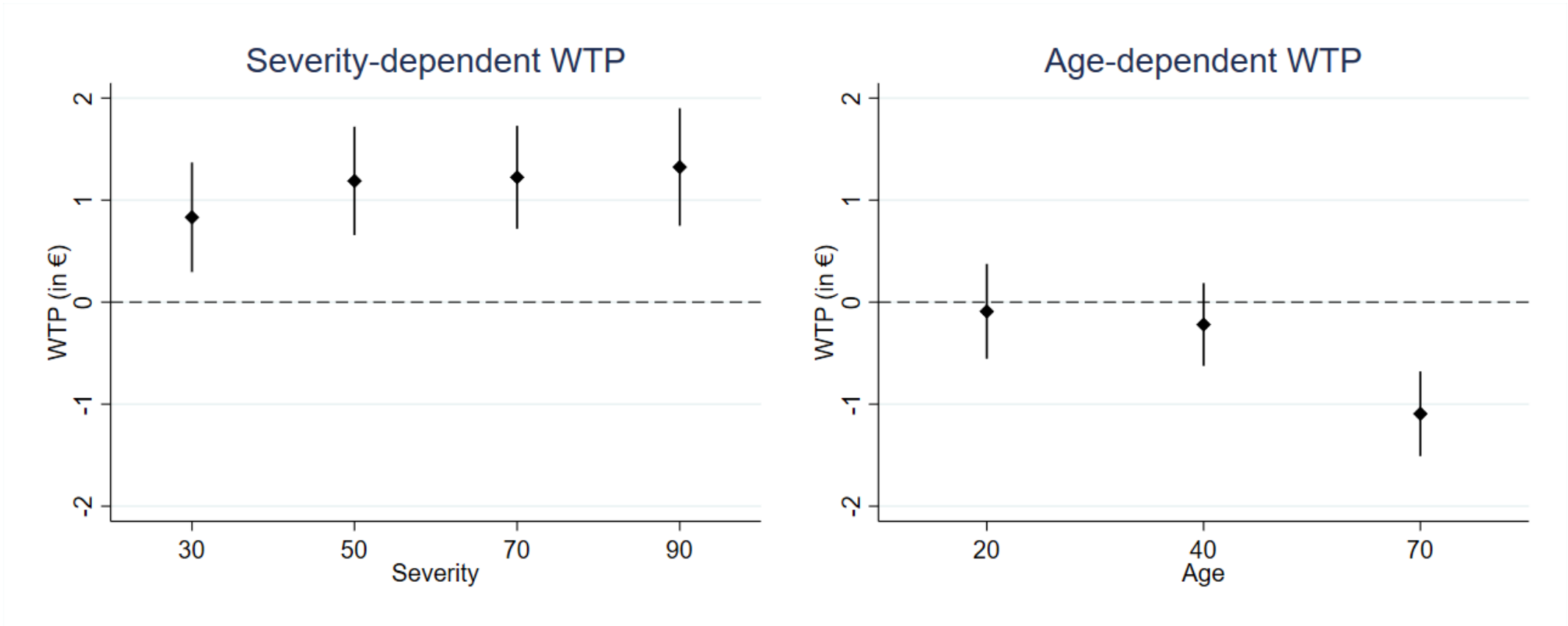

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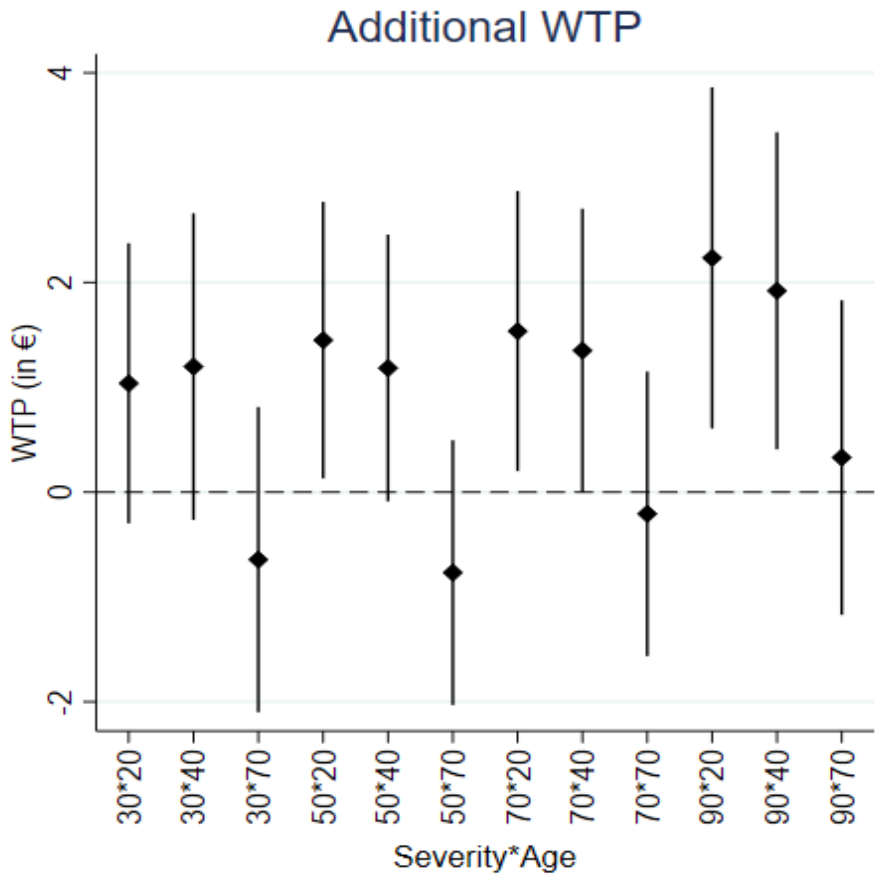

VT Reckers-Droog, NJA van Exel, WBF Brouwer WBF. (2021). Willingness to Pay for Health-related Quality of Life Gains in Patients with Different Severity Levels and Ages. *Value in Health.*

Healthcare priority setting

In reimbursement decisions:

- Elicited preferences *not* used for calculating QALYs
- QALY gains in children and adults are valued equally
- Differential weighting of QALY gains in children and adults *can be* made explicit in decisions:

– To align current decision-making frameworks with societal preferences
– To reduce the possible risk of discrimination on the basis of age between patients

Preferences for child and adult health

Preliminary results from one study:

- DCE and cTTO tasks combined with think-aloud protocol

Performing the HSV tasks	Perceptions about the person living in the health states	Interpreting and assessing the health states	Influencing factors	Stating a preference
Clarity and coherency of health states	Imagining (someone else) living in health states	Experience with health states	Support system	Stating a preference (for someone else)
Maintaining focus	General image of the person living in health states	Dimensions and levels of health states	Life events and goals	Giving up life years
	Cognitive and developmental aspects	Duration of health states	Preferences of persons living in health states	Legitimacy of choice
Hypothetical context	Resilience and coping skills	Consequences of health states for person living in health states	Preferences of persons not living in health states	Egoistic versus altruistic preferences
	Family and peer relationships	Consequences of health states for persons not living in health states	Normal and acceptable health	Consequences of choice
	Relationship to the person living in health states	Lexicographic preferences	(Not) having children	Distancing oneself from the task
	Rephrasing health states		Housing and available facilities	Expectations about (potential differences in) preferences

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	Rephrasing health states		Housing and available facilities	Expectations about (potential differences in) preferences

“A bit of problems... a lot of pain... I choose life A, because I hope that with painkillers and such, and medical aids... I actually want to sort of escape from making a choice... from making a decision. I don't want it, it does not befit children.”
(62 yo male, w children)

“A child is often the most precious thing you have and you don't want to lose it. I can well imagine that in the haze of ‘I don't want to lose my child’ you say ‘we are going to do this’ because then she can be with us longer. I can well imagine that happening.”
(22 yo female, wo children)

Health state valuation

In reimbursement decisions:

- Elicited preferences *are* used for calculating QALYs
- QALY gains in children and adults are valued unequally
- Differential weighting of QALY gains in children and adults implicit in decisions
- Consequences (e.g. in relation to higher values and narrower value range) may not be aligned with societal preferences

Moving forward…

- Evidence supports attaching different values to child and adult health (gains)
- Preferences for prioritisation of children and for child health *are distinctly different*, but some overlap in underlying (equity) considerations may need to be disentangled
- How to move forward may depend on (consensus about) whether age-related preferences should be taken into account in reimbursement decisions.

If no, should different values then be attached to child and adult health?

If yes, should such preferences be incorporated (explicitly) as QALY weights and/or (implicitly) as HRQOL utilities?

Acknowledgement

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The views expressed in this presentation are those of the authors and do not necessarily reflect the views of the funders.

Want to discuss further?

Contact me

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Poll question 2

How should a health system with a limited budget prioritise resources?

- The health system should prioritise the treatment of adults
- The health system should prioritise the treatment of children
- The health system should give equal priority to the treatment of adults and children
- Don’t know

Poll question 3

If the general public feels (on average) that the treatment of children should be prioritised, should this preference be incorporated into HTA decision-making?

- Yes
- No
- It depends
- Don’t know

Q&A session

Some suggested topics for discussion:

- Are there any countries in which child QALYs are given greater weight than adult QALYs?
- Which arguments in favour of age-based QALY weights do you find most persuasive? And which do you find problematic?
- If there is support for a premium for children, should this premium be applied algorithmically or via greater flexibility in the decision making process?
- What are the views of those working for pharmaceutical and life sciences companies?
- What are the main research priorities in this area?

Thank you for joining us

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