The Role of Patient Preferences in Economic Evaluations

Barriers and Opportunities for a Patient-Based QALY

Anja Schwalm
IQWiG
Germany

Martin Henriksson
Linköping University
Sweden

Ben van Hout
University of Sheffield
United Kingdom

Moderator:

Paul Schneider, Valorem health, Germany

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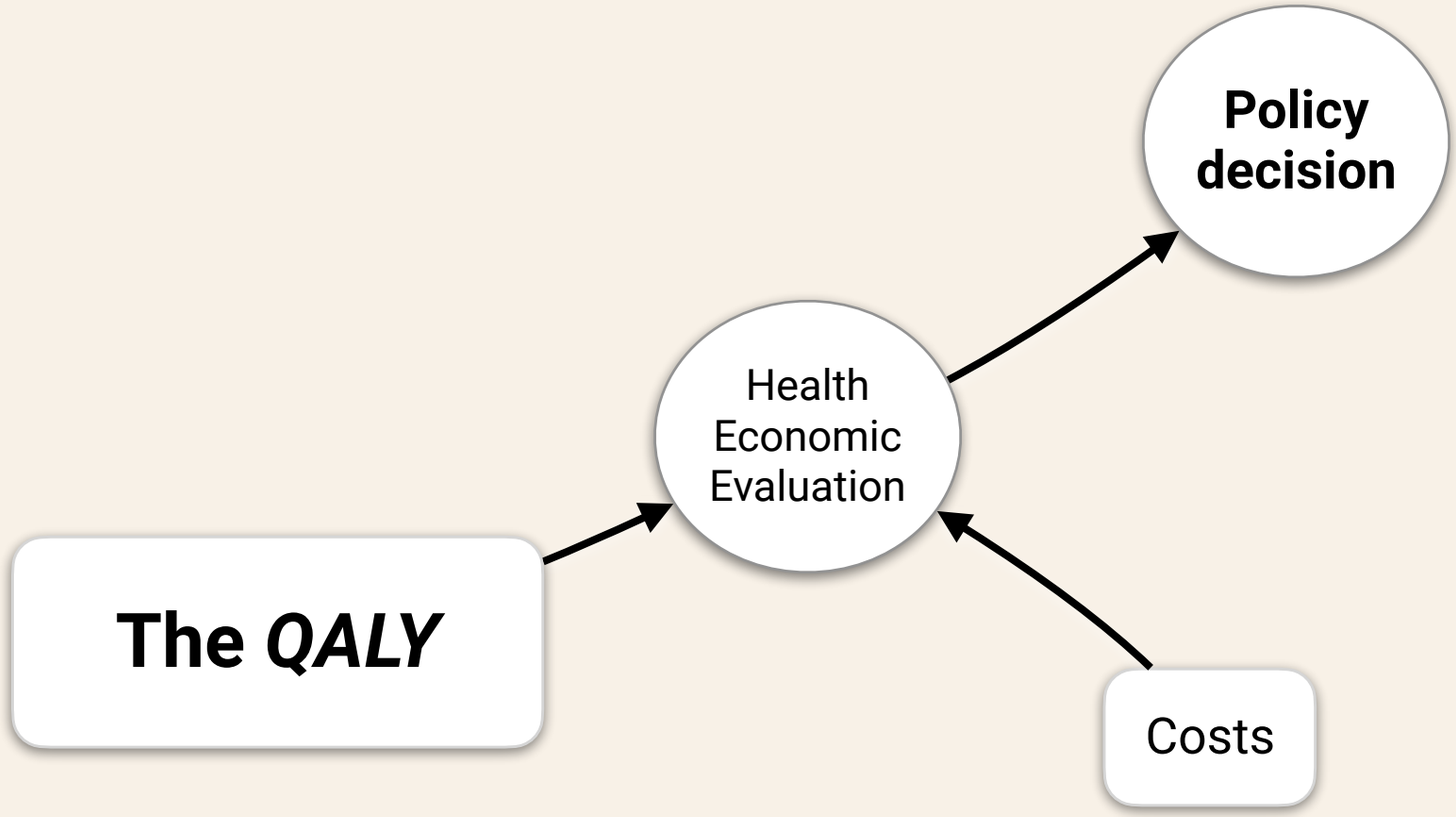
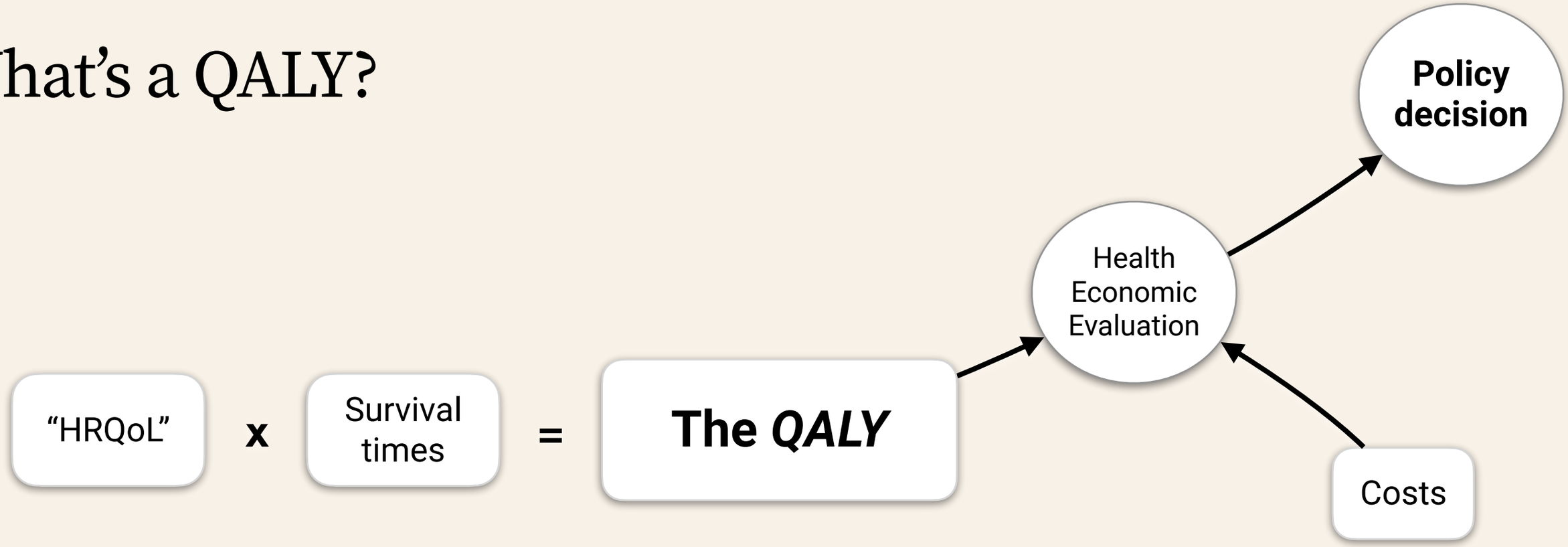
What's a QALY?

The *QALY*

What's a QALY?


Policy decision
Health Economic Evaluation
The *QALY*
Costs

What's a QALY?


"HRQoL"
x
Survival times
=
The *QALY*
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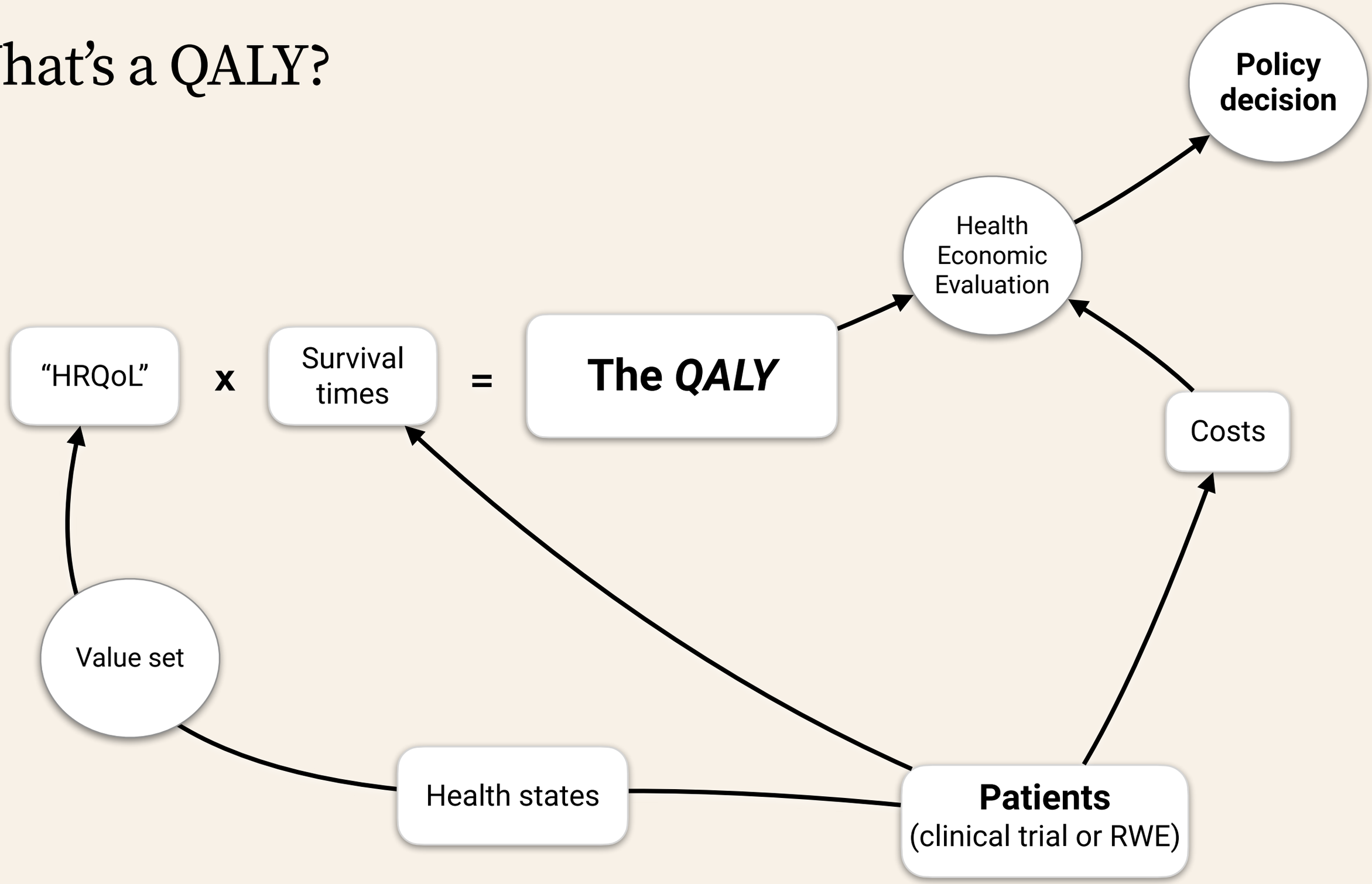
What's a QALY?


Policy decision
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"HRQoL"
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=
The *QALY*
Costs
Value set
Health states
Patients
(clinical trial or RWE)

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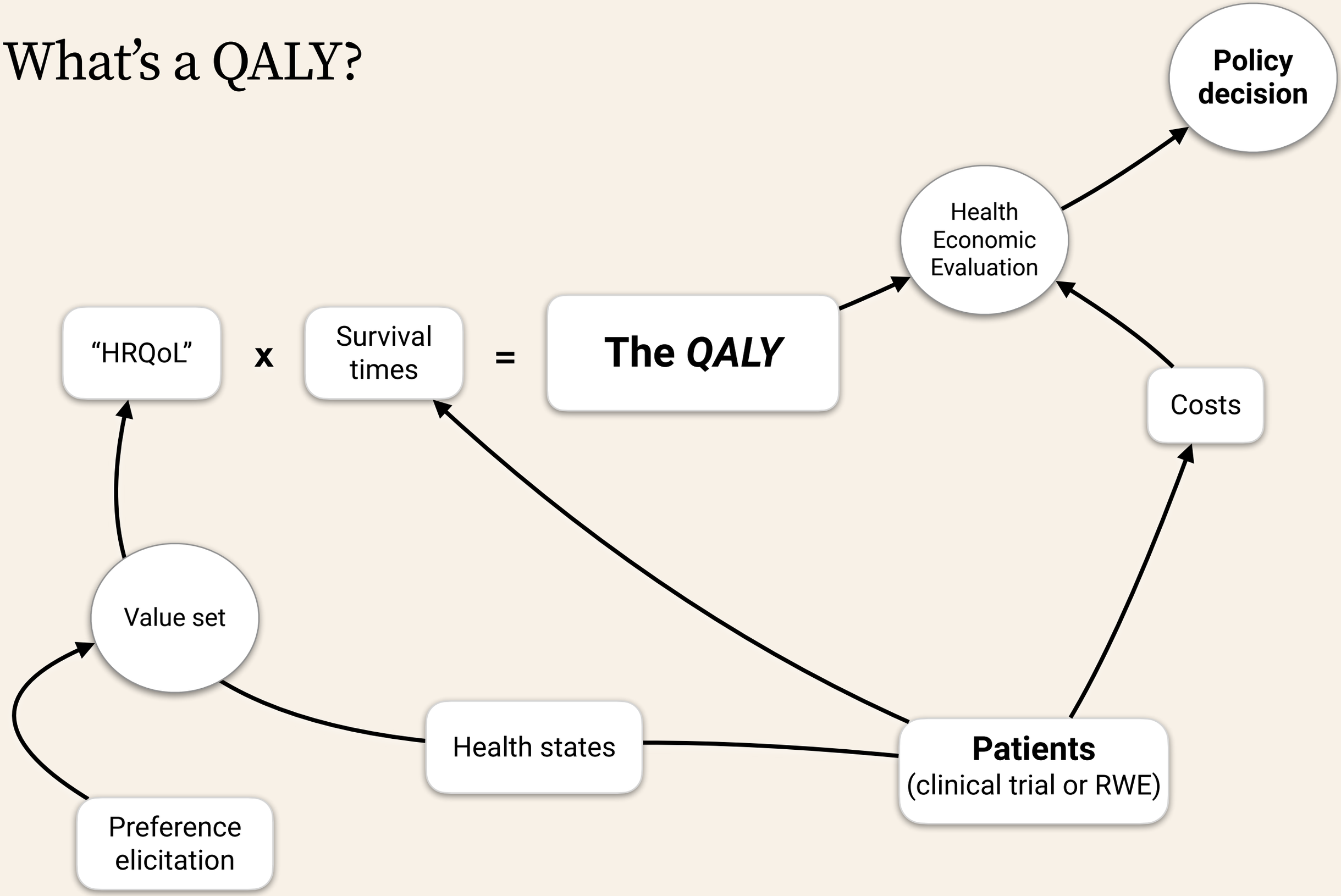
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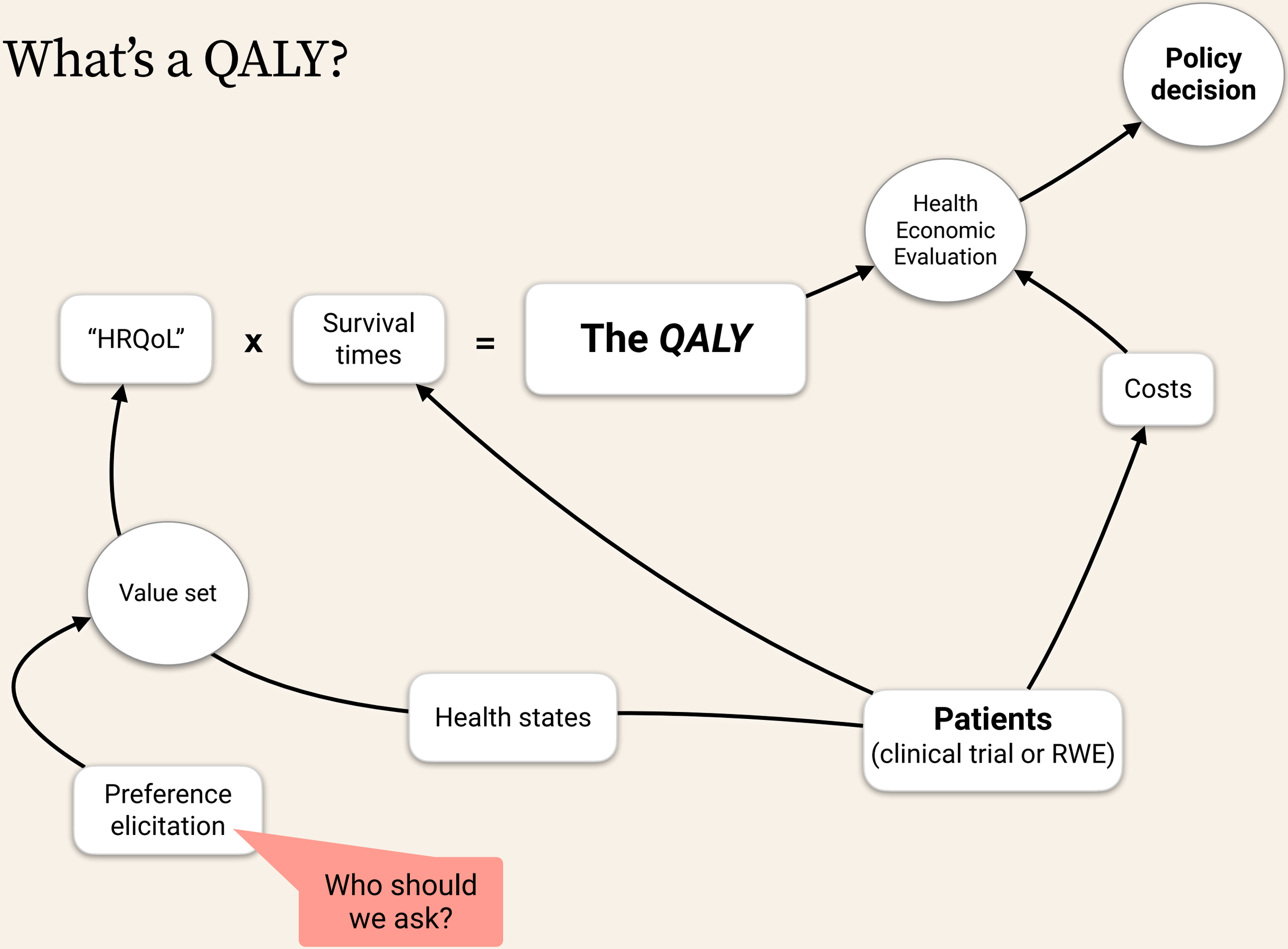
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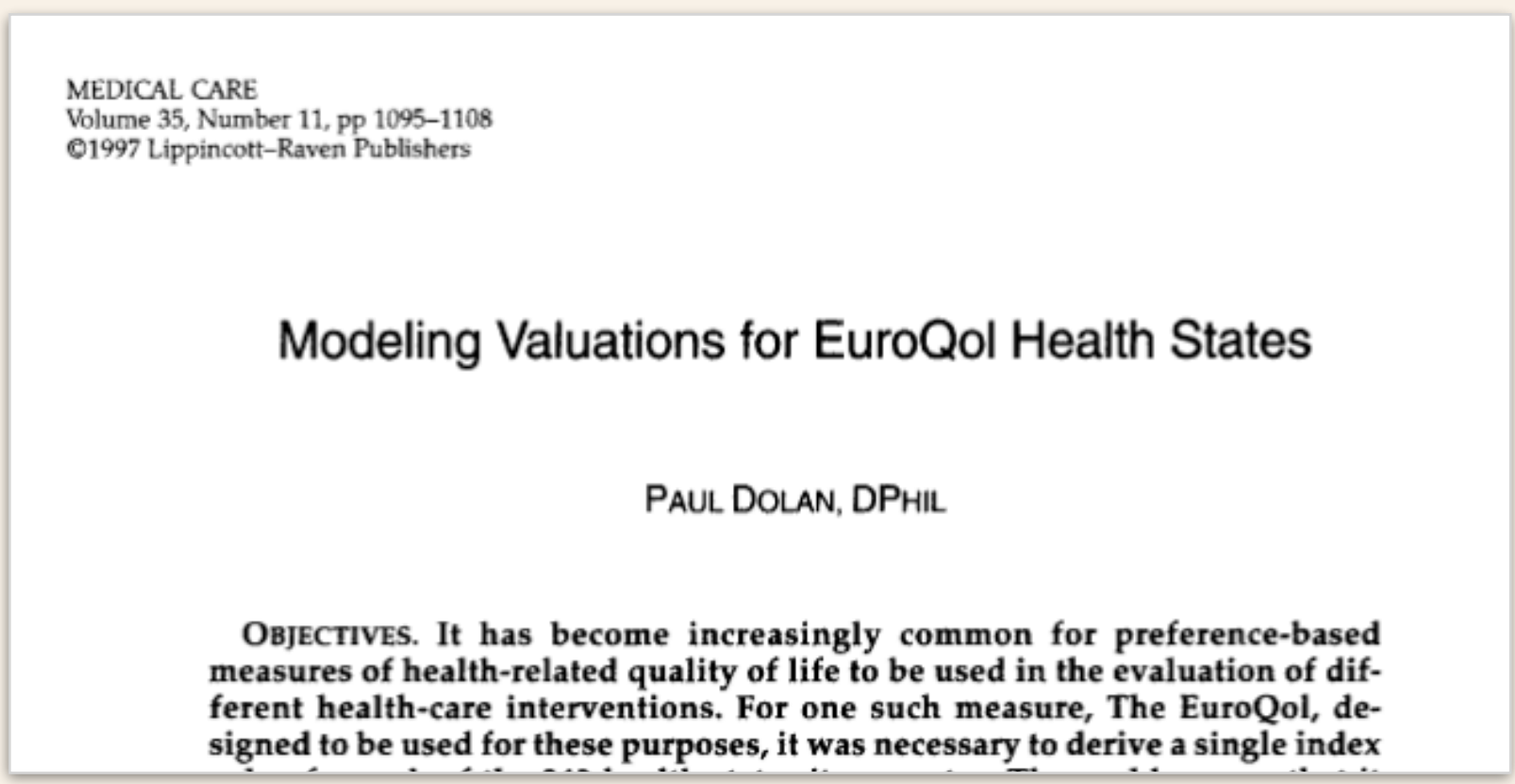
Preference elicitation
Who should we ask?

“[F]or purposes of resource allocation, the relevant preferences are those of the general public”

- The Washington Panel on Cost-effectiveness, 1996



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Modeling Valuations for EuroQol Health States

PAUL DOLAN, DPHIL

OBJECTIVES. It has become increasingly common for preference-based measures of health-related quality of life to be used in the evaluation of different health-care interventions. For one such measure, The EuroQol, designed to be used for these purposes, it was necessary to derive a single index

- Dolan et al., 1997

Sources of preference weights in HTA guidelines

General population	
Australia	Japan
Belgium	Malaysia
Brazil	Mexicof
Canada	The Netherlands
Chile	New Zealand
Columbia	Norway
Croatia	Poland
Czech Republic	Portugal
England	Scotland
France	Singapore
Hungary	South Koreag
Irand	Taiwan
Ireland	Thailand
Israel	

Patients
Germany
Sweden

Kennedy-Martin M, Slaap B, Herdman M, van Reenen M, Kennedy-Martin T, Greiner W, Busschbach J, Boye KS. Which multi-attribute utility instruments are recommended for use in cost-utility analysis? A review of national health technology assessment (HTA) guidelines. The European Journal of Health Economics. 2020 Nov;21:1245-57.

What has changed?

Who should we ask?

The General Public or Patients?

The role of patient preferences in economic evaluations: barriers and opportunities for a patient-based QALY

Issue panel ISPOR Copenhagen 13 November 2023

IQWiG - Scientifically independent

- We assess the benefit or harm of medical interventions for patients.
- The contents of the assessments are not influenced by payers (health insurance funds), service providers, industry or politics.
- Neither the Institute nor its staff members receive any payments by third parties, such as industry.

Where do we come from?

Health Economic Evaluation of drugs in Germany

- High relevance of the benefit assessment of drugs as an essential basis for price negotiations
  - Other possibly relevant information: e.g. price in other European countries, expected annual sales volume, therapy costs of the comparator(s)
- Patient perspective is of great importance
  - Assessment of patient-relevant outcomes, involvement of patients and other affected persons
- Health economic evaluation is a theoretical option under specific circumstances
  - No commission since the start of early benefit assessment in 2011

How to value health states

Who	General public	Patients
What	Hypothetical health state	Own health state
When	Ex ante	Ex post
Examples	Sample of the German general public values health states described with the EQ-5D 5L Ludwig et al. German Value Set for the EQ-5D-5L. Pharmacoeconomics 2018; 36(6): 663-674.	Patients with Coeliac disease valuing their current health state Angyal et al. Health utilities and willingness to pay in adult patients with coeliac disease in Hungary. Qual Life Res. 2023 Sep;32(9):2503-2516.

Who should provide the values?

Helgesson 2020:

- systematic literature review of arguments regarding the most accurate source for valuation of health states - patients or general public
- structural similarity between the two sets of arguments with different interpretations,
  - e.g. distortion due to adaptation, focussing effects, difficulties in providing values

- *„none of the debated positions is flawless..“*
- *“suggests that the most accurate source of information for valuation of health states is that based on experience….”*

Helgesson etal. Whom should we ask? A systematic literature review of the arguments regarding the most accurate source of information for valuation of health states. Qual Life Res 2020; 29(6): 1465-1482.

Does it matter?

- Differences between patients and the general public exist
  - Meta-analysis found significantly higher utility values for patients
- Size of the effect and direction might differ depending on, e.g.:
  - Disease in question, kind of impairment, severity of health status
  - Type of intervention (curative or life extending)
  - Valuation technique (TTO, SG, VAS), design of the valuation study

Peeters et al. Health state valuations of patients and the general public analytically compared: a meta-analytical comparison of patient and population health state utilities. Value Health 2010; 13(2): 306-9.

Brazier et al. Measuring and Valuing Health Benefits for Economic Evaluation. Oxford: Oxford University Press; 2017.

Gandhi et al. Comparison of health state values derived from patients and individuals from the general population. Qual Life Res. 2017 Dec;26(12):3353-3363.

Ludwig et al. To What Extent Do Patient Preferences Differ From General Population Preferences? Value Health. 2021 Sep;24(9):1343-1349.

Should QALYs reflect patients´ instead of societal preferences….

IQWiG’s perspective

- Patients appear to be the most accurate source of information for utilities because it can be assumed that they are better informed about their own state of health.

- The inclusion of the perspective of patients is of great importance to the IQWiG:
  - The German Social Code stipulates that certain aspects of "patient benefit" are to be adequately considered in the health economic evaluation.

- Recommendation in IQWiG methods:
  - Utility values included in the analysis should primarily be based on assessments by patients.

…and if so, how?

Possible approaches for discussion

- Direct utility assessment: e.g. valuations by patients using TTO
- Indirect utility assessment: e.g. patient or experience-based tariffs
- New approaches: e.g. Online Personal Utility Functions (OPUF) tool

Gamper et al. EORTC Quality of Life Group. The EORTC QLU-C10D discrete choice experiment for cancer patients: a first step towards patient utility weights. J Patient Rep Outcomes. 2022 May 4;6(1):42.

Chai et al. Valuation of EQ-5D-5L health states from cancer patients' perspective: a feasibility study. Eur J Health Econ. 2023 Oct 14.

Burström et al. Experience-Based Swedish TTO and VAS Value Sets for EQ-5D-5L Health States. Pharmacoeconomics. 2020 Aug;38(8):839-856

Schneider et al. The Online Elicitation of Personal Utility Functions (OPUF) tool: a new method for valuing health states. Welcome Open Res. 2022 Jan 14;7:14.

Institute for Quality and Efficiency in Health Care (IQWiG)

Im Mediapark 8
50670 Köln

Telefon +49 221 35685-0
Telefax +49 221 35685-1

info@iqwig.de

www.iqwig.de
www.gesundheitsinformation.de
www.themencheck-medizin.de

@iqwig@wisskomm.social
@iqwig_gi@wisskomm.social

Patient-based QALYs

- view from Sweden

Martin Henriksson

Associate professor, Linköping University

INTRODUCTION

- Priority setting on many levels in Sweden
- Perhaps the most formalized process is that of reimbursement of prescription drugs
- The dental and pharmaceutical benefits agency (TLV) decides on drug reimbursement based on cost-effectiveness (and other criteria)

TLV GUIDANCE REGARDING ECONOMIC EVALUATION

- Dental and pharmaceutical benefits agency (TLV) in Sweden recommends QoL-weights based on patients in the condition of interest

7. Calculation of weightings for life quality adjustment

QALY-weightings should be based on methods such as the Standard Gamble (SG) or Time-Trade-Off (TTO) methods. In a second instance, QALY-weightings should be based on the rating scale method. QALY-weightings can be based either on direct measurements with the above-mentioned methods or indirect measurements (where a health classification system such as EQ-5D is linked to QALY-weightings). QALY weightings based on appraisals of persons in the health condition in question are preferred before weightings calculated from an average of a population estimating a condition depicted for it (e.g. the "social tariff" from EQ-5D). Using weightings for current health conditions collected from previous studies may be a solution.

A SWEDISH EXPERIENCE-BASED VALUE SET FOR EQ-5D?

- TLV´s view and practice motivated the development of Swedish experience-based EQ-5D value sets

- Where did this lead us?

SWEDISH VALUE SETS FOR EQ-5D

EQ-5D-5L work completed in 2020

PharmacoEconomics
https://doi.org/10.1007/s40273-020-00905-7

ORIGINAL RESEARCH ARTICLE

Experience-Based Swedish TTO and VAS Value Sets for EQ-5D-5L Health States

Kristina Burström[1,2] · Fitsum Sebsibe Teni[1] · Ulf-G. Gerdtham[3,4] · Reiner Leidl[5,6] · Gert Helgesson[7] · Ola Rolfson[1,8,9,10] · Martin Henriksson[11]

EQ-5D-3L work completed in 2014

Qual Life Res (2014) 23:431–442
DOI 10.1007/s11136-013-0496-4

Swedish experience-based value sets for EQ-5D health states

Kristina Burström · Sun Sun · Ulf-G Gerdtham · Martin Henriksson · Magnus Johannesson · Lars-Åke Levin · Niklas Zethraeus

THE EQ-5D WORK

5. Kryssa under varje rubrik bara i EN ruta som bäst beskriver din hälsa IDAG.

a) Rörlighet
- [x] Jag har inga svårigheter med att gå omkring
- [ ] Jag har lite svårigheter med att gå omkring
- [ ] Jag har måttliga svårigheter med att gå omkring
- [ ] Jag har stora svårigheter med att gå omkring
- [ ] Jag kan inte gå omkring

b) Personlig vård
- [x] Jag har inga svårigheter med att tvätta mig eller klä mig
- [ ] Jag har lite svårigheter med att tvätta mig eller klä mig
- [ ] Jag har måttliga svårigheter med att tvätta mig eller klä mig
- [ ] Jag har stora svårigheter med att tvätta mig eller klä mig
- [ ] Jag kan inte tvätta mig eller klä mig

c) Vanliga aktiviteter
(t ex arbete, studier, hushållssysslor, familje- eller fritidsaktiviteter)
- [x] Jag har inga svårigheter med att utföra mina vanliga aktiviteter
- [ ] Jag har lite svårigheter med att utföra mina vanliga aktiviteter
- [ ] Jag har måttliga svårigheter med att utföra mina vanliga aktiviteter
- [ ] Jag har stora svårigheter med att utföra mina vanliga aktiviteter
- [ ] Jag kan inte utföra mina vanliga aktiviteter

d) Smärtor / besvär
- [ ] Jag har varken smärtor eller besvär
- [ ] Jag har lätta smärtor eller besvär
- [x] Jag har måttliga smärtor eller besvär
- [ ] Jag har svåra smärtor eller besvär
- [ ] Jag har extrema smärtor eller besvär

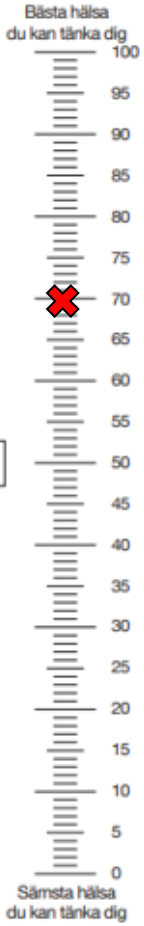
e) Oro / nedstämdhet
- [ ] Jag är varken orolig eller nedstämd
- [ ] Jag är lite orolig eller nedstämd
- [ ] Jag är ganska orolig eller nedstämd
- [ ] Jag är mycket orolig eller nedstämd
- [x] Jag är extremt orolig eller nedstämd

1. I have no problems in walking about
1. I have no problems washing or dressing myself
1. I have no problems doing my usual activities
3. I have moderate pain or discomfort
5. I am extremely anxious or depressed

6.

- Vi vill veta hur bra eller dålig din hälsa är IDAG.
- Den här skalan är numrerad från 0 till 100.
- 100 är den bästa hälsa du kan tänka dig. 0 är den sämsta hälsa du kan tänka dig.
- Sätt ett X på skalan för att visa hur din hälsa är IDAG.
- Skriv nu i rutan nedan det nummer du har markerat på skalan.

DIN HÄLSA IDAG: [ ][ ][ ]

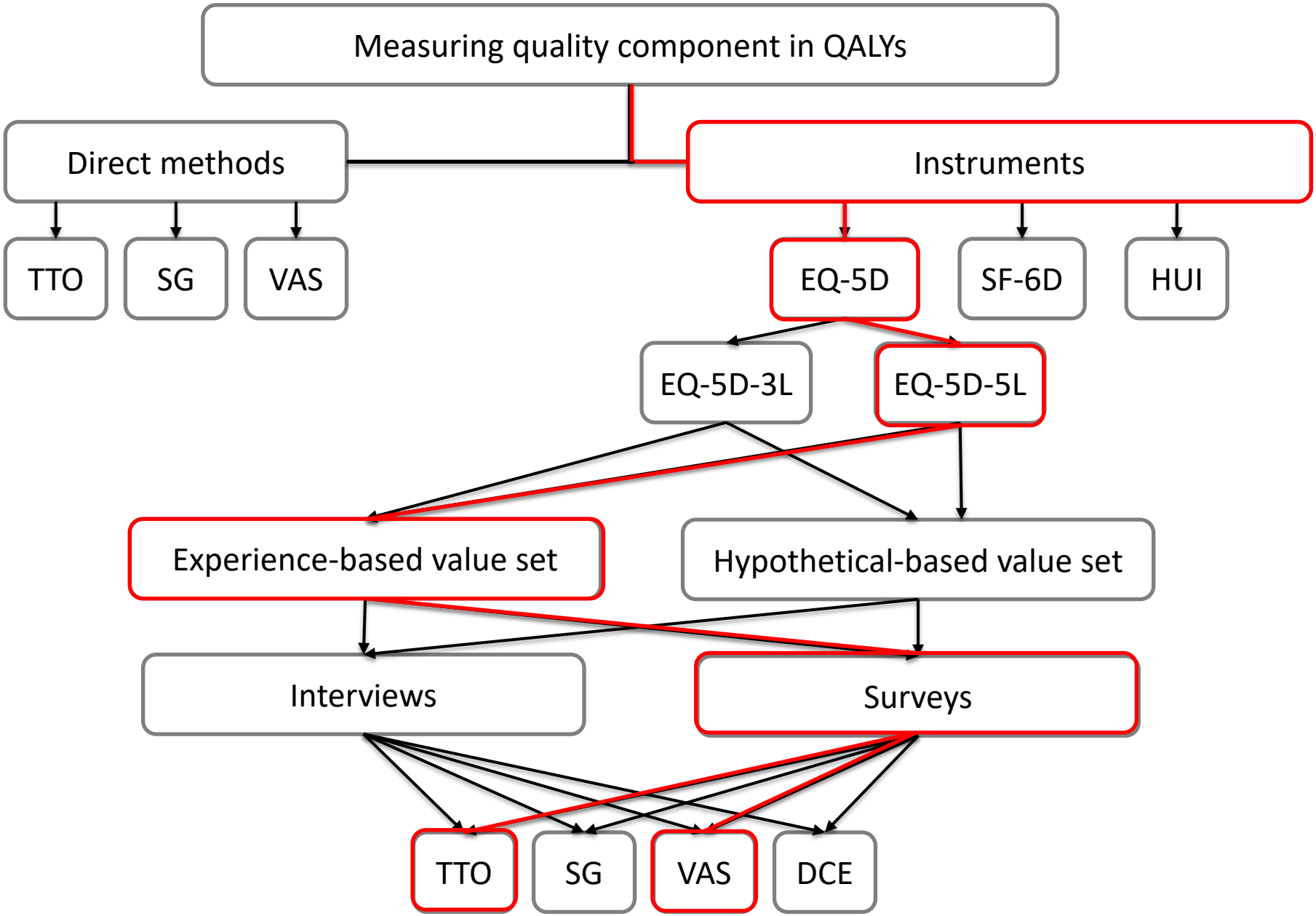


7. Tänk dig att du får reda på att du har 10 år kvar att leva. I samband med detta får du välja mellan att leva dessa 10 år i ditt nuvarande hälsotillstånd, eller att avstå något/några år för att istället leva kortare tid med full hälsa.

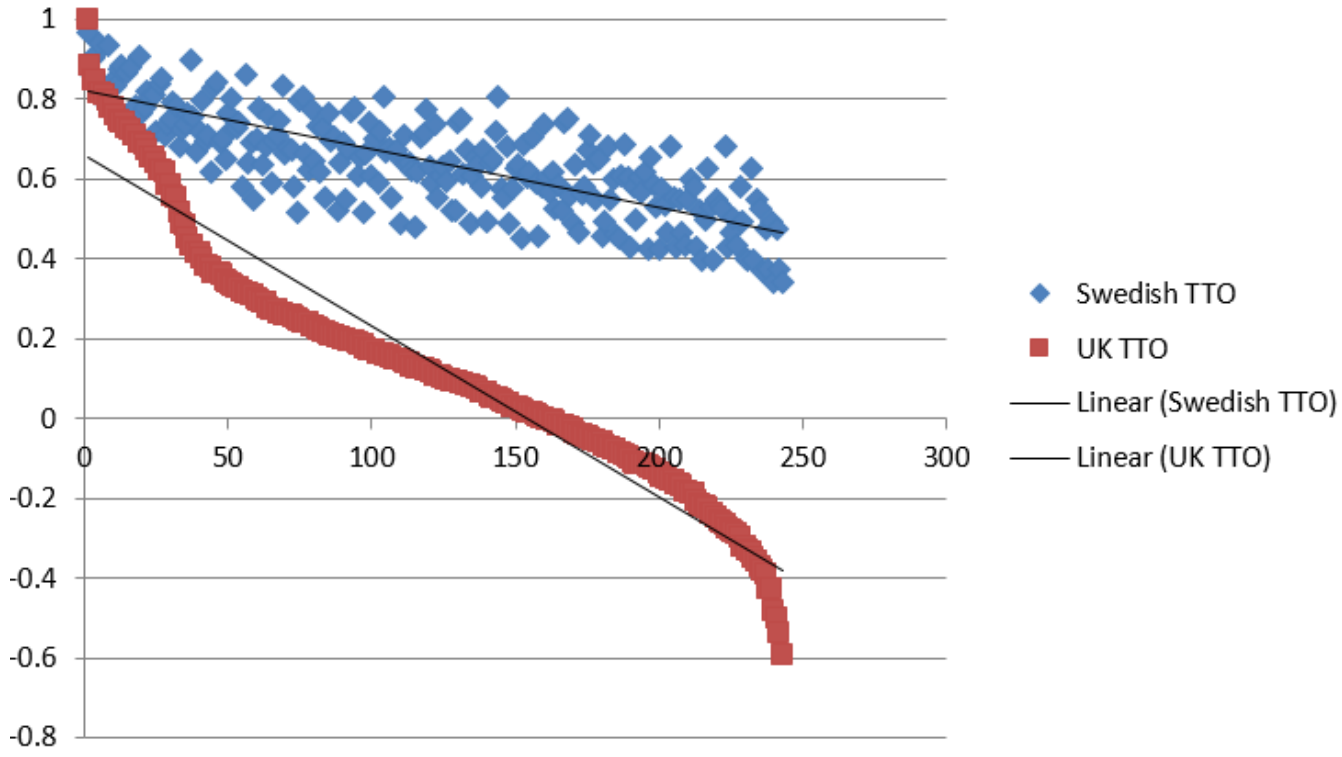
Markera med ett kryss (X) på linjen det antal år med full hälsa som du tycker är likvärdigt med att leva 10 år i ditt nuvarande hälsotillstånd.

Antal år med full hälsa
(Anser du att du för närvarande har full hälsa ska du kryssa i 10 år)

APPROACH TO VALUATION



EXPERIENCE-BASED VALUATIONS VS HYPOTHETICAL



WHAT ABOUT THE EXPERIENCE-BASED VALUATIONS?

- To be fair – not much happened at all

- Why?
  - Not sure to be honest
  - Comparability
    - One should not underestimate the difficulty of changing practice
  - Uncertainty about the methods of the valuation study
    - Survey-based TTO question etc.

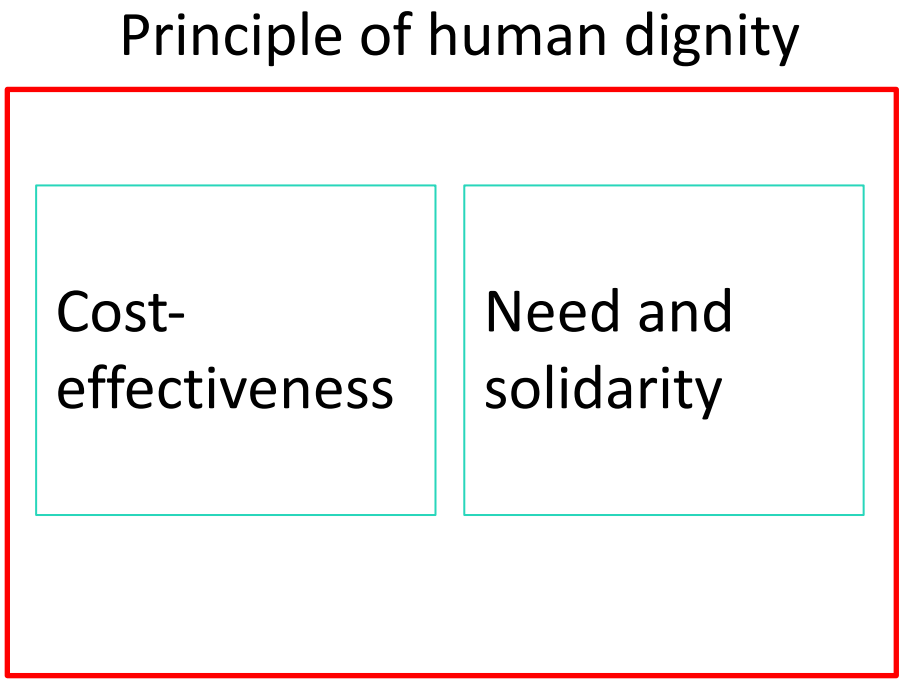
A FINAL DIGRESSION ON EQUITY AND HYPOTHETICAL VALUATIONS

- What are the implications of using experience-based or hypothetical valuations in systems where disease severity is one priority setting criterion?

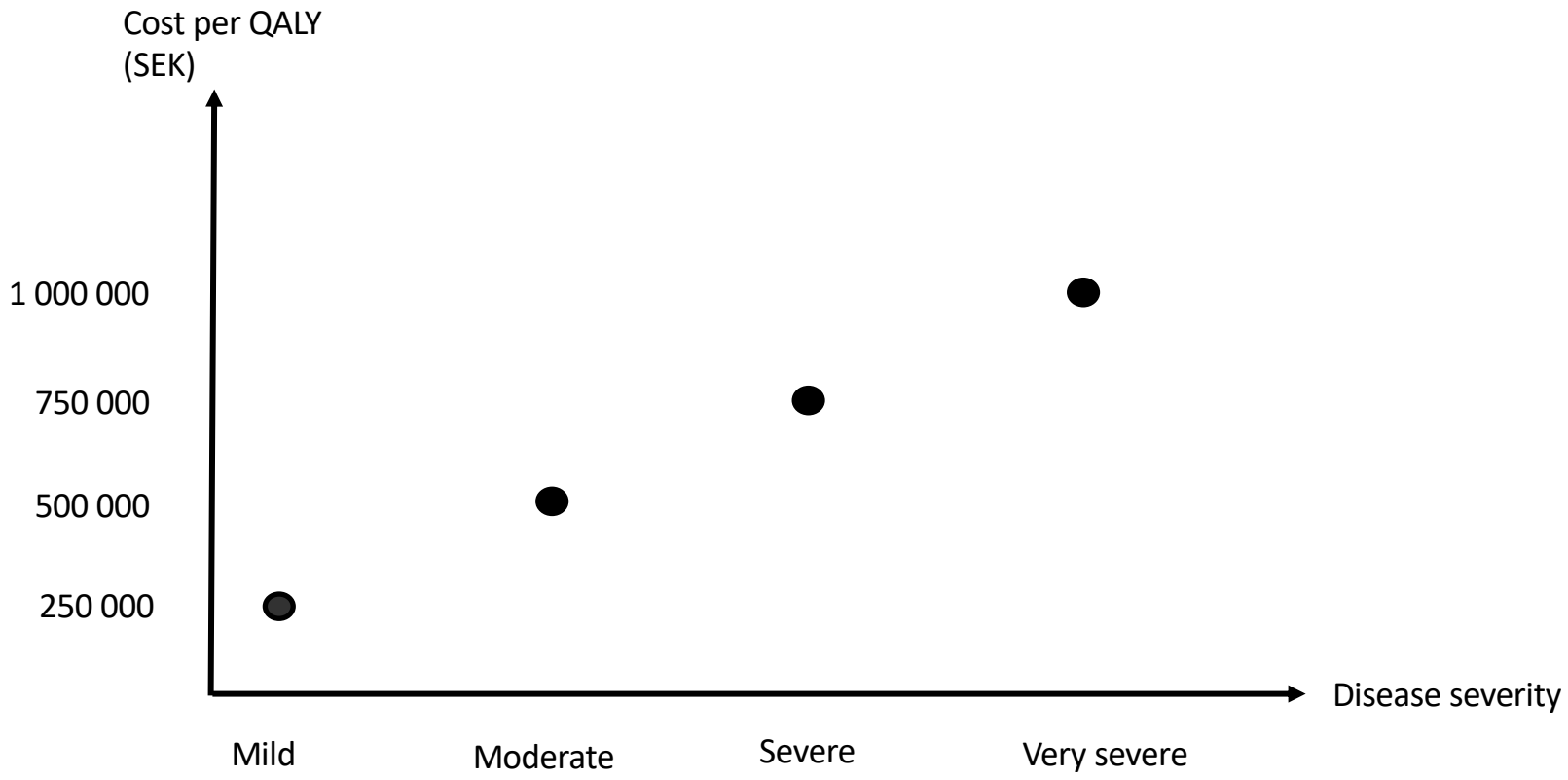
Legislation and the Swedish ethical platform

- The principle of human dignity
  - all individuals have equal rights regardless of personal characteristics and position in society
- The principle of need and solidarity
  - resources should be used in domains (or patients) where needs are considered to be largest
- The principle of cost-effectiveness
  - resources should be used in the most effective way without neglecting fundamental duties concerning the improvement of health and quality of life

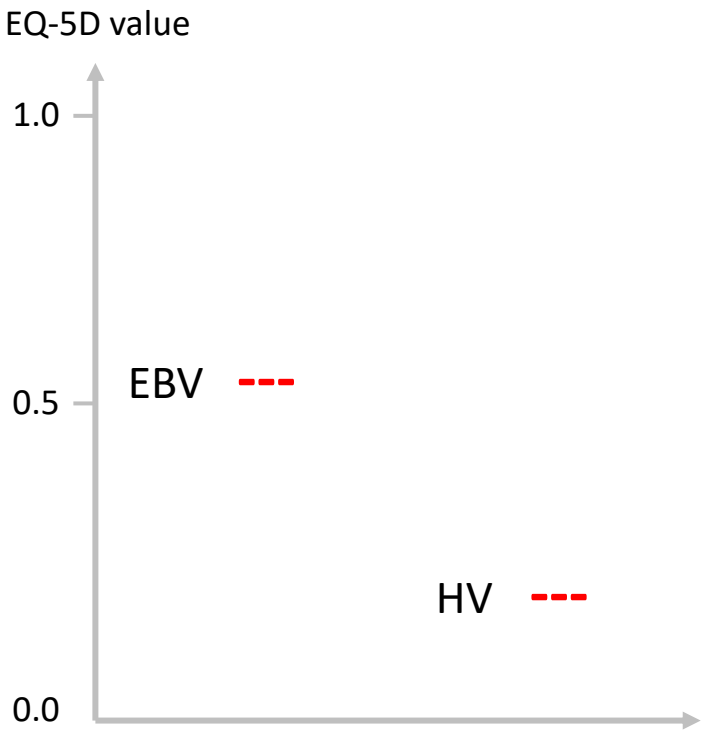
PRIORITY SETTING PRINCIPLES IN SWEDEN



OPERATIONALIZING PRINCIPLES IN DRUG REIMBURSEMENT



EXAMPLE: HEALTH STATE 21223

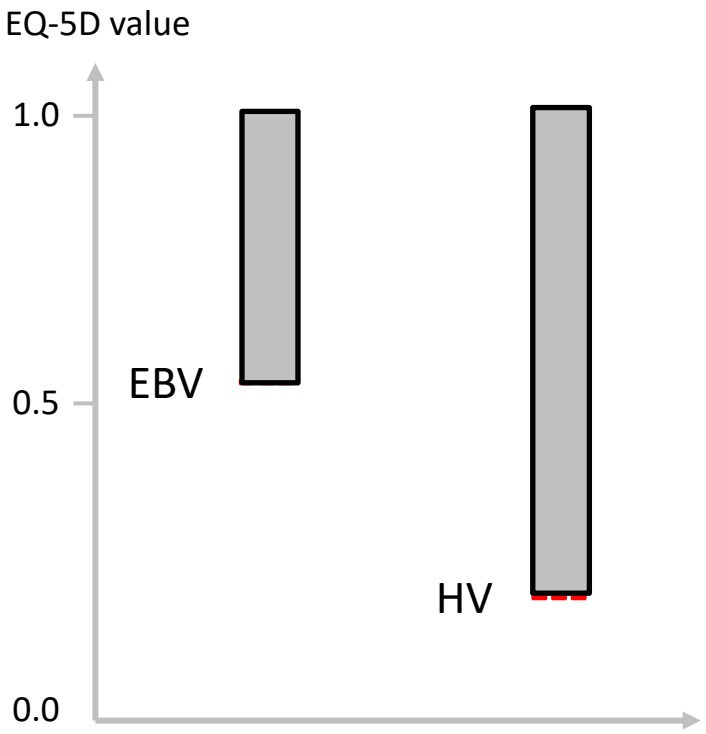


Health state 21223 with

Experience-based valuation (EBV) = 0.5161

Hypothetical valuation (HV) = 0.186

EXAMPLE: HEALTH STATE 21223 WITH A LARGE HEALTH IMPROVEMENT

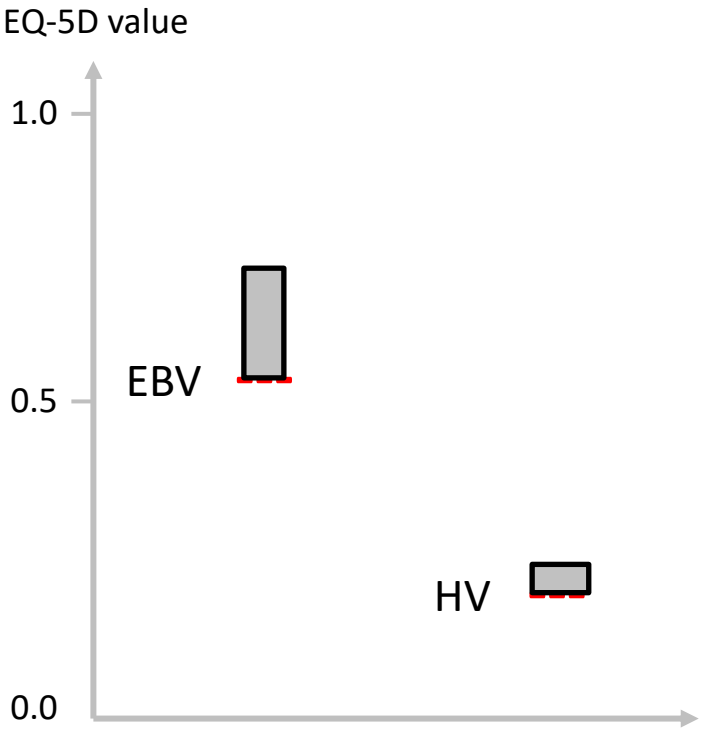


An intervention that takes individuals from 21223 to 11111

Experience-based valuation (EBV): QALY-gain 0.484 in population with disease severity

Hypothetical valuation (HV): QALY-gain 0.814 in population with very high disease severity

EXAMPLE: HEALTH STATE 21223 WITH A SMALL HEALTH IMPROVEMENT



An intervention that takes individuals from 21223 to 32211

Experience-based valuation (EBV): QALY-gain 0.157 in population with moderate severity

Hypothetical valuation (HV): QALY-gain 0.01 in population with very high disease severity

About Patient QALYs

Ben van Hout

University of Sheffield, UK

Open Health, UK

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