CAHTA Center for Analyses and Health Techology Assessment

Introduction to cost and utility assessment methods

Hochschule für Angewandte Wissenschaften Hamburg
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ECON – EPI
Experts in Health Economics and Epidemiology
RESEARCH | CONSULTING | TRAINING

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Outline

- Introduction – why cost and utility assessment?
- Cost assessment methodology
  - Direct and indirect costs
  - Perspectives
  - Process of cost estimation
  - Example
- Cost-of-illness studies and budget impact models
- Utility assessment
  - "unit" of health effects
  - instruments to measure health-related quality of life
- Overview: cost-utility assessment in different countries
- Summary

Why cost and utility assessment?

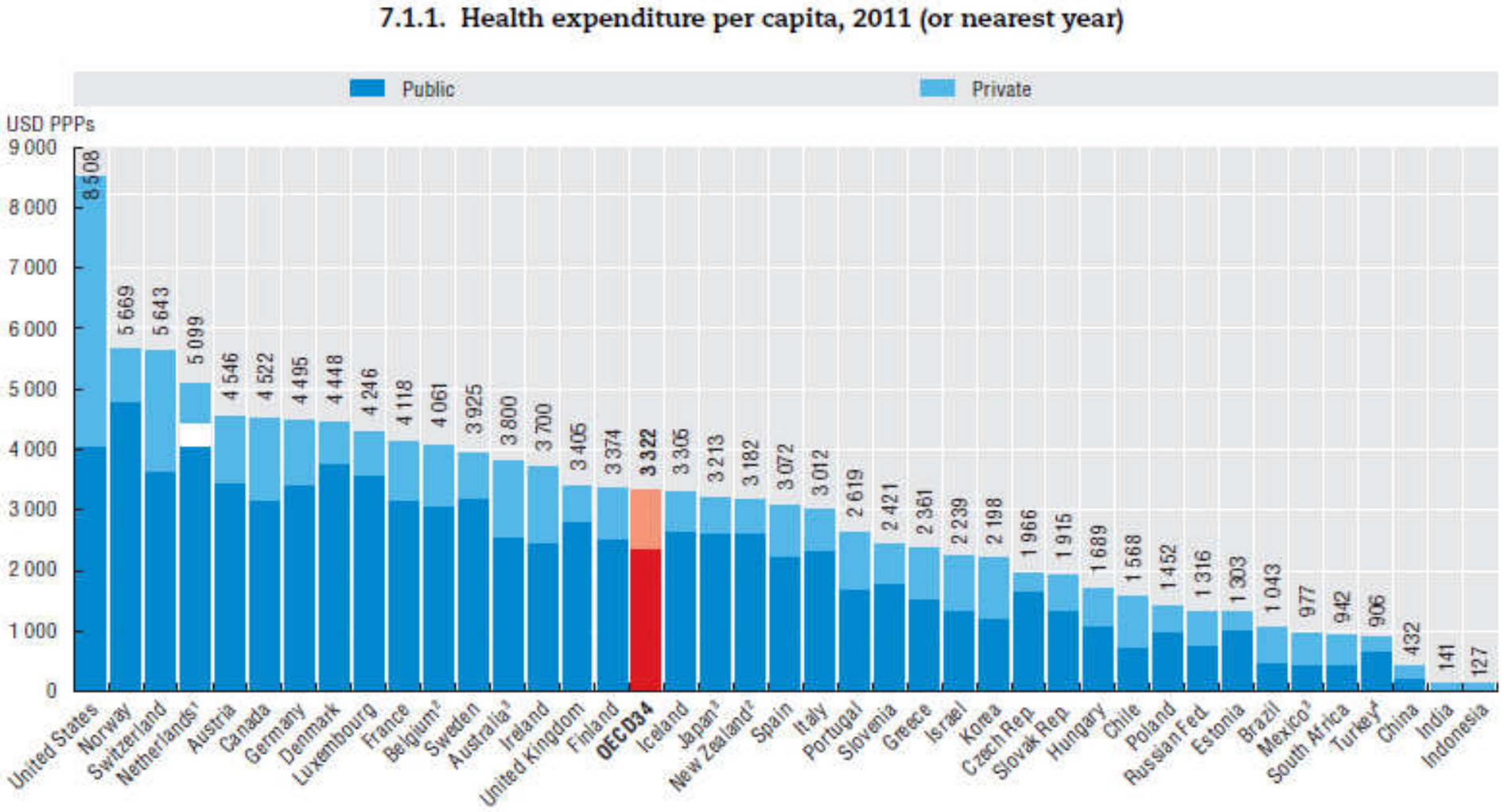
- **fact of life: “scarce“ healthcare resources**

- **evidence-generation: paradigm is to *measure***
  - **cost** of care
  - **quality** of care
  - **efficiency** of care

- **Accountability: transparent decision-making calls for**
  - cost-of-illness analysis (→ public health priority-setting!)
  - budget impact analysis
  - cost-effectiveness and cost-utility analysis

Hutubessy et al. 2003, IQWiG 200

Scarcity: a relative concept



Source: OECD Health at a Glanc

Cost assessment

Cost types: direct and indirect costs

	Direct	Indirect
Medical	Costs related to the disease from the perspective of the health care payer(s) (e.g. hospital stays, procedures and diagnostics, drugs)	(***)
Non-medical	Costs which do not arise in the healthcare sector, but which are still related to the disease, e.g., travel costs, special diet, patient time	• Incapacity to work (illness) • occupational disability (long-term illness or disability) • premature death

(***) some authors allocate future health care costs, incurred during the years of extended life span, to this “box“

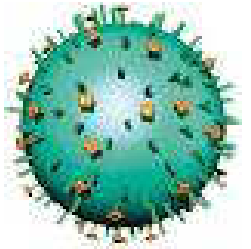
Table adapted from Annemans 2008, IQWiG 2009a

Costs by perspective

pective	Direct Costs		Indirect Costs		Trans paym
	medical	Non-medical	Morbidity-driven	Mortality-driven	
insurance	Hospital stays, Outpatient care, Drugs, devices	e.g. transportation costs	—	—	—
insurance rs	Statutory benefits (e.g. long-term care)	e.g. premature retirement benefits, widow/orphan benefits	—	—	—
esses	Employer contributions	—	Presenteeism Absenteeism	Costs in friction period	—
nment	Gov't contributions	e.g. premature retirement benefits e.g. criminal justice costs (see schizophrenia)	e.g. reduced tax revenue (assuming full employment)	e.g. lost future tax revenue (assumes full employment)	Paid-out (disease-b transfer payments
e holds	Out-of-pocket (OOP) payments	e.g. house remodelling (staircase lift)	Loss of household income or household production	Loss of household income or household production	Received transfer payment
y/ economy	All costs	All costs	All costs	All costs	—

Question 1

In the influenza season, a sick worker (with influenza) reports into work nevertheless, but he/she is less productive at work.

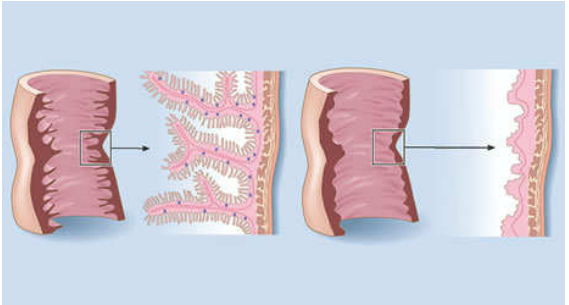


- What cost type are we talking about?
- How difficult or easy to measure?
- What other costs could the aforementioned behaviour lead to?

If he/she stayed at home,
• what cost type?
• how difficult or easy to measure?
• Probability P and length L of home-stay: country-specific?

Question 2

A coeliac disease patient has more physician contacts per year than a healthy patient, and needs to follow a gluten-free (more expensive) diet.



- What cost types need to be collected?
- Which analytical perspective would capture the full cost of coeliac disease?

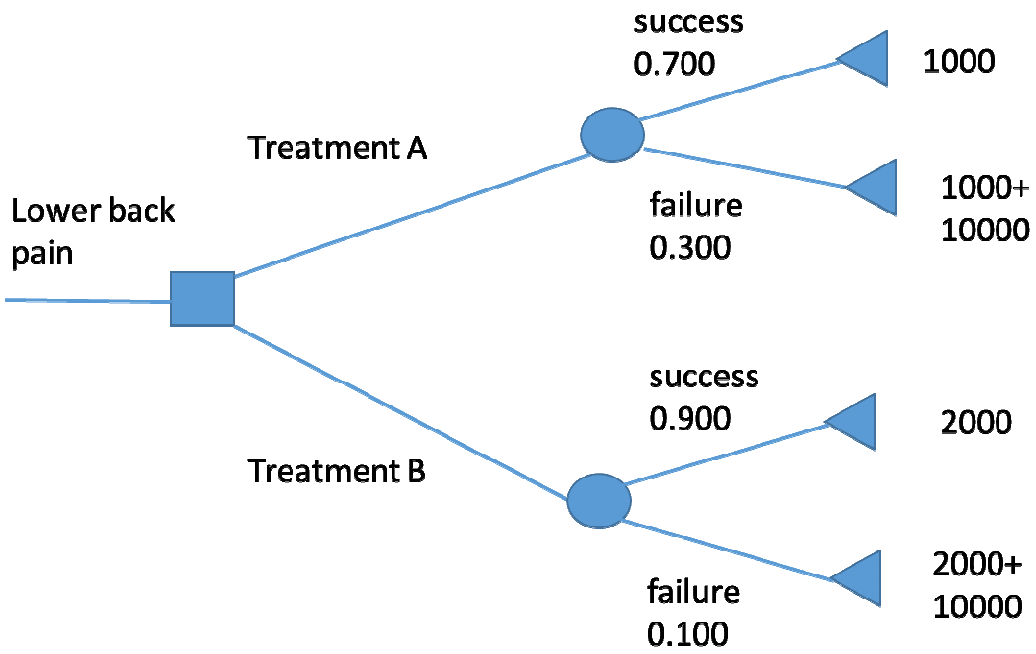
Process of cost estimation

1. **Identification of resource consumption items**
2. **Quantifying resource consumption (RC)**
3. **Valuation of resource units**
4. **Calculating total costs of intervention options**

IQWiG 2009
Krauth *et al*

Process of cost estimation (1): Identification of resource consumption items

- identify **relevant items** along therapeutic pathway!



IQWiG 2009a, Annemans 2008

Process of cost estimation (1), Identifying resource items, *cont'd.:*
Sources for identifying resource consumption items

- original and review studies
- **clinical practice guidelines**
- **administrative and accounting data** (e.g. data from all health insurance funds)
- models (including combining data from various sources)
- expert opinions

IQWiG 2009a

Side-note: guidelines and reality

Neuropsychiatric Disease and Treatment

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ORIGINAL RESEARCH

Do neurologists in Germany adhere to the national Parkinson's disease guideline?

This article was published in the following Dove Press journal:
Neuropsychiatric Disease and Treatment
4 March 2011
Number of times this article has been viewed

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Abstract: Implementation of guidelines can improve clinical practice. The aim in this study was to investigate whether neurologists in Germany adhered to the national Parkinson's disease guideline. Data were obtained from a cross-sectional survey of 60 neurologists. Analyses were performed on 320 patients with idiopathic Parkinson's disease with either low grades of functional impairment (Hoehn and Yahr stage I) or higher grades of functional impairment (stage II–V) but without motor complications. The sample was divided into four groups depending on age and grade of functional impairment. For each group, a biometric parameter on the use of dopamine agonists and L-dopa was defined based on the guideline. In patients aged <70 years, the recommendation to use dopamine agonists without L-dopa (parameter 1) was observed in 53% of patients with lower grades of functional impairment, whilst recommended use of dopamine agonists in more functionally impaired patients (parameter 2) was followed to a greater extent (84%). In patients aged ≥70 years, recommendations to use L-dopa without dopamine agonists were adhered to in only 50% of less functionally impaired (parameter 3) and 52% of more functionally impaired (parameter 4) patients. In conclusion, our results indicated there was moderate but not full adherence to the guideline.

Keywords: Parkinson's disease, dopamine agonists, L-dopa, neurologists, national guideline, Germany

Process of cost estimation (2): Quantifying resource consumption
Micro-costing vs. macro-costing

Macro-costing:

- identification and measurement of **composite intermediate products and services** (e.g. inpatient days)

Micro-costing:

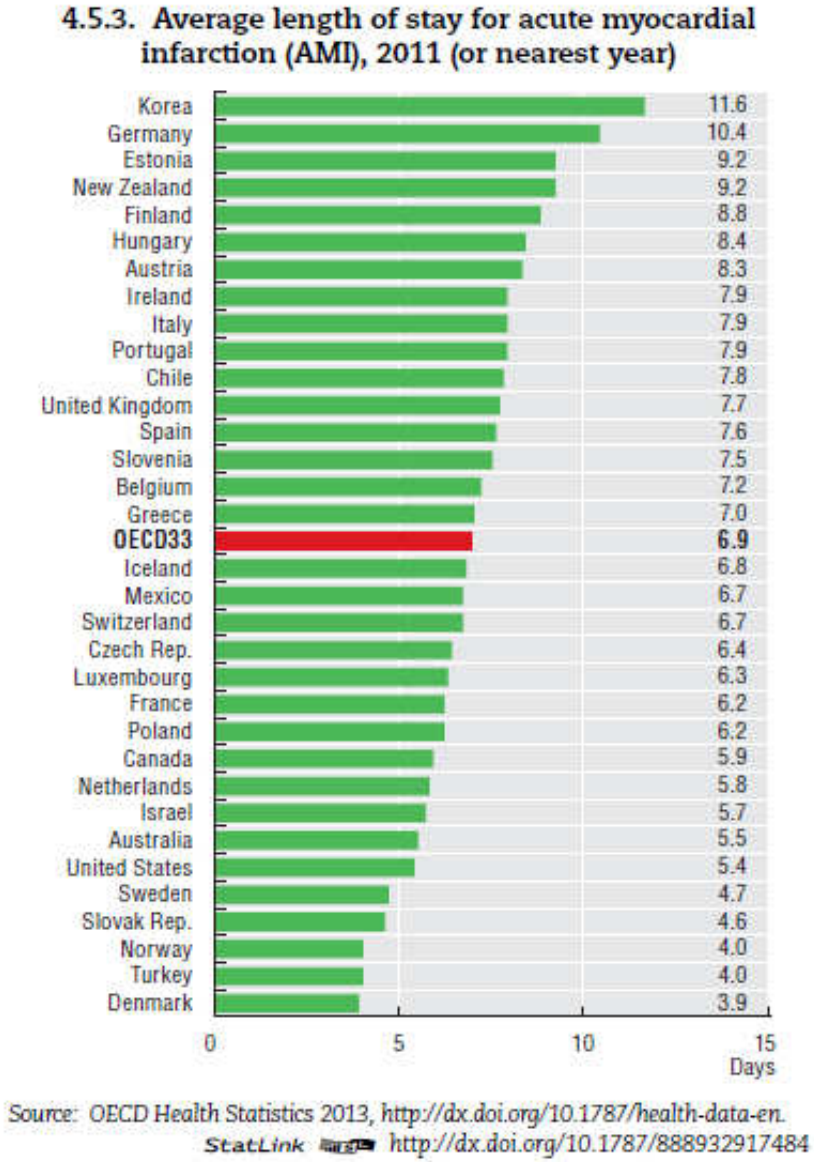
- detailed identification and measurement of services (e.g. a hospital stay broken down into **individual components** such as consultation, operation, medication, diagnostics, nursing, accomodation, food, cleaning, etc.)

- determination of required resources used (personnel, material, equipment, building, overheads, etc.)

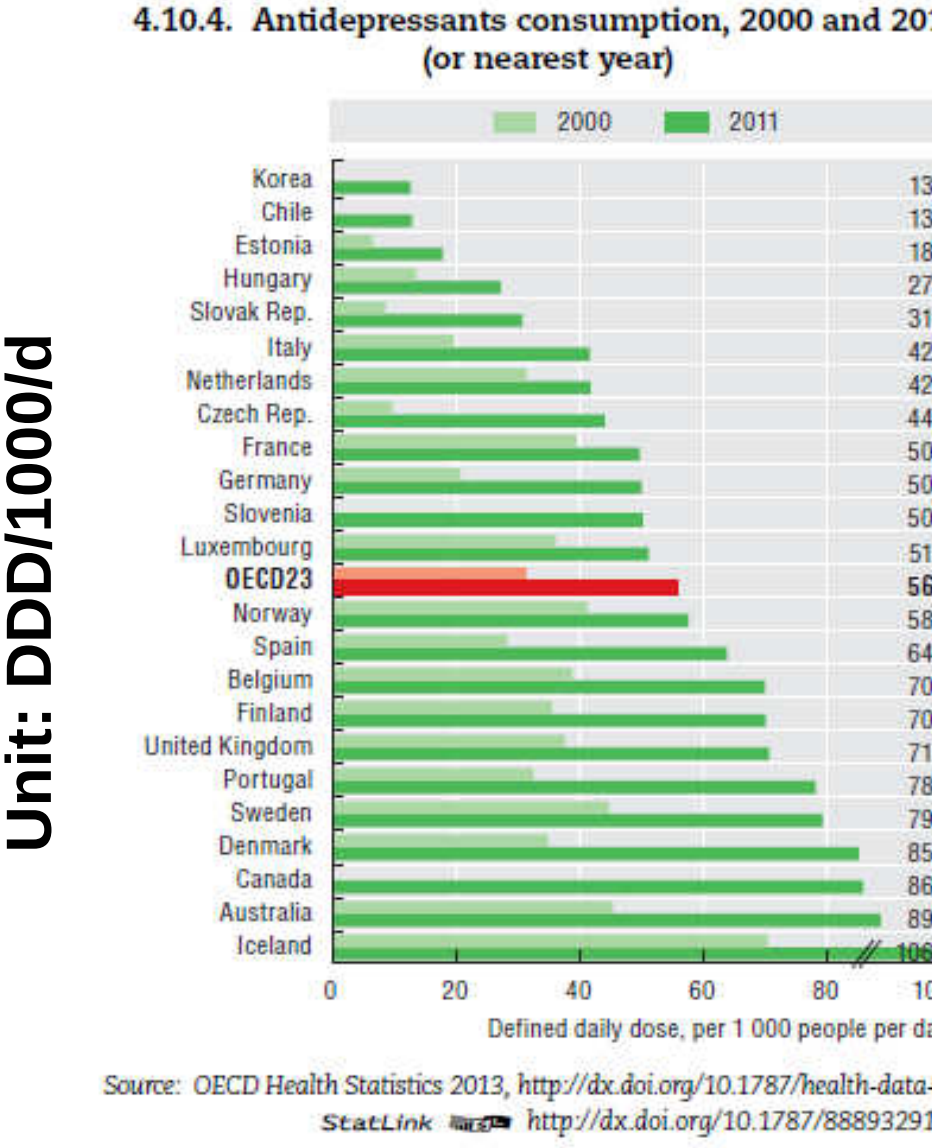
IQWiG 2009a

Macro-level RC quantification

Unit: days



Unit: DDD/1000/d



Process of cost estimation (3): valuation of resource units
(here: micro-costing approach)

$$Cost = \sum_{i=1}^{n} RC_i \cdot P_i$$

where *i represents the specific items/materical/resource considered (from i=1 to the n^{th} item)*
RC = resource consumption
P = unit price

Question on perspectives

For a regular on-term delivery of a baby:

- List a few resource items
- How should these be “costed out” from the **hospital**’s perspective, i.e. what is the cost of the delivery to the provider?
- What is the cost of the delivery to the **payer**?
- What incentives does this difference in numerical results create?

Process of cost estimation (3): Valuation of resource units

Micro-costing vs. macro-costing

nple: Grades of sion in hospital ng

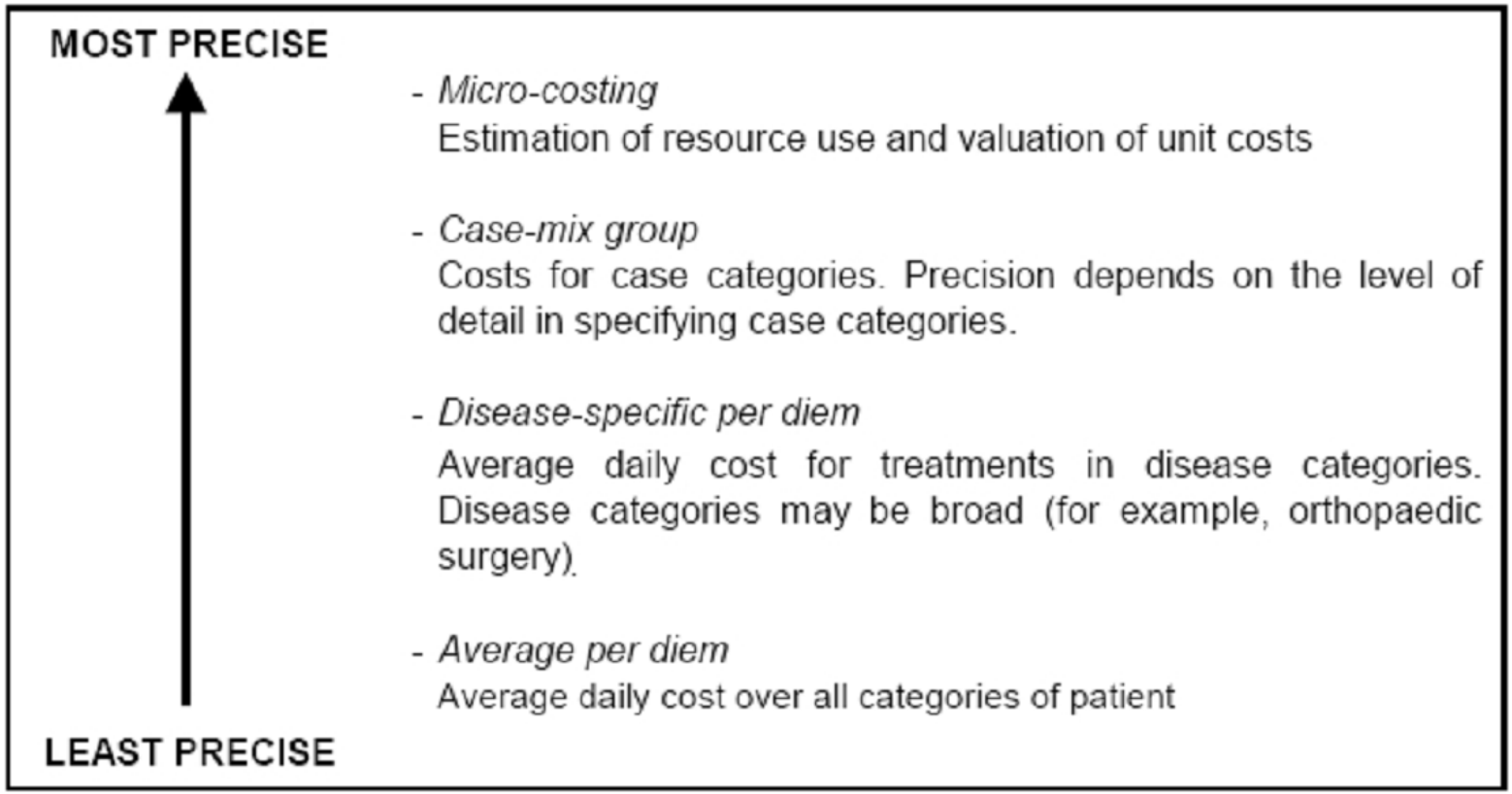


Figure taken from IQWiG 2009a

Process of cost estimation (3), Valuation of resource units

Micro-costing vs. macro-costing, *cont'd.*

Factors influencing the required precision of cost and estimations:

- unit costs of products and services
- frequency of utilization
- point in time in the course of a chronic disease
- variations between patients
- variability in intervention options

IQWiG 2009a

Quiz: macro or micro costing?

- The products and services used are cost-intensive:
- The products and services tend to be used very often:
- Within a chronic disease, the event/endpoint (e.g. fracture, MI, …) is taking place in the distant future:
- The variation between patients is small:
- The variability in intervention options is large:

IQWiG 2009a

Use of micro-costing

- cost-effectiveness analyses
- stand-alone studies

- **micro-costing studies are suitable**
  - for **new interventions**, where no average cost can be calculated
  - for the examination of **within-procedure variation**, and
  - for the incorporation of **non-market goods** for which standardized cost estimates are not available

Frick 200

Caveats in costing and utility assessment (1): prospective health economic evaluations

- protocol-induced costs
- protocol-induced findings
- exclusion of patients
- alternative treatment
- termination of the study
- time horizon

Annemans 2008

Caveats in cost and utility assessment (2): retrospective health economic evaluations

Caveats

- no randomization of treatment allocation (selection bias)
- incomplete data
- time-frame: not suitable for new technologies or treatments
- impossible to measure quality of life (e.g. EQ-5D) *post hoc (ex post)*

Annemans 2008

Example: rheumatoid arthritis in Germany

- aim: development of a systematic set of cost data on rheumatoid arthritis (RA)
- data source: healthcare payer data pool
- methods: 338 RA patients treated by rheumatologists, retrospectively from July 2000 to June 2001, **patient-by-patient micro-costing** approach
- perspective: healthcare payer

Ruof et al. 2003

Example: rheumatoid arthritis in Germany, *cont'd.*

Table 2 RA related direct costs (€) per patient-year by cost domains

Cost domain	Mean (SEM)	Percentage of direct costs	Median	Range
Visits to physicians	323.5 (9.3)	14.0	300	0–972
Outpatient surgery	3.9 (1.6)	0.2	0	0–352
Emergency room visits	0	0	0	0
Non-physician service use	2.4 (0.7)	0.1	0	0–135
Drugs	1019.3 (144.1)	44.1	382.7	0–28975
DMARDs	722.7 (138.6)	31.3	189	0–27949
Steroids	46.9 (3.7)	2.0	28	0–396
NSAIDs	83.7 (12.1)	3.6	15	0–2693
Osteoporosis drugs	73.3 (7.8)	3.2	19	0–890
Analgesics	21.7 (5.3)	0.9	0	0–1032
Gastroprotective drugs	71.1 (12.3)	3.1	0	0–1960
Diagnostic/therapeutic procedures and test	185.3 (5.7)	8.0	168.0	0–608
Imaging of bones and chest	27.2 (1.3)	1.2	24.2	0–132
Laboratory tests	140.1 (4.4)	6.1	126.9	0–462
Other procedures	18.0 (1.9)	0.8	7.7	0–328
Devices and aids	168.4 (34.9)	7.3	0	0–8712
Acute hospital facilities (without surgery)	276.1 (79.0)	11.9	0	0–19150
Acute hospital facilities (surgery)	215.1 (67.5)	9.3	0	0–15690
Non-acute hospital facilities	65.3 (27.9)	2.8	0	0–6544
Transportation	52.7 (10.9)	2.3	0	0–1921
Home healthcare services	0	0	0	0

DMARDs, disease modifying antirheumatic drugs; NSAIDs, non-steroidal anti-inflammatory drugs.

Table taken from Ruof *et al*.

Example: rheumatoid arthritis in Germany

Calculation of total costs:

- **total direct cotst for a RA patient:** € 3815 per patient-year (SEM € 267)

Ruof et al.

Application of the costing methodology: Cost-of-illness studies and Budget Impact Models

Multiple Sclerosis (MS) in Europe
Cost of illness estimates

Country	N	Age	Societal Perspective €	Payer Perspective €
stria	1019	50	40.300	20.000
gium	799	48	32.500	17.700
rmany	2973	45	40.000	19.000
itzerland	1101	53	41.900	19.100

Source: Kobelt G *et al*., JNNP 2007

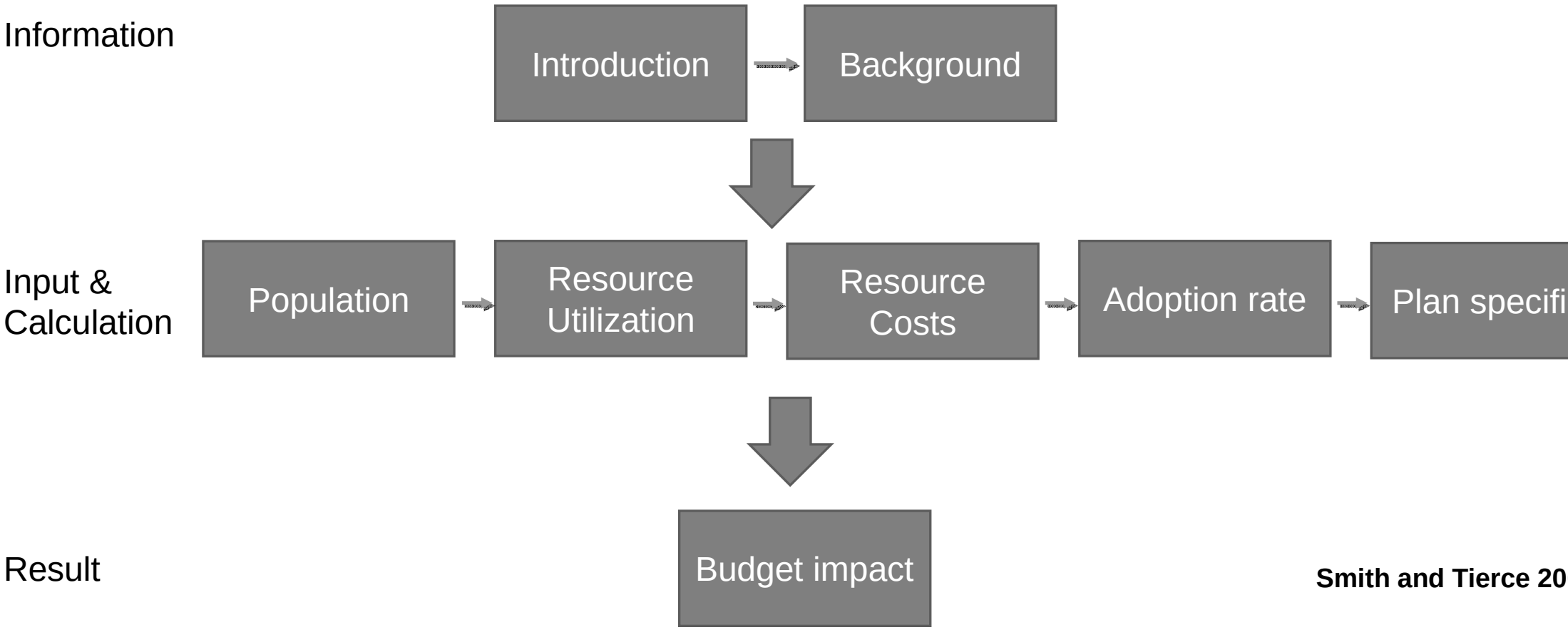
Value of a cost-of-illness analysis

Estimates the economic burden of a disease on a particular society or budget

- Public sector
- insurer
- employer
- Private household

- Provides first estimate of **savings potential**
- Helps setting **system priorities**
- First step in **cost-effectiveness analysis**

Budget impact models: Logic



Smith and Tierce 20

Utility assessment

“Unit“ of health effects

Indicators of health gain:

- Number of cured patients
- Number of symptom-free days
- Number of days in good quality of life; mean improvement in QoL score
- Number of life-years gained (LYG)
- Disability-adjusted life-years (DALYs) avoided
- Quality-adjusted life-years (QALYs) gained

Annemans 200

Instruments to assess quality of life: Categories and examples

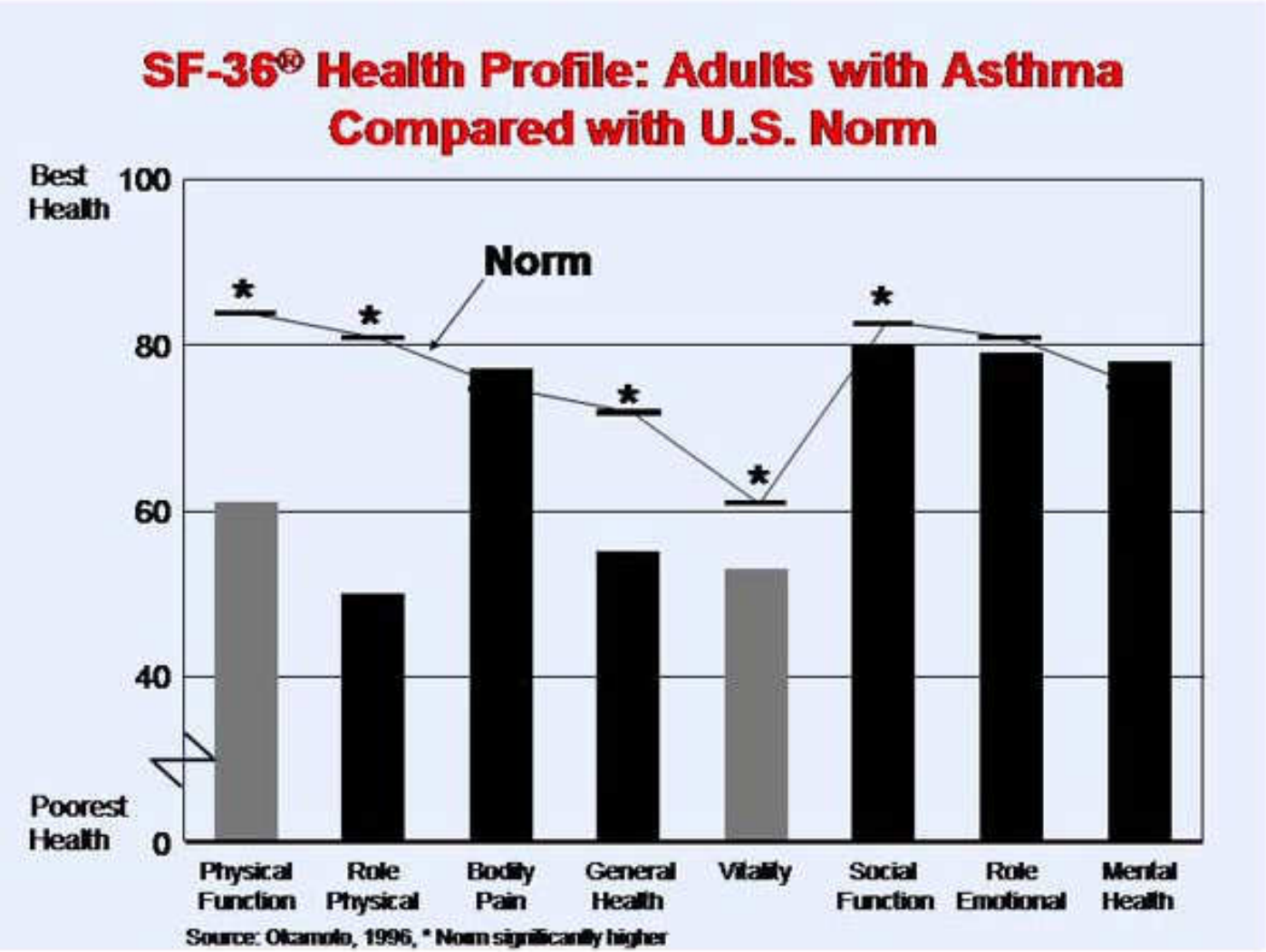
	Profile instruments	Utility instruments
Disease-specific	IBDQ	(some exist)
Generic	SF-36	EQ-5D SF-6D

Instruments to measure health-related quality of life in profile form: the SF-36

- multi-purpose, short-form health survey
- 36 questions
- 8-scale profile of functional health and well-being
- psychometrically validated physical and mental health summary measures

Ware n.y.

Instruments to measure health-related quality of life in profile form: the SF-36, *cont'd.*



Ware n.y.

Instruments to measure health-related quality of life in (utility) index form: the EQ-5D

bility
o problems in
king about
ome problems
walking about
onfined to bed

Self-care
1.No problems with self-care
2.Some problems with self-care
3.Unable to wash or dress self

Pain and discomfort
1.No pain or discomfort
2.Moderate pain or discomfort
3.Extreme pain or discomfort

Anxiety and depression
1.Not anxious or depressed
2.Moderately anxious or depressed
3.Extremely anxious or depressed

Usual activities
1.No problems with performing usual activities (e.g. work, study, housew
2.Some problems with performing usual activities
3.Unable to perform usua acitivities

Morris et al. 200

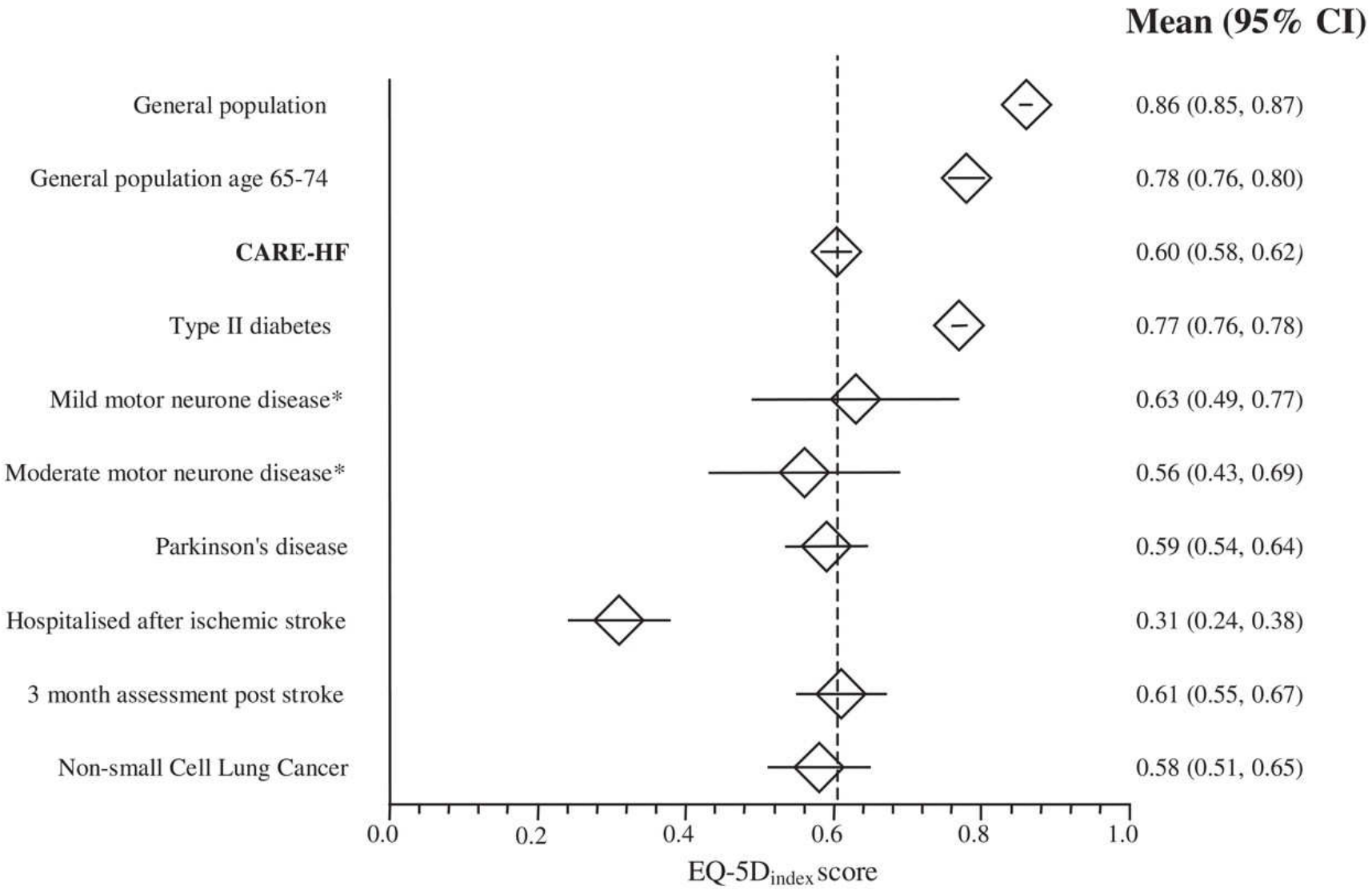
EQ-5D: health profiles and corresponding utility levels

Status	Index
11111	1.000
11222	0.6299
11333	0.1558
33333	-0.1584
Dead	0.000 (by definition)

Table adapted from Annemans 2008

Q: What does the negative number mean?

EQ-5D index for chronic diseases



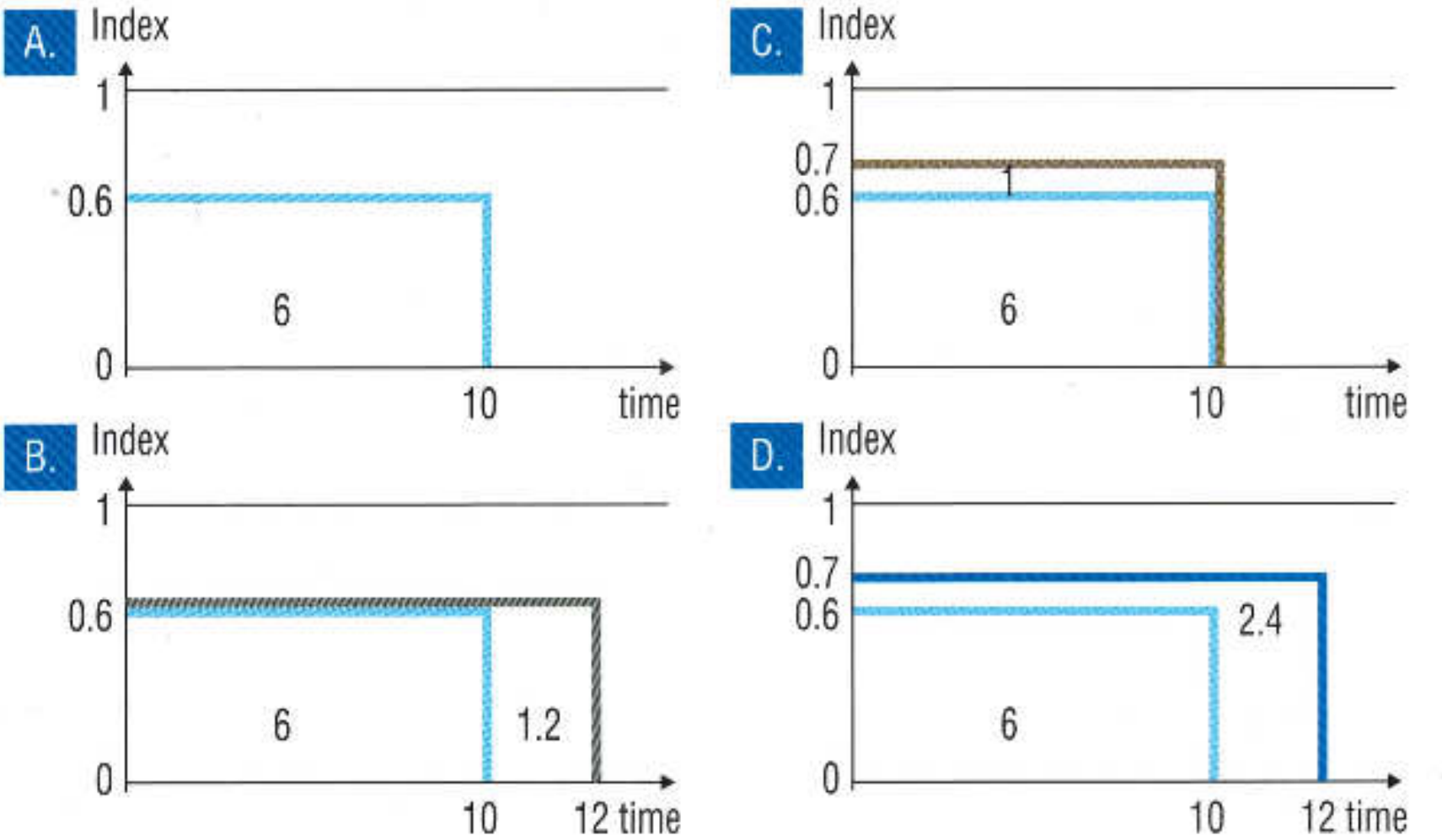
Calvert et al. 20

“Unit“ of health effects: QALYs

- benefit concept based on different theories/models (notably SG, TTO)
- “utility weight“ of each health state = sourced from patient or reference population
- QALYs = Σ expected durations in each particular health state
- Utility weight ranges from 1 to 0
  - if 1 = full health → 1 year = 1 QALY
  - if e.g. 0.6 (≈ post stroke) → 1 year = 0.6 QALYs
  - if 0 = death → 1 year, 2 years, … = 0 QALYs

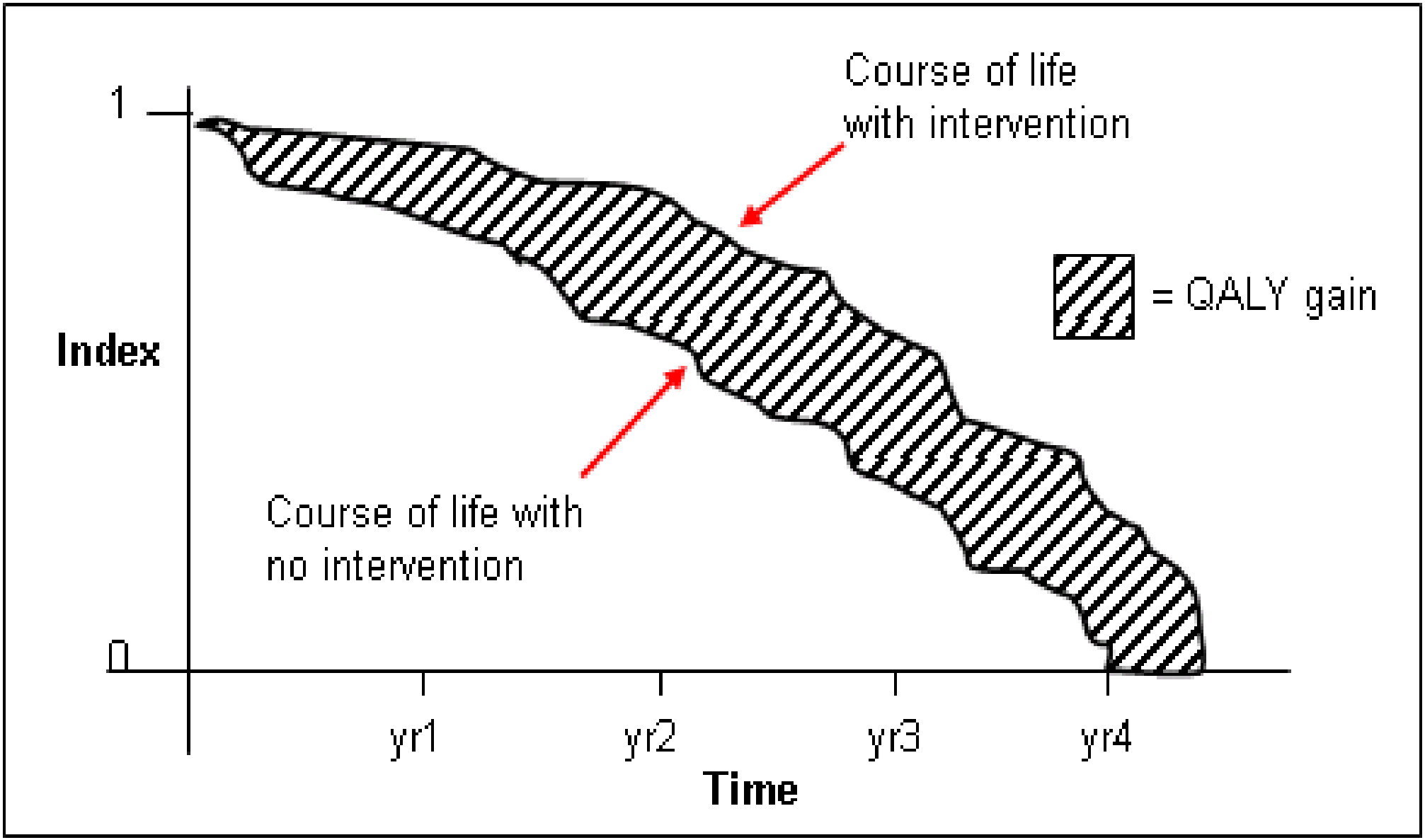
IQWiG 2

Calculating QALY gains



Annemans 200

QALY gain, less schematic



Utility assessment in practice

- Needed? (Cost-per-QALY decision-making in my country?)
- Generic or disease-specific utility-yielding instrument?
- Collection intervals
- Operational aspects:
  - self-complete or interviewer-administered?
  - paper-and-pencil or eCRF/e-diary?

Beyond utilities: Valuation of *patient preference* using discrete choice experiments (DCEs). Example: Insulins

↓4 attributes of importance↓

Table I Levels for noncost attributes and their source

		Source
Attribute 1: Timing of injection before meal		
Level 1	0–15 min (alternative scenario)‡	Humalog Mix25 Product Information
Level 2	30–45 min (fixed scenario)†	Humulin 30/70 Product Information
Level 3	60 min	Additional level for sensitivity analysis
Attribute 2: Two-hour postprandial blood glucose		
Level 1	9.4 mmol/L (alternative scenario)‡	Weighted average level for patients treated with Humalog Mix25, estimated by meta-analysis of available clinical evidence [3,4]*
Level 2	10.3 mmol/l (fixed scenario)‡	Weighted average level for patients treated with Humulin 30/70, estimated by meta-analysis of available clinical evidence [3,4]*
Level 3	11.0 mmol/L	Additional level for sensitivity analysis
Attribute 3: Effect of prandial dosing		
Level 1	Won't make a difference	Pharmacokinetic data
Level 2	Will make a difference	Pharmacokinetic data
Attribute 4: Nocturnal hypoglycemic frequency		
Level 1	One event in 12 months (alternative scenario)‡	Weighted average rate for patients treated with Humalog Mix25, derived from meta-analysis of available clinical evidence [4,5]†
Level 2	Two events in 12 months (fixed scenario)‡	Weighted average rate for patients treated with Humulin 30/70, derived from meta-analysis of available clinical evidence [4,5]†
Level 3	Four events in 12 months	Additional level for sensitivity analysis

Aristides *et al*. 2004. Patient Preference and Willingness-to-Pay for Humalog Mix25 relative to Humulin 30/70. *Value in Health* 7 (4): 442-454.

Measuring patient preference, insulin example, *cont'd.*

Appendix A: Example of scenario pair presented to participants

Attribute	Insulin A		Insulin B
When to inject The recommended time to inject is:	0–15 min before a meal	>	30–45 min before a meal
Blood sugar after meal Imagine before a meal your blood sugar was 9.2 mmol/L and 2 hours after a meal it is:	11 mmol/L	<	10.3 mmol/L
Injecting just before a meal If for some reason the insulin is used just before a meal:	This won't make any difference. After a meal your blood sugar *would be the same* as if you took the insulin at the correct time	>	This *will* make a difference. After a meal your blood sugar *would be higher* than if you took the insulin at the correct time
Night-time very low blood sugar or "hypo" Using the insulin, you can expect to have:	Two night-time "hypos" over 12 months		Two night-time "hypos" over 12 months
Cost to you of this insulin every month:	£18	<	£0
Which insulin do you prefer? Please tick one box only	Insulin A: ☐		Insulin B: ☐

Attributes

Levels insulin A

Levels insulin B

des *et al.* 2004. Patient Preference and Willingness-to-Pay for Humalog Mix25 relative to Humulin 30/70. *Value in Health* 7 (4): 442-454.

Results of insulin DCE

able 5 Incremental WTP utilities for Humalog Mix25 relative to Humulin 30/70

tribute	Variable	Estimated WTP (€)	SE‡	95% CI§	Percentage of aggregate WTP accounted for
me of dose*	0–15 vs. 30–45 mins	40.98	7.07	28.97, 57.48	37%
vo-hour postprandial blood glucose†	9.4 mmol/L vs. 10.3 mmol/L	16.19	6.34	4.87, 29.71	14%
octurnal hypoglycemic event rate†	One vs. two events per 12 months	54.01	8.26	41.80, 77.05	49%
tal*		111.18	16.56	86.71, 156.91	100%

972 successful bootstrap repetitions.
000 successful bootstrap repetitions.
andard error of the bootstrap estimate.
as-corrected confidence interval.
bbreviations: CI, confidence interval; SE, standard error; WTP, willingness-to-pay.

What matters most to insulin users?
How is that preference quantified?

A vignette example from a study in the field of vertigo

- **Please compare “Health State A“ and “Health State B“.**
- **Imagine that “Health State A“ is your current health state.**
- **You could also attain “Health State B“ after a three-month treatment, if you pay a certain monthly payment (for three months).**
- **Which is worse?**

Health State A	Health State B (after three months)
Some nausea or vomiting	No nausea or vomiting
Some balance disturbances	No balance disturbances
Never feel faint	Feel faint a lot
A lot of visual image disturbances	No visual image disturbances
Cost to me: 0 $ per month	Cost to me: 75 $ per month

Which option is worse (please tick one)?

☐ ☐

Summary (1 of 3)

We study cost and utility assessment methods because we want to measure & quantify these key attribute of new healthcare interventions

Costing is a key ingredient to cost-of-illness, budget impact, and cost-effectiveness analyses

Utility assessment is needed when cost-utility (“cost-per-QALY”) studies are required for healthcare decision-making

Summary (2 of 3)

Costs can be broken down by type (direct, indirect) and perspective (payer, household, societal)

The societal perspective is the scientifically warranted perspective, and ist use will yield to allocative efficiency in resource spending

Cost of an intervention = resource use x unit prices (micro-costing)

Payer reimbursement schedules can be based on lump sums (DRGs, capitation), and those should be considered if the analytical perspective is that of the payer

Different degrees of precision in costing exist (micro- vs. macro-costing), with a number of intermediate “shades of grey“;

the disease/intervention specifics, and intended use of the evidence, will determine what degree of precision is reasonable

Summary (3 of 3)

On the “benefits side“, utility – and also patient preference – often warrant further investigation

A number of tandardised instruments, with large underlying datasets, exist to assess utilities

Utilities are a key ingredient to QALY calculations, which themselves are needed where decision-making is based on cost-utility analysis (and, eventually, ± explicit thresholds)

A contemporary approach to assessing the benefit of a new technology is approximating patient preference by willingness-to-pay (WTP), e.g. through DCEs.

Thank you for your attention

Prof. Dr. York F. Zöllner
Hamburg University of Applied Sciences
Workshop Applied Aspects of Health Technology Assessment
February, 22nd 2014, Plovdiv

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