

Kartause Ittingen
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HTA including Economic Evaluation

Objectives of Collectively Financed Health Care,
Decision-Makers' Needs,
and Health Economic Analysis

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INTRODUCTION

Topics to be addressed

- ↪ **Background**
- ↪ **Project Objectives**
- ↪ **International Experience**
- ↪ **Economic Thinking
(Abbreviated!)**
- ↪ **The Logic of Cost Effectiveness**
- ↪ **Some Anomalies**
- ↪ **Decision Support
and the Objectives of Health Care**



WHAT IS HTA?

A Definition Proposed by EUNETHA

Health Technology Assessment

- **Health technology**
is the application of scientific knowledge
in health care and prevention.
- **Health technology assessment (HTA)**
is a multidisciplinary process that summarises information
about the medical, social, economic and ethical issues
related to the use of a health technology
in a systematic, transparent, unbiased, robust manner.
Its aim is to inform the formulation of safe, effective, health policies
that are patient focused and seek to achieve best value.
- Despite its **policy goals**, HTA must always be firmly rooted
in research and the scientific method.



WHAT IS HTA FOR?

A broad range of expectations (and fears) ...

What are Technology Assessments for?

“restricting use”

“containing costs”

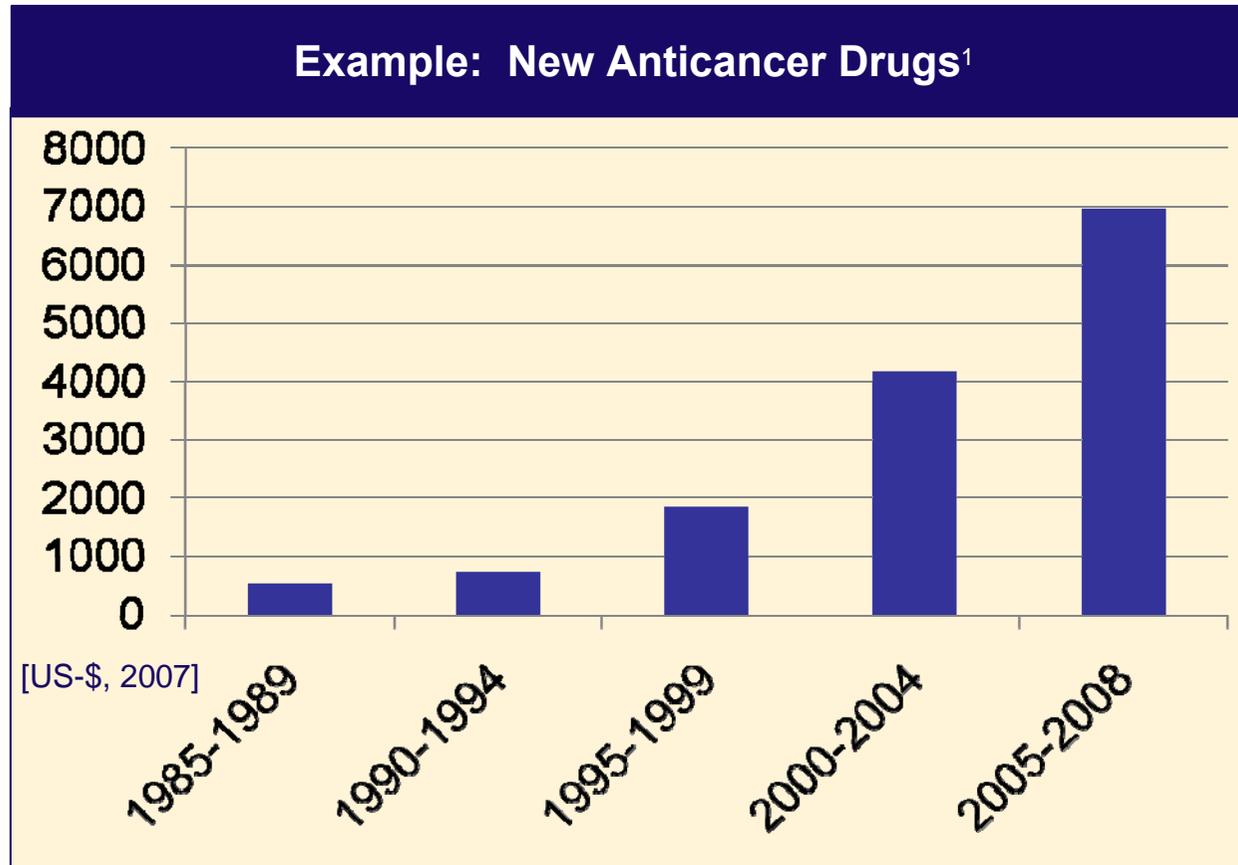
“issuing guidance to
potential users”

“prioritizing for
further evaluation”

“alerting users to future
possibilities”

“AFFORDABILITY” (?)

Median Monthly Costs of New Anticancer Drugs (by Year of Launch)



OBJECTIVES

Overall Project Objective

Swiss HTA Project: Objectives

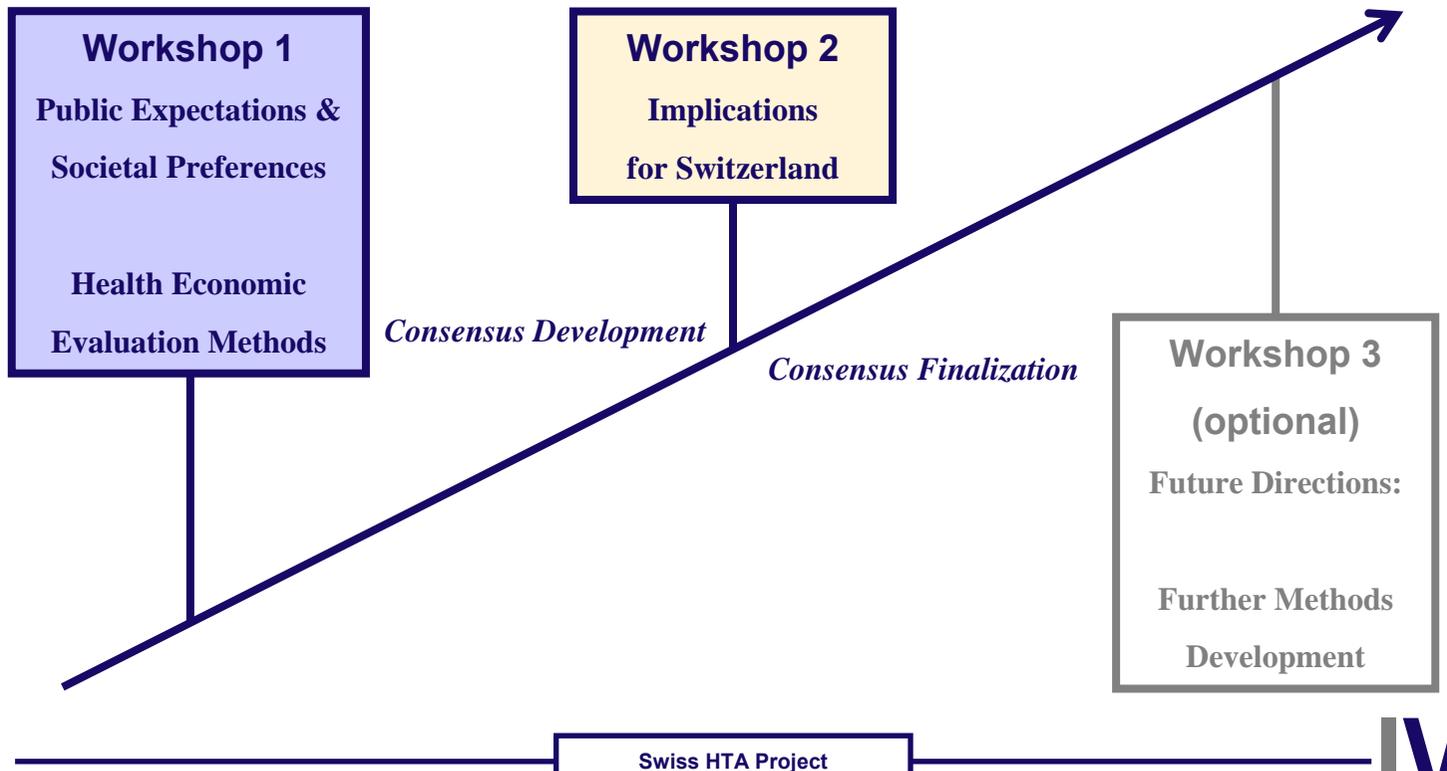
To develop
a **Swiss Consensus Statement**
on the appropriate use
of Health Technology Assessments
(HTAs)
including [health] economic evaluation.



PROCESS

Developing a Swiss Consensus Statement on the Use of HTAs including Economic Evaluation

Swiss HTA Project: Process



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STRUCTURE

Who is Behind the Project?

Swiss HTA Project	
Project Steering Committee	Scientific Steering Committee
<ul style="list-style-type: none">↪ Christian Affolter (santésuisse)↪ Thomas B. Cueni (Interpharma)↪ Pius Gyger (Helsana)↪ Ansgar Hebborn (Roche)↪ Stefan Kaufmann (santésuisse)↪ Heiner Sandmeier (Interpharma)	<ul style="list-style-type: none">↪ Robert E. Leu (U Bern / Switzerland)↪ Gérard de Pouvourville (ESSEC, Paris / France)↪ Michael Schlander (U Heidelberg & InnoVal^{HC}, Wiesbaden / Germany)



OBJECTIVES

Swiss HTA Project

Workshop 1: Objectives

To provide an update on international experience
and areas of debate concerning the use
of Health Technology Assessments (HTAs)
including economic evaluation,

in order to lay the foundations for a process
aimed at the development of a Swiss Consensus Statement

...

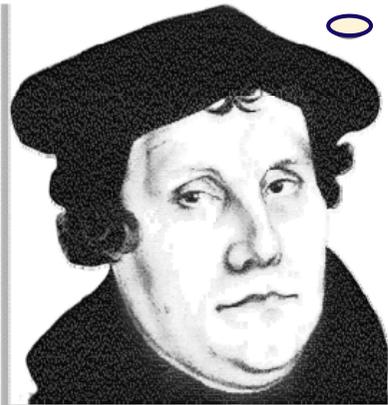


HAVE THE REGULATORS GOT IT RIGHT?

An old German saying ...

“Wer am Wege baut,
hat viele Meister“¹

“A house built by
the wayside
is either too high
or too low.”



¹Martin Luther (1530)

NICE PERSPECTIVES?

A High Profile not only in Europe

“What Could Be Nicer Than NICE?”¹



→ **Pearson and Rawlins (2005):**

“The conditions seem ripe for a NICE in the United States ...”

→ **Smith (2004):**

“*The triumph of NICE*”:

“NICE is conquering the world ... and may prove to be one of Britain’s greatest cultural exports along with Shakespeare, Newtonian physics, The Beatles, Harry Potter, and the Teletubbies ...”

→ **WHO (2003):**

“Published technology appraisals are already being used as international benchmarks ...”

HAS NICE GOT IT RIGHT?

NICE



- Three (distinct) “**Centres of Excellence**“:
- **Centre for Public Health Excellence**
 - Public health guidance
on the promotion of good health and the prevention of ill health
- **Centre for Health Technology Evaluation**
 - Technology appraisals (recommendations on the use of new and existing medicines and treatments within the NHS)
 - Interventional procedure guidance (evaluates the safety and efficacy of such procedures where they are used for diagnosis or treatment)
- **Centre for Clinical Practice**
 - Clinical guidelines
(recommendations, based on the **best available evidence**, on the appropriate treatment and care of people with specific diseases and conditions)



HAS NICE GOT IT RIGHT?

The NICE Approach

NICE Technology Appraisal Process

- **Three (to four) phases**
 - **Scoping**
 - **Assessment**
 - **Appraisal**
 - **Appeal** (if lodged by one or more consultees)

- **Frequently acclaimed features**
 - NICE objective of appraising the evidence in a way that is **“objective, unbiased, and methodologically sound”**¹
 - An appraisal process that can be described as being **“inclusive, consultative, transparent”**¹

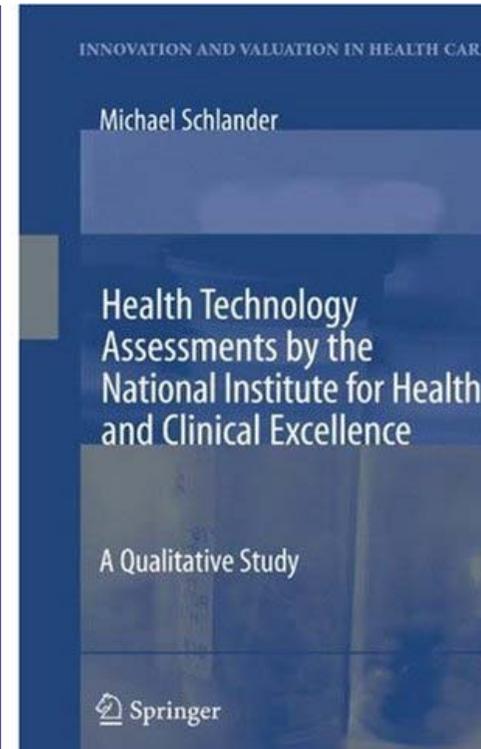
HAS NICE GOT IT RIGHT?

The NICE Approach

How Robust Are NICE Technology Appraisals?

Some Issues

- Timing of Technology Appraisals?
- Approach to Uncertainty?
- Integration of Clinical and Economic Expertise?
- Availability of Sufficient Resources?
- Efficiency-First Approach?
- (Almost) Exclusive Reliance on QALYs?
- Enforcement:
Internal Quality Assurance?
Implementation of Guidance?



HAS NICE GOT IT RIGHT?

Accountability for Reasonableness (A4R)¹

A4R Condition	Key Features	Key Limitations
Publicity	Overall process well-defined structure; detailed timelines, key documents continuously published; predictable opportunities for stakeholders to provide input	Selection of topics for appraisal (sometimes)
	Assessment Phase Assessment Protocol and Assessment Report published	"Commercial-in-confidence" withheld. Economic model withheld ("intellectual property")
	Appraisal Phase Appraisal Committee meeting agendas published meeting minutes published ACD, FAD published	Uniform Appraisal Committee criteria beyond neither codified
	Appeal Phase (optional) Appeal Panel holding public hearings; detailed meeting minutes	
Relevance	Fairness Condition High level of procedural fairness within NICE framework; NICE seeking input from Citizens Council on social value judgments	No codified criteria for fairness "efficiency-first" approach
	Integration of clinical and economic perspectives	Poor alignment of scopes (for technology appraisals and clinical guideline development). Sometimes (?) poor integration of both perspectives
Revisions and appeal	NICE definition of "appeal" differs from that of A4R; appeals may be lodged by consultees only	Conditions for appeal more restrictive than A4R recommendations; this appears unlikely to be fully compensated for by opportunities for stakeholder participation
Enforcement	Consistency of technical quality of assessment reports	Absence of effective quality assurance system for technology assessments
	Implementation	Mixed record of guidance implementation in the NHS

"NICE's use of cost-effectiveness as an exemplar of a deliberative process ..."

"The use of cost-effectiveness by NICE: No(t yet an) exemplar of a deliberative process ..."

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A.J. Culyer (2006)
Health Economics, Policy and Law 1: 299-318

M. Schlander (2008)
Journal of Medical Ethics 34: 534-539

¹M. Schlander (2007, 2008)



HAS NICE GOT IT RIGHT?

“What More Could Anyone Ask For?”

NICE is “the closest anyone has yet come to fulfilling the economist’s dream of how priority-setting in health care should be conducted.”



Alan Williams (1927 – 2005)

... “[NICE] is transparent, evidence-based, seeks to balance efficiency with equity, and uses a cost-per-QALY benchmark as the focus for its decision-making. *What more could anyone ask for?*”

HAS NICE GOT IT RIGHT?

“What More Could Anyone Ask For?”

NICE is “the closest anyone has yet come to fulfilling the economist’s dream of how priority-setting in health care should be conducted.”

However:
“Experience has taught me that it is not uncommon for an-economist’s-dream-come-true to be seen as a nightmare by everyone else.”



Alan Williams (1927 – 2005)

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THE ISSUE

Introductory Remarks

**“Economic Evaluation
in Health Care:
Is It Really Useful
or
Are We Just Kidding Ourselves?”¹**

“Let’s face it: most health economists have an **interest**
in the continued growth of the subdiscipline.”

Obstacles may be “(i) the short-term nature
of the decision making process; (ii) problems in
interpreting studies; (iii) lack of timeliness in study
results; and (iv) importance of **other factors** in
decision making.”¹

¹Michael Drummond (2004)

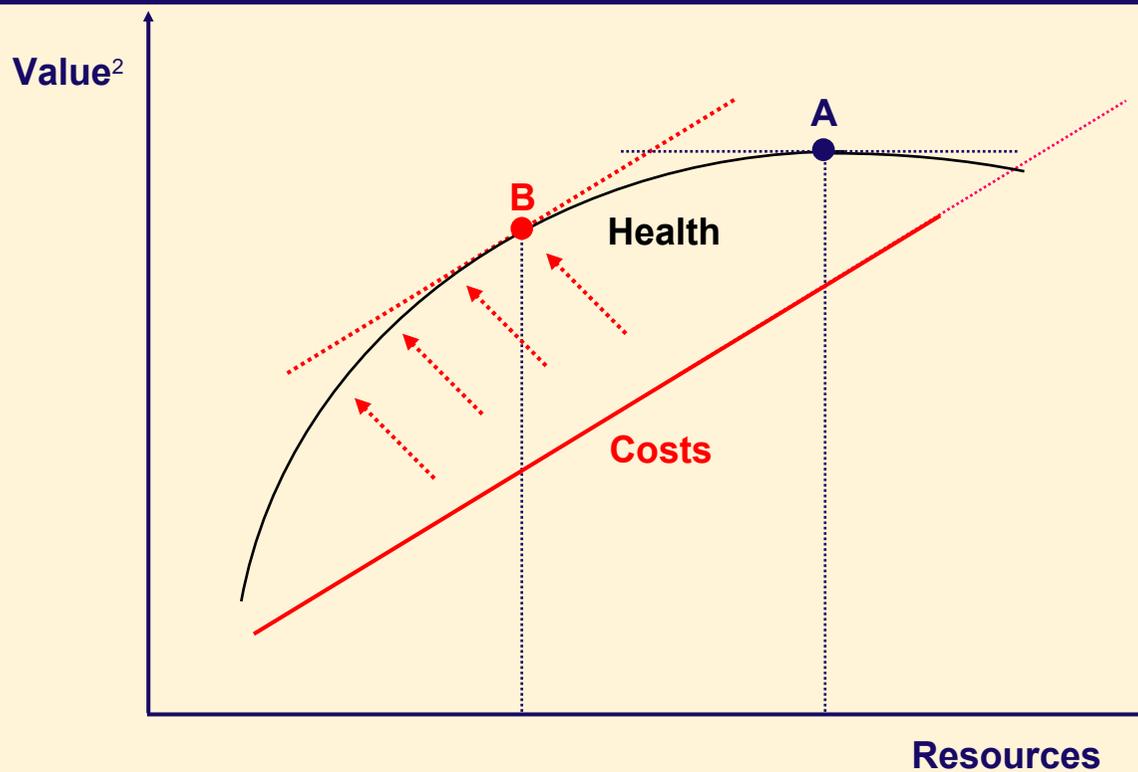
Australian Economic Review 37 (1) : 3-11

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ECONOMIC THINKING

Some Foundations of Economics: Marginal Analysis and Opportunity Costs

Evidence Based Medicine (A) & Economic Evaluation¹ (B)



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¹cf. Victor R. Fuchs: "Health Care and the United States Economic System", *The Milbank Memorial Fund Quarterly*, April 1972: 211-237.

²Note different definitions of "value".

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Value and Valuation of Health Technologies

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VALUES TALK

A Canadian Policy Analysis¹



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Illustration by Athanasius Kircher

A Tower of Babel ...

- Referral to many different and often incommensurate things...
- **A key paradox:**
The discourse about values is both very important and very ambiguous...
- Stakeholders may be tempted to react to this problem with either
reductionism
(focusing on one particular definition of values to the neglect of other relevant types)
or
nihilism...
(either rejecting all values analyses as equally unreliable, or accepting all as equally credible)

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¹M. Giacomini et al. (2004)



“VALUE”

Economic Welfare Theory and “Utility”

What We Teach Our Students

“Political economy has to take as the *measure of utility* of an object the maximum sacrifice which each consumer would be willing to make in order to acquire the object

...

the only real utility is that which people are *willing to pay for*.”¹

– Contemporary Textbooks of Microeconomics:

– “The **value** [of a product] to a given consumer is defined as the maximum amount that the consumer would be **willing to pay** for that [product].”²

¹Jules Dupuit (1844)

²Steven E. Landsburg: *Price Theory and Applications*, 5th ed., Mason, OH: South-Western 2002, p. 238.

EXTRA-WELFARISM

In particular, two assumptions of economic welfare theory have attracted criticism from a group of health economists (often referred to as “extrawelfarists”)

An Extra-Welfarist Critique⁵

1. “The monetary measurement [of benefits in cost-benefit analysis] inherently favors the wealthy over the poor.”¹
 - “Extra-welfarists *and many decision-makers in the real world of health care* are willing to accept an approach that considers outcomes equitably (as CEA using QALYs does), rather than accept an approach in which choices are heavily influenced by ability to pay.”²
2. “Extra-welfarists identify ‘health’ as the principle output of health services.”³
 - Then, in effect (*at least in theory*⁴), health is treated as an independent argument in the welfare function. Now, health can no more be substituted by income or consumption.

¹M.R. Gold et al. (1996), p.26; ²M.C. Weinstein and W. Manning (1997), p. 127; ³A.J. Culyer (1989), p. 51; ⁴C. Donaldson et al. (2002);

⁵Thomas Rice (1998, 2002) has provided a systematic critique of welfare theory as a foundation of health economics.



COMPARATIVE ECONOMIC EVALUATION

Foundations: Two prevailing philosophies¹

Welfare Economics

- Seeking (potential) Pareto improvements
- Focused on efficient allocation of scarce resources²
 - Cost-benefit analysis incorporating the efficiency rationale behind markets
 - Social objective assumed to be to maximize (aggregate) consumer satisfaction (“utility”)
 - Grounded in economic welfare theory
 - Strength of preferences expressed by Willingness to Pay (WTP)²

Decision Support

- Decision analysis as a tool to support social objectives
- In practice, [usually] focused on [aggregated] health maximization
 - Can, in principle, accommodate a variety of objectives and perspectives
 - Background in operations research
 - Striving to adopt the perspective of a ‘rational’ decision-maker
 - Distributive concerns representing a research frontier, not actual practice

¹cf. R.F. Sugden, A. Williams: *The Principles of Practical Cost-Benefit Analysis*. Oxford University Press (1978); cf. also G. Torrance (2006)

²Note that, at least in principle, CBA can accommodate the impact of prior distribution (wealth, income; “ability to pay”)



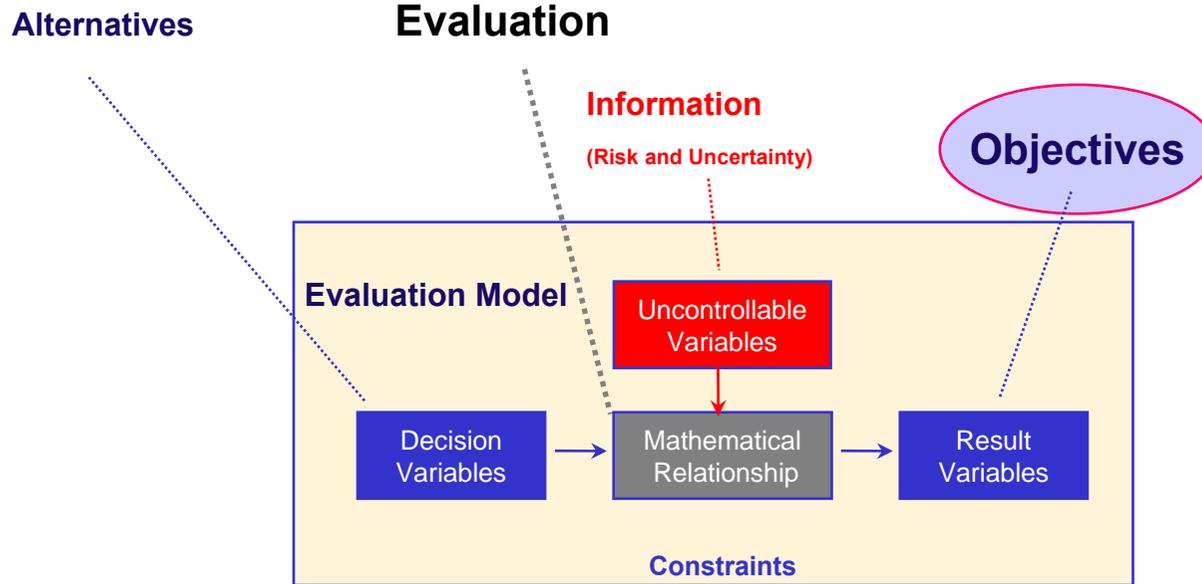
EXTRA-WELFARISM

The Logic of Cost-Effectiveness

What Are the Objectives of Collectively Financed Health Care?

What We Also Teach Our Students

Decision Analytic Principles¹:



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¹From E. Turban and J.R. Meredith (4th ed., 1988)



EXTRA-WELFARISM

The logic of cost-effectiveness:
a promise and a premise

“A QALY
is a QALY
is a QALY
—
regardless of
who gains and who
loses it.”¹

“The principal
objective of the
National Health Service
ought to be to
maximize the
aggregate
improvement in the
health status of the
whole community.”²

“The underlying premise
of CEA in health problems is
that for any given level of
resources available, **society** (or
the decision-making jurisdiction
involved) **wishes to maximize
the total aggregate health
benefit** conferred.”³

²Anthony J. Culyer (1997)

¹D. Feeney and G.W. Torrance (1989)
but there are reasons to suspect that the utility of health states
may be influenced by wealth – cf. C. Donaldson et al. (2002)

³M.C. Weinstein and W.B. Stason (1977)

EXTRA-WELFARISM

The logic of cost-effectiveness

Utilitarian Thought

– **John Stuart Mill (1806-1873):**

– “What is best brings the greatest good for the greatest number ...”

– **Jeremy Bentham (1748-1832):**

– “The greatest happiness of all those whose interest is in question is the right and proper, and the only right and proper and universally desirable, end of human action.”

– **Medical Utilitarianism:**

– A variant of act utilitarian thought, **exclusively focusing on health outcomes** (usually QALYs)

EXTRA-WELFARISM

The ethics of resource allocation decisions

Problems with (Act) Utilitarianism

Case 1:

	U ₁	U ₂	U ₃	U _{tot}
A ₁	+6	+8	+6	+20
A ₂	+7	+9	+2	+18
A ₃	+2	+3	+12	+17

Assumptions:

- Utility can be measured and quantified.
- Measured values can be compared meaningfully.

Case 2:

	U ₁	U ₂	U ₃	U _{tot}
A ₁	+28	+28	-30	+26
A ₂	+2	+9	+14	+25
A ₃	+8	+8	+8	+24

Problem:

- Distribution is ignored.
- Act utilitarianism even will defend negative utilities for some.



THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)

Quality and Quantity of Life as Outcomes

- **Basic idea underlying the QALY**
 - Combination of (health-related) quality of life and length of life into **one comprehensive and universal measure**
 - Intended to facilitate comparisons between different kinds of treatments and diagnoses
 - Should be measured on a **cardinal scale** to enable computations¹
- **The concept of the QALY**
 - If the health state “blind” gives a quality weight (utility index) of 0.4, then one year as blind gives 0.4 QALYs ...
 - ... or 1 year in full health gives the same number of QALYs (1) as 2.5 years as blind

¹According to expected utility theory (EUT), this can be achieved using standard gamble (SG) experiments.

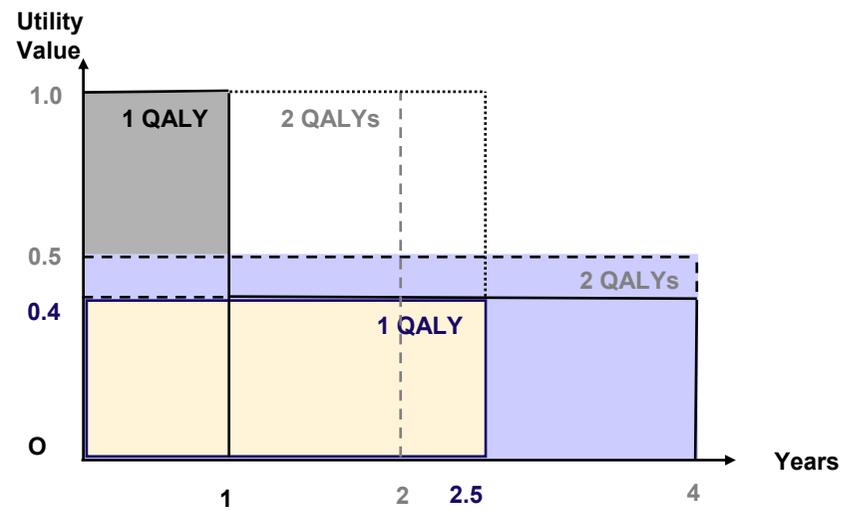


THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)

Quality and Quantity of Life as Outcome

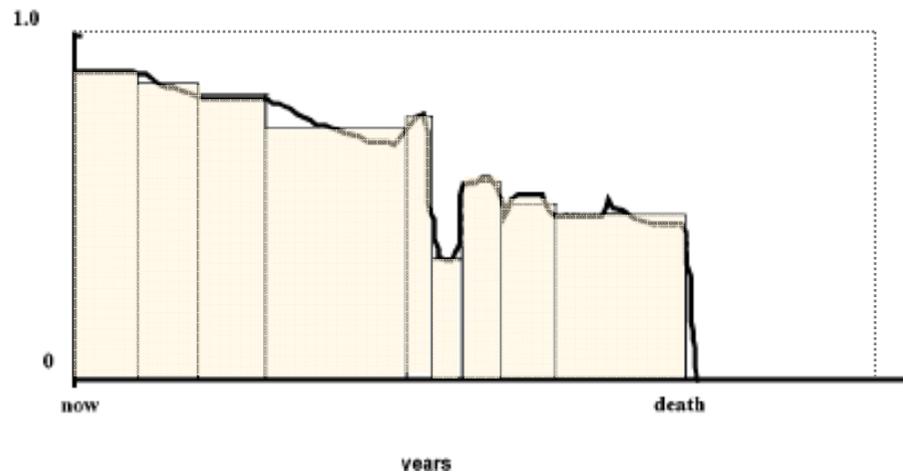
QALY: Quantity and Quality of Life = AUC



THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)

Calculating QALYs



The area under the curve is the QALYs accumulated by the person over the respective portion of her life time.

The area is approximated by summing the areas of the rectangles.

The area of each rectangle is the product of an HRQoL weight and the time for which the individual is assumed to experience this HRQoL level.

Some assumptions underlying the QALY concept:

- Utility independence (quality / quantity of life; from a welfare economic perspective also for health / non-health arguments of the utility function)
- Constant proportional trade-off
- Additive separability

¹From M. R. Gold et al. (2002)

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THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)
as a measure of (health-related) outcomes¹

QALYs: Utility-Adjusted Life Years

Expected Utility (Theory)

Utility = Maximum WTP (Jules Dupuit)
Objective: Maximization of Expected Utility
Fear? Attention? Maximization or else?

Experienced Utility

“Hedonimeter“ (Francis Edgeworth)
“instant utility“ (Daniel Kahneman)
Adaptation? Maximization or else?

Note: Some approaches do not consider each person as an end, but are willing to promote an overall ‘social good’ in ways that may in effect use some people as means to the enrichment of others¹

Limitations of “Utility”

Key entitlements (capabilities)? (Amartya Sen)
Distributional ‘neutrality’; maximin? (John Rawls)
Trade-Offs against primary goods
(e.g., political and religious liberty)?
Preference adjustment?

¹Martha C. Nussbaum, *Frontiers of Justice* (2006)



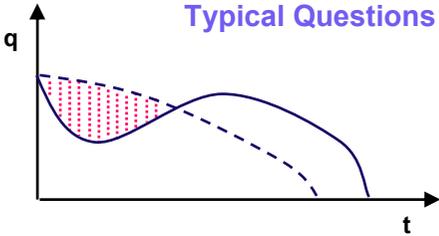
THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)
as a measure of (health-related) outcomes¹

Three Distinct Ways How to Use QALYs

Same intervention
for
Same indication
(same patient group)

“Does the Utility Gain
Outweigh the Disutility
of Treatment?”
e.g., cancer chemotherapy



Different interventions
for
Same indication
(same patient groups)

“How Can We Integrate a
Variety of Clinical Outcomes
in one Summary Measure?”
Alternative: disaggregated (cost-consequence) analysis

Different interventions
for
Different indications
(different patient groups)

“How Can We Determine the Most Efficient Allocation
of Scarce Health Care Resources
across a Wide Range of Competing Interventions?”
“Efficiency” usually defined in terms of QALY maximization

¹This is *not* a comprehensive list. For example, QALYs may also be used in descriptive (non-comparative) economic analyses.

QALYs as a utility measure of health-related consequences

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THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs)

Measurement methods to generate quality weights

HRQoL: Generic Index Instruments¹

Are they all the same?

- Coverage of descriptive system
- Sensitivity of dimensions
- Model used to combine the dimensions / items
- Valuation method (scaling instrument (VAS, SG, TTO, ...))

THE LOGIC OF COST-EFFECTIVENESS

Quality-Adjusted Life Years (QALYs) Measurement methods to generate quality weights

HRQoL: Convergent Validity of Generic Index Instruments¹

	EQ5D	HUI 3	QWB SA	SF6D
EQ5D	1			
HUI 3	0.49	1		
QWB SA	0.41	0.45	1	
SF6D	0.50	0.52	0.43	1
MEAN	0.47	0.49	0.43	0.48

Proportion of variance explained by another instrument (R^2)

R^2 = correlation coefficient squared

¹Source: Fryback et al (2010) p. 8, Tab. 2

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THE LOGIC OF COST-EFFECTIVENESS

Putting the 'Q' Into the Quality-Adjusted Life Year (QALY)

Some Utilities for Health States¹

Health State	Utility
↪ Full health (reference state)	1.00
↪ Myocardial infarction, acute (TTO)	0.87
↪ HIV infection, asymptomatic (TTO)	0.87
↪ Hospital dialysis (TTO)	0.56
↪ Liver cirrhosis, decompensated (SG and TTO)	0.54
↪ Being blind or deaf or dumb (TTO)	0.39
↪ Dead (reference state)	0.00
↪ Confined to bed with severe pain	< 0

¹Data from: G.W. Torrance (1987); T.O. Tengs (2000)

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EXTRA-WELFARISM

The logic of cost-effectiveness

QALY League Tables¹

Ranking Interventions by Their Cost-Effectiveness

Example	Cost/QALY
↳ GP advice to stop smoking	220 £
↳ Antihypertensive treatment to prevent stroke (age 45-64 years)	940 £
↳ Hip replacement	1,180 £
↳ Kidney transplant	4,710 £
↳ Hospital hemodialysis	21,970 £
↳ Neurosurgical intervention for malignant intracranial tumors	107,780 £

¹Data from: A. Maynard (1991); data for United Kingdom (in 1990 £)

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THE LOGIC OF COST-EFFECTIVENESS

Economic evaluation of new medical technologies

Some Cost-Effectiveness Benchmarks

- No scientific basis
- Some international “de facto” benchmarks:
 - **New Zealand** (PHARMAC):
NZ-\$ 20,000 / QALY¹
 - **Australia** (PBAC):
AUS-\$ 42,000 / LYG to AUS-\$ 76,000 / LYG²
 - **England and Wales** (NICE):
£ 20,000 – £ 30,000 / QALY
 - **United States** (MCOs):
US-\$ 50,000 – US-\$ 100,000 / QALY³
 - **Canada** (proposed “grades of recommendation”):
CAN-\$ 20,000 – CAN-\$ 100,000 / QALY⁴

¹C. Pritchard (2002); QALY: “quality-adjusted life year”; ²George et al. (2001); LYG: “life year gained”

³D.M. Cutler, M. McClellan (2001); ⁴A. Laupacis et al. (1992)



THE LOGIC OF COST-EFFECTIVENESS

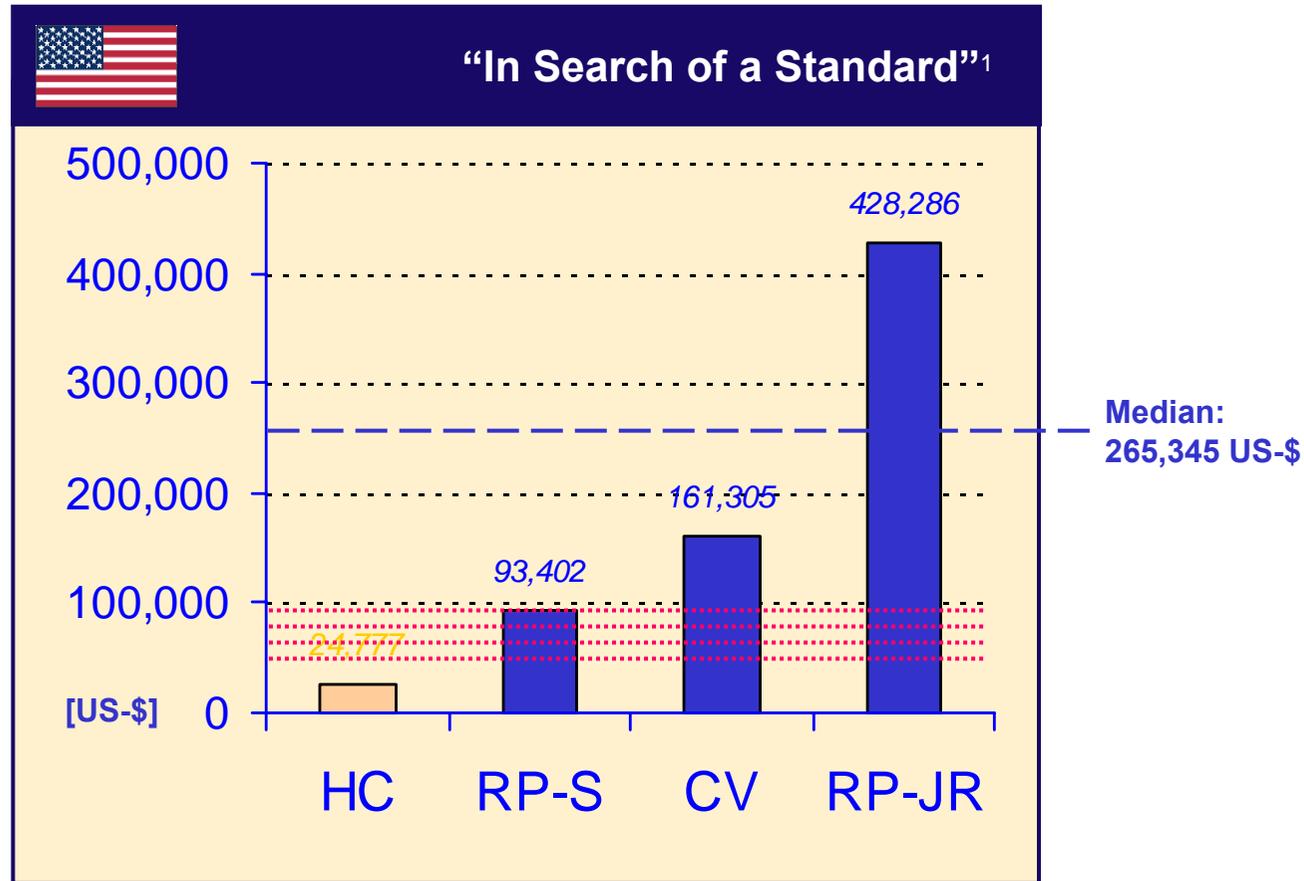


Not so new:

The evaluation
of
human
life time
in
economic /
monetary
terms

THE LOGIC OF COST-EFFECTIVENESS

“Gaining a QALY may be worth more than analysts generally assume.”¹ (?)



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¹R.A. Hirth et al. (2000)

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Value and Valuation of Health Technologies

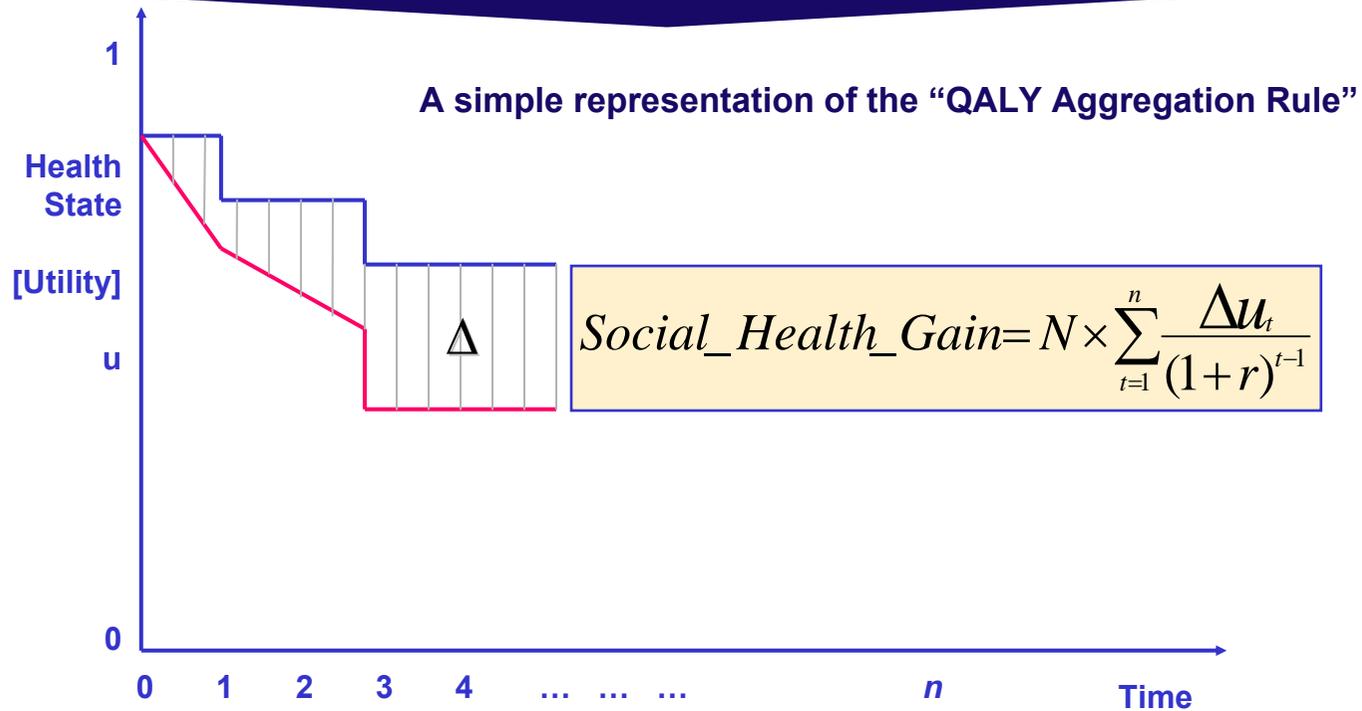
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THE LOGIC OF COST-EFFECTIVENESS

The concept of a cost per QALY “threshold” rests on the linear QALY aggregation assumption

The Conventional Unit of Health Outcomes: QALYs



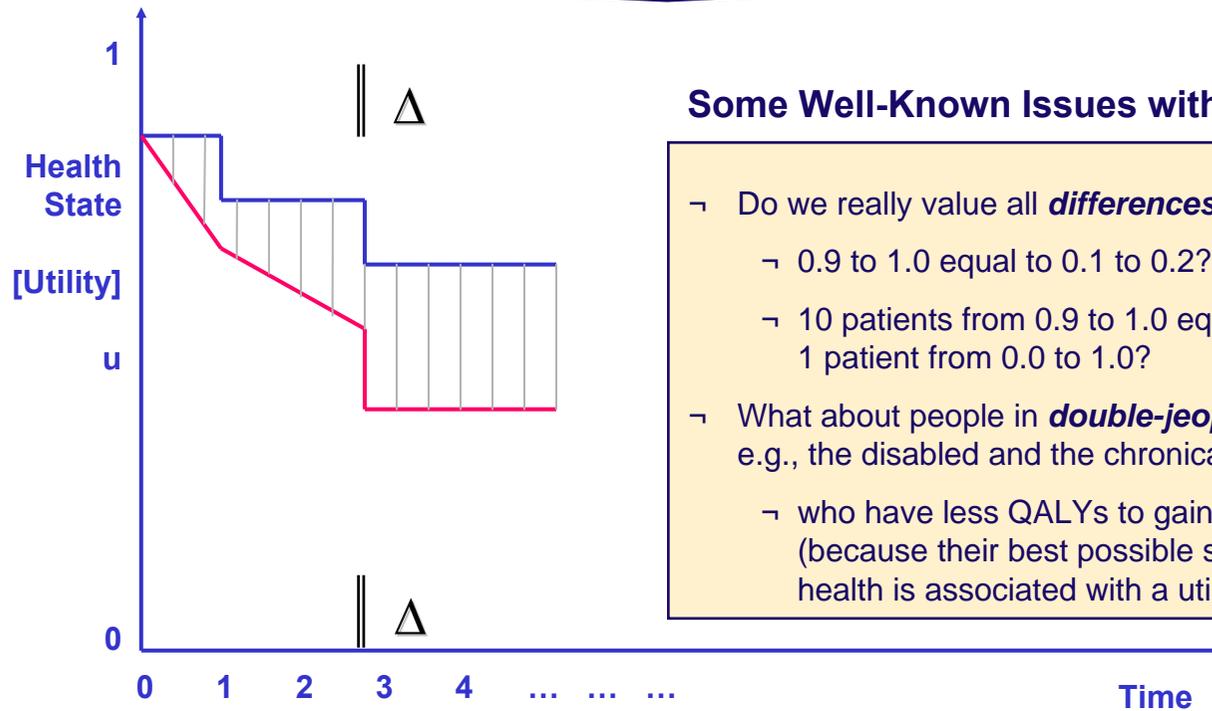
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EXTRA-WELFARISM

The logic of cost-effectiveness

Aggregation of Quality-Adjusted Life Years (QALYs)



Some Well-Known Issues with QALYs

- Do we really value all **differences** equally?
 - 0.9 to 1.0 equal to 0.1 to 0.2?
 - 10 patients from 0.9 to 1.0 equal to 1 patient from 0.0 to 1.0?
- What about people in **double-jeopardy**, e.g., the disabled and the chronically ill,
 - who have less QALYs to gain? (because their best possible state of health is associated with a utility $u < 1$)

The QALY aggregation rule is “descriptively flawed”.¹

¹cf. P. Dolan et al. (2005), M. Schlander (2005)

THE LOGIC OF COST-EFFECTIVENESS

Social WTP: Valuation of Quality-Adjusted Life Years (QALYs)

Does “Context” Matter?

- ↪ **Empirical evidence** supports a role of the following¹:
 - ↪ **Severity** of initial health state
 - ↪ Level of impairment
in addition to improvement (difference)?
 - ↪ **Rule of rescue**
 - ↪ Identifiable individuals
(but is being “visible” morally relevant?)
 - ↪ **Potential** for health improvement
 - ↪ e.g., the permanently disabled and chronically ill?
(who have less QALYs to gain)
 - ↪ Patients with **high-cost illnesses**

¹cf. recent reviews by P. Dolan et al. (2005), J. Richardson and J. McKie (2005), M. Schlander (2005); further considerations include (but are not limited to) age, responsibility for dependants, and number of patients or program size.



THE LOGIC OF COST-EFFECTIVENESS

Extrawelfarism

Guidance based on the EQ-5D

- **Some problems with walking and with usual activities, no other problems** (EQ-5D state 21211)

- Utility gain from prevention ($1 - 0.810 =$) 0.190

- **Fatal heart attack**

- Utility gain from prevention ($1 - 0 =$) 1.000

- **Issue**

Is preventing five cases of “some problems with walking and with usual activities, no other problems” **as valuable as** preventing one case of fatal heart attack?



THE LOGIC OF COST-EFFECTIVENESS

“QALY League Tables” Revisited

Deconstructing Counterintuitive Cost-per-QALY Rankings

- (In)Famous example from the Oregon Health Plan (OHP):
 - Capping a tooth for **150** (not one!) patients was ranked higher than an appendectomy for **one** person.
 - But did this ranking reflect our “powerful proclivity to rescue endangered life”?¹
- **Some issues not adequately addressed by CEA/CUA:**
 - What priority should be given to the worst off? (those with the most serious and/or immediate conditions)
 - When should small benefits to a large number of persons outweigh large benefits to a small number of persons?
 - How can the conflict between fair individual *chances* and best aggregated outcomes be resolved?²

¹cf. D.M. Eddy (1991) and D.C. Hadorn (1991); ²For a more complete account of these and related ethical issues, cf. D. Brock (2004, 2006).



THE LOGIC OF COST-EFFECTIVENESS

“QALY League Tables” Revisited

Ranking of Interventions by Cost per QALY ICERs

Interventions:

- **Sildenafil**
for erectile dysfunction
- **Methylphenidate**
for ADHD in children
- **Riluzole**
for motor neuron disease
- **Beta interferon**
for multiple sclerosis
- **Laronidase**
for mucopolysaccharidosis

ICERs:

- **< ~ 3,600 £ / QALY¹**
- **< ~ 7,000 £ / QALY²**
- **~ 38,500 £ / QALY³**
(34,000–43,500 £/QALY³)
- **~ 120,000 £ / QALY⁴**
(69,000–580,000 £/QALY⁴)
- **> 330,000 £ / QALY⁵**

¹E.A. Stolk et al. (2000); ²S. King et al. (2004); ³G. Ginsberg and S. Lowe (2002), NICE (2001), ⁴A. Stewart et al.(2000); ⁵NICE (2006)

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THE LOGIC OF COST-EFFECTIVENESS

“QALY League Tables” Revisited

A Greater Role for Budgetary Impact Analysis?

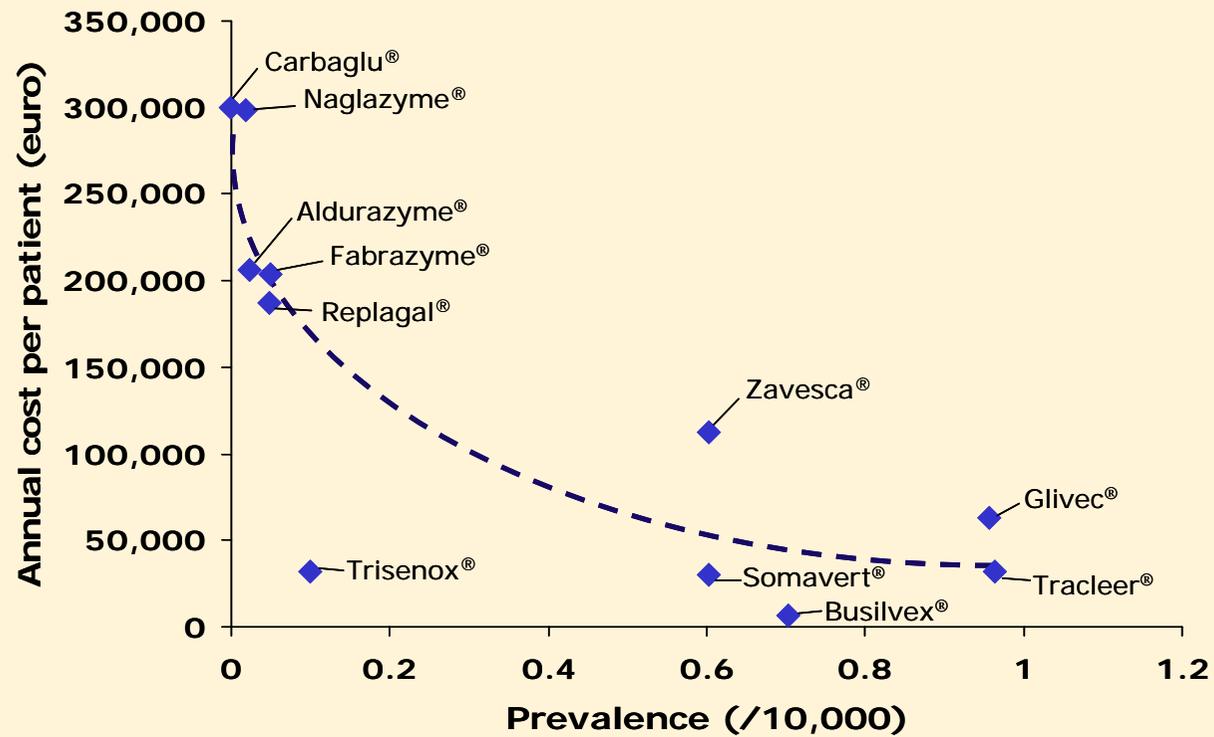
Some ICERs for “Orphan” Treatments			
Condition	Prevalence	Product	ICER (“preliminary estimated £ per QALY”)
M. Gaucher (Type I and III)	270	Imiglucerase (Ceredase ^R)	391,200
MPS Type 1	130	Laronidase Aldurazyme ^R)	334,900
M. Fabry	200	Agalsidase beta Fabrazyme ^R)	203,000
Hemophilia B	350	Nonacog alpha (BeneFIX ^R)	172,500
M. Gaucher (Type I)	270	Miglustat	116,800



THE LOGIC OF COST-EFFECTIVENESS

A Greater Role for Budgetary Impact Analysis?

“Orphan” Treatments: No Distinct Subcategory



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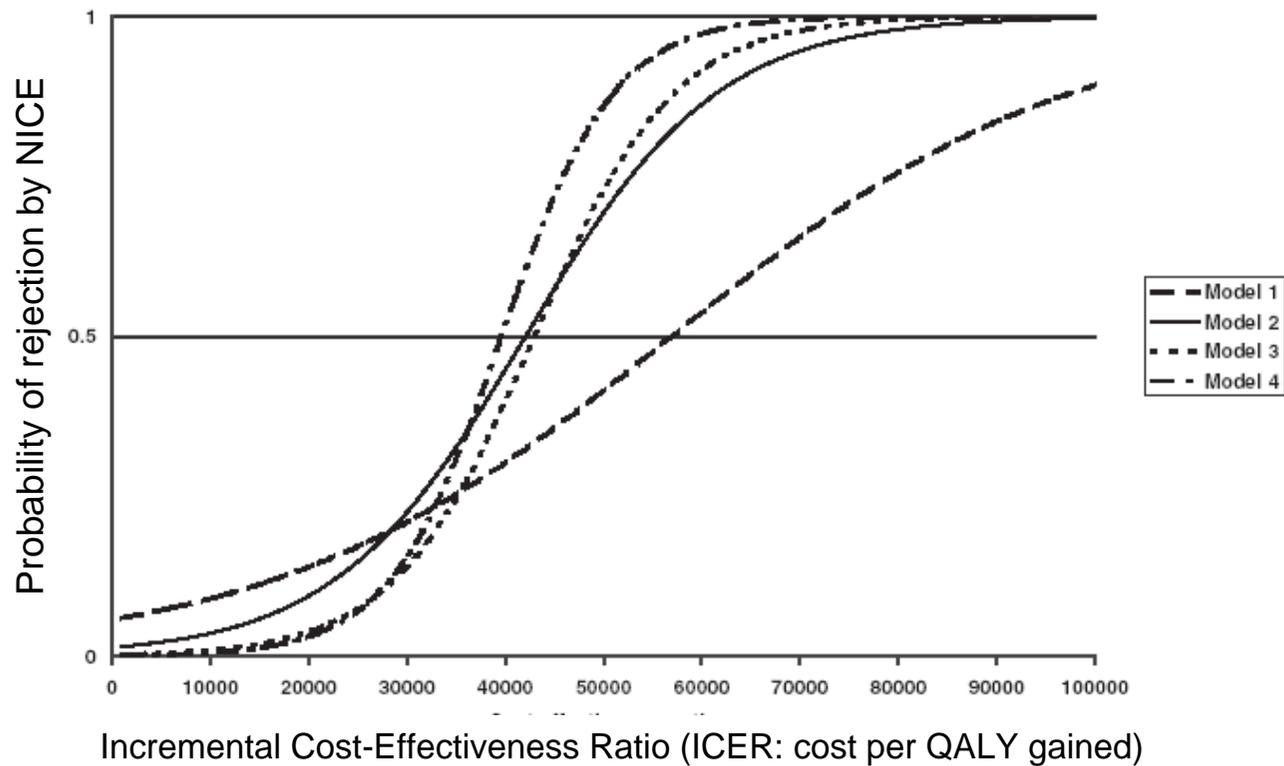
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THE LOGIC OF COST-EFFECTIVENESS

A NICE example of cost-effectiveness benchmarks in practice

'Probabilistic' NICE Cost-Effectiveness 'Benchmarks'¹



¹N. Devlin and D. Parkin (2004)



THE LOGIC OF COST-EFFECTIVENESS

UK Cancer Experts Deplore NICE Decision on Kidney Cancer Drugs



August 26, 2008 – Cancer experts in the United Kingdom have banded together to voice their dismay over the recent draft guidance from the National Institute for Health and Clinical Excellence (NICE) **stating that 4 new cancer drugs should not be used in the treatment of advanced and/or metastatic renal cell cancer**. This draft recommendation, issued on August 7, is now open for consultation; a further review is planned for September 10.

The 4 products involved are bevacizumab (*Avastin*, Roche/Genentech), sorafenib (*Nexavar*, Bayer), sunitinib (*Sutent*, Pfizer), and temsirolimus (*Torisel*, Wyeth). **Although the drugs have been shown to extend patients' lives by some months, NICE ruled that they were not cost effective and hence should not be available on the National Health Service (NHS).**

...

"It just can't be that everyone else around the world is wrong about access to innovative cancer care and the NHS right in rationing it so severely," they comment. The signatories include some of the most prominent cancer specialists in the United Kingdom, and the group of 26 is headed by Karol Sikora, MBBCh, PhD, medical director of CancerPartnersUK, professor of cancer medicine at Hammersmith Hospital, in London, and former chief of the World Health Organization Cancer Programme.



THE LOGIC OF COST-EFFECTIVENESS

UK government backs NICE (2008)

Pharmaceutical
BUSINESS
Review

The UK government's response to a parliamentary committee's report on NICE, the healthcare technology assessment agency for England and Wales, was lukewarm and it **refused to modify the NICE's role or its operating procedures concerning healthcare**, reported PJB news.

The House of Commons health committee report, which was delivered in January, 2008 found [...] irregularities in the NICE's guidance concerning the national health service.

But **the government commended NICE's role in promoting cost-effective health care** and dismissed several of the committee's recommendations, as operational matters for NICE itself.

ALTERNATIVES TO QALYs?

Reliance on QALYs
as a “universal and comprehensive” measure of (health-related) benefits?

Societal WTP as an Alternative Metric?

- **Hypothetical Acute Pain Relief Scenario**¹
 - Assume a surgical intervention for a small group of patients (say, n=1,000 cases per year) results in postoperative pain associated with a health state “worse than dead” (with a utility of -0.2), lasting for one day.
 - Assume further a new postoperative pain treatment results in pain relief leading to a health state with a utility of 0.8 at a total incremental cost of £ 250.
 - This treatment is associated with an ICER (**cost per QALY gained**) of £ 250 / $\{[(0.8 - (-0.2))] \times (1/365)\} = \mathbf{£ 91,250}$.
 - Given the size of the program, the **budgetary impact** (from the perspective of the health care scheme) is **£ 250,000 p.a.**
- **Would we be willing to pay for this intervention?**

¹Note that this scenario may be less hypothetical than it might seem at first glance!
cf. M. Stadler, M. Schlander, M. Braeckman et al. (2004)



ALTERNATIVES TO QALYs?

Reliance on QALYs
as a “universal and comprehensive” measure of (health-related) benefits?

Some Concerns concerning Cost per QALY Benchmarks

- **An Empirically Flawed Decision Rule**
 - The Consistency Argument – A Thinly Disguised Normative Claim
- **Severity of Condition**
 - Capacity to Benefit of Secondary Importance!
 - Life Saving Interventions and Rule of Rescue
- **The Value of Duration (of Life / of Benefit)**
 - Constant Proportional Trade-Off?
- **Mapping of Individual Utility and Societal Value?**
 - Cost-per-QALY League Tables?
 - From Sildenafil ... to Orphan Treatments
 - Small Benefits for Many Outweighing Important Benefits for Few
- **ICER Benchmarks and Opportunity Cost?**



FOUNDATIONS

Objectives of [collectively organized] health care

What are the Objectives of Health Care?¹

Two Concepts²

Utilitarian Thought	Deontological Thought
Economic Welfare Theory (ordinal utilitarianism) Extrawelfarism (cardinal medical utilitarianism)	Health Care Sector Professionals and the Public
	Stated (Official) Objectives Policy Makers, Payers, Providers
	Historic Roots of Medicine and Health Care
	Empirical Ethics (Public Preferences)
	Legal Environment
Moral Intuitions (e.g., Bentham, Mill, Harsanyi)	Moral Intuitions (e.g., Kant; Rawls, Daniels; Sen)

²and a dilemma, resulting from the lack of the one compelling, integrating “grand theory”? – cf. Thomas Nagel: *The Fragmentation of Value* (1979)

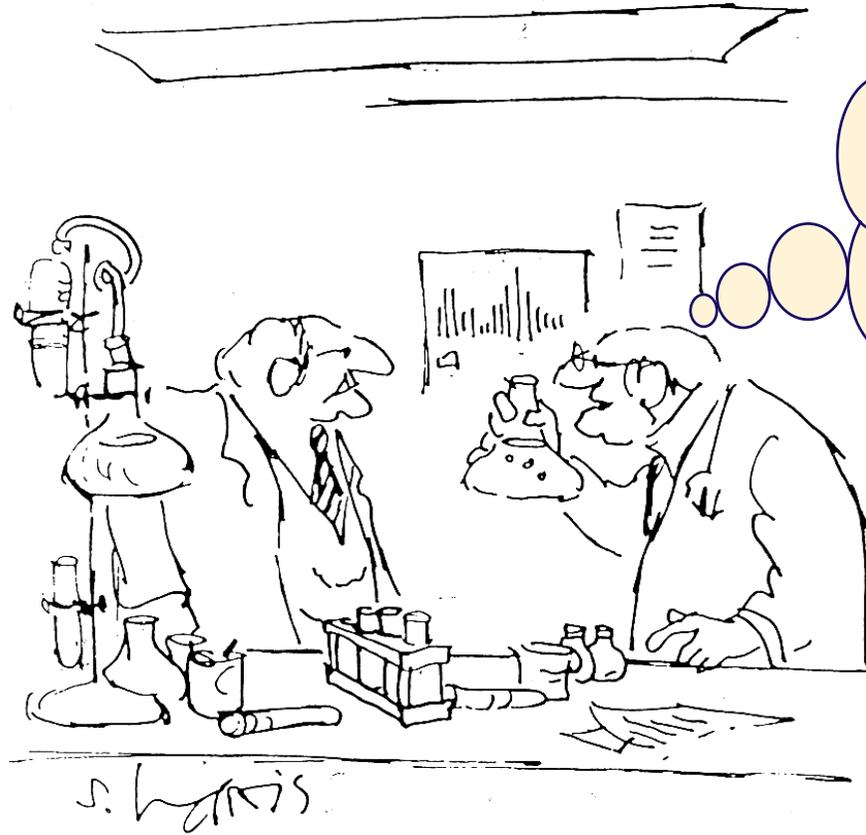
¹Related to collectively organized systems

of health care delivery and financing.



MODELING, UNCERTAINTY AND JUDGMENT

Economic evaluation of new medical technologies



**“It may well
bring about
immortality
—
but it will
take forever
to test it.”**