

Debate – Value and Impact of HTA

Alec Morton & Michael Schlander

“Are Our Current Ways to Conduct HTAs Fit for Purpose?”

II. Against the Motion [A Health Economist’s Perspective]

*Trigger Warning: The “Devil’s Advocate”
view might be perceived as provocative*

Michael Schlander (Heidelberg / Germany)

HTAi Global Policy Forum, The Hague, March 27, 2023

Value and Impact of HTA

Starting Points

There have been many definitions of “Health Technology Assessment” (HTA).¹

Legal and Institutional Context

Common Features

- ▢ a multidisciplinary process
- ▢ using explicit methods
- ▢ to determine the **value** of a health technology at different points in its lifecycle

Purpose

- ▢ to inform decision-making
- ▢ in order to promote an
 - ▢ **equitable**,
 - ▢ **efficient**,
 - ▢ and **high-quality** health system

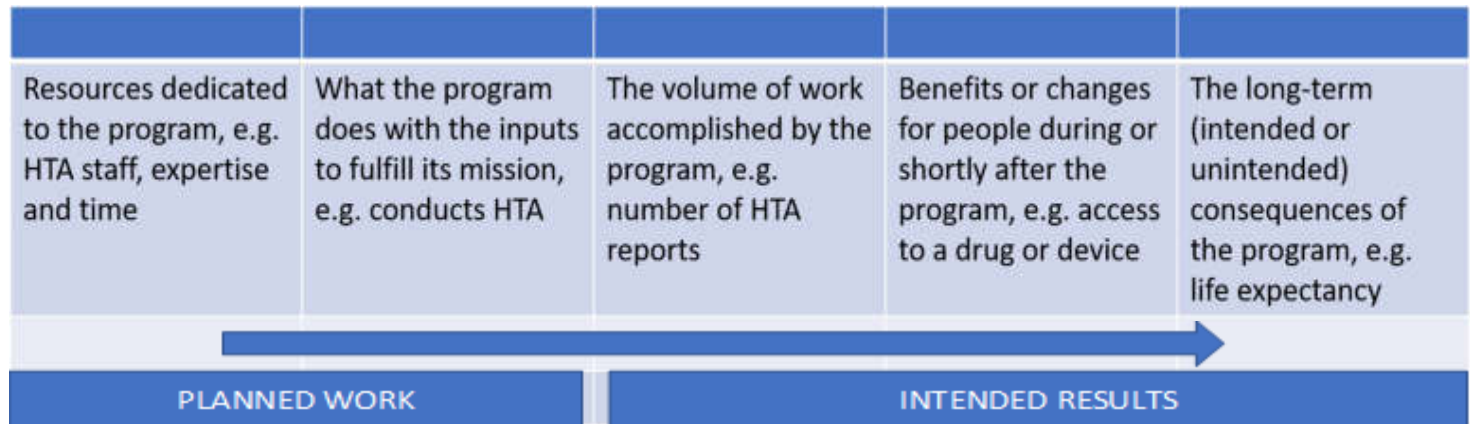
Stakeholders

- ▢ **Citizens**
- ▢ **Members** [of an Insurance Plan]
- ▢ Patients
- ▢ Payers
- ▢ Policy Makers
- ▢ Politicians
- ▢ ...
- ▢ Health Economists (?)

¹cf. B. O'Rourke, W. Oortwijn, T. Schuller et al.,
*International Journal of Technology Assessment
in Health Care* 36, 2020: 187-190

Value and Impact of HTA

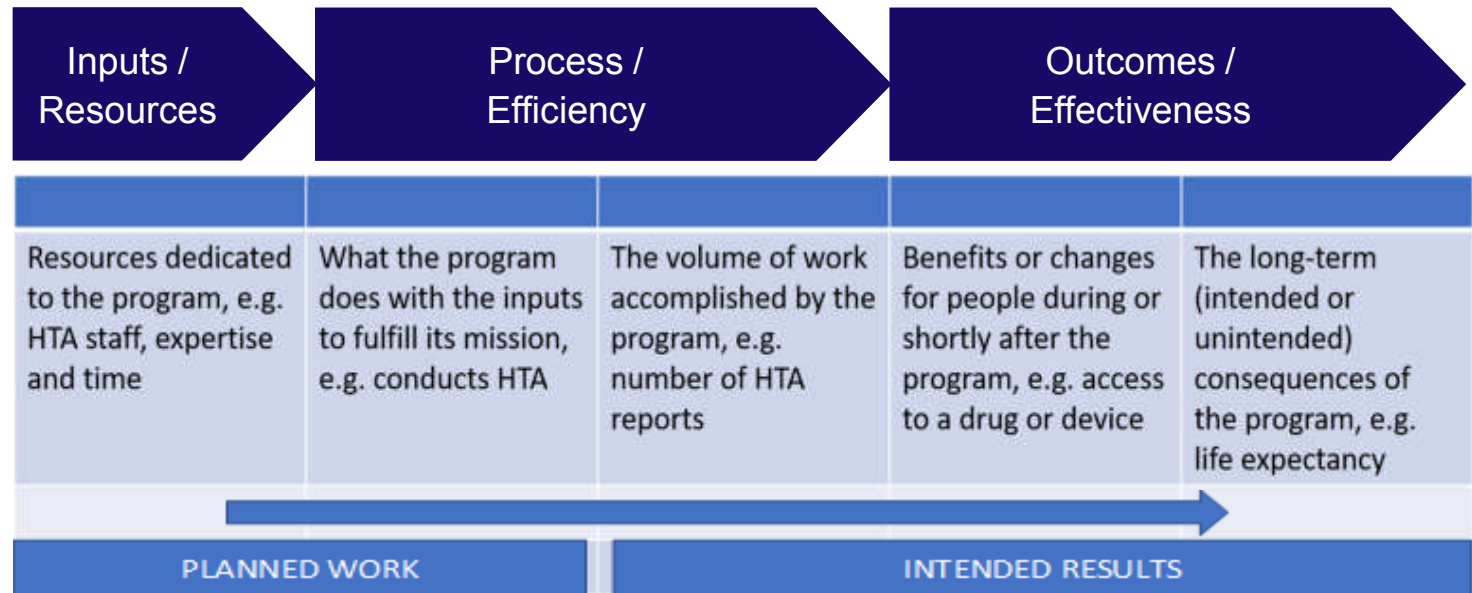
Proposed Framework (I)¹



¹D. Ollendorf et al. *The Value and Impact of Health Technology Assessment. HTAi Global Policy Forum 2023 Background Paper*. Edmonton, March 2023, p. 4

Value and Impact of HTA

Proposed Framework (I)¹



“Effectiveness” and “Efficiency”

– or the need to justify the appropriateness of the chosen effectiveness criterion:

- ▢ by definition, “**efficiency**” is a **secondary** or **instrumental objective**,
- ▢ whereas the “**effectiveness**” criterion invariably represents the **primary objective**.

¹D. Ollendorf et al. *The Value and Impact of Health Technology Assessment. HTAi Global Policy Forum 2023 Background Paper*. Edmonton, March 2023, p. 4:

²e.g., M.L. Barer, Th. Getzen, G.L. Stoddart (1998): M. Schlander (2009, 2017)

Value and Impact of HTA

Proposed Framework (II)¹

| | Domain | Examples and possible metrics/indicators |
|--------------------------|---|---|
| Inputs / Resources | Inputs: The contributions necessary to enable the program to be implemented | <ul style="list-style-type: none"> • Staff numbers (and skillsets) • Infrastructure • Funding (e.g. annual budget) • Relationship with key partners/position in health system |
| Process / Efficiency | Outputs: The program's activities and outputs (direct products/deliverables of the activities). | <ul style="list-style-type: none"> • Number and type of HTA reports/products (e.g. technical reports to inform decision makers or mandatory guidance) • Time to produce reports/recommendations • Stakeholder satisfaction/engagement with the process • Price cuts/negotiations/MEAs (where relevant) |
| Outcomes / Effectiveness | Outcomes: measure of effects/changes in the short- to medium-term | <ul style="list-style-type: none"> • Appropriate technology usage (investment/disinvestment, uptake) • Variation in health care (maps) • Improved health outcomes (behaviors/wellness and QoL measures) • Efficient allocation of system resources (funding/staffing) • Engagement and connectedness of system: inclusivity, transparency, trust (quality, quantity and timing of dialogues) |
| Outcomes / Effectiveness | Health system and societal impacts: measure of long-term, distal effects | <ul style="list-style-type: none"> • Improved life expectancy / quality-adjusted survival • Reduced in health/education/social inequalities • Sustainable funding of health system(s) • Reduction in environmental effects of the medical technology industry • Use of evidence-based, transparent and fair decision-making (within health and beyond) |

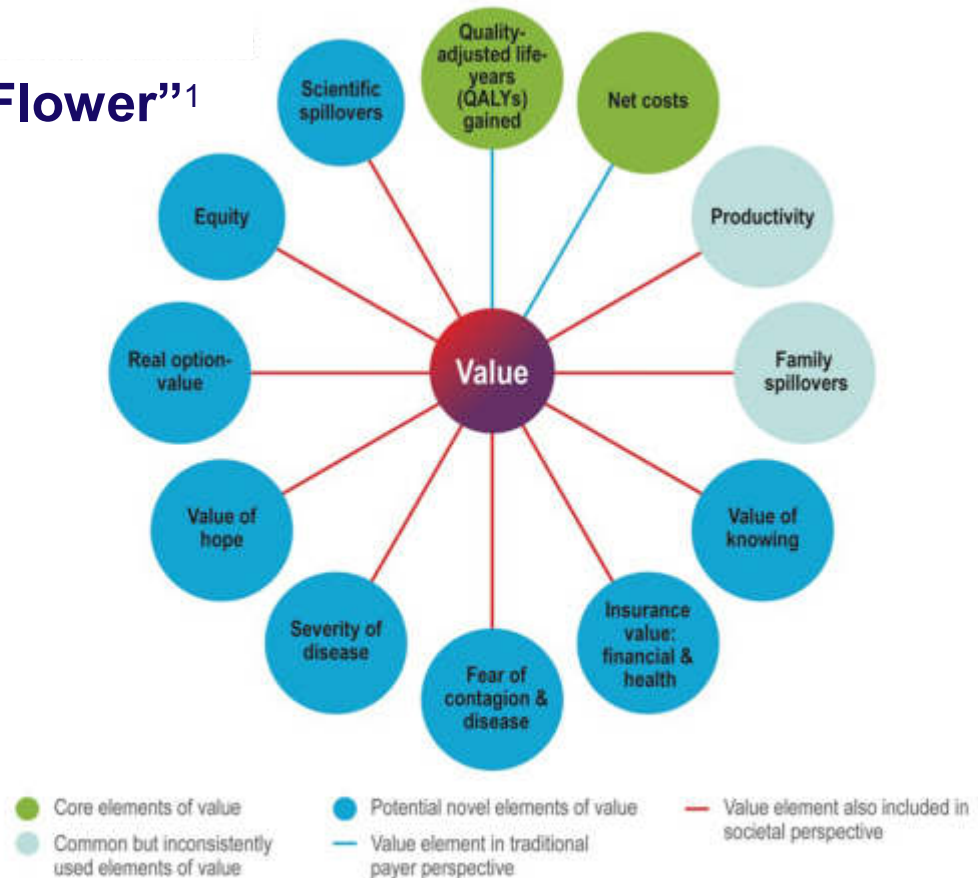
5/30

¹D. Ollendorf et al. *The Value and Impact of Health Technology Assessment. HTAi Global Policy Forum 2023 Background Paper*. Edmonton, March 2023, p. 13

Value and Impact of HTA

Importance of Values (Objectives; “Effectiveness”)¹

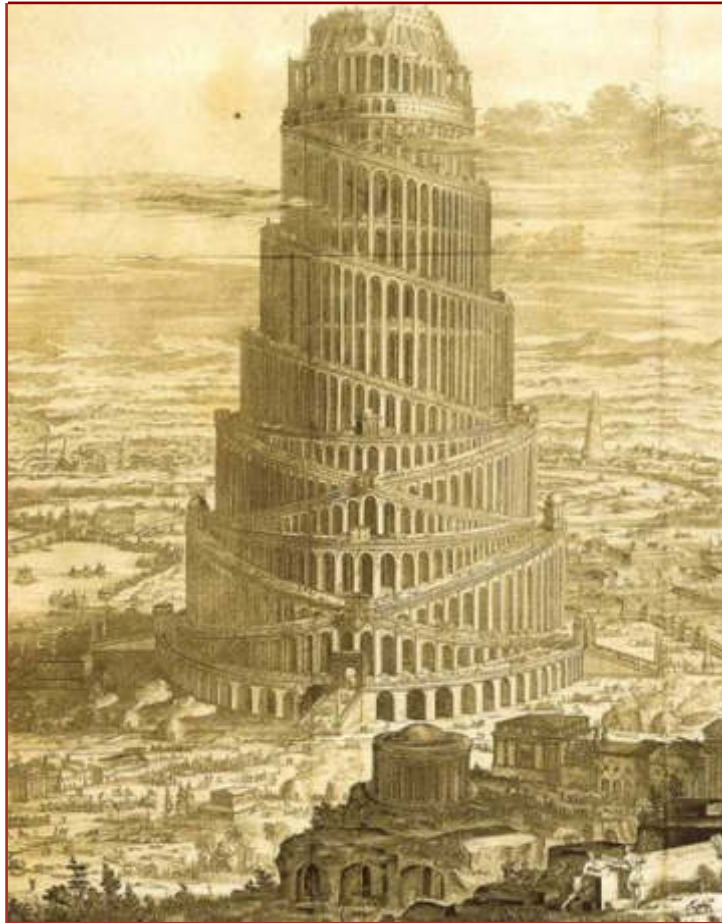
The “Value Flower”¹



¹ISPOR (2018)

Value and Impact of HTA

Values Talk – a Tower of Bable?¹

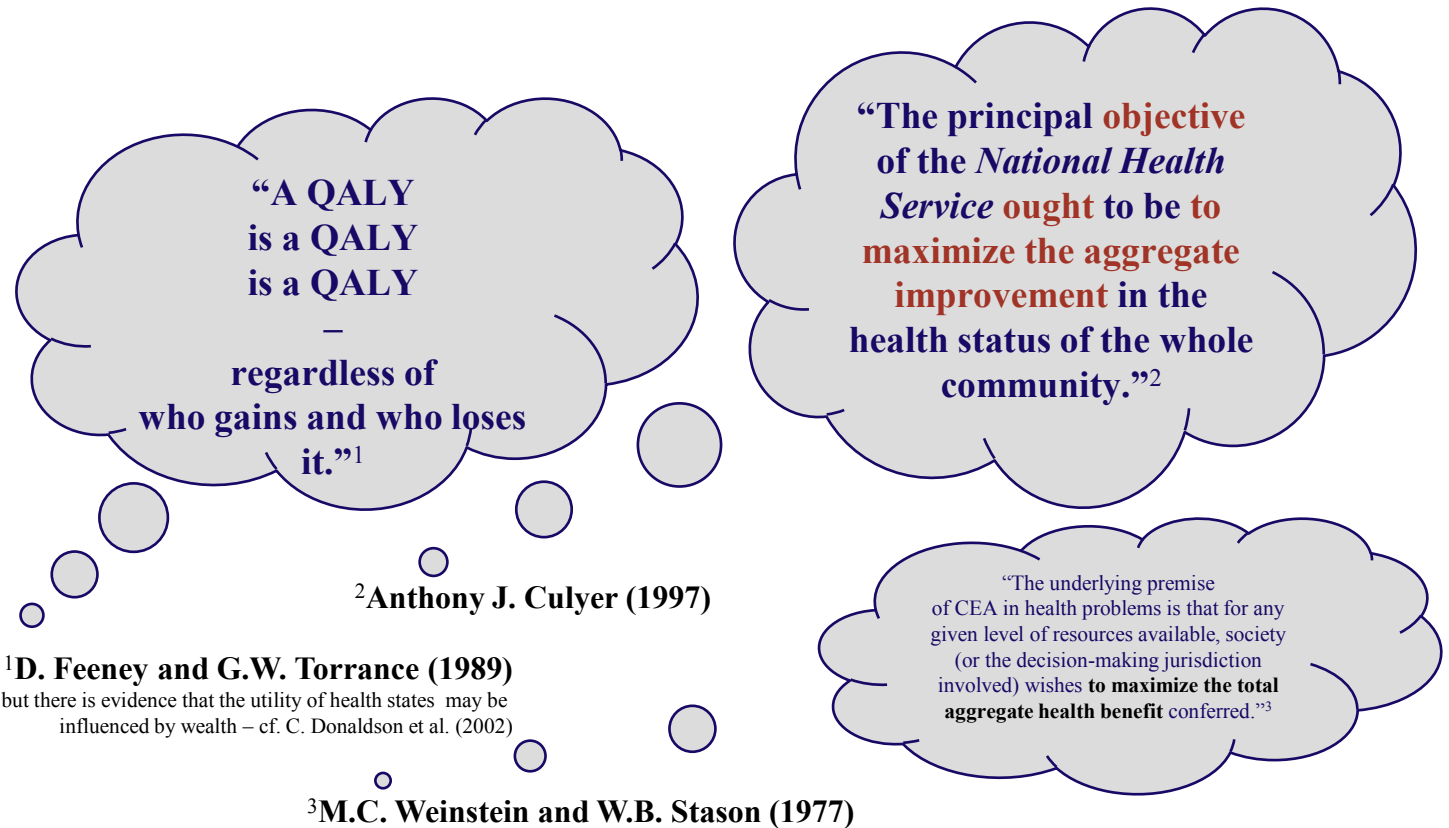


- Referral to many different and often incommensurate things...
- **A key paradox:**
The discourse about values is both very important and very ambiguous.
- Stakeholders may be tempted to react to this problem with either
reductionism
(focusing on one particular definition of values to the neglect of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

¹based on a Canadian policy analysis by Mita Giacomini et al. (2004)

Value and Impact of HTA

The Reductionist (/Technocratic) Approach of Mainstream Applied “Health Economics”



Value and Impact of HTA

Utilitarianism and the Measurement and Aggregation of Quality – Adjusted Life Years

PAUL DOLAN

Health Care Analysis 9: 65–76, 2001.

Pharmacoeconomics (2019) 37:293–299
<https://doi.org/10.1007/s40273-018-0742-2>

CURRENT OPINION

QALY maximisation and people's preferences: a methodological review of the literature

Paul Dolan^{a,b,*}, Rebecca Shaw^c, Aki Tsuchiya^d and Alan Williams^e

HEALTH ECONOMICS
Health Econ. 14: 197–208 (2005)

Determining Value in Health Technology Assessment: Stay the Course or Tack Away?

J. Jaime Caro^{1,2,8} · John E. Brazier³ · Jonathan Karnon⁴ · Peter Kolominsky-Rabas⁵ · Alistair J. McGuire¹ · Erik Nord⁶ · Michael Schlander⁷

Published online: 9 November 2018
© The Author(s) 2018

Abstract

The economic evaluation of new health technologies and the proposed additional costs has become an essential part of value. The determination of value is problematic because there exists a tension between the desire to maximize aggregate health, measured in terms of quality-adjusted life-years (QALYs), and the desire to ensure that the proposed additional costs are justified. A 'cost-effectiveness' threshold is used to gauge whether an intervention is sufficiently efficient in doing so. This approach has come under fire for failing to account for the value of the resources, regardless of pre-existing health status, benefit or adjusting the threshold to implement therapeutic options. The need for alternative methods to evaluate medical interventions for ultra-rare disorders with a view to clarifying the incremental cost per quality-adjusted life year gained? The need for alternative methods to evaluate medical interventions for ultra-rare disorders with a view to clarifying the incremental cost per quality-adjusted life year gained?

JOURNAL OF MARKET ACCESS & HEALTH POLICY
2018, VOL. 7, 1557981
<https://doi.org/10.1080/20016689.2018.1557981>

 **Routledge**
Taylor & Francis Group

ARTICLE

 OPEN ACCESS  Check for updates

Health technology assessment (HTA) and economic evaluation: efficiency or fairness first

Jeff Richardson^a and Michael Schlander^{b,c}

Health technology assessment (HTA) and economic evaluation are both used to inform decisions about the allocation of health resources. Commonly, a single 'cost-effectiveness' threshold is used to gauge whether an intervention is sufficiently efficient in doing so. This approach has come under fire for failing to account for the value of the resources, regardless of pre-existing health status, benefit or adjusting the threshold to implement therapeutic options. The need for alternative methods to evaluate medical interventions for ultra-rare disorders with a view to clarifying the incremental cost per quality-adjusted life year gained?

The Value of Freedom: A Review of the Current Developments and Conceptual Issues in the Measurement of Capability

Jasper Ubels, Karla Hernandez-Villafuerte & Michael Schlander

Value and Impact of HTA

The Myth of Maximization

and Some Further Loopholes of the Conventional Logic of Cost Effectiveness

Linearity Assumptions

- ▮ **Life time gained** (except for discounting)
“QALMS about QALYs”?
- ▮ **Quality of life**
Priority for those in particularly severe health states?
Constant proportional trade-off?
- ▮ **Probability (or “risk”)**
Large and/or immediate risks (cf. also “rule of rescue”)?
- ▮ **Number of patients benefitting**
[in effect, any impact of the dimensions of programs assumed away]
OMP issue, all-or-nothing decisions / “winners take it all”?

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Key Elements of the Logic of Cost Effectiveness

Use value: Quality-Adjusted Life Years (QALYs)

- ▢ (fully?) capture the value of health care interventions;
- ▢ are all created equal (“a QALY is a QALY is a QALY...”).

Aggregation: Maximizing the number of QALYs produced

- ▢ ought to be the primary objective of collectively financed health schemes,
- ▢ leading to the concept of thresholds (or benchmarks) for the maximum allowed cost per QALY gained.

Decreasing cost per QALY

- ▢ implies increasing social desirability of an intervention.

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Textbook Example: “QALY League Table”¹

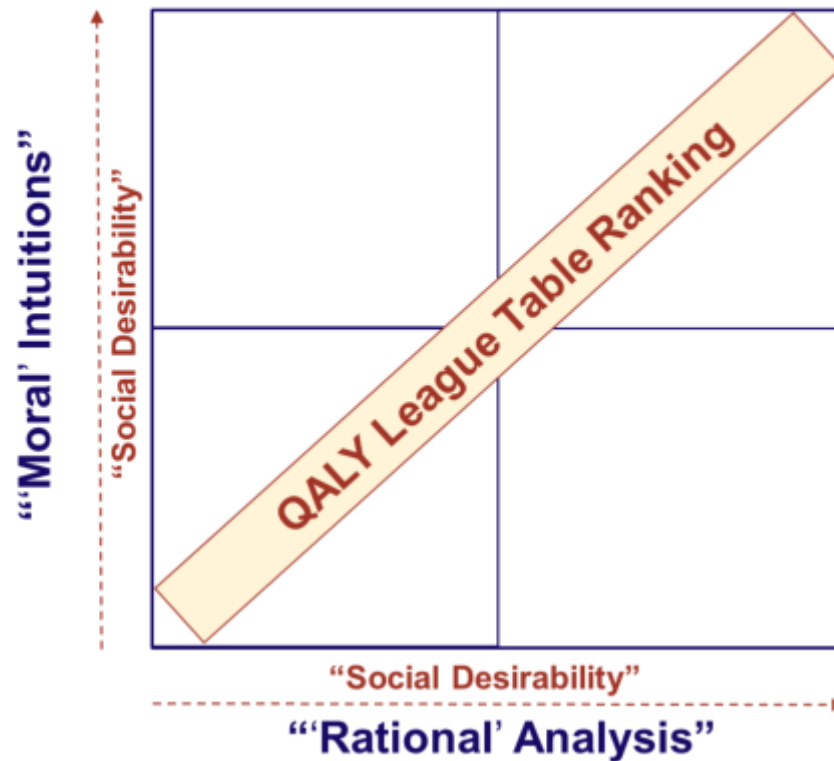
| Ranking [original] | Intervention [abbreviated; comparator not stated in original table] | Cost / QALY [£ (1990)] |
|-----------------------|--|---------------------------|
| 3 | G.p. advice to stop smoking | £ 270 |
| 5 | Antihypertensive therapy to prevent stroke | £ 940 |
| 6 | Pacemaker implantation | £ 1,100 |
| 7 | Valve replacement for aortic stenosis | £ 1,140 |
| 8 | Hip replacement | £ 1,180 |
| 9 | Cholesterol testing and treatment | £ 1,480 |
| 11 | Kidney transplant | £ 4,710 |
| 12 | Breast cancer screening | £ 5,780 |
| 15 | Home hemodialysis | £ 17,260 |
| 18 | Hospital hemodialysis | £ 21,970 |
| 20 | Neurosurgery for malignant intracranial tumors | £ 107,780 |
| 21 | Epoetin alfa therapy for anemia in dialysis patients | £ 126,290 |

¹A. Maynard. *Economic Journal* 1991; 101 (408): 1277-1286

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Reflective Equilibrium



Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Measures of efficiency in healthcare: QALMs about QALYs?

Michael Schlander^{a,b,c,*}

^aInstitute for Innovation & Valuation in Health Care (InnoVal^{HC})

^bUniversität Heidelberg, Medizinische Fakultät Mannheim (Institut für Public Health)

^cHochschule für Wirtschaft Ludwigshafen

Summary

Comparative economic evaluations are concerned with the relative efficiency of alternative uses for scarce resources. Cost-benefit analysis (CBA) is grounded in economic welfare theory and attempts to identify alternatives with a net social benefit, measuring the created value in terms of individual willingness to pay (WTP). In applied health economics, cost-effectiveness evaluation (CEA) is more widely used than CBA, adopting a modified efficiency criterion, minimization of incremental costs per quality-adjusted life year (QALY) gained ("cost-utility analysis," CUA).

CBA has been greeted with skepticism in the health policy field, primarily owing to resistance to a monetary measure of benefit and owing to concerns that WTP may be unduly influenced by ability to pay. The move to CUA, however, has not

been without problems. The framework deviates from economic theory in important aspects and rests on a set of highly restrictive assumptions, some of which must be considered as empirically falsified. Results of CUAs do not seem to be aligned with well-documented social preferences and the needs of healthcare policy makers acting on behalf of society. By implication, there is reason to assume that a context-independent value of a QALY does not exist, with potentially fatal consequences for any attempt to interpret CUAs in a normative way. Policy makers seem well advised to retain a pragmatic attitude towards the results of CUAs, while health economists should pay more attention to the further development of promising alternative evaluation paradigms as opposed to the application of algorithms grounded in poor theory.

Key words: efficiency, cost-benefit analysis, cost-effectiveness analysis, cost-utility analysis, willingness to pay, quality-adjusted life year (QALY)

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Revisiting the Fundamental Premise

“The Social Desirability of an Intervention is Inversely Related to its Incremental Cost per QALY Gained”

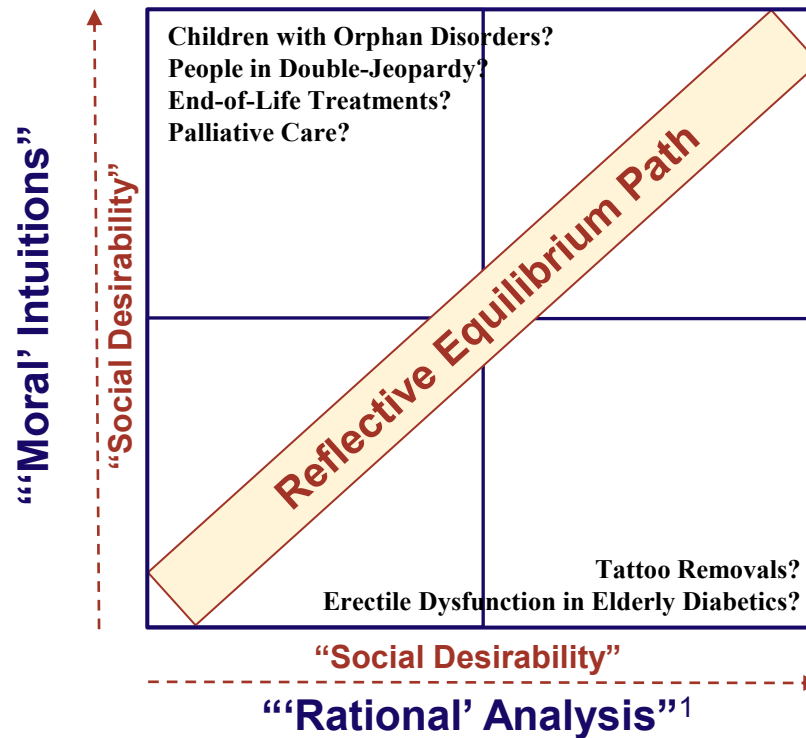
The assumption may create Reflective Equilibrium issues:

- ▢ Sildenafil for elderly diabetics with erectile dysfunction
- ▢ Removal of Tattoos
compared to
- ▢ Palliative Care,
- ▢ Interventions for people with comorbid conditions
(in “Double Jeopardy”, like the chronically disabled)
- ▢ Orphan Medicinal Products (OMPs) for (very) rare disorders

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Reflective Equilibrium?



¹ "rational" according to the "logic of cost effectiveness"

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

A Rapidly Growing Economic Literature ...

... on a broad range of characteristics (or “attributes”)¹
contributing to **Social Value Judgments**, *including*

▮ **Attributes of the Health Condition**

- ▮ individual valuation of health conditions
- ▮ **severity** of the condition
- ▮ **urgency** of an intervention
- ▮ unmet medical need
- ▮ capacity to benefit from an intervention
(however, to a lesser extent than assumed in conventional CEA)

▮ **Attributes of the Persons Afflicted**

- ▮ **non-discrimination** (and claims-based approaches)
- ▮ **age** (and fair innings)
- ▮ other patient attributes
- ▮ fairness objectives; sharing; aversion against *all-or-nothing* decisions

¹for a review and in-depth discussion, see M. Schlander, S. Garattini, S. Holm, et al., *Journal of Comparative Effectiveness Research* 2014; 3 (4): 399-422.

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Uneasiness with Thresholds

HTA Agencies

- NICE (England): end-of-life treatments; ultra-orphan disorders / HST process
- TLV (Sweden): adjustments for severity

Research-Based Biopharmaceutical Industry

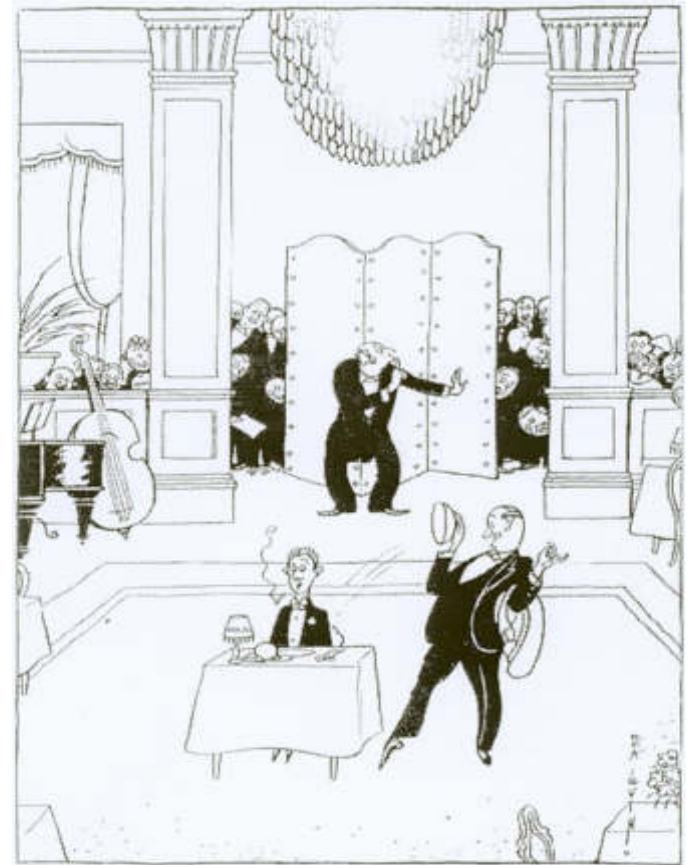
- Barriers to access
- Innovation (dealing with uncertainty and dynamic efficiency)

Payers

- NHS England: Cancer Drugs Fund (CDF)
- A “prescription for uncontrolled growth in expenditures”¹?

Academics

- Increasing recognition of “other criteria”
- Scientific foundation of benchmarks: might be too high² / too low³ / non-existent⁴?



THE ICER SURPRISE

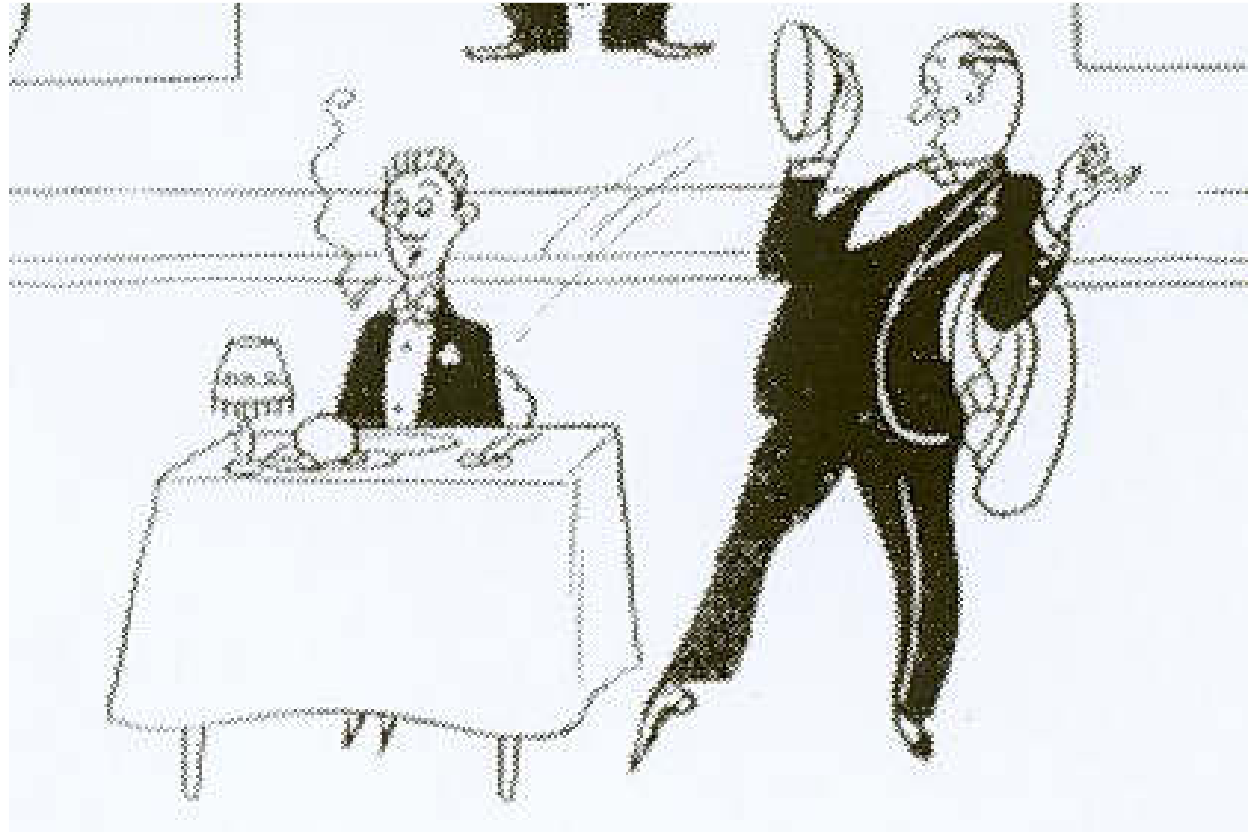
Source of cartoon: THE NEW YORKER 1925

¹A. Gafni, S. Birch (1993)
²K. Claxton et al. (2013)
³M. Schlander et al. (2017)
⁴when social preferences are taken into account

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

ICER: “Information Created to Evade Reality”¹ (?)



THE ICER SURPRISE

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

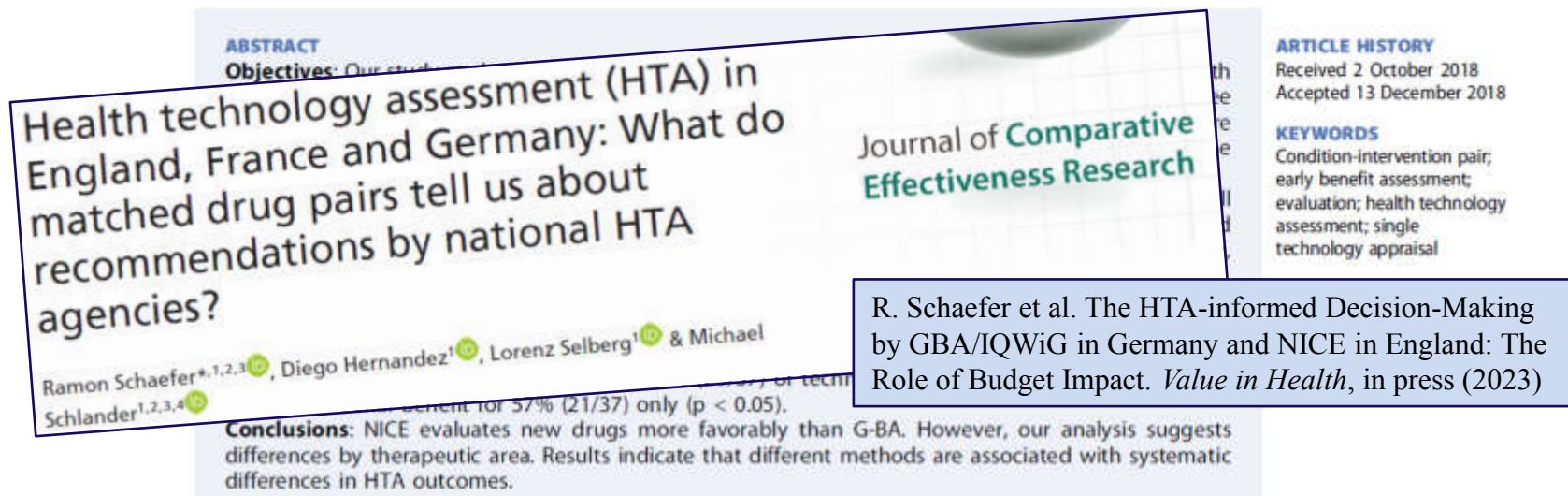
Not a Theoretical Debate

Different HTA Methods, Different HTA Outcomes¹

Is the National Institute for Health and Care Excellence (NICE) in England more 'innovation-friendly' than the Federal Joint Committee (G-BA) in Germany?

Ramon Schaefer^{a,b,c} and Michael Schlander^{a,b,c}

^aDivision of Health Economics, German Cancer Research Center (Deutsches Krebsforschungszentrum, DKFZ), Heidelberg, Germany; ^bMannheim Medical Faculty, University of Heidelberg, Mannheim, Germany; ^cInstitute for Innovation & Valuation in Health Care (InnoVal^{HC}), Wiesbaden, Germany



R. Schaefer et al. The HTA-informed Decision-Making by GBA/IQWiG in Germany and NICE in England: The Role of Budget Impact. *Value in Health*, in press (2023)

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Social Cost Value Analysis (SCVA)

adopting a citizens' perspective implies shifting the focus from cost per patient to cost at program level

- **A decision-makers' (and payers') concern**

has traditionally been overall **budgetary impact** (*transfer cost*)

- **A social value perspective**

(instead of a narrow focus on QALYs as a proxy for individual health-related “utility” and their aggregation) corresponds to the social **opportunity cost** (or [social] value foregone) being reflected by net budgetary impact (*transfer cost*)

- This reflects **the type of decisions informed by HTAs**,

i.e., decisions on the adoption of health technologies at the level of programs (*not* at the level of individual patients)

- Incidentally, a focus on net budgetary impact corresponds to the prevailing strategy of biopharmaceutical companies to maximize **life cycle revenues**.

Value and Impact of HTA

Addressing Anomalies and Critical Assumptions of the Conventional Logic

Social Cost Value Analysis (SCVA)

How different is it from Conventional Cost Effectiveness Analysis?

Moving from CEA to SCVA

would be of little consequence, if and when

- the QALY calculation algorithm offered an adequate proxy for individual [health-related] utility gains,
 - including the transformation of length and quality of life inherent in the QALY model [and some further assumptions],
- individual [health-related] utility gains mapped into social [health-related] utility gains,
- citizens were not risk averse,
- citizens had little (if any) consideration for others,
 - which would eliminate any non-selfish preferences (for sharing health care resources),
- citizens' social WTP was strictly proportional to the number of patients benefitting from the adoption of a health care program.

Value and Impact of HTA

If the Problem is a Distributional One,
So Should be the Proposed Solution...

but applied health economics
appears almost obsessed
with allocative issues

**“Abstracting from
Distributional Effects,
this Policy
is Efficient.”¹**



¹**Uwe E. Reinhardt (1936-2017)**

In: Barer/Getzen/Stoddart: Health, Health Care and Health Economics (1998; pp. 1-52)

Value and Impact of HTA

If the Problem is a Distributional One,
So Should be the Proposed Solution...

Woran bemisst sich Effizienz im Gesundheitswesen?

Zur Klärung fachwissenschaftlicher
Begriffe und Kriterien

Der Begriff der Effizienz hat einen positiven Beiklang. Für Nichtökonomien bleibt jedoch manchmal verborgen, dass sich der Sprachgebrauch der Ökonomen deutlich vom Alltagsverständnis unterscheidet. Das kann erhebliche Konsequenzen haben, wenn Ökonomen maßgebende Aussagen über Effizienz und Ineffizienz im Gesundheitswesen machen. Ein instruktives Beispiel hierfür bietet die Debatte über effizientes Verhalten und die sogenannte „Rule of Rescue.“ Um einige der zugrundeliegenden Zusammenhänge zu verstehen, ist es erforderlich, die verschiedenen Erwartungen an das Gesundheitswesen zu kennen. Sie ergeben sich aus den unterschiedlichen Zielvorstellungen oder Effektivitätskriterien, von denen Ökonomen, Ärzte, Patienten und gesunde Versicherte ausgehen. Denn „Effizienz“ kann per definitionem immer nur ein instrumentelles Ziel sein, über das erst dann sinnvoll diskutiert werden kann, wenn zuvor Einvernehmen über die zu verfolgenden Ziele hergestellt worden ist.



Michael Schlander

M.L. Barer, Th. Getzen, G.L. Stoddart. *Health, Health Care and Health Economics – Perspectives on Distribution*. Wiley 1998.
M. Schlander. Health Economics – In Search of Efficiency. *Z. Evid. Fortbild. Qual. Gesundh. wesen (ZEFQ)* 103 (2009) 117–125.
M. Schlander. Woran bemisst sich Effizienz im Gesundheitswesen? *AMOS International* 11 (2017) 22–31.

Value and Impact of HTA

Focus on “*Due Process*” as a Solution (as an add-on during “Appraisal”)?

Accountability for Reasonableness (“A4R”)¹

▢ **Publicity**

- ▢ Decisions and their underlying rationales must be publicly accessible

▢ **Relevance**

- ▢ These rationales must rest on evidence, reasons, and principles that plan managers, clinicians, patients, and consumers agree are pertinent to deciding how to meet diverse needs under resource restraints

▢ **Revisability and appeals**

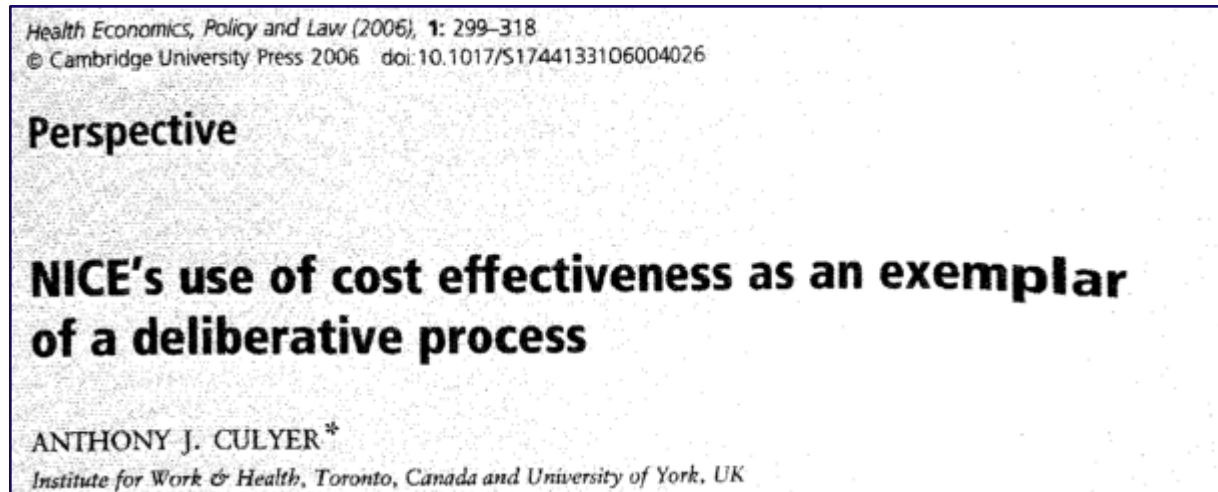
- ▢ A mechanism must allow challenges to limit-setting decisions, help resolve those challenges, and allow revisions in light of further evidence and arguments

▢ **Enforcement**

- ▢ A voluntary or public regulatory process must ensure that decision makers fulfill the first three conditions

Value and Impact of HTA

Debate on “*Due Process*” as a Solution (as an add-on during “Appraisal”)?



The use of cost-effectiveness by the National Institute for Health and Clinical Excellence (NICE): no(t yet an) exemplar of a deliberative process

M Schlander

Journal of Medical Ethics 2008; 34: 534-539

Institute for Innovation & Valuation in Health Care (InnoVal^{HC}); University of Applied Economic Sciences Ludwigshafen, Germany; Mannheim Medical Faculty, University of Heidelberg, Heidelberg, Germany

ABSTRACT

Democratic societies find it difficult to reach consensus concerning principles for healthcare distribution in the face of resource constraints. At the same time the need for legitimacy of allocation decisions has been recognised. Against this background, the National Institute for Health and Clinical Excellence (NICE) aspires to meet the

comparison techniques (in order to enable indirect comparisons of technologies in the absence of head-to-head studies).

THE LOGIC OF COST-EFFECTIVENESS

The logic of cost-effectiveness, as adopted by NICE and in contrast to traditional cost-benefit analysis

Value and Impact of HTA

Multi-Criteria Decision-Making (MCDM) as an Alternative?

Example: SwissHTA¹ – A Multi-Stakeholder Consensus on HTA

Drivers of Social Value (*beyond individual health gain*²)

▮ **Severity and Urgency**

of initial health problem

▮ **“Fair Innings” / Age**

interventions for children and young people who have not had an opportunity to pursue their individual life plans (a decent minimum of health as a “*conditional good*”)

▮ **Nondiscrimination or Fairness**

fair chance of access to effective health care,
even if condition is rare or intervention is expensive (sic!)

▮ **“Bagatellen”**

exclusion of **or low priority** for minor self-limiting health problems
and ‘affordable’ interventions³

▮ **Fast Access to Real Innovation**⁴

¹See www.swisshta.ch.

²Hypotheses, based on literature review and expert consensus; SwissHTA identified a major **research need**;

³‘affordability’ determined from a patient’s out-of-pocket perspective; ⁴‘innovation’ to be defined appropriately

Value and Impact of HTA

Empirical Ethics as an Alternative?

Adopting a Citizens' Perspective:

The need for further research
re. the integration into HTA of
social norms and preferences



“The taste
for improving the health
of others
appears to be stronger
than for improving
other aspects of their welfare.”¹

¹**Kenneth Arrow** (1921-2017)

Uncertainty and the Welfare Economics of Medical Care (1963; p. 954)

Value and Impact of HTA

Some Tentative Conclusions from an Economics Perspective

▮ Purpose

- ▮ Respect the relevant legal and institutional context

▮ Evaluation Criteria (Value Judgments & “Preferences”)

- ▮ Technology Assessments should support appraisal processes in the best possible way – requiring internal alignment of criteria used:
- ▮ Coherent value systems need to bridge the different phases from topic selection and scoping to assessment, appraisal, and decision-making
- ▮ The decision whose values (/preferences) should count, and how to handle interpersonal comparisons, will have far-reaching implications for HTA
- ▮ Beware of an uncritical adoption of [reductionist] “efficiency-first” frameworks

▮ Process-Related Aspects

- ▮ Methodological pluralism
- ▮ Transparent and systematic approach
- ▮ Processes for appeals, enforcement, and implementation
- ▮ Learning system involving the public (citizen representatives)

Value and Impact of HTA

Some Tentative Conclusions from an Economics Perspective

Let's have **more** Health Technology Assessment in the future

- ▢ respecting the need for publicity and consistency
- ▢ in particular w.r.t. its underlying value judgments,
- ▢ which might better reflect its institutional context including
- ▢ prevailing social norms and preferences to share health care resources fairly.

Let's work **more** on improved methods for a [better] alignment of value judgments applied by conventional health economics and prevailing social norms and preferences held by citizens.