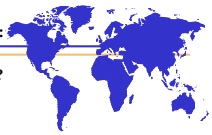
International Health Economics Association (iHEA)
5th World Congress, Barcelona / Spain, July 9-13, 2005

Economic evaluation of medical interventions:

Answering questions people are unwilling to ask?



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IV

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]

[1]**Michael Drummond (2004)**
Australian Economic Review 37 (1) : 3-11

IV

THE ISSUE

Subject of the presentation: a normative dilemma

- For developed market economies, health care expenditures rising faster than the economy as a whole have been shown to be "affordable" for the foreseeable future[1].
- In contrast, **resource constraints** notoriously do plague **collective systems** (both government ["Beveridge"] and insurance ["Bismarck"] type systems) of health care financing.
- **Rational resource allocation** within these collective systems is a challenge that has contributed to an increasingly high profile of health economics as a scientific discipline.
- Rational decision making (and support hereof) requires a consensus on the primary **objectives** to be pursued.
- Do the objectives **assumed** for most health economic evaluations of medical interventions meet this criterion?
- In case they fail to, what other questions regarding "rational resource allocation" do (and/or should) people ask?

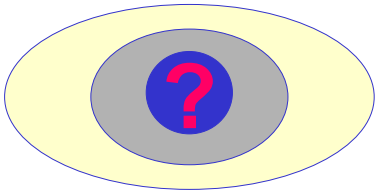
[1]M. Chernew et al., Increased Spending On Health Care: How Much Can The United States Afford? *Health Affairs* 2003, 22: 15-25; also M. Schlander et al, Affordability Sensitive To Economic Growth Rates. *Health Affairs* 2004, 23: 276-277; M. Schlander and O. Schwarz, Affordability of Increasing Health Care Expenditures in Germany: a Macroeconomic Analysis. *Gesundheitsoekonomie & Qualitaetsmanagement* 2005, 10: 178-187.

IV

THE SCOPE

A word of warning before:
The scope of the presentation will be limited to a core area of "essential" health care[1]

Health is defined by **WHO's Constitution** as a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity.



[1]While recognizing that, apart from theoretical reasoning, there is no simple universally accepted approach to define "essential" health care in practical terms.

WELFARISM

Some Foundations of Health Economics

What We Teach Our Students (1)

- **Economic Welfare Theory**
  - "Clearly, the Paretian approach has **the theoretical high ground**, although even the most committed Paretians acknowledge that distributional issues as well as efficiency issues need to be dealt with."[1]
- **Principle: "The No-Loser Constraint"**
  - The Absolute No-Loser Constraint: "Pareto Principle"
  - The **Theory of Cost-Benefit-Analysis**: No-Loser Constraint with hypothetical compensation in terms of goods "Potential Pareto Improvement (Kaldor-Hicks Criterion)"[1]
  - **Practical Cost-Benefit Analysis**: No-Loser Constraint with hypothetical compensation in terms of money "Potential Pareto Improvement (Kaldor-Hicks Criterion)"[2]

[1]M. Drummond et al., *Methods for the Economic Evaluation of Health Care Programmes*, 2nd ed. 1997, p.287.
[2]Note that the criterion does not require that the compensation (redistribution) actually takes place.

WELFARISM

Some Foundations of Health Economics

Welfare Economics[1]

- **Key Assumptions:**
  - Social welfare is made up from the welfare ("utilities") of each individual member of the society.
  - Individuals are the best judges of their own welfare.
  - The three postulates of welfare theory are frequently described as innocuous:
- **Consumer sovereignty**
- **Non-Paternalism**
- **Unanimity**
  - Consumer choice, consumer empowerment, individual responsibility, economic efficiency, enhanced economic welfare…

[1]Given time constraints of this presentation, the following necessarily is an incomplete account of the theoretical frameworks discussed.

What We Teach Our Students (2)

"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]

[1] **Jules Dupuit (1844)**

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

"Efficiency"

- "The **efficiency criterion** is an example of a consequentialist **normative theory**. … It pronounces that between two policies, we should always prefer the one that yields the higher social gain."[1]
- "A change is a good thing if it would be possible in principle for the winners **to compensate the losers** for their losses and still remain winners. If a policy increases Jack's income by $10, reduces Jill's by $5, and has no other effects, … the policy is a good one … according to the efficiency criterion."[1]
- "The mere fact that it is **possible to create potential Pareto improving redistribution possibilities** is enough to rank one state over another on efficiency grounds."[2]

[1] Steven E. Landsburg: *Price Theory and Application*, 5th ed., Mason, OH: South-Western 2002, pp. 293ff.
[2] Robin Broadway and Neil Bruce, *Welfare Economics*, Oxford: Basil Blackwell 1984, p. 97.
The question arises whether there exist compensation possibilities (in money or else) in the core area of "essential" health care.
This includes, in other words, the issue: is there a meaningful and acceptable "marginal rate of substitution" across the full spectrum of health?

Policy Prescriptions

- "The ranking of social states is inevitably a normative procedure; that is, it involves making value judgments. … **Some value judgments** might, in fact, **command widespread support**, and rankings based on them might therefore legitimately form the basis for actual policy prescriptions.
- The use of welfare economics for policy purposes is … based on this premise.
- Much of the welfare economic analyses underlying policy prescriptions is based on a certain set of value judgments which are widely accepted among economists."[1]

[1] Robin Broadway and Neil Bruce, *Welfare Economics*, Oxford: Basil Blackwell 1984, p. 2.
It has been shown, however, that non-economists do not necessarily share these value judgments. – cf. R. Feldman and M.A. Morrissey: Health economics: a report on the field. *Journal of Health Politics, Policy, and Law* 1990, 15: 627-646.

WELFARISM

In particular, two assumptions of economic welfare theory have attracted criticism from a group of health economists ("extra-welfarists")

An Extra-Welfarist Critique[5]

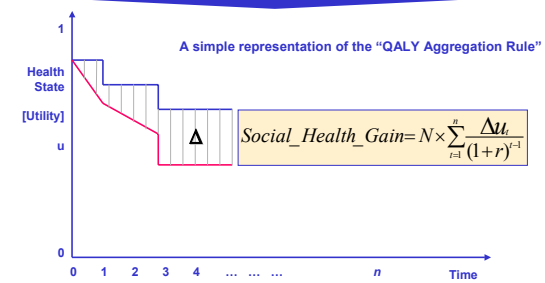
1. **"The monetary measurement [of benefits in cost-benefit analysis] inherently favors the wealthy over the poor."**[1]
   - "Extra-welfarists *and many decision-makers in the real world of health care* are willing to accept an approach that considers outcomes equitably (as CEA using QALYs does), rather 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.

[1]M.R. Gold et al. (1996), p.26; [2]M.C. Weinstein and W. Manning (1997), p. 127; [3]A.J. Culyer (1989), p. 51; [4]C. Donaldson et al. (2002); [5]Thomas Rice (1998, 2002) has provided a systematic critique of welfare theory as a foundation of health economics.

EXTRA-WELFARISM

The logic of cost-effectiveness

The Conventional Unit of Health Outcomes: QALYs



EXTRA-WELFARISM

The logic of cost-effectiveness

Standard Extra-Welfarist Methods: Really "Good Enough"?

- **Claim:** "**Cost-utility analysis** [*i.e., cost-effectiveness analysis with QALYs as the effectiveness measure*] **should** be used … when the programmes being compared have a wide range of different kinds of outcomes and you wish to have **a common unit of output for comparison**."[1]
  - Do our **methods** (*in principle*) live up to this promise?
    - Assumptions of presumably "technical nature" (e.g., constant proportional trade-off), etc.?
    - Assumption of additive separability and time preference?
    - (In)Sensitivity to small differences (time; HRQoL)?
    - Reflecting co-existing conditions (comorbidity)?
    - Arbitrary conventions regarding "thresholds"; unclear opportunity costs
  - ***Beyond* methods:**
    - Assumption of "distributive neutrality"?
    - (When) Does "context" matter?
    - Whose health state preferences should count (cf. adaptation of patients)?

[1]M.F. Drummond et al. (1997), p. 141f.

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]

[1] **D. Feeney and G.W. Torrance (1989)**
but there is evidence that the utility of health states may be influenced by wealth – cf. C. Donaldson et al. (2002)

[2] **Anthony J. Culyer (1997)**

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

EXTRA-WELFARISM

The logic of cost-effectiveness

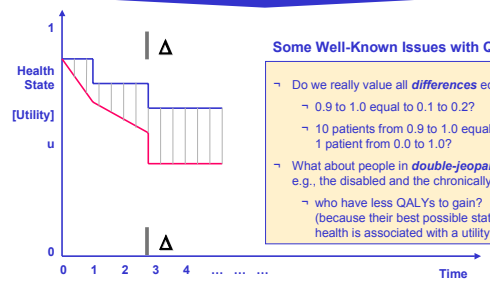
Utilitarian Thought

- **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 logic of cost-effectiveness

The Conventional Unit of Health Outcomes: 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].

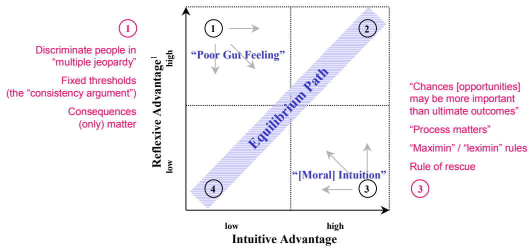
[1] cf. recent review by P. Dolan et al. (2005)

The logic of cost-effectiveness

Concerns (1): Rehabilitating [moral] intuitions

What We Also Teach Our Students (1)

"Seek to reconcile rational analysis with your intuitions."



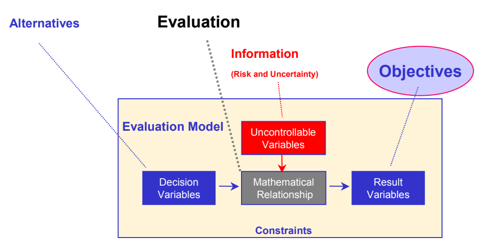
[1]Reflexive: "rational" according to the extra-welfarist theoretical framework as currently applied

The logic of cost-effectiveness

Concerns (2): Objectives matter

What We Also Teach Our Students (2)

Decision Analytic Principles[1]:



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

The logic of cost-effectiveness

Expected Utility Theory (von Neumann-Morgenstern)

- **Decision analysis:** "The axioms define rational behavior and their **repeated application** ensures that …the probability that the achieved utility differs from the maximum achievable utility by an arbitrarily small number approaching unity."[1]
  - **Yet, when "essential" health care is concerned, how many times can any given patient repeat the "resource allocation game"?**[2]
- **An extra-welfarist claim:** "The theory and the methods of measurement were developed as a normative (prescriptive) model for **individual decision-making** under uncertainty. The model is general; it applies to decision-making in all fields, including health."[3]
  - **Yet, (like EV / EMV) EU maximization is just one out of a number of alternative rational approaches for decision-making.** Other criteria include the "most probable state of nature", the "minimum regret", and the "maximin" decision rules.[4]
- **Expected utility theory: "Von Neumann Morgenstern theory is inappropriate in any context where the agents have reasons to differ in their assessment of factual matters as well as ultimate objectives."**[5]

[1]J. Marschak (1950), p. 139; [2]Here we encounter again the "compensation problem" – in **decision analysis** classes for managers, **we teach our students** to distinguish one-time decisions, for instance on corporate strategy, from repeated decisions (by type or dimension); [3]G.W. Torrance and D. Feeny (1989), p. 560; [4]EV: expected value; EMV: expected monetary value; EU: expected utility; cf. E. Turban and J.R. Meredith (4th ed., 1988), pp. 70ff.; [5]P. Mongin and C. D'Aspremont in *Handbook of Utility Theory, Vol. 1* (1998), see p. 404.

What are the Objectives of Health Care?[1]

(1) Stated (Official) Objectives – (a.) Policy Makers

Canada:

- Canadian Medicare "is widely regarded as an **important symbol of community**, a concrete manifestation of mutual support and concern.
  - It expresses a **fundamental equity** of Canadian citizens in the face of death and disease. As the Premier of Ottawa pointed out, there is no social program that we have that more defines Canadianism."[2]

Norway:

- "Two recent Norwegian commissions on priority setting in health care have identified that
  - **an important rationale for government involvement in health care is to provide benefit to those with the worst health prospects**."[3]

[2]R. Evans, M. Law, in: World Bank Seminar Series (1995); [3]J.Olsen (1997), taken from P. Dolan et al. (2005)
[1]Related to collectively organized systems

FOUNDATIONS

Objectives of [collectively organized] health care

What are the Objectives of Health Care?[1]

(1) Stated (Official) Objectives – (b.) Payers

"The principles of the NHS require it to be:

- Universal in its reach, available to anyone who wishes to use it;
- High quality, applying the latest knowledge and the highest professional standards;
- Available on the basis of **clinical need**, without regard for the patient's ability to pay."[2]

Kaiser Permanente:

- "As a nonprofit health plan, we are driven by the **needs of our members** rather than the needs of shareholders."
- "Our core values: Our mission is to provide affordable, high-quality health services and improve the **health of our members** …"[3]

Statutory Health Insurance (SHI / GKV):

- §1 ("Solidarity, Individual Responsibility"): "The mission of the SHI is to maintain, to restore, and to improve the health status of its members."
- §12 ("Economic Efficiency"): "Services provided have to be sufficient, appropriate, and efficient; they should not exceed **medical need**."[4]

[2]White Paper published by UK Department of Health (1996); [3]Taken from the official website, www.kaiserpermanente.org; [4]Sozialgesetzbuch V
[1]Related to collectively organized systems

FOUNDATIONS

Objectives of [collectively organized] health care

What are the Objectives of Health Care?[1]

(1) Stated (Official) Objectives – (c.) Providers

- **Physicians: The Hippocratic Oath**
  - "I will prescribe treatment to the best of my ability and judgement **to help the sick** …"
  - "I will enter the houses I visit in order **to help the sick**, and will not intentionally do harm or act corruptly …"

American Medical Association (AMA)[2]: Code of Medical Ethics

- "(I.) A physician shall be dedicated to providing competent medical care, with **compassion and respect for human dignity and rights**." …
- "(VIII.) A physician shall, while caring for a patient, regard responsibility to **the patient as paramount**."
- "(IX.) A physician shall support **access** to medical care **for all** people."

Code of Ethics for Nurses (developed from the "Nightingale Pledge")

- Respect for **human dignity**, primary **commitment to the patient**, protection of patient privacy…
- The ideas are based on Kantianism as well as Judeo-Christian tradition.

[2]Of course, economists are well aware of the fact that revealed preferences may differ from stated preferences. However, Ises and Oughts should not be confused. [1]Related to collectively organized systems

What are the Objectives of Health Care?[1]

(2) Historic Roots

- **"From Monastery to Hospital"**[2]:
  - The "Monastic Health Care System" as a starting point, beginning with Basil of Caesarea:
- **The Birth of the Hospital: Social Services at Basil's Hospital (ca. 330)**
  - **The Poor** were in the forefront of Basil's conception of Christian praxis and thus of his hospital. A concern for the poor is demonstrated throughout Basil's writings.
  - **Strangers and the Homeless; Orphans** – housing, car, and education were central to the charitable program of the Basileias.
  - **Lepers**, caring for the terminally ill, something unheard of before in ancient medicine.
  - **The Elderly and the Infirm**, who were physically unable of providing for themselves.
  - **The Sick** were destigmatized for the first time; unlike virtually any other type of ancient medical care, monastic medicine offered inpatient hospital care under the supervision of trained health care providers, including doctors and nurses.

"The Care of Strangers", "A Once Charitable Enterprise", …

[2]A.T. Crislip: *From Monastery to Hospital – Christian Monasticism & the Transformation of Health Care in Late Antiquity*. Ann Arbor, MI, 2005.
[1]Related to collectively organized systems of health care delivery and financing.

What are the Objectives of Health Care?[1]

(3) Empirical Ethics

NICE Citizen Council[2]:

- "Cost-utility analysis in the economic evaluation of particular interventions is a necessary, but insufficient, basis for decisions about cost-effectiveness."
- "Nevertheless, … philosophers are generally prepared to accept cost-utility analyses provided they are used to inform, rather than direct, decisions about setting priorities, and that other considerations are available to constrain morally offensive trade-offs."

Public expectations:

- **Fair distribution of health care services:** People think the efficiency with which society distributes health care resources must be balanced with the perceived fairness, or equity, of this distribution.
- **Give priority to severely ill patients** "even when their care is less cost-effective".
- **Avoid discrimination against people with chronic illness or disability.**[3]

Numerous Public Surveys:

- Confirming "solidarity" (e.g., no risk-adjusted premiums) as desired guiding principle[4]

[2]NICE: *Social Value Judgements*, Draft for consultation (April 8, 2005); [3]cf. Peter A. Ubel (2001); [4]75-84% of respondents in population studies.
[1]Related to collectively organized systems of health care delivery and financing.

"Hume's Guillotine"[1]

- **"One cannot deduce an ought from an is."**

  (David Hume, A Treatise of Human Nature)

  - *Though*:

  - "Hume's guillotine" may be overstated:
  - Oughts are powerfully influenced by Ises.

[1]cf. Mark Blaug (1992);
note that much of what has been said about the normative interpretation of "orthodox" welfare economics (claiming to be based on a set of principles most economists agree on) is also relevant to a normative interpretation of "empirical ethics".

What are the Objectives of Health Care?[1]

(4) Legal Environment

Oregon Health Plan (OHP)

- Explicit ranking based on cost-effectiveness of condition-treatment pairs inconsistent with the **Americans with Disabilities Act**?
- Oregon Health Plan implemented in a **political process** frequently praised for its public participation. In fact, **funding** was achieved in "the traditional way", i.e., by raising revenues directly and by sensible contracts with providers[2].
- ***Overall*, to date there has been little litigation that directly raised or challenged the use of CEA.**

Constitutional Provisions

- Rationing criteria must be status-blind[3].
- "Quality of life hardly acceptable for prioritization."[4]
- Broad agreement among the legal profession that there is a constitutional right for access to "essential" care restoring "normal functioning without stigma"[5].

[2]cf. L. Jacobs et al. (1999); [3]cf. V. Schmidt (1998); [4]J. Taupitz (1999), p. 128 –on methodological grounds (!); [5]cf. I. Ebsen (1997) pp.109, 119ff.
[1]Related to collectively organized systems of health care delivery and financing.

What are the Objectives of Health Care?[1]

(5) Normative Ethics

- **John C. Harsanyi** (e.g., 1977): A Defence of Utilitarianism
  - "Ethics as a branch of the general theory of rational behaviour"
- **Tom L. Beauchamp and James F. Childress**: (Bio-)Medical Ethics
  - Respect for autonomy; nonmaleficence, beneficence, justice
- **John Rawls** (1971): Notion of Primary Goods (and *basic liberties*)
  - Each person deserves consideration as a person, and this [neglect of a person's autonomy] militates against a distribution-indifferent view.
- **Norman Daniels** (1985): "Just Health Care"
  - A "decent minimum" of health -> "a normal range of opportunities"
- **Amartya Sen** (e.g., 1992): A Capability Perspective
  - Need to distinguish between achievements and capabilities
  - Importance of procedural justice (Sen's example: gender differences)

[1]Related to collectively organized systems of health care delivery and financing.

What are the Objectives of Health Care?[1]

Two Concepts[2]

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)

[2]and a dilemma, resulting from the lack of the one compelling, integrating "grand theory"? – cf. Thomas Nagel: *The Fragmentation of Value* (1979)
[1]Related to collectively organized systems of health care delivery and financing.

IMPLICATIONS

Some questions to answer before calling for "more consistency" in the implementation of the results of cost-effectiveness analyses

What are the Objectives of Health Care?[1]

- **More specifically, when and why do we distrust market allocation of health care?**
  - **Market failures** (allocative inefficiency) due to
    - Information asymmetry, moral hazard, …?
    - Externalities?
    - Public goods?
  - **Distributive concerns**
    - Objectives incompatible with market results?
    - A decent minimum of health as a conditional good?
    - Are such social objectives adequately captured by current standards for economic analyses of health technologies?

CONCLUSIONS

Some questions to ask ourselves

- Are the actual **objectives** of [collectively organized] health care systems (and their constituents, with a further specific emphasis on "essential" health care) compatible with conventional [health] economic theory *as currently applied* (welfarism, extrawelfarism)?
  - If this is not the case (as is suggested here):
- **Should** the objectives of health care systems (or, of which of their parts) be altered to achieve alignment with conventional economic theory and its assumptions, perhaps moderated by distributive concerns?
- **Should** the methods of *applied* health economic evaluations be developed to better reflect these objectives?
  - What are the implications for the discipline?
- A "normative" interpretation of health economic appraisals?
- Do current methodological [and professional] standards suffice to be accepted by non-economists as impartial analysts?
- Do current priorities within the discipline adequately reflect this?

CONCLUSIONS (CONT.-D)

From the perspective of many constituents of health care, the current state of affairs may imply that a needs-based approach to "essential health care" should be moderated by economic factors, not vice versa as thought (hoped for?) by some health economists, as evidenced by their calls for "more consistency" in implementation of the results of economic appraisals.

If health economic evaluation is to be used to its full potential,

there remains much to be done within the field itself!