Will Progress in Applied Health Economics (H.E.) Depend on a New Evaluation Paradigm?

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Evolution of H.E. Evaluation Principles

Comparative Economic Evaluation of Health Care Programs

Traditional Economic Welfare Theory

"The objective of Pareto or potential Pareto efficiency can be achieved by an aggregation of individual utility (preferences or maximum WTP), with individual preferences taken as given."

- **Cost Benefit Analysis**
  - Human Capital Approach
    - Mushkin 1958
    - Weisbrod 1960
  - Value of "Life and Limb"
    - Schelling 1968
    - Mishan 1971
  - Contingent Valuation
    - Developed in environmental economics in the 1960s/1970s
  - Value of Life
    - Jones-Lee 1985
    - Viscusi 1992
  - Health Care
    - Johanneson 1996; but relatively few applications to date
      … for equity concerns
      … for acceptance problems in health care

The 'Extrawelfarist' Proposition

"Maximizing Health Gain Given a Budget Constraint"

- No monetary valuation of human life (at first glance only)
- Health as an independent argument of the utility function
  - Compensation of losers within health care system ("benefits in kind") only
- No contamination of choices (willingness to pay) by ability to pay (marginal utility of money)
  - Considering outcomes equally ("distributive neutrality")

- **Cost Effectiveness (/"Cost Utility") Analysis**
  - Quality-Adjusted Life Years (QALYs)
    - Klarman 1968, Packer 1968
    - Pliskin 1980
  - Additive QALY Aggregation (Maximization)
    - Weinstein and Zeckhauser 1973
    - Culyer, Maynard and Williams 1981
    - Washington Panel 1993 (/1996)
  - Reflections on "Utility"
    - Richardson 1994
    - Kahneman 1997
  - Critique Focused on Methods
    - Utility measurement dependent on instrument (/ poor cross-correlation of index instruments)
    - Linearity assumption and undervaluation of small and short-term health benefits
    - Treatment of comorbid conditions in CEA (and people in "double-jeopardy"?)

Evolution of Health Care Objectives

Core Value Judgments Underlying Health Care Provision

Traditional Values

Shared by Health Care Providers and Policy Makers

- Stated Objectives of Health Care Providers:
  Hippocratic Oath 5th century BC
  AMA Code of Medical Ethics 1847-
  Nightingale Pledge 1893
- Stated Objectives of Health Care Policy Makers and Providers
- Historic Roots – Birth of the Hospital: A Charitable Enterprise – Basil of Caesarea 330
- "Empirical Ethics": Fairness as a Dominant Public Expectation; *cf. also below*
- Rights-Based Legal Traditions (cf. also Dworkin 1977)
- Normative Ethics (Beauchamp and Childress; Rawls, Daniels; Sen…) – but also Harsanyi

Collectively Financed Health Schemes

- Social Health Insurance (SHI-based "Bismarckian" systems)
- National Health Service (NHS) model ("Beveridge-type" systems)
- Cost Containment / Increased Spending Driven by New Technology
  - Goddeeris 1984
  - Newhouse 1992

Evidence-Based Medicine (EBM)

- Cochrane 1972 (/Wennberg and Gittelsohn 1973)
- Eddy, Sacket, Guyatt et al. 1990s

Health Technology Assessments (HTAs) *and 'economic evaluation'*

- U.S. Congress Office of Technology Assessment (OTA) 1975 (-1995)
  - Report "*Development of Medical Technology – Opportunities for Assessment*" (1976)
- Institute of Medicines (IoM) 1970
  - Report "*Assessing Medical Technologies*" (1985)
    "[…] Development of […] technologies has outpaced evaluation of their safety, efficacy, **cost-effectiveness**, and ethical and social consequences."
- Use of HTA in U.S. Managed Care (Blue Cross/Blue Shield (1977/1983)
- ISTAHC 1985 / (HTAi) 2003; INAHTA 1993

Regulatory Use of HTAs *with 'cost utility evaluation'* (i.e., QALYs)

- Australia: Pharmaceutical Benefits Advisory Committee (PBAC) 1992
- Canada: Ontario Ministry of Health 1994
- England and Wales: National Institute for Clinical Excellence (NICE) 1999

Regulatory Use of HTAs *without 'cost utility evaluation'* (i.e., QALYs)

- France: Haute Autorité de Santé (HAS) 2004-
- Germany: Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (IQWiG) 2004 /2007
- USA: Patient-Centered Outcomes Research Institute (PCORI); Patient Protection and Affordable Care Act 2010
- Switzerland: Swiss HTA Consensus 2011 (www.swisshta.ch)

The QALY Maximization Hypothesis

$$ICER = \frac{C_A - C_B}{E_A - E_B} = \frac{\Delta Costs}{\Delta Effects} = \frac{\Delta Costs}{\Delta QALYs} < \lambda \qquad QALYs = \sum_{h=1}^{n} w_h \times t_h$$

where w_h = quality weight (utility index), a preference-based measure reflecting the utility of health-related quality of life in a given health state h, and t_h = time (expressed as number of years) spent in that health state.

Note that the QALY does not represent a measure of health-related utility, but the sum of [health-related] utility adjusted time periods (years, fragments or multiples hereof). This is often referred to as the assumption of "additive separability" – which is a simplification that allows for convenient computation of QALYs, but must be considered as theoretically unsound and empirically falsified,

An additive (quasi-utilitarian) aggregation rule for QALYs (i.e., the assumption that the principal objective of a collectively financed health scheme "ought to be to maximize the aggregate improvement in the health status of the whole community" (Culyer, 1997) is **a necessary requirement for the existence of a universal ICER (cost per QALY) threshold** (often referred to as lambda, λ).

$$Social\_Health\_Gain = \sum_{j=1}^{k} (m_j \times \sum_{t=1}^{n} \frac{\Delta w_{j,t}}{(1+r)^{t-1}})$$

where m = the number of beneficiaries of an intervention j

On actual objectives of collectively financed health schemes, -> cf. "Core Values" -> see above, top of right column.

Some Underlying Issues

Normative Concerns:

There is a fundamental clash between the rights-based legal traditions of Western jurisdictions and the quasi-utilitarian premises underlying the "QALY maximization hypothesis", which is a prerequisite for universal cost per QALY benchmarks.

Empirical Ethics:

In addition to these normative concerns, there is an increasing body of research on societal preferences for health care resource allocation, indicating that the QALY maximization hypothesis must be considered as empirically falsified.

Apparently, social preferences may differ from individual ones, and social value is not identical with the sum of individual "utility" (whether health-related or else).

The broad range 'contextual' variables, which have been documented already now, implies that a uniform 'social value' of a QALY does not exist, and suggests that projects designed to determine the monetary value of a QALY will either fail or mislead policy.

It is therefore predicted here that the future role of conventional cost effectiveness / "cost utility" analysis using QALYs as the presumably universal and comprehensive measure of health outcomes and their "value" will be a much more limited one than currently believed by many scholars. It may be more useful in evaluations of technical efficiency as opposed to allocative decision support.

Context Matters – Some Evidence

Examples of Contextual Factors Influencing "Societal WTP for a QALY"

- Severity of Initial Health State
- Potential for Health Improvement
  (Nord 1993; and many others)
- Dimension of a Health Care Program
  (cf. Orphan Treatments, see Illustration;
  cf. Gafni and Birch, "The Silence of the Lambda")
- Should direct cost be relevant at all?
  (Nord et al. 1995; …)
- As a result, QALY league tables may not pass tests of reflective equilibrium
  (e.g., sildenafil for erectile dysfunction versus many cancer treatments; cf. Stolk et al. 2000)

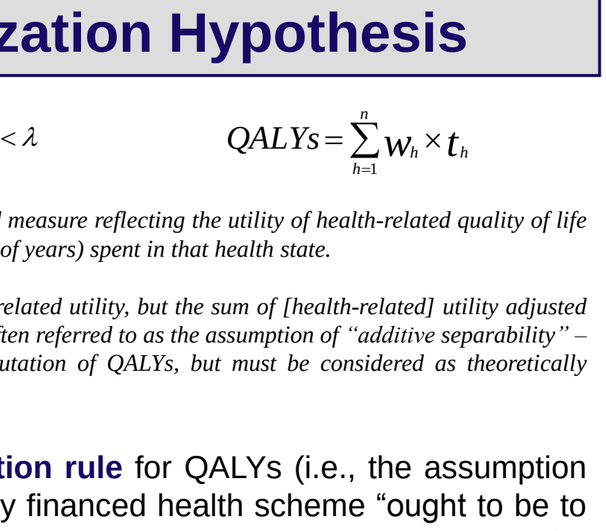

Illustration: Rarity and Cost per Patient
(from Schlander and Beck 2009; Alcimed 2005)

Implications and Future Directions

If economic evaluation is expected to be closer in touch with society's values, there is a need for a new health evaluation paradigm, placing more emphasis on fairness-related considerations.

Hence, there is an urgent need to further explore promising alternative economic evaluation paradigms, such as (but not limited to) the "Relative Social Willingness To Pay" (RS-WTP) approach.

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