



The Evaluation of Economic Methods to Assess the Social Value of Medical Interventions for Ultra-Rare Disorders (URDs)

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Key Challenges for URDs

- **Establishing “Value for Money” (Efficiency)**
 - international heterogeneity in institutional arrangements and established methodologies to determine “value for money”;
 - the still prevailing “logic of cost-effectiveness”, relying on cost per QALY benchmarks, in applied health economics;
 - the broadly held assumption that the social desirability of an intervention would be inversely related to its associated incremental cost per QALY gained;
 - the adoption of “efficiency-first” instead of “fairness-first” evaluation approaches in a number of jurisdictions;
 - the high fixed (i.e., volume-independent) cost of R&D and the need to recoup this investment from a small number of patients during limited periods of market exclusivity;
 - ...



Economic Welfare Theory:

Value & Valuation: Utility

“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: *De la Mesure de l'Utilité des Travaux Publics. Annales des Ponts et Chaussées* 1844; 2: 8. Reprint: *International Economic Papers* 1952, 2: 83-110.

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



¹Jules Dupuit (1844)

De la Mesure de l'Utilité des Travaux Publics. *Annales des Ponts et Chaussées* 1844; 2: 8.

Reprint: *International Economic Papers* 1952, 2: 83-110.



Extrawelfarism: Cost-Effectiveness

→ 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** (some MCOs):
US-\$ 50,000 – US-\$ 100,000 / QALY³
- **Canada** (proposed “grades of recommendation”):
CAN-\$ 20,000 – CAN-\$ 100,000 / QALY⁴

→ No scientific basis

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 Underlying Premise

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

- **Sildenafil** for elderly diabetics with erectile dysfunction and **removal of tattoos** appear to be associated with a relatively (very) low cost per QALY gained,
whereas
- **palliative care**, interventions for people with comorbid conditions (in “**double jeopardy**”, like the disabled) or (very) **rare disorders** appear to be associated with (very) high cost per QALY gained.

**Individual Preferences versus Social Preferences;
Individual Utility versus Social Utility:**

- Do individual preferences map into social utility, i.e., is social WTP simply the sum of individual WTP?
- As to WTP and ATP, what is the appropriate budget constraint?



Vertical versus Horizontal Equity

Rights as Goals:

- “To fail to satisfy people’s basic needs and provide essential skills and opportunities is to leave people without recourse, and people without recourse are not free.”
(A. Sen, 1984; C. Korsgaard, 1993)
- Vertical equity as “positive discrimination” (cf. G. Mooney, 2000)

Relevant Legal Provisions:

- Human Rights Legislation
- Constitutional Provisions (...)
- Nondiscrimination and Rights of Persons with Disabilities
- EU Disability Legislation
- UK Equality Act
- ...



Three Areas of Concern

Normative Reasons for Concern

- (quasi) utilitarian “efficiency-first” framework, based on *individual* preferences, *implying*
- distinct difficulties to incorporate rights-based reasoning.

Empirical Reasons for Concern

- studies overwhelmingly indicate that the majority of people do not wish QALY maximization, *and suggest*
- a wide range of *social* preferences (other than QALY maximization).

Methodological Reasons for Concern

- valuation results (for VSL / QALYs, and for health state utilities alike) differ greatly as a function of the methodology chosen.



Empirical Ethics

The “Sharing Perspective”:

A Broad Range of Social Preferences

- **severity** of the initial health state, i.e., a stable preference to prioritize health care for the worse off;
- **urgency** of the initial health problem, especially if life-threatening, i.e., the so called “rule of rescue”;
- **capacity to benefit** of relatively lower importance, i.e., people appear to value additional health gains lower once a certain minimum effect has been achieved;
- certain **patient attributes** (such as [younger] age, parent or caregiver status, [non] smoker);
- a strong dislike for “**all-or-nothing**” resource allocation decisions;
- **rights**-based considerations (such as nondiscrimination).



How to Evaluate Evaluation Methods:

How well do they capture

- **Normative Premises**, in particular
 - Links to Moral Theory
 - Links to Economic Theory
- **Empirical Preferences** related to
 - Attributes of the Health Condition
 - Attributes of the Persons Afflicted
- **Pragmatic Aspects / Practical Experience** regarding
 - Feasibility
 - Implementation



How to Evaluate Evaluation Methods:

How well do they incorporate / capture

1. Normative Premises

▸ Links to Moral Theory

- pure utilitarianism
- (quasi) act utilitarian (or medical utilitarian) logic
- rule- and non-utilitarian consequentialist variants
- nonconsequentialist claims based (principled) ethics
- discourse ethics (e.g., A4R)

▸ Links to Economic Theory

- formal recognition and reflection of the scarcity condition
- consideration of opportunity costs
- marginal (or incremental) analysis
- perspective(s) of analysis (...)



How to Evaluate Evaluation Methods:

How well do they capture

2. Empirical Preferences

▸ Attributes of the Health Condition

- individual valuation of health conditions
- severity of the condition
- unmet medical need
- urgency of an intervention
- capacity to benefit from an intervention

▸ Attributes of the Persons Afflicted

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



How to Evaluate Evaluation Methods:

How well do they capture

3. Pragmatic Aspects

▸ Feasibility

- practical experience and robustness
- ease of measuring and modeling
(as opposed to analytic complexity and resource intensity)
- possibilities to engage stakeholders
- gaps in our current understanding

▸ Implementation

- validation of criteria, weights, and aggregation algorithm
(as applicable)
- potential for bias and abuse by stakeholders
- addressing the economic realities of R&D (incentives, cost structure, time and risk; second order or dynamic efficiency)