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dkfz. DEUTSCHES
KREBSFORSCHUNGSZENTRUM
IN DER HELMHOLTZ-GEMEINSCHAFT

Forschen für ein Leben ohne Krebs

Panel Session 3:

Determining Value in Health Technology Assessment Consistent with Societal Aims

Professor **Michael Schlander**

Head of Division of Health Economics at German Cancer Research Center (DKFZ) & University of Heidelberg – see www.michaelschlander.com and www.dkfz.de
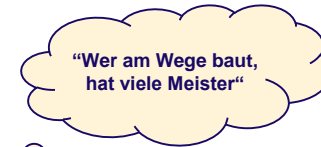
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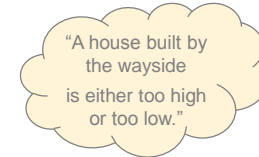GLASGOW, SCOTLAND

The Evolution of Value in Health Care

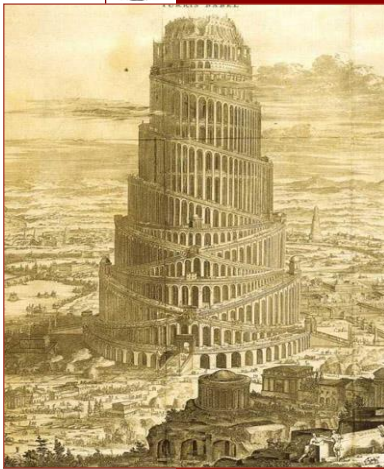ISSUE PANEL – Hall 2 – Tuesday, November 07, 2017 – 14:00 - 15:00

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Determining Value in Health Technology Assessment Consistent with Societal Aims

Health Technology Assessment (HTA)

"Wer am Wege baut, hat viele Meister"

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

Martin Luther (1530)

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"Values Talk" - A Tower of Babel[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)

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

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"So why abandon an extended cost per QALY approach?"[1]

¬ **HTA is here to stay**
  ¬ International practice of HTA has been heterogeneous (in particular w.r.t. the role of economic evaluation)

¬ **Lack of compelling alternatives?**
  ¬ "MCDA may have a role in local decision-making, but still likely to use cost and QALYs"

¬ **Can't measure all things anyway?**
  ¬ Yet formal evaluations need to reflect multiple criteria, in order to minimize degree of "taking into account"

¬ **No single "right method" anyway?**
  ¬ Jurisdictions ["will"] vary on what they value in decision making attributes

[1]J. Brazier (2017)
[presentation to ISPOR Annual European Congress]

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From CUA to [Health-Related] Social "Utility"

$$U = f(H, W, ...)$$
$$U = f(H) + f(W, ...)$$
$$QALYs = \sum_{h=1}^{n} u_h \times t_h$$
$$QALYs = \sum_{t=1}^{n} \frac{u_t}{(1+r)^{t-1}}$$
$$Social\_Health\_Gain = m \times \sum_{t=1}^{n} \frac{\Delta u_t}{(1+r)^{t-1}}$$
$$ICER = \frac{c_A - c_B}{e_A - e_B} = \frac{\Delta costs}{\Delta effects} = \frac{\Delta costs}{\Delta QALYs}$$
$$ICER = \frac{\Delta C}{\Delta E} = \frac{\Delta C}{\Delta QALY} (< \lambda)$$

"The **principal objective** of the **National Health Service** ought to be to maximize the aggregate improvement in the health status of the whole community."[1]

Usual HTA Perspective:
¬ incremental cost *per patient*
¬ health insurance or NHS perspective [sometimes incl. social insurance / PSS / …; controversial: caregiving / productivity loss]

¬ incremental gain in *individual* "utility" (health-related quality of life x length of life)

"The Silence of the Lambda"[2]

"Information Created to Evade Reality"[3]

[1] A.J. Culyer (1997); also M.C. Weinstein and W.B. Stason (1977): *"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."*
[2] A. Gafni, S. Birch (2006)
[3] S. Birch, A. Gafni (2006)

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Increasing Uneasiness with Thresholds

HTA Agencies
¬ NICE (England): end-of-life treatments, ultra-orphans
¬ 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
¬ A "prescription for uncontrolled growth in expenditures"[1]?

Academics
¬ Increasing literature on the importance of "other criteria"
¬ Scientific foundations of actual benchmarks for cost effectiveness: might be too high[2] / too low[3] / non-existent[4]?

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

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Key Elements of the Conventional Logic

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.

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Loopholes of the Conventional Logic

Effectiveness and Efficiency

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.

Efficiency

Need to distinguish explicitly between

¬ technical efficiency, productive efficiency, and allocative efficiency;

¬ static and dynamic efficiency.

Social Value ("Utility")

Existence of

¬ components different from individual utility and its aggregation;

¬ social (and non-selfish) preferences; rights and duties.

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Valuation of Health: A Framing Issue?

1. **Use value** (consumer perspective)
2. **Option value** (due to uncertainty and risk averse citizens)
3. **Externalities** (caring externalities and altruistic behaviors)

Perspective on incremental costs and WTP:

1. direct out-of-pocket payments
2. private (voluntary) health insurance premiums
3. public (compulsory) health insurance premiums (or tax)

$$WTP_{direct\_oop} \leq WTP_{private\_ins} \leq WTP_{public\_tax}$$

¬ But can we expect this additive relationship[1] to be (always) true?

[1] cf. D. Gyrd-Hansen (2013)

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Economic Literature: Preferences for Health

Contingent Valuation (CV) of Health[1]

¬ Smith and Sach identified 265 CV Studies
(published from 1985 – 2005):

  ¬ Focus on **Use Value** of Health only, 73%
  ¬ Focus also on **Option Value**, 13%
  ¬ Focus also on **Externalities**, 5%
  ¬ Focus including **Option Value and Externalities**, 9%

¬ Arguably, **Option Value** and **Externalities** will be most important when access to high technology and/or costly interventions is at stake – *i.e., in practice, when most*

¬ **Health Technology Assessment**s *(HTAs) are conducted*

[1] cf. R.D. Smith, T.C. Sach, *Health Economics, Policy and Law* 2010; 5: 91-111.

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A Rapidly Growing Economic Literature

on a Broad Range of Characteristics[1]

contributing to **Social Value Judgments**

¬ 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

[1]cf., for example, M. Schlander, S. Garattini, S. Holm, et al., *Journal of Comparative Effectives Research* 2014; 3 (4): 399-422.

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Social Preferences in the Economic Literature

"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)

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Research Need: "Social Preferences"

¬ many studies of social preferences …

- ¬ most of them small
- ¬ limited in scope
- ¬ likely to be impaired by framing effects
- ¬ other study types (not choice-based experiments)
- ¬ some studies of questionable methodology

¬ … very difficult to generalize

- ¬ severity probably best documented contextual variable
- ¬ distinct difficulties to quantify effects observed
- ¬ if measures of willingness-to-pay were incorporated, they typically reflected maximal individual WTP
- ¬ social willingness-to-pay in exchange for health care programs covered under a collectively financed health scheme might be more relevant

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ESPM Project: Research Objectives

1. To investigate systematically how the general public valuates selected characteristics ("attributes") of health care interventions,
   - ¬ and how they weigh them against each other (including their interaction).

2. To compare the valuation results obtained in the study with those based on the logic of cost effectiveness by means of a utility comparator.

3. To assess the sensitivity of weights to the level of information offered to respondents and to potential framing effects.

ESPM: "*European Social Preferences Measurement*" **project**; currently, project phase I (SoPHI study: "*Societal Preferences for Health Care Interventions*" in Switzerland is undergoing final evaluations, after completion of qualitative and quantitative pretests and of main DCE survey during 2017.

4. (in Phase II:) To identify international similarities and differences with regard to the valuation of the attributes tested.

5. (in Phase II:) to explore the agreement of respondents between their choices in the experimental setting, their policy implications, and their policy preferences.

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ESPM Project:
Attributes Investigated

1. **Severity** of the initial health state: lost life expectancy
   (i.e., *ex ante*, before / without an intervention)
2. **Severity** of the initial health state: lost quality of life
   (i.e., *ex ante*, before / without an intervention)
3. **Effectiveness** of an intervention: life expectancy gained
4. **Effectiveness** of an intervention: quality of life gained
5. **Age** of patients (or "fair innings")
6. **Rarity** of disorder
   (i.e., prevalence or number of persons benefitting)
7. **Cost** of intervention:
   perspective of a compulsory health scheme ("OKP");
   payment vehicle = social willingness-to-pay

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ESPM Project: Design Elements

1. **Representative population sample**
   ¬ 1,501 respondents from Switzerland in Study Phase I
2. **Discrete Choice Experiment (DCE) design**
3. **Initial Preference Formation Phase**
   ¬ prior to DCE experiment
4. **Testing for framing effects** (by randomization):
   ¬ different levels of information on implications of rarity
   ¬ information on cost per patient (either provided or withheld)
5. **Perspective on costs**:
   ¬ incremental compulsory health insurance premiums
6. **Utility comparator** (with generic health state descriptions)
7. **Econometric evaluation**
   ¬ incl. testing for interaction of attributes;
      subsamples, latent class, and random coefficient models

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From CUA to MCDA and SCVA

SCVA: Social Cost Value Analysis

¬ **Social WTP**
   capturing the will to share health care resources[1]
   (option value and externalities)

Potential attributes influencing the will to share may include

¬ **severity** of the initial health state
¬ certain **patient attributes**
¬ a strong dislike for "**all-or-nothing**" resource allocation decisions
¬ **rights**-based considerations

[1]cf. J. Richardson et al. (2012; 2017); see also E. Nord (2017), and further references

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SCVA: How Different is it from CUA?

**Moving from CUA 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 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' WTP was proportional to the number of patients
   benefitting from the adoption of a health care program.

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SCVA: A Changing Perspective

**shifting the focus
from cost per patient to cost at program level**

¬ A **decision-makers**' (and payers') perspective
has been traditionally 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
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)

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Thank You for Your Attention!

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