Ethical and economical challenges of using CER & HTA for priority setting in health care

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Justice in Modern Health Care – Perspectives for the 21st Century
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Overview

- Background: Why setting limits ("rationing") is unavoidable...
- Theoretical foundations of just health care: The special moral importance of health
- Procedural criteria: Setting limits fairly...
- Substantive criteria: What services should be included in a basic benefit package?
- Perspective: Utility maximization with fairness constraints – balancing cost-effectiveness with other values
  - Instrument: Cost-conscious guidelines (CCGL)
- Questions & Discussion
Biomedical innovations

Demographic change

Limited financial resources

Demand ↑ + Supply ↓

Scarcity of resources in health care
Biomedical innovations  
Demographic change  
Limited financial resources

Demand ↑ + Supply ↓

Scarcity of resources in health care

Strategies?
Biomedical innovations

Demographic change

Limited financial resources

Demand $\uparrow$ +

Supply $\downarrow$

Scarcity of resources in health care

Increase efficiency („Rationalization“)
Biomedical innovations

Demographic change

Limited financial resources

Demand ↑ + Supply ↓

Scarcity of resources in health care

Increase efficiency („Rationalization“)

Increase funding for health care

Limit services („Rationing“)
Intermediate conclusion (1)

- Efficiency can and should be increased, but not enough to compensate the cost pressure by biomedical innovations and demographic change.

- There are convincing ethical (!) arguments to limit public health care spending.

- Setting limits ("rationing") becomes inevitable.

⇒ Challenge: setting limits fairly and efficiently!
Rationing in Germany: Empirical evidence

- BMBF-collaborative research project: representative survey 2008 among 1137 German clinicians from intensive medicine & cardiology, 507 answered (45%)
- Item: *During the last 6 months, how often have you withheld a potentially beneficial intervention from a patient for cost reasons or substituted the intervention by a less effective alternative?*

- Never: 22%
- Less than monthly: 32%
- Monthly: 33%
- Weekly: 11%
- Daily: 2%

Just health (care)

- Function of health care: restore or maintain normal species functioning
- Impairment of normal species functioning through disease and disability restricts an individual's opportunity
  ⇒ Health care promotes **equal opportunity** by preventing and curing disease
- Fair equality of opportunity = a requirement of social justice (John Rawls: “Theory of justice” 1971)
  ⇒ Justice requires universal access to (basic) health care irrespective of ability to pay
  ⇒ Strong ethical argument for a **regulated universal health care system with equitable financing (“solidarity”)**
Setting limits fairly

Trilemma of ethics:

(1) Pluralism of ethical theories of justice/just health care

(2) Limited applicability of most theories
   - Too general to give guidance on concrete allocation decisions

(3) Health care priorities depend on substantial conceptions of the good life \iff\ ideal of neutrality of liberal theories of justice (e.g. Rawls)

→ We cannot infer a concrete hc allocation scheme from an ethical theory of justice or just health care!

→ Fair decision procedures!
  (e.g. “accountability of reasonableness” by Daniels & Sabin)
Health priorities and the good life

Examples
- Health care for the elderly
- Life extending technologies vs. palliative care
- Intensive care for very low birth weight babies
- Prevention vs. acute care
- Infertility services, organ transplantations

Conceptions of the good life determine
- the overall health-care expenditure
- the allocation to different health-care sectors
- the services that are included in a basic benefit package
- what services individual patients demand
Intermediate conclusion (2)

- Ethically legitimate allocation decisions (i.e. setting limits) require

  (1) Fair decision procedures ⇒ *Procedural* ethical allocation criteria

  (2) Good ethical justification ⇒ *Substantive* ethical allocation criteria
Fair procedures: criteria

(1) Transparency
(2) Legitimacy
(3) Consistency
(4) Justification based on relevant reasons
(5) Evidence-based concerning benefits & costs
(6) Participation of relevant stakeholder groups
(7) Minimize conflicts of interest
(8) Revision and appeal mechanisms
(9) Regulation & control (of these conditions)

cf. Daniels & Sabin, Emanuel, et al.
Fair procedures in practice: examples

- Assessment of interventions (HTA) should be procedurally independent of coverage decision
  - E.g. IQWiG vs. G-BA (Federal Joint Commission), NICE vs. DoH

- Explicit democratic legitimization for “rationing” bodies
  - Social code book V - sufficient for G-BA??

- Participation of patient representatives in assessment
  - Importance of different outcomes
  - Quality-of-life assessment

- Transparent data basis and rationale of decisions
  - Stakeholders should have opportunity to review the process and comment on decisions
Who should decide? Physicians` opinions

- If in a health care system not all beneficial services can be covered, physicians should decide **case by case** which patient should get which service.
  - 53% (completely agree + somewhat agree)

- If in a health care system not all beneficial services can be covered, it should be regulated **in general rules** (e.g. positive lists, guidelines) „above“ the individual physician-patient relationship, which services are covered by the statutory health care system.
  - 74% (completely agree + somewhat agree)

- Similar ambivalence in the in-depth interviews!

Substantive allocation criteria (1)

- **Empirical**: Public rationing preferences (e.g. Ubel, Nord)
  - Priority to severely ill patients (even if less cost-effective)
  - No discrimination of people w/ chronic illness / disability
  - Fair distribution of health care services and outcomes
- **Political**: political deliberative process (N, S, NL...)
- **Analytical**: Ethical arguments

⇒ Most appropriate substantial ethical criteria

- Individual **medical need** for the treatment
  - severity of disease; urgency of treatment
- Expected (incremental) **medical benefit** for the patient
- **Cost-benefit ratio**
- Meta criterion: *strength of evidence*
Substantive allocation criteria (2)

- Main Challenge: How much weight shall we assign to the different criteria?
  - Efficiency-equity trade-off
  - Also: Equity-equity trade-off (benefit vs. need)!
  - Trade-off cannot be derived from ethical theory

- Ethically most appropriate: exclude services with
  - Small incremental benefit
  - High incremental costs
  - (if more cost-effective alternative available)

  ⇒ “Utility maximization with fairness constraints”
Ethical justification

- **Justice – population perspective**
  - Limited resources \(\Rightarrow\) take into account opportunity costs
  - Maximize achievable health gain w/ given resources
  - Consider cost-effectiveness of interventions

- **Beneficence – individual perspective**
  - Minimize the benefit withheld from individual patients
  - Alternative treatment should be available

\(\Rightarrow\) Obligation to perform CEA & CUA (cf. NICE, IQWiG)

\(\Rightarrow\) Several methodological challenges, e.g.:
  - assess utilities
  - distributive consequences of the QALY
  - balancing of competing values
# Utilities: comparison of methods

<table>
<thead>
<tr>
<th>Health condition</th>
<th>Rating scale</th>
<th>Standard gamble</th>
<th>Time trade-off</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mild hand pain</td>
<td>0.92</td>
<td>0.91</td>
<td>0.99</td>
</tr>
<tr>
<td>Moderate knee pain</td>
<td>0.63</td>
<td>0.83</td>
<td>0.94</td>
</tr>
<tr>
<td>Severe headache pain</td>
<td>0.37</td>
<td>0.75</td>
<td>0.90</td>
</tr>
</tbody>
</table>

Source: Ubel P, Pricing life. 2000, 54
QALY – distributive consequences

- Health benefits are maximized with the available resources 😊
- Distribution of benefits does not matter 😞
- Severity of disease is neglected
  - 0.1 → 0.2 is equivalent to 0.8 → 0.9?
  - Undervalues life-saving interventions (cf. Oregon)
- Positive or negative age discrimination?
  - Negative: Age ↑ → possible QALY gain ↓
  - Positive: age does not matter
  - 3 QALYs [50 year old] ≈ 9 QALYs [70 year old]
- Discrimination of disabled persons
  - Lower gain of QALYs in comparable conditions
- Advantage for common disorders
Methodological options

(1) **Quantitative integration**
- Incorporate distributional concerns into utility elicitation (e.g. PTO instead of TTO or SG)

(2) **Quantitative transformation**
- Transform “conventional” QALYs (utilities elicited with TTO, SG) to include other values

(3) **Qualitative supplementation**
- CEA/CUA based on conventional QALYs
- Add other values informally in fair (political) decision making process (e.g. NICE)

⇒ (Currently) most feasible, justifiable option: (3)
- Validity of quantitative methods still unclear
- More *transparent* (trade-off not hidden in one figure)
Cost-effectiveness plane

Inefficient ⇒ ethically not acceptable!

Burden of justification increases w/ α!
α big: reject!
α small: accept!

Efficiency gain ⇒ ethically mandated!

With small β ⇒ ethically acceptable!

Cost difference

Cost ↑ Effect ↓

Cost ↓ Effect ↓

Cost ↓ Effect ↑

Cost ↑ Effect ↑

Modified according to Drummond et al. (1997)
Possible ethical justifications:
- Large expected individual benefit
- No alternative intervention available
- High severity of disease
- Innovative character of intervention (potential benefit for future patients)

Example: lysosomal storage diseases (M. Gaucher, Fabry)
- Very expensive enzyme substitute therapies
- High ICER: >400.000£/QALY
- Without treatment: fatal diseases
- Great individual benefit
- No alternative treatment available

Efficiency gain ⇒ ethically mandated!

With small β ⇒ ethically acceptable!

Cost-effectiveness plane

Cost difference
Difference in effectiveness

Efficiency gain

Inefficient ⇒ ethically not acceptable!

Modified according to Drummond et al. (1997)
Cost-conscious guidelines (CCGL)

- Assess effectiveness & cost-effectiveness of medical interventions
- Identify patient subgroups with different incremental benefit & cost-effectiveness
  - Exclude subgroups with no additional net-benefit ⇒ efficiency gain
  - Exclude subgroups with small incremental benefit & high ICER ⇒ limit services with net-benefit (“rationing”)
- Intervention limited to those patients that benefit most!
- Cf. our collaborative research project
  - BMBF-Forschungsverbund “Allokation”
  - Develop & evaluate CCGL for selected cardiologic interventions: ICD & DES
Example: DES vs. BMS

- Basis: NICE guidance TA152
- Main effect: reduced rate of revascularization with DES vs. BMS (5% vs. 10-25%)
- Mortality: No statistically significant difference

<table>
<thead>
<tr>
<th>Price difference DES vs. BMS</th>
<th>400€</th>
<th>800€</th>
</tr>
</thead>
<tbody>
<tr>
<td>All patients</td>
<td>98.000 €/QALY</td>
<td>227.000 €/QALY</td>
</tr>
<tr>
<td>Pts. w/ long lesion (&gt;15 mm)</td>
<td>62.000 €/QALY</td>
<td>167.000 €/QALY</td>
</tr>
<tr>
<td>Pts. w/ small vessel (&lt;3 mm)</td>
<td>33.000 €/QALY</td>
<td>126.000 €/QALY</td>
</tr>
</tbody>
</table>

- Guidance: DES in PCI recommended, if
  - artery has calibre <3mm or lesion longer 15mm and
  - price difference between DES & BMS is < 400€
CCGL: Physicians’ opinions

- CCGL should limit those interventions that provide a **small incremental benefit** to the patient at **comparably high cost**
  - 92% (completely agree + somewhat agree)

- Physicians should **follow** official CCGLs, which limit the use of interventions that provide only a **small incremental benefit** for the patient at **high costs**
  - 78% (completely agree + somewhat agree)

- To guarantee a **consistent and fair** allocation of scarce resources, physicians **should not deviate** from the recommendations in official CCGLs
  - 30% (completely agree + somewhat agree)
Research & policy implications

- Best feasible, justifiable option at the moment:
  - “Qualitative supplementation” (QALY + informal value judgment) ⇒ fair & open decision making process!!
  - HTA should provide information on other values

- Further research required:
  - Conceptualize equity concerns
  - Further develop & evaluate tools to quantify equity concerns

- Comparative evaluation of different decision-making strategies ("policy research")
  - Assess same set of programs with quantitative transformation vs. qualitative supplementation
  - Compare outcomes with different strategies
Questions for further discussions

- What *ethical criteria* (societal preferences) should be applied in addition to the cost-effectiveness/utility ration?
- How can these criteria be *integrated* into the cost-benefit assessment?
  - qualitative vs. quantitative?
- How can we deal with the "*threshold-problem*"?
  - Balancing cost-effectiveness with other values
- How can we organize a *fair decision procedure* to implement the results of CER & HTA in a health care system?
Biomedical progress  
Demographic change  
Limited financial resources

Scarcity of resources in health care

Demand ↑ + Supply ↓

Increase efficiency („Rationalization“)

Increase funding for health care

Limit services („Rationing“)

Charybdis

Scylla
Thank you very much for your attention!

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Increasing demand for hc

- Biomedical & technological progress
  - Product innovations >> process- & organizational innovations
  - Add-on-technologies >> substitute technologies

- Increasing life expectancy
  - Change of disease spectrum
    - chronic & degenerative diseases↑
    - multimorbidity↑
  - Increasing demand for long-term care (e.g. Alzheimer’s disease)

- Especially: Interaction between technological progress and increasing life expectancy

- “Sisyphus-Syndrome”
  - e.g. Japan: highest life-expectancy + highest cancer mortality

- Increasing demand for health care
- Rising health care expenditures
Limited financial resources

- Declining economic growth
  - High unemployment
  - Decreased tax revenues

- Change in age structure of the population (demographic transition)
  - Life expectancy \(\uparrow\) + Birth rates \(\downarrow\) (1.4 in Ge)
    - Aging at the top + aging at the bottom
    - „Double aging“
    - Aging of the population
    - Increasing dependency ratio
      (ratio of working age to dependent population)
    - Increasing financial pressure on public hc systems