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

Georg Marckmann

Ludwig-Maximilians-University of Munich, Germany
Institute of Ethics, History & Theory of Medicine

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

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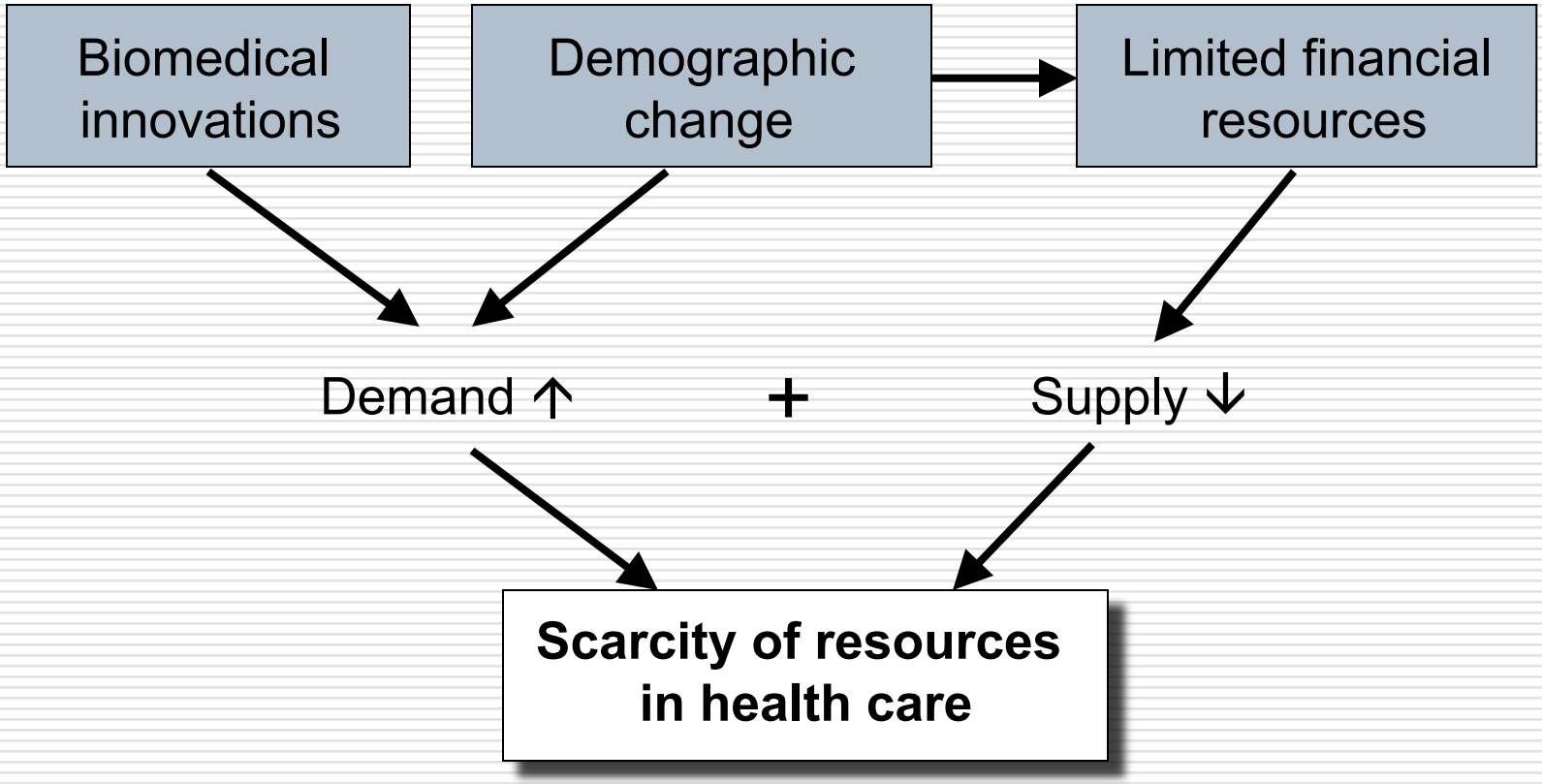
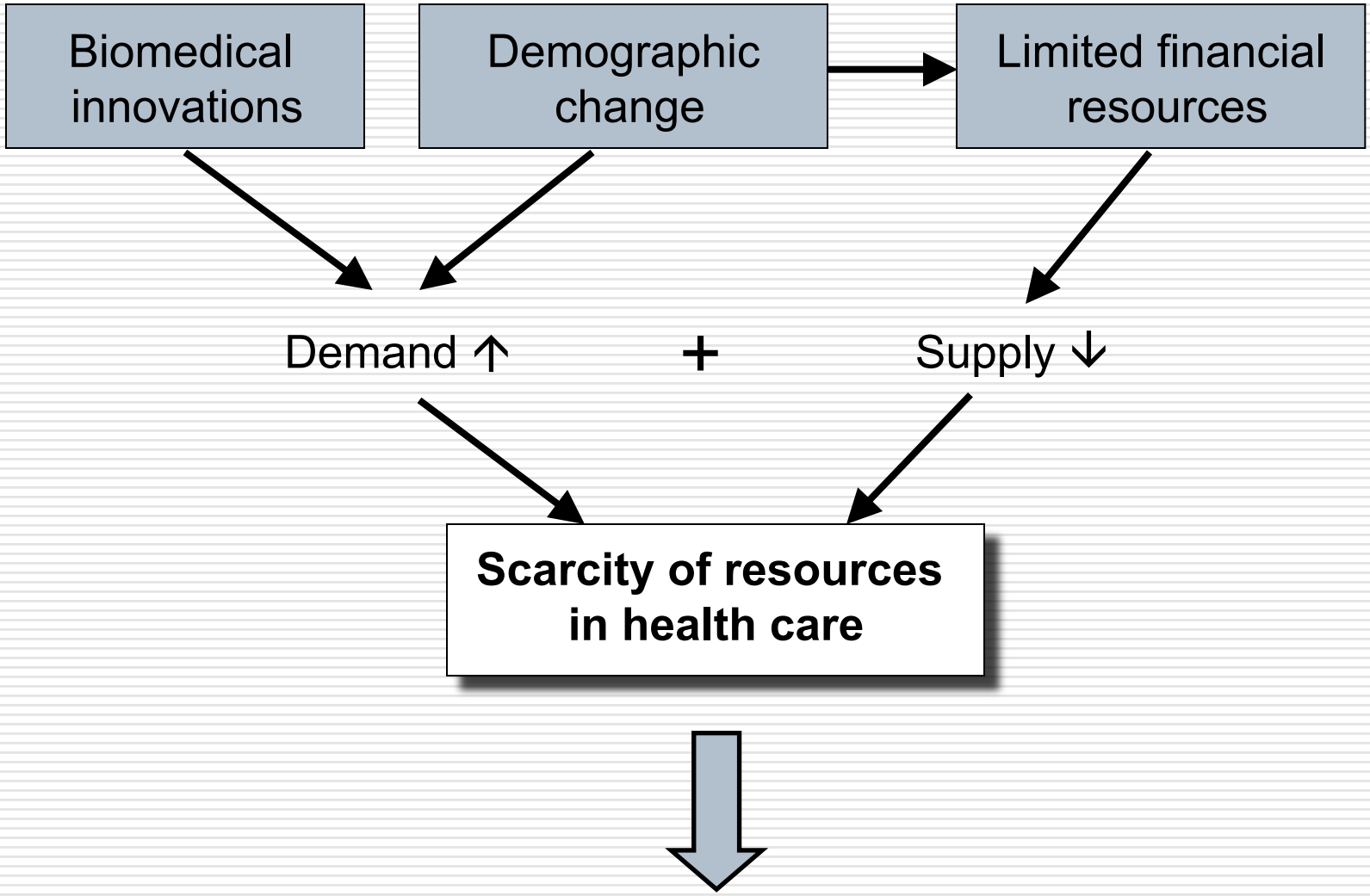
Overview

- 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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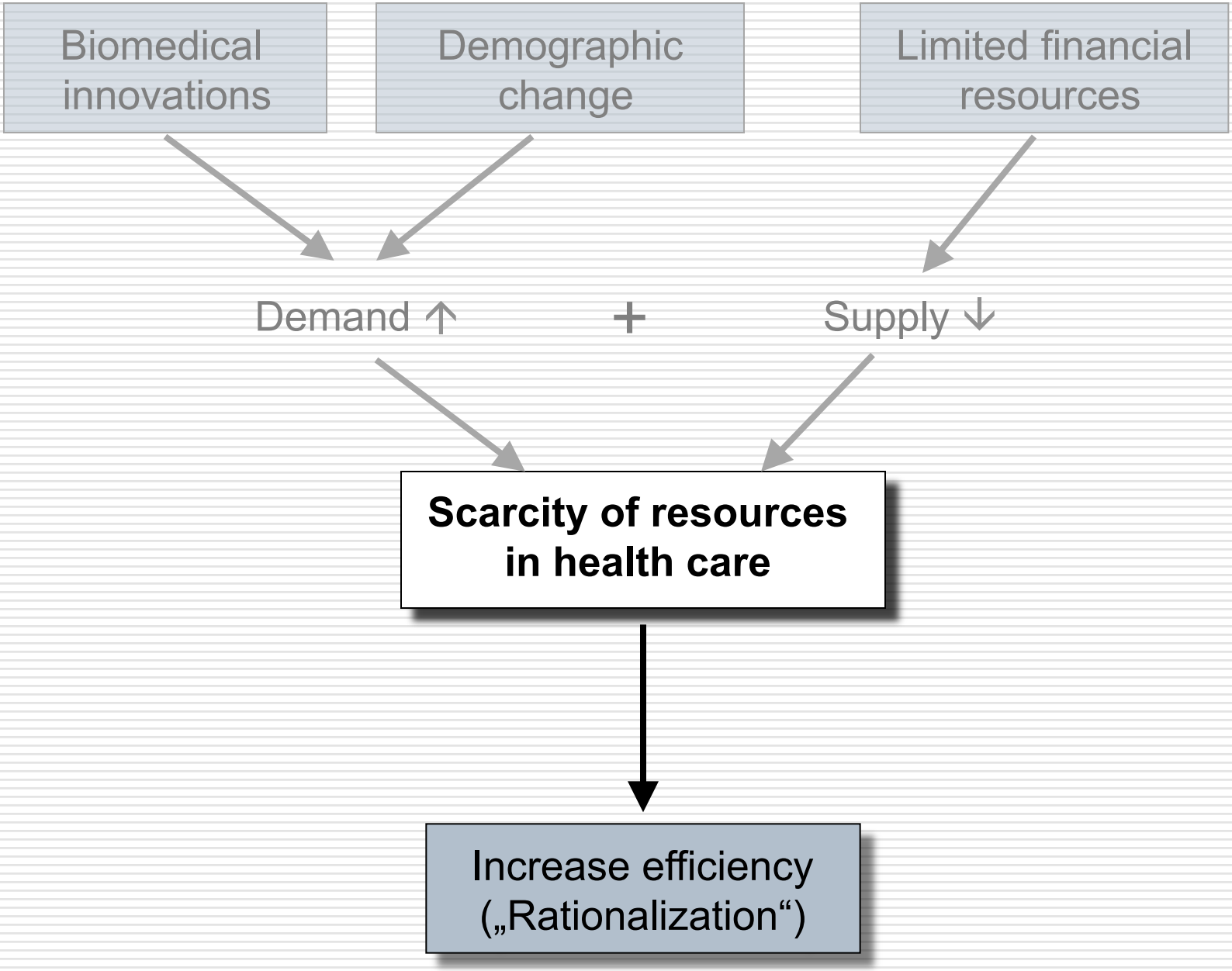
Strategies?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Increase efficiency („Rationalization“)

Biomedical innovations

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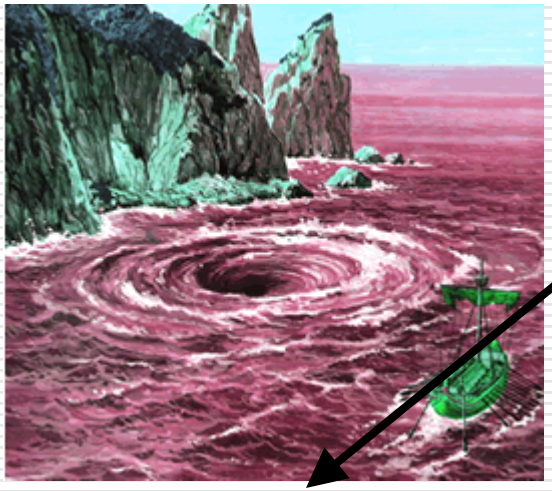
Scarcity of resources in health care

Charybdis



Scylla

Increase funding for health care

Increase efficiency („Rationalization“)

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%

78%

13%

Strech, D. et al. (2009) Ausmaß und Auswirkungen von Rationierung in deutschen Krankenhäusern. **DMW** 2009;134:1-6.

Just health (care)

- Why is health care special? ⇨ Norman Daniels: "Just health care" (1985)/ "Just health" (2008)
- 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* ⇔ 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!

Strech, D. et al. (2009) Ausmaß und Auswirkungen von Rationierung in deutschen Krankenhäusern. **DMW** 2009;134:1-6.

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 ⇨ 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

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

⇨ Several methodological challenges, e.g.:

- ⇨ assess utilities
- ⇨ distributive consequences of the QALY
- ⇨ balancing of competing values

Utilities: comparison of methods

Health condition	Rating scale	Standard gamble	Time trade-off
Mild hand pain	0,92	0,91	0,99
Moderate knee pain	0,63	0,83	0,94
Severe headache pain	0,37	0,75	0,90

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

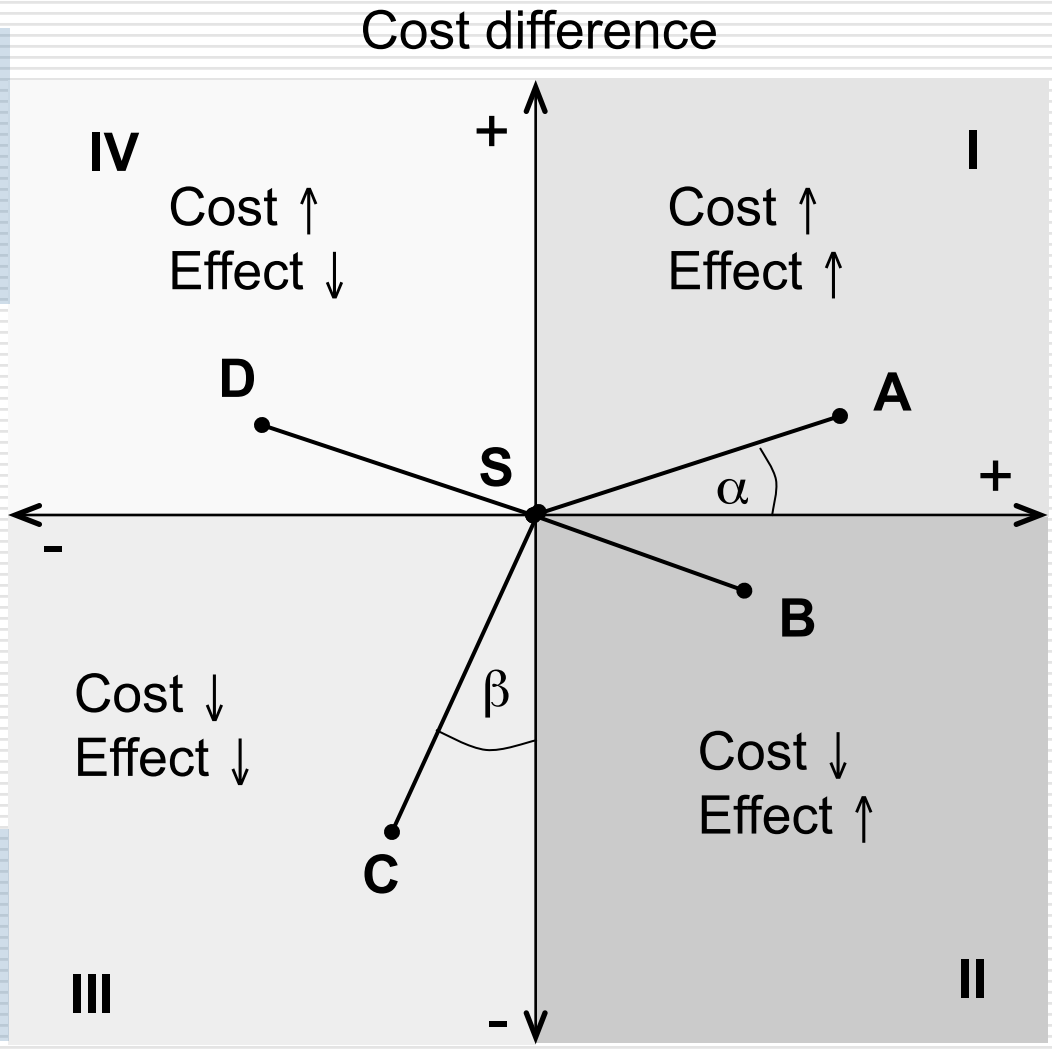
- CEA/CUA based on conventional QALYs
- Add other values informally in fair (political) decision making process (e.g. NICE)

⇨ (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!

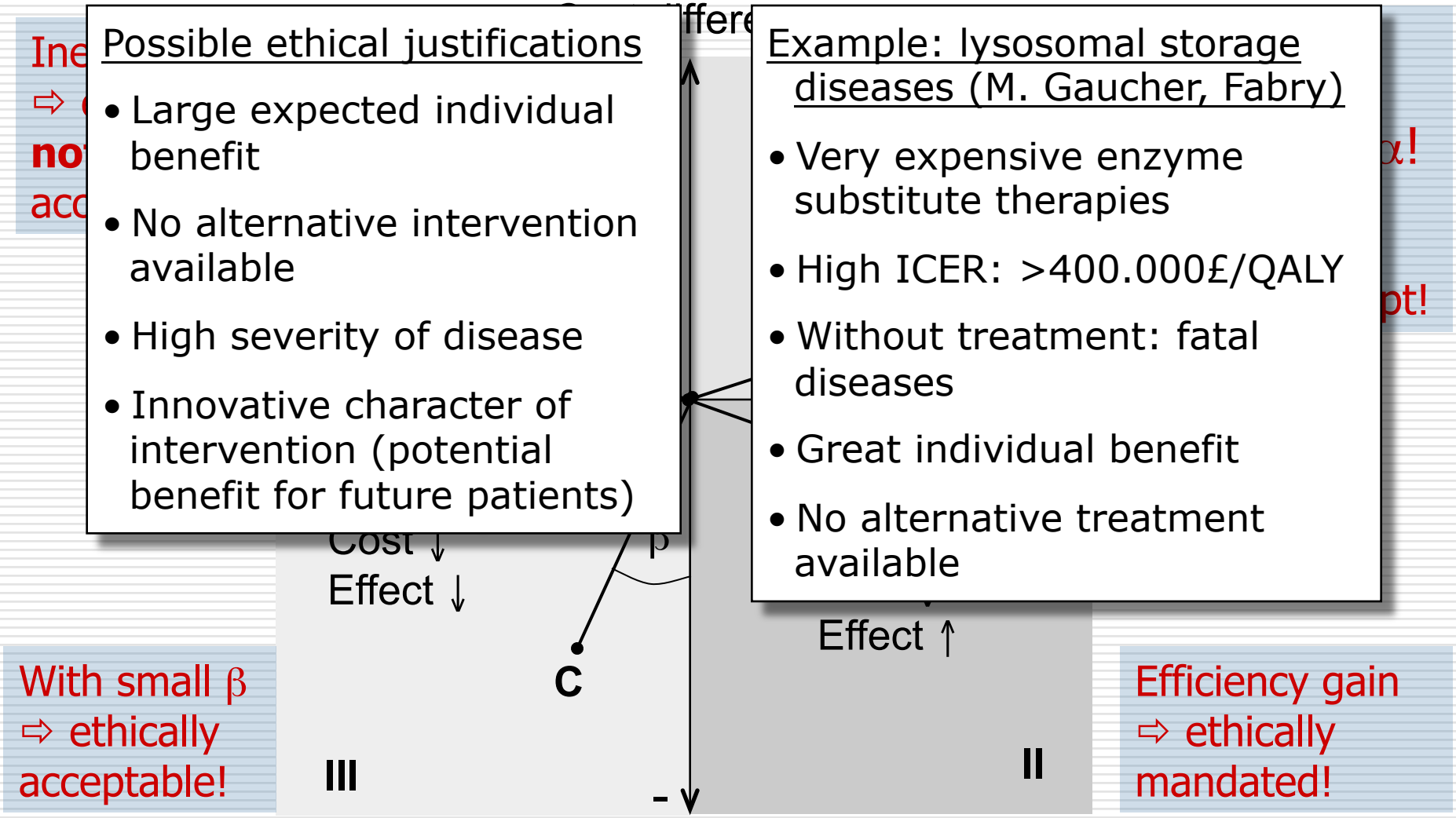
α small: accept!

With small β ⇨ ethically acceptable!

Efficiency gain ⇨ ethically mandated!

Modified according to Drummond et al. (1997)

Cost-effectiveness plane



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

Price difference DES vs. BMS	400€	800€
All patients	98.000 €/QALY	227.000 €/QALY
Pts. w/ long lesion (>15 mm)	62.000 €/QALY	167.000 €/QALY
Pts. w/ small vessel (<3 mm)	33.000 €/QALY	126.000 €/QALY

- 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** form 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

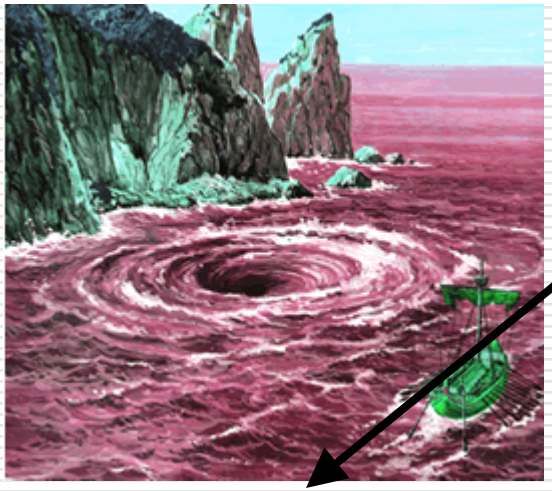
Demographic change

Limited financial resources

Demand ↑ + Supply ↓

Scarcity of resources in health care

Charybdis



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Increase funding for health care

Increase efficiency („Rationalization“)

Limit services („Rationing“)

Thank you very much for your attention!

Contact:
marckmann@lmu.de

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 ↑ + Birth rates ↓ (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