UNIVERSITÄT HEIDELBERG

dkfz. DEUTSCHES KREBSFORSCHUNGSZENTRUM IN DER HELMHOLTZ-GEMEINSCHAFT

Forschen für ein Leben ohne Krebs

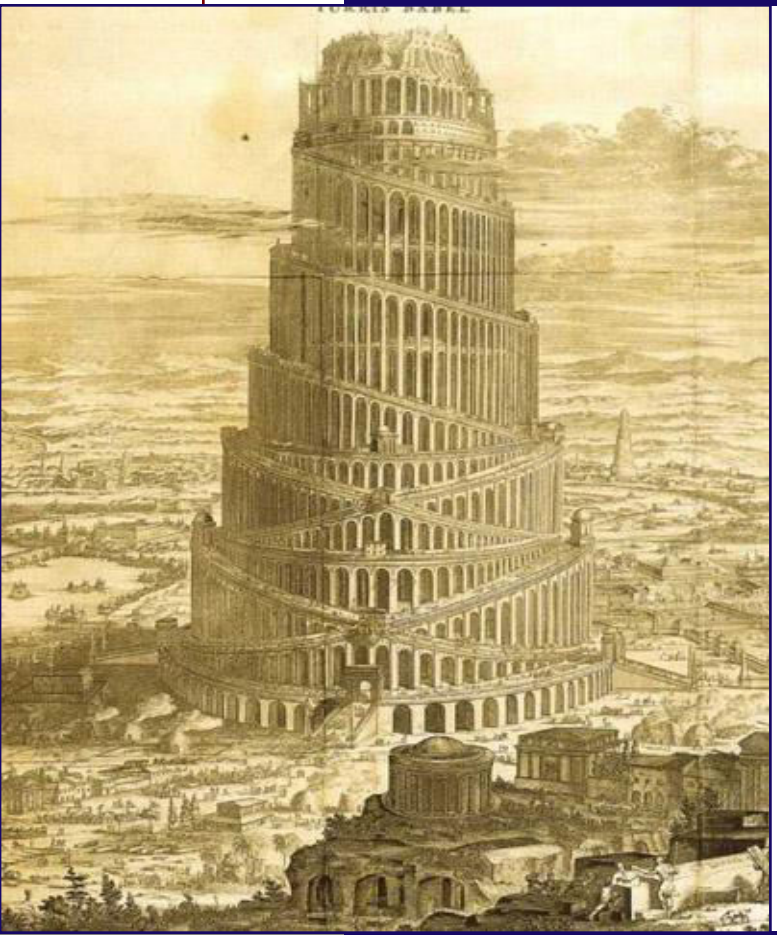
Economical Perspectives in Rare Diseases

"A Paradigm Shift in Value Frameworks for Access"

Moderators:	Michael Schlander (Heidelberg) Mondher Toumi (Aix-Marseille)
Speakers:	Jeff Richardson (Melbourne) Michael Schlander (Heidelberg) Sheela Upadhyaya (London)
Theme Leaders:	Ruediger Gatermann (Marburg) Michael Schlander (Heidelberg)

9th European Conference on Rare Diseases and Orphan Products

Vienna / Austria, May 12, 2018: 09:00 a.m. – 10:30 a.m.



"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)

Orphan drugs and the NHS: should we value rarity?

Christopher McCabe, Karl Claxton, Aki Tsuchiya

The growing number and costs of drugs for rare diseases are straining healthcare budgets. Decisions on funding these treatments need to be made on a sound basis.

[…]

The justification for special status for rare diseases must rest on the question: should we value the health gain to two individuals differently because one individual has a common disorder and the other has a rare disorder?

[…]

While orphan drugs were rare, healthcare systems were able to deal with them in an ad hoc manner. But there are now over 6000 orphan diseases with over 200 treatments approved by the US Food and Drugs Administration and 64 trials currently sponsored by the US Office of Orphan Products Development. […] Genomics is expected to disaggregate currently prevalent diseases into many genetically defined distinct conditions. Orphan status is thus likely to become increasingly common.

[…]

Special status for orphan drugs in resource allocation will avoid difficult and unpopular decisions, but it may impose substantial and increasing costs on the healthcare system. The costs will be borne by other, unknown patients, with more common diseases who will be unable to access effective and cost effective treatment as a result.

British Medical Journal 2005, 331: 1016-1019

Orphan drugs policies: a suitable case for treatment

Michael Drummond, Adrian Towse

A starting point for designing any health policy is to clarify society's views and objectives in relation to the issues concerned.

Although there is scant evidence on what the general public in different countries expect from their health care system, the utilitarian perspective of maximising the total benefits to the population as a whole is a reasonable starting point, particularly in jurisdictions where public financing of health care predominates.

This notion also underpins most of the assessments of value for money conducted in those jurisdictions where these are explicitly required. Namely, the implicit or explicit objective is to maximise the total health gain from the use of health care resources, although the methods for measuring health gain vary from jurisdiction to jurisdiction.

However, since orphan drugs are never as cost-effective as drugs for more prevalent diseases, **departures from a strict utilitarian perspective would have to be justified** if they were to be funded. That is, society would have to be willing to give up some of the health gain to the population as a whole.

European Journal of Health Economics 2014, 15: 335-340

"Departures from a strict utilitarian perspective would have to justified…"[1]

- **John Stuart Mill (1806-1873):**

  "What is best brings the greatest good for the greatest number"

- **Jeremy Bentham (1748-1832):**

  "The greatest happiness of all those whose interest is in question is the right and proper, and the only right and proper and universally desirable, end of human action."

Medical Utilitarianism

- A variant of act utilitarian thought, exclusively focusing on individual health outcomes (usually *QALYs*), and their maximization

[1]M. Drummond, A. Towse, *European Journal of Health Economics 2014, 15: 335-340*

The Underlying Premise

"A QALY is a QALY is a QALY – regardless of who gains and who loses it."[1]

"The principal objective of the *National Health Service* ought to be to maximize the aggregate improvement in the health status of the whole community."[2]

"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."[3]

[1] D. Feeney and G.W. Torrance (1989)

[2] Anthony J. Culyer (1997)

[3] M.C. Weinstein and W.B. Stason (1977)

Key Assumptions of the Conventional Logic

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…").

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.

Objectives of Collectively Financed Health Care

Maximizing the satisfaction of individual (selfish?) preferences?

- Utility?
- Well-being?
- Health?

Maximizing the number of QALYs "produced"?

- The assumption has been shown to be "descriptively flawed"

What about social / non-selfish preferences?

- Normative ethics?
- Empirical ethics?