# Improving the Usefulness of Health Economic Evaluations for HTA Decision Makers

## Social Value and the Case of Interventions for Rare and Ultra-Rare Disorders (URDs)

Panel Presentation to 12th HTAi Annual Meeting

Deborah Marshall

Oslo, June 17, 2015 – 09:00 am - 10:30 am

Radisson Blu Plaza Hotel – 33th Floor – Room Oslofjord

#HTAiOslo2015

OSLO HTAi 2015
12th Annual Meeting

# Background

## Treatments for Rare and Ultra-Rare Disorders (URDs): Some of "The Most Expensive Drugs in the World"

1. **Eculizumab** (Soliris®)
   paroxysmal nocturnal hemoglobinuria (PNH),
   atypical hemolytic uremic syndrome (aHUS);
   estimated annual drug acquisition cost (U.S.[1]): **US-$ 409,500**
2. **Idursulfase** (Elaprase®)
   Hunter syndrome (ERT); **US-$ 375,000** p.a.
3. **Galsulfase** (Naglazyme®)
   mucopolysaccharidosis (MPS) VI (ERT); **US-$ 365,000** p.a.
4. **Human C1 Esterase Inhibitor** (Cinryze®)
   hereditary angioedema (HAE); **US-$ 350,000** p.a.
5. **Alglucosidase alfa** (Myozyme®)
   Pompe disease (ERT); **US-$ 300,000** p.a.

[1]S. Williams, The Motley Fool, June 29, 2013.
http://www.fool.com/investing/general...
[last accessed Jan. 22, 2014]

# Background & Objectives

- **URD Project: Three International Expert Workshops** *(to date)*
  - in Berlin / Germany, November 08, 2012,
    in Dublin / Ireland, November 07, 2013,
    in Amsterdam / The Netherlands, November 13, 2014
  - supported by three biopharmaceutical companies[1]
    under an unrestricted educational grant policy
- **Agreement (Expert Consensus[2])**
  - on scope of project and prioritization of issues to be addressed
  - on issues underlying the failure of (many) URD treatments
    to meet conventional standards of cost effectiveness
  - on the need for (improved or) alternative evaluation methods
  - on promising ways forward, overcoming
    the shortcomings of the currently prevailing evaluation paradigm

[1]BioMarin (since 2012);
Genzyme (since 2013;);
Alexion (in 2012)
[2]available onlne at www.innoval-hc.com

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# Objectives & Approach

- **Approach Chosen (Method)**
  - open exchange of views under the Chatham House Rule
  - initial consensus statement of 2013
    finalized subsequent to the first workshop in an iterative process
- **Subject of Analysis**
  - technologies targeting ultra-rare disorders (URDs),
    excluding cancer and personalized medicine
  - URDs under consideration should be
    - severe,
    - chronic,
    - represent clearly defined biological entities (i.e., are not created by artificial “slicing” of a biologically much broader and more prevalent indication),
    - are associated with a broadly accepted high unmet medical need

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# Objectives & Approach

## ¬ Situation Analysis

- ¬ The expert group agreed to begin with a comprehensive review of the current situation and challenges.
- ¬ The group agreed to initially focus on a high-level analysis (1, below), and to move on from there top-down in a hierarchical process:

## ¬ Levels of Analysis

1. **principles underlying the current evaluation framework**
2. actual evaluation policies implemented by HTA agencies and regulatory bodies (primarily those concerned with pricing and reimbursement decisions)
3. evaluation practice when principles and policies are applied to real-world problems. – In particular, the third level of analysis would have to include case studies, including cases where existing regulation has been potentially misused.

# The "URD Project" Group

- **Silvio Garattini** (Mario Negri Institute, Milan / Italy)
- **Søren Holm**[1] (U of Manchester / England)
- **Peter Kolominsky-Rabas** (U of Erlangen / Germany)
- **Deborah Marshall**[1] (U of Calgary, AB / Canada)
- **Erik Nord**[1] (U of Oslo / Norway)
- **Ulf Persson** (IHE, Lund / Sweden)
- **Maarten Postma** (U of Groningen / The Netherlands)
- **Jeffrey Richardson**[1] (Monash U, Melbourne, Vic / Australia)
- **Michael Schlander**[1] (InnoVal$^{HC}$ & U of Heidelberg / Germany)
- **Steven Simoens** (U of Leuven / Belgium)
- **Oriol de Sola-Morales** (IISPV, Barcelona / Spain)
- **Keith Tolley** (Tolley HE, Buxton / England)
- **Mondher Toumi** (U of Lyon / France)

[1]presenting on behalf of International Expert Group

# Panel Overview

- **Deborah Marshall**: **Introduction**
  [09:00 – 09:05]
- **Michael Schlander** on **The Logic of Cost Effectiveness**
  [09:05 – 09:15]
- **Søren Holm** on **A Normative Perspective – Social Preferences**
  [09:15 – 09:30]
- **Erik Nord** on **An Empirical Perspective – Severity and Rarity**
  [09:30 – 09:45]
- **Jeffrey Richardson** on **The Need for a Revised Framework**
  [09:45 – 10:00]
- **Deborah Marshall** moderatoring **Discussion with Audience**
  [10:00 – 10:25]
- **Michael Schlander** on **Which Way Forward?**
  [10:25 – 10:30]

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# Questions to be Addressed Today

- Do Conventional Health Economic Evaluations Capture the Full Social Value of Interventions (*not only for URDs*)?
- Social Preferences
  - What Are They and Why Should They Matter?
  - What About Concerns for Severity and Rarity?
  - What About a Sharing Perspective?
- What Might Be the Implications for Applied Health Economics and HTA?

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# The Social Value of Interventions for Ultra-Rare Disorders and the Logic of Cost Effectiveness

[The Need for Alternative Methods to Evaluate Medical Interventions for Ultra-Rare Disorders]

**Michael Schlander**

INNOVAL HC
Institute for Innovation & Valuation in Health Care

**HTAi 2015: 12th Annual Meeting, Oslo / Norway, June 14-17, 2015**

& Mannheimer Institut für Public Health – www.miph.uni-hd.de

# The 5 Most Expensive Drugs in the World[1]

1. **Eculizumab (Soliris®)**
   paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS); estimated drug acquisition cost (in U.S.[1]): **US-$ 409,500** p.a.
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## 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]

## Utilitarian Thought

- **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."

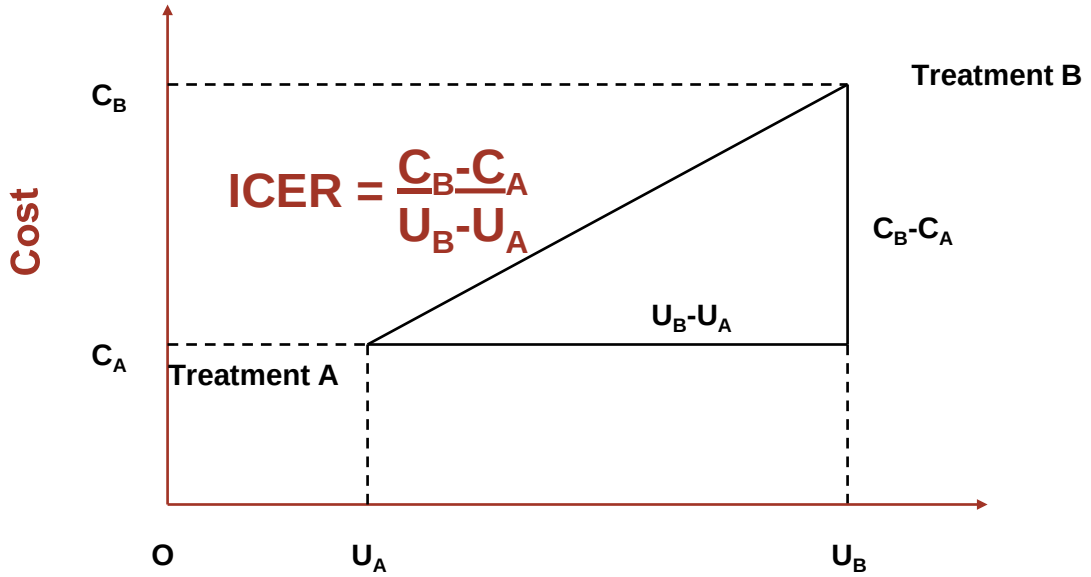
## Medical Utilitarianism

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

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

# The Logic of Cost-Effectiveness

## Incremental Analysis




Note: Quality-Adjusted Life Years (QALYs) are not a measure of [health-related] utility!

**ICER: *Incremental Cost-Effectiveness Ratio***

*or:* ***"Information Created to Evade Reality"?***[1]

[1]S. Birch, A. Gafni: Information created to evade reality (ICER): things we should not look to for answers. *PharmacoEconomics* 2006: 24: 1121-1131

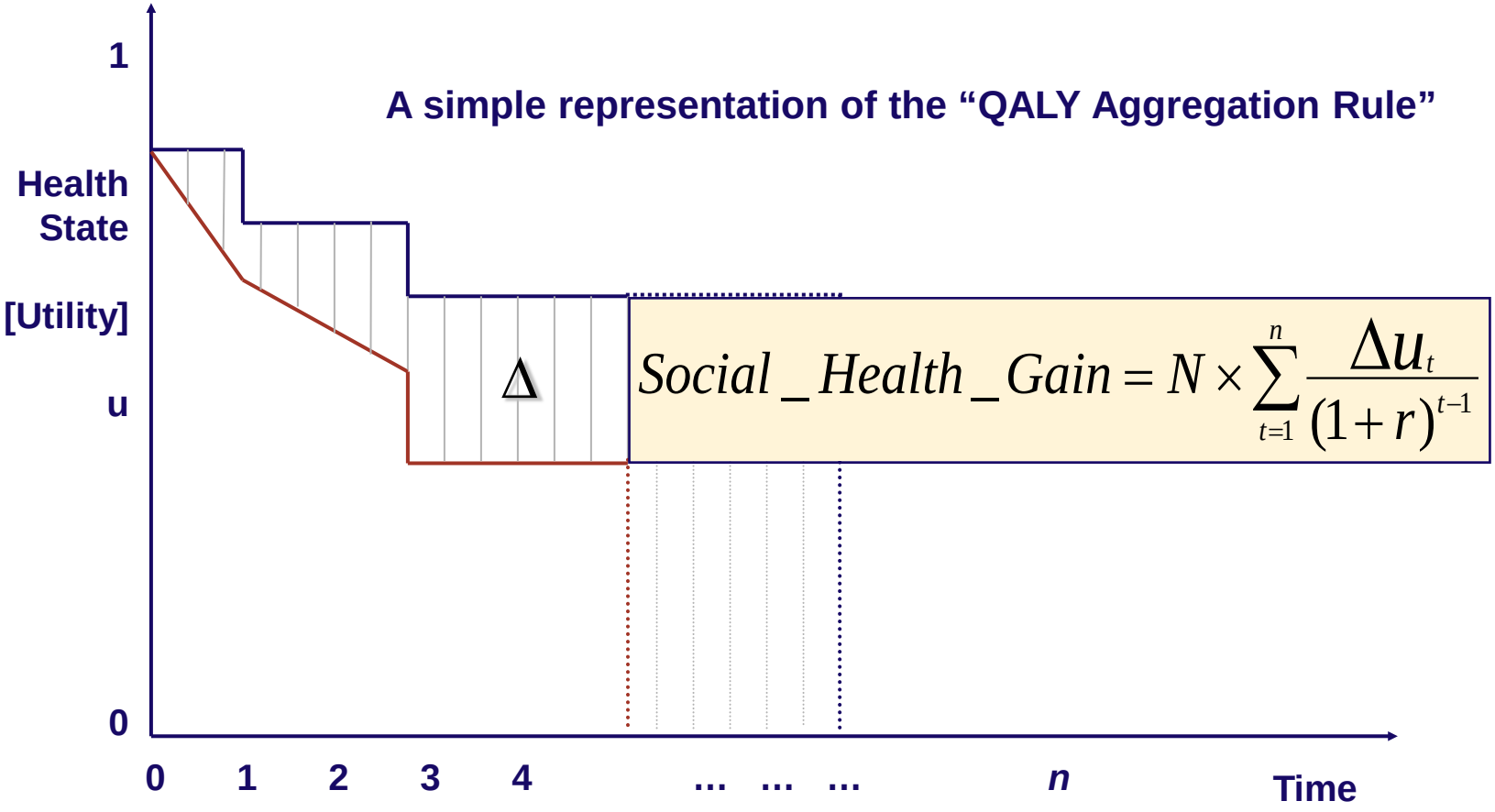
# The Logic of Cost-Effectiveness

## The Cost-Effectiveness Decision Rule:

$$ICER = \frac{\Delta C}{\Delta E} = \frac{\Delta C}{\Delta QALY} < \lambda$$

Note that the size of numerator and denominator will cancel out.

# Social Value as Sum Total of QALYs Gained



# 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.

# A Fundamental Premise

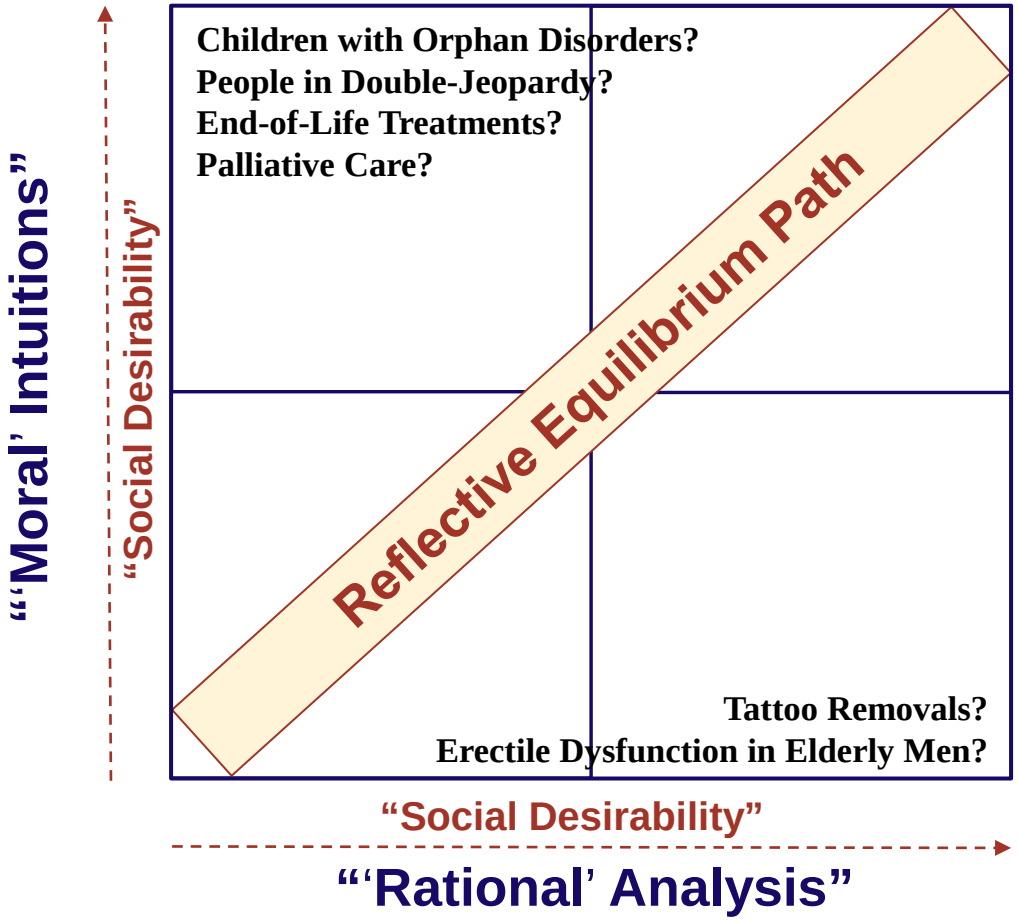
**"Social Desirability of an Intervention is Inversely Related to its Incremental Cost per QALY Gained"**

but this assumption may create **Reflective Equilibrium** issues:

- Sildenafil for elderly diabetics with erectile dysfunction
- Removal of Tattoos

  *compared to*
- Palliative Care,
- Interventions for people with comorbid conditions (in "Double Jeopardy", like the chronically disabled)
- Orphan Medicinal Products (OMPs) for (very) rare disorders

# Reflective Equilibrium



# Economic "Efficiency" (1)

| Effectiveness | Efficiency |
| --- | --- |
| **Realized Output** / **Intended Output** | [Realized] **Output** / [Realized] **Input** |
| (Value[s], Objective[s]) | (By definition, efficiency is a secondary objective) |

# Economic "Efficiency" (2)

| Technical Efficiency | Allocative Efficiency |
|---|---|
| ¬ **Ability to produce the maximum possible output from a given set of inputs** | ¬ **Choosing the most cost-effective set of programs for the given level of expenditure** (i.e.,optimal choice of input proportions, given their respective prices) |
| ¬ **Does not routinely imply choosing between different patient (group)s** – *hence individual persons* | ¬ **Does imply allocating resources across different patient (group)s** – *hence individual persons* |

# Efficient Allocation & Strict Act Utilitarian Logic

## Case 1:

| | $U_1$ | $U_2$ | $U_3$ | $U_{tot}$ |
|---|---|---|---|---|
| $A_1$ | +6 | +8 | +6 | **+20** |
| $A_2$ | +7 | +9 | +2 | **+18** |
| $A_3$ | +2 | +3 | +12 | **+17** |

### Assumptions:

- Utility can be measured and quantified.
- Measured values can be compared meaningfully.

## Case 2:

| | $U_1$ | $U_2$ | $U_3$ | $U_{tot}$ |
|---|---|---|---|---|
| $A_1$ | +28 | +28 | -30 | **+26** |
| $A_2$ | +2 | +9 | +14 | **+25** |
| $A_3$ | +8 | +8 | +8 | **+24** |

### Problem:

- Distribution is ignored.
- Act utilitarianism even will defend negative utilities for some.

Note that conventional cost benefit analysis assumes that the “winners“ may compensate the “losers” so that after compensation nobody loses.

# An Alternative Premise

"**Right of Access**:

An individual suffering from a rare disease has the same **right** to the necessary treatments and medication as someone with a more common disease."[1]

[1]**European Charter of Patients' Rights (Rome, 2002)**

# 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" (G. Mooney, 2000)

## Relevant Legal Provisions:

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

# "Social Preferences" – Non-Selfish Motives

A person exhibits social preferences if the person not only cares about the material resources allocated to her but also cares about the material resources allocated to relevant reference agents.[1]

In addition to material self-interest, these are

- **Reciprocity or Reciprocal Fairness**
  with fairness being determined by the equitability of the payoff distribution (relative to the set of feasible payoff distributions)
- **Inequity Aversion**
  resulting in altruism or envy towards other people
- **Pure Altruism**
  a form of unconditional kindness
- **Spiteful or Envious Preferences**
  *always* valuing a payoff of relevant reference agents negatively

Note heterogeneity of motives at the individual level.

[1] cf. E. Fehr and U. Fischbacher (2002)

# Sources of Social Value

How should we address

- **Prior Normative Commitments**, in particular
  - with regard to Moral Theory
  - with regard to Economic Theory
- **Empirical "Social" Preferences** related to
  - Priorities related to Attributes of the Health Condition
  - Priorities related to Attributes of the Persons Afflicted
- **Pragmatic Aspects / Practical Experience** regarding
  - Feasibility
  - Implementation

# Social norms and preferences – Why should they matter?

Søren Holm

Manchester & Oslo & Aalborg

# Outline

What is a social preference?

Different types of social preferences

Possible restrictions on social preferences

The normative importance of social preferences for health economics and policy-making

*Background – treatments for URD*

# What is a social preference?

Initial definition:

A preference held by an individual A for a particular social arrangement in a specific social context and area S

and/or

a preference for particular weight being given to a social outcome

But, note that the ambiguity of 'social' as a descriptor merely moves from 'preference' to 'arrangement' and 'outcome' if we choose the second definition

Economists use 'social preference' with a very wide scope, including 'fairness' preferences in one-off two person games

# Different types of social preferences 1

Does 'social' map on to the philosophical self- v other-regarding distinction in preference consequentialism?

I.e. are social preferences identical with other-regarding preferences (preferences regarding outcomes for other people)?

Not completely. Some other-regarding preferences are individualised and not social in any meaningful way.

A further problem is that some social preferences may be both self- and other-regarding at the same time (e.g. a preference for a public health care system).

But, never the less for something to count as a social preference it must be significantly other-regarding (or general).

# Different types 2

- Structural preferences
  - Distributive preferences (for many different kinds of resources)
    - Preferences for fairness
  - Preferences for specific structural outcomes
- Preferences for non-structural outcomes

# Restrictions? Should only some preferences count?

Anonymity:

Should social preferences be 'anonymous' in order to count as social preferences, i.e. not refer directly or indirectly to identifiable individuals?

Or is it sufficient that they are general, i.e. would be held irrespective of who the individuals are?

Non-discrimination / fanaticism:

Should social preferences only count if they are not discriminatory or fanatical?

Rationality:

Should social preferences only count if they are rational?

Perhaps, but according to what account of rationality?

# Why and when do social preferences matter?

They matter to preference consequentialists if they are 1. satisfiable, 2. not fanatical or perverse, and 3. not ruled out by restrictions on other-regarding preferences; simply by the fact that they are preferences that are of equal status to any other preference

They may matter to other philosophers as well

They should matter to policy makers for a variety of reasons:

- In contexts where preference satisfaction is thought to matter independently
- In contexts where (the perception of) preference satisfaction is necessary for the function and/or sustainability of a particular social arrangement
- In contexts where common resources are being distributed, or where the state acts as an intermediary in a social arrangement

# Why should they matter 2

They should matter to economists because we know that people have such preferences and that they continue to hold them even after reflection.

In so far as economic analysis is about working out how economic agents can maximise the satisfaction of the preferences they **actually have** then social preferences should matter.

They should also matter because we cannot accurately model and predict behaviour if we ignore them.

Note, that the 'fact' that some social preferences are inconsistent with other preferences held by the same person is not sufficient to discard them, if the person continues to hold both set of preferences after reflection

# Implications for URD ressource allocation

- Public health care systems are the type of social context where social preferences should matter
- Need to identify the relevant social preferences
  - Rarity
  - Unmet (desperate?) need
    - Only option
  - Ameliorating tragic situations
  - Family impact
  - *De minimis* budgetary impact

# Take home messages

- Social preferences are ubiquitous
- Understanding and incorporating social preferences is important for both health economics and for decision makers
- We need more and better research into what social preferences people actually hold in relation to URDs (*and these may vary from country to country*)

# Availability of orphan drugs: Background, ethics and societal preferences.

Erik Nord

Senior Researcher, Norwegian Institute of Public Health,

Professor of Health Economics, University of Oslo

# Issue 1: Rare diseases and R&D - rationality and fairness

Conventional explanation: Lack of market incentives.

Deeper reason: Rational use of societal resources:

If pharmaceutical R&D were organised and paid by governments, the results would be much the same.

Cfr the intended use of Burden of Disease (DALYs): Priority to the biggest problems.

Is it unfair?

It is always bad luck to be born with rare needs.

Nevertheless we feel compassion and a moral obligation to help.

# Issue 2: Incorporation of general concerns for fairness in economic evaluation may particularly help those with rare diseases

Daniels, Harris, Brock, Menzel, Culyer, Loomes & McKenzie 1985-1990.

Nord & Richardson 1991-2014.

1999-2000: Main papers in Health Economics, Medical Care and Hastings Center Report with Paul Menzel, Jose-Luis Pinto and Peter Ubel.

=> (Social) Cost-Value Analysis (CVA).

Based on theories of justice, government guidelines for priority setting, and preference studies.

# CVA 1: Emphasis on severity (50 Grades of Pay)

Severity as proportional shortfall (PS).

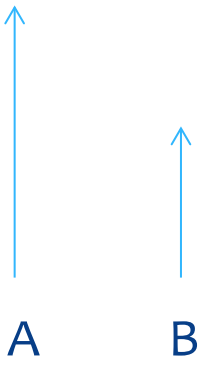
Review of preference studies (Nord&Johansen, Health Policy 2014).

Tentative gradient of willingness to pay for a QALY by PS:

| PS (%) | 10 | 20 | 30 | 40 | 50 | 60 | 70 | 80 | 90 |
|---|---|---|---|---|---|---|---|---|---|
| NOK (1000) | 200 | 300 | 400 | 500 | 600 | 700 | 800 | 900 | 1.000 |

Rare diseases are often severe and will tend to profit from such a graded willingness to pay.

# CVA 2: Realising potentials



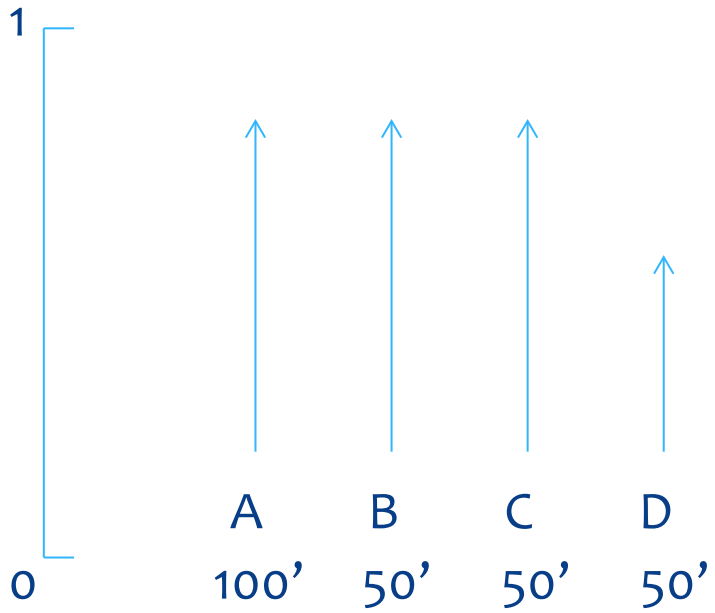

Proposition: When severity is the same, and both can be significantly helped, B has the same moral claim as A.

Norway 1987.

Cfr the rejection of cost/QALY in Germany.

Norheim et al, 'Equity Check List', JCERA 2014.

# Realising potentials judged by strength of interest




Who has an interest in giving priority to C over D?

Bottom line: If drugs for rare diseases often offer only partial cure, the ethic of realising potentials will tend to increase the value of QALYs obtained in rare diseases.

# Issue 3: Possible arguments for special treatment of orphan drugs

When severity and benefit are equal, how can we argue in favor of greater willingness to accept costs for orphan drugs than for other drugs?

1 Rarity does not have value per se.

2 Unfair to restrict access just because a person happens to belong to a small group (and therefore is costly).

But we do restrict access for people who happen to be costly for other reasons (for instance complexity of disease).

The problem: How can we justify to pay 200 000 euro per QALY for rare diseases and deny treatment for a common disease at 100 000 euro per QALY?

# Possible arguments, continued

3 *'Winners take all' / 'Some get nothing' = bad.*

*Some degree of sharing is desirable.*

a. Per se (deontological reason).

b. A minimum of sharing provides hope (consequentialist reason), and hope is a source of value.

Preference data:

Nord and Richardson, 1995.

Richardson et al, 2012.

# Issue 4: Preference studies: Some alarming lessons

Complex issue, difficult for most people:

Desser et al, BMJ 2010. How to spend a given sum of money?

100 rare vs 100 common, equal cost per patient: 65 % indifferent.

Cost of rare = 4 x cost of common: 47 % still indifferent.

=> inconsistent.

## … but also difficult for researchers:

Mentzakis et al, 2011. DCE. 'Other things equal, higher WTP for rare?

Which of two drugs to list? => The drug for rare would target fewer patients.

So other things were not equal. => Not about rarity per se.

Also: Prioritising between patients vs between research programs.

# A pragmatic point about visibility

In the absence of clear theoretical justifications for accepting higher costs per QALY:

Easier to support R&D than to increase WTP/QALY because of lower visibility of the action (=> less envy/conflict).

# Conclusions

General concerns for equity/fairness (severity, realising potentials) are well documented in preference studies and consistent with theories of justice.

Systematic incorporation of such concerns in formal econonomic evaluation will tend to advantage people with rare diseases.

Regarding societal willingness to stretch cost acceptance per QALY for orphan drugs in particular:

Thinking fast (and compassionately): Yes!

Thinking slow: Difficult to find a clear theoretical justification.

Further studies of population views would nevertheless be of interest.

Need for quality assurance of preference studies.

- Little information value if respondents are only asked to make choices.
- Deliberations in focus groups.

MONASH University

Business and Economics

# Health Technology Assessment International

2015 Annual Meeting, 14-17 June Oslo Norway

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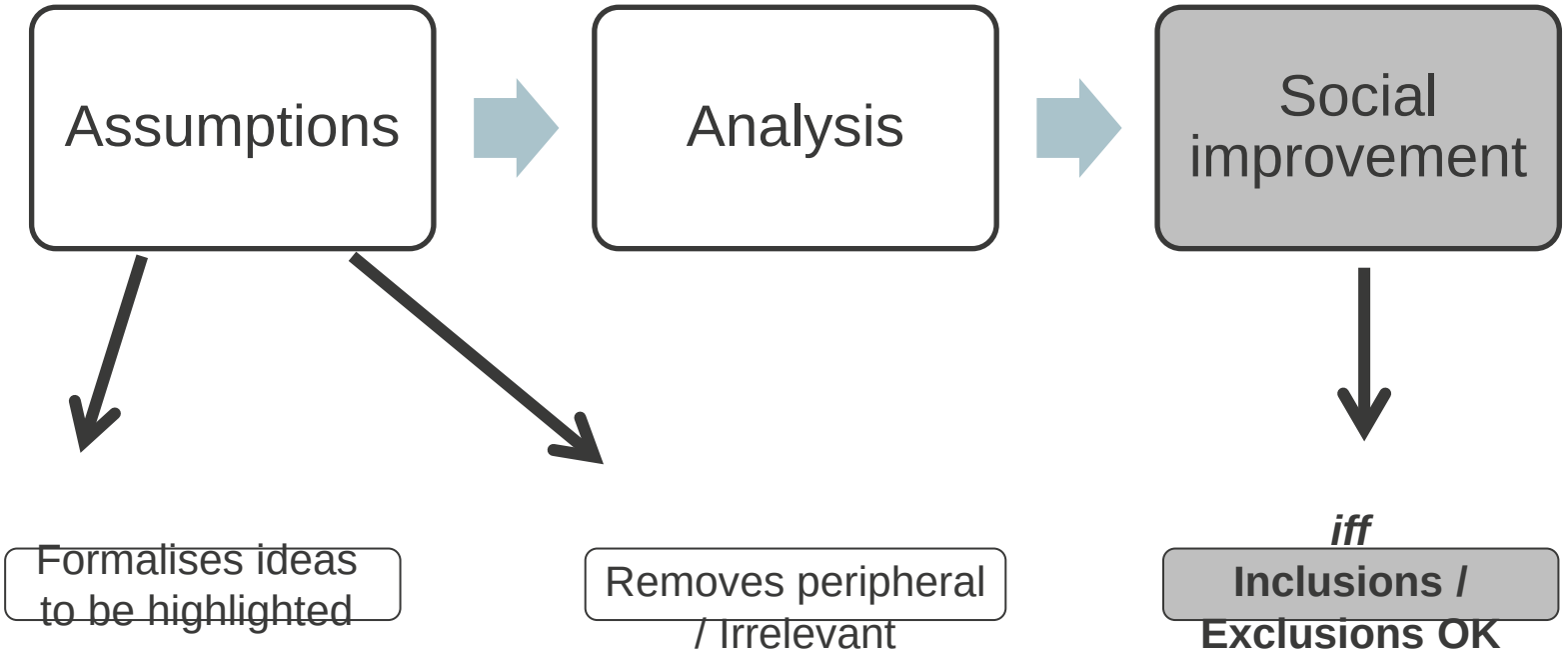
http://www.aqol.com.au http://www.buseco.monash.edu.au/centres/che/

# Contents

1. Theory
2. Theme
3. Results from 3 surveys
4. Revising the framework

# 1 Theory determines the framework



# 2. Present theme

- Exclusions --- not OK

**Avoiding extreme health states with ‘Cost Ineffective’ services**

- Severity per se …Nord
- Personal extreme cases …Survey 1
- Sharing life years …Survey 2
- Sharing QoL …Survey 3

# Survey 1
# Personal Preferences

n = 403

# CEA vs Personal Preferences

- CEA
  - Benefits = value of consequences (outcome ie QALYs)

  ***BUT***
- Personal preferences are for both:
  - Outcomes and
  - Protection against fear (risk) of dire outcomes

# Hypothesis

- People minimise worst outcome (irrespective of C-E)

  (Maxi-min hypothesis – Rawls, Keynes et al)

# Survey 1

Describe

Illness A
Illness B
} Services ↑ → QoL ↑ from death to full health

Probability A = 0.5 = Probability B

Service A … expensive

Service B … cheap

# Survey 1

Task

- ‘Pre-purchase’ of services A and B via insurance st budget
- Measure utility of health states (VAS → TTO)

# Survey 1

Task

- ‘Pre-purchase’ of services A and B via insurance st budget
- Measure utility of health states (VAS → TTO)

- Relative price $P_A/P_B$ varied
  - predict ‘optimal’ mix of insurance for A, B

$$\frac{MU_A}{P_A} = \frac{MU_B}{P_B}$$

[ MU = marginal utility]

  - Compare with hypothesised maximin

$$(U_A = U_B)$$

# TOTAL BUDGET $80,000

**Illness A** Problems with:

1. Walking, 2. Self care , 3. Usual daily activities

**Illness B** Problems with:

1. Anxiety, 2. Depression, 3. Pain

| Funds left for insurance B | Insurance A Cover % | Insurance A Cost (000) | Insurance A Guaranteed health state | Insurance B Cover % | Insurance B Cost (000) | Insurance B Guaranteed health state |
|---|---|---|---|---|---|---|
| $30,000 | 100 | $50 | 100% cover<br>**No problems** with walking, self care and activities | 100 | $100 | 100% cover<br>**No problems** with anxiety, depression and pain |
| $35,000 | 90 | $45 | | 90 | $90 | |
| $40,000 | 80 | $40 | | 80 | $80 | |
| $45,000 | 70 | $35 | 75% cover<br>**Slight problems** with walking, self care and activities | 75 | $70 | 75% cover<br>**Slight problems** with anxiety, depression and pain |
| $50,000 | 60 | $30 | | 60 | $60 | |
| $55,000 | 50 | $25 | 50% cover<br>**Moderate problems** with walking, self care and activities | 50 | $50 | 50% cover<br>**Moderate problems** with anxiety, depression and pain |
| $60,000 | 40 | $20 | | 40 | $40 | |
| $65,000 | 30 | $15 | | 30 | $30 | |
| $70,000 | 20 | $10 | 25% cover<br>**Severe problems** with walking, self care and activities | 20 | $20 | 25% cover<br>**Severe problems** with anxieties, depression and pain |
| $75,000 | 10 | $5 | | 10 | $10 | |
| $80,000 | $0 | $0 | 0% cover<br>**Unable to** walk, self care and do activities | $0 | $0 | 0% cover<br>**Extreme problems** with anxiety, depression and pain |

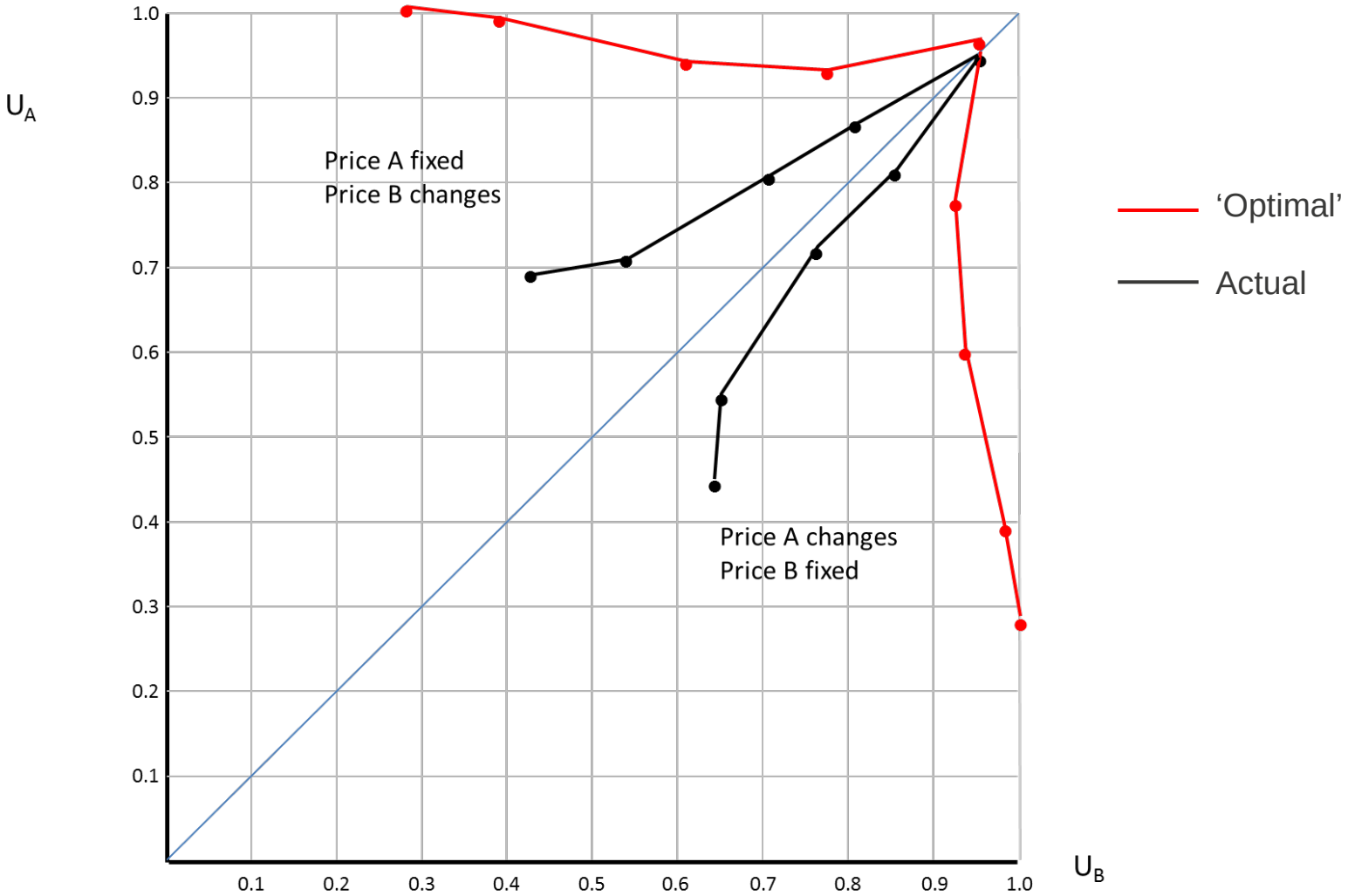
Your spending on Insurance A $..... Your spending on Insurance B $.....

MONASH University

# Result
# (Price B)/(Price A) increases 16 fold

| Framework | Insurance B | Utility B |
|---|---|---|
| **'Optimal' (EU max)** | 100 → 18% | 1.00 → 0.28 |
| **Actual** | 90 → 30% | 0.95 → 0.54 |

# ‘Optimal’ and observed utility ($U_A$, $U_B$) after illness as price varies



# Conclude

‘Cost ineffective’ services for extreme outcomes

are undervalued by CEA

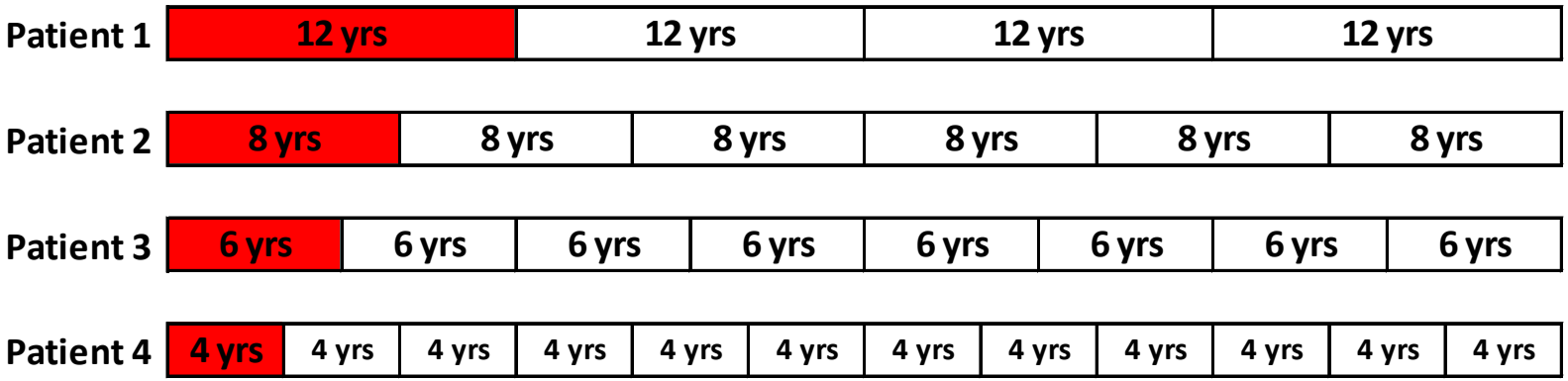
*if preferences measured when outcomes unknown*

*ie as part of an insurance scheme*

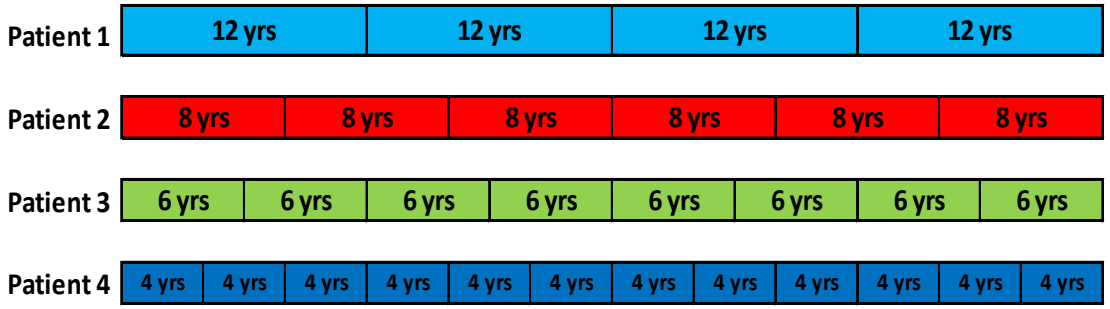
# Survey 2: Social Preferences Sharing life years

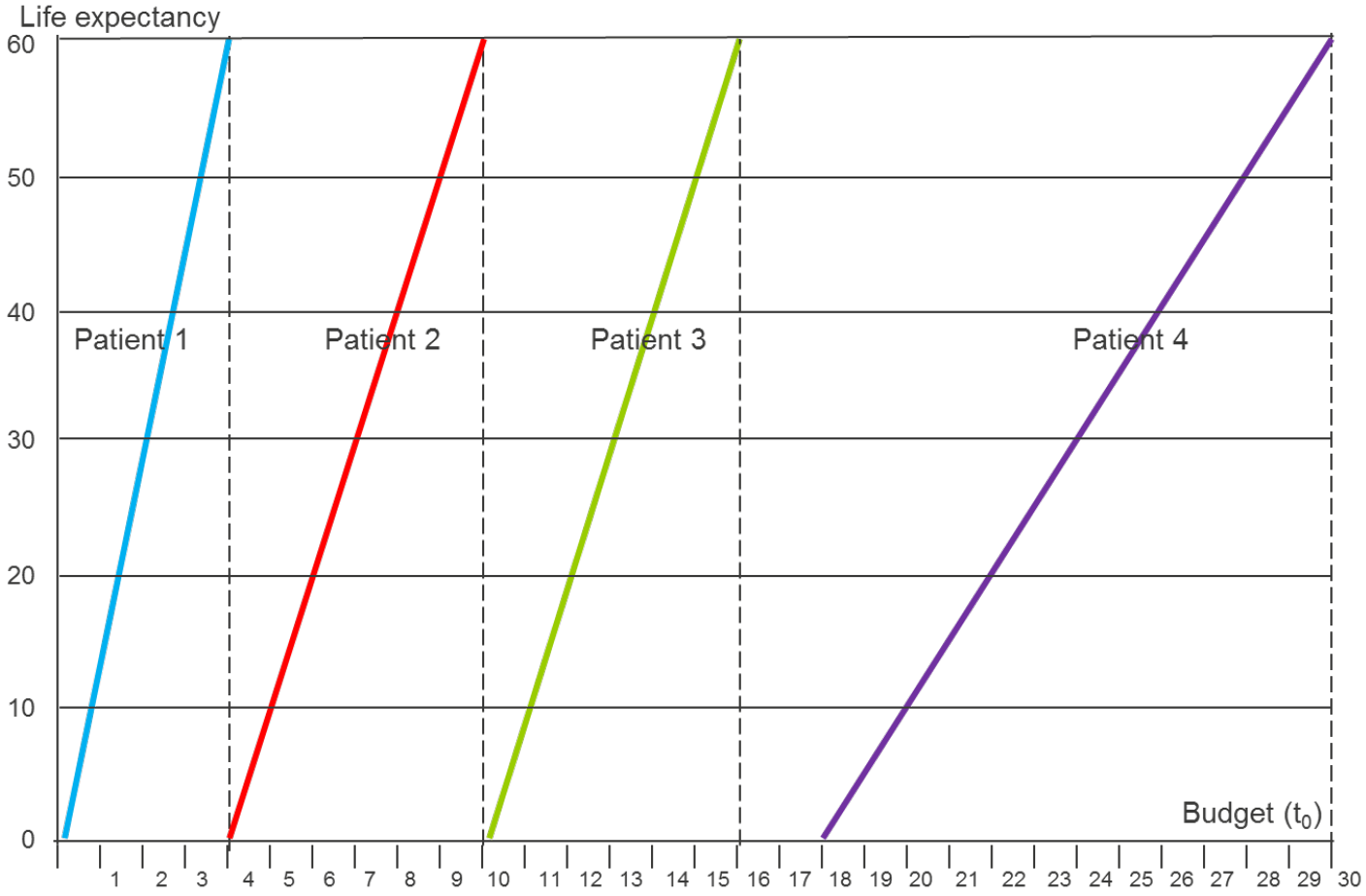
n = 511

# Web based allocation exercise

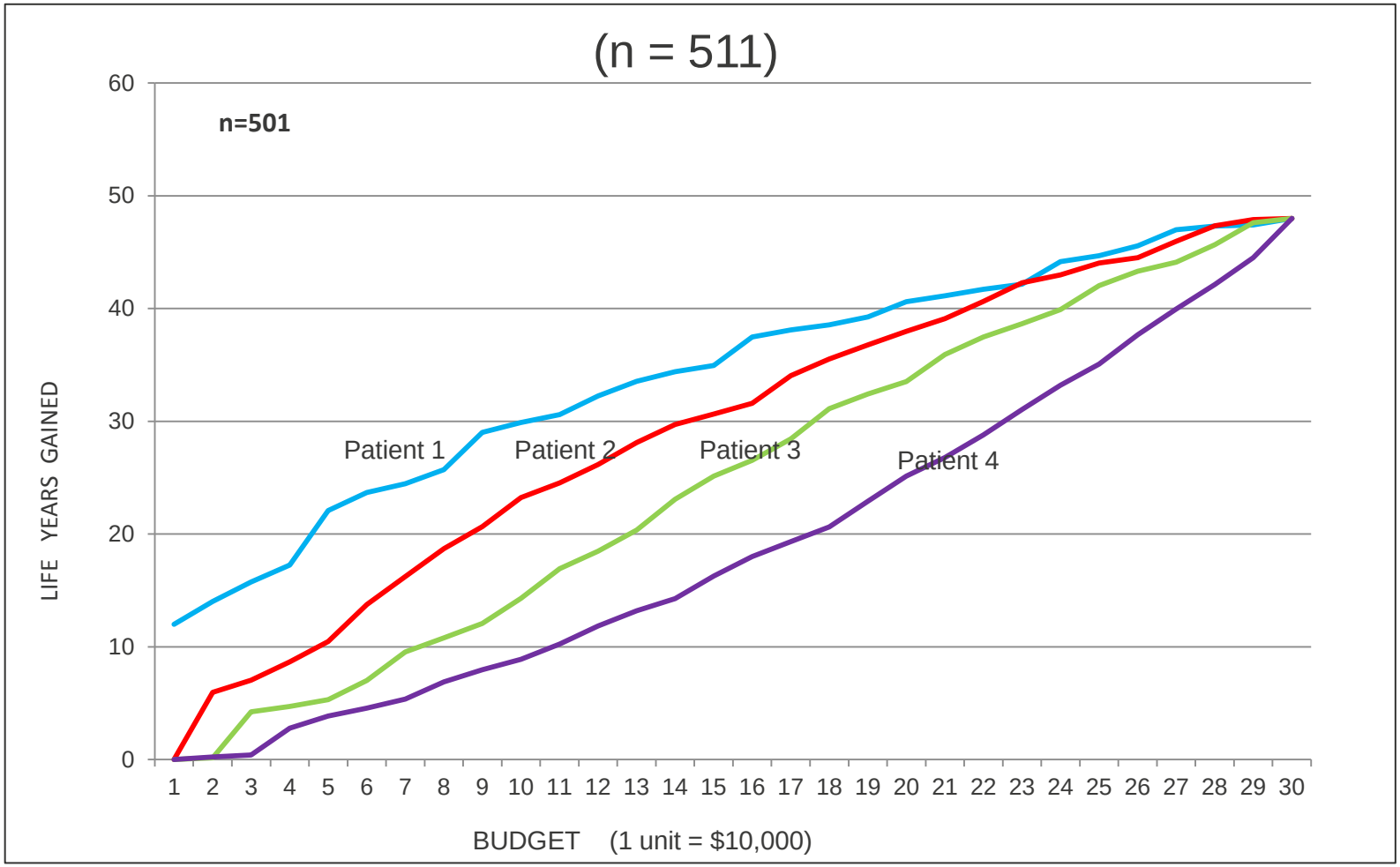


# CEA and life years allocated



# Survey result



# Conclude

- Sharing commenced immediately,
  ie high cost/LY selected over low cost/LY

# Survey 3: Social Preferences Sharing quality of life

n = 432

# Hypothesis

- Public preferences
  - ← shared (average) cost per person affected
- With a fixed budget
  - Importance cost ↓
  - Importance severity ↑

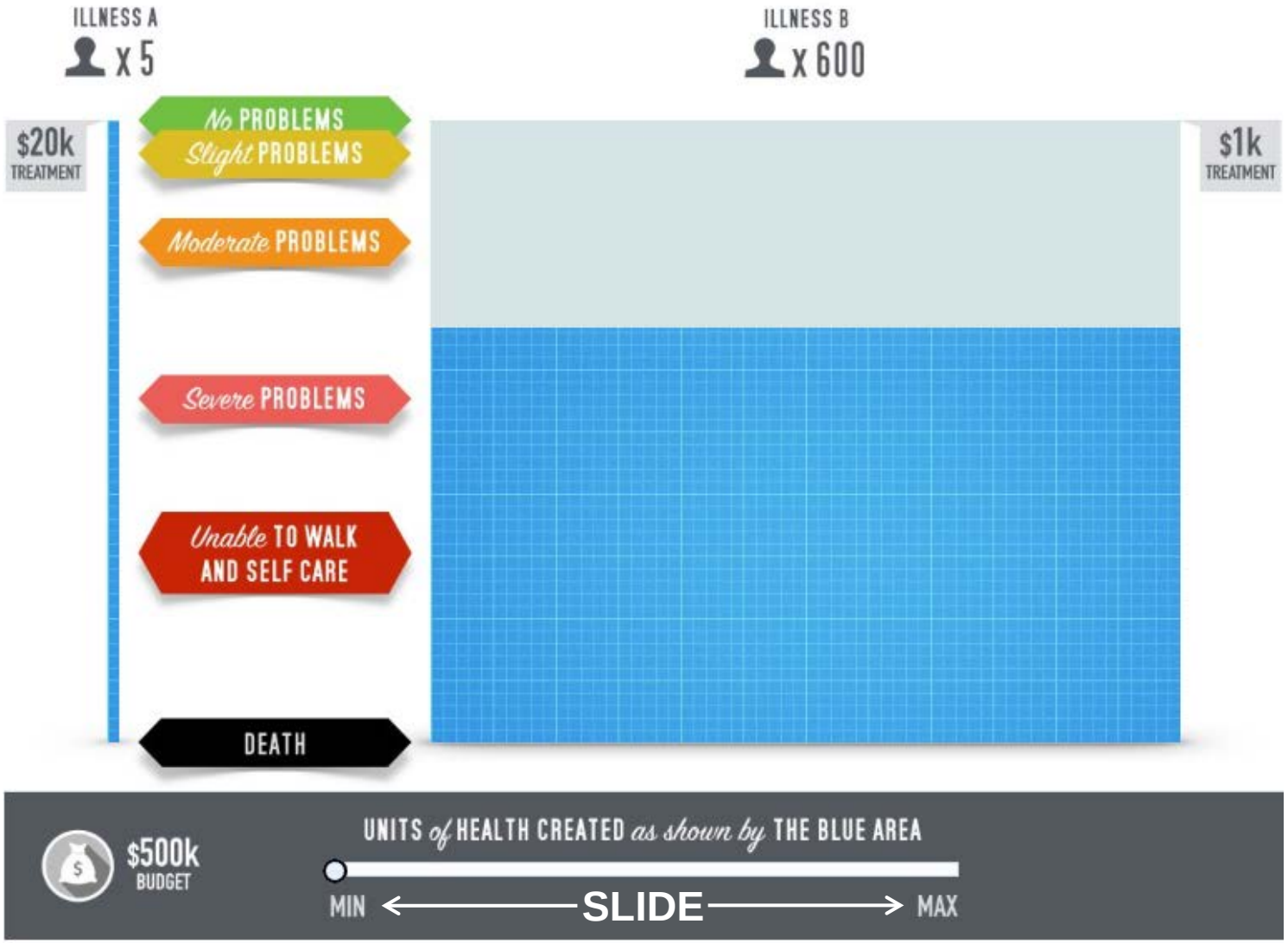
# Example of the intuition

- Sailor lost at sea
  - Would you personally sacrifice €2 to save him?
    … Yes.
  - But 1 million people must sacrifice €2
    Total cost = €2m
    It is not cost effective
  - Would you change your mind
    … No.

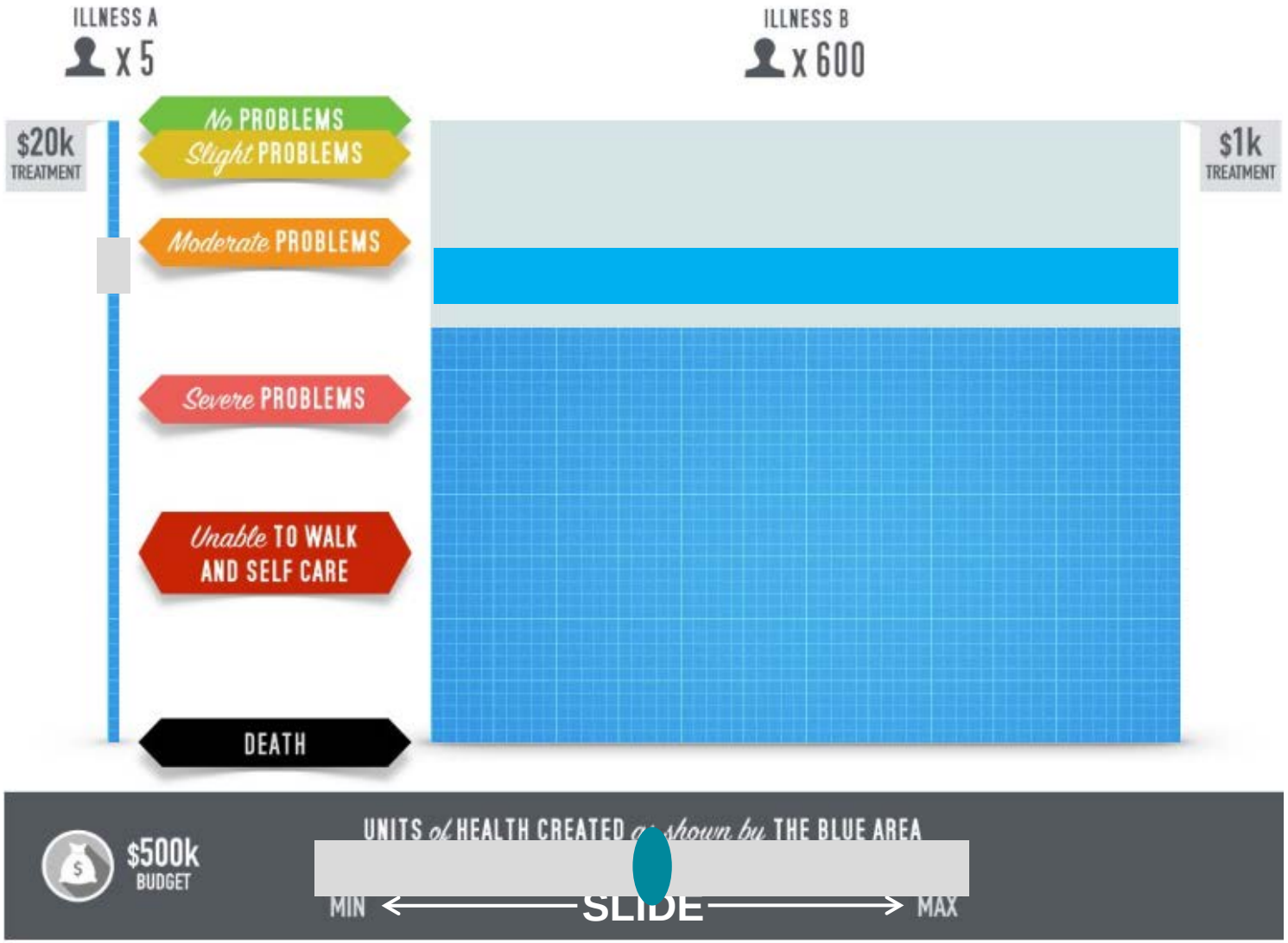
# Survey

- Allocate a budget to partial (full) cure of:
  - Illness A: High Cost, 5 patients
  - Illness B: Low Cost, many patients
- Cost A
  - 20, 15, 10, 5, 2 x Cost B
- Group B
  - n = 100, 300, 600

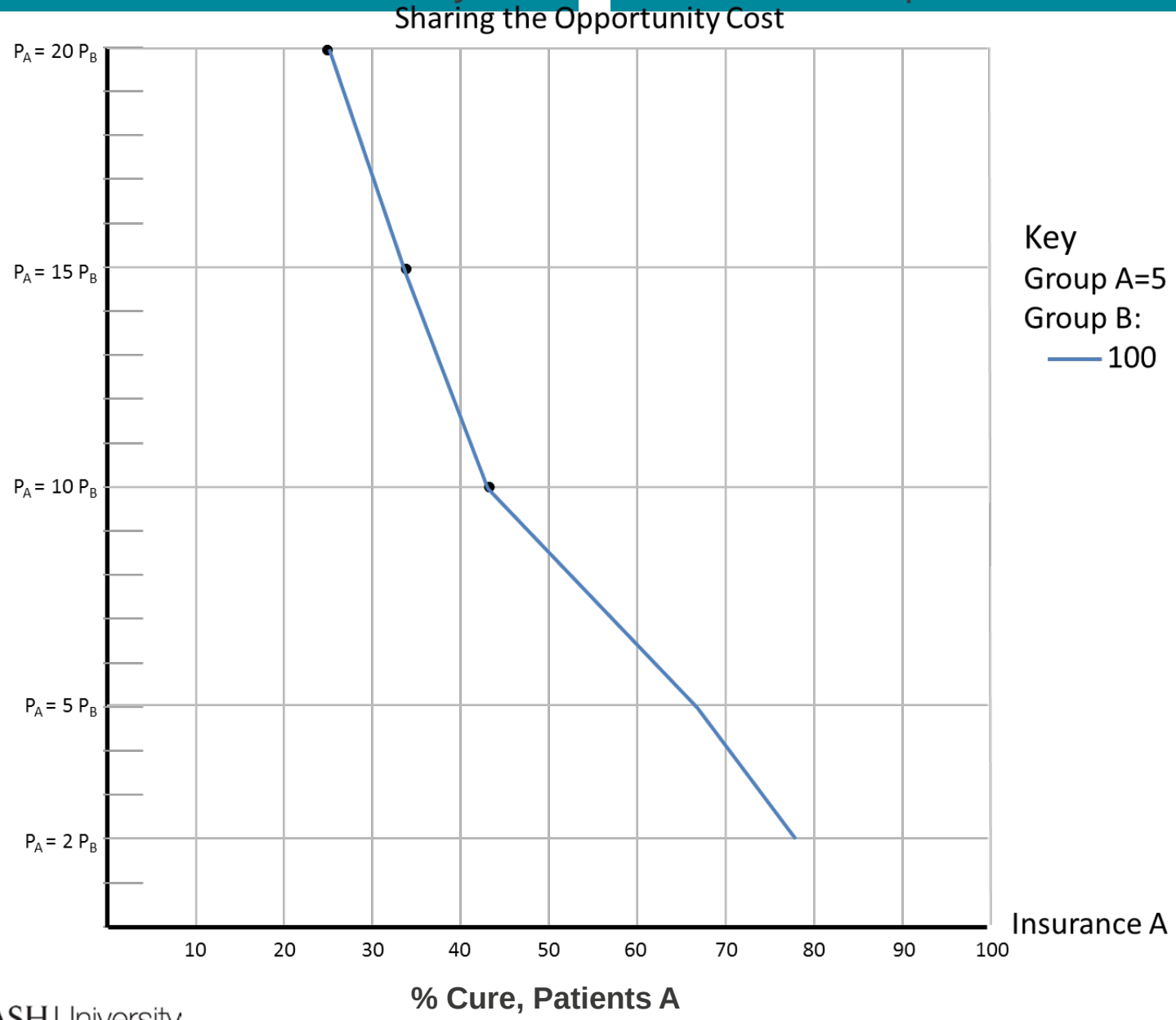
# Test of hypothesis: Shift the slide



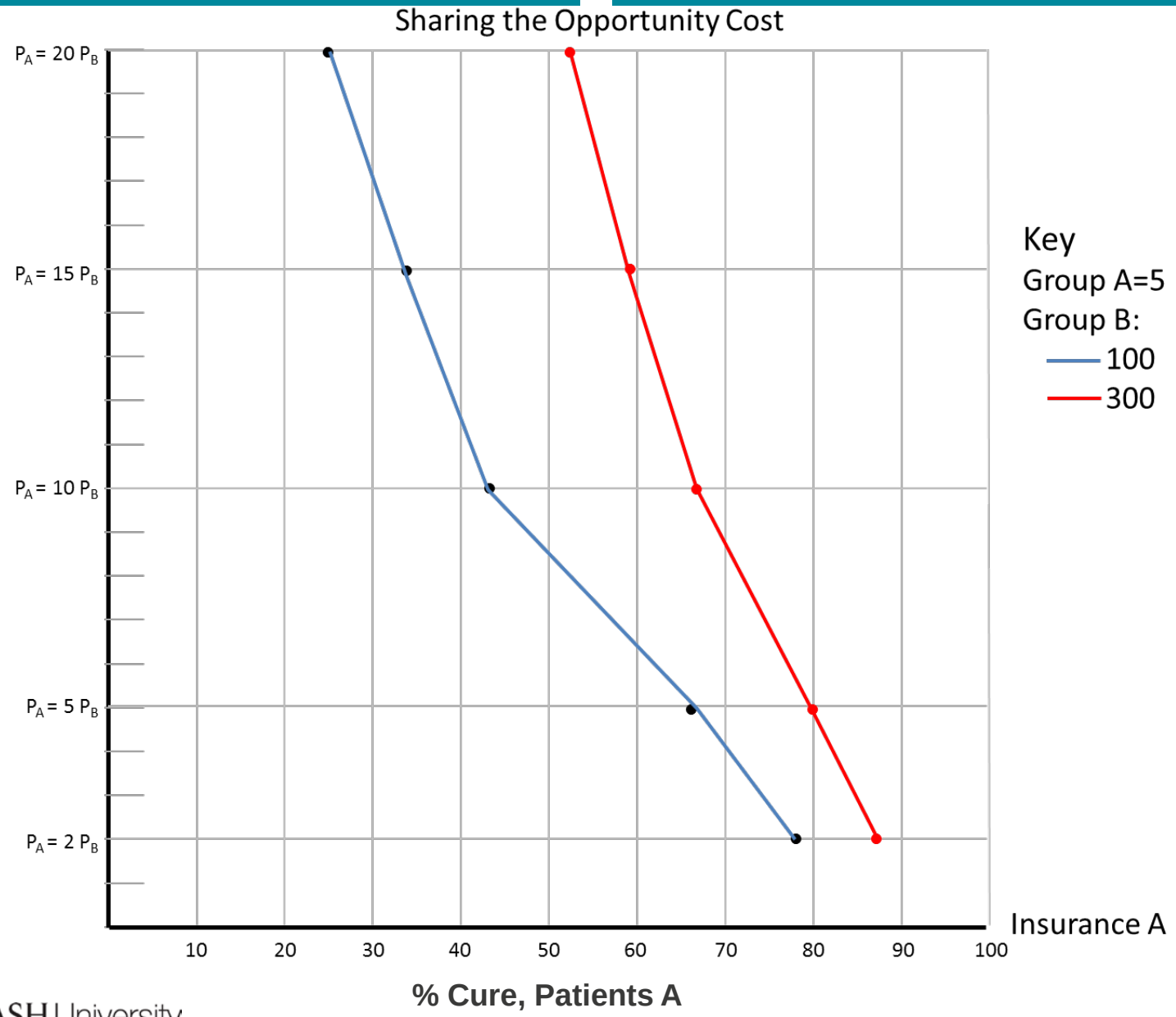
# Test of hypothesis: Shift the slide



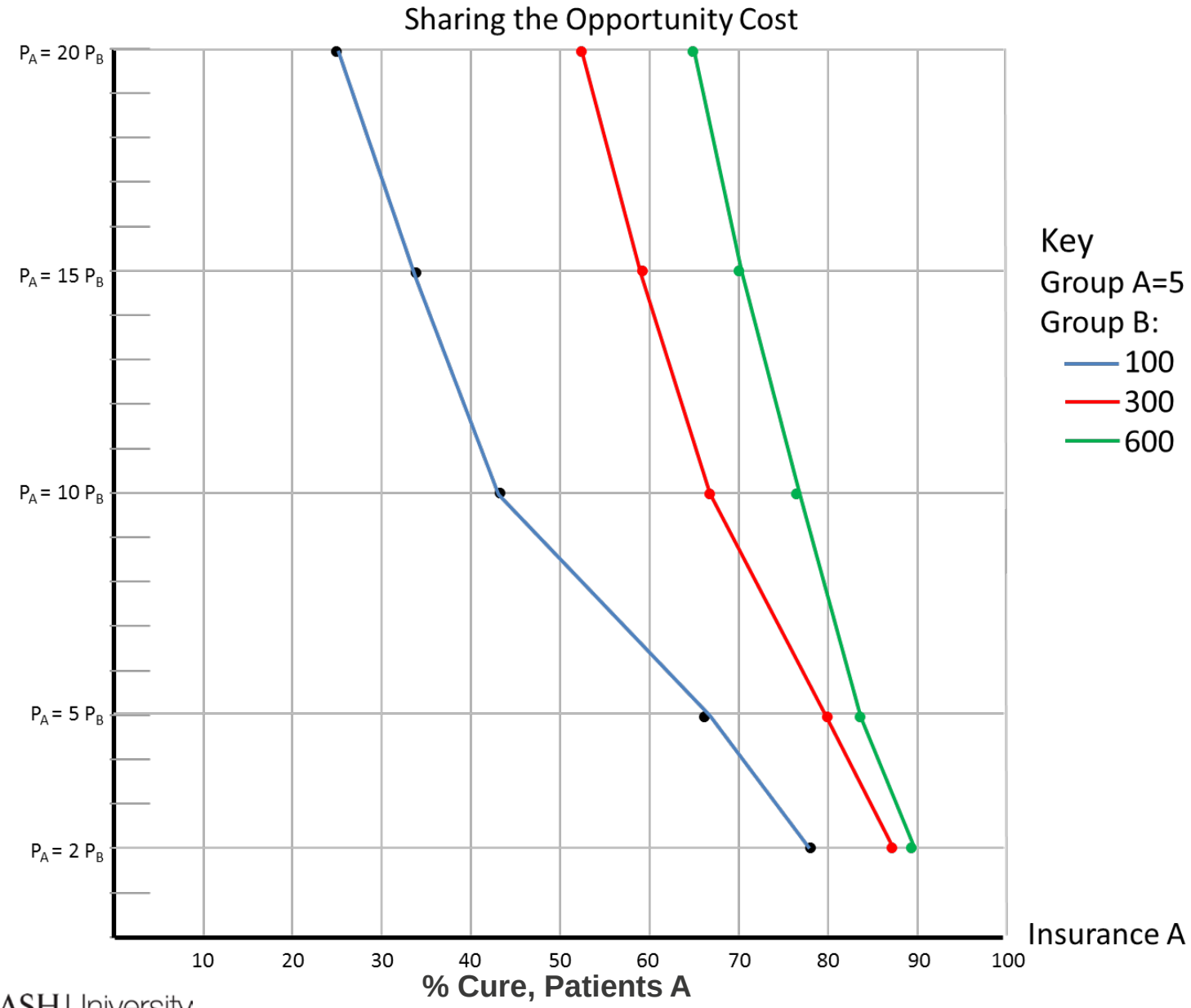
# Insurance A by Price A and size of Group B



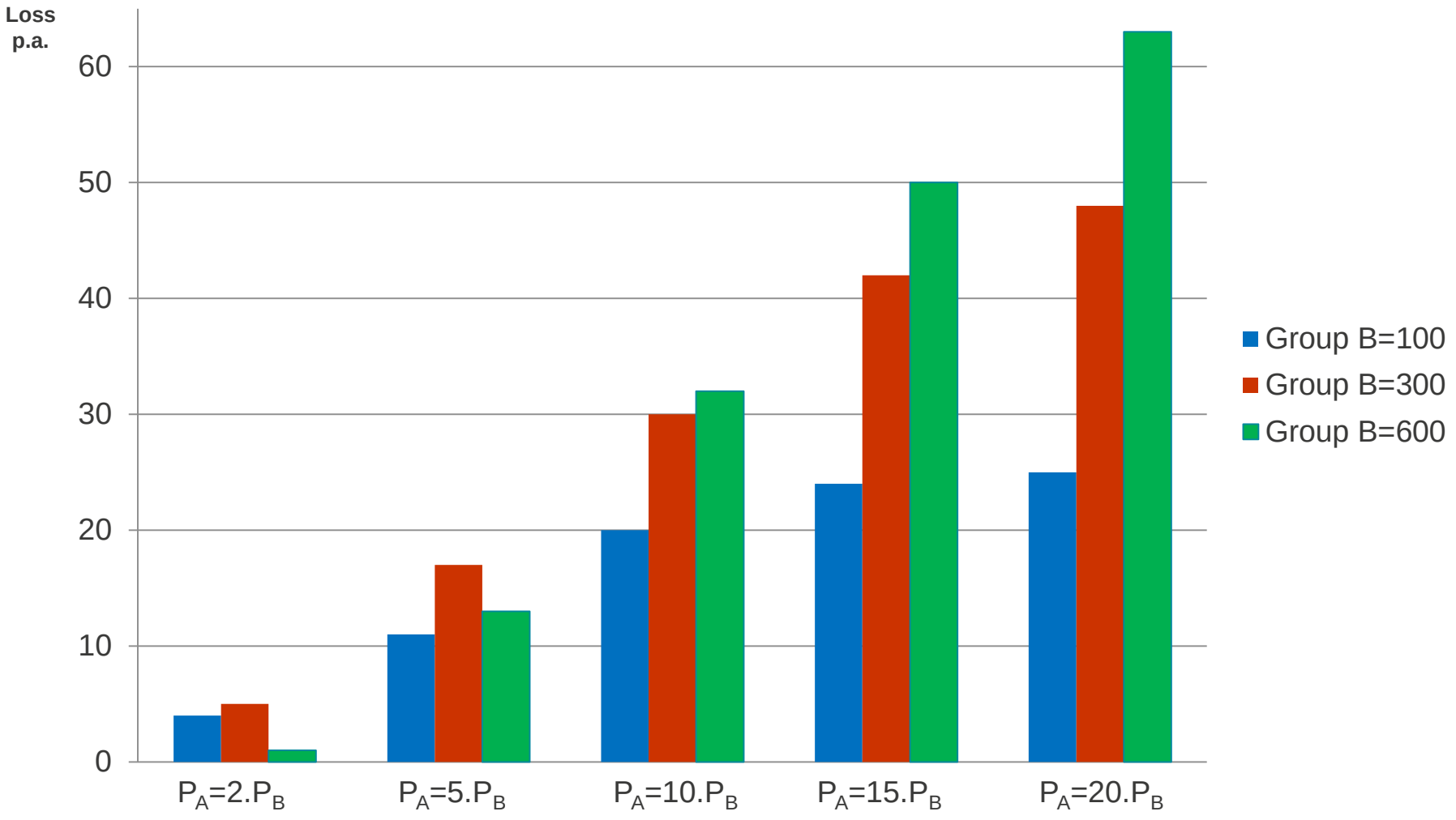
# Insurance A by Price A and size of Group B



# Insurance A by Price A and size of Group B



# Total QALY loss by Price A and size of Group B



# Survey Result vs CEA

- CEA
  - No/miniscule funding of Service A
  - 5 People die/assigned minimal QoL
- Actual result
  - Significant sharing
  - Funding of A ↑ as Sharing ↑
  - Sharing ↑ → QALYs ↓

# Summary of 3 studies

- People seek to avoid worst outcome health states:
  - For themselves (survey 1)
  - For others (survey 2, 3)
- To achieve this, people:
  - Reduce their own E(U) ie E(health)
  - Reduce overall population health and share resources
- This implies:
  - Purchase ‘cost ineffective’ services for severe cases

# 4 Revising the framework

- Step 1 Determine relevant concepts
- Step 2 Quantify concepts
  Severity/Sharing
- Step 3 determine decision rule/algorithm

# Revising the framework: An example

- CEA Benefit [QoL, LY] - Cost > 0
- Revised Benefit [QoL, LY, ***severity*** …] - ***k*** cost > T

*Where*

k = discount factor for sharing cost
T = threshold to achieve a given budget

- Result Severity ↑, k ↓
  Priority
  high cost, high severity, services ↑
  low cost, low severity services ↓

# Conclusion

- Precise formula
  - ← Evidence of social value
  - Need for (country specific) research
- Likely effect
  - Reduced importance of cost
  - Increased importance severe health states

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# The Social Value of Interventions

## [Not Only] for Ultra-Rare Disorders:

## Which Way Forward?

**Michael Schlander**

INNOVAL HC
Institute for Innovation & Valuation in Health Care

**HTAi 2015: 12th Annual Meeting, Oslo / Norway, June 14-17, 2015**

& Mannheimer Institut für Public Health – www.miph.uni-hd.de

# What are the Alternatives?

## 1: "Efficiency-Only" Framework ?

- currently predominant "extrawelfarist" paradigm?

## 2: "Efficiency-First" Framework ?

- extended by incorporating "social value judgments"
  - e.g., by multiple adjustments of cost per QALY thresholds by (disorder- and/or patient-related) contextual variables?

## 3: "Fairness-First" Framework ?

- adopting a "sharing perspective" driven by "empirical ethics"
  - (relative) social willingness-to-pay as a proxy for social value?
  - budget impact reflecting social opportunity cost?

## 4: Outright Rejection of Health Economic Analysis ?

- then, what about opportunity costs?
- appropriate role for multi-criteria-decision analysis (MCDA)?

# Perspectives on Value

**A Broad Range of Empirical "Non-Selfish" Preferences**
indicating objectives apart from simple QALY maximization:

Prioritization criteria supported by empirical evidence include

- **severity** of the initial health state,
- **urgency** of the initial health problem,
- **capacity to benefit** of relatively lower importance,
- certain **patient attributes**,
- a strong dislike for "**all-or-nothing**" resource allocation decisions,
- a "**sharing**" perspective (with less emphasis on cost per patient),
- and **rights**-based considerations.

# Perspectives on Cost

- A **decision-makers**' perspective:

  overall **budgetary impact** (*transfer cost*)

- A **social value** perspective:

  (instead of an almost exclusive narrow focus on individual utility):

  social **opportunity cost** (or [social] value foregone)
  better reflected by net budgetary impact (*transfer cost*)?
  Move focus from cost per patient to cost on the program level?

- A **pragmatic** perspective

  should reflect the commercial realities of the research-based biopharmaceutical industry, which is showing signs of a shift from price maximization to **life cycle revenue management**.

# Elements of a Roadmap

towards **Social Cost Value Analysis (CVA)**,
better approximating the public's expectations

## Multi-Criteria Decision Analysis (MCDA)

- including a more prominent role for budgetary impact

## Social Preferences Measurement Project

- generating more robust empirical evidence on "social preferences"
- in an inclusive effort, inviting multiple stakeholders to participate (cf. the example of SwissHTA)

# For Further Exploration of Ways Forward:

- Smith RD, Richardson J:
  **Can we estimate the 'social' value of a QALY? Four core issues to resolve.**
  *Health Policy*. 2005; 74 (1): 77-84.

- Schlander M, Garattini S, Holm S, Kolominsky-Rabas P, Nord E, Persson U, Postma M, Richardson J, Simoens S, de Solà Morales O, Tolley K, Toumi M:
  **Incremental cost per quality-adjusted life year gained? The need for alternative methods to evaluate medical interventions for ultra-rare disorders.**
  *J Comp Eff Res*. 2014; 3(4): 399-422.

- **Heidelberg Health Economics Summer School**:
  **"Economic Evaluation & Health Technology Assessment"**
  Heidelberg / Germany, September 14-18, 2015
  See: ***www.innoval-hc.com***

# Thank You for Your Attention!

on behalf of the "URD Project Group"

**Professor Michael Schlander, M.D., Ph.D., M.B.A.**

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The *URD Consensus Documents* are available at the Institute's website **www.innoval-hc.com**