UNIVERSITÄT HEIDELBERG

The Social Value of Orphan Medicinal Products (OMPs):

The European Social Preferences Measurement (ESPM) Study Project

[Presentation and Break-Out Session for
Multi-Stakeholder Symposium
on Improving Patient Access to Rare Disease Therapies]

Michael Schlander
on behalf of the URD / ESPM Study Group

INNOVAL HC
Institute for Innovation & Valuation in Health Care

EURORDIS Roundtable of Companies (ERTC) Meeting
Brussels / Belgium, February 24, 2016, 15:45h - 17:15h

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

Overview

- **Background & Rationale**
  - The Most Expensive Treatments of the World
  - The Standard Answer of Health Economists
  - What's Wrong with the Conventional Logic?
- **Perspectives on and Sources of Value**
  - Should Social Preferences Matter?
  - A Preference for Rarity?
  - Which Values Should Count?
- **Social Norms and Preferences**
  - Alternative Frameworks for Evaluation
  - The Need for More Robust Empirical Evidence
  - The ESPM Research Project

Overview

Presentation
Michael Schlander
15:45 - 16:30

¬ Background & Rationale

- ¬ The Most Expensive Treatments of the World
- ¬ The Standard Answer of Health Economists
- ¬ What's Wrong with the Conventional Logic?

Discussion
Vinciane Pirard
Mohit Jain
16:30 - 17:00

¬ Perspectives on and Sources of Value

- ¬ Should Social Preferences Matter?
- ¬ A Preference for Rarity?
- ¬ Which Values Should Count?

Next Steps
Michael Schlander
Stakeholder Engagement
17:00 - 17:15

¬ Social Norms and Preferences

- ¬ Alternative Frameworks for Evaluation
- ¬ The Need for More Robust Empirical Evidence
- ¬ The ESPM Research Project

INNOVAL HC
Institute for Innovation & Valuation in Health Care

Background: Who We Are

- Independent Not-for-Profit Organization
  - Not a Commercial Contract Research Organization
- Founded in Aschaffenburg/Germany in June 2005
  - Offices in Wiesbaden/Germany since December 2008
- Member of the Stockholm Network
  - Group of European Market-Oriented Think Tanks
- Formally associated with University of Ludwigshafen
- Funding of Projects
  - Under an "unrestricted educational grant" policy
  - Supported by National Institute of Mental Health (NIMH), Bethesda, MD; NHMRC, Canberra, AUS; HTA Agencies; DFG; DKFZ; Physician and Payer Organizations; SAMW; Industry … (>90% international projects)
- Prof. Michael Schlander, MD, PhD, MBA (Heidelberg & Ludwigshafen)
- Prof. Oliver Schwarz, PhD (Heilbronn)
- Prof. Erik Trott, MD, PhD (Würzburg & Aschaffenburg)

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Background: What We Do (Examples)

- **Normative Analysis**
  - Normative Health Economics and "Empirical Ethics"
  - Evaluation Principles for Rare & Ultra-Rare Disorders (URDs)
- **Health Care Policy Analysis**
  - Pharmaceutical Market Regulation
  - "Appraising the Appraisers"
- **Health Technology Assessment (HTA)**
  - Systematic Reviews and Value Assessments
  - Swiss HTA Consensus Project
- **Applied Health Economics**
  - Cost Effectiveness Analyses & Modeling
  - Health Economic Methods Development
- **Health Care Utilization Research**
  - Nordbaden Project (using German administrative data)
- **Education, Outreach & Consulting**
  - Heidelberg Health Economics Summer School

Starting Point:
How to Evaluate Interventions for URDs?

¬ Five International Expert Workshops

- ¬ in Berlin / Germany, November 08, 2012
- ¬ in Dublin / Ireland, November 07, 2013
- ¬ in Amsterdam / The Netherlands, November 13, 2014
- ¬ in Heidelberg / Germany, September 16, 2015
- ¬ in Milan / Italy, November 12, 2015[1]

¬ Agreement on Issues and on Way Forward

- ¬ on challenges that arise when applying conventional HTA methodologies to rare and ultra-rare disorders (URDs)
- ¬ on the need for (improved or) alternative evaluation methods
- ¬ on promising ways forward (notably, social cost value analysis), overcoming the shortcomings of currently prevailing evaluation paradigms
- ¬ need for more empirical research into “social preferences” (ESPM Study)

[1]supported by unrestricted educational grants from BioMarin and Genzyme (2013 - 2015); in 2012, from BioMarin and Alexion

Starting Point:
How to Evaluate Interventions for URDs?

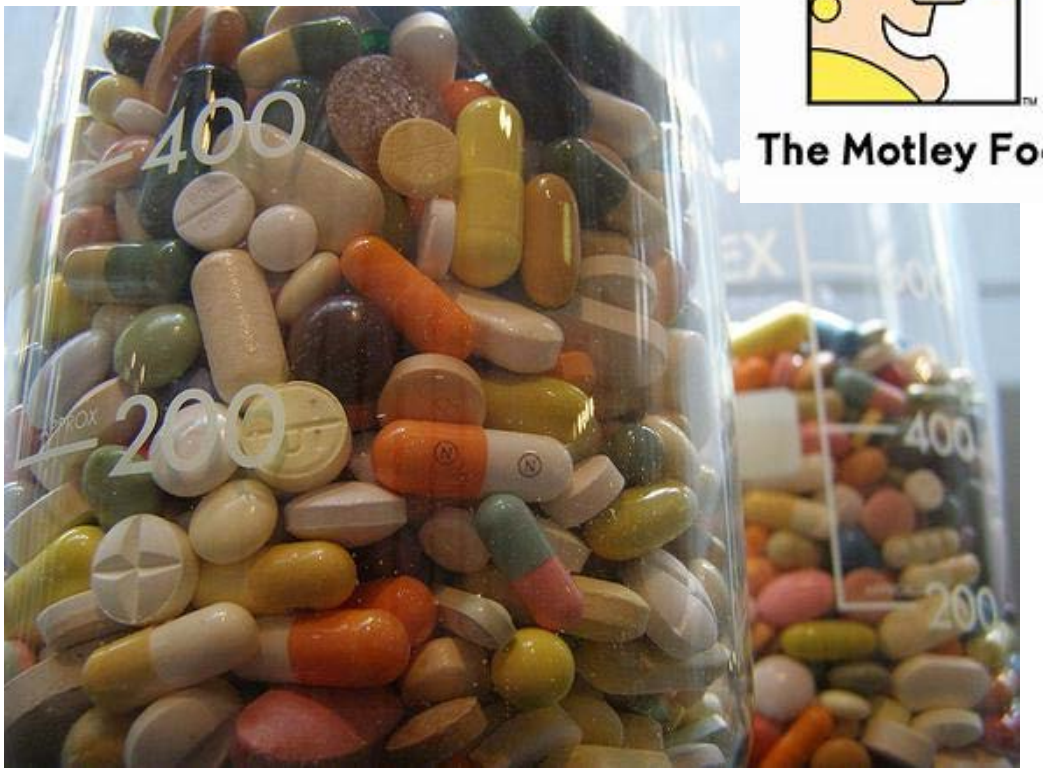
¬ International Expert Group

- ¬ **Silvio Garattini** (Mario Negri Institute, Milan / Italy)
- ¬ **Sören Holm** (U of Manchester / England)
- ¬ **Peter Kolominsky** (U of Erlangen / Germany)
- ¬ **Deborah Marshall** (U of Calgary / Canada)
- ¬ **Erik Nord** (U of Oslo / Norway)
- ¬ **Ulf Persson** (IHE, Lund / Sweden)
- ¬ **Maarten Postma** (U of Groningen / The Netherlands)
- ¬ **Jeffrey Richardson** (Monash U, Melbourne / Victoria)
- ¬ **Michael Schlander** (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)

"Hand clapping for science is now inextricably linked to hand wringing over affordability."[1]

[1]**Peter B. Bach**
New England Journal of Medicine 2015 (November 05); 373 (19): 1797-1799.

"The Most Expensive Drugs in the World"[1]



[1]S. Williams, The Motley Fool, June 29, 2013. http://www.fool.com/investing/general... [last accessed Jan. 22, 2016]

The 5 Most Expensive Drugs in the World[1]

1. Soliris (Alexion)
   paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS); average annual cost: US-$ 409,500
2. Elaprase (Shire)
   Hunter syndrome (ERT); US-$ 375,000 p.a.
3. Naglazyme (BioMarin)
   mucopolysaccharidosis (MPS) VI (ERT); US-$ 365,000 p.a.
4. Cinryze (ViroPharma)
   hereditary angioedema (HAE); US-$ 350,000 p.a.
5. Myozyme (Sanofi / Genzyme)
   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, 2016]

The 5 Most Expensive Drugs in the World[1]

1. Soliris (Alexion)
   (8,000 [PNH] + [aHUS]) x US 09,500 =
   = US-$ 3,400 n p.a. (U.S. al
2. Elaprase (Shire)
   2,000 [Hunter s.] x US-$ 375 - US-$ 750 million p.a. (WW)
3. Naglazyme (BioMarin)
   1,100 [MPS VI] x US-$ 000 = US-$ 400 million p.a. (WW)
4. Cinryze (ViroPharma)
   6,000 [HAE] x US-$ 350,000 = US-$ 2,100 million p.a. (U.S.)
5. Myozyme (Sanofi / G me)
   900 [Pompe dis.] x US-$ 300,000 = US-$ 270 million p.a. (WW)

Five Drugs (back of the envelope estimate): ≥ US-$ 6.9 billion p.a.

[1]S. Williams, The Motley Fool, June 29, 2013. http://www.fool.com/investing/general... [last accessed Jan. 22, 2016]

Health Technology Assessment (HTA)

Martin Luther (1530)

**"Wer am Wege baut,
hat viele Meister"**

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

Definition of Health Technology Assessment (by EUnetHTA)

- **Health Technology Assessment (HTA)**
  is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value.

- Despite its **policy goals**, HTA must always be firmly rooted in research and the scientific method.

What are Technology Assessments for?

A broad range of expectations (and fears) …

"restricting use"

"containing costs"

"issuing guidance to potential users"

"prioritizing for further evaluation"

"alerting users to future possibilities"

The Conventional 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)

"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*

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…").

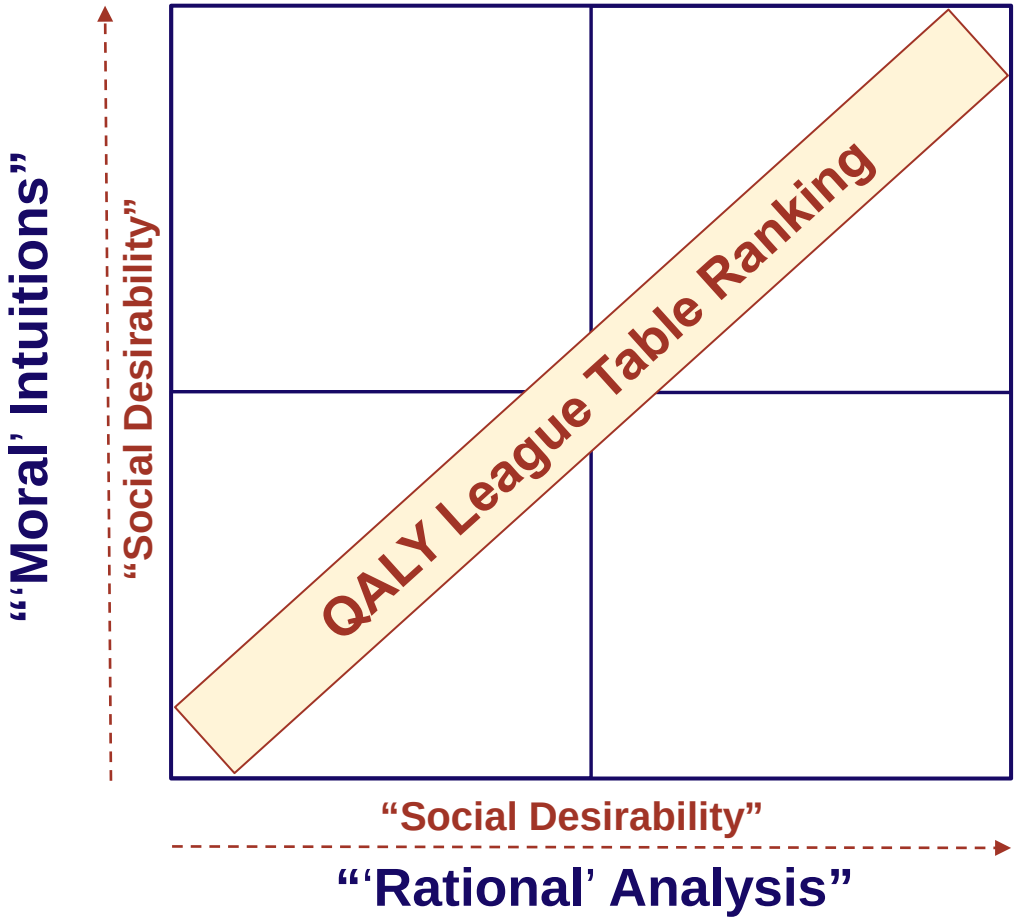
Maximizing the number of QALYs "produced"

- 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.

Reflective Equilibrium



Textbook Example: "QALY League Table"[1]

Ranking [original]	**Intervention** [abbreviated; comparator not stated in original table]	**Cost / QALY [£ (1990)]**
3	G.p. advice to stop smoking	£ 270
5	Antihypertensive therapy to prevent stroke	£ 940
6	Pacemaker implantation	£ 1,100
7	Valve replacement for aortic stenosis	£ 1,140
8	Hip replacement	£ 1,180
9	Cholesterol testing and treatment	£ 1,480
11	Kidney transplant	£ 4,710
12	Breast cancer screening	£ 5,780
15	Home hemodialysis	£ 17,260
18	Hospital hemodialysis	£ 21,970
20	Neurosurgery for malignant intracranial tumors	£ 107,780
21	Epoetin alfa therapy for anemia in dialysis patients	£ 126,290

[1]A. Maynard. *Economic Journal* 1991; 101 (408): 1277-1286

Some Cost-Effectiveness Benchmarks

- Some international "de facto" benchmarks:
  - New Zealand (PHARMAC):
    NZ-$ 20,000 / QALY[1]
  - Australia (PBAC):
    AUS-$ 42,000 / LYG to AUS-$ 76,000 / LYG[2]
  - England and Wales (NICE):
    £ 20,000 – £ 30,000 / QALY
  - United States (some MCOs):
    US-$ 50,000 – US-$ 100,000 / QALY[3]
  - Canada (proposed "grades of recommendation"):
    CAN-$ 20,000 – CAN-$ 100,000 / QALY[4]
- No scientific basis

[1]C. Pritchard (2002); QALY: "quality-adjusted life year"; [2]George et al. (2001); LYG: "life year gained"
[3]D.M. Cutler, M. McClellan (2001); [4]A. Laupacis et al. (1992)

Prevalent Unease with Thresholds

for example:

HTA Agencies

- NICE (England): end-of-life treatments, ultra-orphans
- TLV (Sweden): adjustments for severity

Research-Based Biopharmaceutical Industry

- Barriers to access
- Innovation (and dealing with uncertainty)

Payers

- NHS England: Cancer Drugs Fund
- Thresholds actually too high?

Adopting the Logic of Cost Effectiveness

… using
Incremental Cost-per-QALY-Gained
Benchmarks …

… would have the potential

to necessarily and inevitably disenfranchise

many patients with rare and ultra-rare disorders

from any chance to ever get access

to innovative effective interventions.

Revisiting the Fundamental Premise

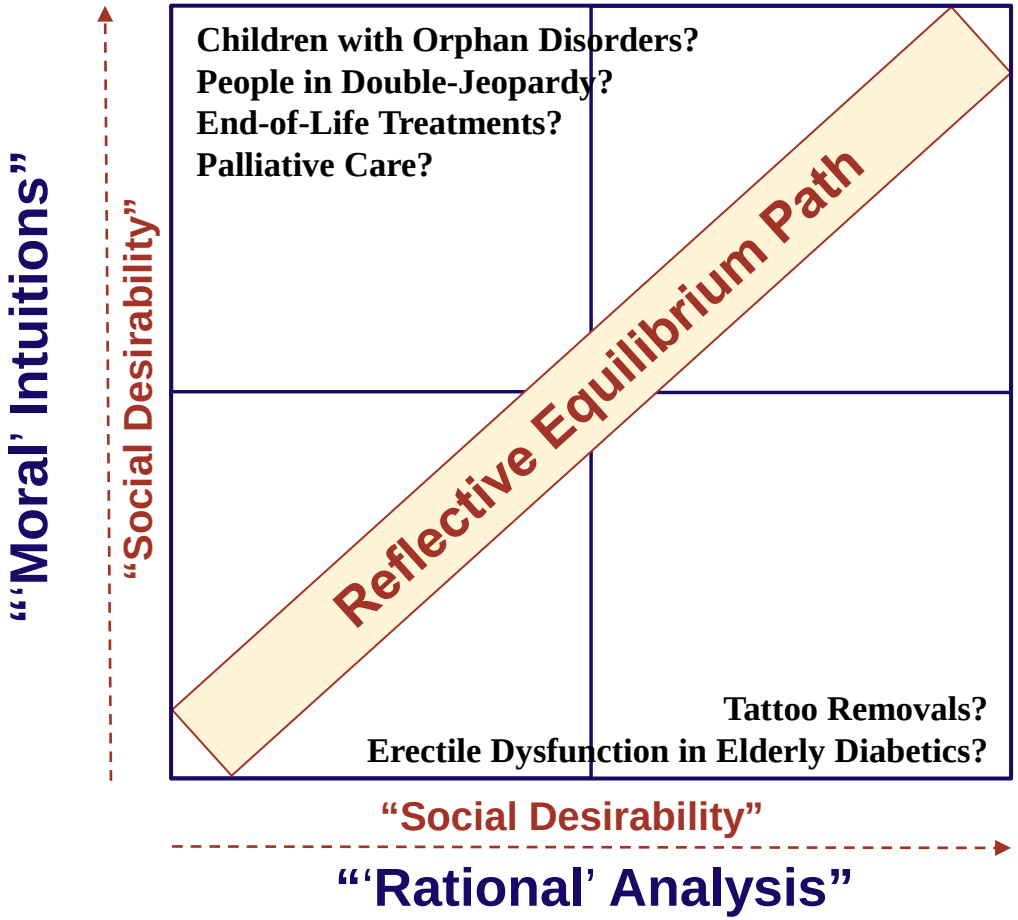
"Social Desirability of an Intervention is Inversely Related to its Incremental Cost per QALY Gained"

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



What's Wrong with the Conventional Logic?

Effectiveness and Efficiency

- by definition, "efficiency" is a secondary or instrumental objective,
- whereas the "effectiveness" criterion invariably represents the primary objective.

Efficiency

Need to distinguish between

- technical efficiency, productive efficiency, and allocative efficiency;
- static and dynamic efficiency.

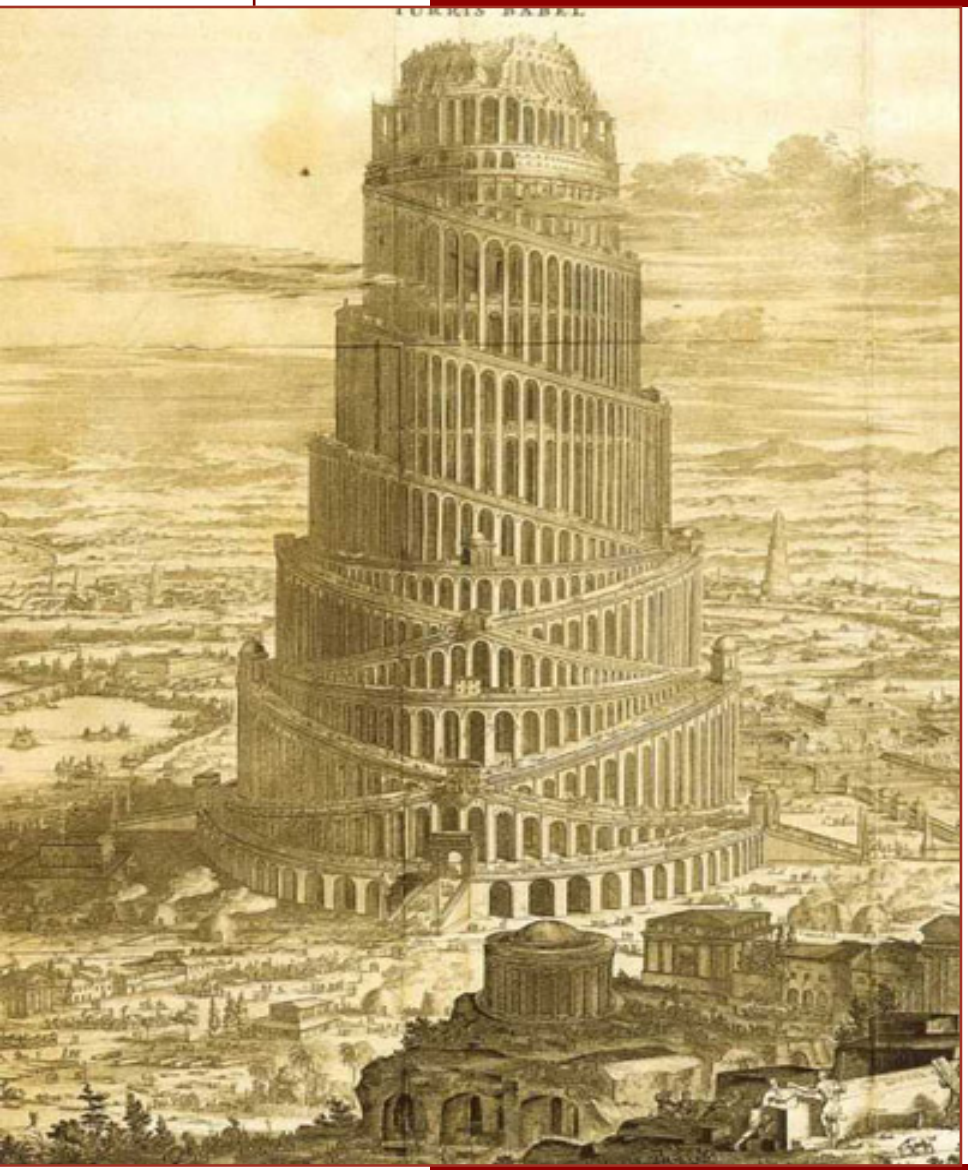
Social Value ("Utility")

Existence of

- components different from individual utility and its aggregation;
- social (i.e., non-selfish) preferences, rights and duties.

Economic "Efficiency"

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



"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

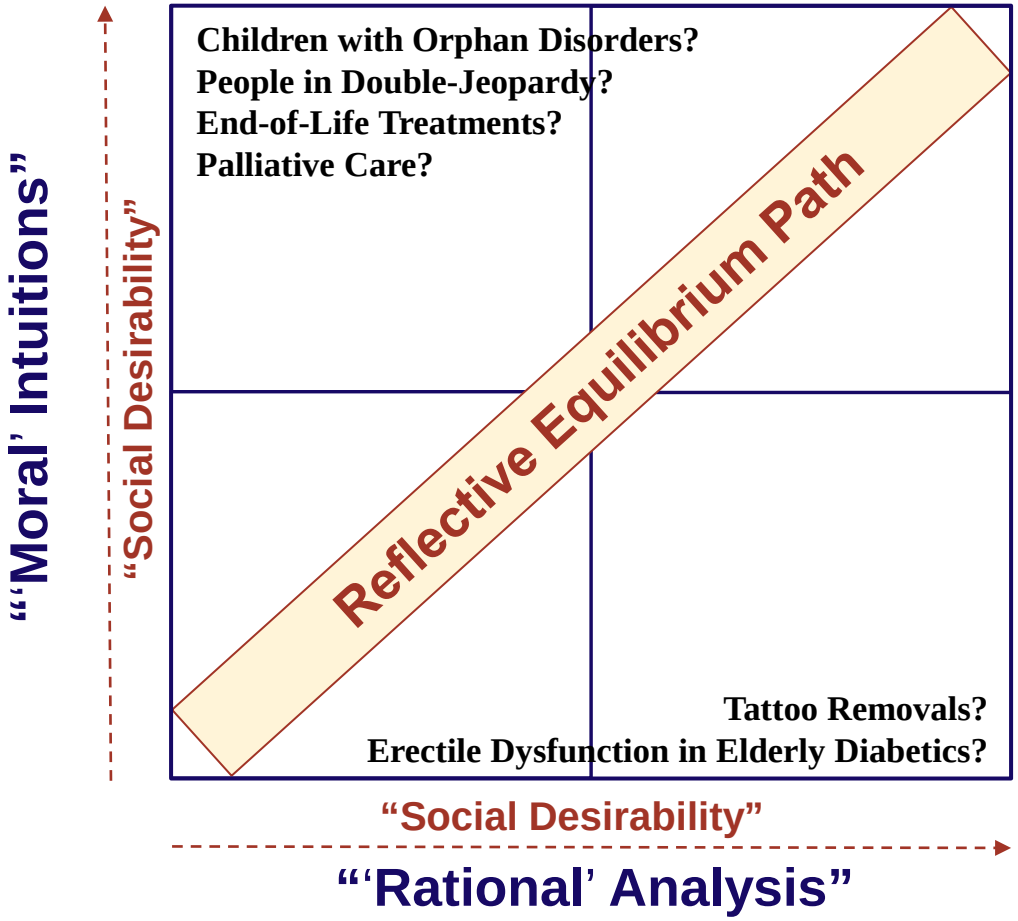
  nihilism…
  (either rejecting all values analyses as equally unreliable, or accepting all as equally credible)

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

DISCUSSION

FOR DISCUSSION

Reflective Equilibrium



FOR DISCUSSION

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

FOR DISCUSSION

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)

FOR DISCUSSION

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
- …

FOR DISCUSSION

"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)

FOR DISCUSSION

Discussion

Does "Context" Matter?

Empirical evidence supports a role of the following:

- Severity of initial health state
  - Level of impairment in addition to improvement (difference)?
- Rule of rescue
  - Identifiable individuals
    (but is being "visible" really morally relevant?)
- Potential for health improvement
  - e.g., the permanently disabled and chronically ill?
    (who have less QALYs to gain)
- Patients with high-cost illnesses

FOR DISCUSSION

Discussion

Guidance based on the EQ-5D

- Some problems with walking and with usual activities, no other problems (EQ-5D state 21211)
  - Utility gain from prevention (1 – 0.810 =) 0.190
- Fatal heart attack
  - Utility gain from prevention (1 – 0 =) 1.000

- **Issue**:

  Is preventing fifty cases of "some problems with walking and with usual activities, no other problems" **as valuable as** preventing ten cases of fatal heart attack?

FOR DISCUSSION

Discussion

Defining an International Research Project

Systematically Assessing Social Preferences

- 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

ESPM STUDY

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:

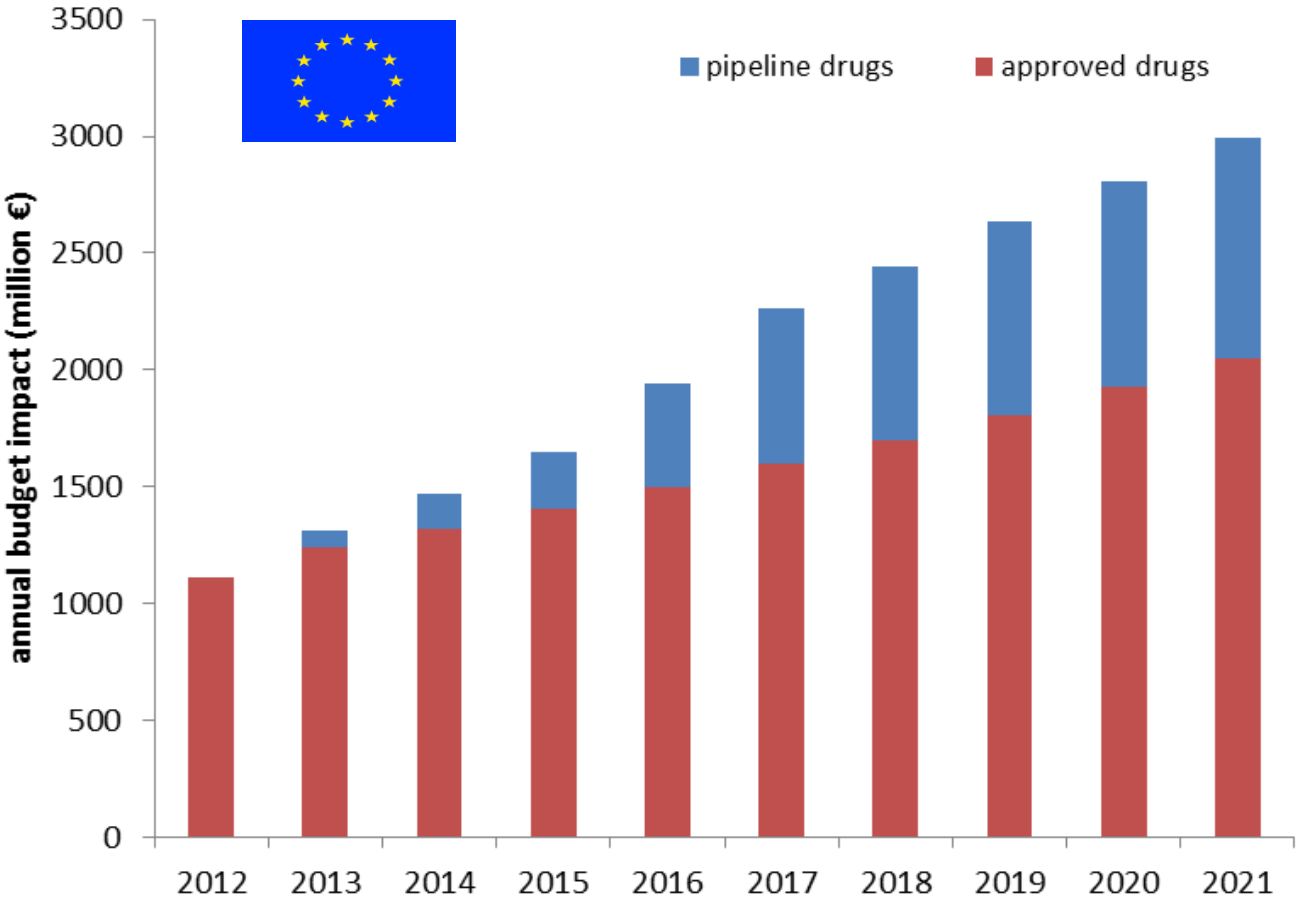
  (instead of an almost exclusive narrow focus on individual utility):

  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

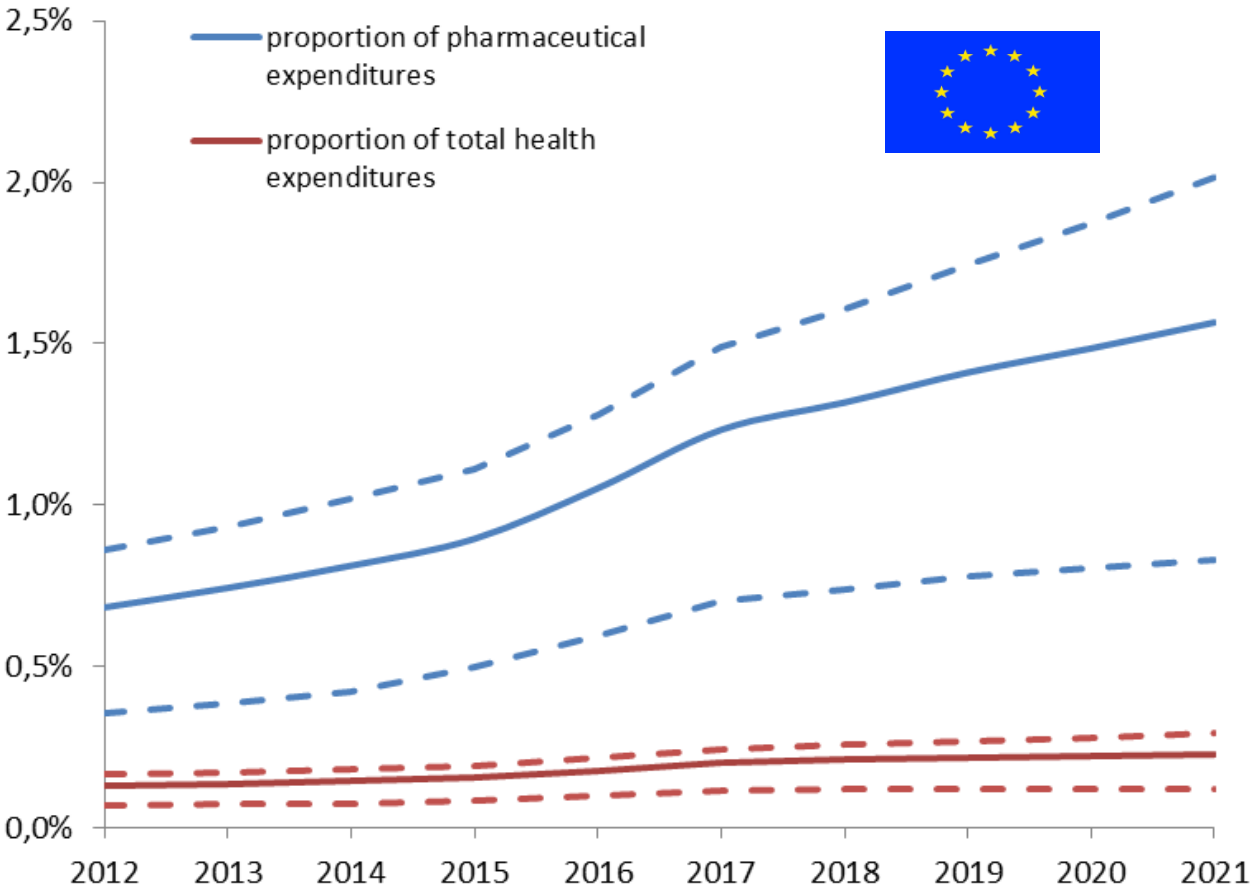
  should reflect the commercial realities of the research-based biopharmaceutical industry, which is showing signs of a shift from price maximization to **life cycle revenue management**.

A Side Note: Projected URD Budget Impact



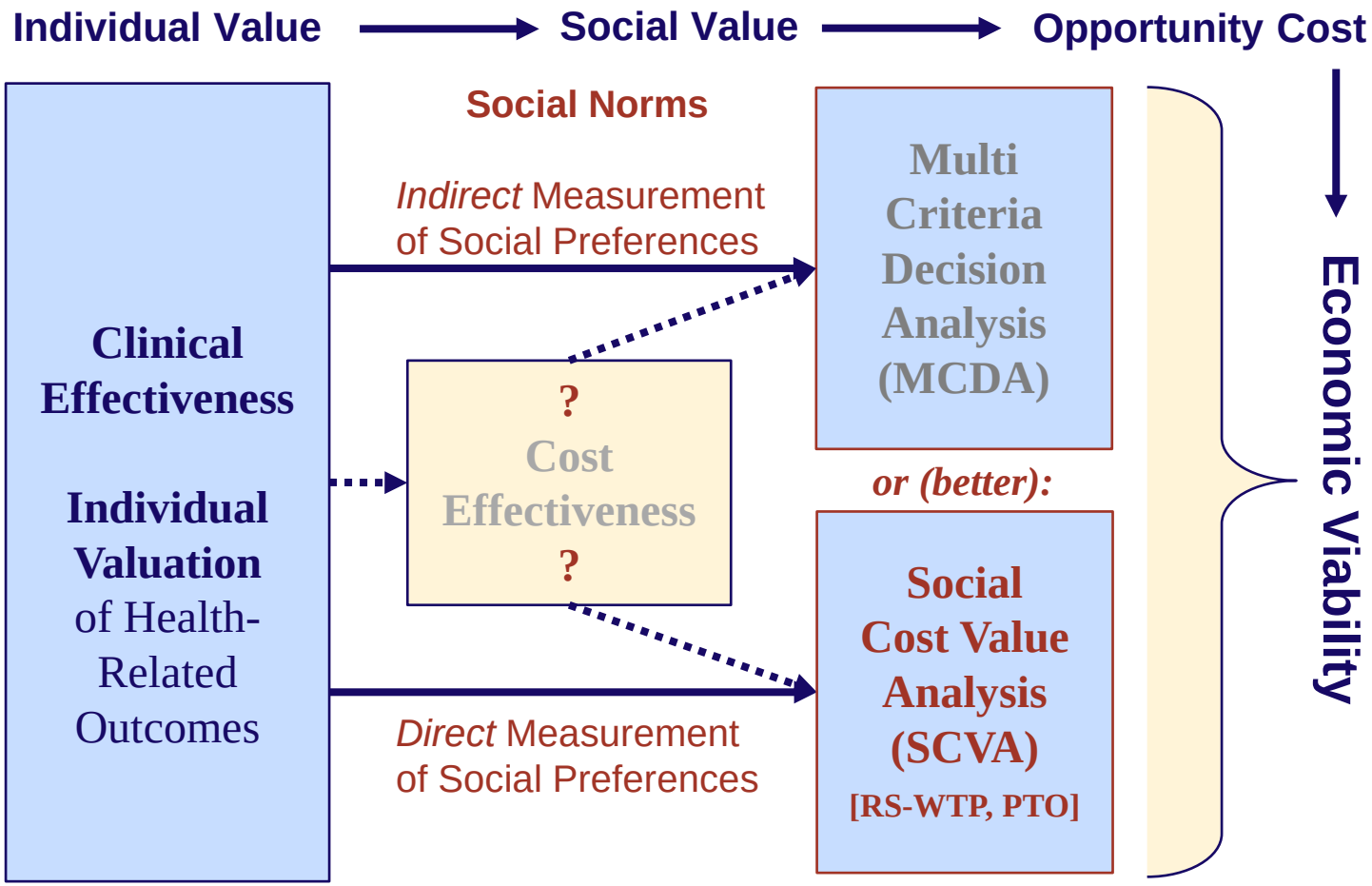
Annual budget impact of approved and pipeline drugs for ultra-rare diseases over 10 years (2012 to 2021) in Europe from a payer's perspective (Schlander et al., 2014).

A Side Note: Projected URD Budget Impact



Proportion of pharmaceutical and total health expenditures in Europe spent on drugs for ultra-rare diseases (URDs). Dashed lines indicate ranges provided by the extreme-case scenario analyses. Source: Schlander et al. (2014).

Elements of a Roadmap: Ways Forward



Elements of a Roadmap

towards **Social Cost Value Analysis (SCVA)**,
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 www.**SwissHTA**.ch)

Research Need

- many studies of social preferences …
  - most of them small
  - many studies limited in scope
  - many studies 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

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.
4. 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.

ESPM Project: Characteristics Investigated[1]

1. **Severity** of the initial health state
   (i.e., *ex ante*, before intervention)
2. **Urgency** of an intervention
   (in order to avoid major irreversible health impairments)
3. **Uncertainty** of outcomes ("risk")
   (i.e., probability of effectiveness / consequences)
4. **Clinical effectiveness** (or consequences);
   health gain; length and quality of life
5. **Age** of patient (or "fair innings")
6. **Rarity** of disorder (or fair chance of access);
   i.e., prevalence or number of persons benefitting
7. **Cost** (from different perspectives; t.b.c.)

[1]Note that concept presented here reflects status as at Feb.04, 2016, and may undergo change and revision during subsequent Work Packages.

ESPM Project: Design Elements[1]

1. **Representative population sample(s)**
2. **Discrete Choice Experiment (DCE) design**
3. **Testing for framing effects** (primarily by randomization):
   - uncertainty (certain outcomes versus specified probabilities)
   - rarity (different levels of information on implications)
   - perspective on cost (cost per patient treated vs. cost per member of a collectively financed health scheme; "zero sum" assumption)
4. **Utility comparator**
5. **Testing for potential cognitive overload**
6. **Econometric evaluation**
   - analyzing subsamples
   - latent class and random coefficient models

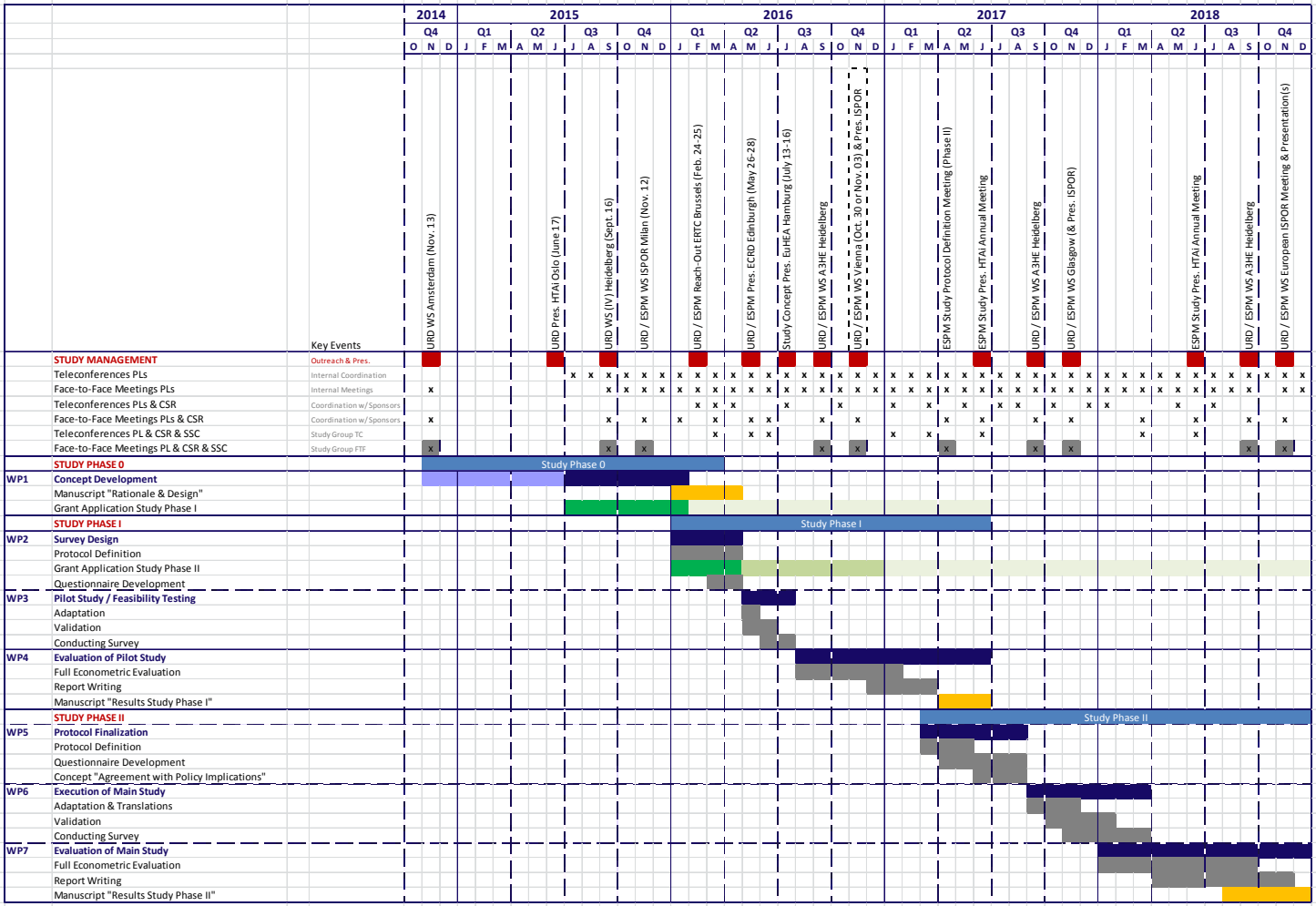
[1]Note that concept presented here reflects status as at Feb.04, 2016, and may undergo change and revision during subsequent Work Packages.

ESPM Project: Study Phases and Funding[1]

Study Phase	Work Packages	Major Activities	Funding
Phase 0 - Q1 2016	1	Concept Development	URD Project Sponsors (Biomarin and Genzyme)
Phase I Q1 2016 - Q2 2017	2-4	Pretests and Pilot Study in Switzerland; generating initial results and demonstrating feasibility of concept	Co-Funding equally split between (1) URD Sponsors and (2) SwissHTA Stakeholders (equally split between sick funds and industry)
Phase II Q2 2017 - Q4 2018	5-7	Finetuning of concept, incoporating learnings from pilot study and stakeholder input; pan-European study execution	Public/private co-funding will be sought on European level ; striving for broad stakeholder involvement

[1]Note that concept presented here reflects status as at Feb.04, 2016, and may undergo change and revision during subsequent Work Packages.

ESPM Project Plan (Tentative Overview)



Color Code:

- Study Phase
- Work Package
- Idea Generation & Concept Development
- Writing Funding Request(s)
- Grant Application Follow-Up
- Grant Application Management
- Execution of Specified Activities
- Writing Manuscripts for Publication
- Outreach & Presentation

Abbreviations:

PL: Project Leaders
CSR: Core Stakeholder Representation (cIAB)
ESR: Extended Stakeholder Representation (eIAB)
SSC: Scientific Steering Committee

ESPM: Major Steps in Study Phases I and II[1]

Selecting Social Preferences for Study	Are Experiments Understood? *"Willingness-To-Be-Taxed"*	Execution of Study Econometric Evaluation	Interpretation of Study Normative Analysis
Type of Preferences Operation-alization	Pre-Test Feasibility Parametric DCE Design *Randomization*	Validity and Rel. Weights; 'WTT'; subgroups, latent class analyses, random coefficient models	Social Norms and Social Preferences Legal and institutional context *Limitations*

[1]Note that concept presented here reflects status as at Feb.04, 2016, and may undergo change and revision during subsequent Work Packages.

ESPM Project: Who Will Benefit?

1. Health care decision-makers and payers

- seeking to incorporate the **social values of the population** covered by a collectively financed health scheme into priority-setting decisions;
- applying the **logic of cost effectiveness** with a serious interest in ist scope and ist limitations;
- interested in the exploration of the empirical rationale in favor of **alternative evaluation paradigms**, such as social cost value analysis;
- believing in the usefulness of **multi-criteria decision analysis** (MCDA) and seeking robst information on characetristics to be included in such frameworks, as well as their relative weights.

2. Policy-makers and stakeholders

- in Switzerland (Study Phase I)
- interested in the potential of increased international harmonization and integration of HTA process in Europe (Study Phase II)

3. Patients and R&D-based biopharmaceutical industry

SwissHTA Multi-Stakeholder Consensus: Hierarchy of Objectives

1. A Prior Normative Commitment
   - Starting Point: Swiss Legal Tradition
   - Human Rights / "Rights" or "Principles" -Based Approach
     1. Personality, Integrity and Autonomy of the Individuum
     2. Principles of Nondiscrimination (*Chancengerechtigkeit*)
2. Expectations of the Insured Population ("Social Preferences")
   1. "Empirical Ethics"
   2. Research Need to close gaps in our understanding
3. Operationalization of WZW Criteria
   1. W*irksamkeit* (Effectiveness)
   2. *Zweckmässigkeit* (Appropriateness)
   3. W*irtschaftlichkeit* (Economic Viability)

SwissHTA: A Prior Normative Commitment

Starting Point:

- Principle-Based Reasoning ('Rights' and 'Claims'):
  personality, integrity and autonomy of individuum
- Health as a 'Conditional Good'
  i.e., a prerequisite needed to pursue life plans
  (a normal range of opportunities)
- Echoing the Philosophical Thinking
  of Immanuel Kant, Ronald Dworkin,
  John Rawls and Norman Daniels
- Reflected in (parts of) Economic Theory
  for example by Amartya Sen and Martha Nussbaum

SwissHTA: A Prior Normative Commitment

Federal Constitution of the Swiss Federation:

- **Principle of Equality** (Article 8)

  1: Every person is equal before the law.
  2: No person may be discriminated against […]
  3: The law shall provide for the elimination of inequalities that affect persons with disabilities. .

- **Protection of Children and Young People** (Article 11)

  1: Children and young people have the right to the special protection of their integrity and to the encouragement of their development.

- **Right to Assistance When in Need** (Article 12)

  Persons in need and unable to provide for themselves
  have the right to assistance and care,
  and to the financial means required for a decent standard of living.

SwissHTA: Social Value (beyond individual health gain[1])

- Severity and Urgency of initial health problem
- "*Fair Innings*"

  interventions for children and young people who have not had an opportunity to pursue their individual life plans
  (a decent minimum of health as a "*conditional good*")
- Nondiscrimination or Fairness

  fair chance of access to effective health care
  even if condition is rare or intervention is expensive
- "*Bagatellen*"

  exclusion of or low priority for minor self-limiting health problems and 'affordable' interventions[2]
- Fast Access to Real Innovation[3]

[1]Hypotheses; SwissHTA identified a major research need;
[2]'affordability' determined from a patient's out-of-pocket perspective;
[3]'innovation' to be defined appropriately

Multi-Criteria Decision Analysis (MCDA)

There are many definitions of Health Technology Assessment (HTA).

Some Commonalities:

- A Multidisciplinary Endeavor:
  Clinical Medicine, Epidemiology, [Health] Economics, „*Policy Makers*“
- Systematic Evaluation of **Evidence of Clinical Benefit**
  of medical interventions and clinical strategies

Some Differences:

- Systematic Inclusion of Costs (…)
  of medical interventions and clinical strategies
- Types and Roles of **Economic Evaluation**

All definitions have in common that HTA (by definition) represents a variant of multi-criteria decision making.

Multi-Criteria Decision Analysis (MCDA)

There are many methods for Multi-Criteria Decision-Making.

Some Strengths:

- Integration of multiple (sometimes conflicting) objectives
- Decomposing complex decision problems
- Comprising a broad set of methodological approaches
- Building on many disciplines
  (incl. operations research, decision sciences, economics, psychology, …)

Some Problems:

- It is doubtful if any identification of the “best” MCDA method can be performed
- Appropriate consideration of opportunity cost?

Some Commonalities:

All need to be informed by

- criteria,
- weights,
- and ranking principles.

Uncertainty and Value Judgments

"It may well bring about immortality – but it will take forever to test it."

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- **SwissHTA**: *www.swisshta.ch*

Thank You for Your Attention!

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