Back to the future: should we live in a post-QALY world?

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Presenters:
- Andrew Walker
- Ad Rietveld
- Oriol Solá Morales

Session Structure

1. Andrew Walker
   Arguments for the cost per QALY

2. Ad Rietveld
   Arguments against the cost per QALY

3. Oriol Sola Morales
   Future challenges facing the cost per QALY

"I hate QALYs! They are entirely logical, when you build up the argument for them. Until you realise that what you are doing is, putting a number between 0 and 1 (and in some cases less than 0), on a person's life. That is when it all falls apart." (Former NHS Commissioner)

"The cost per QALY approach is not perfect, but it is based upon a robust, transparent and established methodology which is intertwined with the utilitarian nature of the NHS" (NHS Policy Lead)
“No issue in HTA provokes such passion as the QALY”

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In common with other systems

Focus on patient relevant endpoints such as survival and ‘quality of life’

Evaluates new medicines using a common framework

A system decision-makers can grow familiar with and becomes a second language for them

Provides a definition of value to guide medicines developers when making decisions on study design and ‘go/no go’ investments

Spotlight on value claims

‘Value’ can be one or more of improved quality life, longer survival or savings to health care budget

Shines light on case for a new treatment:
• Unmet need
• Disease-modifying
• Patient centric
• Innovative
• Granted breakthrough designation by [whoever]
Opportunity cost

If we say yes to this new treatment what do we gain (measured in QALYs)? And, by combining with data on net cost, how many QALYs do we have to give up from the money we have to take from other services?

Potential to evaluate all new treatments in the same terms
Hence maximise health gain from the health care budget

Ask those involved...

The cost per QALY is not about how much we are willing to spend but how much we are willing to take from someone else.

One man’s QALY is another man’s opportunity cost, (or even several men’s opportunity cost.)

It’s impossible to capture the value of human life in a single number like the QALY. But without it, choices are made by history and politics, and rarely with wisdom.
Debates with politicians

• A way to explain the decision made
• Transparent in the sense the calculations can be put on Powerpoint
• Helps to ensure consistency as decision-makers come and go and decisions are made by different agencies
• Helps with accountability of the agencies to government
• Partial defence when media asks why we don’t pay for every new medicine

Questions about ‘special cases’

Includes an ethical assumption health gains are of equal value irrespective of the disease and recipient

What about ‘special cases’
• Cancer
• End of life
• Start of life
• Rare diseases
• Diseases that are a bit rare but not ultra rare

Do we have a higher willingness to pay? Framework to decide
Based on society’s values

- Members of UK public took part
- TTO exercise on EQ-5D states
- Produced value set
- EQ-5D measured in clinical study, then values of UK public applied
- Shows the values used in decisions take (some) account of the opinions of the public

Economic value of a new oncology medicine

- Comparator price: £10k
- QALY gain valued at £50k/qaly: £30k
- Saving 1: £3k
- Saving 2: £1k
- Extra cost AE: £-3k
- Maximum willingness to pay: £41k
Practical advantage

- Can take a TPP
- Use predicted endpoint differences to estimate QALY gain and savings
- Convert QALY gain to £-value using guidance on willingness-to-pay
- Estimate a total willingness to pay (QALYs-in-£ plus savings)
- Consider what this needs to cover (monitoring, admin, etc)
- Divide what is left by treatment duration to give a maximum willingness to pay per unit of time

QALYs raise uncomfortable issues

- Raises key questions (e.g. about health benefit from achieving surrogate endpoint) and gives framework for discussion
- Predicting long-term benefits from RCT with limited follow-up
- Or with single-arm clinical study
- Value of certain benefits today vs uncertain benefits 20y from now
- ‘Subjectiveness of valuing health’
- Dislike of reducing things to numbers
- Adding QALYs across individuals
The QALY and payer decision-making: a good fit?

By Ad Rietveld
RJW&partners
12 November 2018

Fundamental questions that drive healthcare funding decision-making

Do we think patients need financial access to the intervention?

Given all our other priorities, can we afford to fund it?

How can we ensure that funding this intervention does not take up too much of our budget?
Regulations, rules and methodologies have been developed to assist in healthcare funding decision-making

Ad hoc (= political) decision-making ultimately leads to inequality and lack of transparency

Application of rules and accepted methodologies aim to ‘automate’ decision-making

Desired result: equality and transparent decision-making

Expectations at time of introduction of the use of the QALY for drug reimbursement decision-making

- Increase transparency
- Promote rational decision-making
- Assist resource allocation
- Make opportunity costs visible
- Maximise health gains of health insurance funds
- Provide a framework for further discussions
- Stable & predictable methodology
- Give insights in length & quality of life trade-offs
- Industry perspective – another way for supporting the value of products.
Let’s see what the value of the cost/QALY approach is for deciding on the funding of different types of new treatments

For non-innovative products cost minimization is the preferred approach
For products with important benefits, achieving an acceptable cost/QALY is usually hampered by a low comparator price

- Generally much lower-priced comparators
- High differential benefit or low price needed
- Clinical data usually sufficient for decision-making
- Budget impact will drive decision-making

For products for previously untreatable diseases, product prices are such that cost/QALYs are usually disregarded

- Often very high priced ultra-orphans
- Cost/QALY outcomes usually pushed aside
- Clinical data, a high price and low patient numbers usually enough information for decision-making?
- Prioritization of treatments to fund?
Irrespective of the cost/QALY, budget impact will be the main hurdle for curative treatments

- Very high priced, increasingly personalized medicine
- High ‘upfront’ payment and patient numbers are a problem
- Budget impact will be main issue
- Prioritization of treatments to fund?

Irrespective of any cost/QALY outcome, budget impact will be the main hurdle for preventative treatments

- Patient numbers are a problem
- Budget impact will be main issue
- Prioritization of treatments to fund?
Healthcare decision-making is actually not so much about budgets as it is about ethics

- Do we think patients need financial access to the intervention?  
  Ethical

- Given all our other priorities, can we afford to fund it?  
  Ethical

- How can we ensure that this intervention does not take up too much of our budget?  
  Financial / ethical

Usefulness of the cost/QALY is hampered by a fundamental contradiction......

- Cost/QALY analysis is a technological approach for assessing the monetary value of an investment in a medical intervention
- but
  Most of healthcare funding decision-making is not about investments but about ethics
- Cost/QALY analysis is applied as a technological solution to an ethical problem
- Technology cannot solve ethical dilemmas
- No universally accepted algorithms exist for healthcare prioritization
If it is not cost/QALY, what then?

- Focus on robust clinical data support allowing for clinical comparisons and assessment of clinical benefits
- Use of conditional reimbursement as a way of dealing with uncertainty on benefits and supporting data at launch
- More flexibility in price negotiations and contracting options with less importance attached to list prices
- New pricing models urgently required to deal with personalized medicine and gene therapies
- Prepare for increasing demand for transparency of setting prices

*Priority in decision-making will remain event driven and political*

Let’s stop asking industry to throw in more data and additional analyses – decomplicate!
QALY and future demands

Personal position on CEA

- It is far from being perfect

- Costs and utilities are prone to many uncertainties / methodological limitations
Personal position on CEA

• It is far from being perfect
  • Do we value equal paediatric and elderly care?
  • Is cancer equal to high blood pressure?
  • QALYs do not relate to VALUE

• ..... But you may want to take an extra-welfarist approach
  • It is not (pareto) optimal, but it is better than not having it

• CEA is a good tool in combination with a full HTA analysis
  • Provides good framework analysis
  • Enables comparison
  • Facilitates repetition
  • Obliges to make explicit decision-making, and therefore to justify deviations from the criterion
Potential usages of Economic Evaluation

EE can / should still be used in

• Early Market Access
  • To model the potential outcomes of drug / device development
  • To model sensitivity analysis and complete investment decisions

• In developing economies
  • To ensure most efficient technologies are introduced
  • To avoid non efficient technologies
  • To improve transparency
  • To help build the healthcare system

New Market Access Requirements

What to do in an unsustainable situation? What are the new challenges? What to do?
What can we do?

<table>
<thead>
<tr>
<th>No one does Nothing (Business as Usual)</th>
<th>Do Something Transformational</th>
<th>Be Reactive</th>
</tr>
</thead>
<tbody>
<tr>
<td>The Alzheimer’s Paradox</td>
<td>Demand Side Policies</td>
<td>Cannibalism</td>
</tr>
</tbody>
</table>

Cannibalism

What’s in a loaf of bread?

<table>
<thead>
<tr>
<th>Ingredient</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td>Water</td>
<td>10 €</td>
</tr>
<tr>
<td>Flour</td>
<td>20 €</td>
</tr>
<tr>
<td>Heat</td>
<td>15 €</td>
</tr>
<tr>
<td>Salt</td>
<td>5 €</td>
</tr>
<tr>
<td>TOTAL</td>
<td>50 €</td>
</tr>
</tbody>
</table>

- What happens when the government decides it has to introduce Flour in bread to prevent caries?
Cannibalism

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<tr>
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<tr>
<td>Flour</td>
<td>18 €</td>
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<tr>
<td>Heat</td>
<td>13 €</td>
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<tr>
<td>Salt</td>
<td>4 €</td>
</tr>
<tr>
<td>Flour</td>
<td>7 €</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>50 €</strong></td>
</tr>
</tbody>
</table>

- Everybody loses.
- The (public) insurer will limit its expenditure / ability to pay to a fixed amount (OECD minimum)
- It will maximise competition, driving production costs up.

The Alzheimer’s Paradox

- Under current market perspectives, manufacturers may want to maximise their revenue, and compensate for previous losses.
  - This is leading to 10% GDP average annual increase
- The potential market is huge
- The potential price is huge
- **The potential risk for no-approval is huge**
The Alzheimer’s Paradox

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Managing Demand: Priority Setting Exercises

<table>
<thead>
<tr>
<th>Dimension</th>
<th>Weight (%) 2010</th>
<th>Dimension</th>
<th>Weight (%) 2012</th>
</tr>
</thead>
<tbody>
<tr>
<td>Impact on QoL</td>
<td>38</td>
<td>Clinical Impact</td>
<td>66</td>
</tr>
<tr>
<td>Risk of no Treatment</td>
<td>30</td>
<td></td>
<td></td>
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<tr>
<td>Waiting Time</td>
<td>8</td>
<td>Clinical Effectiveness</td>
<td>13</td>
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<tr>
<td></td>
<td></td>
<td>Expected Benefit</td>
<td>12</td>
</tr>
<tr>
<td>Resource Use</td>
<td>11</td>
<td>Social Role</td>
<td>22</td>
</tr>
</tbody>
</table>

Setting priorities sends a clear message to the EcoSystem and allows alignment of policies to results
Demand Side Policies

- Pre-commercial Procurement
- Innovative Public Purchasing

Demand Side Policies

- Purchasers may sign pre-commercial agreements
  - Lowers the cost of (borrowing) money
  - Reduces commercial costs
  - Ensures ‘early adopters’ / experience (reduces commercial uncertainty)

- There are societal benefits
  - Needs are fulfilled
- And business benefits
  - Companies make the same profit at less cost /risk
Demand Side Policies

- Under agreed developments ...
  - Incentives are possible
    - OMP legislation
    - Antibiotic development incentives
    - GAVI alliance
  - Externalities appear
    - New markets are developed
    - Triggers science
    - Rewards Universities / RnD
    - Fulfils Lisbon treaty (knowledge based society)
Example: Hypercholesterolemia

- Need: high prevalence, high related mortality
- Gene- autosomal dominant pattern
  - Polygenic, but LDLR, APOB, or PCSK9 have been identified
- Can we develop a gene therapy?
  - Define potential targets
  - Define timings, expectations
  - Ensure access + reimbursement
  - Would outcomes be better than for PCSK9i

Example: ADA-SCID

- The treatment was developed at San Raffaele Telethon Institute for Gene Therapy and developed by GlaxoSmithKline (GSK) through a 2010 collaboration with Fondazione Telethon and Ospedale San Raffaele.
- Italian authorities
  - co-develop it
  - accept to pay for it
  - Speed access
  - Price is ‘better’
- MOLMED is a biotechnology company founded in 1996 in Milan.
What **shall** we do?

High Cost
Limited Value

Low Cost
High Value

High ICER

Low ICER

High Cost
Limited Value

High ICER

Demand Side Policies

Early MA

Develops HC (eco)Systems

High Cost
Limited Value

Low Cost
High Value

Low ICER

High ICER
Discussion and Q&A