ASSESSING THE ADDED VALUE OF HEALTH TECHNOLOGIES
Reconciliationing Different Perspectives

Michael Drummond
Centre for Health Economics, University of York
Acknowledgements

- Rosanna Tarricone
  Director and Associate Professor, CeRGAS

- Aleksandra Torbica
  Assistant Professor, CeRGAS
Introduction to the Issues

- **Patients**: Value therapies that improve their length or quality of life. Want to recoup their investments in research by charging a premium price for their products.

- **Payers**: Seek to justify their decisions by comparing the extra benefits provided by the therapy with the extra costs (Health Technology Assessment).

- **Manufacturers**: Want to recoup their investments in research by charging a premium price for their products.

Mismatch of expectations???
Reconciling the Value Positions

Patients

- Increasing patient participation in HTA

- Aligning research with social objectives

Payers

Rethinking the notion of value in healthcare

Manufacturers

Aligning research with social objectives
1. Rethinking the Notion of Value in Healthcare

- The quality-adjusted life-year (QALY) became health economists’ ‘big idea’ when cost-benefit analysis was abandoned.

- Several methodological problems with QALYs.

- The QALY may not capture all the relevant aspects of the social value of healthcare investments (eg. benefits other than health gain, equity considerations).
The Relationship Between Social Value and Incremental Cost Per Quality-Adjusted Life-Year (QALY)
# Incremental Cost Per Additional Life-Year Gained: League Table

<table>
<thead>
<tr>
<th>Number</th>
<th>Incremental cost per additional life-year gained at 1998/1999 prices ($AU)</th>
<th>PBAC decision</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>5517</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>2</td>
<td>8374</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>3</td>
<td>8740</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>4</td>
<td>17387</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>5</td>
<td>18762</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>6</td>
<td>18983</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>7</td>
<td>19807</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>8</td>
<td>22255</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>9</td>
<td>26800</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>10</td>
<td>38237</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>11</td>
<td>39821</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>12</td>
<td>42697</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>13</td>
<td>43550</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>14</td>
<td>43550</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>15</td>
<td>43550</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>16</td>
<td>56175</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>17</td>
<td>57901</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>18</td>
<td>63703</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>19</td>
<td>71582</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>20</td>
<td>75286</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>21</td>
<td>85385</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>22</td>
<td>88865</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>23</td>
<td>98323</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>24</td>
<td>229064</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>25</td>
<td>231650</td>
<td>Recommend at price</td>
</tr>
<tr>
<td>26</td>
<td>256950</td>
<td>Recommend at price</td>
</tr>
</tbody>
</table>

$AU$ = Australian dollars. The average interbank exchange rate to US dollars for 1998/1999 was 0.63772 (range 0.68760 to 0.54850).

PBAC = Pharmaceutical Benefits Advisory Committee.

NICE’s Supplementary Guidance for “End of Life” Therapies

• If the therapy:
  - is for a small patient population with life expectancy of less than 24 months;
  - where the therapy adds three months or more to life expectancy.

• Then:
  - the QALYs gained should assume full quality of life in the added months;
  - in addition the Committee can consider that the QALYs gained should be weighted sufficiently high for the therapy to be approved given NICE’s current threshold.
Alternatives to the QALY

- Cost-consequences analysis (using the main clinical outcomes).
- Contingent valuation (willingness-to-pay).
- Discrete choice experiments (conjoint analysis).
Recent Developments in Health Benefit Measurement

- Continuing research into discrete choice experiments and other stated preference techniques (eg RTI, University of Sydney, University of Aberdeen, Johns Hopkins).


- Projects commissioned by IQWiG to explore the relative weighting of outcomes within therapeutic areas.

- Still only limited experience of the use of these approaches in decision-making.
Patients’ Preferences for Attributes of Hepatitis C Therapy
(Source: Muhlbacher, 2011)
Comparison of Patients’ and Clinical Experts’ Opinions (Source: Muhlbacher, 2011)
Incorporating Equity Considerations

• Develop a series of distributive weights for QALYs?
  Wailoo et al Pharmacoeconomics 2009;27:983-9

• Establish a ‘deliberative’ decision-making process to incorporate other relevant factors (beyond the incremental cost per QALY)?
  Culyer Health Economics Politics and Law 2006;1:299-318

• Encourage more transparency and public debate about healthcare resource allocation decisions?
  Drummond et al Int J Tech Assess Health Care 2007;23:36-42

• Conduct an ‘equity analysis’ alongside the economic evaluation?
2. Aligning Manufactures’ Research with Social Objectives

- In the past, much research has been about pursuing a clinical hypothesis, rather than meeting an identifiable health need.

- The main emphasis in companies has been to design clinical studies to meet the needs of the regulator (eg FDA, EMA).

- In Europe, there is a trend towards requiring studies that will be more useful to payers, clinicians and their patients.

- A paradigm shift in manufacturers’ clinical development programmes is required.

- Some companies are responding to this challenge.
The Trend Towards Assessments of Relative Efficacy and Effectiveness in Europe

- The EU High-level Pharmaceutical Forum has:
  - Endorsed the aim of relative effectiveness assessments to characterise added therapeutic value.
  - At the same time, acknowledged the distinction between relative effectiveness assessments and health economic assessments of costs and benefits.

- The European Medicines Agency is now calling for more assessments of 'relative efficacy' as part of its procedures, in order to produce summaries of clinical value (e.g. European Public Assessment Reports) that are of more relevance to payers.

- The EMA and payers have been holding discussions to explore the extent to which their respective data needs can be met.
Anticipated Evolution of Relative Efficacy Assessment at the Interface Between Regulatory Agencies and Payers
Possible Impacts on the Clinical Development Process

- Fewer placebo-controlled trials.
- More superiority studies.
- More arms in trials, or more trials in total.
- Measurement of endpoints of more interest to payers/patients.
- Longer duration of trials.
- More ‘pragmatic’ trial designs.
Actions Manufacturers Should Consider

Internally
- review clinical trial plans with payers’ needs in mind.
- make sure that the clinical evidence from the trials supports the desired product positioning.
- review the clinical portfolio to assess the potential for comparative studies, studies using different endpoints or studies conducted in ‘real-world’ settings.

Externally
- engage in early dialogue with payers as well as regulators, prior to deciding on Phase III trial designs.
Early Dialogue Between Manufacturers, Payers and Regulators

• A major pharmaceutical company produced a dossier of a product in early clinical development.

• The dossier was shown to 2 regulatory agencies and 7 payers in 5 countries.

• Questions were asked about:
  - the most relevant target population
  - the alternative to which the drug should be compared
  - the outcomes that should be measured
  - the length of follow-up in the trials
  - the relevant sub-groups in the patient population

• Several interesting differences in perspective were identified but, overall, an evidence generation strategy satisfying all parties could be defined.

• Further studies are required in different therapeutic areas. (See Backhouse et al Value in Health 2011;14:608-15)
3. Increasing Patient Participation in HTA

• Patients are regarded as important stakeholders in HTA.

• It is often that the patient’s perspective would be a useful addition to the scientific assessments that are made.

• The main question is that of how to secure effective patient input.
Is Patient Participation Conditioned by the Type of Healthcare System?

- ‘Patient-focused’ systems
  - individuals select their health plan
  - substantial co-payments
  - wide range of choice, providing patient is willing-to-pay

- ‘Population-focused’ systems
  - single health plan for all publicly-insured
  - limited co-payments
  - choice from among a range of publicly-approved options only
What Information from Patients is Useful in HTA?

• Preferences for health states (for incorporation into societal/health plan decision-making).

• Information about costs borne by the family in seeking/receiving care
  - out-of-pocket expenses
  - time costs
  - informal care

• Views on the experience of care
  - involvement in the care process
  - convenience of different forms of treatment (eg oral versus IV infusion, different dosing schedules)
How Can Patients be Involved in HTA?

- Membership of expert committees.
- Scoping of technology assessments.
- Commenting on draft reports.
- Appealing the reimbursement/coverage decision.
Is there Evidence that Engaging Stakeholders Makes a Difference?

• Adds to the integrity of the process of HTA.
• Can help identify mistakes (e.g. NICE’s technology appraisal of treatments for glioma).
• Can provide evidence that is not available in the public domain (e.g. manufacturers).
• Can help improve the wording and balance of key recommendations.
• Appeals can change decisions.
# Summary of NICE Appeal Decisions

1 March 2000 to 31 October 2011

<table>
<thead>
<tr>
<th>Category</th>
<th>Number of appeals</th>
<th>(% of total)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Dismissed:</strong> Final guidance published</td>
<td>25</td>
<td>(35%)</td>
</tr>
<tr>
<td><strong>Request changes</strong> to final appraisal but no further consideration by the Appraisal Committee</td>
<td>21</td>
<td>(30%)</td>
</tr>
<tr>
<td><strong>Upheld:</strong> final appraisal returned to the Appraisal Committee</td>
<td>23</td>
<td>(32%)</td>
</tr>
<tr>
<td><strong>Referred</strong> to the Department of Health</td>
<td>2</td>
<td>(3%)</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>71</td>
<td></td>
</tr>
</tbody>
</table>
Key principles for the improved conduct of health technology assessments for resource allocation decisions

Michael F. Drummond
University of York

J. Sanford Schwartz
University of Pennsylvania

Bengt Jönsson
Stockholm School of Economics

Bryan R. Luce
United BioSource Corporation

Peter J. Neumann
Tufts University

Uwe Siebert
UMIT—University for Health Sciences, Medical Informatics and Technology

Sean D. Sullivan
University of Washington
Principle 10. Those conducting HTAs should actively engage all key stakeholder groups (eg professional bodies, patient organizations, manufacturers).

- Is the HTA organization formally required to engage stakeholders in its activities?
- Does the HTA organization involve stakeholders in the scoping of HTAs?
- Does the HTA organization have a mechanism for identifying the relevant stakeholders?
- Does the organization encourage or require submissions of evidence from stakeholders?
- Does the organization allow stakeholders to comment on reports at the draft stage?
- Does the organization allow stakeholders to appeal against recommendations/decisions?
- Do the organization’s committees include stakeholder representation (eg patient groups, technology manufacturers, clinical specialists)?
Conclusions

- Different parties (ie patients, payers and manufacturers) have differing perspectives on the value of therapy.
- It is important that these different value positions are recognised.
- The various value positions can be reconciled by taking the initiatives identified here.