COMPARATIVE EFFECTIVENESS FOR REIMBURSEMENT: A COMPARISON OF INTERNATIONAL POLICIES AND METHODS

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HTAi Symposium, Dublin, June 2010
Defining ‘comparative effectiveness’

AHRQ

“Comparative effectiveness research is designed to inform health-care decisions by providing evidence on the effectiveness, benefits, and harms of different treatment options. The evidence is generated from research studies that compare drugs, medical devices, tests, surgeries, or ways to deliver health care.” (AHRQ, 2010)
Defining ‘comparative effectiveness’

Congressional Budget Office

• ...a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients....The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options. (Congress of the United States Congressional Budget Office, 2007).
For our purposes...

- Establishing differential health benefit
- Necessary whether cost-effectiveness is relevant to the decision or not
- Focus on the payer’s decision
  - Effectiveness in routine use
  - Against existing forms of management
Objectives of the Symposium

- Share perspectives on CER from different jurisdictions
- Address conflicts between regulation and reimbursement
- Consider methodological issues in CER
- Discuss the transferability of CER across jurisdictions
- Identify methodological and policy research questions
Our speakers

• **Adrian Towse** (OHE, London, UK)
  – The European Vision: Increasing needs in Comparative Effectiveness from European HTA authorities

• **Clifford Goodman** (Lewin Group, Falls Church, Virginia, USA)
  – The US Vision: The comparative effectiveness in the US today and potential directions for the future

• **Bong-Min Yang** (Seoul National University, South Korea)
  – The Asian Vision: How does comparative effectiveness fit in HTA in Asia?
Some key features of CER

• Health outcomes in
  – Routine practice
  – Routine patients
• Compared with standard interventions
• To support decisions
Some methodological challenges

• Measuring health
• The challenge of comparators
• Assessing transferability of evidence across jurisdictions
• Recognizing heterogeneity in effectiveness
• Dealing with uncertainty in evidence
• Prioritising research
• Appropriately rewarding innovation
## The need for a summary measure (net benefit)

<table>
<thead>
<tr>
<th></th>
<th>Treatment A</th>
<th>Treatment B</th>
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<tbody>
<tr>
<td>Diarrhoea</td>
<td>Moderate</td>
<td>Absent</td>
</tr>
<tr>
<td>Hot flushes</td>
<td>Present, mild</td>
<td>Present, mild</td>
</tr>
<tr>
<td>Breast swelling</td>
<td>Absent</td>
<td>Present</td>
</tr>
<tr>
<td>Physical energy</td>
<td>Lacking energy</td>
<td>No problems</td>
</tr>
<tr>
<td>Life expectancy</td>
<td>Option A better by 2 months</td>
<td></td>
</tr>
</tbody>
</table>
Net benefit – what are the options?

- Need for single, generic scale
- Needs to avoid being arbitrary – reflect individual preferences
- Widely used options
  - The quality-adjusted life year (QALY)
  - Discrete choice/conjoint methodology
Example – HRT and breast cancer

Minelli et al. BMJ, 2004;328:371
The challenge of comparators – network of evidence

<table>
<thead>
<tr>
<th>No. of RCTs</th>
<th>SK</th>
<th>t-PA</th>
<th>Acc t-PA</th>
<th>Sk+t-PA</th>
<th>r-PA</th>
<th>TNK</th>
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<td>✓</td>
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<td></td>
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</tr>
</tbody>
</table>

Acc t-PA = accelerated alteplase; r-PA = reteplase; SK = streptokinase; Sk+t-PA = streptokinase + alteplase; TNK = tenecteplase; t-PA = alteplase.

Caldwell et al. BMJ 2005; 331: 897-900
The challenge of comparators – presenting results

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Fixed effect (%)</th>
<th>35-day mortality</th>
<th>probability treatment the best</th>
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<tbody>
<tr>
<td>SK</td>
<td>6.5</td>
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<td>0</td>
</tr>
<tr>
<td>t-PA</td>
<td>6.4</td>
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<tr>
<td>Acc t-PA</td>
<td>5.6</td>
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<td>40</td>
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<td>SK+t-PA</td>
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<td>r-PA</td>
<td>5.8</td>
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<td>15</td>
</tr>
<tr>
<td>TNK</td>
<td>5.6</td>
<td></td>
<td>43</td>
</tr>
</tbody>
</table>

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Caldwell et al. BMJ 2005; 331: 897-900
Generalisability across jurisdictions

- Preferred methods may vary
- Decision problem may vary
  - Comparators
  - Context of use
- Estimates might vary
  - Baseline risk
  - Relative treatment effect
  - Preferences
- Implications for design of studies
Example – the ATLAS study