Priority setting: towards evidence-informed deliberative processes

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Outline

• Assessing the value of health technology to inform decision-making

• Towards evidence-informed deliberative processes
A drug that cures doesn’t do any good if the people who need it...

Countries with HPV vaccine in the national immunization programme

- **Introduced** to date (129 countries or 66.5%)
- **Not Available, Not Introduced** (50 countries or 33.5%)
- **Not applicable**

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**DA VINCI SURGICAL ROBOT**
Is High Cost Justified by Benefits?

Negatives or costs
Priority setting – value frameworks

• HT assessed by a judgment on the *relative importance* of certain criteria that may differ between stakeholders, countries
• The criteria considered traditionally include:
  - the level of clinical benefit
  - and in some cases a measure of the incremental cost-effectiveness
• Recently also indirect, unintended or ‘hidden’ outcomes (e.g. potential benefits and harms for other stakeholders), and ethical, legal, and organizational issues
• Criteria potentially differ across conditions and type of technology (e.g. end-of-life medicines in the UK)
Different value frameworks around the globe

- Frameworks have evolved over the years by using public consultation / multiple stakeholder involvement
- HTA agencies around the globe intend to support population health / payer considerations (coverage decisions)
- US: mainly targeting patient-clinician conversations (e.g. ASCO, NCCN)
- Other ‘emerging’ frameworks >>> often not used in practice

# Value frameworks: evidence-informed…

<table>
<thead>
<tr>
<th>Indication</th>
<th>Brand name (generic)</th>
<th>Added benefit</th>
<th>Equal benefit</th>
<th>Added benefit</th>
<th>Equal benefit</th>
<th>HTA recommendation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Breast cancer</td>
<td>Eribulin</td>
<td>Equal benefit</td>
<td>Added benefit</td>
<td>Added benefit</td>
<td>Negative</td>
<td>Negative</td>
</tr>
<tr>
<td>Colorectal cancer</td>
<td>Aflibercept</td>
<td>Added benefit</td>
<td>Equal benefit</td>
<td>Not assessed</td>
<td>Negative</td>
<td>Negative</td>
</tr>
<tr>
<td>Melanoma</td>
<td>Ipilimumab</td>
<td>Added benefit</td>
<td>Added benefit</td>
<td>Added benefit</td>
<td>Positive</td>
<td>Negative</td>
</tr>
<tr>
<td>Non-small cell lung cancer</td>
<td>Crizotinib</td>
<td>Equal benefit</td>
<td>Added benefit</td>
<td>Not assessed</td>
<td>Negative</td>
<td>Negative</td>
</tr>
<tr>
<td>Prostate cancer</td>
<td>Abiraterone</td>
<td>Added benefit</td>
<td>Added benefit</td>
<td>Equal benefit</td>
<td>Positive</td>
<td>Negative</td>
</tr>
<tr>
<td>Renal cell carcinoma</td>
<td>Axitinib</td>
<td>Added benefit</td>
<td>Added benefit</td>
<td>Not assessed</td>
<td>Positive</td>
<td>Negative</td>
</tr>
</tbody>
</table>

Towards evidence-informed deliberative processes (EDPs)
“It is the decision-making process that warrants the legitimacy of (reimbursement) decisions and not only the robustness of evidence or the formal procedure followed”


- EDPs combines two existing frameworks:
  - A4R – deliberative process to identify relevant values
  - MCDA – Rational decision-making through evaluation of identified values
Evidence-informed deliberative processes
Step-by-step practical guide for HTA organisations

Version: 1
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Value Assessment Frameworks for HTA Agencies: The Organization of Evidence-Informed Deliberative Processes
Rob Baltussen, PhD, Maarten Paul Maria Jansen, MSc, Leon Bijlmakers, PhD, Janneke Grooters, PhD, Anneke Kruymans, MSc, Rob P. Ruuska, PhD, Marita Timmers, PhD, Gerrit Jan van der Will, Msc (Tei), PhD
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Progressive realisation of universal health coverage: what are the required processes and evidence?
R Baltussen,1 M P Jansen,1 L Bijlmakers,1 N Tromp,1,2 A E Yamin,3,4 O F Norheim2,5

Stakeholder Participation for Legitimate Priority Setting: A Checklist
Maarten P.M. Jansen1, Rob Baltussen1, Kristine Barrow3

Multi criteria decision analysis to support HTA agencies – benefits, limitations and the way forward
Baltussen R1, Marsh K1, Thokala P1, Diaby V1, Castro Jaramillo H1, van der Cleemput P1, Garau M1, Goethebeur M1, Iskrov G1,2, Manesh A1, Mirelman A1,2, Mobilizadeh M1,3, Morton A1,3, Tringali M1,3, van der Wijk J3, Valentim J3, Wagner M1, Youngkong S1,3, Zah V1, Toll A1, Jansen1, Leon Bijlmakers1, Wija Oortwijn1, Henk Broekhuizen1

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How to do it?

- Targeting HTA agencies

- Five steps
  - Methodological guidance and best practices in each step

- Most countries already have processes in place
  - And can improve – ‘menu of options’
Examples of countries *perceived* as “best practices”

Note: there is **no single country** that may be referred to as best practice country for its system as a whole. A country may only be a best practice for a (subset of) element(s).

Step 1: Appraisal committee

- Process guidelines
- Ideally involving relevant stakeholders as members
- Many other options to involve stakeholders
- Decision-makers remain responsible
  - consensus not required
  - stakeholder dominance

Examples: 🇧🇷 🇨🇦 🇳🇱 🇳゜ 🇬🇧
Step 2: Selecting of technologies and criteria

- Selection of interventions for evaluation
- Selection of criteria
  -> generic and contextual

Examples: 🇨🇦 🇪🇸 🇹🇭 🇬🇧
Step 3: Assessment

- Evidence collection
- On all identified relevant criteria
- Quantitative or qualitative information

Also:
- Use of evidence reports
- Stakeholder involvement

Examples: 🇦🇺 🇨🇦 🇩🇪 🔴 🇬🇧
Step 4: Appraisal

• How can an appraisal committee balance all relevant criteria?

• ‘Menu of options’ – different impact on quality, consistency and transparency
  Always: deliberate to assess evidence and make judgements on criteria
  1. Interpretation of criteria – e.g.
  2. Use of criteria weights (E-European agencies)
  3. Use of simple decision rules – e.g.

• Depending on the context, HTA agencies can make their own choice
Step 5: Communication & appeal

• Make all decisions and underlying argumentation public
• Install mechanisms for appeal
• Monitoring and evaluation

Examples: 🇪🇺️ 🇫🇷 🇬🇧 🇦🇪 🇪🇸
Added value...

• Explicit focus on legitimacy

• Theoretically sound, very practical

• Decision-making values visible

• Explicit guidance on balancing values
Key messages

• EDPs can facilitate legitimate decision-making:
  • It supports organizations to be more systematic, explicit and transparent, by making recommendations/decisions sensitive to a wider range of needs and values, and by promoting consistency across decisions

• Transparency and explicitness of processes and methods will not automatically appear - it takes time

• Broad, multidisciplinary, stakeholder involvement will be necessary throughout the process, and should start from the beginning of the HTA process to determine relevant criteria

• It will increase accountability and predictability for all stakeholders
THANK YOU!

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