The decision making process and the application of value judgments

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# Process matters! The “ideal situation”…?

<table>
<thead>
<tr>
<th>Principles</th>
<th>Putting them into practice...</th>
</tr>
</thead>
<tbody>
<tr>
<td>Independence</td>
<td>“Arm’s length” from government, payers, industry and professional groups; strong and enforced conflict of interest policies</td>
</tr>
<tr>
<td>Transparency</td>
<td>Meetings open to the public; material placed on the web; decision criteria and rationale for individual decisions made public</td>
</tr>
<tr>
<td>Inclusiveness</td>
<td>Wide and genuine consultation with stakeholders; willingness to change decision in light of new evidence</td>
</tr>
<tr>
<td>Scientific basis</td>
<td>Strong, scientific methods and reliance on critically appraised evidence and information</td>
</tr>
<tr>
<td>Timeliness</td>
<td>Decisions produced in reasonable timeframe; minimise delays in publishing decisions</td>
</tr>
<tr>
<td>Consistency</td>
<td>Same technical and process rules applied to all cases</td>
</tr>
<tr>
<td>Legal framework</td>
<td>Reference in country’s legal framework; institutional role in informing coverage and payment decisions</td>
</tr>
<tr>
<td>Regular review</td>
<td>Regular updating of decisions and of methods</td>
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</tbody>
</table>
NICE: Evidence assessment to decision making ("Appraisal")...

- Published evidence

- Unpublished evidence; expert input; industry submissions

- Identification, critical appraisal and synthesis of clinical and economic evidence

- University group or professional association/Royal College

- Standing (or ad hoc) independent advisory committee/expert group

- Healthcare professional groups

- Patients and service users

- Policy making: evidence, values, UK reality

- Academia

- Industry

- NHS; public sector
Stakeholder engagement…

Consultation

“Scoping” ➔ Submission

Review

“Decision” ➔ “Appraisal”

Assessment
What are stakeholders?

A large constituency

- Those responsible for delivering the care (professionals, managed care programmes).
- Those receiving it (consumers or patients and their caregivers).
- Those financing it (governments, health insurers, the public, and employers).
- Those managing care (policy makers, public health services).
- Those monitoring care
- Employers
- Pharmaceutical/device industry
Why involve stakeholders? (1)

• Evidence is imperfect
  – Varying quality
  – Complex to interpret
  – May not address appropriate outcomes

• HTA based decisions/recommendations are constructed through a *deliberative process*
  – Evidence rarely translates directly into recommendations
  – Process includes consideration of evidence quality, weighing harms & benefits
  – Also includes preferences, values, judgments
  – A process that should be inclusive…
Why involve stakeholders? (2)

• HTA outputs are interventions
  – Aim at improving outcomes
  – Excluding stakeholders’ perspective jeopardizes uptake

• The process needs to be transparent
  – Conflicts of interest need disclosing
  – Involving the public opens the process to scrutiny

• HTA outputs can have “policy status”
  – E.g. linking recommendations to contractual obligations and / or “pay-for-performance” incentives
  – Guidance can challenge professional freedom and commercial interests (e.g. drug companies) → may take legal action
NICE “Social value judgements”: principles for the development of NICE guidance

• Principles that NICE should follow in designing the processes it uses to develop its guidance and in developing individual pieces of guidance.

• Mainly about the judgements that NICE and its advisory bodies should apply when making decisions about the effectiveness and cost effectiveness of interventions, especially where such decisions affect the allocation of NHS resources.

• The current (2008) edition of Social Value Judgements is the second and it pre-dates the Equality Act 2010. The Act's requirements now govern NICE's application of social value principles when considering legally protected groups.

• Eight principles – together fulfil the requirements of ‘accountability for reasonableness’.
SVJ on… *Evidence*

- NICE should not recommend an intervention (that is, a treatment, procedure, action or programme) if there is no evidence, or not enough evidence, on which to make a clear decision. But NICE’s advisory bodies may recommend the use of the intervention within a research programme if this will provide more information about its effectiveness, safety or cost (Principle 1).
SVJ on…individual choice

• Although NICE accepts that individual NHS users will expect to receive treatments to which their condition will respond, this should not impose a requirement on NICE’s advisory bodies to recommend interventions that are not effective, or are not cost effective enough to provide the best value to users of the NHS as a whole (Principle 5).
SVJ on… Responding to comments and criticism

• NICE should consider and respond to comments it receives about its draft guidance, and make changes where appropriate. But NICE and its advisory bodies must use their own judgement to ensure that what it recommends is cost effective and takes account of the need to distribute health resources in the fairest way within society as a whole (Principle 6).
Role of cost effectiveness in NICE guidance

• “Those developing *clinical guidelines, technology appraisals or public health guidance* must take into account the relative costs and benefits of interventions (their ‘cost effectiveness’) when deciding whether or not to recommend them.” (Principle 2, SVJ, NICE 2008)

BUT

• “Decisions about whether to recommend interventions should *not be based on evidence of their relative costs and benefits alone*. NICE must consider other factors when developing its guidance, including the need to distribute health resources in the fairest way within society as a whole.” (Principle 3) → Equity issues!

• See: [http://www.nice.org.uk/media/C18/30/SVJ2PUBLICATION2008.pdf](http://www.nice.org.uk/media/C18/30/SVJ2PUBLICATION2008.pdf)
Why exploring equity is important for decisions

• Decisions in health and health policy will often reflect issues beyond efficiency
• Should we provide equal access to those with equal need?
• Should we provide different access to those with differing need?
• Do we have an accurate idea of need:
  – capacity to benefit, burden of disease, availability of alternatives…?
**NICE cost-effectiveness threshold**

<table>
<thead>
<tr>
<th>Probability of rejection</th>
<th>Cost per QALY (£’000)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>10</td>
</tr>
<tr>
<td></td>
<td>20</td>
</tr>
<tr>
<td></td>
<td>30</td>
</tr>
<tr>
<td></td>
<td>40</td>
</tr>
<tr>
<td></td>
<td>50</td>
</tr>
</tbody>
</table>

- **Rituximab for follicular lymphoma**
- **Imatinib for chronic myeloid leukaemia (blast phase)**
- **Trastuzumab for early stage HER-2 positive breast cancer**
What will be the threshold in future???

- In 2010, University of York, Imperial College and Office of Health Economics begins project on the empirical estimation of NHS cost effectiveness threshold
- Research supported by the Medical Research Council
- Final report now available…
- Builds on econometrics work by Martin et al (2008) using NHS “Programme Budgeting” data:
  - Information on NHS spending in 23 broad programmes of care
  - Explores relationship between local spending on particular types of care and health outcomes


Call to cut NHS price cap for new drugs
By Andrew Jack

Ministers are set to face fresh pressure to lower the price threshold above which new medicines are rejected for the National Health Service, amid claims that pharmaceutical companies are charging too much for groundbreaking treatments.

In research to be completed next month, academics at the University of York will make the case for a reduction by a third in the cap on new drug costs used by the National Institute for Health and Clinical Excellence (Nice), the medicines advisory board, which already rejects a significant number of new treatments as not being cost effective.

The findings are likely to fuel debate between those who believe new drugs consume a disproportionate share of the NHS budget and the pharmaceutical industry, which says price cuts would deter innovation and threaten patient access to the most advanced treatments.

NICE threshold ‘could be reduced to £13,000 per QALY’
By Macion Davies | 14 May 2012

The NICE threshold above which drugs are considered too expensive for NHS could be lowered to £13,000 for some diseases, academics have suggested.

Researchers at the University of York are looking into whether the threshold that currently sits at around £30,000 per quality-adjusted life year (QALY) should be altered to ensure costs do not rise.

They have calculated that it could be lowered by at least £1,000 per QALY, and potentially reduced to as little as £13,000, in a move would have meant that drugs such as dabigatran and insulin glargine would never have been approved for use on the NHS.
5. How uncertain are the estimates and what are the implications?

5.1 Simulation of uncertainty in the economic evaluation shows that the cost-effectiveness of the intervention is uncertain. The probability that the cost-effectiveness ratio is less than £20,000 per QALY is 0.64 and the probability that it is less than £30,000 is 0.92.

“The Government has agreed with industry that the baseline cost effectiveness threshold should be kept at a level consistent with the current range (£20,000 per QALY up to £30,000 per QALY subject to the application, in individual cases, of a number of modifying factors)” [Ongoing consultation on “value based assessment”]
Most decision-makers don’t use a fixed threshold… why?

Making Judgements

- Cost-effectiveness
- Legal and policy constraints
- Practicalities of implementation
- Effectiveness
- Extent of uncertainty & Irreversibility of decision
- Other social values: ethics, equity, rights
Application of ‘special circumstances’

Table 1
Application of ‘special circumstances’ in the appraisal of some products with incremental cost-effectiveness above £30 000 per quality adjusted life year

<table>
<thead>
<tr>
<th>Topic</th>
<th>ICER (£000s)</th>
<th>Severity</th>
<th>End of life*</th>
<th>Stakeholder persuasion</th>
<th>Significant innovation</th>
<th>Disadvantaged population</th>
<th>Children</th>
</tr>
</thead>
<tbody>
<tr>
<td>Riluzole (motor neurone disease)</td>
<td>38–42</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
<td></td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Trastuzumab (advanced breast cancer)</td>
<td>37.5</td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Imatinib (chronic myeloid leukaemia)</td>
<td>36–65</td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Imatinib (gastrointestinal stromal tumour)</td>
<td></td>
<td>✓</td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pemetrexed (malignant mesothelioma)</td>
<td>34.5</td>
<td>✓</td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Ranizumab (age-related macular degeneration)</td>
<td>&gt;&gt;30</td>
<td></td>
<td></td>
<td>✓</td>
<td>✓</td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Omalizumab (severe asthma)</td>
<td>&gt;30</td>
<td>✓</td>
<td></td>
<td></td>
<td>✓</td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Sunitinib (advanced renal cancer)</td>
<td>50</td>
<td>✓</td>
<td>✓</td>
<td></td>
<td>✓</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lenalidomide (multiple myeloma)</td>
<td>43</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Somatotropin (growth hormone deficiency)</td>
<td>n/a</td>
<td></td>
<td></td>
<td></td>
<td>✓</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Chronic subcutaneous insulin infusion (childhood Type 1 diabetes)</td>
<td>n/a</td>
<td></td>
<td>✓</td>
<td></td>
<td></td>
<td></td>
<td>✓</td>
</tr>
</tbody>
</table>

*End-of-life considerations have only been explicitly taken into account since January 2009 on the basis of supplementary advice from the Institute to the Appraisals Committee. ICER, incremental cost-effectiveness ratio (£ per quality-adjusted life year.

Rawlins, Barnett, Stevens Br J Clin Pharmacol 2010
Question: what value judgments matter to you and your setting?
From evidence to setting standards and improving quality: going beyond HTA to implementation

- Clinical Trials and Evidence Reviews
- Clinical Guidelines and Health Technology Assessment
- “Clinical Pathways” of Care / “quality standards”
- Medical education and professional training
- Performance management
- Budget management
- Provider payment mechanisms incl. case-based payment
- Communication of entitlement to patients and their families
- Clinical audit and provider benchmarking
- Provider regulation and accreditation
Thank you… Any Questions?

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