The application of the cost-effectiveness threshold in five countries

UCL Workshop, Department of Statistical Sciences – 15th December 2014

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Director, HEOR & Strategic Market Access
“**Q14 Valerie Vaz:** Have you done an assessment on displaced services? Obviously you will get a new technology and you will do an assessment on that; what about an assessment on the displaced services?

**Sir Andrew Dillon:** This is an important point, and it is one focused on particularly by the group of economists who responded to us and also published on this as well. They are very concerned, as they have been for some time, about the methodology that NICE have used to assess new drugs. [...] 

It is an important point, but it illustrates the difference between what NICE do and what economists do, with enormous respect, because we use them frequently and extensively to support us in our methodology and in the individual topics that we look at. **They quite properly are guardians of the methodology we use; we are the users and appliers of that in the difficult world of decision making for the NHS.** The advisory committees that NICE set up have a methodology, but they also have discretion to do what they believe is the right thing: the right balance between the interests of a particular group of patients in the case of a drug that might benefit and the broader group of patients in the NHS for whom there will be consequences with any decision to do anything new.”

*Source: Work of NICE, Oral evidence: Work of NICE, HC 612 6 2nd September 2014*
Overview of the presentation

- What are the requirements for cost-effectiveness evidence by country?
- What is the cost-effectiveness threshold in theory?
- Cost-effectiveness threshold in practice in five countries:
  - Australia
  - Netherlands
  - United Kingdom
  - France
  - Japan
- Some thoughts and questions for discussion
Cost-effectiveness requirements by country

- Bending & Smith 2012 outline requirements by country

<table>
<thead>
<tr>
<th>Reimbursement processes requirements for submission of HEA</th>
<th>Mandatory for first assessment of new medicine</th>
<th>Mandatory for certain medicines on first assessment</th>
<th>Optional for first assessment of new medicine</th>
<th>Not Required for first assessment of new medicine</th>
</tr>
</thead>
<tbody>
<tr>
<td>Australia (PBAC)</td>
<td>✤ Canada (CADTH CDR)</td>
<td>✤ Finland (PPB)</td>
<td>✤ Israel (MoH)</td>
<td>✤ Hungary (OHTA)</td>
</tr>
<tr>
<td>✤ South Korea (HIRA)</td>
<td>✤ Mexico (GHC)</td>
<td>✤ Poland (AHTAPol)</td>
<td>✤ Portugal (INFARMED)</td>
<td>✤ Sweden (TLV)</td>
</tr>
<tr>
<td>✤ England &amp; Wales (NICE) +</td>
<td>✤ Scotland (SMC)</td>
<td>✤ Austria (HEK)</td>
<td>✤ Belgium (NIHDI)</td>
<td>✤ Ireland (CPU)</td>
</tr>
<tr>
<td>✤ Netherlands (CVZ)</td>
<td>✤ New Zealand (PHARMAC)</td>
<td>✤ Norway (NOMA)</td>
<td>✤ Spain (MoH)</td>
<td>✤ France (HAS)</td>
</tr>
<tr>
<td>✤ Denmark (DMA)</td>
<td>✤ Italy (AIFA)</td>
<td>✤ Switzerland (FOPH)</td>
<td>✤ Germany (G-BA)*</td>
<td></td>
</tr>
</tbody>
</table>

+ Does not appraise all new medicines but is selective based on priorities.
* Following arbitration the manufacturer can be required to provide a cost benefit analysis but no submissions have been required to date.
Brief history of the use of cost-effectiveness

- Providing comparisons between interventions using the ICER required some notion of a threshold ratio above which an intervention would be deemed not cost-effective.

First published cost-effectiveness study
(Klarman, 1965)

First cost-utility analysis:
(Klarman, 1968)

Office of Technology Assessment setup (HTA)

First WTP study for value of risk ↓ in heart attacks
(Action, 1976)

League table notion of threshold
(Williams, 1985)

PBAC uses cost-effectiveness in decisions

NICE/ZIN Established

Empirical estimation of threshold
(Martin et al, 2007)

HAS sets CE brief
The ‘critical ratio’ or $\lambda$ cut-off

- The use of an ICER for informing decisions can be traced back to public economics (Weinstein and Zeckhauser 1973)

- Weinstein and Zeckhauser introduced the critical ratio ($\lambda$)

- The description of this critical ratio is:
  - Under a fixed budget constraint, divisibility and constant returns to scale of programs, an ICER threshold value can be defined above which interventions do not improve efficiency and below which they do improve efficiency (‘$\lambda$’)
  - Efficiency is defined as maximising total health from the available resources (budget)

- Critical ratio/decision rule: \[ \frac{\Delta C}{\Delta E} < \lambda \]

- ‘$\lambda$’ from this point onwards became known as: ‘The cost-effectiveness threshold’
Early notions of a CE threshold: League tables

<table>
<thead>
<tr>
<th>Intervention</th>
<th>ICER (cost per QALY)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pacemaker for atroventricular heart block</td>
<td>£700</td>
</tr>
<tr>
<td>Hip replacement</td>
<td>£750</td>
</tr>
<tr>
<td>Valve replacement for aortic stenosis</td>
<td>£900</td>
</tr>
<tr>
<td>CABG (severe angina, LMD)</td>
<td>£1,040</td>
</tr>
<tr>
<td>CABG (moderate angina, LMD)</td>
<td>£1,330</td>
</tr>
<tr>
<td>CABG (mild angina, LMD)</td>
<td>£2,280</td>
</tr>
<tr>
<td>Kidney transplantation</td>
<td>£3,000</td>
</tr>
<tr>
<td>CABG (moderate angina, DVD)</td>
<td>£4,000</td>
</tr>
<tr>
<td>Heart transplantation</td>
<td>£5,000</td>
</tr>
<tr>
<td>CABG (mild angina, TVD)</td>
<td>£6,300</td>
</tr>
<tr>
<td>Haemodialysis at home</td>
<td>£11,000</td>
</tr>
<tr>
<td>CABG (mild angina (DVD))</td>
<td>£12,600</td>
</tr>
<tr>
<td>Haemodialysis in hospital</td>
<td>£14,000</td>
</tr>
</tbody>
</table>


- Williams first published the ‘league table’ in 1985 approach in coronary artery bypass grafting (CABG)
  - ICER vs medical management
  - Decline with less severe disease

- ICER Threshold value is “The value of the last intervention in the league table of ICERs that would still be financed from a given fixed budget.”
Cost-effectiveness threshold

1. Shadow price of the budget constraint

- This approach requires that interventions with an ICER less than or equal to the shadow price of the budget be adopted.

- Budget is set and it is a question of opportunity cost:
  "The cost effectiveness threshold is an estimate of health forgone as other NHS activities are displaced to accommodate the additional costs of those technologies recommended (....).”
  (Claxton et al. 2013)

- The shadow price approach is based on several assumptions:
  - Fixed health care budget
  - Objective is to maximise health benefits of the population
  - Perfectly divisibility and constant returns to scale of all programs
  - Programs are independent of each other
  - Complete information on costs and benefits of existing programs
Cost-effectiveness threshold

2. Social willingness to pay

- Value society places on health gains estimated by survey
  - WTP of members of the public for (typically small) health gains
  - Sums are then aggregated to provide overall value of a QALY
  - Implemented by value of preventing a statistical fatality (VPF) and value of serious injury (VSI) (used in other public sectors)

- The health care budget is then determined as the sum of the values for each program
  - Flexible health care budget with no constraint (private, insurance based systems)

- Key assumptions of the WTP approach for the threshold:
  - Distributional assumptions (association with ability to pay)
  - Hypothetical nature of the elicitation process
  - Detached from budgeting process by considering individual values
Cost-effectiveness thresholds in practice

- The thresholds can be explicit or implicit and is determined by the decision-making authority:
  
  1. Previous analysis of reimbursement decisions
  2. Societal willingness to pay
  3. Empirical estimation of opportunity cost
  4. Stated by decision-maker (in combination with other factors)
  5. Other methods to determine the threshold (i.e. GDP per capita, WHO 3 x GDP, expert elicitation)

- Depends whether the decision-authority is a threshold *searcher* or *setter* (& surveyors)? (Culyer et al. 2007, Baker et al. 2011)
Past decisions implying a CE threshold
Cost-effectiveness threshold in Australia

- The Pharmaceutical Benefits Advisory Committee (PBAC) is established under the National Health Act 1953 (the Act)
  - Recommends to the Minister of Health which medicines should be subsidised under the Pharmaceutical Benefits Scheme (PBS)
  - Funded through taxation (AUS$9.8bn – ‘12/’13) and co-payments
  - Direct budgetary impact (estimated to the scheme) but potentially fixed for a year?
  - > AUS$20m considered by the cabinet following PBAC (in 2011)

**Figure 1: PBS expenditure is falling**

![Bar chart showing PBS expenditure from 2007/8 to 2012/13 in millions of dollars](Source: Final Budget Outcomes Appendix A: Expenses by Function and Sub-function 2013/14, 2012/13, 2011/12

Factors relevant in decision-making

- The PBAC does not apply a fixed threshold but takes account of a number of quantitative and qualitative factors (PBAC, 2013)

<table>
<thead>
<tr>
<th>Factors that are more readily quantified</th>
<th>Factors that are less readily quantified</th>
</tr>
</thead>
<tbody>
<tr>
<td>Comparative cost-effectiveness</td>
<td>Uncertainty</td>
</tr>
<tr>
<td>Comparative health gain</td>
<td>Equity</td>
</tr>
<tr>
<td>Patient affordability in the absence of PBS subsidy</td>
<td>Presence of effective alternatives</td>
</tr>
<tr>
<td>Financial implications for the PBS</td>
<td>Severity of medical condition treated</td>
</tr>
<tr>
<td>Financial implications for government health budgets</td>
<td>Ability to target therapy</td>
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<td></td>
<td>Development of resistance</td>
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</tbody>
</table>

*Source: Adapted from PBAC, Guidelines for preparing submissions to the Pharmaceutical Benefits Advisory Committee June 2013 version 4.4*
Past decisions

- Chair of PBAC on record as AUS$50k per outcome ‘on the high side’
- George et al. 2001 presented <AUS $42k per LYG were unlikely to reject
- Henry et al. 2005 presented highest recommended was AUS$52k
- Harris et al. 2008 found
  - Clinical significance, cost-effectiveness, budget impact & severity important
  - ↑ ICER by AUS$10k → ↓ plist by 0.06
- Mauskopf et al. 2013 considered the importance of budget impact controlling for cost-effectiveness

Figure 2  Predicted probability of the Australian Pharmaceutical Benefits Advisory Committee recommendation by incremental cost per quality-adjusted life year by severity of condition and confidence in clinical significance at the mean of other variables. ICER = incremental cost-effectiveness ratio.

Australia

- Considering an implicit threshold based on past decisions is problematic
  - PBAC reports do not provide the exact ICER but a range of ICERs
  - Previous decisions do not necessarily reflect the current situation
  - Studies can only provide a limited representation of technologies to determine the threshold
  - Controlling for the impact of all (un)observed factors for decisions

- Key questions
  1. Is it appropriate to take into account budget impact if cost-effectiveness is a decision factor?
  2. What are the implications of the dynamic changes in budget through Cabinet intervention?
  3. Does this system of considering decisions on a case by case basis improve access and health or provide a lack of clear signal of value to manufacturers?
Cost-effectiveness threshold, WTP & severity
Netherlands

- Social health insurance system funded by:
  - Government (budget fixed?)
  - Employers premiums (potential flexibility?)
  - Individual premiums (potential flexibility?)

- Cleemput et al. 2012 and other authors refer to the Netherlands system in terms of willingness to pay

- Social value of health for informing decisions:
  - Contingent valuation study (Bobinac et al. 2010) estimated €24,000 per QALY
    - Wide variation with income group from €5k to €75k per QALY
ZiN reimbursement process

- The ZiN uses four formal priority setting principles that include (based on the Dunning funnel):
  - Necessity (medical need)
  - Effectiveness
  - Cost-effectiveness
  - Feasibility

- The ZiN advises the minister on the robustness and does not advise on the actual cost-effectiveness estimate
  - Mandatory for drugs with added therapeutic value (List 1B), exempt for budget impact of lower than €500k per year and HIV medications
  - HEE evidence only available for 35% of applications (Franken et al. 2014)

- No explicit threshold - The ZiN advises the minister on the robustness and does not advise on whether cost-effective
Implicit cost-effectiveness threshold

- Cleemput et al. 2012 developed a decision framework including the Netherlands considering:
  - Medical, therapeutic and or societal need
  - Preparedness to pay for a indication
  - Preparedness to pay for a particular treatment
  - Preparedness to pay more than an alternative
  - Willingness to pay: price and reimbursement basis

- A report suggested a cost-effectiveness range depending on severity of disease of between €10,000 to 80,000 per QALY (RVZ, 2006)
  - Based on a balance of considerations
  - Dutch Minister has never confirmed or endorsed such range
Netherlands – ZiN (formerly CVZ) criteria

- A background study provides guidance to the ACP on the meaning of cost-effectiveness in relation to other factors

### Table: CVZ criteria that affect the interpretation of cost-effectiveness

<table>
<thead>
<tr>
<th>Criteria that affect the interpretation of cost-effectiveness</th>
<th>Increase the leniency with respect to the cost-effectiveness requirement</th>
<th>Make the cost-effectiveness requirement stricter</th>
<th>Should not be included</th>
</tr>
</thead>
<tbody>
<tr>
<td>High burden of disease</td>
<td></td>
<td></td>
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<tr>
<td>Rareness</td>
<td></td>
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<td></td>
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<tr>
<td>Informal care (positive effect)</td>
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<tr>
<td>Public health risk</td>
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<tr>
<td>Little overlap with health care domain</td>
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<tr>
<td>High budget impact</td>
<td></td>
<td></td>
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<tr>
<td>Future medical costs not included</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Unsuitable to insurance because of high prevalence</td>
<td></td>
<td></td>
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<tr>
<td>Unsuitable to insurance because of excessive patient influence on the dose</td>
<td></td>
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<tr>
<td>Uncertainty about the appropriateness of the intervention</td>
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<tr>
<td>Lifestyle/high risk behaviour</td>
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<td></td>
</tr>
<tr>
<td>Age, gender, ethnicity, sexual preference and social economic status</td>
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</tbody>
</table>

Threshold by severity

Netherlands key challenges

- No explicit threshold range that some drugs received reimbursement far higher than the (€80k maximum)

- To date the ZiN has decided not to begin a wider debate on cost-effectiveness threshold following focus groups with patients and the general public (Franken, et al. 2014)

Some questions:
1. What is the theoretical basis/conceptual basis?
2. Is the fear of the political unacceptability and inducing strategic behaviour a sufficient reason? (Franken, et al. 2014)
3. Who should be responsible for setting or searching for the threshold (ZiN or the Minister, or academia)?
Searching for the cost-effectiveness threshold
NICE – Past 15 years

1999 → 2014

2. Evidence

2.1 Permanent wisdom teeth normally erupt from the age of six onwards, with the third molar (wisdom tooth) being the last to emerge usually between the ages of eighteen and twenty-four years old. The third molar may erupt in all directions causing pathological changes as well as the early periodontal removal of pathology-free impacted third molar. Variations in the course of the latter procedures across the country, which suggest that in the past, up to 60% of wisdom teeth removal and periodontal surgery may have been inappropriate. However, in recent years, change in the practice of removal of wisdom teeth may have taken place in response to Faculty of Dental Surgery, Royal College of Surgeons guidelines. It has been wider range of procedures may still be inappropriate. In 2010/11 there were approximately 36,000 extraction and 64,000 day case admissions in England. More recent figures (2012/13) for Wales indicate that there were up to 5000 procedures. It is estimated that the total cost to the NHS in England and Wales of wisdom teeth extraction is up to €12 million per year.

5.1 There is an evidence to support a health benefit to patients from the prophylactic removal of pathology-free impacted third molar teeth.

5.2 Every procedure for the removal of an impacted third molar carries risk for the patient, including temporary or permanent nerve damage, abscess, osteitis, infection and haemorrhage as well as temporary local swelling, pain and restricted mouth opening. There are also risks associated with the use of the general anaesthesia in some of these procedures, including sea and unpredictable death. Such patients are therefore being exposed to the risk of undergoing a surgical procedure unnecessarily.

4.4 The Committee concluded that apixaban was more clinically effective than warfarin for the primary outcome of stroke and systemic embolism.

4.5 The Committee concluded that apixaban was less effective than warfarin in reducing the risk of intracranial bleeding for people with atrial fibrillation when compared with warfarin.

4.14 The Committee concluded that apixaban had been shown to be cost effective compared with warfarin, the most plausible ICER being less than £20,000 per QALY gained, and could be recommended as an option for preventing stroke and systemic embolism for people with nonvalvular atrial fibrillation who have 1 or more risk factors for stroke.

Summary of Appraisal Committee’s key conclusions

TA375 Appraisal title: Apixaban for preventing stroke and systemic embolism in people with nonvalvular atrial fibrillation

Key conclusion

Apixaban is recommended as an option for preventing stroke and systemic embolism within its marketing authorisation.

The decision about whether to start treatment with apixaban should be made after an informed discussion between the clinician and the person about the risks and benefits of apixaban compared with warfarin, dabigatran etexilate or rivaroxaban. For people who are taking warfarin, the potential risks and benefits of switching to apixaban should be considered in light of their level of international normalised ratio (INR) control.

The Committee concluded that apixaban was more clinically effective than warfarin for the primary outcome of stroke and systemic embolism.

The Committee concluded that apixaban resulted in fewer bleedings than warfarin and it recognised the particular importance of the effects of apixaban in reducing the risk of intracranial bleeding for people with atrial fibrillation when compared with warfarin.

The Committee concluded that apixaban had been shown to be cost effective compared with warfarin, the most plausible ICER being less than £20,000 per QALY gained, and could be recommended as an option for preventing stroke and systemic embolism for people with nonvalvular atrial fibrillation who have 1 or more risk factors for stroke.

Current Practice
Searching for the cost-effectiveness threshold

- NICE established (1/4/99)
- Devlin et al. 2004 Past NICE decisions
- Rawlins & Culyer publish in BMJ
- Kennedy report
- End of life guidance
- VBP proposed
- MRC CE threshold study, Jun & Nov
- UCL Workshop 15/12/14

- 1999
- 2001
- 2003
- 2005
- 2007
- 2009
- 2011
- 2013
- '14

- '99

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First statement of cost-effectiveness threshold

- Rejection of the idea of an absolute threshold in 2004

“The main considerations in making judgments about cost-effectiveness for ratios of £25,000-£35,000/QALY are: The degree of uncertainty surrounding the estimate; The particular features of the condition and population using the technology; The innovative nature of the technology; When appropriate the wider societal costs and benefits; When appropriate, reference to previous appraisals.” (Rawlins and Culyer, 2004)

Source: Rawlins and Culyer 2004

A range of £20,000-£30,000/QALY
CE threshold for NICE by previous decisions

Figure 5. Probabilistic cost-effectiveness thresholds for NICE decisions

NICE CE threshold in the 2008 methods guide

6.2.23 Above a most plausible ICER of £20,000 per QALY gained, judgements about the acceptability of the technology as an effective use of NHS resources will specifically take account of the following factors.

- The degree of certainty around the ICER. In particular, the Committee will be more cautious about recommending a technology when they are less certain about the ICERs presented.
- Whether there are strong reasons to indicate that the assessment of the change in HRQL has been inadequately captured, and may therefore misrepresent the health utility gained.
- The innovative nature of the technology, specifically if the innovation adds demonstrable and distinctive benefits of a substantial nature which may not have been adequately captured in the QALY measure.

6.2.24 As the ICER of an intervention increases in the £20,000 to £30,000 range, the Committee’s judgement about the acceptability of the technology as an effective use of NHS resources will make explicit reference to the relevant factors listed above.

6.2.25 Above a most plausible ICER of £30,000 per QALY gained, the Committee will need to identify an increasingly stronger case for supporting the technology as an effective use of NHS resources, with regard to the factors listed above.

Developments between 2009 to 2013

- Three developments following the 2008 guide:
  - Kennedy report published suggesting other elements of benefit important such as:
    - Severity of disease
    - End of life
    - Further research required
  - End of life supplementary criteria (NICE, 2009)
  - Cancer Drugs Fund setup in 2010 to fund drugs rejected by NICE

- Guide to methods in 2013 added two more factors to the list most plausible ICERs above £20,000:
  - The technology meets the criteria for special considerations as a ‘life extending treatment at the end of life’
  - Aspects that relate to non-health objectives of the NHS

- HST programme introduced (May 2013) considering wider benefits (cost saving outside NHS)

- VBP, VBA...VBPostponed
Is the search for the NICE CE threshold over?

- Dakin et al. 2013 suggested that a technology costing £40,000 per QALY has a 50% chance of NICE rejection
  - Range of 25% at £27,000 per QALY; 75% at £52,000 per QALY
  - May be explained by other factors not considered

- Methods for estimating the NICE cost-effectiveness (Claxton et al. 2013)
  - A 2 year study funded by a MRC methodological research grant:
    - Advanced econometric analysis of 23 programme budgeting categories
    - First draft £18,317, Second draft provided £12,936 per QALY
  - Critique of study by OHE authors (Barnsley et al. 2013) are concerned with:
    - The assumption to ignore that current spending leads to current and future reductions in mortality so tends to overestimate the threshold
    - The assumption of those deaths averted having the same life expectancy as average members of the population
    - Optimistic assumptions for the unobservable QALY gains in the analysis
    - The analysis does not accurately discount the benefits for the threshold
    - The proposition that increases in budget would increase the threshold but this would be offset by productivity increases in the NHS
Some questions for discussion

1. Have we now identified the CE threshold for England?

2. Has the search been too attached to the budgetary process?

3. What are the implications of initiatives such as the Cancer Drugs fund for the cost-effectiveness threshold?

4. What are the implications of the different reimbursement processes on the consideration of the cost-effectiveness threshold (HST, vaccines etc.)?

5. How can the optimal threshold be adjusted for the relationship that the future value depends on the NICE threshold when data was gathered?

6. How can other factors be considered within the health economics framework for the cost-effectiveness threshold?

7. What about new considerations in Scotland through the Patient and Clinician Engagement (PACE) on the CE threshold?
Battle of the “ASMR” versus “λ”
France - Battle of the “ASMR” versus “λ”

- Comparison of SMC & HAS 2012 common decisions (Bending et al. 2012)
  - Contrast of the process differences and links with evidence requirements
  - Common themes in matched decisions with respect to potential reasons for differences in recommendations
    - Differences in comparators and clinical guidelines
    - Dealing with uncertainty (NMA and use of economic analysis)
    - Requirement to use a generic measure of health benefit in cost-utility analysis (as opposed to judgement by ASMR)
    - Clinical benefits but price to high to justify benefit in Scotland but in France judged to be an important improvement (level 2)

- The results at this stage indicated divergence due to difference in HTA perspective, process and evidence requirements
France

- 2008: Haute Autorité de Santé (French HTA agency)
  - Set an objective to evaluate efficiency of therapeutic strategies
  - CEESP (Committee for economic evaluation and Public Health)

- Nov 2011: HAS produces Economic analysis guidelines

- 2012: Law & Decree for efficiency assessment of individual technologies
  - In addition to SMR and ASMR (medical benefit and additional benefit) by the Transparency Committee
  - Only for drugs / devices claiming from moderate to major additional benefit (level I to III), with sales forecasts year 2 > €20 millions (public prices)

- Oct 2013: implementation of cost-effectiveness analysis
Process for introduction of health economics

1. Is a C/E analysis required?
   - Yes/No

2. If Yes:
   - Economic Evaluation
   - Submission to CEESP
   - 90 days
   - CT Opinion (published)

3. If No:
   - Economic Committee of Health Products (CEPS)
HE experience from Oct’13 to Sept’14

- **CEESP submission**
  - 73 submitted

- **Stage in process**
  - 53 in process
  - 10 CE opinions
  - 10 not eligible

- **Provisional Opinion**
  - 1 with minor reservations
  - 9 with major/important reservations

- **Published**:
  - Price negotiation with CEPS required before publication.
    - ICER > 100 000 €/QALY for 2
    - ICER < 30 000 €/QALY for 3
    - One dominant


No consensus on the use of thresholds
France key questions

1. What would be the best way of achieving consensus on the cost-effectiveness threshold in this system?

2. There is still currently the SMR/ASMR but potential move towards one added therapeutic benefit. Can this work besides CE?

3. Can there be a cost-effectiveness threshold with price being determined ex-post?

4. Does the French system achieve the same outcomes in terms of expenditure as the Scottish system?
Implementing cost-effectiveness in decision-making
Japan

- Public health insurance system
  - Mix of insurance premium and taxation funded
  - Ministry of Health, Labour and Welfare provides coverage decisions
  - Questions around sustainability of increasing expenditure

Figure 3. Japan: Health Spending, 1960–2011
(In percent of GDP, unless otherwise noted)

Sources: Ministry of Health, Labor, and Welfare; IMF World Economic Outlook database; and IMF staff estimates.
Guidelines for the Analysis Methods of Health Economics Evaluation Research

(Version 1.0, March 10, 2015)

When these guidelines are quoted the following title should be used for the time being:

These guidelines are part of the research achievements of the “Research of Medical Benefit System Incorporating Health Economics Evaluation” team, Fiscal 2014 MEXT Grants-in-Aid for Scientific Research (KAKENHI), Policy Science Integrative Research Project (Team Fukuda), and are based on discussions by researchers and other members who participated in this team.
Japan – Introducing cost-effectiveness

- Central Social Insurance Council ("Chuikyo") setup for consideration of cost-effectiveness principle for health insurance coverage:
  - Motivated by the current spending challenges
  - Until now health economic evaluation had been rarely applied
  - Pilot scheme running for a group of selected companies
  - Full implementation planned in FY2014

- Proposed that technologies would be prioritized based on:
  - Existence of alternative treatments
  - Large financial impact
  - Not to include rare disease

- A cost-effectiveness threshold has been referred to but not applied in decision-making:
  - Range of JPY5m to JPY6m ($\approx €37k - €44k$ per QALY) (Shiroiwa et al. 2010)
WTP per QALY in Japan

- Shiroiwa et al. recently performed a contingent valuation study to obtain the willingness to pay based on severity:
  - 2,400 respondents using double bounded dichotomous choice
  - 16 health states by severity
  - Mean & median for states was JPY5 million (Shiroiwa et al. 2013)
  - Participants were willing to pay more for worse health states
  - Range from low severity to high severity estimated at JPY2 million to JPY8 million (≈ €15k - €58k per QALY)

- Chuikyo anticipates formally reviewing CE evidence in 2016
  - Suggested other factors will be taken into account alongside the CE analysis
  - No clear consensus or description of CE threshold in the new guideline

- What can Japan learn from the experience of the threshold setters?
  - How should a CE threshold be implemented when multiple funding sources?
Further thoughts and questions

- What is the theoretical basis of the threshold for countries with multiple funding sources?

- How should the optimal cost-effectiveness threshold be determined in fixed budget systems?

- There is evidence that severity is important across the five countries. How should this and other factors be considered?

- Is it appropriate for countries to implement processes that vary the strictness of the cost-effectiveness threshold?

- What can countries that wish to implement a cost-effectiveness threshold learn from previous experience?
Mapi is purpose-built for Patient-Centered research

- Pharma identifies Endpoint Strategy
- Pharma identifies COA options and begins licensing discussions
- Patient-Centered Outcomes Services
- Linguistic Validation Services
- Health Economic Outcomes Research Services
- Value Dossier and Strategic Market Access Services
- Real World Evidence Services
References


References


21. PBAC, Guidelines for preparing submissions to the Pharmaceutical Benefits Advisory Committee June 2013 version 4.4


Thank You