The emerging discipline of Health Technology Assessment

NHSRC, August 2012
Kalipso Chalkidou, MD, PhD
Director, NICE International
7.1.3 Total health expenditure per capita and GDP per capita, 2009 (or nearest year)

Health spending per capita (USD PPP)

0 1000 2000 3000 4000 5000 6000 7000 8000

GDP per capita (USD PPP)

0 15000 30000 45000 60000 75000 90000

USA

Source: OECD Health Data 2011; WHO Global Health Expenditure Database.

StatLink: http://dx.doi.org/10.1787/888932526046

and private, 2009 (or nearest year)

Private expenditure on health

Share related to investments.

StatLink: http://dx.doi.org/10.1787/888932526084

National Institute for Health and Clinical Excellence
Median Monthly Costs

new anti-cancer drugs at launch

Bach et al 2009
How much is enough?
"The NHS, just like every other healthcare system in the world—public or private—has to set priorities and make choices. The issue is not whether there are choices to be made, but how those choices are made. There is not a service in the world, defence, education or health, where this is not the case."

UK Parliamentary Health Committee
Our starting point

• If a country’s commitment to the principle of universal access to a basic package of services for its population is to be met, the long-term financial sustainability of providing the listed services to those who need them is of the essence.

• To ensure this, a prioritisation process to determine which services are to be provided and for whom, has to be designed, implemented and regularly reviewed.

• For such a process to be legitimate and relevant, it needs to adhere to a set of core principles of scientific rigour, transparency, consistency, independence from vested interests, inclusiveness of all stakeholders, contestability, timeliness and enforcement.
## Process matters

<table>
<thead>
<tr>
<th>Principles</th>
<th>Putting them into practice…</th>
</tr>
</thead>
<tbody>
<tr>
<td>Independence</td>
<td>Arm’s length 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, 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; minimal 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>Referenced in country’s legal framework; institutional role in informing coverage/payment decisions;</td>
</tr>
<tr>
<td>Regular review</td>
<td>Regular updating of its decisions and of its methods</td>
</tr>
</tbody>
</table>
HTA: WHAT IS IT?
What is Health Technology Assessment?

- **Health Technology**: “The drugs, devices, and medical and surgical procedures used in health care, and the organisational and supportive systems within which such care is provided”
  - Contraceptives; dialysis machines; mastectomy; screening for cancer; intensive care unit

- **Health Technology Assessment**: “a multi-disciplinary field of policy analysis, which studies the medical, social, ethical and economic implications of development, diffusion and use of health technology.”

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*b*: International Network of Agencies for Health Technology Assessment (INAHTA)
Why do an appraisal?

• Regulators (EMEA, FDA) à safety and efficacy compared to nothing (placebo)
  – Not enough!
• NICE Technology Appraisal à clinical and cost effectiveness compared to next best alternative
  – clear standards for high quality consistent clinical practice across the country
  – faster uptake of effective innovative treatments
  – better use of resources
The real challenge

One in, one out?
Criteria for decision-making: assessing cost-effectiveness

1. How well does the technology work compared to standard practice in OUR healthcare system?
2. How much does the technology cost compared to standard practice in OUR healthcare system?
   - cost of technology, monitoring, length of inpatient or outpatient stay, costs of treating adverse events
3. Health gain is measured using quality adjusted life years (QALYs):
   - Difference in costs
   - Difference in effect
Quality adjusted life years (QALYs)

• **For NICE appraisals and guidelines:**
  - “...(C)ost-effectiveness (specifically cost–utility) analysis is the preferred form of economic evaluation. This seeks to establish whether differences in costs between options can be justified in terms of changes in health effects. Health effects should be expressed in terms of QALYs.”

• **What is a QALY?**
  - Combines quantity & quality of life in single measure
  - Time spent in a health state weighted by quality of life (QoL)
  - QoL scores should reflect peoples’ preferences over health
  - QoL is usually scored with ‘perfect health’=1 and death=0

• **Why use QALYs?**
  - Can weigh up net effects of treatment for patients
  - Provides common unit of health benefit
  - Benchmark for comparison of different treatments
The **Quality Adjusted Life Year**

<table>
<thead>
<tr>
<th>Health-related quality of life</th>
<th>Length of life (years)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Current treatment</td>
<td></td>
</tr>
<tr>
<td>New treatment</td>
<td></td>
</tr>
</tbody>
</table>

- Initial QALY loss due to side effects

QALYs gained
Trading off benefits, harms and costs

<table>
<thead>
<tr>
<th>Cost (£)</th>
<th>Effect (QALYs)</th>
</tr>
</thead>
<tbody>
<tr>
<td>YES</td>
<td>- cheaper &amp; better</td>
</tr>
<tr>
<td>NO</td>
<td>- more expensive &amp; worse</td>
</tr>
<tr>
<td>?</td>
<td>- better but more expensive</td>
</tr>
</tbody>
</table>

Current treatment

New treatment

YES - cheaper & better

NO - more expensive & worse
... but is it cost-effective?
Where is the threshold?

Cost (£) vs. Effect (QALYs)

NICE threshold about £20,000 to £30,000 per QALY

Treatment cost-effective anywhere in shaded area
USING HTA TO DECIDE WHAT TO PAY FOR
In theory, you can, using league tables

1. List all possible health care interventions for all groups of patients
2. Estimate cost & health gain (e.g. QALY/DALY) for each intervention
3. Eliminate any options where an alternative costs more and gives smaller health gain
4. Rank remaining options in order of decreasing value for money (e.g. cost per QALY gained)

https://research.tufts-nemc.org/cear
### The fixed budget approach

<table>
<thead>
<tr>
<th>Selected interventions</th>
<th>$/QALY</th>
</tr>
</thead>
<tbody>
<tr>
<td>Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and high risk for stroke</td>
<td>Cost-saving</td>
</tr>
<tr>
<td>Thrombolytic therapy with intracoronary streptokinase vs. conventional therapy in patients with ECG evidence of AMI and duration of symptoms &lt; 4 hours</td>
<td>$4,800</td>
</tr>
<tr>
<td>Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and medium risk for stroke</td>
<td>$8,800</td>
</tr>
<tr>
<td>Captopril therapy vs. No captopril in 60 year-old patients surviving myocardial infarction</td>
<td>$11,000</td>
</tr>
<tr>
<td>Thrombolytic therapy with tissue plasminogen activator vs. streptokinase in patients presenting within 6 hours after onset of symptoms of AMI</td>
<td>$32,000</td>
</tr>
<tr>
<td>Captopril therapy vs. No captopril in 50 year-old patients surviving myocardial infarction</td>
<td>$73,000</td>
</tr>
<tr>
<td>Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and low risk for stroke</td>
<td>$410,000</td>
</tr>
</tbody>
</table>

### The Willingness To Pay approach

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### The reallocation approach

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<td>Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and high risk for stroke</td>
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<td><strong>$410,000</strong></td>
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Summary

• If correctly used, these methods should improve efficiency
• **Comprehensive** approaches: WTP and fixed budget
  – May be feasible for part of budget (e.g. growth money), *but*,
  – Impossible to list absolutely everything!!!
  – WTP threshold difficult to identify
  – No account of value judgements and equity considerations
  – Political acceptability less than guaranteed!
• **Incremental** approaches: threshold and reallocation
  – More practical, but take longer to make an impact
  – Require strong topic selection processes to target high priority
disease areas or groups of technologies for analysis
  – Room for more focus on process and social values
  – But, if threshold is not calibrated, may have perverse effects
Things are never as easy as they seem!

“This, then, is the reality of rationing: countless, day to day decisions by clinicians and others taken in the light of the resources available and the particular circumstances of the patient concerned.

Rationing, in effect, is a continuous attempt to reconcile competing claims on limited resources, a balancing act between optimising and satisfying treatment. It is about the exercise of judgment, not about the drawing up of lists of what should or should not included in the NHS's menu.”

Ruldolf Klein *BMJ* 1997; 314
Multiple uses for HTA

HTA

- Regulation and licensing
- Listing and coverage
- Appropriate use by professionals and patients
- Pricing and reimbursement
BACK TO THE REAL WORLD: COUNTRY CASE STUDIES
The NHS (currently…)

- UK parliament
  - NHS vote

- Secretary of State for Health/Department of Health

- NICE

- Other bodies (e.g. HPA)

- Primary Care Trusts

- 1<sup>st</sup> care doctors

- Hospitals

- Hospital doctors

Tax-revenue allocation by government
Limited co-pays and out-of-pocket

152 regions across the country

Guidance
NICE: the organisation

- Special Health Authority – part of NHS
- Board (& Chair) appointed by Secretary of State for Health
- Budget and Staff:
  - 1999: £10m / 10 WTE
  - 2005: £27m / 185 WTE
  - 2009: £61m / 390 WTE
  - 2011: £68m / ~ 430 WTE
- ~2,000 experts – physicians, nurses, health economists, clinical epidemiologists, statisticians, lay people- across the UK
NICE brings together …

**Technical**
- Selection of priority topics
- Critical appraisal and synthesis
- Economic analysis (costing, incentive ceiling, CEA)

**Clinical**
- Clinical input: evidence base and baselines
- Feasibility assessment and field testing
- Buy-in and implementation

**Process**
- Stakeholder engagement, QA, contestability, independence of vested interests
- Institutional and operational platforms
## Published NICE guidance

(1\textsuperscript{st} June, 2011)

<table>
<thead>
<tr>
<th>Type</th>
<th>Numbers</th>
</tr>
</thead>
<tbody>
<tr>
<td>Technology appraisals</td>
<td>224</td>
</tr>
<tr>
<td>Clinical guidelines and cancer service guidance</td>
<td>133</td>
</tr>
<tr>
<td>Interventional procedures</td>
<td>349</td>
</tr>
<tr>
<td>Medical Technologies</td>
<td>3</td>
</tr>
<tr>
<td>Public health</td>
<td>35</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>744</strong></td>
</tr>
</tbody>
</table>
Technology appraisals

Guidance on the use of new and existing medicines, treatments and procedures within the NHS

Two types of appraisals:

Multiple Technology Appraisal (MTA)

- Independent academic groups carry out systematic review and develop economic model (MTA)

Single Technology Appraisal (STA)

- Critique the evidence submitted by manufacturer (STA)
- 4 standing Committees (33 members each)

Recommendations to be implemented within 3 months
MTA

Company submission(s) → Assessment team report*
→ Appraisal committee produce consultation document
→ Appraisal committee finalise recommendations
→ Guidance issued

* Consulted on

Appeal

STA

Company submission → Assessment by external review group
→ Appraisal committee produce restricted advice
→ Guidance issued

→ Appraisal committee produce unrestricted advice
→ Guidance issued

→ Appraisal committee finalise recommendations
→ stakeholder comments

Appeal
Technology appraisals  
**all decisions**  
(1 March 2000 to 30 April 2011)

<table>
<thead>
<tr>
<th>Recommendation type</th>
<th>Number (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>‘Recommended’ (Full use)</td>
<td>266 (63%)</td>
</tr>
<tr>
<td>‘Optimised’ (Restricted use)</td>
<td>80 (19%)</td>
</tr>
<tr>
<td>‘Only in research’</td>
<td>24 (6%)</td>
</tr>
<tr>
<td>‘Not recommended’ (No use)</td>
<td>50 (12%)</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>420 (100%)</strong></td>
</tr>
</tbody>
</table>

Overall, 82% of decisions made by NICE (346 of 420) were ‘recommended’ or ‘optimised’. 
The evidence NICE needs

- Research Evidence
- User Experience
- Clinical Practice
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)

• See: http://www.nice.org.uk/media/C18/30/SVJ2PUBLICATION2008.pdf
Opportunity cost

- The NHS budget is limited
- It is about choice
- If the NHS spends more on one thing, it has to do less of something else
- Could we do more good by spending the extra money in other ways?
A Stalinist NHS quango and British cancer victims denied drugs available in Europe

By KAROL SIKORA
Last updated at 8:56 PM on 20th November 2009

The Government continually trumpets its commitment to fighting cancer.

Gordon Brown made a guarantee of early diagnosis for patients one of the flagship measures of his recent speech at the Labour Party conference, while the Department of Health boasts that it is bringing ‘world-class cancer services’ to Britain.

But those fine words have been exposed as hollow rhetoric by the decision of the National Institute for Health and Clinical Excellence (Nice), the Government’s health watchdog, to reject a cancer drug called Nexavar.
Value based pricing and multiple thresholds

• “We will pay drug companies according to the value of new medicines…”

The Coalition: our programme for government, July 2010

• “…the Government would set a range of thresholds or maximum prices reflecting the different values that medicines offer…”

Consultation document on VBP, Dec 2010

We will uphold all of the patient rights in the NHS Constitution. Where necessary we will adapt the way these rights are given legal force, to ensure they have the same legal force under the new legislation. This includes the right to drugs and treatments recommended by NICE, which we will retain after the introduction of value-based pricing for new drugs from January 2014.” (Government response to the NHS Futures Forum, June 2011)

• Price premium for disease severity, therapeutic innovation and wider societal benefits

Consultation document on VBP, Dec 2010
The deal provides for a straight 12.5 percent discount to bring the cost of Votrient to the NHS into line with that of Pfizer's Sutent, and also guarantees a financial rebate if Votrient proves inferior to Sutent in the clinical trial.

“We are moving in the direction where price is driven by value and value is driven by evidence, and therefore we can start to construct different sorts of arrangements where we can balance this off.” Simon Jose, GSK – CEO of ABPI
# NICE: a negative list for technologies

<p>| | | | | | |</p>
<table>
<thead>
<tr>
<th></th>
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<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>TA007</td>
<td>2000</td>
<td>MTA</td>
<td>Rabeprazole</td>
<td>Dyspepsia</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA008</td>
<td>2000</td>
<td>MTA</td>
<td>Digital hearing aids</td>
<td>Deafness</td>
<td>Not Recommended</td>
</tr>
<tr>
<td>TA008</td>
<td>2000</td>
<td>MTA</td>
<td>Analogue hearing aids</td>
<td>Deafness</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA009</td>
<td>2000</td>
<td>MTA</td>
<td>Rosiglitazone</td>
<td>Type 2 diabetes</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA010</td>
<td>2000</td>
<td>MTA</td>
<td>Dry powder inhalers (DPI)</td>
<td>Asthma (children under 5 years)</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA010</td>
<td>2000</td>
<td>MTA</td>
<td>Nebulised therapy</td>
<td>Asthma (children under 5 years)</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA010</td>
<td>2000</td>
<td>MTA</td>
<td>Pressurised metered dose inhalers (pMDI) and spacer system</td>
<td>Asthma (children under 5 years)</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA011</td>
<td>2000</td>
<td>MTA</td>
<td>Implantable cardioverter defibrillators (ICDs)</td>
<td>Arrhythmias</td>
<td>Recommended</td>
</tr>
<tr>
<td>TA012</td>
<td>2000</td>
<td>MTA</td>
<td>Abciximab (intravenous)</td>
<td>Acute coronary syndromes</td>
<td>Recommended</td>
</tr>
</tbody>
</table>

- Guidance has been incorporated in CG17. Recommendation in line with marketing authorisation.
- The Department of Health made digital hearing aid technology available across the NHS after TA008 was published which made guidance obsolete. **Guidance withdrawn from May 2003.**
- The Department of Health made digital hearing aid technology available across the NHS after TA008 was published which made guidance obsolete. **Guidance withdrawn from May 2003.**
- Guidance has been replaced by TA63 and incorporated in CG66. Recommendation in line with marketing authorisation.
- Recommendation in line with marketing authorisation.
- Recommendation in line with marketing authorisation.
- Recommendation in line with marketing authorisation.
- Guidance has been replaced by TA95. Recommendation in line with clinical practice.
- Guidance has been replaced by TA47. Recommendation in line with marketing authorisation.

Regime announced in Dec 2011 for automatic inclusion in local formularies.
Cannot avoid judgements

- Innovative mode of action
- No previous exposure at blast phase suggests omission
- Disease severity

<table>
<thead>
<tr>
<th>Cost per QALY (£’000)</th>
<th>Probability of rejection</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>10</td>
<td>0.2</td>
</tr>
<tr>
<td>20</td>
<td>0.5</td>
</tr>
<tr>
<td>30</td>
<td>0.7</td>
</tr>
<tr>
<td>40</td>
<td>0.8</td>
</tr>
<tr>
<td>50</td>
<td>0.9</td>
</tr>
</tbody>
</table>

- Rituximab for follicular lymphoma
- Imatinib for chronic myeloid leukaemia (blast phase)
- Trastuzumab for early stage HER-2 positive breast cancer
<table>
<thead>
<tr>
<th>Health Interventions</th>
<th>comparators</th>
<th>Baht/QALY (2009)</th>
<th>Coverage decisions</th>
</tr>
</thead>
<tbody>
<tr>
<td>AZT+3TC+LPV/r for PMTCT</td>
<td>AZT plus single dose NVP</td>
<td>cost-saving</td>
<td>Yes</td>
</tr>
<tr>
<td>Provider-initiated HIV testing</td>
<td>Voluntary HIV counseling-testing</td>
<td>70,000</td>
<td>Yes</td>
</tr>
<tr>
<td>Statins in pop ≥30% CVD risk</td>
<td>exercise &amp; diet control</td>
<td>82,000</td>
<td>Yes</td>
</tr>
<tr>
<td>IV/OR form of gancyclovir for CMVR</td>
<td>Intraocular injection form</td>
<td>185,000</td>
<td>Yes</td>
</tr>
<tr>
<td>Pioglitazone for diabetes</td>
<td>Rosiglitazone</td>
<td>211,000</td>
<td>No</td>
</tr>
<tr>
<td>HPV vaccine for girls aged 15 years</td>
<td>Pap smear q 5 years aged 35-60</td>
<td>247,000</td>
<td>No</td>
</tr>
<tr>
<td>Alendronate or Residronate for osteoporosis</td>
<td>calcium + vitamin D</td>
<td>296,000 - 328,000</td>
<td>No</td>
</tr>
<tr>
<td>Cochlear implantation for profoundly deaf</td>
<td>training hand language</td>
<td>400,000</td>
<td>No</td>
</tr>
<tr>
<td>Fordable lens for cataract</td>
<td>Rigid intraocular lens</td>
<td>507,000</td>
<td>No</td>
</tr>
<tr>
<td>Atorvastatin in pop ≤30% CVD risk</td>
<td>exercise &amp; diet control</td>
<td>600,000</td>
<td>No</td>
</tr>
<tr>
<td>Peritoneal dialysis for ESRD</td>
<td>palliative care</td>
<td>435,000</td>
<td>Yes</td>
</tr>
<tr>
<td>Hemodialysis for ESRD</td>
<td>palliative care</td>
<td>449,000</td>
<td>Yes</td>
</tr>
<tr>
<td>Erythropoitin for anemia in cancer</td>
<td>blood transfusion</td>
<td>2,700,000</td>
<td>No</td>
</tr>
</tbody>
</table>

Source: HITAP
Example of using HTA in price negotiation
analysis of pricing threshold of the HPV vaccine against the WTP threshold

In Feb 09 a company announced a price reduction of the vaccine to 7,000 Baht

Source: HITAP
BACK TO THE PROCESSES...
Processes matter

• Comprehensive evidence base
• Expert input
• Independent advisory committees
• Genuine consultation
• Support for implementation
• Regular review
The decision cycle

- Evidence review
- Appraisal
- Consultation
- Guidance
- Update decision
Our Decision Making Process

- Published evidence
- Unpublished evidence; expert input; industry submissions

**POLICY MAKING:** evidence, values, UK reality

- HEALTHCARE PROFESSIONAL GROUPS
- PATIENTS AND SERVICE USERS
- ACADEMIA
- INDUSTRY
- NHS; PUBLIC SECTOR

**University group or professional association/Royal College**

**Standing (or ad hoc) independent advisory committee/expert group**
Committee Day – Part 1 and 2

ERG = Evidence Review Group

45 - 55 participants
Governance and System Strengthening

• Procedural fairness and stakeholder buy-in
  – **Transparency**: methods, evidence base and decisions are public
  – **Independence**: insulation from lobbyists and vested interests
  – **Inclusiveness**: meaningful broad public consultation and committee membership
  – **Scientific basis**: peer review and methods development
  – **Timeliness**: to meet the needs of decision makers
  – **Contestability**: appeal mechanisms
  – **Conflicts of interest**: clear policy for managing vested interests and bias
Building consensus

• Identification of key stakeholders
• Multistakeholder involvement
• Stepwise processes for evaluation and consideration of different types of evidence (from RCT to colloquial evidence)
• Enabling challenge and review
• Clear rules of engagement with different interested parties
Stakeholder input

* Published on NICE web site
Public Recruitment Process for Decision-Making Committees

Apply for the role of member to the GDG on management of hyperglycaemia in acute coronary syndrome in patients both with and without diagnosed diabetes mellitus

NICE have been commissioned by the Department of Health to develop a short clinical guideline on management of hyperglycaemia in acute coronary syndrome in patients both with and without diagnosed diabetes mellitus. We are currently seeking to recruit the following healthcare professionals for the guideline development group (GDG):

- Consultant Cardiologist
- Consultant Physician in one of the following areas; Acute Medicine, Diabetology, Accident & Emergency
- Inpatient diabetes/cardiology nurse specialist
- Clinical Pharmacist with specialist interest in patient safety
- GP
- Patient/Carer x2
Managing Vested Interests: Code of Practice for Declaring Interests (NICE 2007)

- Applies to:
  - NICE employees, NICE Chairman & non-executive board members and their families
  - Chairs and members of the advisory bodies to NICE
  - Expert advisors testifying
  - Employees of organisations contracted by NICE (including academic and professional associations)
Is there a personal pecuniary interest?

A personal pecuniary interest involves a current personal payment, which may either relate to the manufacturer or owner of a product or service being evaluated.

Example:
Any consultancy, directorship, position in or work for a healthcare industry that attracts regular or occasional payments in cash or in kind, both those which have been undertaken in the 12 months preceding the meeting at which the declaration is made and which are planned but have not taken place.
# Methods - Reference Case

## Table 5.1 Summary of the reference case

<table>
<thead>
<tr>
<th>Element of health technology assessment</th>
<th>Reference case</th>
<th>Section providing details</th>
</tr>
</thead>
<tbody>
<tr>
<td>Defining the decision problem</td>
<td>The scope developed by the Institute</td>
<td>5.2.5 &amp; 5.2.6</td>
</tr>
<tr>
<td>Comparator</td>
<td>Therapies routinely used in the NHS, including technologies regarded as current best practice</td>
<td>5.2.5 &amp; 5.2.6</td>
</tr>
<tr>
<td>Perspective on costs</td>
<td>NHS and PSS</td>
<td>5.2.7 to 5.2.10</td>
</tr>
<tr>
<td>Perspective on outcomes</td>
<td>All health effects on individuals</td>
<td>5.2.7 to 5.2.10</td>
</tr>
<tr>
<td>Type of economic evaluation</td>
<td>Cost-effectiveness analysis</td>
<td>5.2.11 &amp; 5.2.12</td>
</tr>
<tr>
<td>Synthesis of evidence on outcomes</td>
<td>Based on a systematic review</td>
<td>5.3</td>
</tr>
<tr>
<td>Measure of health effects</td>
<td>QALYs</td>
<td>5.4</td>
</tr>
<tr>
<td>Source of data for measurement of HRQL</td>
<td>Reported directly by patients and/or carers</td>
<td>5.4</td>
</tr>
<tr>
<td>Source of preference data for valuation of changes in HRQL</td>
<td>Representative sample of the public</td>
<td>5.4</td>
</tr>
<tr>
<td>Discount rate</td>
<td>An annual rate of 3.5% on both costs and health effects</td>
<td>5.6</td>
</tr>
<tr>
<td>Equity weighting</td>
<td>An additional QALY has the same weight regardless of the other characteristics of the individuals receiving the health benefit</td>
<td>5.12</td>
</tr>
</tbody>
</table>

HRQL, health-related quality of life; NHS, National Health Service; PSS, personal social services; QALYs, quality-adjusted life years.
Priority setting for setting priorities!

1. Country's own needs – systematic needs assessment
2. Criteria consistently applied (e.g., health impact,)
3. Explicit, transparent, fair process with expert topic selection panels
4. Technical support and preliminary analyses
5. Government/MoH approval part of process – MoH main client
RIGHT TO APPEAL AND JUDICIAL REVIEW
## Summary of grounds cited for appeals

1 March 2000 to 31 July 2010

<table>
<thead>
<tr>
<th>Ground</th>
<th>Number of appeals</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ground 1: fairness</td>
<td>53</td>
<td>(36%)</td>
</tr>
<tr>
<td>Ground 2: perversity</td>
<td>63</td>
<td>(43%)</td>
</tr>
<tr>
<td>Ground 3: NICE has exceeded its powers</td>
<td>32</td>
<td>(22%)</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>148</strong></td>
<td></td>
</tr>
</tbody>
</table>

The percentages in the table may not add up to 100% because appeals may be made on multiple grounds.

There are three possible grounds for appeal:

- Ground 1 - NICE has failed to act fairly and in accordance with its published procedures as set out in the ‘Guide to the technology appraisal process’
- Ground 2 - NICE has prepared a Final Appraisal Determination that is perverse in the light of the evidence submitted
- Ground 3 - NICE has exceeded its powers (that is, NICE has acted outside its remit or unlawfully in some other way)
Right to Appeal

- **Patients and Carers**: National groups representing patient and carers
- **Professionals**: Healthcare professional organisations (Colleges and Associations)
- **Industry**: Manufacturer(s) or sponsor(s) of the technology
- **Government**: The Department of Health and the Welsh Assembly Government
- **Payers**: Specialised commissioning groups, primary care trusts and local health boards
Appeals’ Panel

- Non-executive NICE directors incl. vice-chair of NICE (chair of Appeals Panel) (x2)
- NHS representative (x1)
- Industry expert (x1)
- Lay member (x1)
- + NICE’s legal advisor

30% of appeals are upheld and guidance revised! But…clear sifting process pre-appeal, so only genuine complaints go forward and process remains timely.
Who decides?

“If a (middle-income) country is perceived not to have the money to pay for vaccines, we need to go into the country to get them to prioritize that spending.”

“Disease burden estimations…cost-effectiveness studies of interventions… independent evaluations of programme implementation are examples of the kind of work that needs to be undertaken. In the absence of such capacity, current policy-making is ad hoc and driven by individual perceptions.”
Thank you!
kalipso.chalkidou@nice.org.uk