The emerging discipline of Health Technology Assessment

NHSRC, August 2012

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Director, NICE International

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National Institute for
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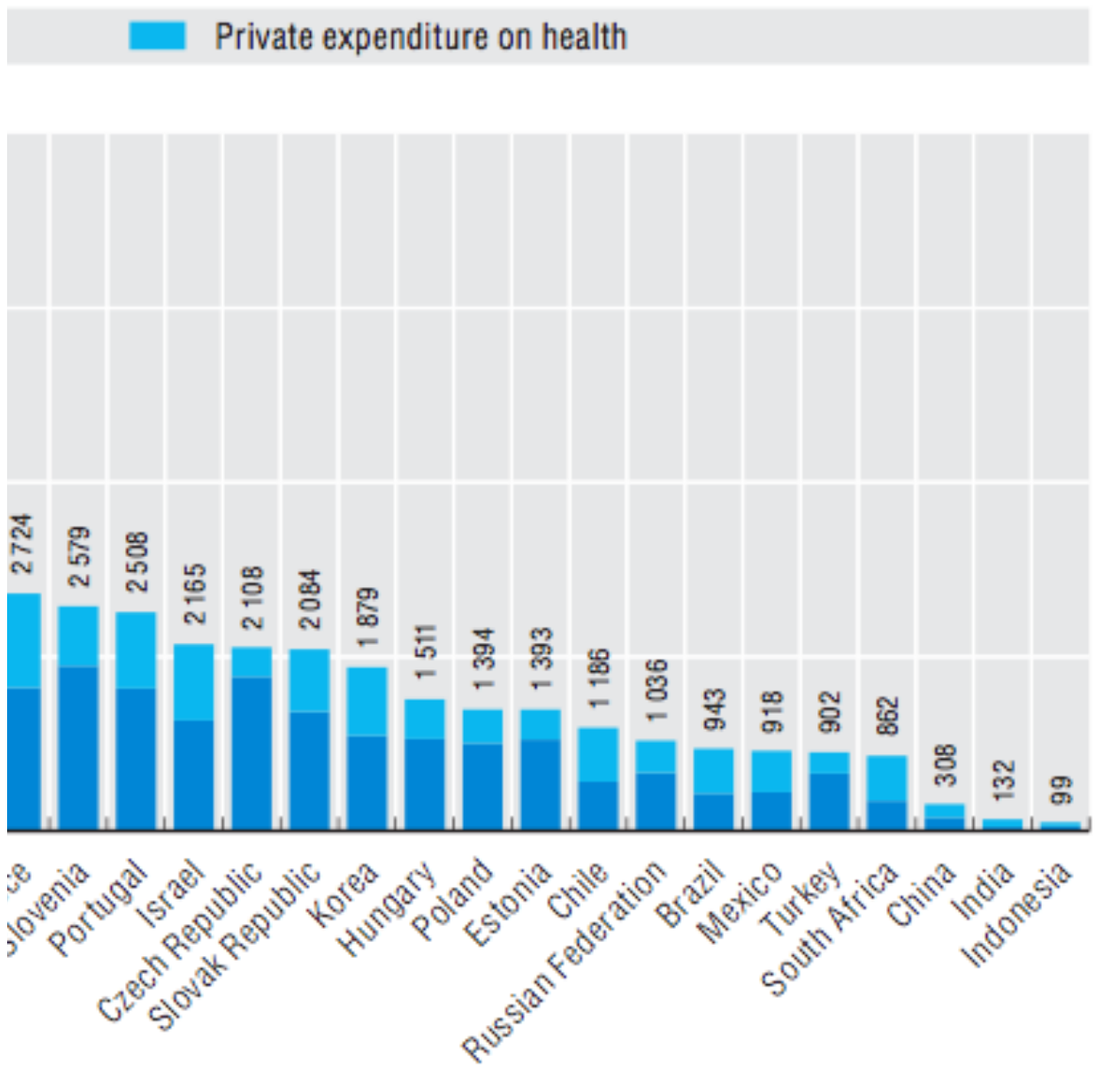
7.1.3 Total health expenditure per capita and GDP per capita, 2009 (or nearest year)

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

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

and private, 2009 (or nearest year)

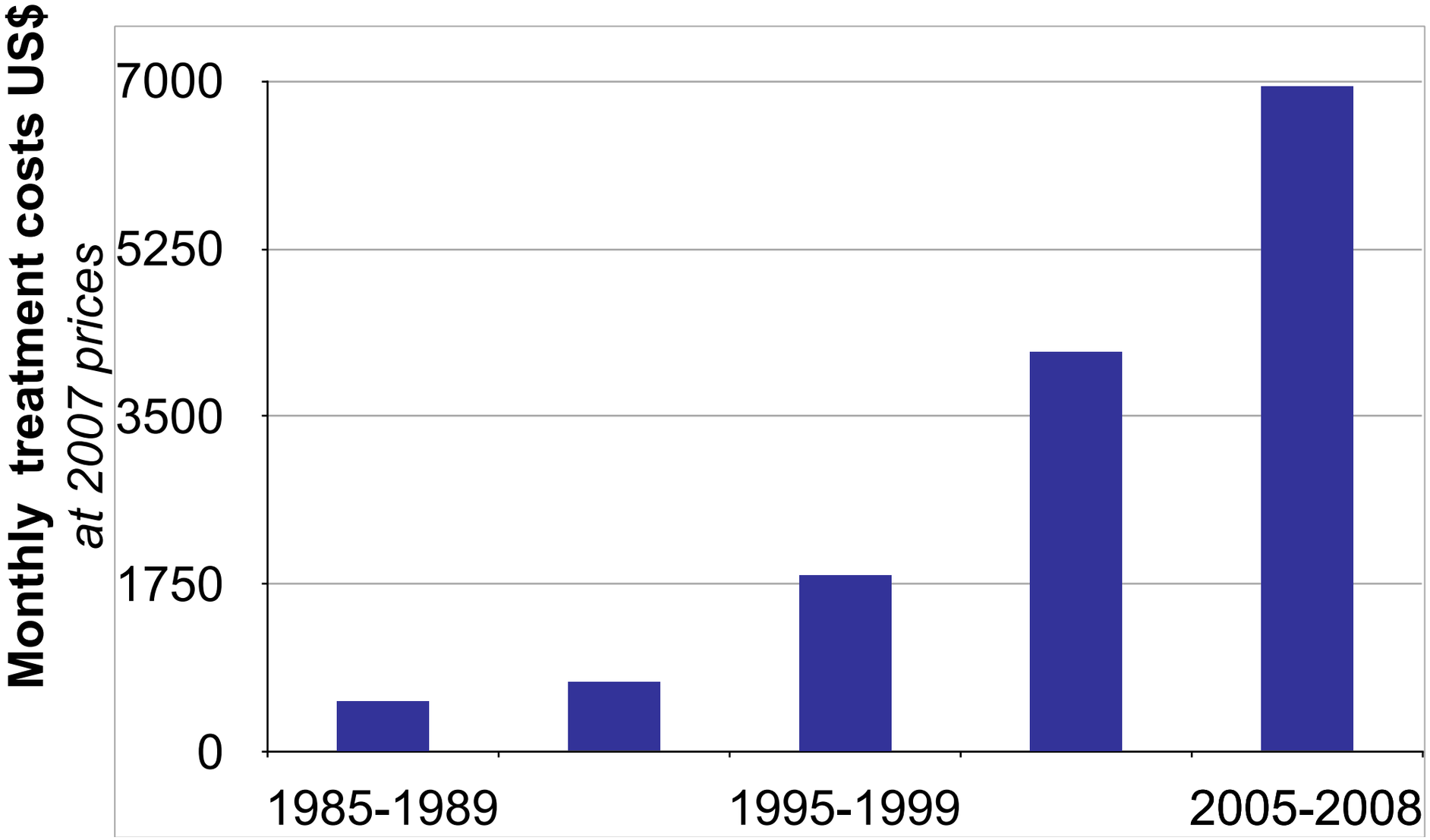


share related to investments.
tion.

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

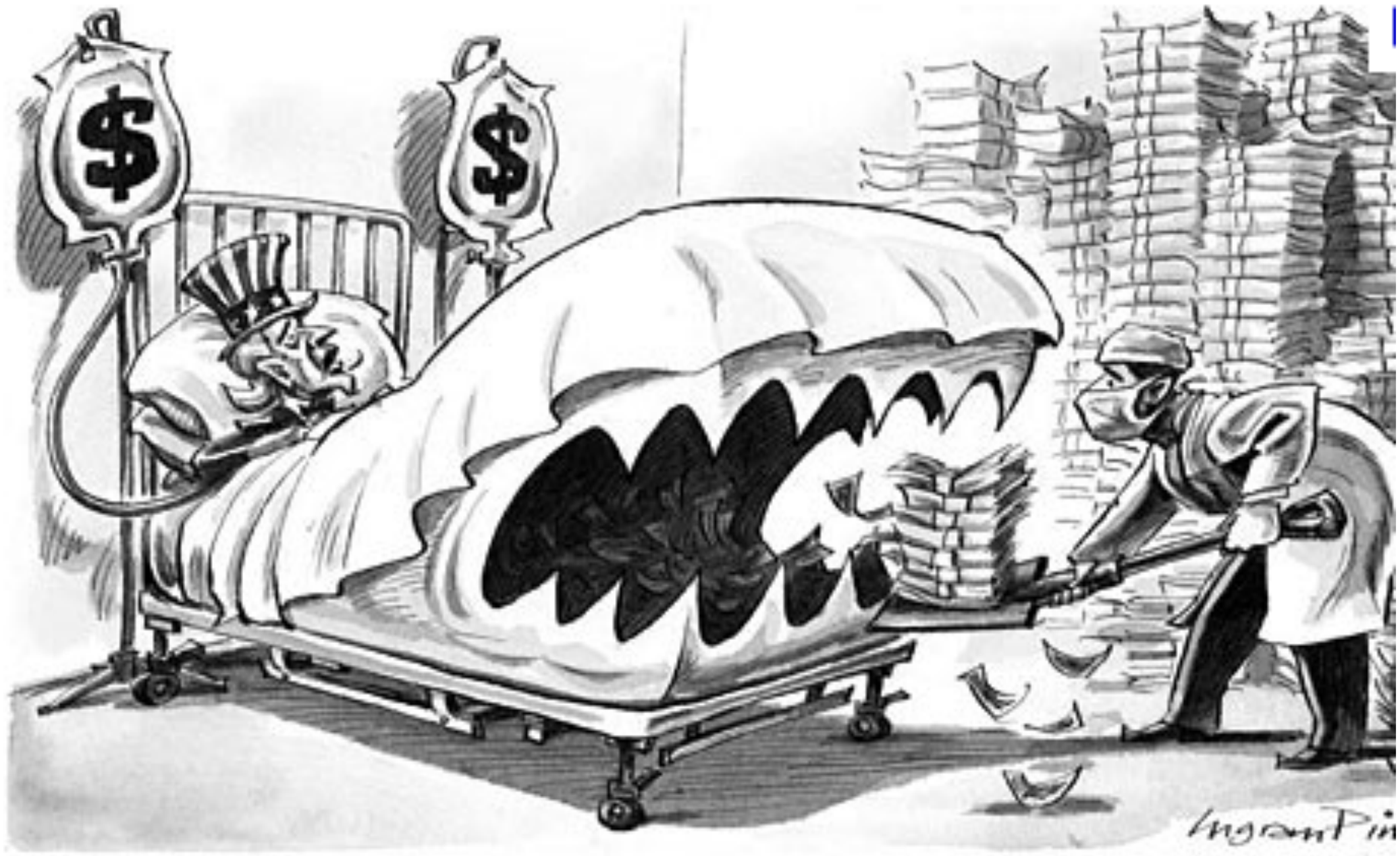
Median Monthly Costs

new anti-cancer drugs at launch



Bach et al 2009

How much is enough?



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“We cannot afford everything that is clinically effective”

"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

Principles	Putting them into practice…
Independence	***Arm's length payers, industry and professional groups; strong and enforced conflict of interest policies***
Transparency	Meetings open to the public; material placed on the web; decision criteria and rationale for individual decisions, public
Inclusiveness	***Wide and genuine consultation with stakeholders; willingness to change decision in light of new evidence***
Scientific basis	Strong, scientific methods and reliance on critically appraised evidence and information
Timeliness	***Decisions produced in reasonable timeframe; minimal delays in publishing decisions***
Consistency	Same technical and process rules applied to all cases
Legal framework	***Referenced in country's legal framework; institutional role in informing coverage/payment decisions;***
Regular review	Regular updating of its decisions and of its methods

HTA: WHAT IS IT?

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What is Health Technology Assessment?

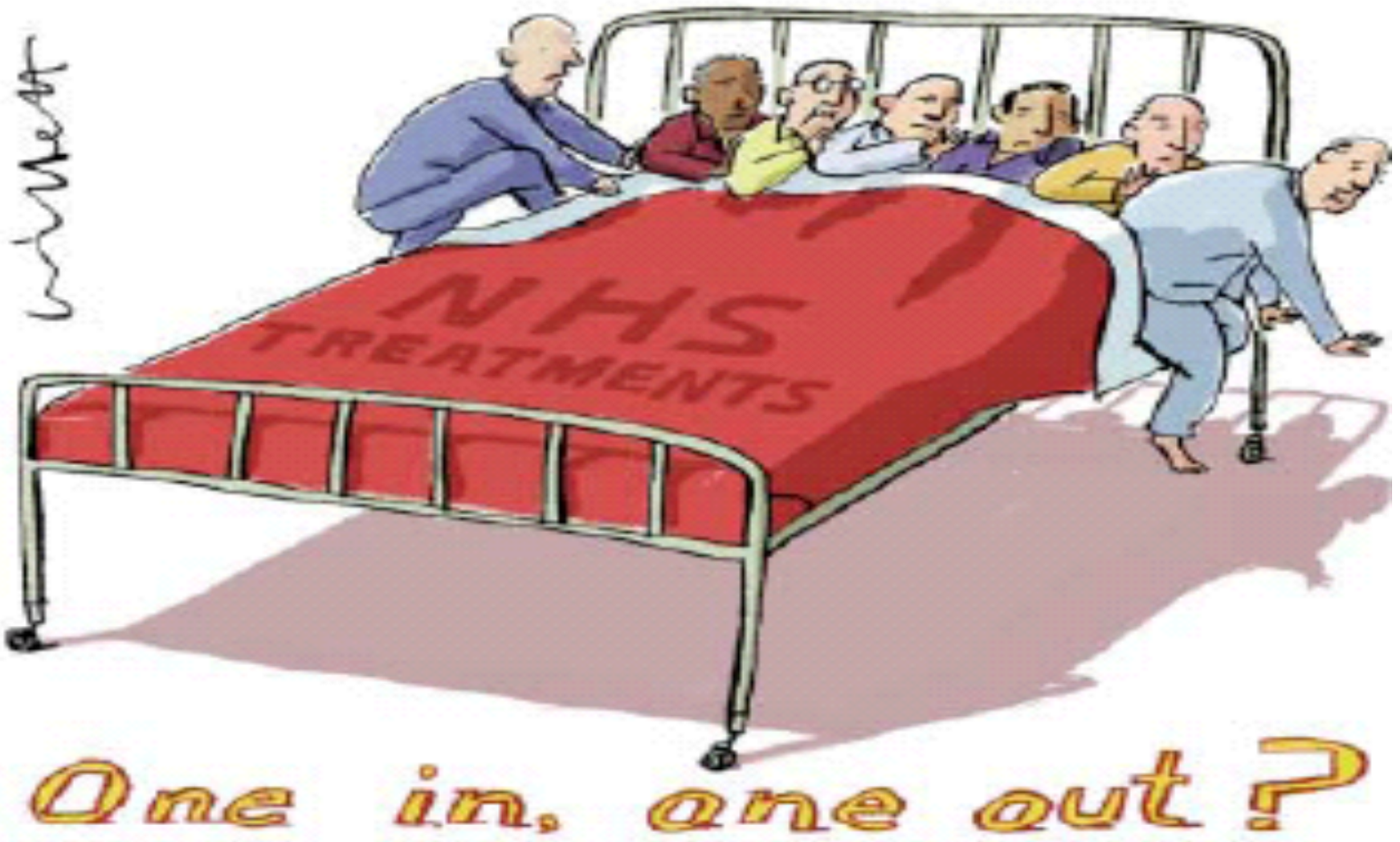
- Health Technology: “The drugs, devices, and medical and surgical procedures used in health care, and the organisational and supportive systems within which such care is provided”[a]
  - Contraceptives; dialysis machines; mastectomy; screening for cancer; intensive care unit
- Health Technology Assessment: “a multi-disciplinary field of policy analysis, which studies the medical, social, ethical and economic implications of development, diffusion and use of health technology.”[b]

a: Office of Technology Assessment. Assessing the efficacy and safety of medical technologies. Washington DC: U.S. Government Printing Office, 1978;
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



Criteria for decision-making: assessing cost-effectiveness

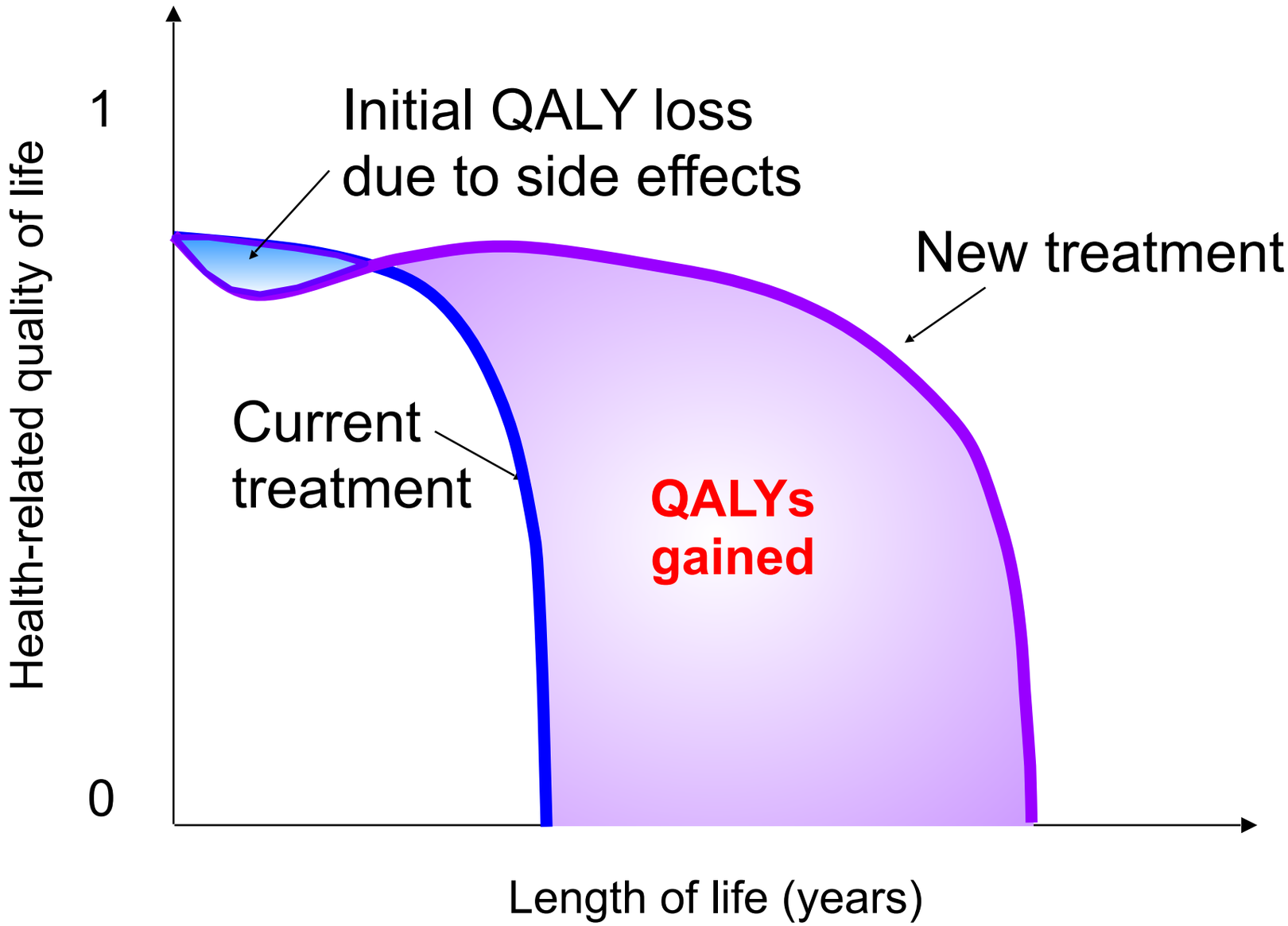
1. How well does the technology work compared to standard practice in OUR healthcare system?
2. How much does the technology cost compared to standard practice in OUR healthcare system?
   - cost of technology, monitoring, length of inpatient or outpatient stay, costs of treating adverse events
3. Health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

$$\frac{\text{Difference in costs}}{\text{Difference in effect}}$$

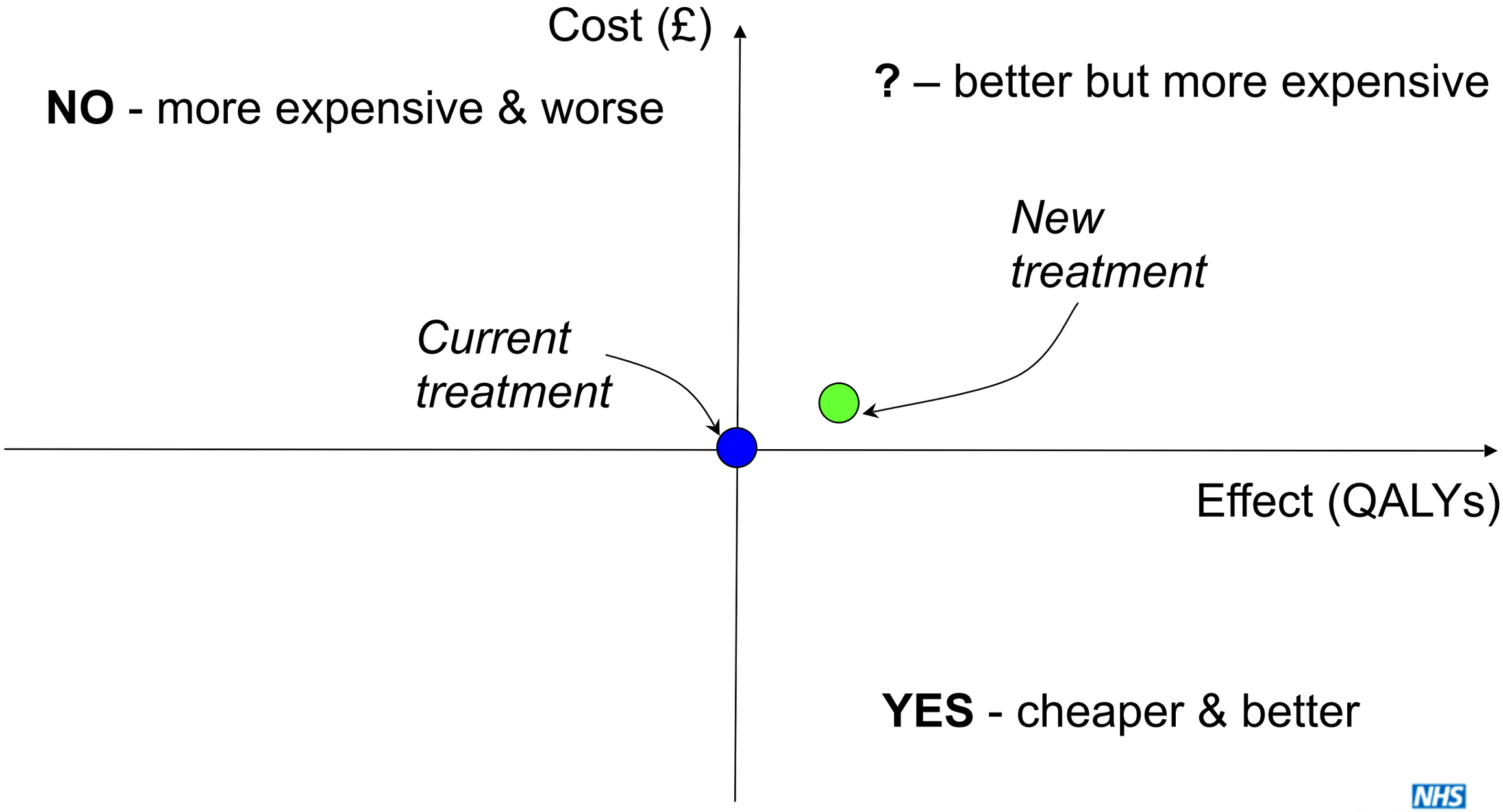
Quality adjusted life years (QALYs)

- **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

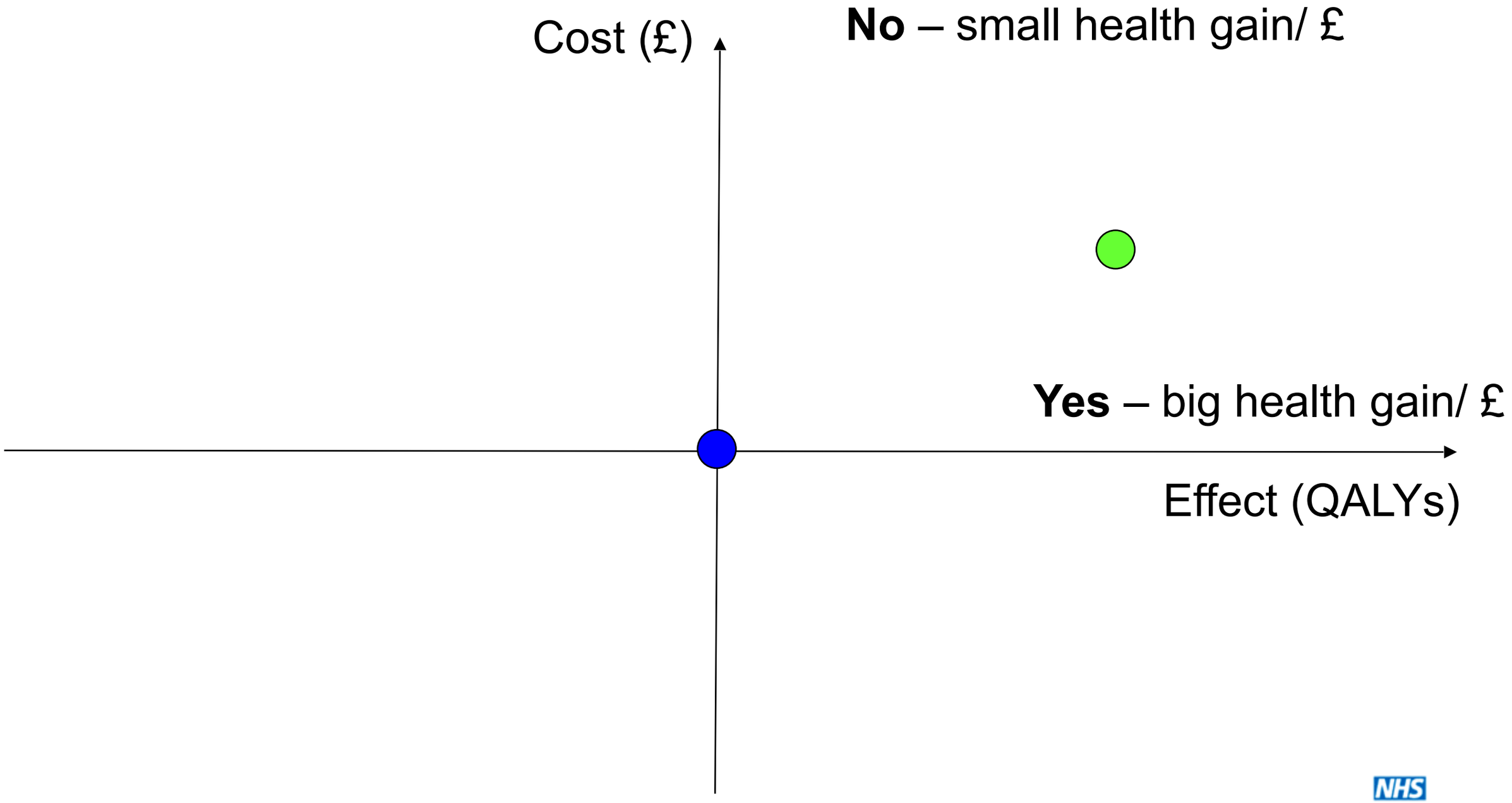
The **Q**uality **A**djusted **L**ife **Y**ear



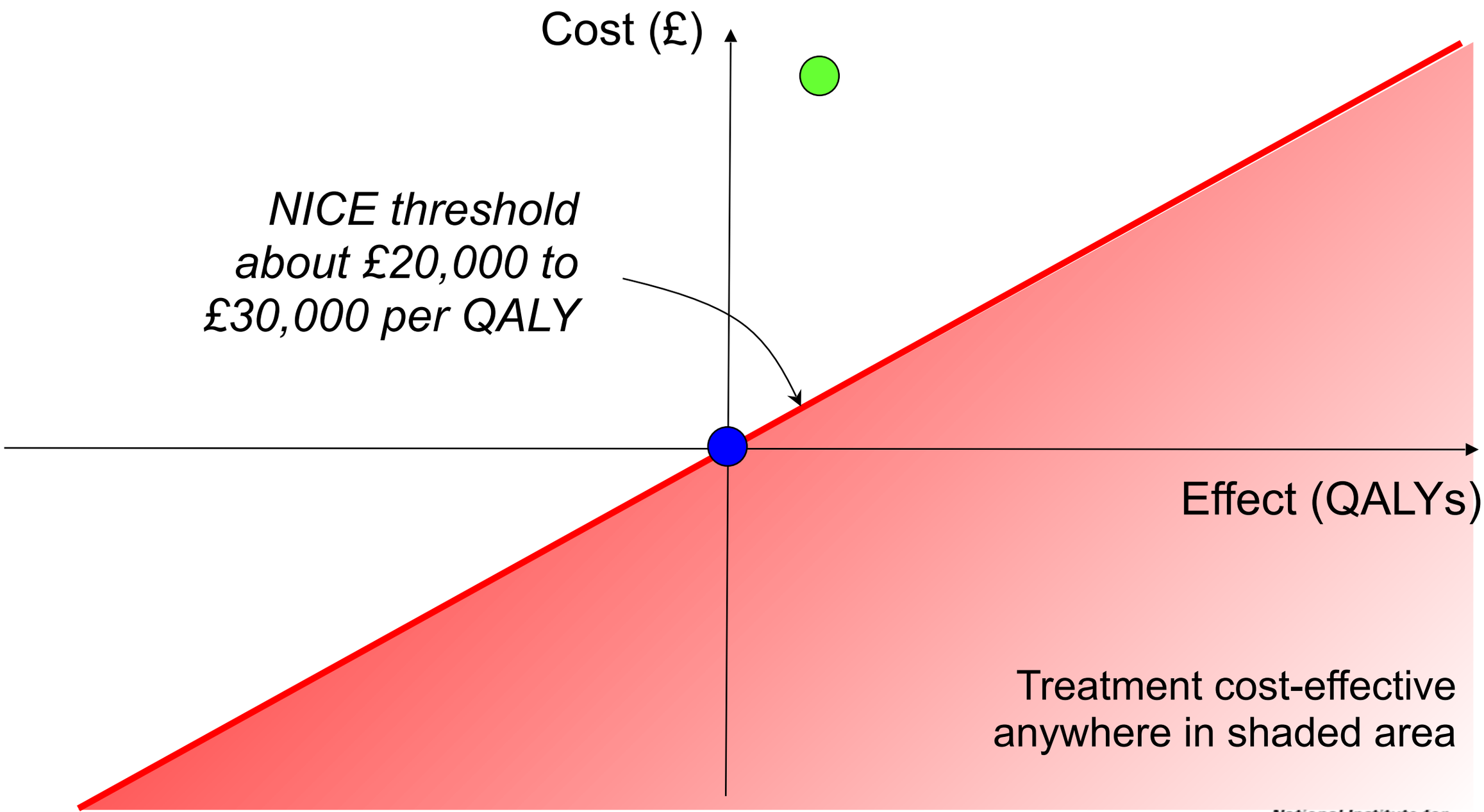
Trading off benefits, harms and costs



… but is it cost-effective?



Where is the threshold?



USING HTA TO DECIDE WHAT TO PAY FOR

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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

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

Shadow price

Healthcare budget fixed

The Willingness To Pay approach

Selected interventions	$/QALY
Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and high risk for stroke	Cost-saving
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Healthcare budget needed

WTP threshold

The threshold approach

Selected interventions	$/QALY
Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and high risk for stroke	Cost-saving
Thrombolytic therapy with intracoronary streptokinase vs. conventional therapy in patients with ECG evidence of AMI and duration of symptoms < 4 hours	$4,800
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Estimated threshold

?

Budget impact

The reallocation approach

Selected interventions	$/QALY
Warfarin vs. aspirin in 65 year-old with nonvalvular atrial fibrillation and high risk for stroke	Cost-saving
Thrombolytic therapy with intracoronary streptokinase vs. conventional therapy in patients with ECG evidence of AMI and duration of symptoms < 4 hours	$4,800
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Estimated threshold

Budget neutral

HERG

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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

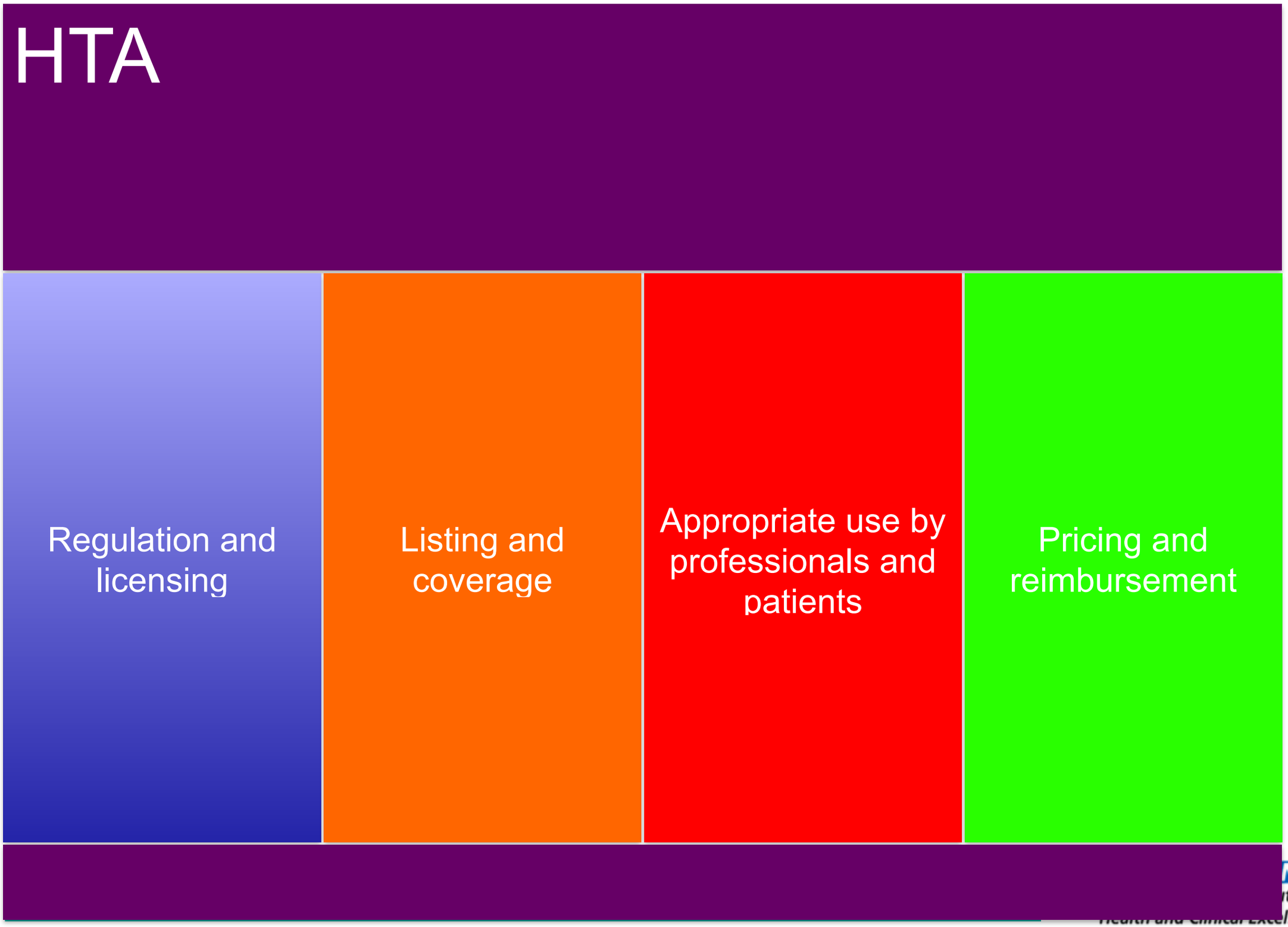
Things are never as easy as they seem!

"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



BACK TO THE REAL WORLD: COUNTRY CASE STUDIES

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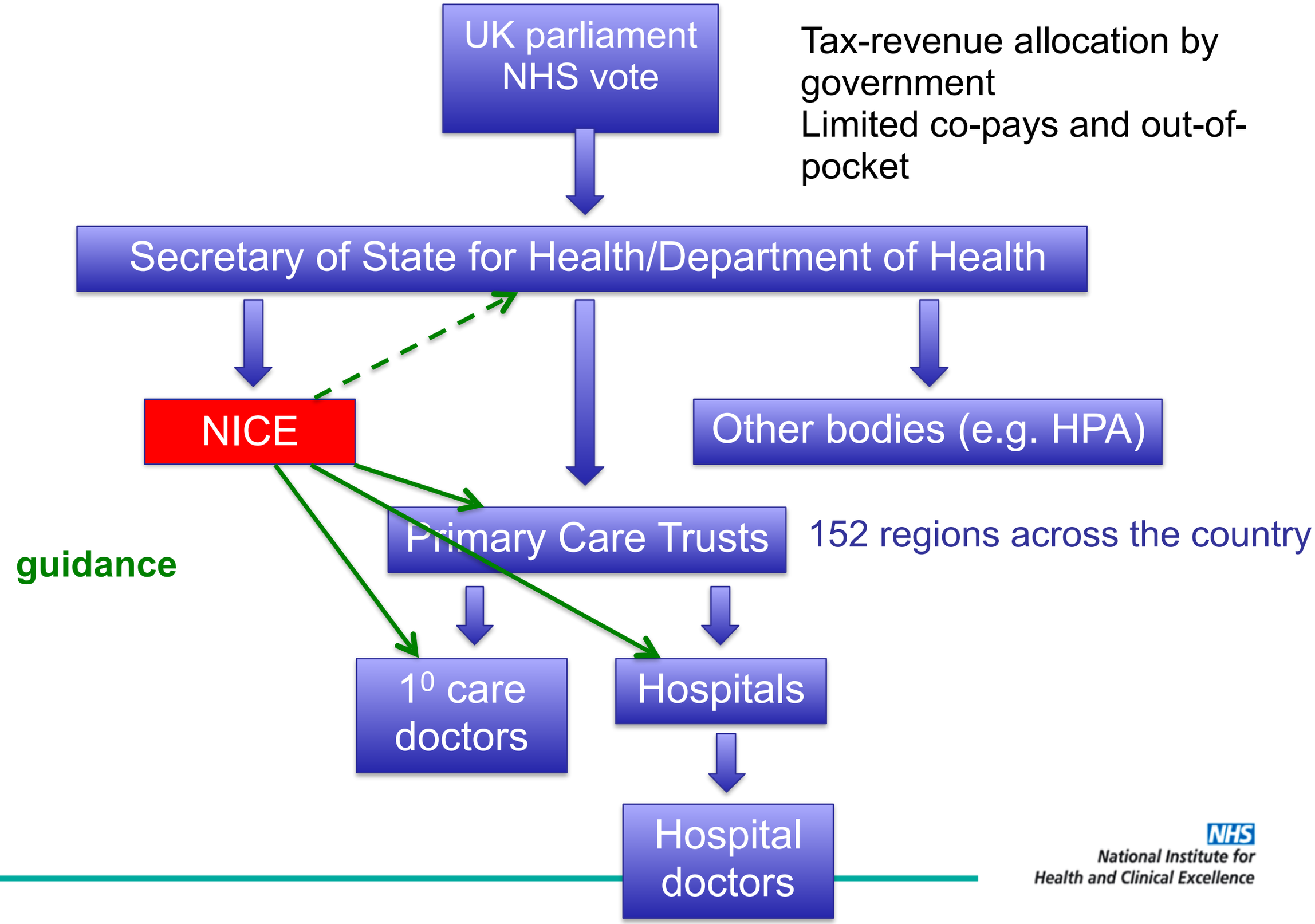
The NHS (currently…)



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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
(1st June, 2011)

Type	Numbers
Technology appraisals	224
Clinical guidelines and cancer service guidance	133
Interventional procedures	349
Medical Technologies	3
Public health	35
Total	744

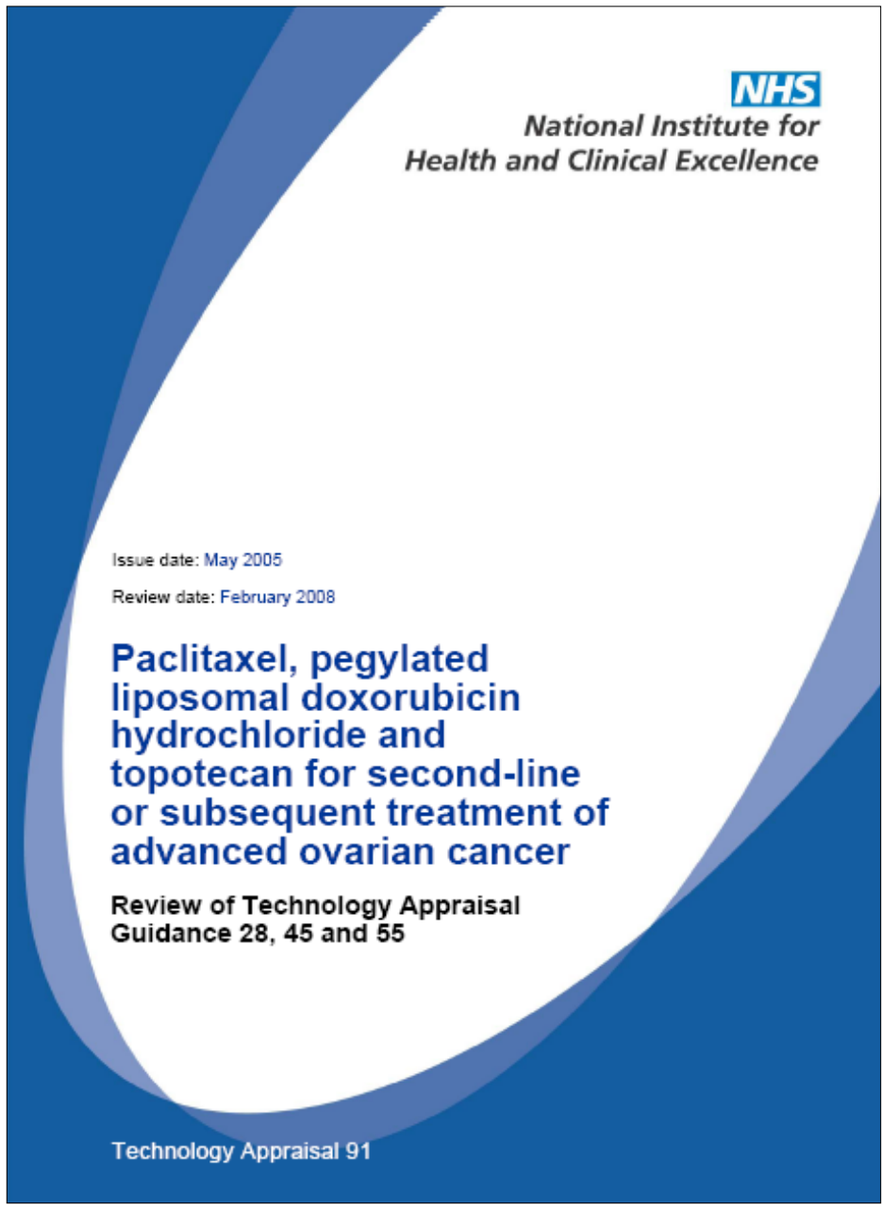
Technology appraisals

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

Two types of appraisals:

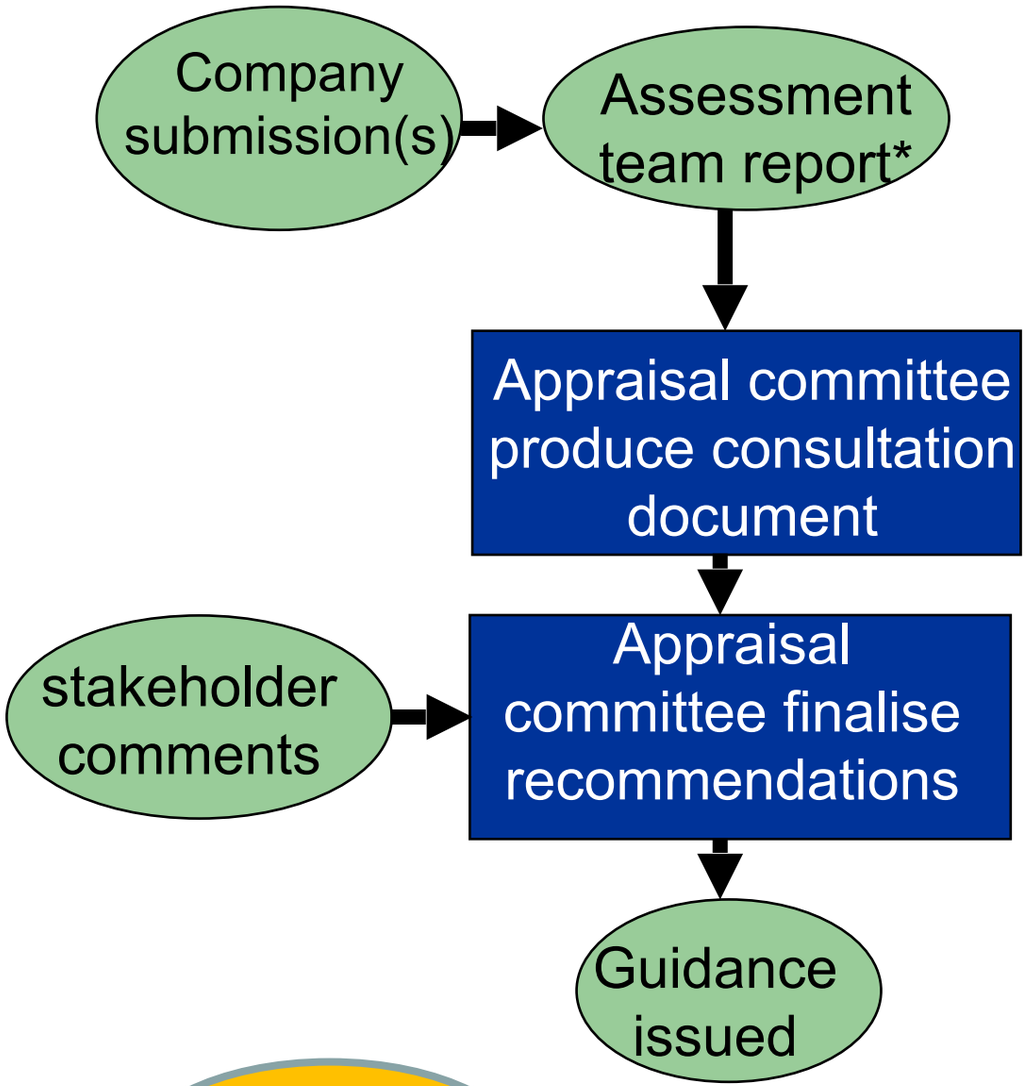
Multiple Technology Appraisal (MTA)
Single Technology Appraisal (STA)

- Independent academic groups carry out systematic review and develop economic model (MTA)
- Critique the evidence submitted by manufacturer (STA)
- 4 standing Committees (33 members each)

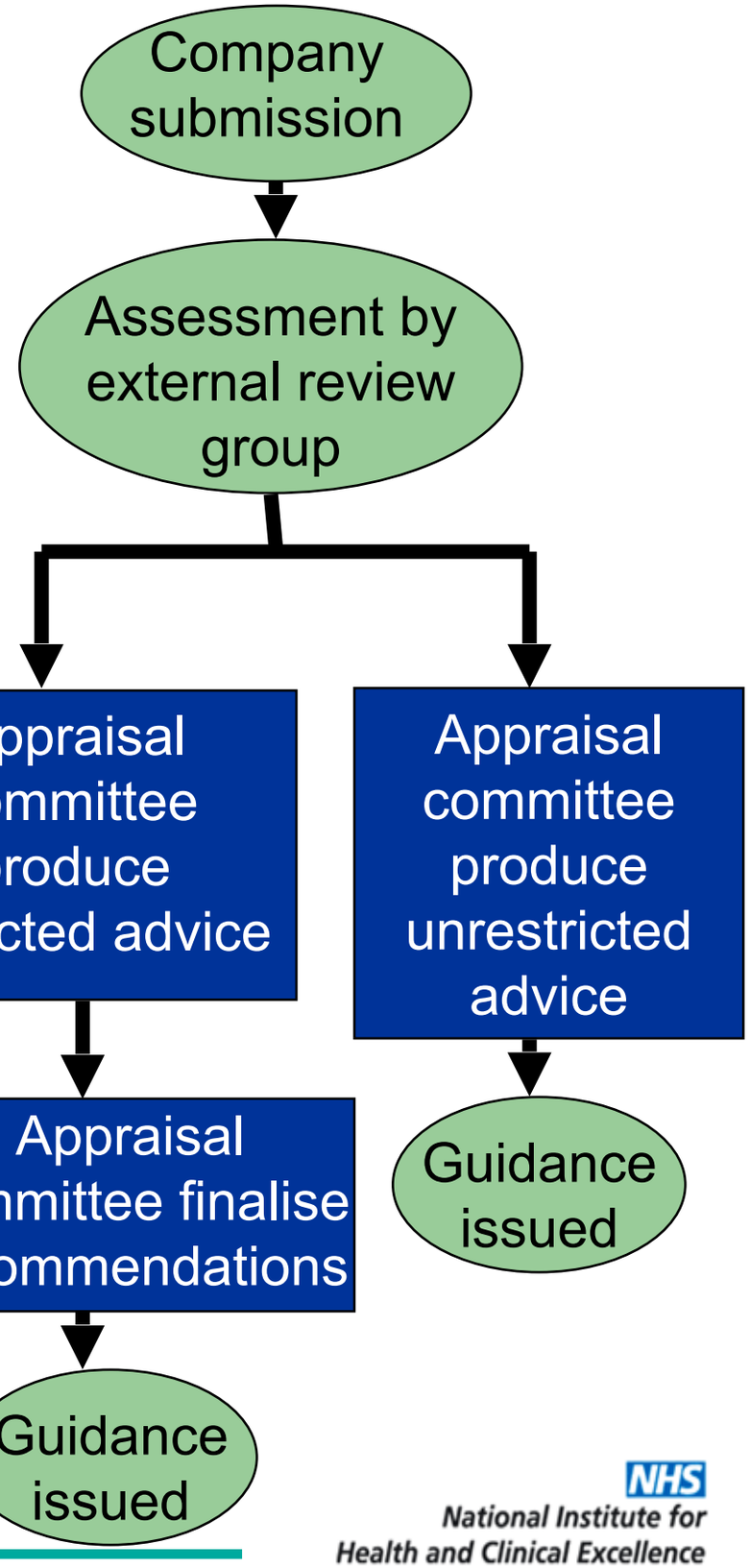


Recommendations to be implemented within 3 months

MTA



STA



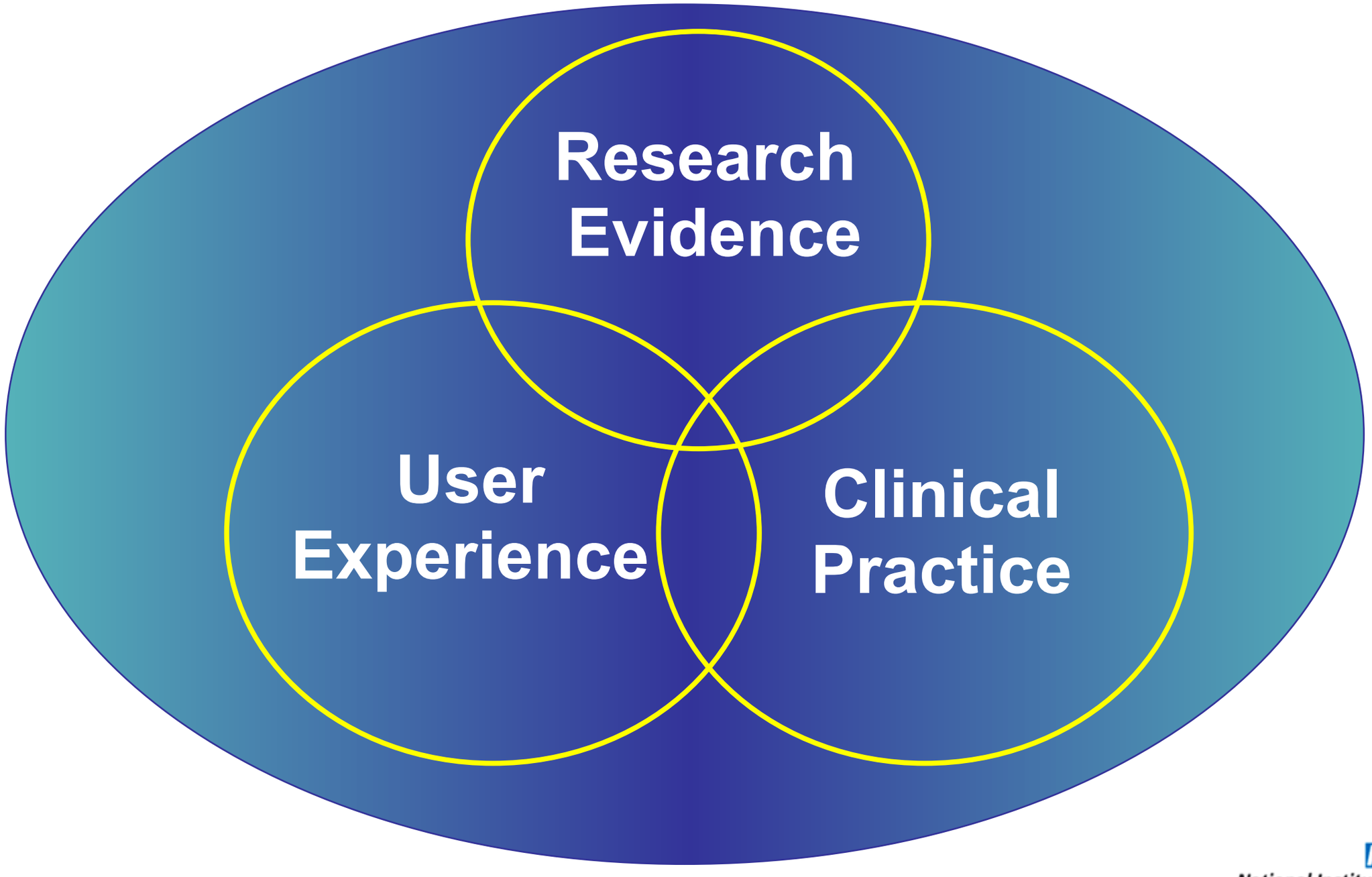
* Consulted on

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

Recommendation type	Number (%)
‘Recommended’ (Full use)	266 (63%)
‘Optimised’ (Restricted use)	80 (19%)
‘Only in research’	24 (6%)
‘Not recommended’ (No use)	50 (12%)
TOTAL	420 (100%)

Overall, 82% of decisions made by NICE (346 of 420) were ´recommended' or ´optimised´.

The evidence NICE needs



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

Comments (71) | Add to My Stories

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

Value based pricing and multiple thresholds

- “We will pay drug companies according to the value of new

Th
go

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

Consultation document on VBP, Dec 2010

DH Department

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

?

A new value-based approach to the pricing of branded medicines

A consultation

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THE WALL STREET JOURNAL.
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WSJ.com MarketWatch BARRON'S All Things Digital. FINS SmartMoney
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GSK offers UK rebate if Pfizer cancer drug better

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.

(Reuters) - Britain's state-run health service will get a

"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

clinical trial.

Share this

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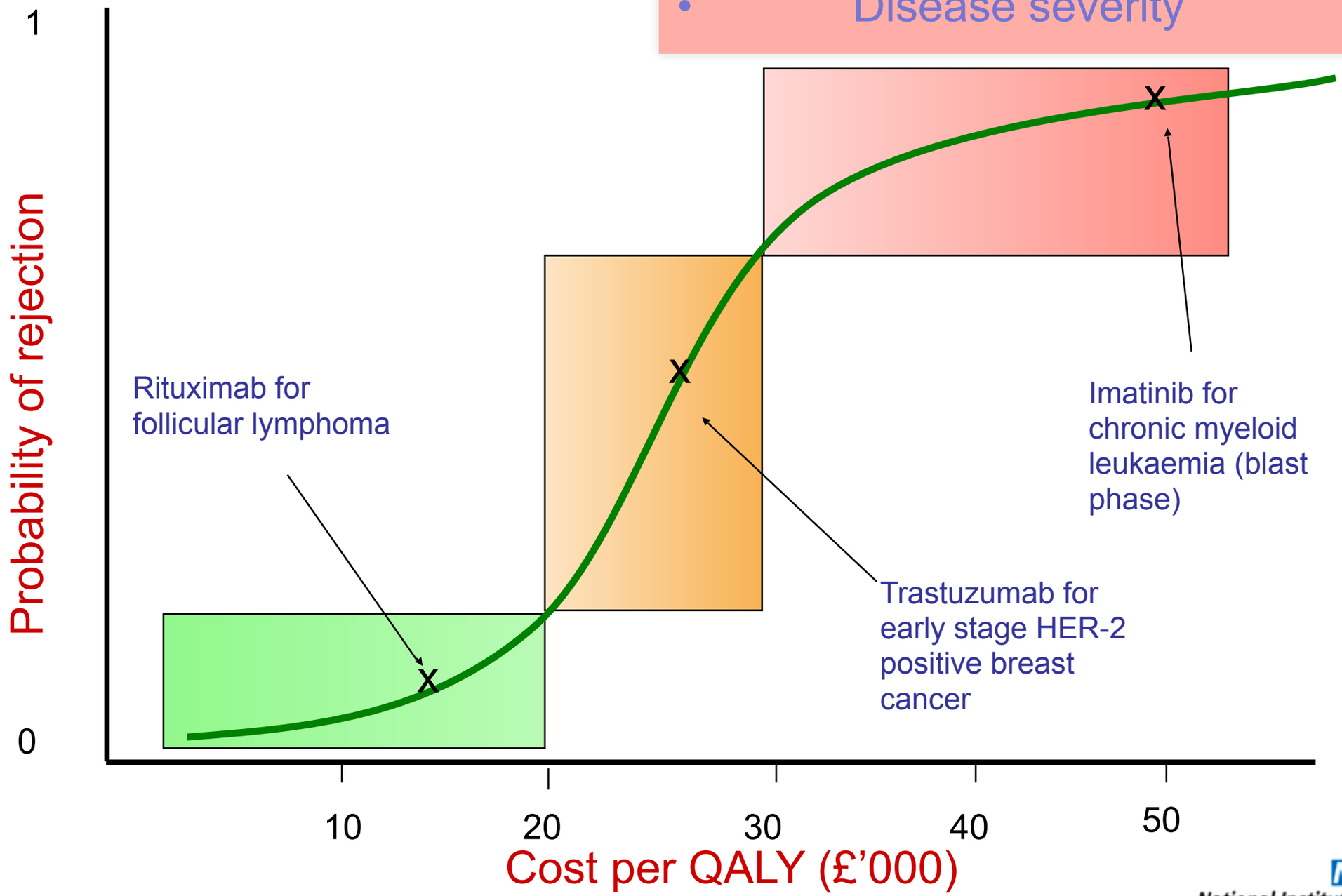
NICE: a negative list for technologies

TA007	2000	MTA	Rabeprazole	Dyspepsia	Recommended	Guidance has been incorporated in CG17. Recommendation in line with marketing authorisation.
TA008	2000	MTA	Digital hearing aids	Deafness	Not Recommended	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.
TA008	2000	MTA	Analogue hearing aids	Deafness	Recommended	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.
TA009	2000	MTA	Rosiglitazone	Type 2 diabetes	Recommended	Guidance has been replaced by TA63 and incorporated in CG66. Recommendation in line with marketing authorisation.
TA010	2000	MTA	Dry powder inhalers (DPI)	Asthma (children under 5 years)	Recommended	Recommendation in line with marketing authorisation.
TA010	2000	MTA	Nebulised therapy	Asthma (children under 5 years)	Recommended	Recommendation in line with marketing authorisation.
TA010	2000	MTA	Pressurised metered dose inhalers (pMDI) and spacer system	Asthma (children under 5 years)	Recommended	Recommendation in line with marketing authorisation.
TA011	2000	MTA	Implantable cardioverter defibrillators (ICDs)	Arrhythmias	Recommended	Guidance has been replaced by TA95. Recommendation in line with clinical practice.
TA012	2000	MTA	Abciximab (intravenous)	Acute coronary syndromes	Recommended	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



Cost-effectiveness league table of selected interventions in Thailand

Health Interventions Source: HITAP	comparators	Baht/QALY (2009)	Coverage decisions
AZT+3TC+LPV/r for PMTCT	AZT plus single dose NVP	cost-saving	Yes
Provider-initiated HIV testing	Voluntary HIV counseling-testing	70,000	Yes
Statins in pop ≥30% CVD risk	exercise & diet control	82,000	Yes
IV/OR form of gancyclovir for CMVR	Intraoccular injection form	185,000	Yes
Pioglitazone for diabetes	Rosiglitazone	211,000	No
HPV vaccine for girls aged 15 years	Pap smear q 5 years aged 35-60	247,000	No
Alendronate or Residronate for osteoporosis	calcium + vitamin D	296,000 - 328,000	No
Cochlear implantation for profoundly deaf	training hand language	400,000	No
Fordable lens for cataract	Rigid intraoccular lens	507,000	No
Atorvastatin in pop ≤30% CVD risk	exercise & diet control	600,000	No
Peritoneal dialysis for ESRD	palliative care	435,000	Yes
Hemodialysis for ESRD	palliative care	449,000	Yes
Erythropoitin for anemia in cancer	blood transfusion	2,700,000	No

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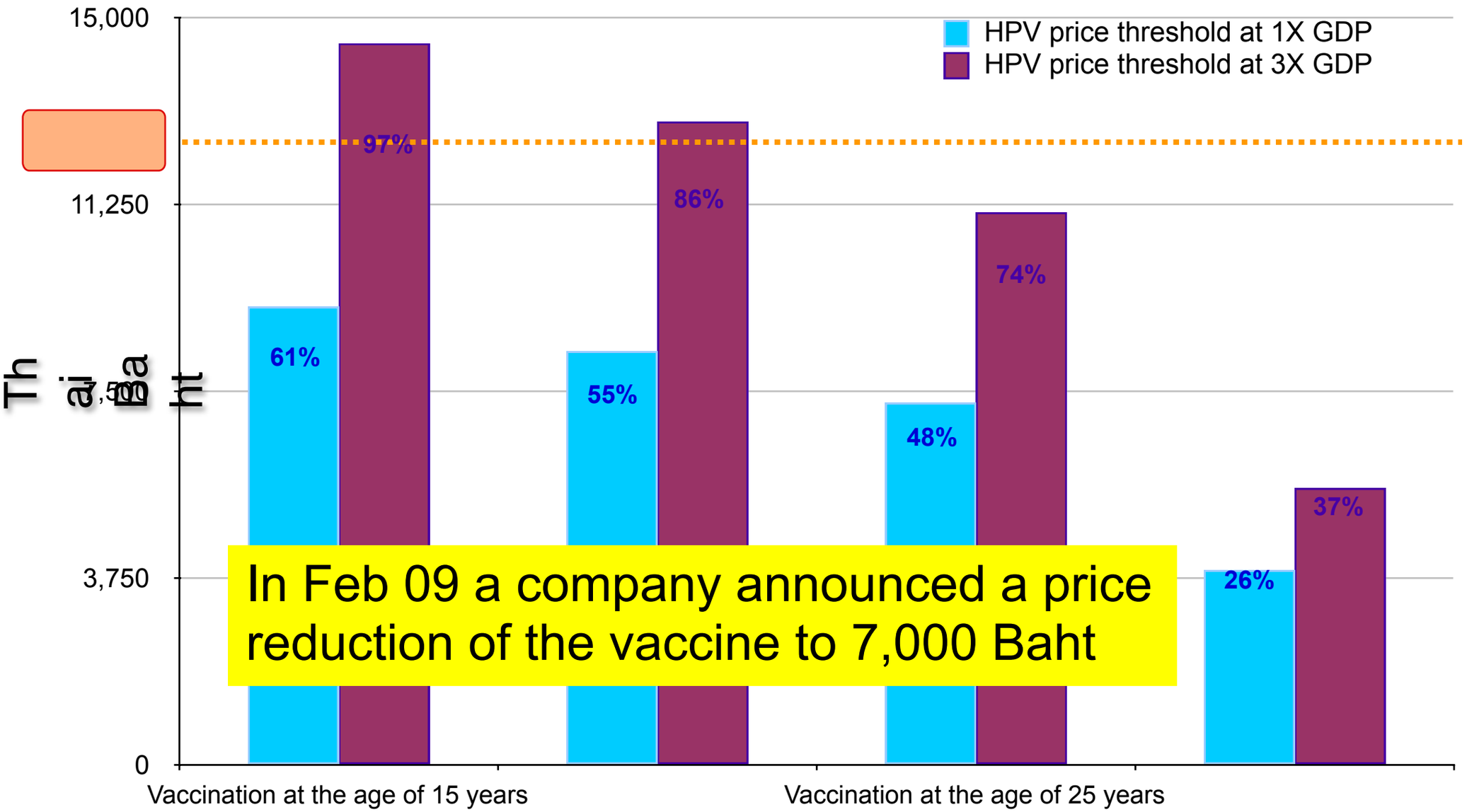
Example of using HTA in price negotiation

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

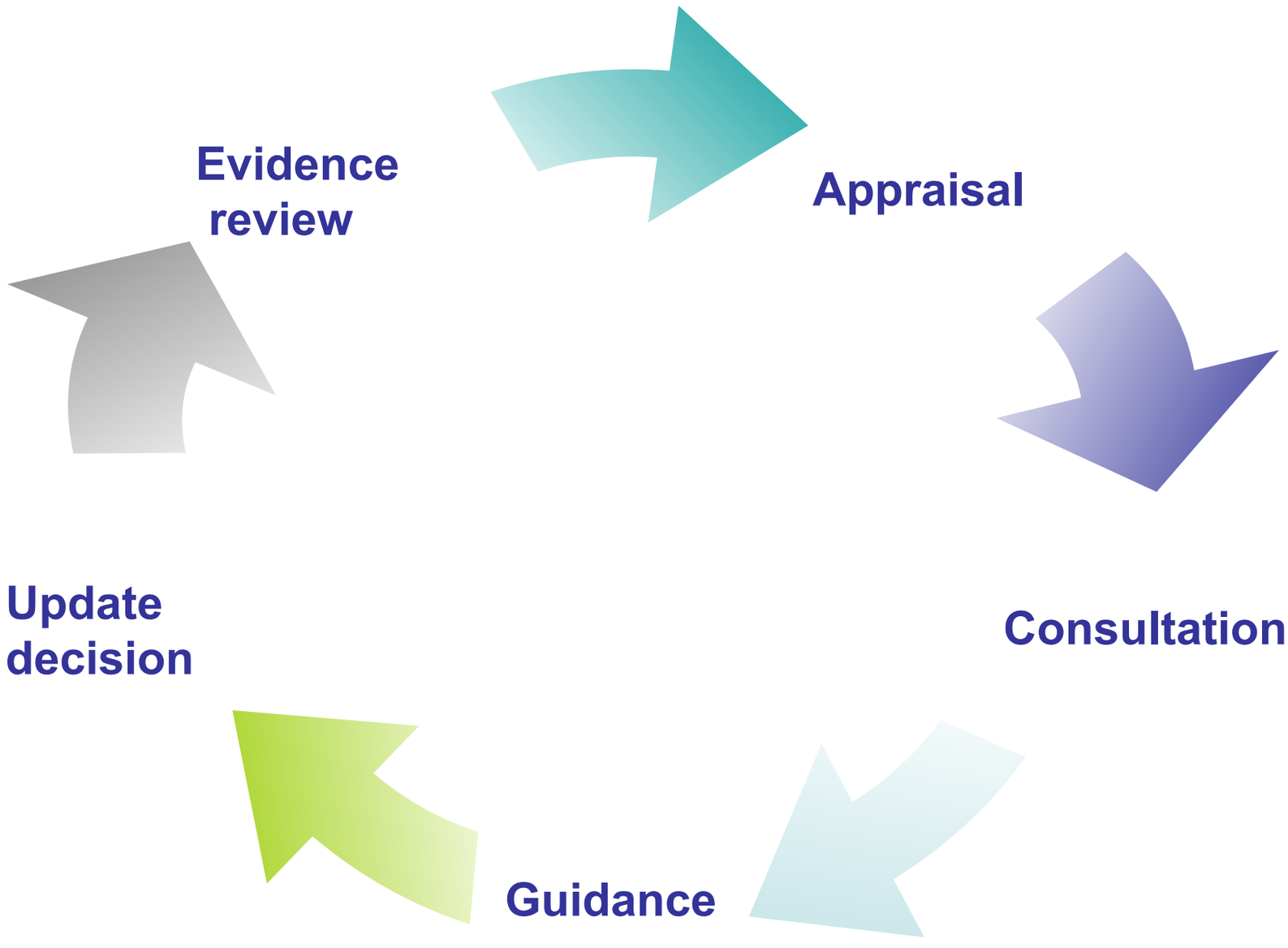
Source: HITAP

BACK TO THE PROCESSES…

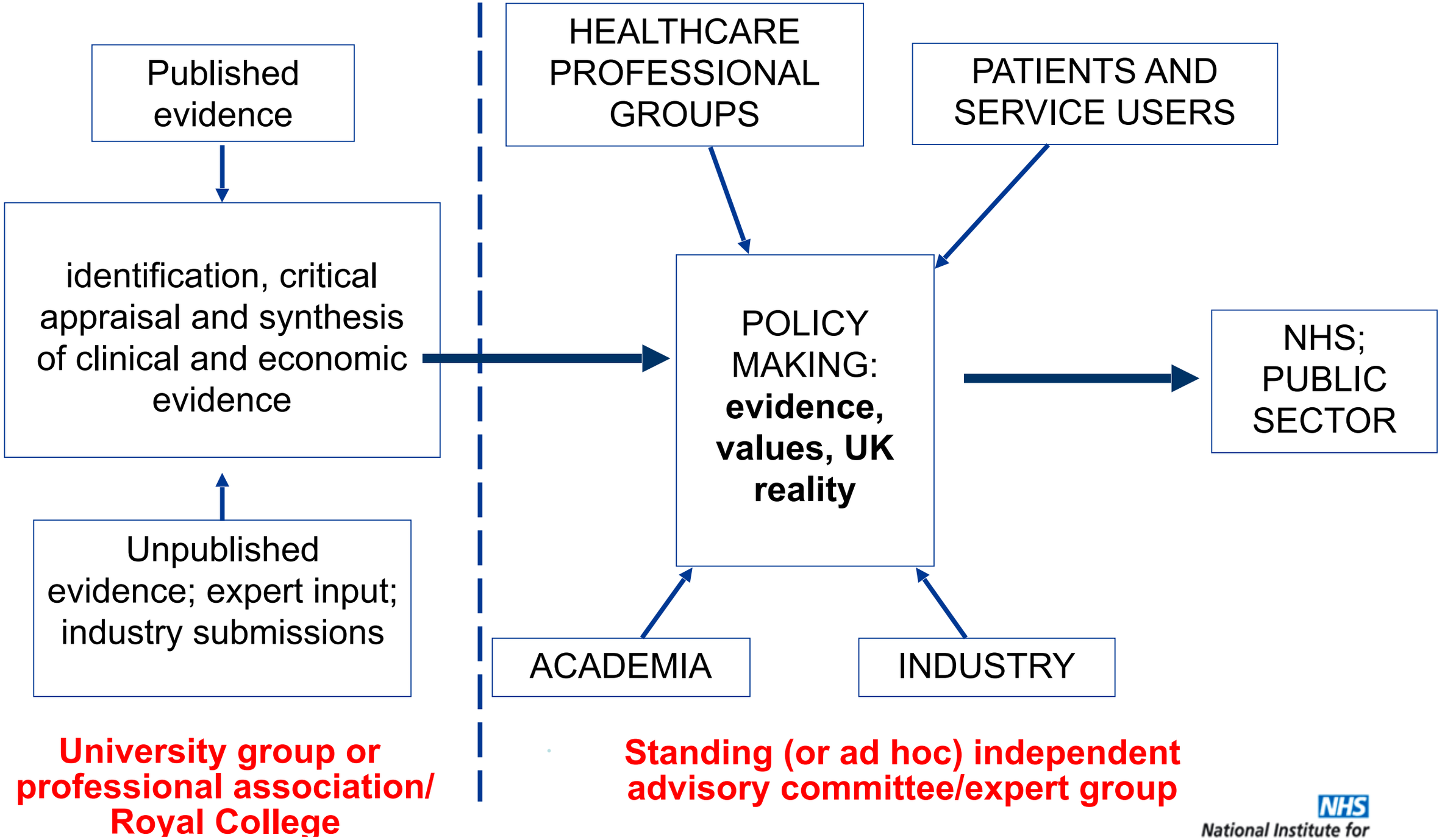
Processes matter

- Comprehensive evidence base
- Expert input
- Independent advisory committees
- Genuine consultation
- Support for implementation
- Regular review

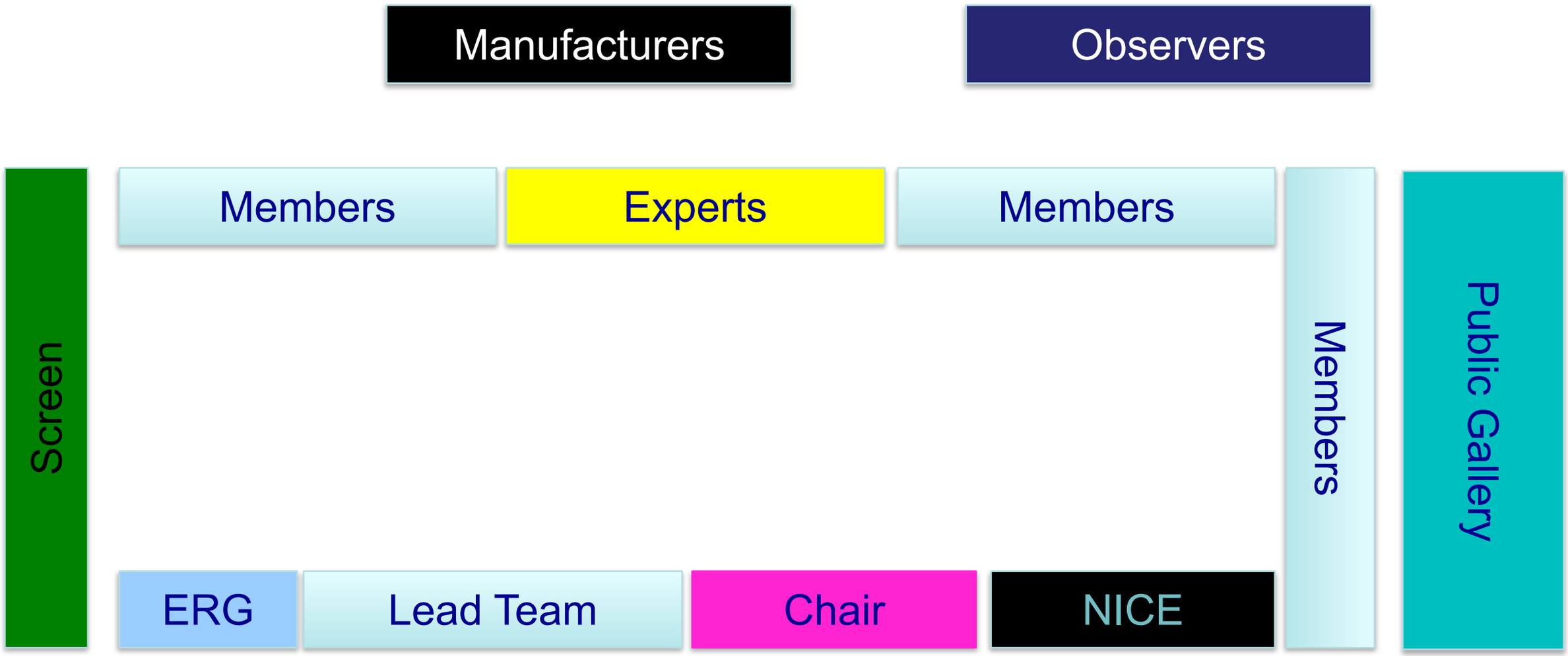
The decision cycle



Our Decision Making Process



Committee Day – Part 1 and 2



ERG = Evidence Review Group *45 - 55 participants*

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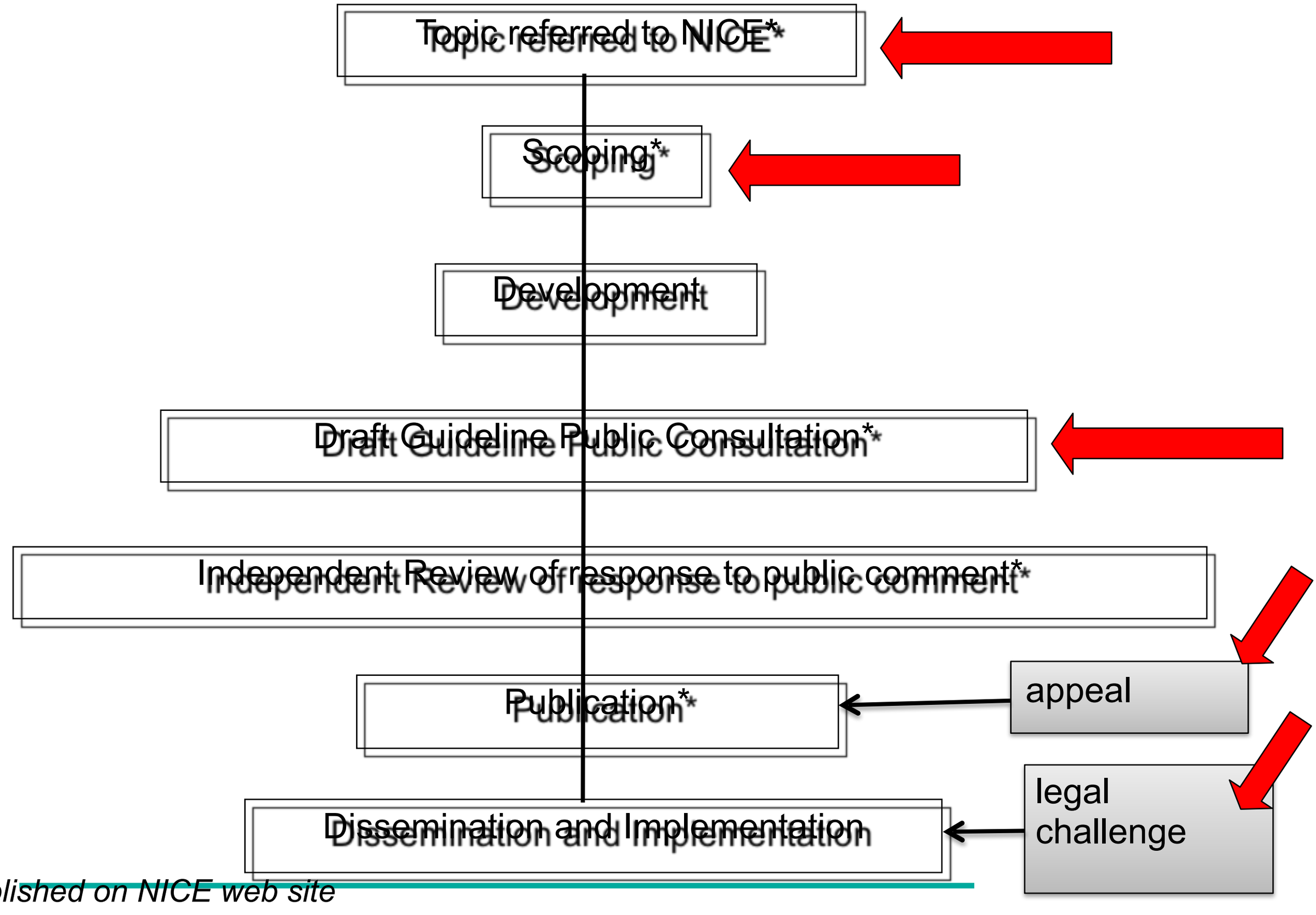
Governance and System Strengthening

- 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

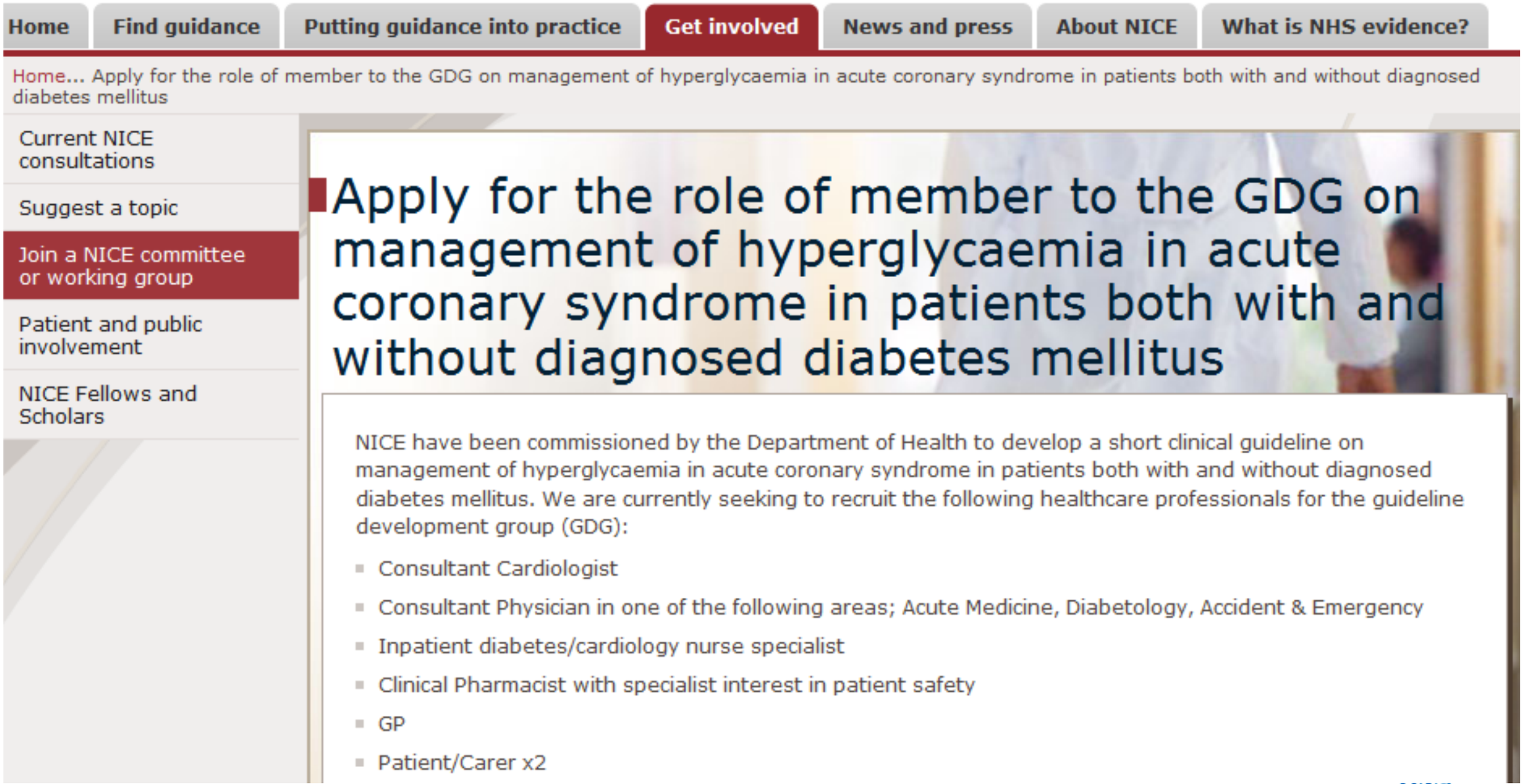
- Identification of key stakeholders
- Multistakeholder involvement
- Stepwise processes for evaluation and consideration of different types of evidence (from RCT to colloquial evidence)
- Enabling challenge and review
- Clear rules of engagement with different interested parties

Stakeholder input



* *Published on NICE web site*

Public Recruitment Process for Decision-Making Committees



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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

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

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

Priority setting for setting priorities!

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RIGHT TO APPEAL AND JUDICIAL REVIEW

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Summary of grounds cited for appeals

1 March 2000 to 31 July 2010	Number of appeals	
Ground 1: fairness [a]	53	(36%)
Ground 2: perversity [b]	63	(43%)
Ground 3: NICE has exceeded its powers [c]	32	(22%)
Total	**148**	

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:
[a] 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'
[b] Ground 2 - NICE has prepared a Final Appraisal Determination that is perverse in the light of the evidence submitted
[c] 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."

- Bill Gates, GAVI fundraising meeting, London, June 13, 2011

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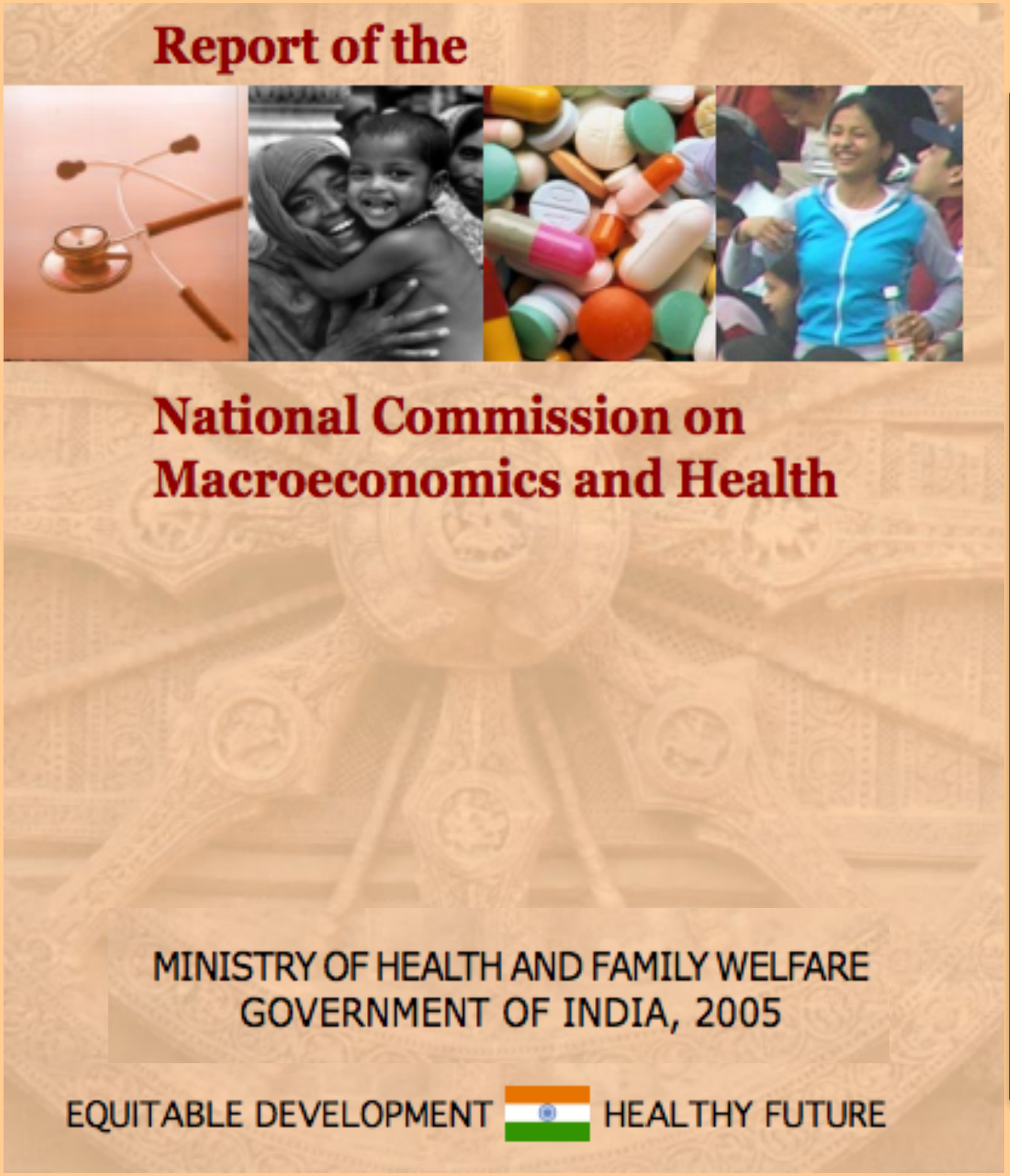

"Disease burden estimations…cost-effectiveness studies of interventions… independent evaluations of programme implementation are examples of the kind of work that needs to be undertaken. In the absence of such capacity, current policy-making is ad hoc and driven by individual perceptions."

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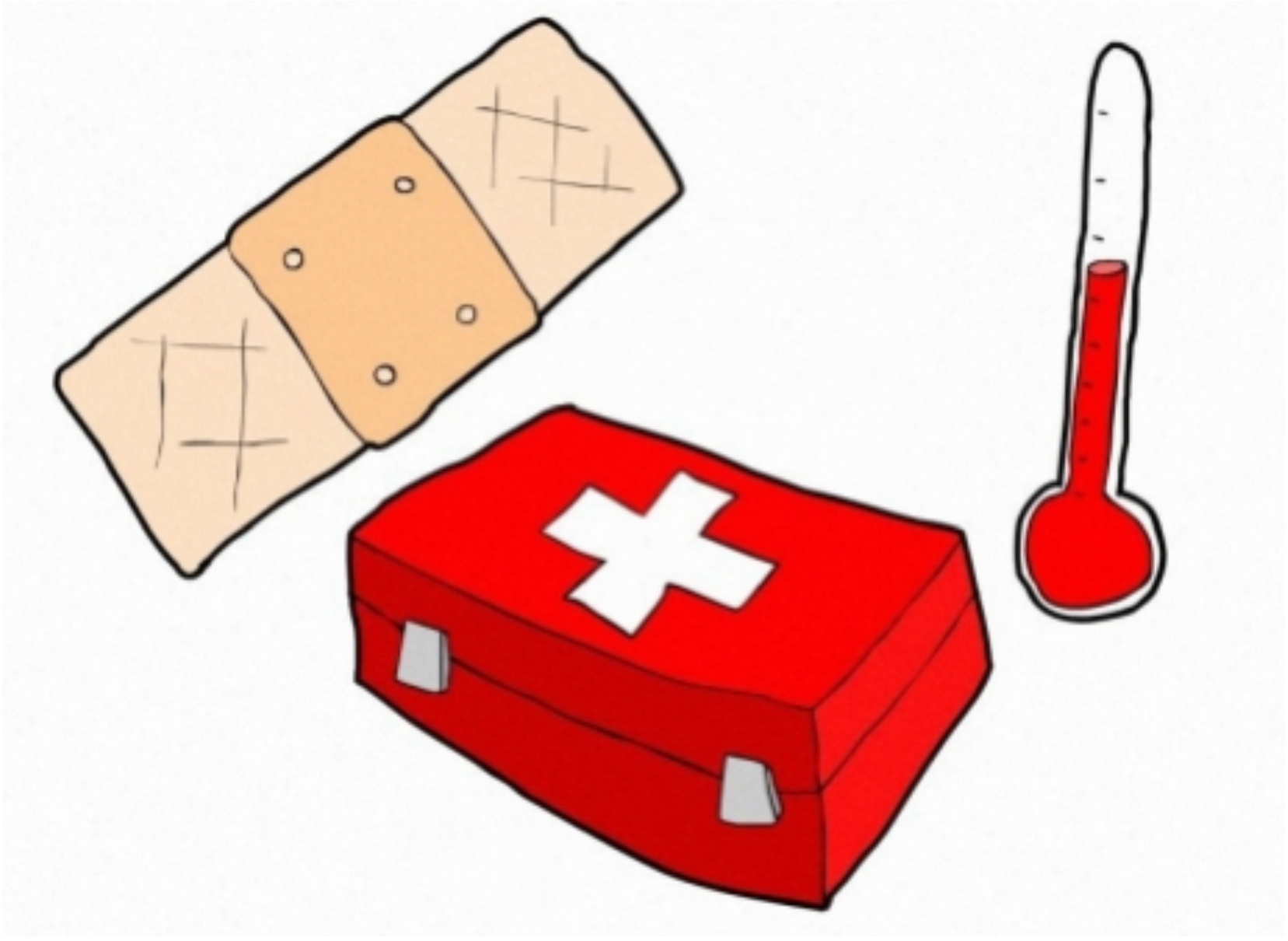

Thank you!

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