

# **The Role of HTA in Health care and the National Institute for Health and Clinical Excellence**

Panos Kanavos, PhD

London School of Economics

Zürich, 5 November 2010



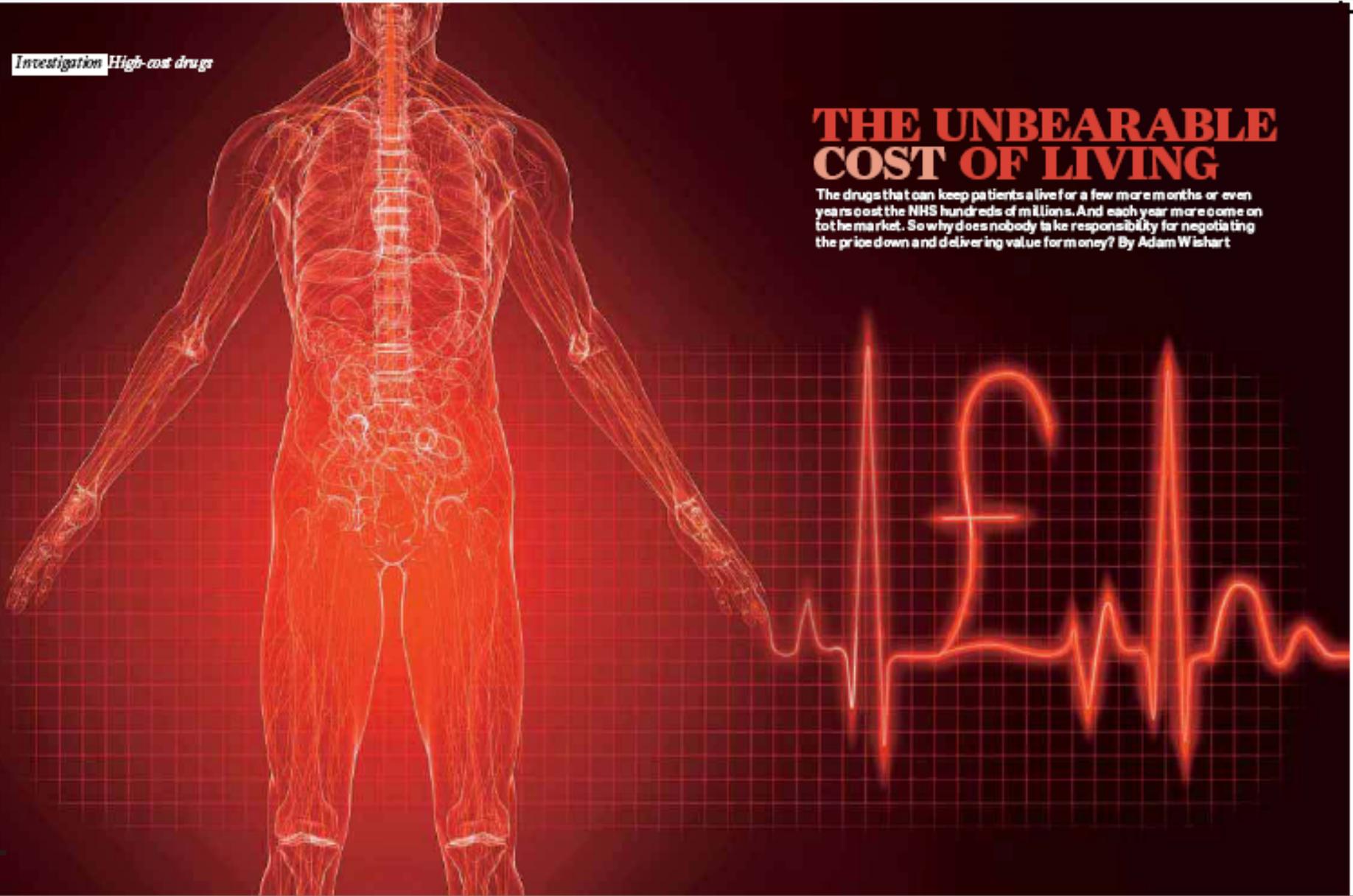
# Outline

- (i) Role of NICE: HTA or more than that?
- (ii) Nature of evidence and its interpretation
- (iii) Identifying and filling the gaps in evidence
- (iv) Future developments

Investigation *High-cost drugs*

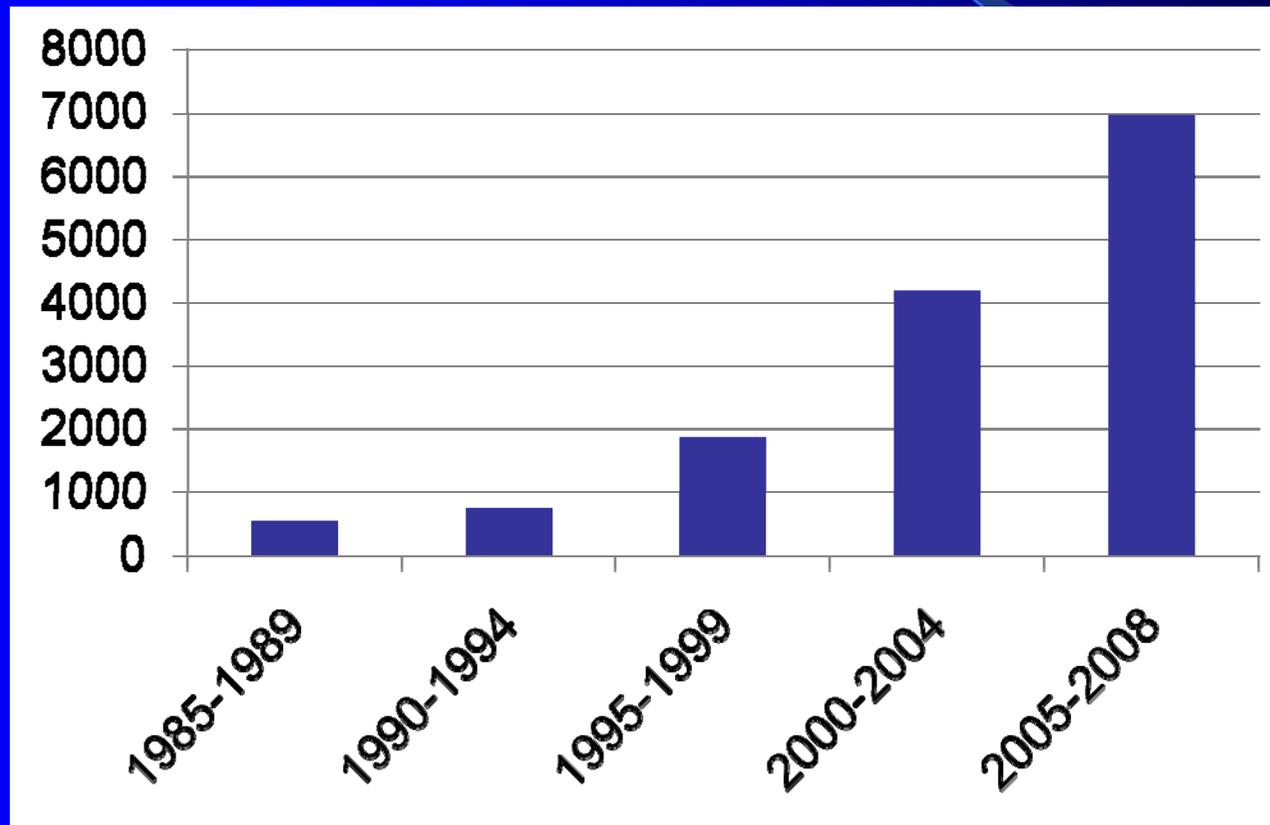
## THE UNBEARABLE COST OF LIVING

The drugs that can keep patients alive for a few more months or even years cost the NHS hundreds of millions. And each year more come on to the market. So why does nobody take responsibility for negotiating the price down and delivering value for money? By Adam Wishart



# Median Monthly Costs of new anti-cancer drugs at launch, 1985 - 2008

Monthly treatment costs US\$  
at 2007 prices



Bach et al 2009

Year



# NICE....what is it ?

The National Institute for Health and Clinical Excellence (NICE) is the independent organisation responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health. It was established in 1999 as a Special Authority and in 2005 it was expanded to include the functions of Health Development Agency



# Justification for the establishment of NICE

- (i) Variation in care
- (ii) Speed of innovation and surge in new information
- (iii) Need to assess “value for money”

# NICE's Programmes

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## 1. Clinical:

- Technology appraisals
- Clinical guidelines
- Interventional procedures

## 2. Public health:

- Interventions and programmes

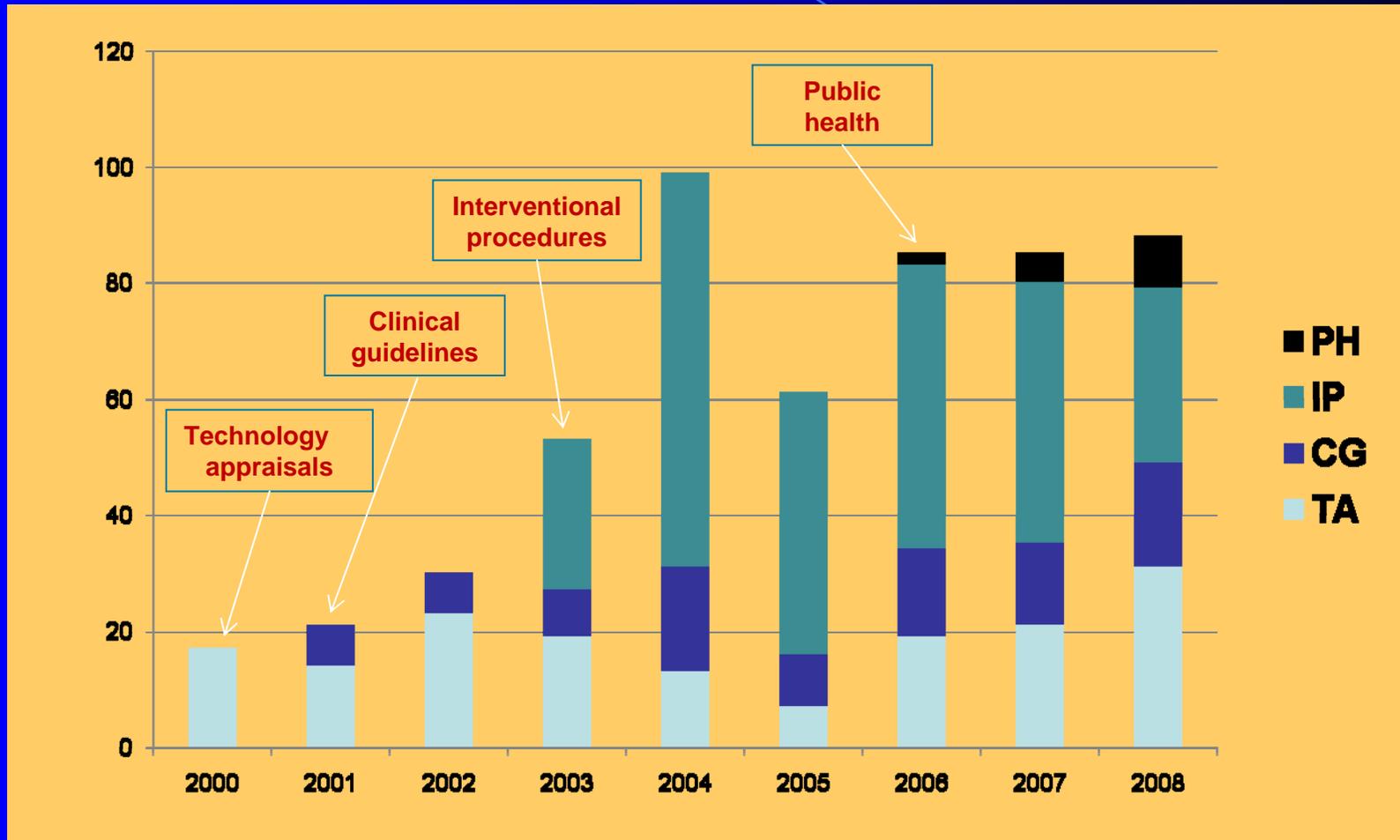
## 3. NICE quality standards

## 4. NHS evidence

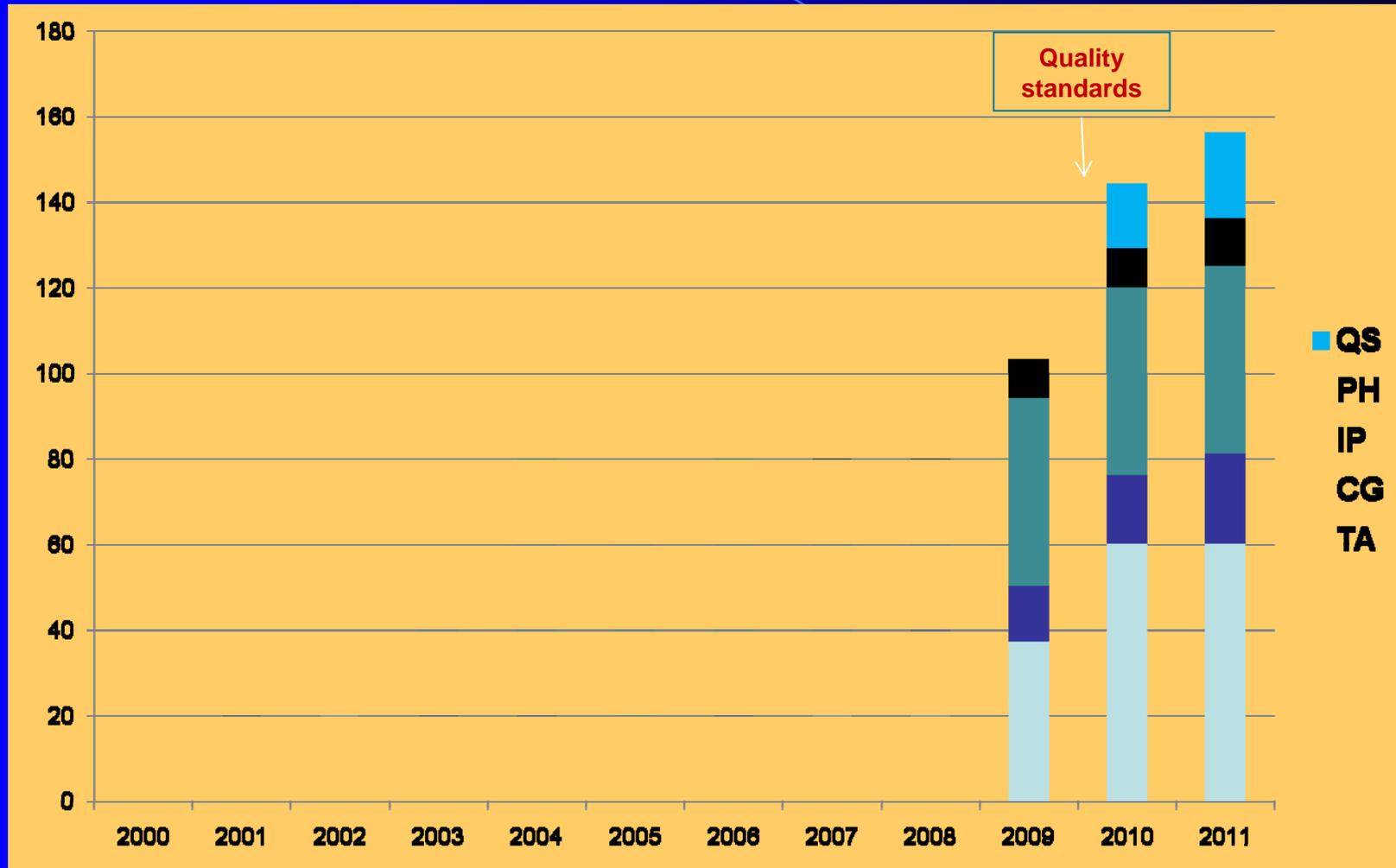
# The Institute produces guidance in three areas

- **Public health** – guidance on the promotion of good health and the prevention of ill health for those working in the NHS, local authorities and the wider public and voluntary sector
- **Health technologies** – guidance on the use of new and existing medicines, treatments and procedures within the NHS including interventional procedures
- **Clinical practice** – guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS.

# NICE guidance 2000-2008



# NICE guidance 2009-2011



# Health Technology Assessment

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Encompasses all approaches to:

- Prevention
- Screening
- Diagnosis
- Treatment

# Technology appraisals *all decisions*

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<b>Full use</b>	<b>247 (67%)</b>
<b>Restricted use</b>	<b>59 (16%)</b>
<b>Only in research</b>	<b>22 (6%)</b>
<b>No use</b>	<b>39 (11%)</b>
<b>TOTAL</b>	<b>367 (100%)</b>

# Principles of NICE guidance

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- 1. Robust**
- 2. Inclusive**
- 3. Transparent**
- 4. Independent**

# Clinical Evaluation

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1. Randomised controlled trials
2. Observational studies
3. Systematic reviews

**Avoid “hierarchies” of evidence**

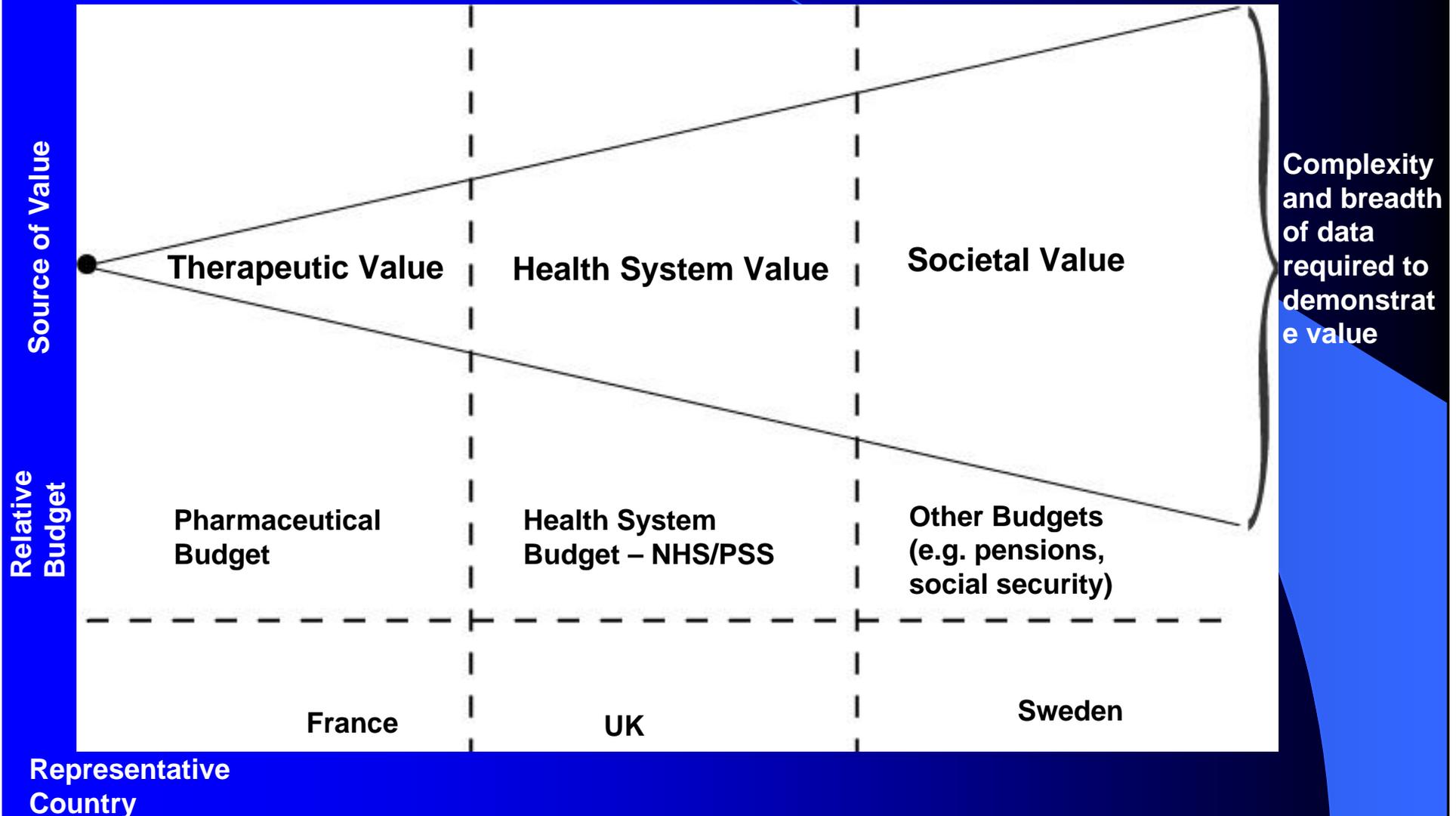
# Economic Evaluation

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## Overarching principles:

1. Economic perspective
  - NHS and PSS
2. Cost effectiveness
  - Not affordability or budgetary impact
3. Balance between:
  - Efficiency (utilitarianism)
  - Fairness (egalitarianism)

# Spectrum of Value (Payers) – Broad Sources and Perspective of HTAs



# Cost Utility Analysis

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## Costs (and savings):

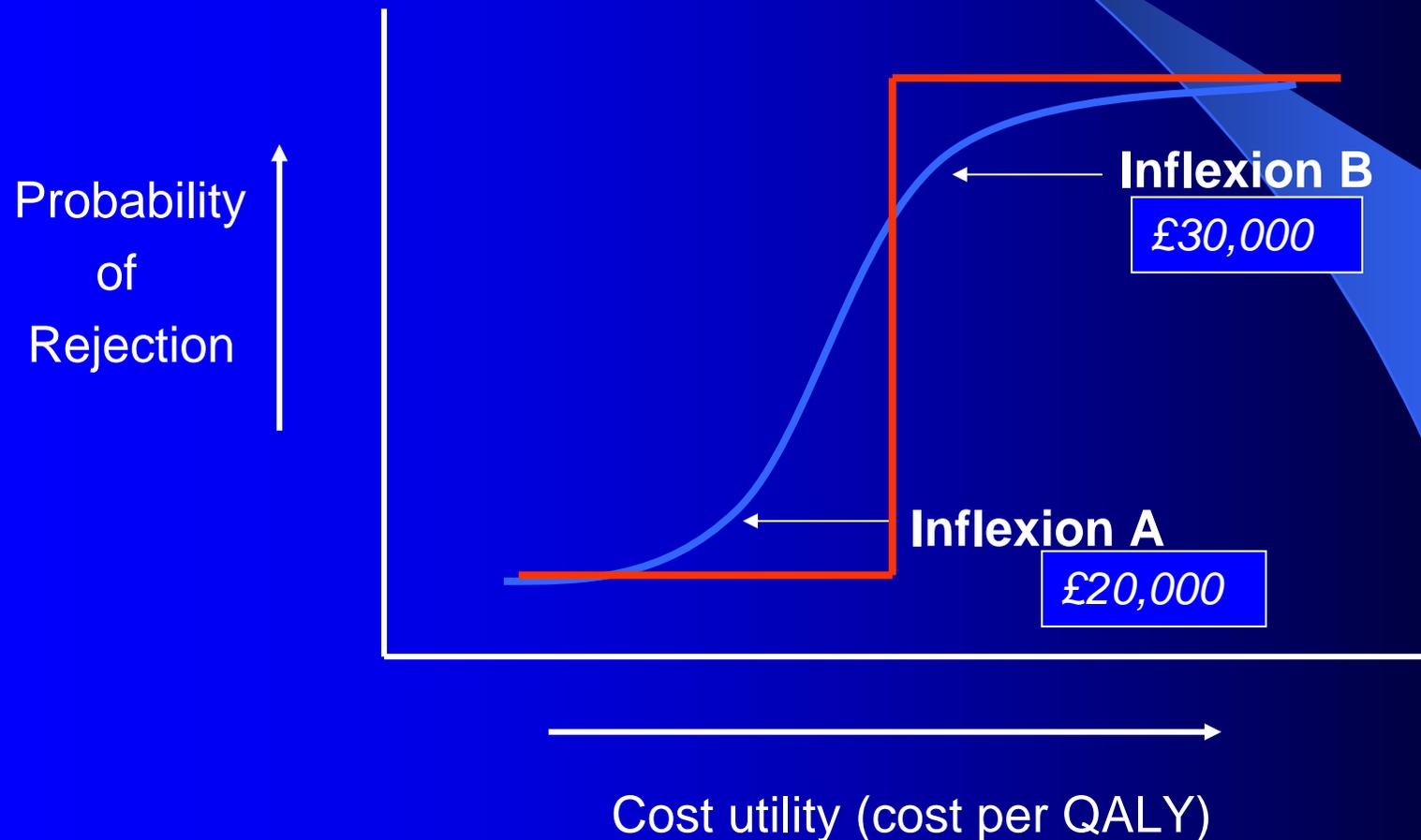
- direct
- indirect

## Benefits:

- improvement (change) in HRQoL (utility)
- time for which it is “enjoyed”

**Incremental cost effectiveness ratio**

# The Cost Effectiveness Threshold and how NICE works it out



Source: Cookson, 2007

# NICE Appraisal Consultation Document. Prevention and treatment of osteoporosis in postmenopausal women.

- Bisphosphonates are recommended as treatment options for postmenopausal women younger than 65 years of age *with a fragility fracture* if they have either of the following:
  - T-score below  $-3.2SD$  established by a DEXA scan;
  - T-score below  $-2.5SD$  and either a history of maternal hip fracture or long-term use of systematic corticosteroids.
- Bisphosphonates are *not* recommended for the treatment of osteoporosis in *postmenopausal women of any age who do not have a fragility fracture*.

# Media View on Osteoporosis Treatments

Daily Mail, Monday, January 12, 2004

## If you want NHS drugs, you must break a bone first

By **Jenny Hope**  
Medical Correspondent

**WOMEN** with osteoporosis will have to break a bone to qualify for drug treatment under proposed Health Service rules. Even then, some fracture victims may be refused.

Millions of women would lose the chance to take drugs to halt bone-thinning while some already being treated could have their medication stopped.

The proposals from the National Institute for Clinical Excellence are aimed at cutting spending on treatments which are not 'cost-effective'.

But critics warn that the move could lead to a massive increase in bone fractures which would actually cost more than continuing to prescribe the drugs.

Specialists say the changes will set the battle against the disease back by many years, just as new treatments are making an impact.

The NICE proposals - which could take effect as early as June - are another blow for postmenopausal women who were told two months ago that they can no longer use Hormone Replacement Therapy to prevent osteoporosis because of possible cancer risks.

The National Osteoporosis Society has launched a campaign



### How this treatment transformed my life

**AUDREY Capy** says the new drug Forsteo 'has given me my life back'.

But 68-year-old Mrs Capy could be stopped from taking it within months - because she is deemed too young.

Although she meets one of the proposed criteria - she failed to respond to other treatments - she would have to wait until she is 70.

'It's completely ludicrous,' she said yesterday. 'It's judging by age, not clinical condition. This drug should be available to stop problems from getting worse. Women shouldn't have to wait until it is too late.'

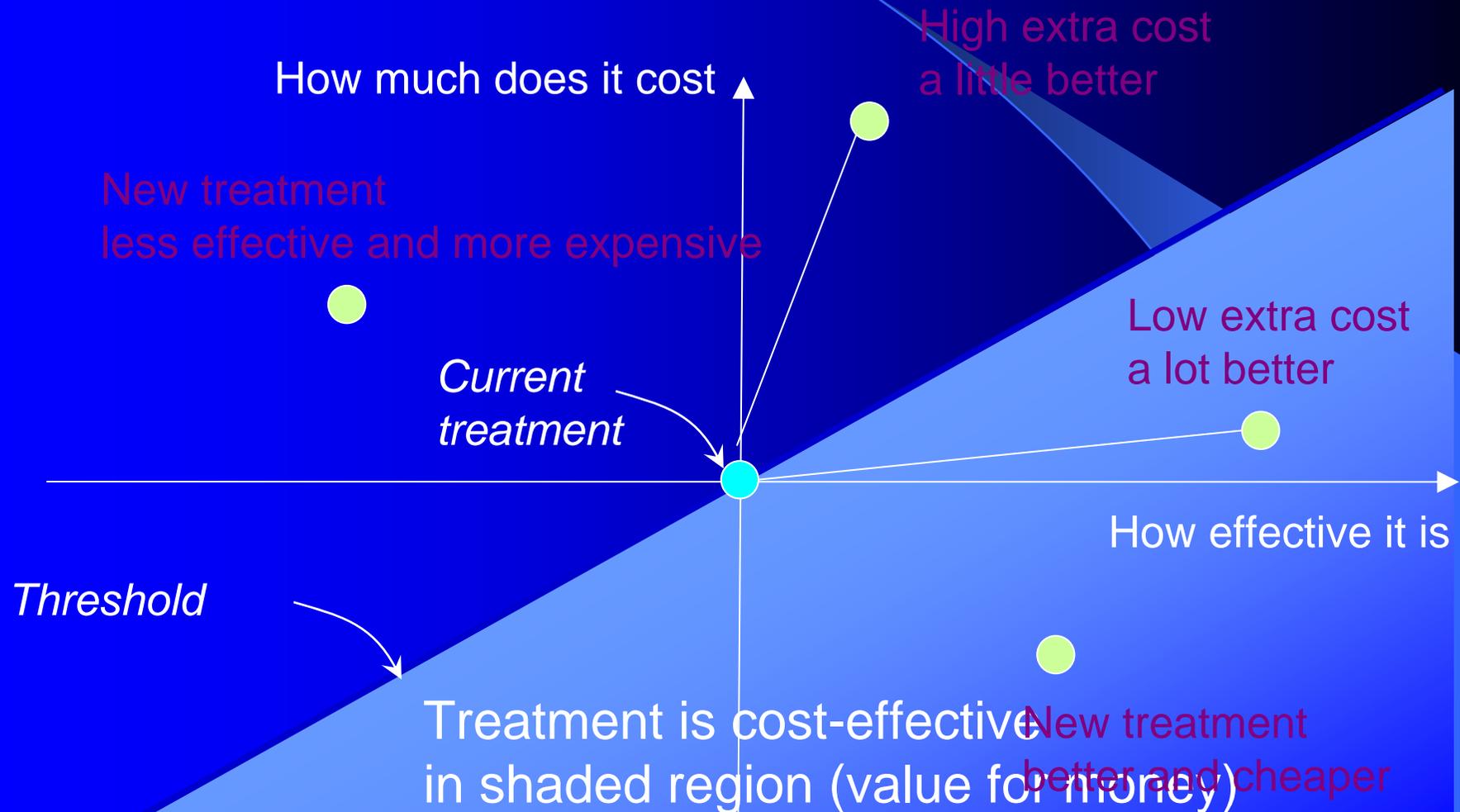
The former teacher's agony began when she suffered two fractures in the upper part of her spine during an adventure ride in Disney World, Florida, two years ago.

At first, no one could explain the problem, which left her in constant pain and forced to spend much of her time lying down. An MRI scan of her lower spine showed nothing.

Mrs Capy, a mother of three from Wheathampstead, Hertfordshire, said: 'I had to take strong painkillers to get out of bed in the morning. Then all I could do was lie on the carpet in the living room to minimise the pain.

'If I went anywhere by car I had to lie on the floor. My family began to wonder what was going on because nobody could find anything wrong.'

# Assessing “value for money”



# Decision-making

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## 1. Scientific judgements

- Reliability of the evidence-base
- Appropriateness of sub-groups
- Generalisability
- Capture of quality of life
- Handling uncertainty

## 2. Social value judgements

- Severity of disease
- End of life interventions (“rule of rescue”)
- Age
- Health inequalities

# Evaluating the evidence

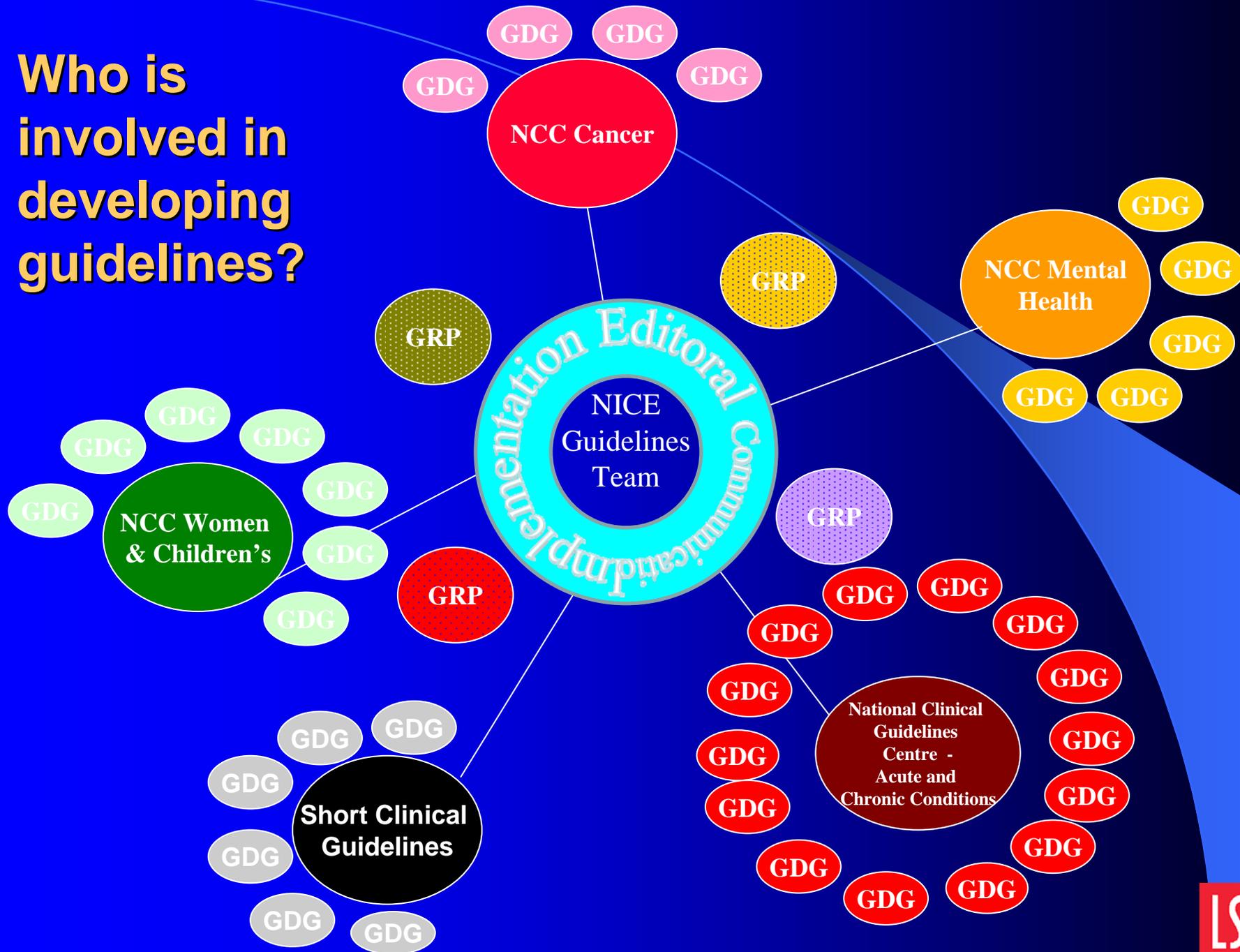


Judgements to be made

# Recommendations >£30,000 per QALY

Topic	QALY ('000)	Severity	End of life	Significant innovation	Disadvantaged population	Children	Corporate responsibility
Riluzole	38-42	★					
Temozolomide (glioma)	35	★	★				
Trastusumab (breast cancer)	37.5	★	★	★			
Imatinib (CML)	36-65	★	★	★			
Bortezomib (myeloma)	32.5	★	★	★			
Pemetrexed (mesothelioma)	34.5	★	★	★	★		
Sunitinib (renal cancer)	55	★	★	★			★
Human growth hormone	Uncertain					★	
Insulin pumps	Uncertain					★	
Lenalidomide (myeloma)		★	★				

# Who is involved in developing guidelines?



# Versions of the guideline

- The full guideline (owned by the National Collaborating Centre [NCC])
- The NICE version
- The quick reference guide (QRG)
- Understanding NICE guidance (UNG)



## Resources

[NCBI Home](#)[All Resources \(A-Z\)](#)[Literature](#)[DNA & RNA](#)[Proteins](#)[Sequence Analysis](#)[Genes & Expression](#)[Genomes](#)[Maps & Markers](#)[Domains & Structures](#)[Genetics & Medicine](#)[Taxonomy](#)[Data & Software](#)[Training & Tutorials](#)[Homology](#)[Small Molecules](#)[Variation](#)

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## Genome Reference C

Formed to improve human and m... assemblies, GRC will fix loci misre... reference assembly, fill remaining... alternate representations of comp...

1 2 3 4

## How To...

- Obtain the full text of an art...
- Retrieve all sequences for...
- Find a homolog for a gene...
- Find genes associated wit...
- Design PCR primers and...
- Find the function of a gene...
- Determine conserved synt...

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## NLM/NCBI H1N1 Flu Reso

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## Guidelines

National Institute for Health and Clinical Excellence



National Institute for  
Health and Clinical Excellence

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NICE clinical guidelines are recommendations on the appropriate treatment and care of people with specific diseases and conditions within the NHS in England and Wales. They are based on the best available evidence and aim to improve the quality of healthcare by changing the process of healthcare and improving people's chances of getting as well as possible. NICE clinical guidelines:

- provide recommendations for the treatment and care of people by health professionals
- are used to develop standards to assess the clinical practice of individual health professionals
- are used in the education and training of health professionals
- help patients to make informed decisions
- improve communication between patient and health professional

NICE clinical guidelines help healthcare professionals in their work, but they do not replace their knowledge and skills.

[CG80Early and locally advanced breast cancer: diagnosis and treatment](#)

National Collaborating Centre for Cancer

[CG82Schizophrenia: core interventions in the treatment and management of schizophrenia in primary and secondary care](#)

National Collaborating Centre for Mental Health

[CG83Rehabilitation after critical illness](#)

Centre for Clinical Practice

[CG86Coeliac disease: recognition and assessment of coeliac disease](#)

Centre for Clinical Practice

[CG88Low back pain: early management of persistent non-specific low back pain](#)

National Collaborating Centre for Primary Care; Royal College of General Practitioners

# Key topics in published and planned public health guidance

- Physical activity
- Smoking and tobacco
- Sexual health
- Alcohol
- Drugs
- Maternal and child health
- Health and work
- Older people's health and wellbeing
- Cancer
- Immunisation
- Accidental injury.
- Obesity
- Mental well being
- Cardio vascular disease.
- Diabetes
- Communicable disease

# Darzi report: High quality care for all

The report stated that:

*‘NICE will manage the synthesis and spread of knowledge through NHS Evidence – a new single portal through which anyone will be able to access clinical and non-clinical evidence and best practice, both what high quality care looks like and how to deliver it. Greater clarity on standards, and where to find them, will support the commissioning and uptake of the most clinically and cost-effective diagnostics, treatments and procedures’.*

# Categories of information in NHS Evidence

## Clinical

- Guidelines
- Systematic Reviews
- Other syntheses content (summaries & overviews)
- Primary research and ongoing trials

## Commissioning & Improvement

- Service guidance
- Tools & models
- Care pathways
- Indicators & metrics
- Improvement information

## Social care

- Social care information – *assured by SCIE*

## E-Learning & Education

- On-line learning modules

## Drug & Technologies

- Prescribing & safety information
- Technology appraisals
- Significant new drugs
- Devices, diagnostics & IP guidance

## Public Health

- Public Health guidance
- *Systematic reviews*
- *Primary research*

# NICE approach has 4 steps to assessing evidence

Step 1: Define the clinical or public health question

Step 2: Identify the evidence

Step 3: Synthesise and assess the body of evidence

Step 4: Issue the recommendations

# Voice of the Public – the citizens council

The screenshot displays the NICE website's 'Reports' page for the Citizens Council. The navigation menu includes Home, Our guidance, Using guidance, Get involved, News & Events, and About NICE. The main content area is titled 'Reports' and explains that the Council submits reports to NICE for use in developing guidelines and appraisals. It lists several reports with their dates:

- Citizen's Council report: Rule of rescue** (16/06/2006)
- Citizens Council report: Mandatory public health measures** (05/10/2005)
- Open University Evaluation report** (23/03/2005)
- Citizens Council report: Ultra Orphan drugs** (14/02/2005)
- Citizens Council report: Confidential enquiries** (27/07/2004)
- National Children's Bureau report** (05/07/2004)
- National Children's Bureau report, Appendix 1** (05/07/2004)
- National Children's Bureau report, Appendix 2** (05/07/2004)

On the right side, there are three boxes: 'Search NICE guidance' with a search bar and 'Advanced guidance search' link; 'Order NICE guidance' with a link to 'Order printed copies of our guidance'; and a small image of a person reading.



# Stakeholder involvement: Patients

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## 1. Clinical effectiveness

- What the condition is *really* like?
- What are the expectations of treatment?
- Has the QoL measures captured all the relevant dimensions of the condition and its treatment?

## 2. Cost effectiveness

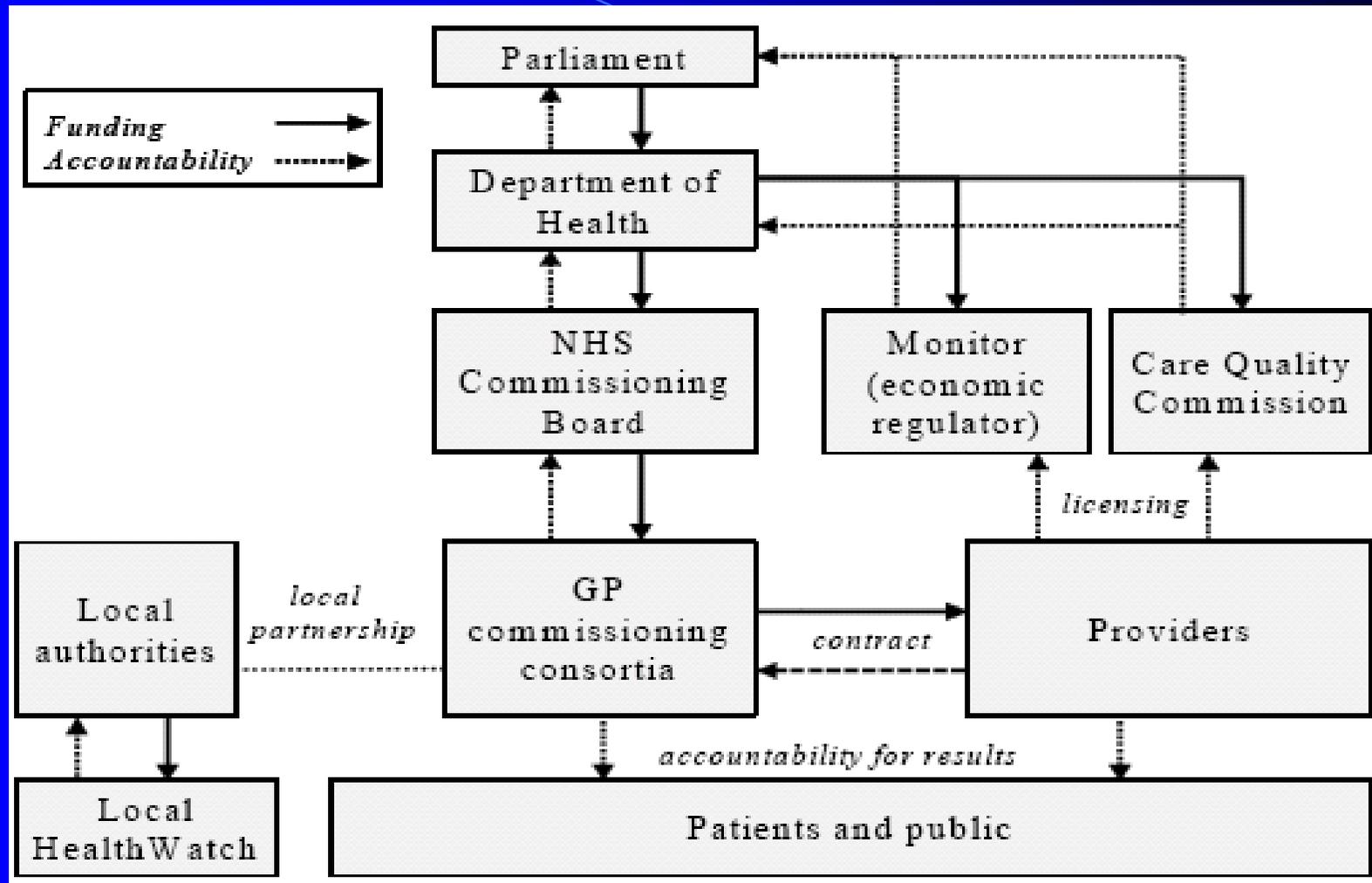
- Are there special considerations?
- If so, have these been taken into account?

# The future

# 1. 2010 Reforms – White Paper & Consultation

- Overall reforms to NHS in England set out in the July 2010 White Paper *“Equity and Excellence: Liberating the NHS”*
- Reforms to NHS commissioning in England are set out in the consultation document *“Commissioning for Patients”*
- Commissioning reforms sit alongside numerous other changes, including:
  - Care Quality Commission to monitor NHS services against a new “outcomes framework”, including “quality standards” from NICE
  - Economic regulation (i.e. of prices, competition, finances) by Monitor
  - Patient involvement via local and national “Health Watch”
  - Local Authorities become responsible for public health
- Following diagram summarises the new structure

# 1. 2010 Reforms – The new structure



Source: WP 2010, Figure 2

# 1. NHS 'commissioning' is:

- “The process of assessing the needs of a local population and putting in place services to meet those needs.” (WP 2010, Glossary)

## IN THE CONTEXT OF:

- **Patient choice** over most non-emergency care: “We expect choice of treatment and provider to become the reality for patients in the vast majority of NHS funded services by no later than 2013/14.” (WP 2010, para.2.23)
- And an “**any willing provider**” approach, in principle (CfP 2010, para.5.11)

# 1. Elements of commissioning

1. Assessing population health needs
2. Determining/designing services to meet needs, including clinical service specification
3. Contracting with service providers
4. Monitoring services provided

# 1. NICE's inputs to commissioning

- “Quality standards, developed by NICE,” [150 over 5 years] “will inform the commissioning of all NHS care and payment systems.” Inspection by the CQC will be “against essential quality standards”. (WP 2010, para.5j)
- “NICE will rapidly expand its existing work programme to create a comprehensive library of standards for all the main pathways of care” (WP 2010, para.3.12) and will expand to cover social care too.
- NB “The Health Bill will put NICE on a firmer statutory footing, securing its independence and core functions and extending its remit to social care.” (WP 2010, para.3.14)

# Recent developments and likely interpretations

- GP Commissioning Groups
- OFT report on PPRS (2007)
- Re-negotiation of PPRS and new agreement (Jan. 2009 – Dec. 2013)
- PPRS abolished and shift to VBP
- Societal perspective in HTA, NICE loses mandate
- HTA bodies produce guidance based on societal perspective
- GP Commissioning Groups to make decisions based on issued guidance (but applying it as they see fit)
- Post code lottery returns?