Cost-effectiveness thresholds and pharmaceutical policy

Dr Darren Ashcroft
Director, Centre for Innovation in Practice
School of Pharmacy and Pharmaceutical Sciences
University of Manchester, UK

darren.ashcroft@manchester.ac.uk

Outline

- What is the cost-effectiveness threshold?
- What should the threshold represent (in principle)?
- How can the threshold be estimated (in practice)?
- Policy context and methods of NICE
- Should the threshold change?

System objectives

- Key underlying concepts in health economics relate to issues of scarcity and choice
- If resources were not scarce, choices would not have to be made
- In clinical practice, choices about allocation of resources have to be made, and are being made routinely.

Supply and Demand

Supply	=	Demand
Providers supply		*Purchasers demand*

In ideal setting, health care services which meet the 'needs' of their local population of residents.

In reality: Supply < Demand

Efficiency

- How do we allocate scarce resources so that benefit is maximised?

- *Allocative efficiency*: using resources, across the whole economy, so that benefit is maximised (or the opportunity cost is minimised).

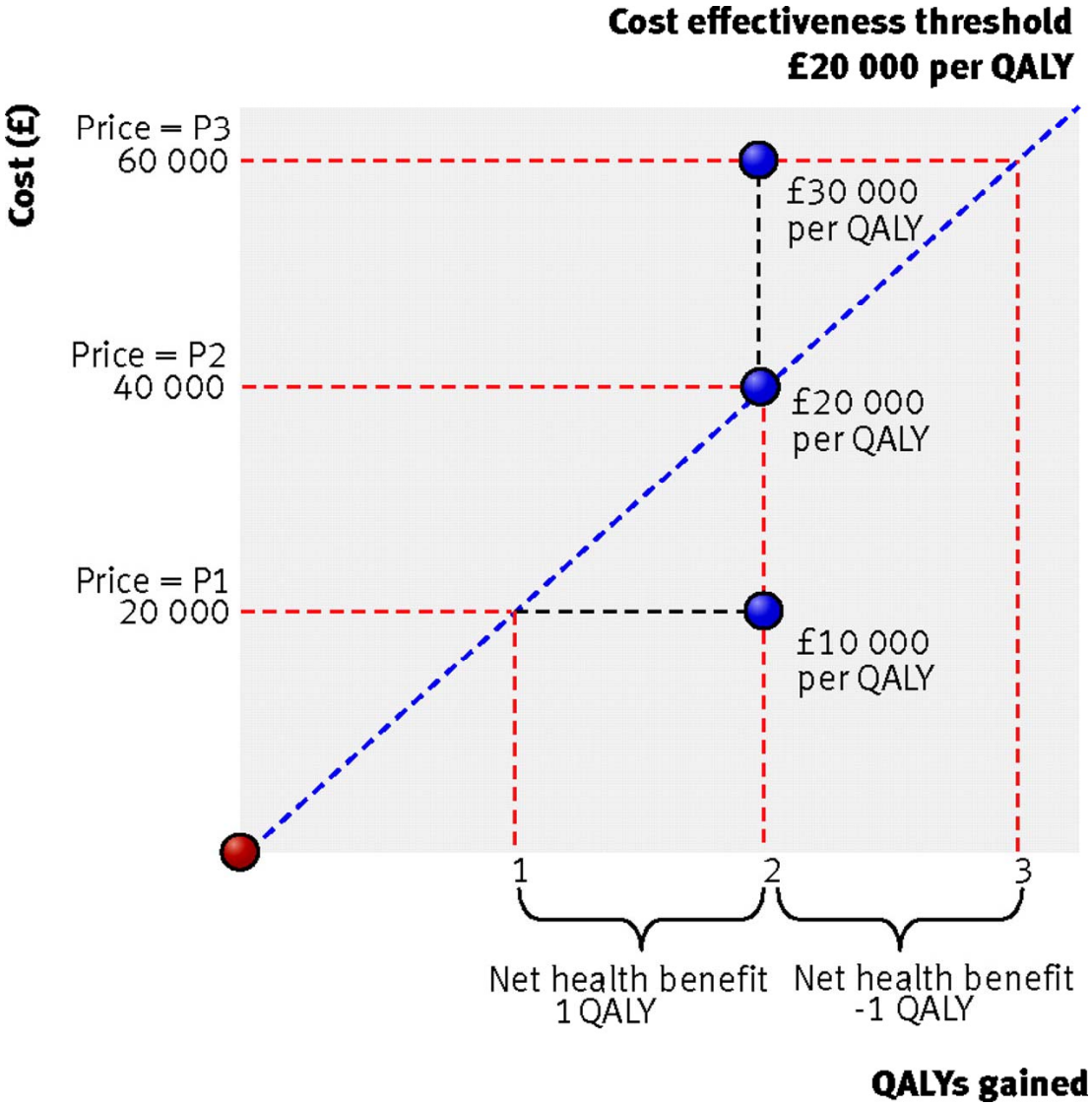
- *Technical efficiency*: means producing a given output for the least cost, or maximising output for a given cost.

System objectives

- Maximise some form of health benefit (e.g. QALY) subject to a financial budget constraint

- No budget constraint – budget always expand to fund treatments which offer health benefits. Does such a system exist?

Cost-effectiveness threshold



What should the threshold represent (in principle)?

- In principle, it is the ICER of the least cost-effective intervention currently being funded
- Such programmes should be forced out by more cost-effective options
- But the threshold will vary:
  - by budget impact of the health technology
  - across localities
- What should be displaced is not what is displaced
  - Major information challenge of finding least cost-effective
  - Often a range of constraints to disinvestment: political and economic

Empirical estimates of the threshold

- Past funding decisions
- Social willingness to pay
- Value of health used elsewhere in the public sector
- Data from the health service on cost-effectiveness of existing services

A societal willingness to pay

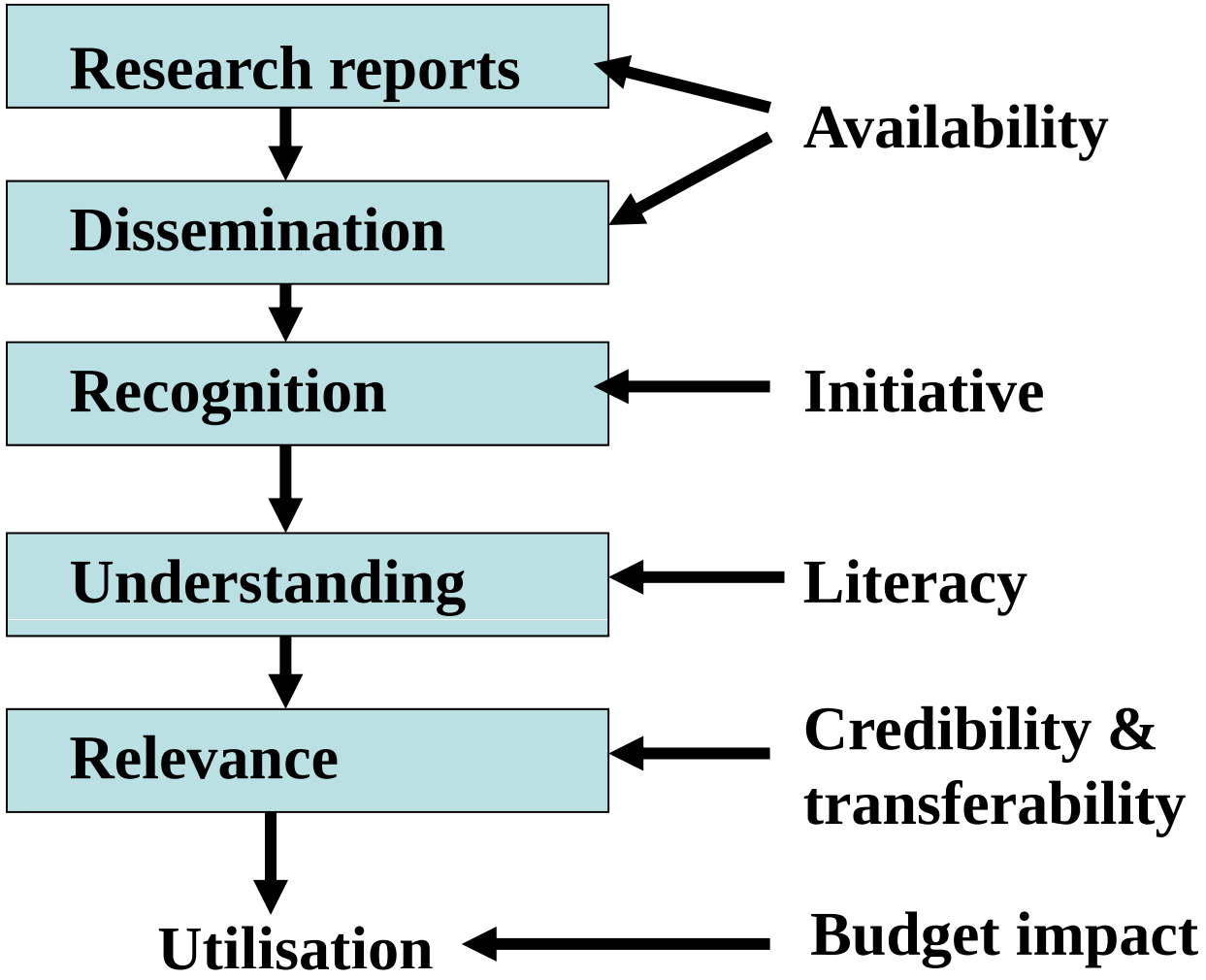
- A number of empirical studies on ‘social valuation’ of health against consumption
- Some studies estimating social value of the QALY
- Possible to value of health from other sectors?
- The value of a statistical life is used in the UK to inform transport investment decisions
- Also considered by other sectors (e.g. environment)
- Tend to imply a higher valuation of health than NICE

Using data on existing services

- Local decision makers mainly consider technical efficiency rather than cost-effectiveness
- Budget impact (irrespective of cost-effectiveness) used
- Some decisions involve trade-off between added costs and benefits, but quite rare

Appleby J, et al. Health Policy 2009; 91: 239–245

Barriers to use of economic evidence



Chen LC, et al. Pharmacy World & Science 2007; 29: 661-670

Estimating the threshold from aggregate data

- Cost per life year saved
- Based on NHS programme budgets and mortality rates
- Econometric modelling to control for medical need
- Modelling variation between local health commissioning organisations

- £ 15,387 for cancer (£19,070 per QALY)
- £ 9,974 for circulation problems (£11,690 per QALY)
- £ 5,425 for respiratory problems
- £ 21,538 for gastro-intestinal problems
- £ 26,428 for diabetes

Martin S, et al. Journal of Health Economics 2008; 27: 826–842

National Institute for Health and Clinical Excellence (NICE)

- Established 1999 following election of Labour government
- Prolonged controversy about ‘post-code prescribing’ in the UK National Health Service
- Wish to ‘de-politicize’ decisions about which new health technologies to cover in NHS
- Desire to use best available methods to address difficult questions
- Major focus on medicines but devices also included
- January 2002: NHS legally obliged to fund treatments recommended by NICE

The NICE process

- Specific technologies
- Lacking in transparency
- Subject to some criteria

- Independent group
- Review plus model
- Good methods supported
- Assess company submissions
- 6 months or more
- Companies can also provide unpublished data

- Multi-disciplinary committees
- Take information from range of sources
- Range of decisions possible

NICE and methods

- Methods guidance more prescriptive than most international guidelines
- Based on a clear view about the use of economic evaluation for decision making
- A need for a consistent approach to analysis to inform decision-making: the Reference Case

National Institute for Health and Clinical Excellence (NICE). *Guide to the Methods of Technology Appraisal*. London: NICE, 2008.

The requirements of economic evaluation for NICE-decision making

Objective function	→	Generic measures of health; QALYs
Decision problem	→	Clarity about population; full specification of options
Appropriate time horizon	→	Time over which options might differ
Evidence base	→	Inclusion of all relevant evidence
Context	→	Relevant to specific decision maker(s)
Uncertainty	→	Quantify decision uncertainty; feed in research prioritisation

What is the appropriate framework for economic evaluation?

Evidence synthesis →

- Systematic review
- Meta-analysis
- Mixed treatment comparisons
- Differing endpoints and follow-up
- Patient-level and summary data

Decision analysis →

- Structure reflecting disease
- Incorporation of evidence on range of parameters
- Facilitates extrapolation and separation of baseline and treatment effects
- Probabilistic methods

Limitations of trials as a vehicle for decision making

Trial limitations	NICE Examples
Inappropriate or partial comparisons	Temozolomide (recurrent malignant glioma)
More than one trial	Drugs for Alzheimer's
Partial measurement	Riluzole (resource use)
Unrepresentative practice	Glycoproteins
Intermediate outcomes	Beta interferon (MS)
Limited follow-up	Implantable cardioverter defibrillators
No trials	Liquid-based cytology

Making trials more ‘naturalistic’

The design continuum

What NICE says about the threshold value?

Below a most plausible ICER of £20,000 per QALY gained, the decision to recommend the use of a technology is normally based on the cost-effectiveness estimate

. …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
- the change in HRQL has been inadequately captured
- the innovative nature of the technology

National Institute for Health and Clinical Excellence (NICE). *Guide to the Methods of Technology Appraisal*. London: NICE, 2008.

Should the threshold be changed?

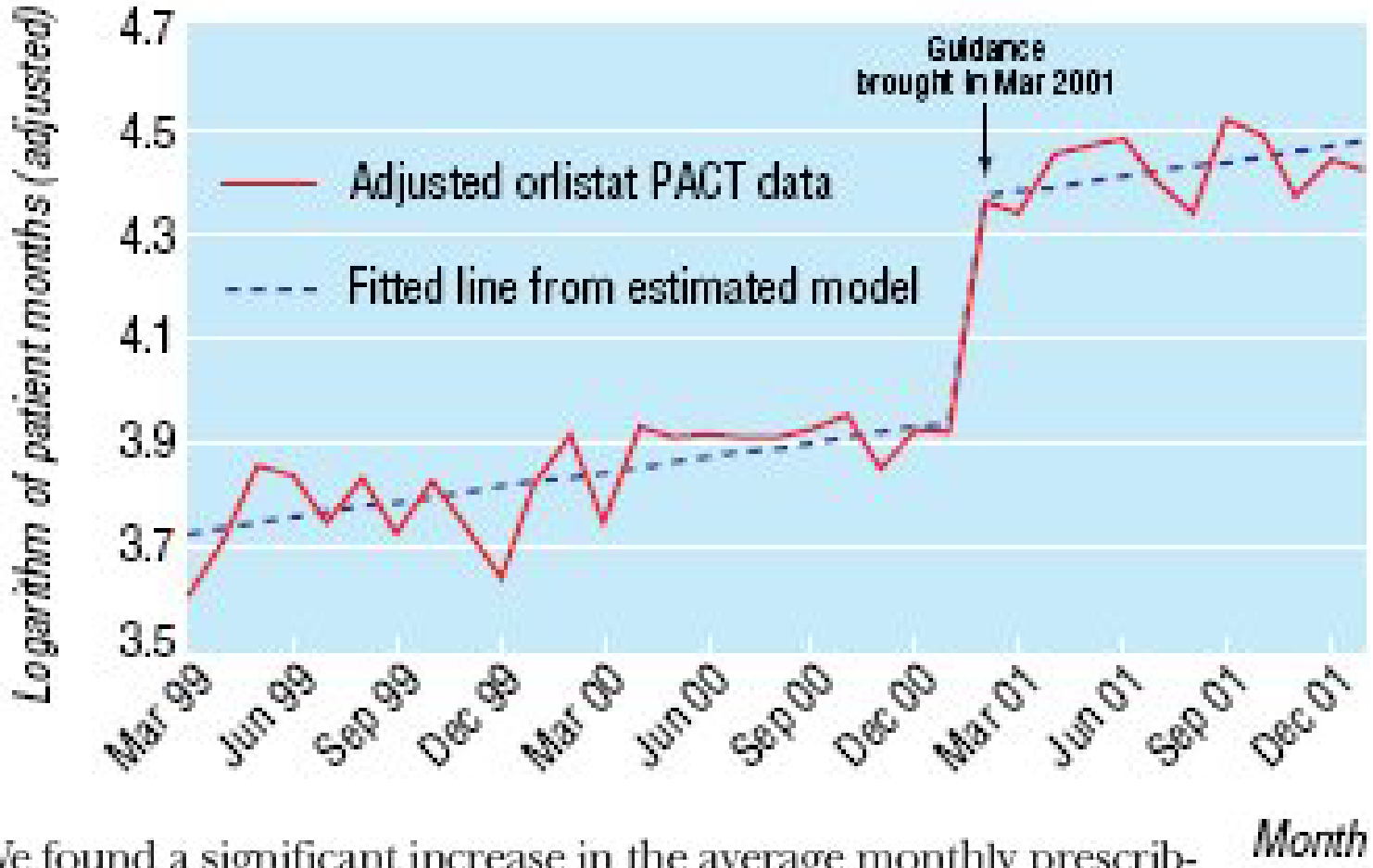
- Pressure to increase because of increased NHS budget and inflation
- Considerable uncertainty regarding whether existing threshold correct
- But change will depend on budget, uses of budget and NHS productivity
  - Much additional budget spent on things not easily displaced (e.g. doctors salaries)
  - NICE can drive threshold down (if services displace less cost-effective treatments)
  - Prices of potentially displaced services may not increase (e.g. use of generic drugs)

NICE decisions overall

Recommendation	Number	%
Yes	27	23%
Yes, with major restrictions	38	32%
Yes, with minor restrictions	30	26%
No	22	19%
Total	117	100%

Raftery J. *BMJ* 2006; 332: 1266-1268.

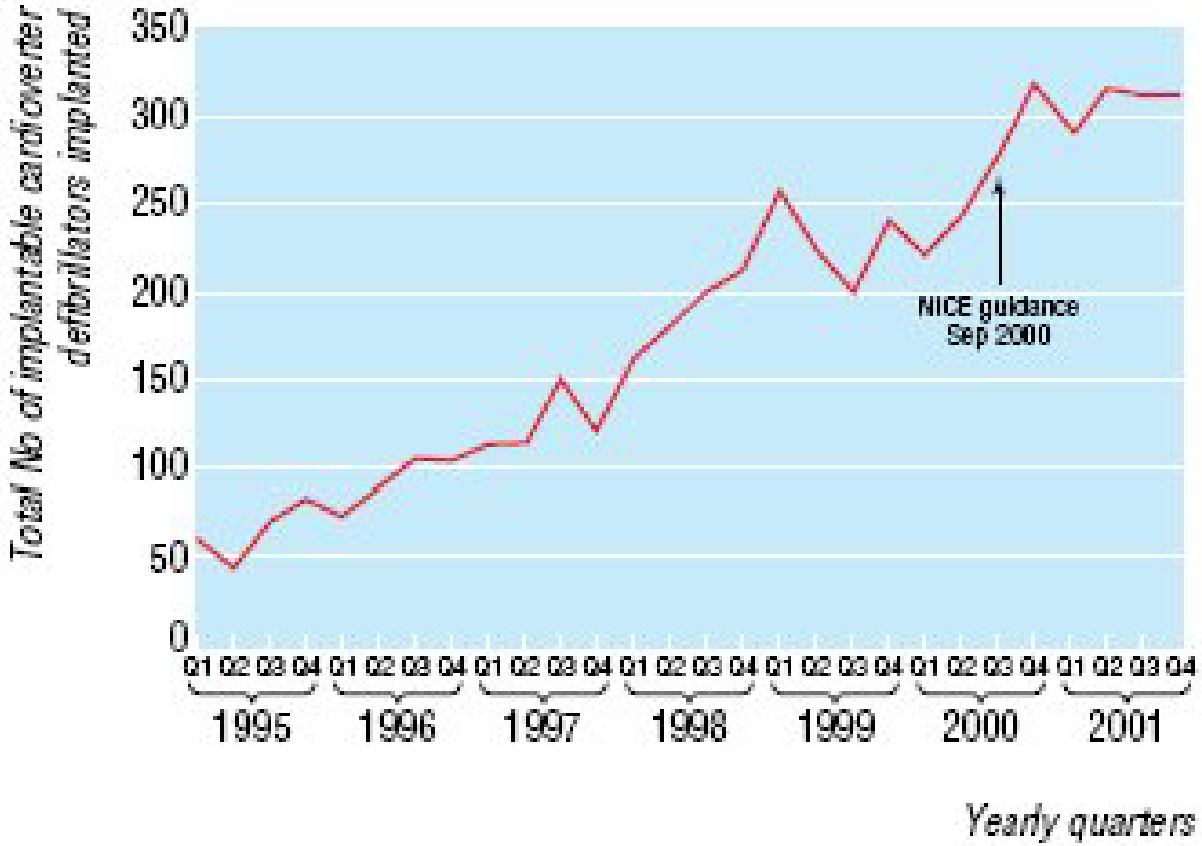
Evidence on Orlistat for obesity



We found a significant increase in the average monthly prescribing of orlistat after the guidance had been published 22 per month (0.43, 95% confidence interval 15.9 to 27.8, $P < 0.001$; fig

Sheldon et al. *BMJ* 2004;329:999.

Evidence on implantable defibrillators for arrhythmias



implanted has risen, we found no evidence of a significant change after NICE guidance had been published (fig 3). Given

Sheldon et al. *BMJ* 2004;329:999.

What influences uptake?

- Commitment to managing process of implementing guidance
- Proactive assessment of local costs and implications of implementation
- Responsibility for funding and implementation vested in locality-wide group
- Recognition of legitimacy of NICE
- Financial stability
- Expectation that compliance is mandatory, subject to identification of funding
- Targeted audit of areas of non-compliance

Sheldon et al. *BMJ* 2004;329:999.

Conclusions

- Any system with a budget constraint needs to define a threshold
- NICE part of an international trend towards greater use of economics in decision making
- Reasonably clear about principles of what the threshold is and should represent
- Much more uncertainty about its empirical value
- Need more research to estimate its value and how it will change over time