Health Technology Assessment: Priorities for Methods Development

Mark Sculpher, PhD

Centre for Health Economics
University of York, UK

Spanish Association for Health Technology Assessment (AEETS): Rationing and Prioritisation: Two strategies claiming HTA

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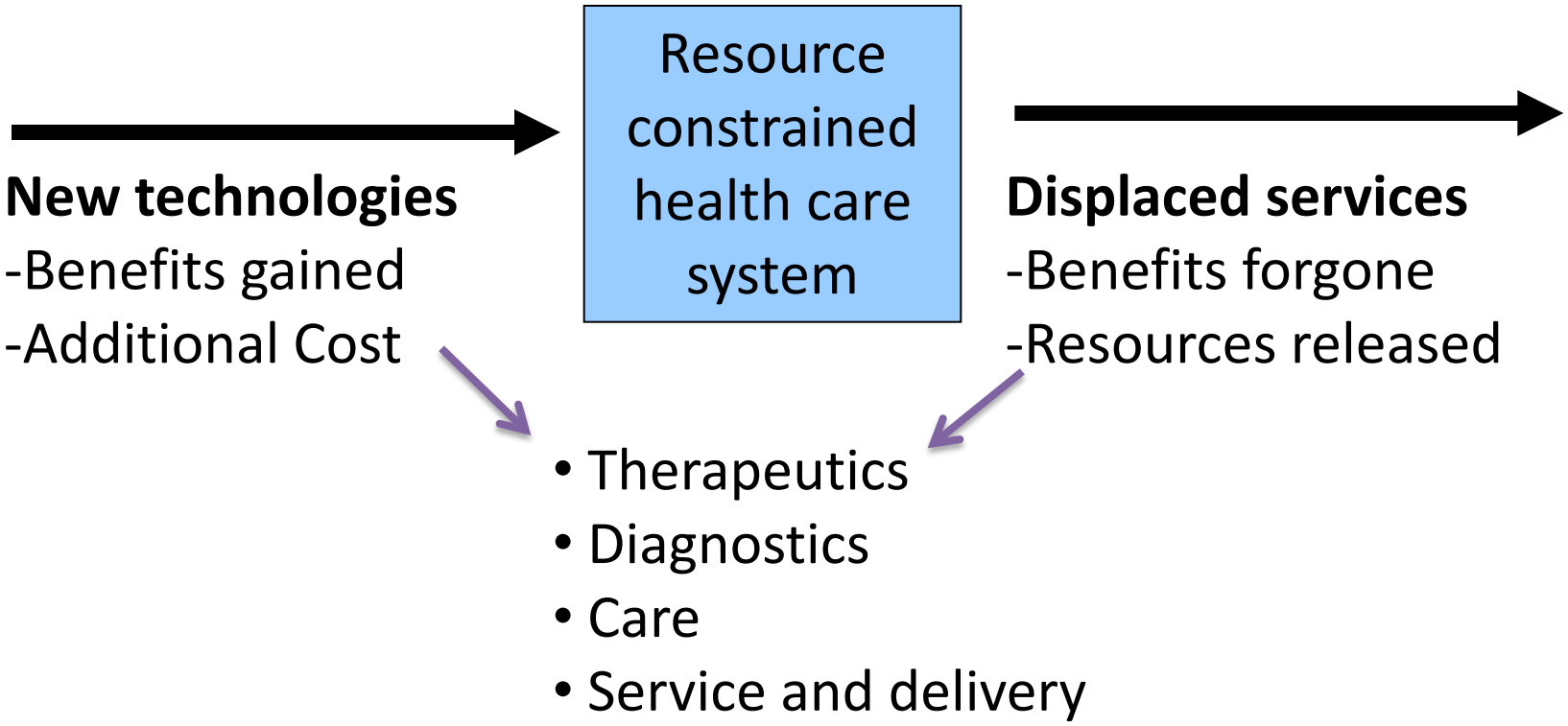
Introduction

- Pressure on budgets
- Perceived need and technological opportunities expanding
- Need to hold health systems accountable for performance
- Range of research ‘movements’ claiming value
  - HTA
  - Comparative effectiveness
- What are the key elements of analysis to support decisions?
- What are the priorities for methods development?

The ultimate question regarding new technologies



Is the benefit gain from the new treatment greater than the benefit foregone through displacement?

Implications and methods challenges

- Need for a generic measure of benefit which reflects the gains and opportunity costs of new technologies
- From net health benefits to social value of health
- A means of reflecting opportunity costs for each decision about new opportunities
- A way of defining sufficient evidence for a positive decision
- Implementing a societal perspective
- Methods to reflect heterogeneity in evidence
- Reflecting non-budgetary constraints

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Reflecting opportunity costs

Efficiency, Equity, and Budgetary Policies: Informing Decisions Using Mathematical Programming

David M. Epstein, MSc, Zaid Chalabi, PhD, Karl Claxton, PhD, Mark Sculpher, PhD

The standard decision rules of cost-effectiveness analysis either require the decision maker to set a threshold willingness to pay for additional health care or to set an overall fixed budget. In practice, neither are generally taken, but instead an arbitrary decision rule is followed that may not be consistent with the overall budget, lead to an allocation of resources that is less than optimal, and is unable to identify the program that should be displaced at the margin. Recent work has shown how mathematical programming can be used as a generalization of the standard decision rules. The authors extend the use of mathematical programming, first to incorporate more complex budgetary rules about when expenditure can be incurred, and show the opportunity loss, in terms of health benefit forgone, of each budgetary policy. Second, the authors demonstrate that indivisibility in a patient population can be regarded as essentially a concern for horizontal equity and represent this and other equity concerns as constraints in the program. Third, the authors estimate the different opportunity costs of a range of equity concerns applied to particular patient populations, and when imposed on all patient populations. They apply this framework of analysis to a realistic and policy-relevant problem. **Key words:** *cost-effectiveness analysis; cost-benefit analysis; mathematical programming; resource allocation.* **(Med Decis Making 2007;27:128–137)**

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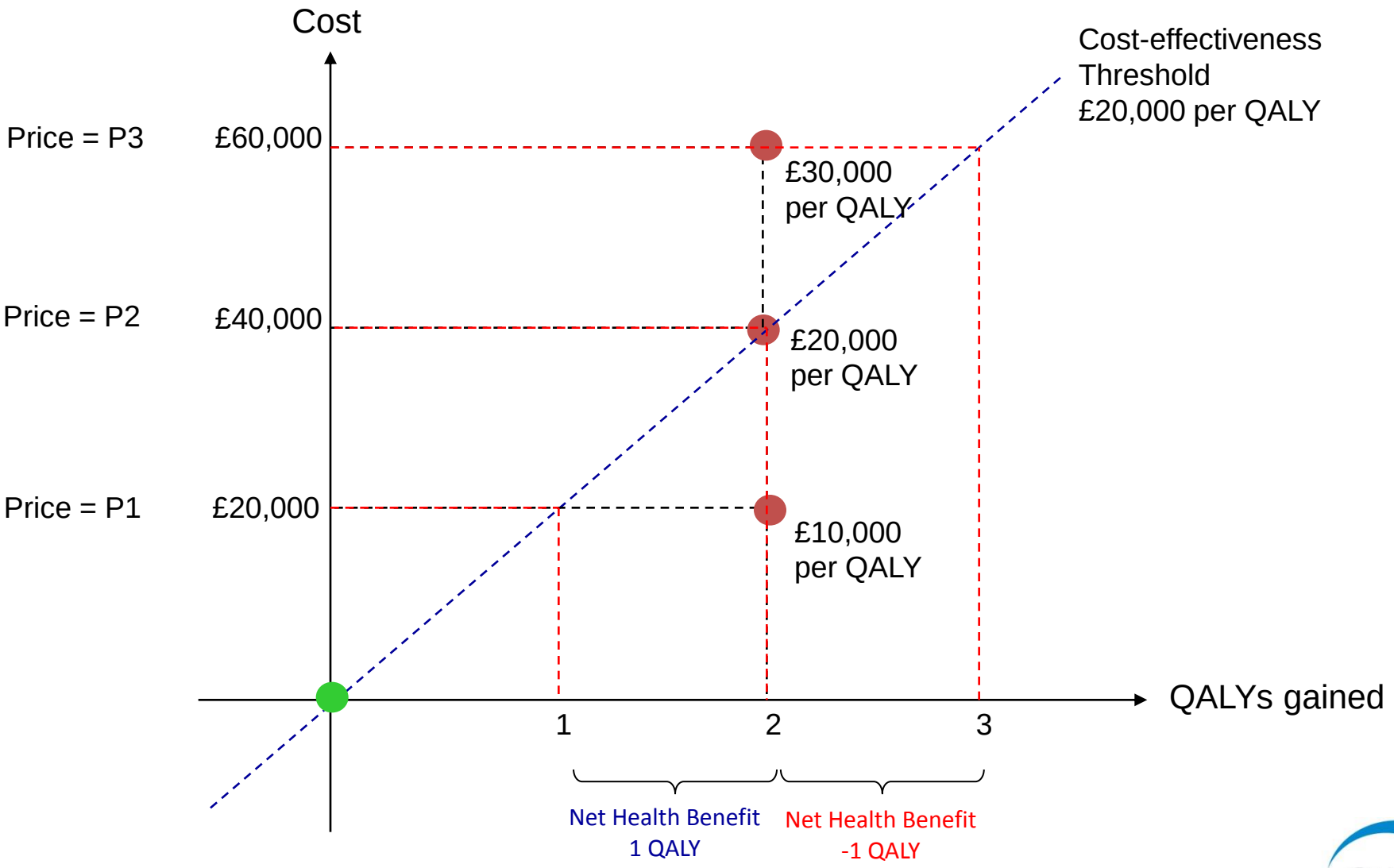
The cost-effectiveness league table

Data source	Disease	Treatments compared and target population	Incremental cost/QALY ($US)
Reference A	Post myocardial infarction	Compare treatment 1 with treatment 2 In people over 65 years of age with severe symptoms	5 000
Reference B	HIV infection	Compare treatment 3 with treatment 4 In people who have early stage disease	9 000
New drug X analysis	HIV infection	Compare drug X with treatment 3 In people who have early stage disease	10 000
Reference C	Urinary tract infection	Compare treatment 5 with treatment 6 In people over 65 years of age with serious disease	16 000
Reference D	HIV infection	Compare treatment 7 with treatment 3 In people who have early stage disease	20 000
Reference E	Post myocardial infarction	Compare treatment 1 with treatment 2 In people over 65 years of age with mild symptoms	50 000

Mauskopf *et al. Pharmacoeconomics* 2003; 21 (14): 991-1000

The cost-effectiveness threshold



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Claxton et al. British Medical Journal, 336, 251-4.

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Appropriate cost-effectiveness thresholds

NHS threshold to guide NICE decisions

Methods for the Estimation of the NICE Cost Effectiveness Threshold

Final Report

Karl Claxton,[1,2] Steve Martin,[3] Marta Soares,[1] Nigel Rice,[1] Eldon Spackman,[1] Sebastian Hinde,[3] Nancy Devlin,[3] Peter C Smith,[4] Mark Sculpher[1]

1. Centre for Health Economics, University of York, UK
2. Department of Economics and Related Studies, University of York, UK
3. Office of Health Economics, London, UK
4. Imperial College, London, UK

http://www.york.ac.uk/media/che/documents/papers/researchpapers/CHERP81_Methods_estimation_NICE_costeffectiveness_threshold.pdf

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Estimated NICE threshold

	[1]	[2]	[3]	
QoL associated with life extension:	*1*	*Norm*		
QoL during disease:	*0*	*0*	*Based on burden*	
			Best estimate	
Effect of expenditure on mortality:	*1 year*	*1 year*	*1 year*	
YLL per death averted:	*~ 4.5 YLL*	*~ 4.5 YLL*	*~ 4.5 YLL*	
QALYs per death averted:	*~ 4.5 QALY*	*~ 3.8 QALY*	*~ 15.0 QALY*	
big 4 PBC's	£10,220	£12,338	£4,872	[1]
11 PBCs (with mortality)	£23,360	£28,045	£8,308	[2]
All 23 PBCs	£25,214	£30,270	**£12,936**	[3]

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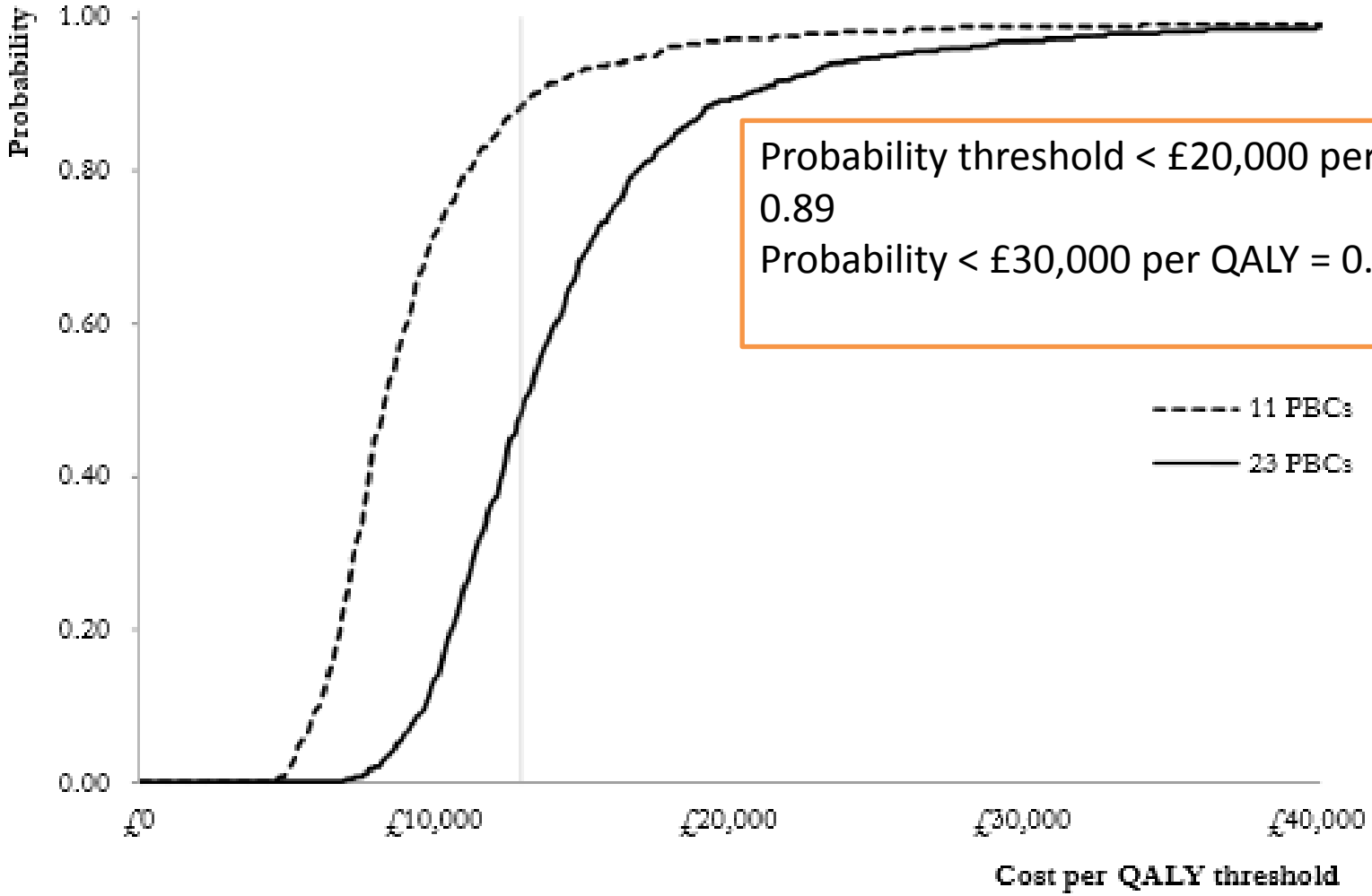
Reflecting uncertainty in the threshold



Probability threshold < £20,000 per QALY = 0.89

Probability < £30,000 per QALY = 0.97

Health consequences of £10m budget impact

	Change in spend (£000)	Additional deaths	LY lost	Total QALY lost	Due to premature death	Quality of life effects
Totals	£10,000	37	167	546	107	439
Cancer	£324.000	3	27	19	18	1
Circulatory	£550.000	17	84	78	53	25
Respiratory	£332.000	10	12	166	7	159
Gastro-intestinal	£232.000	2	18	32	12	20
Infectious diseases	£237.000	1	4	11	3	9
Endocrine	£137.000	< 0.5	4	44	2	42
Neurological	£433.000	1	5	79	3	76
Genito-urinary	£336.000	2	2	8	1	6
Trauma & injuries*	£558.000	0	0	0	0	0
Maternity & neonates*	£495.000	< 0.05	< 0.5	0	< 0.5	< 0.5
Disorders of Blood	£292.000	< 0.5	1	10	1	10
Mental Health	£2,532.000	2	7	51	4	46
Learning Disability	£147.000	< 0.5	1	2	< 0.5	1
Problems of Vision	£275.000	< 0.05	< 0.5	4	< 0.5	3
Problems of Hearing	£124.000	< 0.05	< 0.5	6	< 0.05	6
Dental problems	£409.000	< 0.05	< 0.05	7	< 0.05	7
Skin	£279.000	< 0.5	1	2	1	1
Musculo skeletal	£514.000	< 0.5	2	25	1	24
Poisoning and AE	£132.000	< 0.05	< 0.5	1	< 0.5	1
Healthy Individuals	£501.000	< 0.05	< 0.5	0	< 0.05	< 0.5
Social Care Needs	£426.000	0	0	0	0	0
Other (GMS)	£735.000	0	0	0	0	0

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Estimating thresholds internationally

Journal of the Royal Statistical Society
SERIES A Statistics in Society
J. R. Statist. Soc. A (2015)

Broader health coverage is good for the nation's health: evidence from country level panel data

Rodrigo Moreno-Serra and Peter C. Smith

Imperial College London, UK

[Received July 2012. Revised October 2013]

Summary. Progress towards universal health coverage involves providing people with access to needed health services without entailing financial hardship and is often advocated on the grounds that it improves population health. The paper offers econometric evidence on the effects of health coverage on mortality outcomes at the national level. We use a large panel data set of countries, examined by using instrumental variable specifications that explicitly allow for potential reverse causality and unobserved country-specific characteristics. We employ various proxies for the coverage level in a health system. Our results indicate that expanded health coverage, particularly through higher levels of publicly funded health spending, results in lower child and adult mortality, with the beneficial effect on child mortality being larger in poorer countries.

Keywords: Health coverage; Health financing; Mortality; Panel data econometrics; Reverse causality

Implementing a societal perspective

Pharmacy budgets Health system + Other public sector

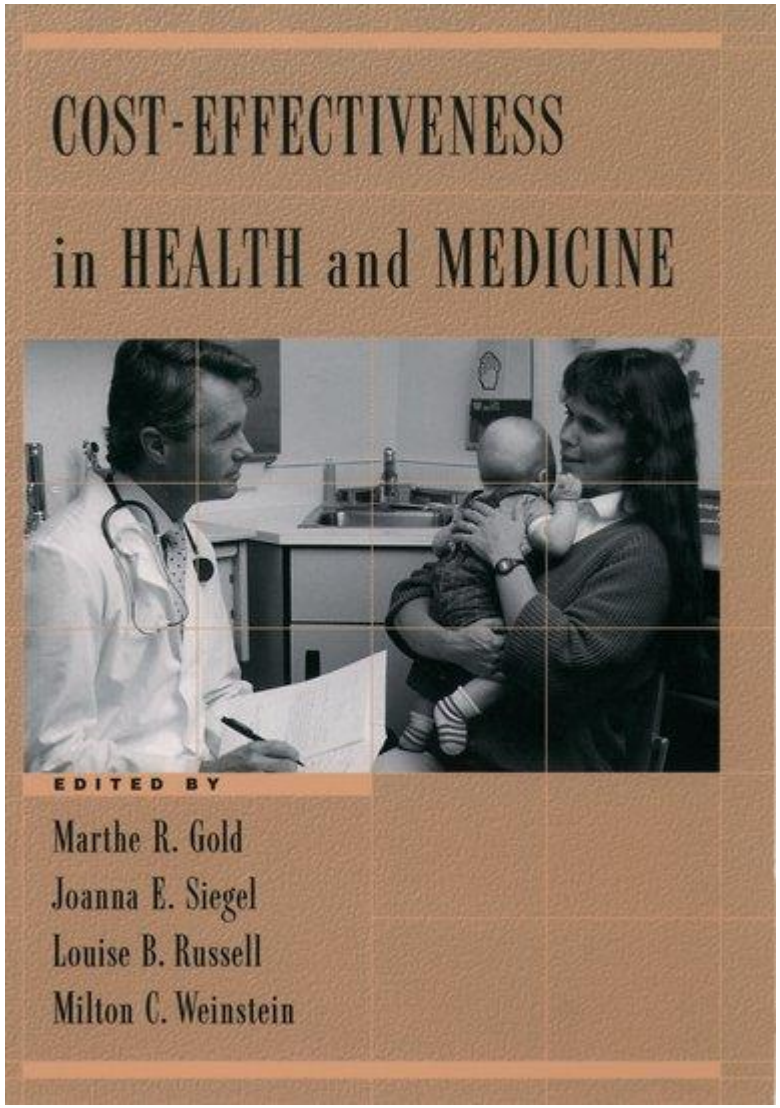
Singe payer costs + patients' costs + net production

Disease effects Generic health + Process effects

HRQoL effects + Equity weighted health Other sectoral effects

Societal perspective

Strong ‘textbook’ case



In:

- Health system costs
- Patient out of pocket costs
- Patient time costs
- Carer costs
- Costs falling on other sectors
- (Some) productivity costs

Out:

- Non health effects
- Productivity net of consumption
- ‘Unrelated’ future costs

Not reflected in decision making (1)

Organisation	Societal	Health system	Both	Unclear
Australia			√	
Austria				√
Baltic		√		
Belgium		√		
Brazil		√		
Canada		√		
Cuba	√			
Eng and Wales		√		
Finland	√			
France	√			
Germany		√		
Hungary		√		
Ireland		√		

Claxton, K., et al. (2010), *Appropriate Perspectives for Health Care Decisions. CHE Research Paper 54 (http://www.york.ac.uk/inst/che/pdf/rp54.pdf)* (York: Centre for Health Economics, University of York).

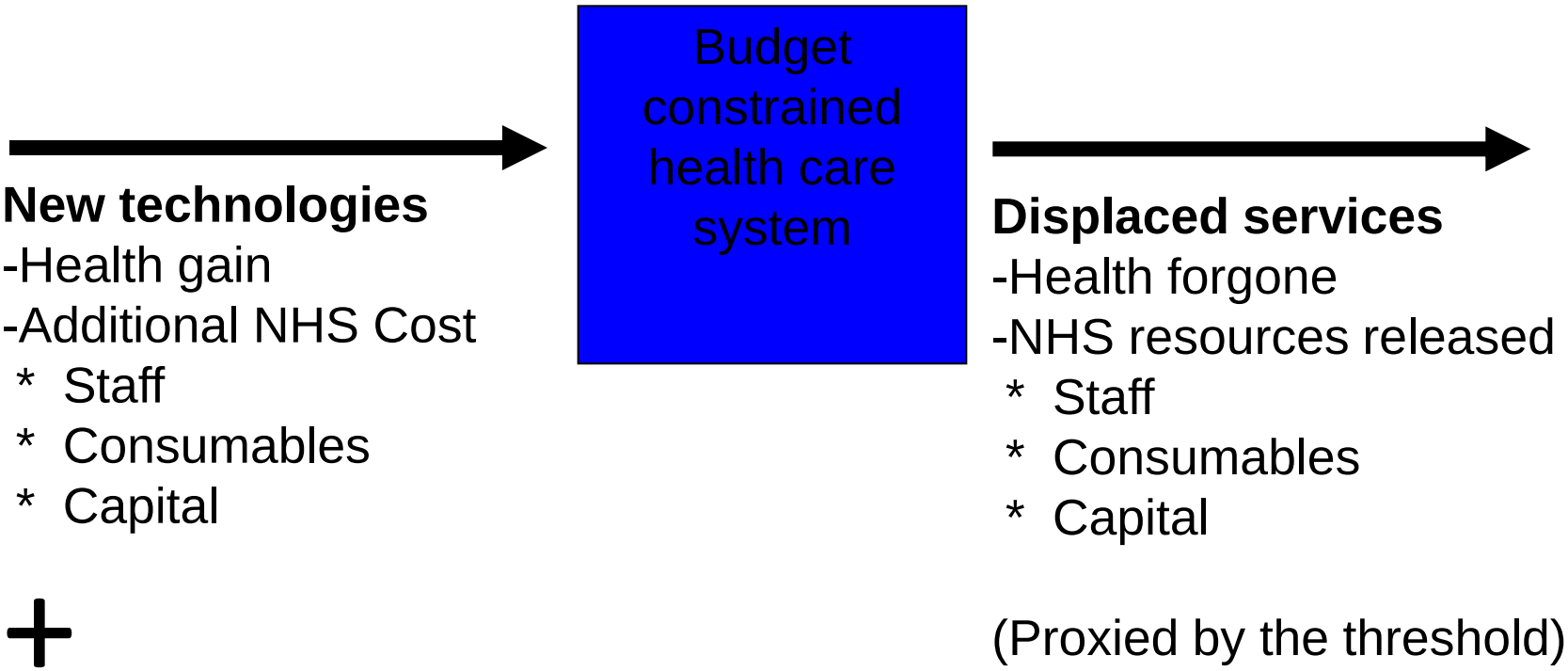
Not reflected in decision making (2)

Organisation	Societal	Health system	Both	Unclear
Isreal		√		
Italy			√	
Mexico		√		
Netherlands	√			
New Zealand		√		
Norway			√	
Poland		√		
Portugal	√			
Russia			√	
Scotland		√	√	
Spain				
Sweden	√			
US (AMCP)		√		
Totals	**6/26**	**14/26**	**5/26**	**1/26**

Claxton, K., et al. (2010), *Appropriate Perspectives for Health Care Decisions. CHE Research Paper 54 (http://www.york.ac.uk/inst/che/pdf/rp54.pdf)* (York: Centre for Health Economics, University of York).

Implementing a societal perspective

Approach 1: Assume costs fall NHS budget



Effects outside the NHS

-Direct costs on patients/families

-Indirect effects (productivity)

-Other public sector effects

Implementing a societal perspective

Approach 2: Use of an external threshold

New technologies
-Health gain
-Additional NHS Cost
* Staff
* Consumables
* Capital

+

Effects outside the NHS
-Direct costs on patients/families
-Indirect effects (productivity)
-Other public sector effects

Is the cost per QALY less than the public's willingness to pay for health in terms of consumption (V)?

Implementing a societal perspective

Approach 3: 'Textbook' cost-benefit analysis

New technologies
-Health gain
-Additional NHS Cost
* Staff
* Consumables
* Capital

+

Effects outside the NHS
-Direct costs on patients/families
-Indirect effects (productivity)
-Other public sector effects

- Focus on individual preferences
- Value all effects according to preferences expressed through market prices
- Shadow prices for imperfect markets
- Contingent valuation where no market

Problems with methods of implementation

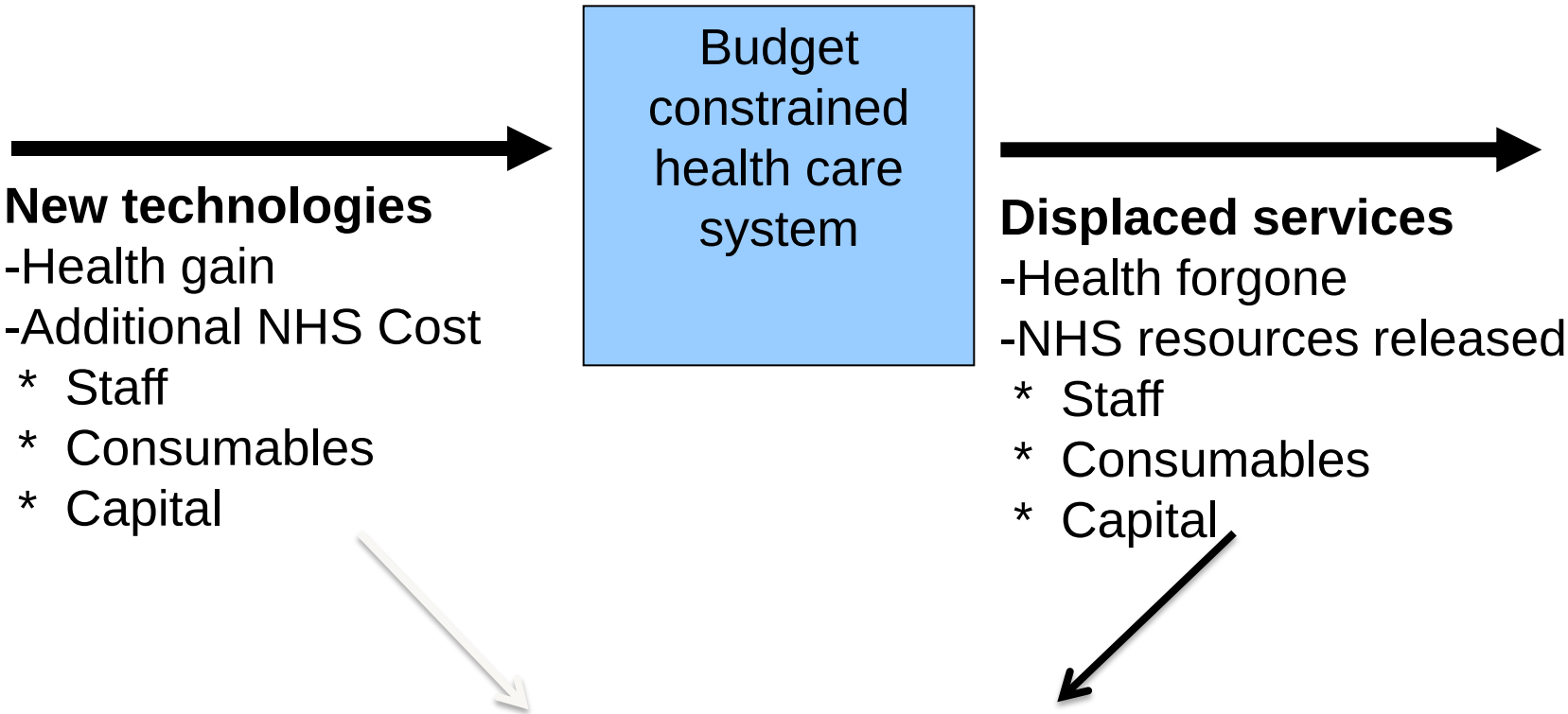
Ignores budget constraints

- Assumes can define and implement optimal budgets
- Assumes budgets adjust instantaneously
- Characterises budgets as ‘administrative nuisances’

Strong value judgements about social value

- Focus on individual preferences imposes strong value judgements
- Impossible to define a complete and consensual social welfare function
- Should analysts define one social welfare function?

The external effects of displaced services



Wider social benefits: gains and losses

- Appraisal of ranibizumab (Lucentis) for diabetic macular oedema 2011
  - Retinal thickness ≥ 400 subgroup before PAS
  - Additional costs = £3,506 per patient
  - Incremental cost-effectiveness = £25,000 per QALY
  - 23,000 eligible patients each year

Attributes	Investment	Disinvestment	Net effects
	Lucentis for diabetic macular oedema (£80m pa)	Expected effects of £80m pa	
Deaths	0	-411	-411
Life years	0	- 1,864	-1,864
QALYs	3,225	- 6,184	-2,959
Burden of disease QALY loss	2.68	2.07	0.61
Wider social benefits Consumption QALY equivalent (£60,000 per QALY)	£85.2m 1,420	- £49.8m - 830	£5.4m 590