Making Economic Evaluation Fit for Purpose to Guide Resource Allocation Decisions

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Outline

- Policy context – successes and failures of economic evaluation in supporting decisions

- A new lease if life – empirical estimates of opportunity cost to inform decisions

- There's always something else – reflecting wider considerations in economic evaluation

- Breaking down barriers – linking economic evaluation with health system research
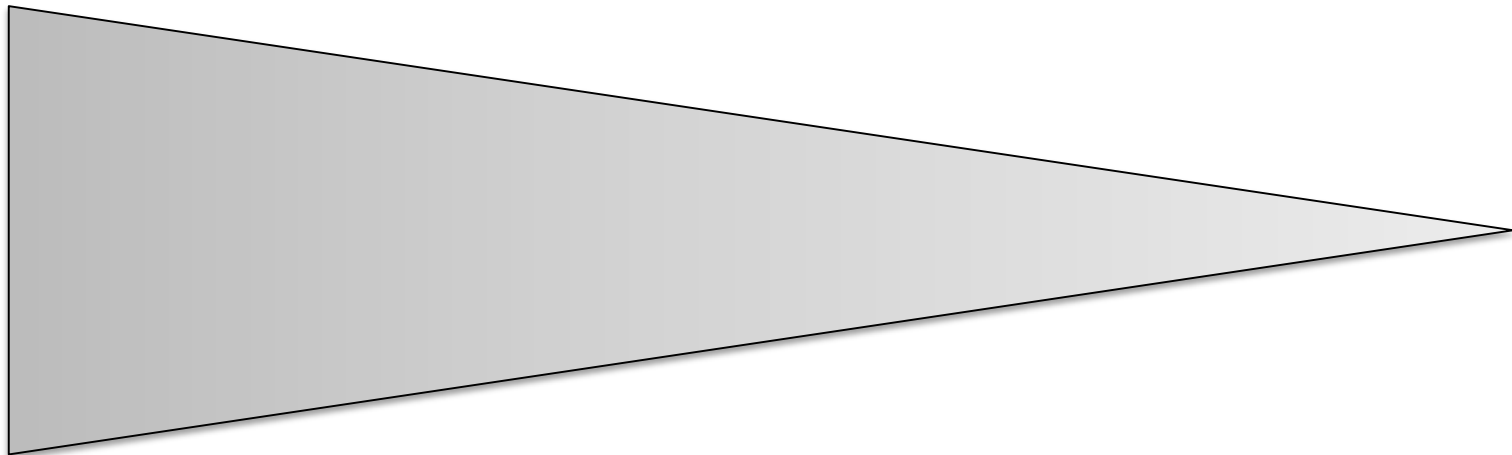
Policy context
Routine use of economic evaluation internationally

World map

■ Formal use of economic evaluation

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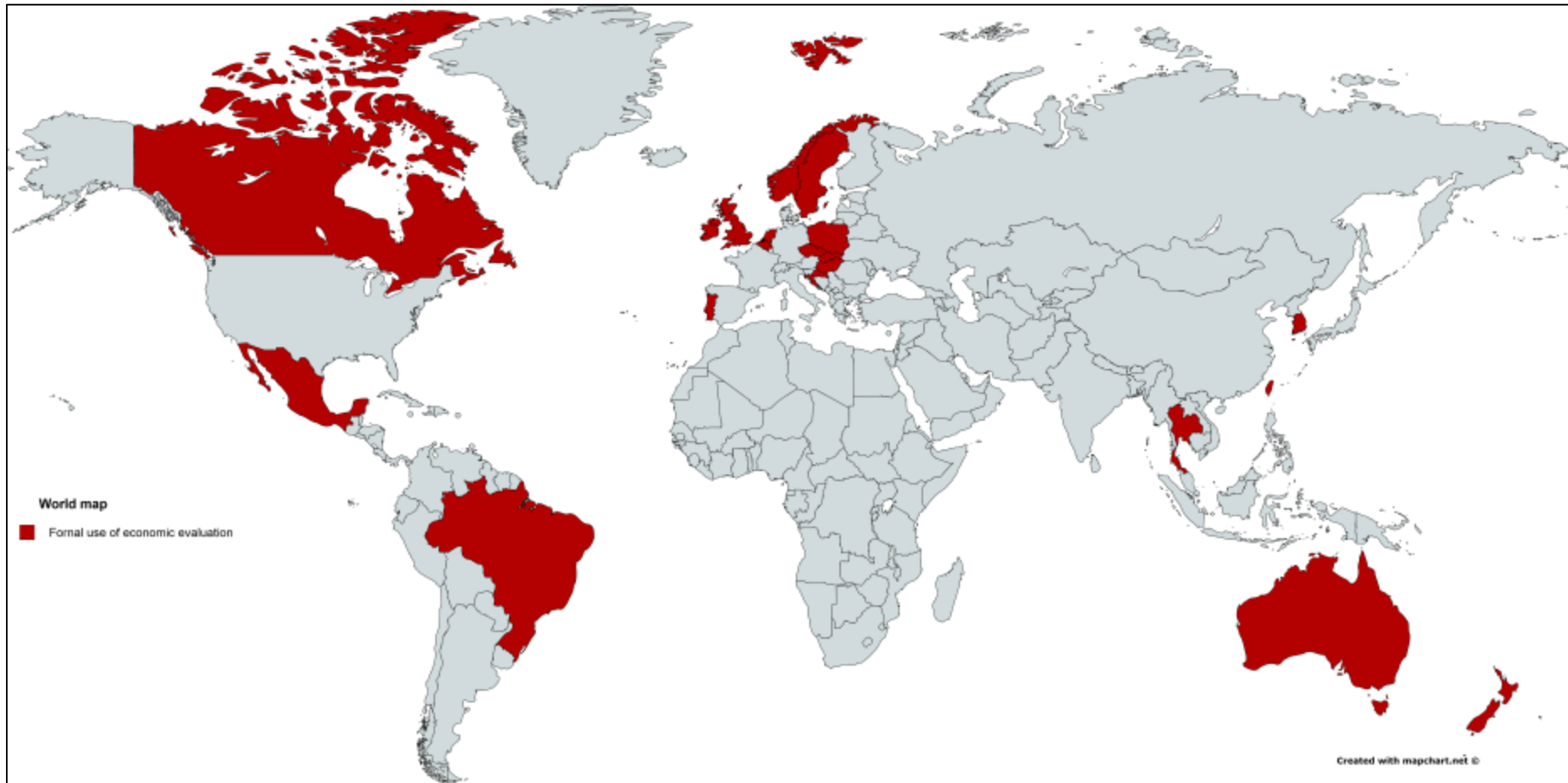
Policy context

Decisions routinely informed by evaluation

Frequency →

Branded drugs

Medical devices

Surgical procedures

Public health

Service development

Infrastructure investment

System reform

Staffing levels

The spectrum of quantification

Increasing use of formal quantification of trade-offs

→

Limited quantification
-Examples: Germany, France, US?
-Focus on individual effects
-Possible interest cost, not CEA

Greater use of quantification
-Examples: UK, Sweden
-CEA countries
-Use of QALYs

The future?
-UK value-based pricing
-Weights to QALYs
   - Severity
   - Unmet needs
   - End of life

Policy context

Calls for changes in methods



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

A consultation

"higher thresholds for medicines that tackle diseases where there is greater burden of illness,…for medicines that can demonstrate greater therapeutic innovation,… for medicines that can demonstrate wider societal benefits."

Department of Health, *A New Value-Based Approach to the Pricing of Branded Medicines - a Consultation*. 2010, London Department of Health. Para 4.10, p.13.

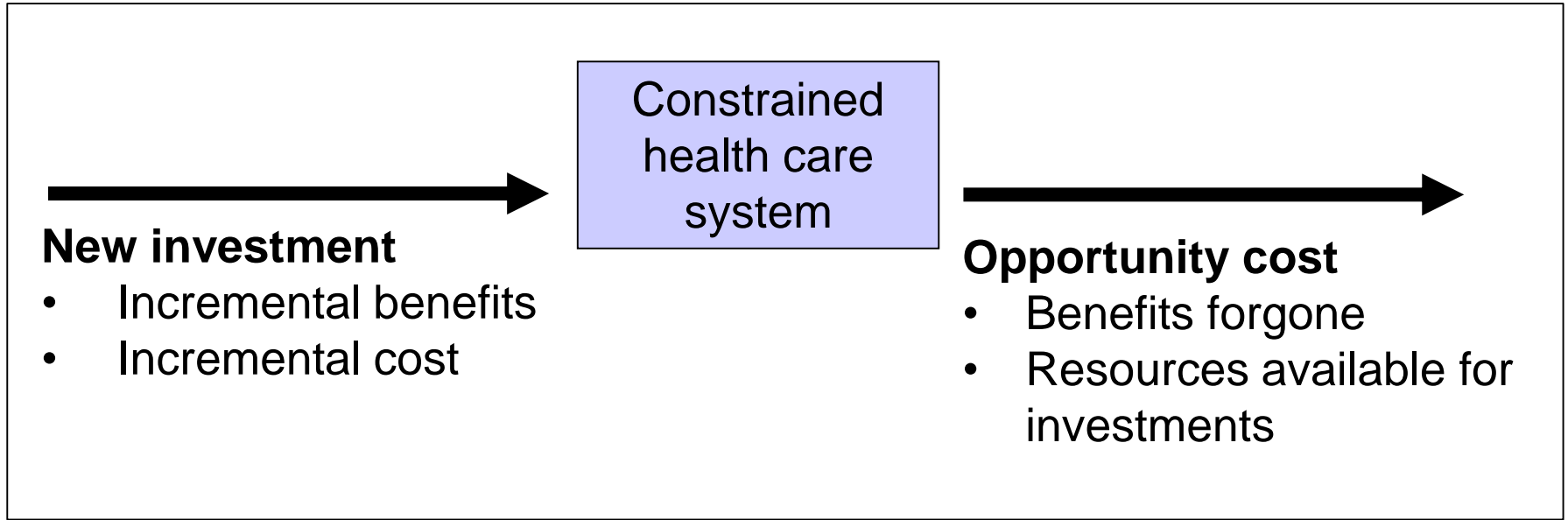# Policy context

Multi-criteria decision analysis (MCDA)
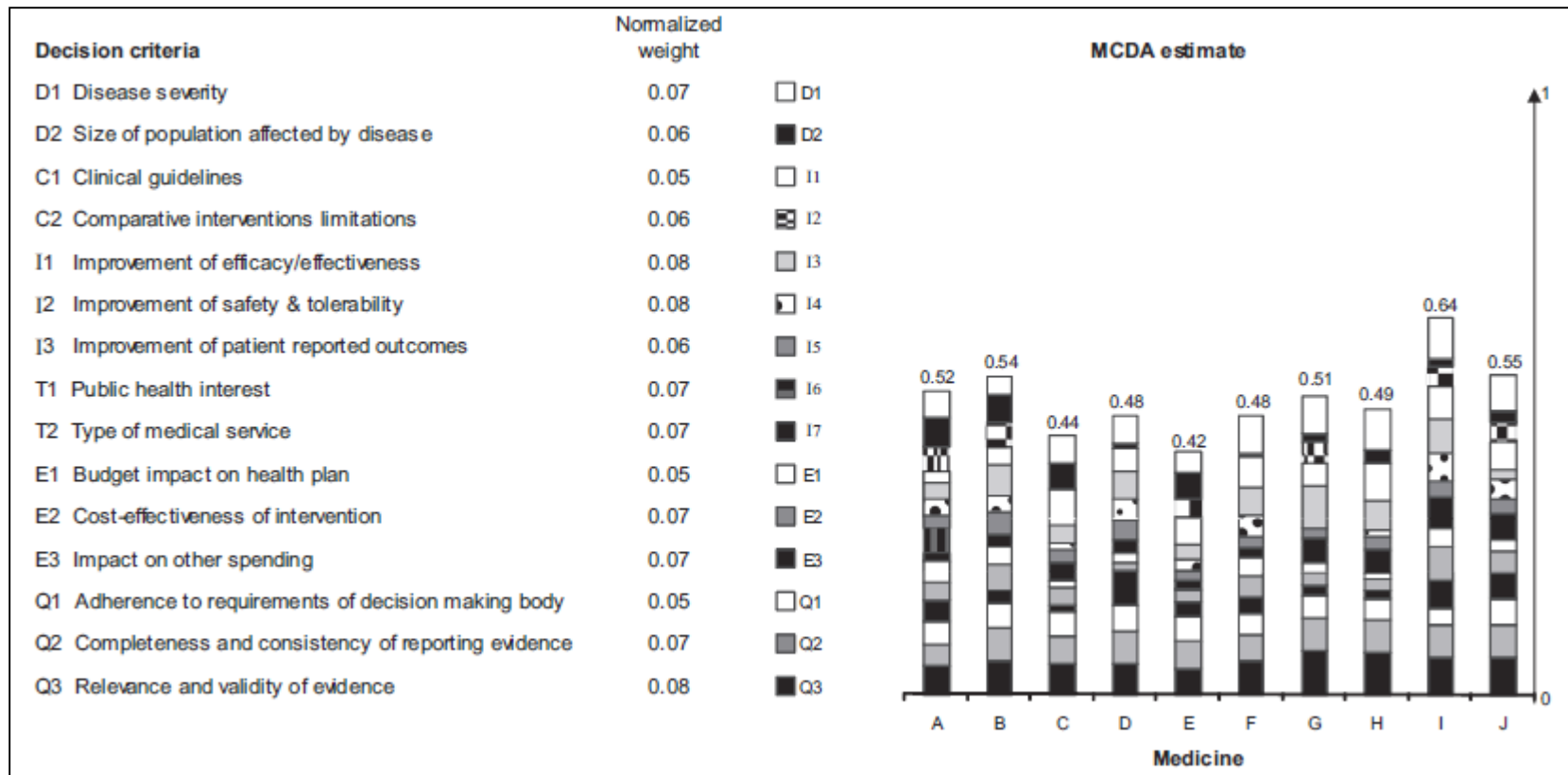
"In the current debate about limitations of reliance on cost-effectiveness for decision making, MCDA approaches open a path to fully exploit the available evidence and move beyond the cost-effectiveness paradigm for decision making." (p.385)

Goetghebeur *et al.* *Medical Decision Making* 2012 Mar-Apr;32(2):376-88

Raises a series of questions

- What are the key principles of economic analysis to support decision making?

- What role should economic analysis have in decision making?

- How do reflect multiple objectives/criteria?

- How can economic analysis support decisions beyond medical technologies?

Assessments necessary for decision making

Constrained health care system

New investment
- Incremental benefits
- Incremental cost

Opportunity cost
- Benefits forgone
- Resources available for investments

What's the threshold mean?

Claxton et al. *British Medical Journal 2008;336:251-4.*

Analysis for value frameworks

Analytical challenges	Methods developments	Success?
Identifying all existing evidence	• Systematic review	****
Generating appropriate new evidence quickly and efficiently	• Novel trial design • Observational data analysis	**
Comparing full range of alternative options	• Evidence synthesis • Network meta-analysis	***
Estimating costs and benefits over suitable time horizon	• Epidemiological modelling • Decision analysis	***
Measuring health to facilitate comparison	• Preference elicitation • Trading length and quality of life	**
Analysing the implications of uncertainty	• Value of information methods	***
Reflecting heterogeneity	• Statistical modelling • Value of individualised care	***

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Quantifying opportunity costs as consistent benchmarks of value	• Other uses of resources for health and other benefits	*
Incorporating other aspects of benefit	• Agreeing additional benefits • Trading off health with other benefits	*

Quantifying health opportunity costs to guide decisions

- Specific new investment
- Imposing additional cost
- No increasing budget

→

- What interventions are displaced?
- What health is forgone?
- Direct disinvestment vs leave to system

- Specific new investment
- Imposing additional cost
- Increasing budget

→

- What other new investments are possible?
- What health could have been generated?
- Direct investments vs leave to system

- Specific new investment
- Generating net savings

→

- What additional activities are funded?
- What are the health gains?
- Direct investment vs leave to system

- **What health does the health system generate from increases or decreases in expenditure?**
- **What is the marginal productivity of the health system?**

Empirical basis for health opportunity costs

HEALTH TECHNOLOGY ASSESSMENT
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ISSN 1366-5278

Methods for the estimation of the National
Institute for Health and Care Excellence
cost-effectiveness threshold

Karl Claxton, Steve Martin, Marta Soares, Nigel Rice, Eldon Spackman,
Sebastian Hinde, Nancy Devlin, Peter C Smith and Mark Sculpher

NHS
National Institute for
Health Research

DOI 10.3310/hta19140

- Estimate of marginal productivity of English NHS
- Based on linking expenditure to mortality
  - Variation between local commissioners
  - Across clinical areas
- Extrapolation to QALYs
- Central estimate £12,936
  - 2008 expenditure
  - 2008-10 mortality

Claxton *et al. Health Technol Assessment,* 2015. 19(14): p. 503.

Health opportunity costs in drug evaluation

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

Attributes	Investment	Forgone	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

Claxton *et al. Health Technol Assessment,* 2015. 19(14): p. 503.

Using economic evaluation in decision making?

- Guiding rather than dictating

- Range of other factors may be relevant to a decision
  - Equity (e.g. burden, severity, rarity)
  - Innovation
  - Wider economic effects

- Requires transparency, supports accountability

Attributes	Investment	Forgone	Net effects
	Lucentis for diabetic macular oedema (£80m pa)	Expected effects of £80m pa	
QALYs	3,225	- 6,184	-2,959

Do expected non-health effects compensate for negative impact on expected population health?

Opportunity costs

Claxton K, *et al.*, *Health Economics,* 2015, DOI: 10.1002/hec.3130.

Burden of disease (QALY loss)			Wider Social Benefits (net production)		
C22	Liver cancer	10.70	M05	Rheumatoid arthritis	£30,034
C25	Pancreatic cancer	9.97	E11	Diabetes	£27,421
C34	Lung cancer	9.68	M45	Ankylosing spondylitis	£26,190
F20	Schizophrenia	7.62	F30	Depression	£23,489
G35	Multiple sclerosis	6.18	F20	Schizophrenia	£22,697
C92	Myeloid leukaemia	6.15	J45	Asthma	£20,100
G20	Parkinson's disease	4.60	M81	Osteoporosis	£17,910
C90	Myeloma	4.45	G35	Multiple sclerosis	£15,482
J43	Emphysema and COPD	3.80	J43	Emphysema and COPD	£14,525
C64	Kidney cancer	3.75	G40	Epilepsy	£14,245
F30	Depression	3.63	L40	Psoriasis	£11,890
M05	Rheumatoid arthritis	2.83	Displaced	Average of displaced QALYs	£11,611
E11	Diabetes	2.68	E66	Obesity	£8,138
Displaced	Average of displaced QALYs	2.07	C53	Cervical cancer	£6,912
J45	Asthma	1.86	K50	Irritable Bowel Syndrome	£6,284
G30	Alzheimer's disease	1.68	J30	Allergic rhinitis	£5,234
F03	Dementia	1.68	G20	Parkinson's disease	£3,102
G40	Epilepsy	1.32	C50	Breast cancer	£2,888
C18	Colon cancer	1.28	G30	Alzheimer's disease	£351
I26	Embolisms, fibrillation, thrombosis	1.16	A40	Streptococcal septicaemia	-£513
C61	Prostate cancer	1.06	F03	Dementia	-£2,430
I21	Acute myocardial infarction	1.00	I64	Stroke	-£6,949
I64	Stroke	0.83	C18	Colon cancer	-£8,061
C53	Cervical cancer	0.60	C61	Prostate cancer	-£10,602
C50	Breast cancer	0.55	C64	Kidney cancer	-£13,211
A40	Streptococcal septicaemia	0.38	I21	Acute myocardial infarction	-£14,395
J30	Allergic rhinitis	0.30	I26	Embolisms, fibrillation, thrombosis	-£16,752
M81	Osteoporosis	0.28	J10	Influenza	-£21,568
K50	Irritable Bowel Syndrome	0.26	C90	Myeloma	-£23,382
J10	Influenza	0.19	C92	Myeloid leukaemia	-£24,813
L40	Psoriasis	0.19	C22	Liver cancer	-£32,709
E66	Obesity	0.18	C34	Lung cancer	-£36,067
M45	Ankylosing spondylitis	0.11	C25	Pancreatic cancer	-£53,860

Broadening the objective function - example

Attributes	Investment	Forgone	Net effects
	Lucentis for diabetic macular oedema (£80m pa)	Expected effects of £80m pa	
QALYs	3,225	- 6,184	-2,959
Burden of disease QALY loss	2.68	2.07	0.61
Wider social benefit	£85.2m	- £49.8m	£35.4m

Broader perspectives: how should we decide?

Perspective	Value
Health and health care	• Net health benefits = 3,225 – 6,184 = - 2,959 QALYs
Net societal cost: ignore opportunity costs	• Net costs = £80m - £85.2m = - £5.2m
Broader perspective: account for health and wider social benefits opportunity costs	• Net health loss = -2,959 QALYs • Net wider social benefits = £85.2m – £49.8m = £35.4m • Worthwhile if consumption value of health < £11,900 per QALY

Conclusions

- Economic evaluation's impact on policy mixed

- Pressure for change from methodologists and policy makers

- Empirical estimates of opportunity cost open up new vistas

- Provides framework for broadening benefit measure

- Offers link between evaluation and system research

Reserve Slides

What if we valued health using willingness to pay?

- When we recognize financial constraints, valuing health using consumption forgone makes no difference
- k: health opportunity cost, marginal productivity (threshold)

Standard 'decision rule':

$$\frac{\mathrm{D}c}{\mathrm{D}h} < k$$

Net health benefits:

$$\mathrm{D}h - \frac{\mathrm{D}c}{k} > 0$$

Net health benefits
(consumption value of health):

$$v[\mathrm{D}h] - v\left[\frac{\mathrm{D}c}{k}\right] > 0$$

NICE and population health 2009-11

Drug	No of eligible patients/year in England and Wales	Total change in costs (£)	Total QALYs gained	Net effect on QALYs	
				£30 000/ QALY	£20 000/ QALY
Sunitinib (renal cell carcinoma)	3095	98 795 495	1 837	−1 457	−3 103
Lenalidomide	3562	193 384 542	4 417	−2 029	−5 252
Sunitinib (gastrointestinal stromal tumour)	240	3 822 720	120	−7	−71
Topotecan	1600	9 910 400	293	−38	−203
Trabectedin	600	11 074 800	321	−48	−233
Pemetrexed	4642	62 105 318	1 322	−749	−1 784
Trastuzumab	492	6 067 644	124	−79	−180
Pazopanib	4000	111 600 000	2 868	−852	−2 712
Azacitidine	700	53 182 500	1 099	−674	−1 560
Total	18 931	549 943 419	12 401	−5 933	−15 098