

# **Making Economic Evaluation Fit for Purpose to Guide Resource Allocation Decisions**

**Mark Sculpher, PhD**

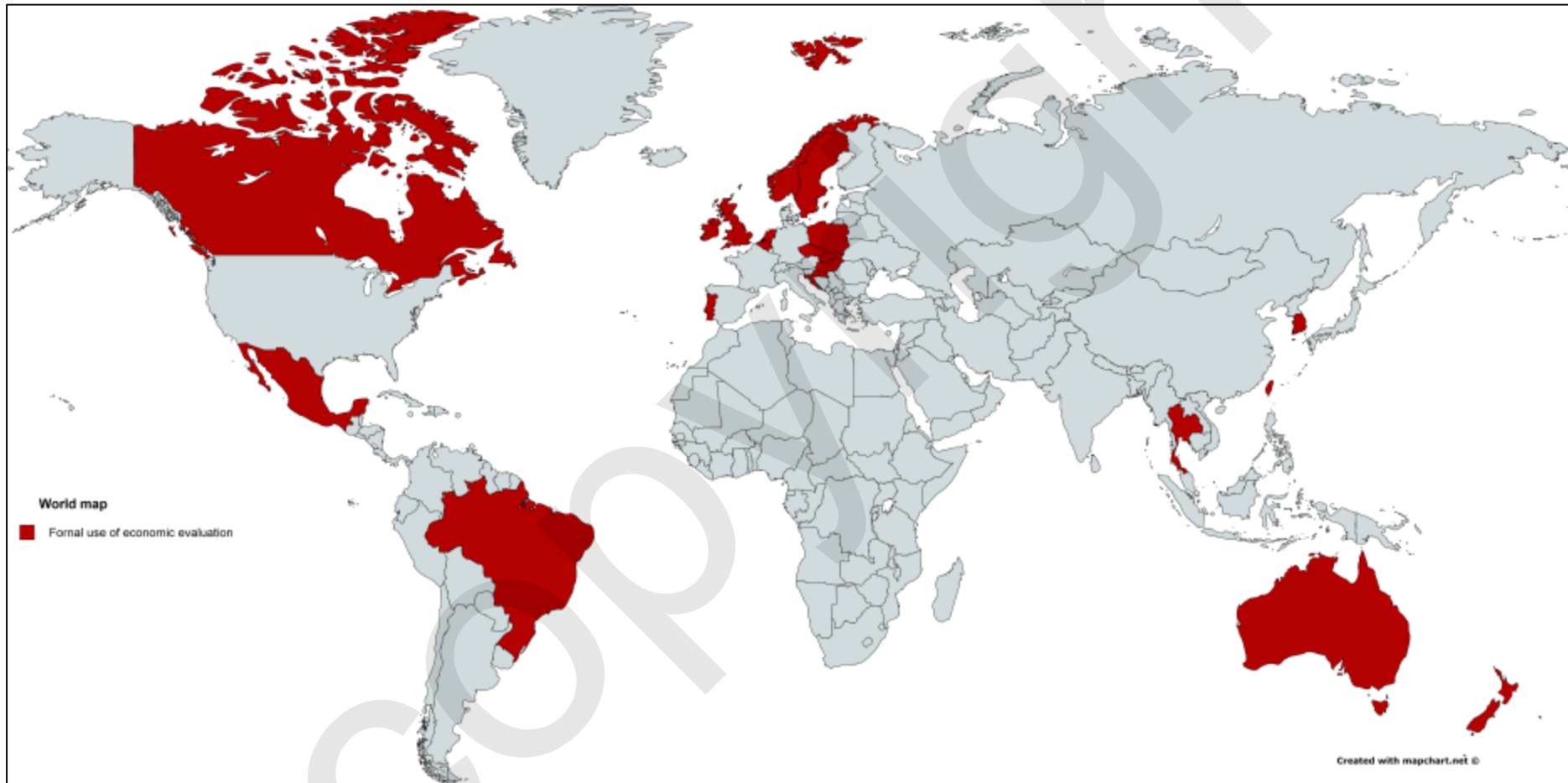
***Professor of Health Economics  
Centre for Health Economics  
University of York, UK***

## 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

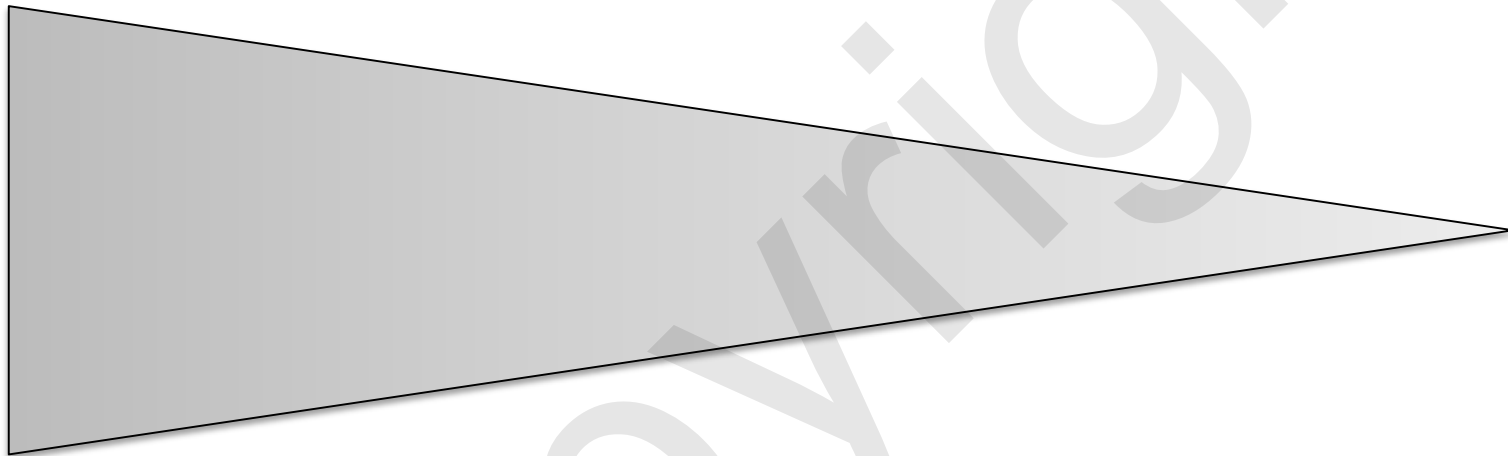
## Routine use of economic evaluation internationally



# 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**

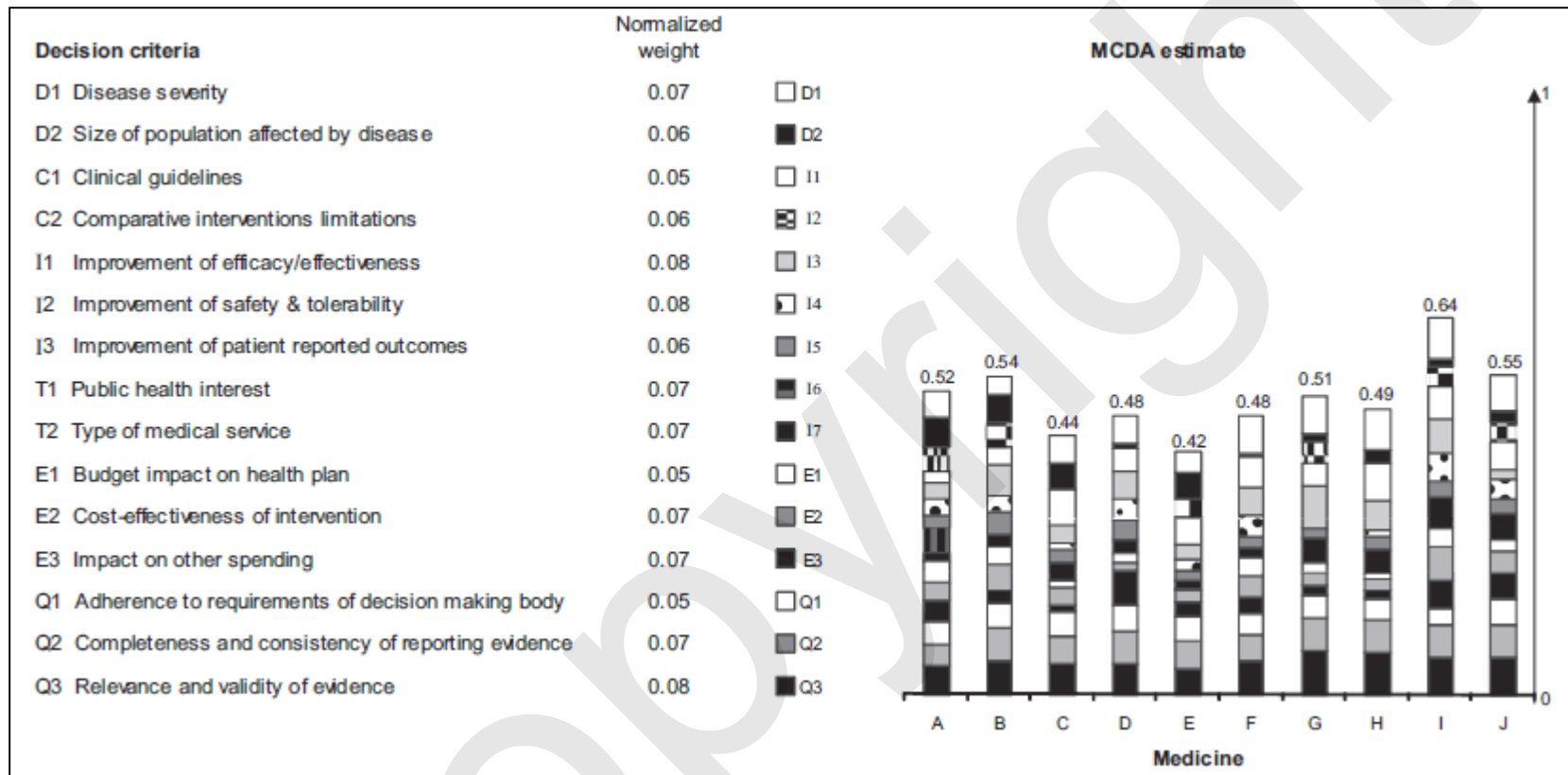
*A consultation*

“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)



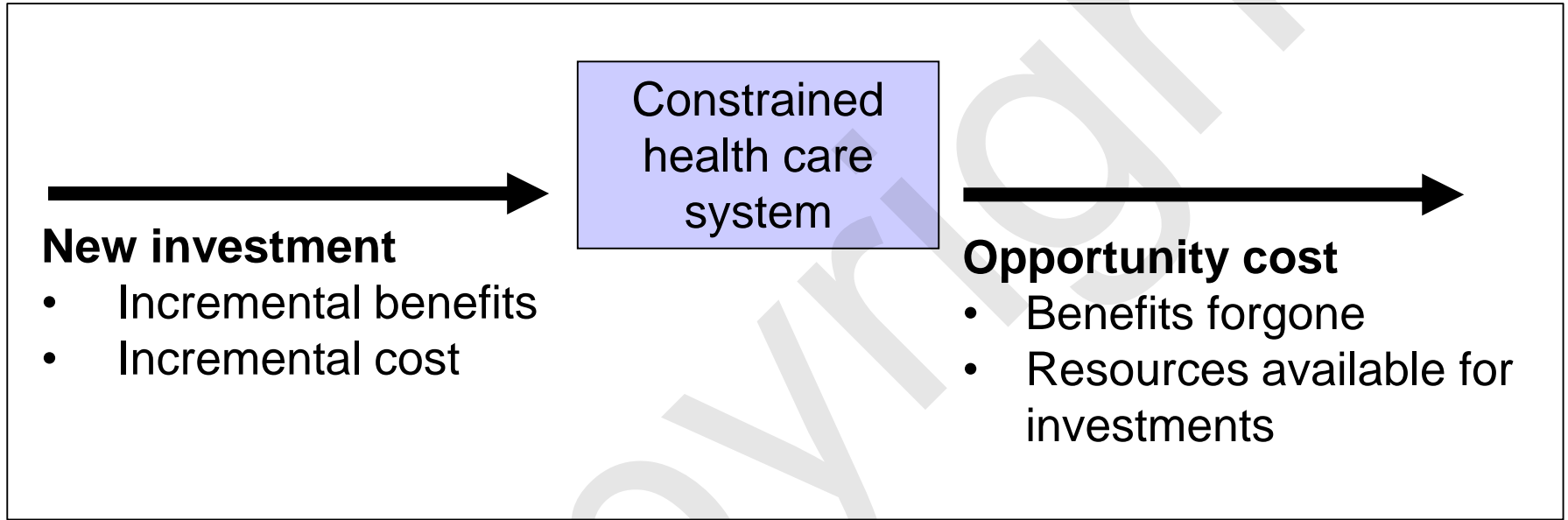
“In the current debate about **limitations of reliance on cost-effectiveness** for decision making, MCDA approaches open a path to fully exploit the available evidence and **move beyond the cost-effectiveness** paradigm for decision making.” (p.385)

## **Raises a series of questions**

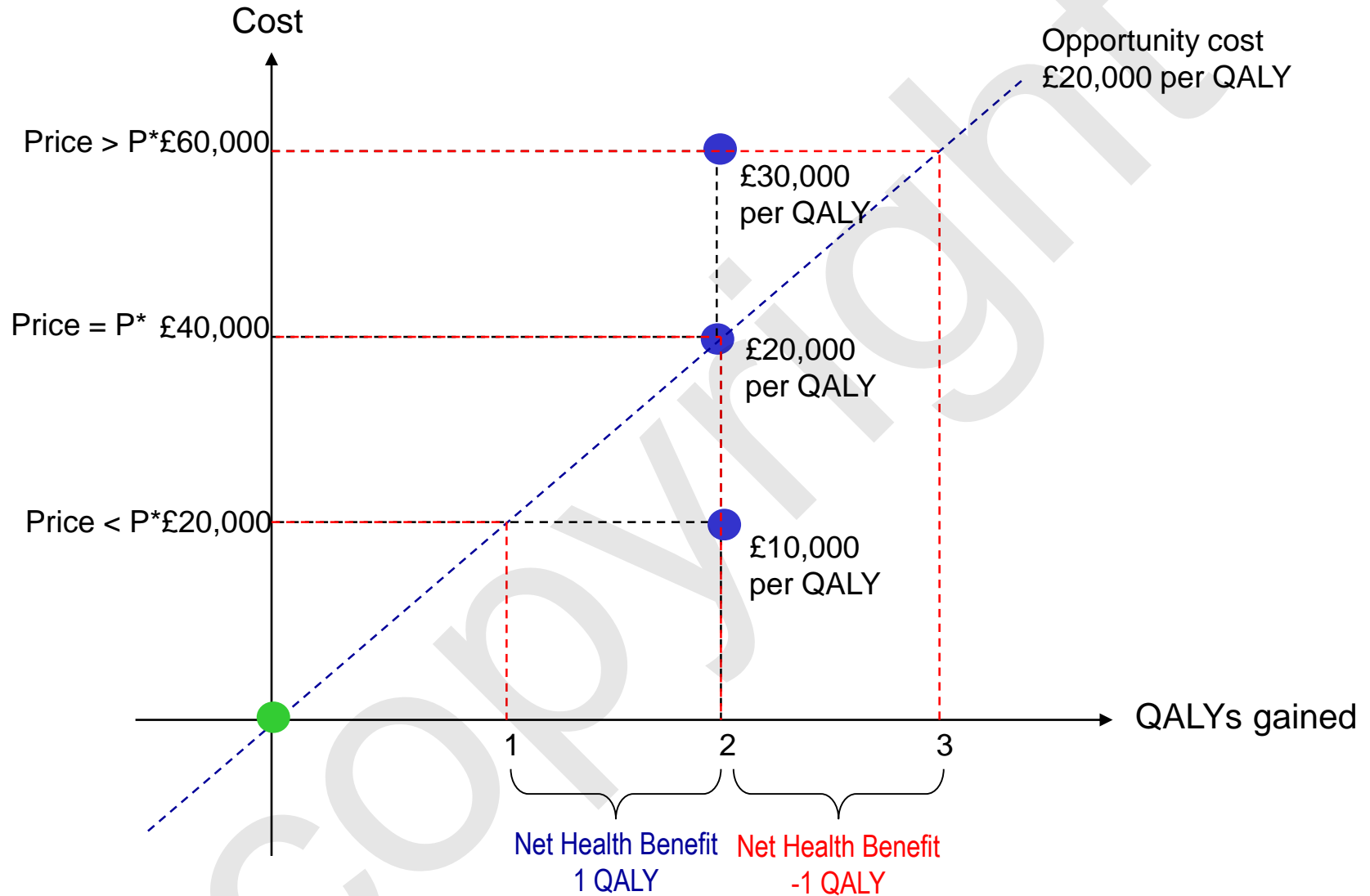
- What are the key principles of economic analysis to support decision making?
- What role should economic analysis have in decision making?
- How do reflect multiple objectives/criteria?
- How can economic analysis support decisions beyond medical technologies?



# Assessments necessary for decision making



# What's the threshold mean?



# Analysis for value frameworks

Analytical challenges	Methods developments	Success?
Identifying all existing evidence	<ul style="list-style-type: none"><li>• Systematic review</li></ul>	****
Generating appropriate new evidence quickly and efficiently	<ul style="list-style-type: none"><li>• Novel trial design</li><li>• Observational data analysis</li></ul>	**
Comparing full range of alternative options	<ul style="list-style-type: none"><li>• Evidence synthesis</li><li>• Network meta-analysis</li></ul>	***
Estimating costs and benefits over suitable time horizon	<ul style="list-style-type: none"><li>• Epidemiological modelling</li><li>• Decision analysis</li></ul>	***
Measuring health to facilitate comparison	<ul style="list-style-type: none"><li>• Preference elicitation</li><li>• Trading length and quality of life</li></ul>	**
Analysing the implications of uncertainty	<ul style="list-style-type: none"><li>• Value of information methods</li></ul>	***
Reflecting heterogeneity	<ul style="list-style-type: none"><li>• Statistical modelling</li><li>• Value of individualised care</li></ul>	***

# Analysis for value frameworks

Analytical challenges	Methods developments	Success?
Identifying all existing evidence	<ul style="list-style-type: none"> <li>• Systematic review</li> </ul>	****
Generating appropriate new evidence quickly and efficiently	<ul style="list-style-type: none"> <li>• Novel trial design</li> <li>• Observational data analysis</li> </ul>	**
Comparing full range of alternative options	<ul style="list-style-type: none"> <li>• Evidence synthesis</li> <li>• Network meta-analysis</li> </ul>	***
Estimating costs and benefits over suitable time horizon	<ul style="list-style-type: none"> <li>• Epidemiological modelling</li> <li>• Decision analysis</li> </ul>	***
Measuring health to facilitate comparison	<ul style="list-style-type: none"> <li>• Preference elicitation</li> <li>• Trading length and quality of life</li> </ul>	**
Analysing the implications of uncertainty	<ul style="list-style-type: none"> <li>• Value of information methods</li> </ul>	***
Reflecting heterogeneity	<ul style="list-style-type: none"> <li>• Statistical modelling</li> <li>• Value of individualised care</li> </ul>	***
Quantifying opportunity costs as consistent benchmarks of value	<ul style="list-style-type: none"> <li>• Other uses of resources for health and other benefits</li> </ul>	*
Incorporating other aspects of benefit	<ul style="list-style-type: none"> <li>• Agreeing additional benefits</li> <li>• Trading off health with other benefits</li> </ul>	*

# 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

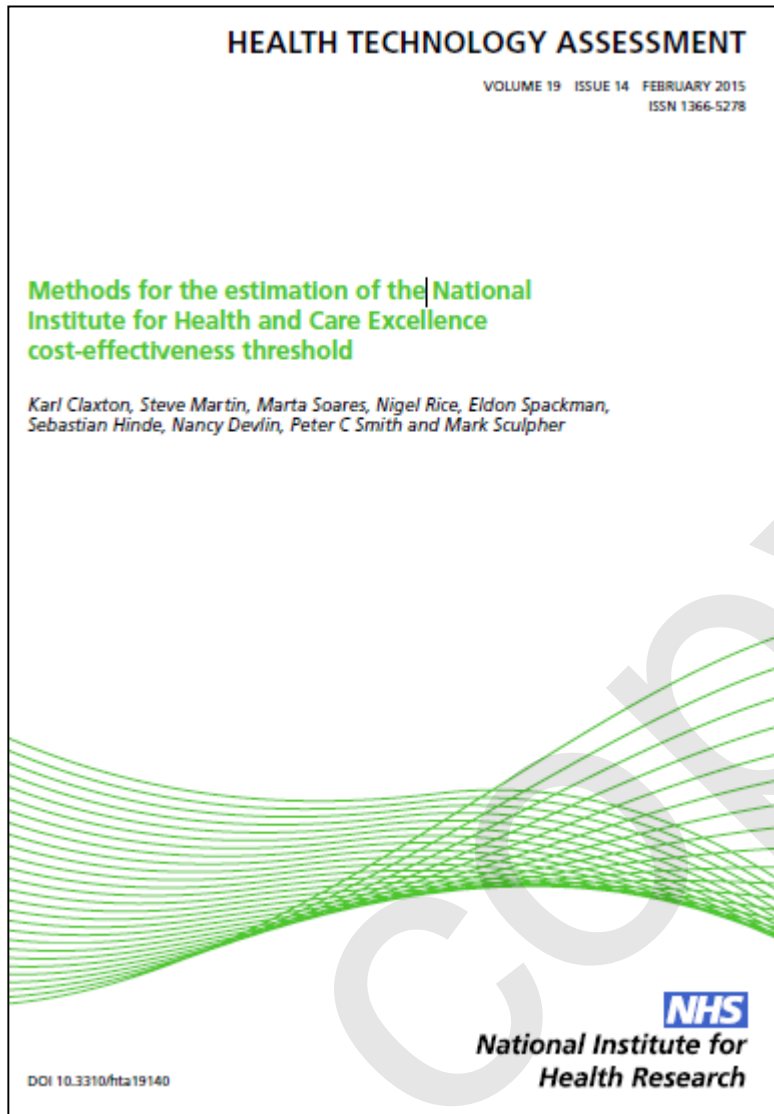
- What other new investments are possible?
- What health could have been generated?
- Direct investments vs leave to system

- 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



- 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  $\geq 400$  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

# 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

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

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

# 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	<ul style="list-style-type: none"><li>• Net health benefits = <math>3,225 - 6,184 = -2,959</math> QALYs</li></ul>
Net societal cost: ignore opportunity costs	<ul style="list-style-type: none"><li>• Net costs = <math>£80\text{m} - £85.2\text{m} = -£5.2\text{m}</math></li></ul>
Broader perspective: account for health and wider social benefits opportunity costs	<ul style="list-style-type: none"><li>• Net health loss = <math>-2,959</math> QALYs</li><li>• Net wider social benefits = <math>£85.2\text{m} - £49.8\text{m} = £35.4\text{m}</math></li><li>• Worthwhile if consumption value of health <math>&lt; £11,900</math> per QALY</li></ul>

# 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

Copyright

# 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':

$$\frac{Dc}{Dh} < k$$

Net health benefits:

$$Dh - \frac{Dc}{k} > 0$$

Net health benefits  
(consumption value of health):

$$v[Dh] - v\left[\frac{Dc}{k}\right] > 0$$

# 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
<b>Total</b>	<b>18 931</b>	<b>549 943 419</b>	<b>12 401</b>	<b>-5 933</b>	<b>-15 098</b>