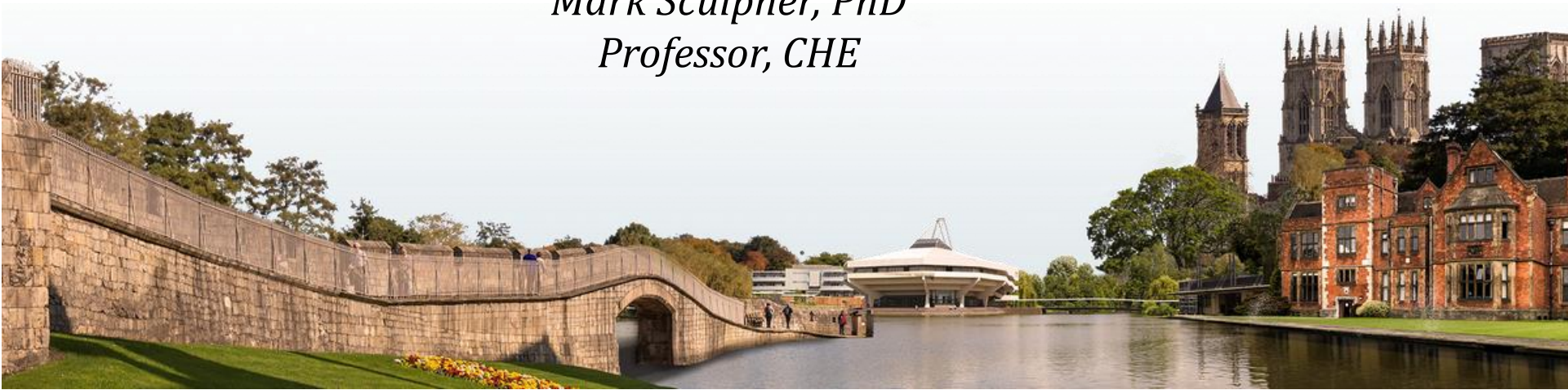
Online Advanced Methods for Cost-Effectiveness Analysis

Presentation 2: Planning and Conceptualising an Economic Evaluation
2.1: Overview and objectives

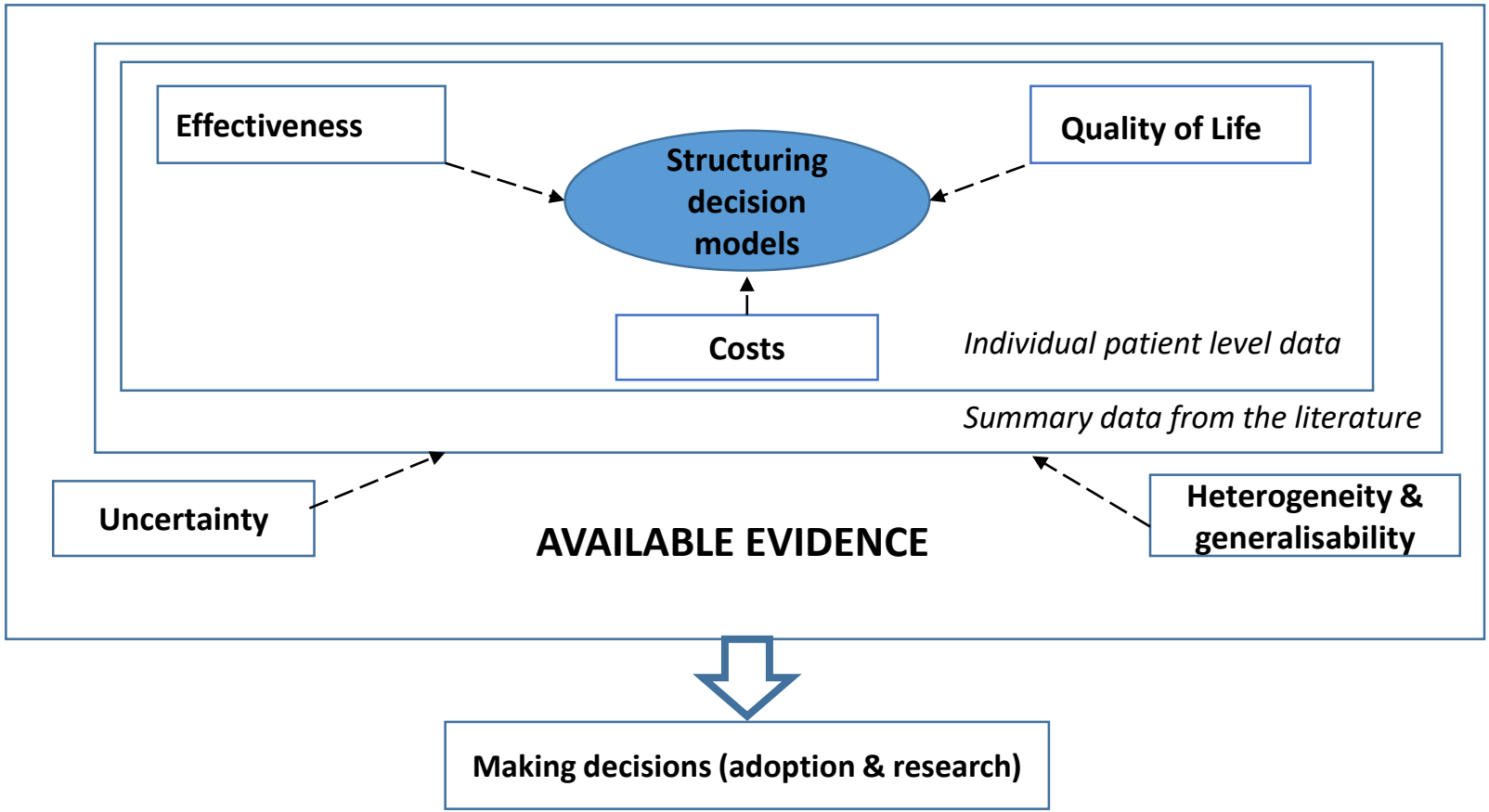
Mark Sculpher, PhD
Professor, CHE



Overview

- Laying the ground-work for an economic evaluation crucial
- Planning:
  - Defining the decision problem
  - Determining key methods choices
- Conceptualising:
  - How does a disease impact on health and resources?
  - How do available interventions influence that impact?

Course structure – where are we up to?



Sections

- Presentation 2.2 Planning and conceptualizing - planning
- Presentation 2.3 Planning and conceptualizing - methods
- Presentation 2.4 Planning and conceptualizing - conceptualization
- Presentation 2.5 Planning and conceptualizing - conclusions

Objectives

- Understand the key elements of a decision problem
- Consider the key methods for planning
- Appreciate the importance of conceptualisation of an analysis
- Understand the process of conceptualisation

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Presentation 2: Planning and Conceptualising an Economic Evaluation
2.2: Planning an analysis

Mark Sculpher, PhD
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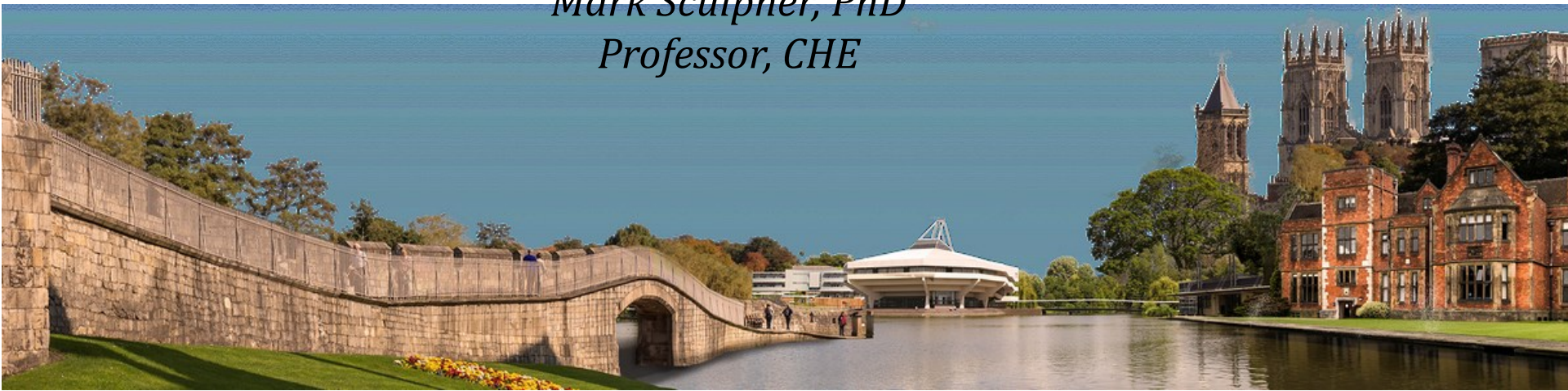


Objectives

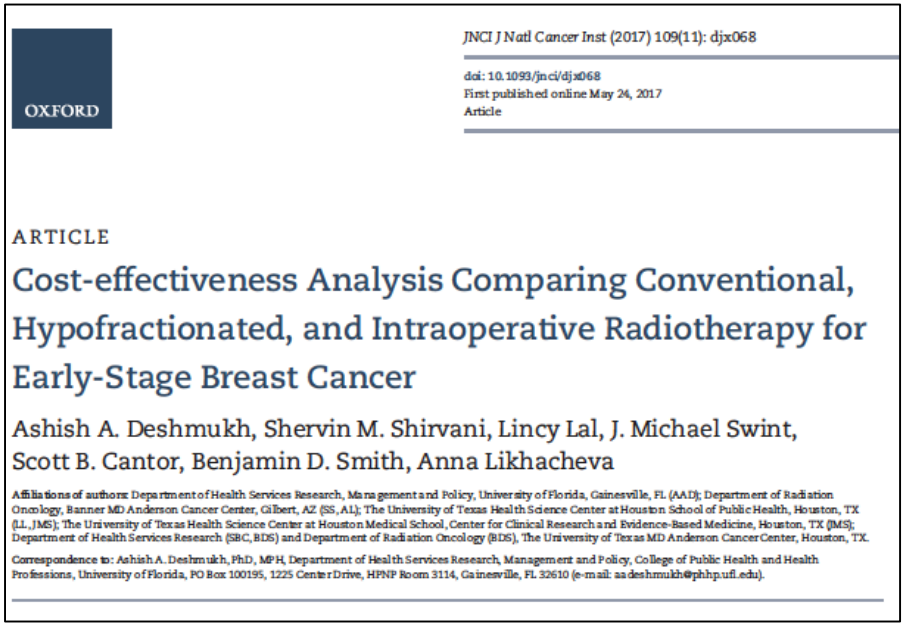
- Understand the key elements of a decision problem
- Appreciate the principles associated with selecting a study population
- Understand the challenges of defining sub-populations/sub-groups
- Determine the ways of identifying relevant options for comparison

Defining the decision problem

- Population and sub-populations
- The intervention of interest
- Comparators

} Options under comparison

Defining the relevant population



OXFORD

JNCI J Natl Cancer Inst (2017) 109(11): djx068

doi: 10.1093/jnci/djx068
First published online May 24, 2017
Article

ARTICLE

Cost-effectiveness Analysis Comparing Conventional, Hypofractionated, and Intraoperative Radiotherapy for Early-Stage Breast Cancer

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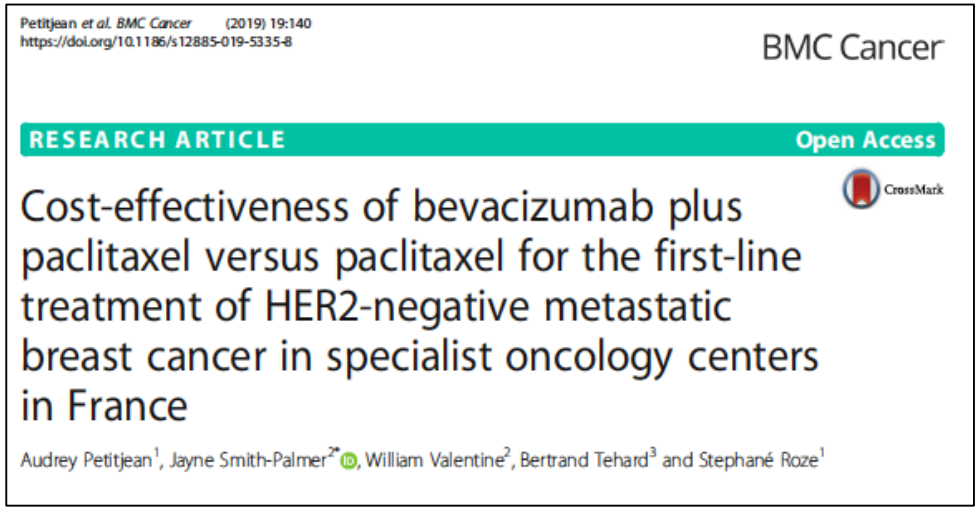


Petitjean et al. BMC Cancer (2019) 19:140
https://doi.org/10.1186/s12885-019-5335-8

BMC Cancer

RESEARCH ARTICLE Open Access

Cost-effectiveness of bevacizumab plus paclitaxel versus paclitaxel for the first-line treatment of HER2-negative metastatic breast cancer in specialist oncology centers in France

Audrey Petitjean[1], Jayne Smith-Palmer[2*], William Valentine[2], Bertrand Tehard[3] and Stephané Roze[1]

Defining the relevant sub-populations (sub-groups) (1)

EUROPEAN JOURNAL OF CANCER 47 (2011) 2517–2530

available at www.sciencedirect.com

ScienceDirect

journal homepage: www.ejconline.com

The cost-effectiveness of adjuvant chemotherapy for early breast cancer: A comparison of no chemotherapy and first, second, and third generation regimens for patients with differing prognoses ☆

H.E. Campbell [a,*], D. Epstein [b], D. Bloomfield [c], S. Griffin [b], A. Manca [b], J. Yarnold [d], J. Bliss [e], L. Johnson [e], H. Earl [f], C. Poole [g], L. Hiller [h], J. Dunn [h], P. Hopwood [e], P. Barrett-Lee [i], P. Ellis [j], D. Cameron [k], A.L. Harris [l], A.M. Gray [a], M.J. Sculpher [b]

Defining the relevant sub-populations (sub-groups) (2)

	ICER (Probability that strategy is cost-effective at £20,000 per QALY[a])			
	No chemotherapy	CMF chemotherapy	E-CMF/FEC60 chemotherapy	FEC-D chemotherapy
Reference-case results				
Average risk woman aged 40 years and ER negative[b]	–(0)	Dom (0)	£603 (0.28)	£13,704 (0.72)
Sub-group analyses				
Average risk altering age and ER status				
Average risk woman aged 60 years and ER negative[b]	–(0)	Dom (0)	£4,172 (0.46)	£18,550 (0.54)
Average risk woman aged 40 years and ER positive[b]	–(0)	Dom (0)	£1,730 (0.66)	£24,107 (0.34)
Average risk woman aged 60 years and ER positive[b]	–(0.23)	Dom (0)	£14,324 (0.74)	£45,918 (0.03)
High risk altering age				
High risk woman aged 40 years and ER negative[c]	–(0)	Dom (0)	£249 (0.12)	£8,770 (0.88)
High risk woman aged 60 years and ER negative[c]	–(0)	Dom (0)	£2,317 (0.21)	£11,195 (0.79)
Low risk altering age				
Low risk woman aged 40 years and ER positive[d]	–(0.01)	Dom (0.02)	£7,151 (0.97)	£70,116 (0)
Low risk woman aged 60 years and ER positive[d]	(1)	Dom (0)	Dom (0)	£539,470 (0)

Campbell *et al, European Journal of Cancer*, 2011; 47: 2517-30

Selecting populations

- Select based on the nature of the decision
- “What is the best way of treating patients with disease X?”
  - Relevant to many guideline decisions
- “Is intervention Y worth funding?”
  - Many ‘HTA’/reimbursement decisions
  - How is the population defined?
- Relevant populations defined by product licences
  - Drives many decisions
  - Focus may be a sub-group
- How is an intervention used/expected to be used in practice?
  - Some drug licenses very broad (e.g. antibiotics)
  - Some technologies can be used in many different ways (e.g. diagnostics)
- Some interventions relate to broad and heterogenous populations
  - Public health
  - Health system changes

Population driven by the product licences

Rheumatology 2007;46:1729–1735 doi:10.1093/rheumatology/kem221

The cost-effectiveness of etanercept and infliximab for the treatment of patients with psoriatic arthritis

Y. Bravo Vergel, N. S. Hawkins, K. Claxton, C. Asseburg, S. Palmer, N. Woolacott[1], I. N. Bruce[2] and M. J. Sculpher

Objective. Tumour necrosis factor (TNF) antagonists have been shown to improve the outcomes in patients with rheumatoid arthritis (RA) and psoriatic arthritis (PsA). We assess the cost-effectiveness of two TNF antagonists and so-called 'palliative care' for the treatment of active PsA from the perspective of the UK National Health Service (NHS).

Methods. Bayesian statistical methods were used to synthesize evidence from three Phase III trials, identified through a systematic review, and estimate the relative efficacy of etanercept, infliximab and palliative care. A probabilistic decision analytic model was then used to compare these treatments after the failure of at least two conventional disease-modifying anti-rheumatic drugs (DMARDs), following the British Society for Rheumatology (BSR) guidelines for use. The primary outcome measure, quality-adjusted life years (QALYs), was derived from utility values estimated as a function of disability measured by the Health Assessment Questionnaire (HAQ). The deterioration experienced in HAQ at treatment withdrawal (rebound) was incorporated using alternative scenarios to represent best- and worst-case assumptions. The model was extended beyond the trial duration to a 10-yr and lifetime horizon, using available evidence and expert opinion-based assumptions on disease progression. Resource utilization was based on literature, national databases and expert opinion. Prices were obtained from routine NHS sources and published literature.

Results. At a 10-yr time horizon, the incremental cost-effectiveness ratio (ICER) for etanercept compared with palliative care was £26 361 per QALY gained for the best-case rebound scenario, which increased to £30 628 for the worst-case. The ICERs for infliximab compared with etanercept were £165 363 and £205 345 per QALY, respectively. These findings are mainly explained by the fact that infliximab has higher acquisition and administration costs without substantially superior effectiveness compared with etanercept. Results were sensitive to estimates of rebound assumptions at withdrawal and the time horizon.

Conclusions. Only results for etanercept remained within the range of cost-effectiveness estimates considered to represent value for money in the NHS by the National Institute for Health and Clinical Excellence. Further research appears most valuable in relation to the short-term effectiveness, utility parameters and assumptions regarding the effect of rebound.

Key words: Cost-effectiveness, Etanercept, Infliximab, Psoriatic arthritis, Bayesian evidence synthesis

Selecting sub-populations (sub-groups)

- Rationale: cost-effectiveness can vary by different types of patient
- Includes (but broader than) clinical sub-group analysis
- Ideally identified in advance of analysis (pre-specified)
- Post-hoc analyses cannot be ruled out to inform decisions

The ‘intervention’

Intervention(s)	Donepezil and galantamine
Population(s)	People with vascular (multi-infarct) dementia, dementia with Lewy bodies, including Parkinson’s dementia, or any other non-Alzheimer dementia for which there is robust clinical evidence of efficacy. People with mixed dementia whose predominant dementia is considered to be non-AD.
Current standard treatments (comparators)	• Pharmacological (e.g. aspirin or a hypertensive drug) • Management without donepezil or galantamine
Other considerations	Outcomes include: • Health-related quality of life of patients and carers (analyses should be carried out separately for patients alone, and for patients and carers combined) • Ability to remain independent • Likelihood of admission to residential/nursing care • Survival • Long-term management of patients with

National Institute for Clinical Excellence
Invitation to participate in the appraisal of
new pharmaceutical treatments for non-Alzheimer dementias
Issue date: January 2004

Page 2 of 3

- Defined by a reimbursement process
- Defined by research funder
- Defined by researcher

Selecting comparators (options)
The principles

All other mutually exclusive ways that the population group could be managed

- May include ‘do nothing’
- Could include strategies (e.g. combination of options)
- May add to complexity

Selecting comparators (options) (1)
Planning questions

Planning question	Issues	Guidance
Can we identify options from the available RCTs?	• RCTs may not include all interventions used in practice • RCTs may relate to other jurisdictions	Do not base comparator section on what's been compared in RCTs
What can the literature tell us about option selection?	• Good and recent overviews may be helpful to get a list of possible options	Literature is helpful but cannot be the sole basis of option selection
Can we use clinical/specialist opinion?	• Helpful if this is broad and representative of jurisdiction	Always use suitable expert opinion
What can be learned from licenses, guidelines and funding decisions?	• Licenses only relate to certain interventions and are only a guide • Guidelines may not be current and may not be mandatory? • Are funding decisions mandatory and do they cover all types of interventions?	Official documents and guidelines are a useful source of information

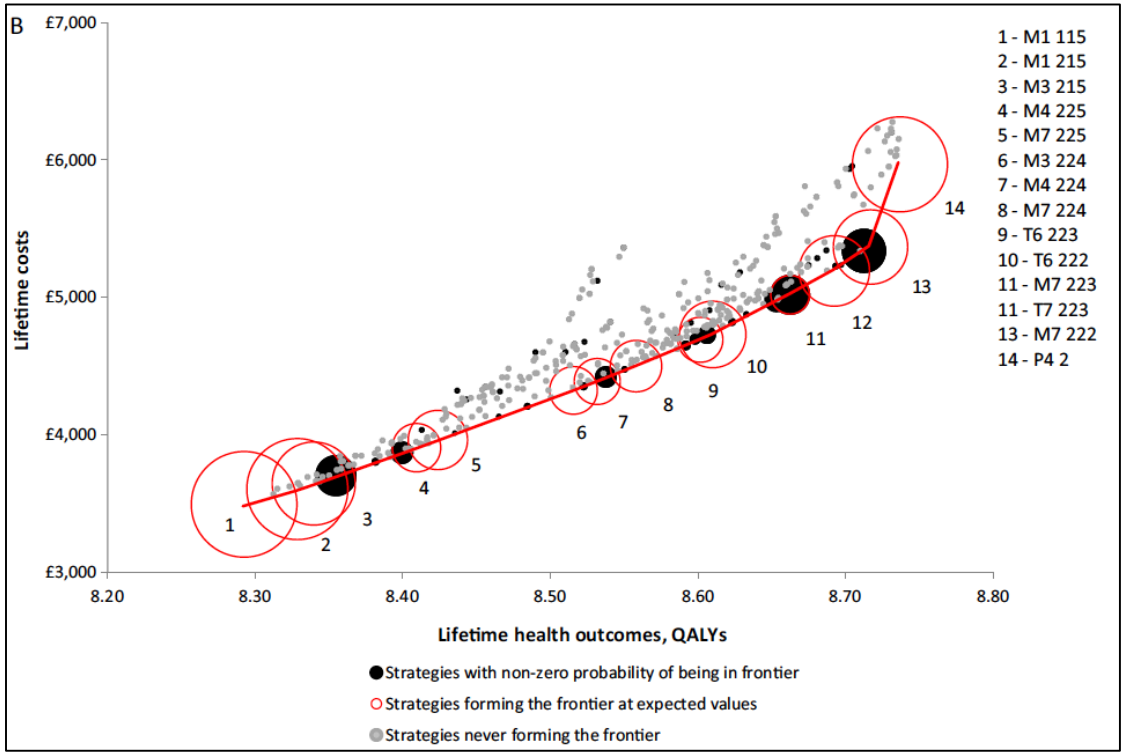
Selecting comparators (options) (2)
Planning questions

Planning question	Issues	Guidance
Is their scope for the analyst to include (as-yet) unused options?	• Different ways of using options may be revealed through analysis • These become additional options • Examples: stopping rules, repeat testing	Always need to consider different management strategies for a given set of 'core' options
How should treatment or test sequences be used?	• Each potential sequence is an option but may get complex	Sequence comparison is fundamental to some evaluations
How should comparisons of numerous options be handled?	• Sometimes options can get into the thousands + • Some may not be feasible • Can simplify by asking clinicians what would be used/are used • Need to beware of missing cost-effective options	Using clinical or other expert opinion to 'rule out' needs to be used cautiously Use flexible modelling and analysis Attempt to identify options with very low chance of being cost-effective

Managing complexity in options
Example of diagnostics and prostate cancer

Test	Strategies
MPMRI	
First test	M1–M7; N1–N7
Second test after TRUSB	T5–T9; P5–P9
TRUSB	
First test	T1–T9; P2–P9
Repeat TRUSB in men with no cancer detected	T2, T4
Repeat TRUSB in men with non-CS cancer detected	T3, T4
Second test after MPMRI: MRI-targeted TRUSB, in men with lesions visible at the MPMRI	M1–M7
Repeat MRI-targeted TRUSB in men with no previous cancer or non-CS cancer at first MRI-targeted TRUSB, but with lesions visible at MRI	M3–M7; T5–T9; N3–N7
TPMB	
First test	P1
Second test	P2–P4; N1–N4
Third test	P5–P9; N3–N7

MPMRI = multiparametric magnetic resonance imaging; TRUSB = transrectal ultrasound-guided biopsy; TPMB = template prostate mapping biopsy; CS = clinically significant. MRI-targeted TRUSB is a TRUSB informed by a prior MPMRI. All TRUSB post-MPMRI are assumed to be MRI-targeted TRUSB. Diagnostic strategies were labelled according to their test combination first (M1–M7, N1–N7, T1–T9, P1–P9), and then their biopsy TRUSB definition (1 or 2), MPMRI definition (1 or 2), and cut-off (2 to 5). T strategies start with TRUSB, M strategies start with MPMRI, P strategies are the same as T strategies, and N strategies are the same as M strategies but have TPMB as the last biopsy. For example, strategy M1 125 refers to test combination M1, in which all men were first assessed using MPMRI definition 2 and cut-off 5 and then followed up with biopsy definition 1 for those with a suspicion of CS cancer. See the Supplementary material, section 1, for full details on the test sequences for each diagnostic strategy.



Faria *et al*. *European Urology*, 2017; 73: 23-30

Summary

- Defining a decision problem focusses on the population, sub-populations and options
- Range of ways to determine the appropriate population
- Sub-populations (sub-group) ideally identified in advance of analysis
- The concept of a primary intervention and comparators is not always relevant: often simply comparing a set of options
- Identifying all relevant options can be challenging and may lead to complexity

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Presentation 2: Planning and Conceptualising an Economic Evaluation
2.3: Methods

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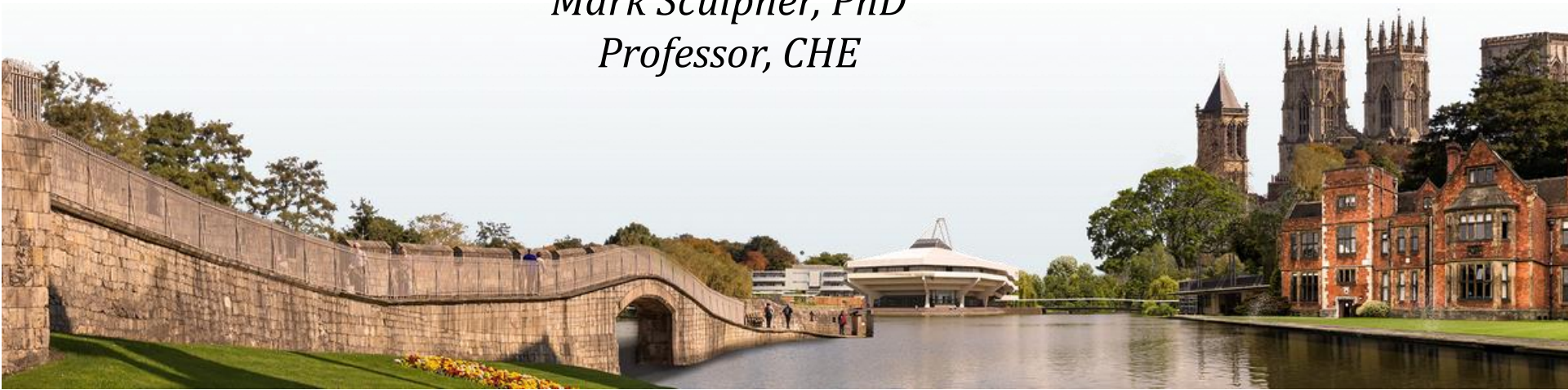
Professor, CHE



Objectives

- Understand the core principles and methods to be employed
- Appreciate the choices to be made regarding perspective
- Differentiate health and non-health outcomes

Core principles

Appropriate specification of decision problem	• See Presentation 2.2
Reflect all evidence	• No selective use of evidence • Clear justification for choices • Replicable if not comprehensive
Select time horizon over which costs and benefits could differ between options	• Will be lifetime if mortality differs • Decision makers may consider shorter period, but implications should be reflected
Capture uncertainty and relate to decision	• Analysis to show likelihood of an option being cost-effective • Consider all sources of uncertainty
Reflect heterogeneity	• Sub-group economic analysis • Failure to reflect will reduce outcomes

Different views on perspective

Theoretical societal perspective

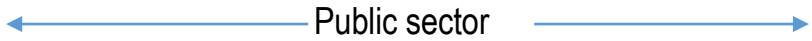
- Linked to ‘textbook’ cost-benefit analysis
- Budgets reflect social preferences that can be measured
- Hence budgets are ‘efficient’
- Budgets are flexible
- Outcomes should reflect social preferences
- See: Jonsson B. *European Journal of Health Economics*. 2009;10:357–9.

A practical societal perspective

- Budgets are set based on a range of political considerations
- Budgets do not have a measurable relationship with social preferences
- Budgets are slow to adjust
- Outcomes should reflect decision makers’ responsibilities
- See: Walker *et al. Applied Health Economics and Health Policy*. 2019 17:577–90.

Note: budgets may be interpreted as ‘funding rules’ in some systems (e.g. USA)

The knotty issue of perspective and outcomes



Health care

Education

Criminal justice

Environmental

Wider production and consumption

Largely confined to health system
- New cancer treatments
- Other low incidence treatments
- Diagnostics related to such treatments

Budgets set independently of other sectors

Opportunity costs reflect budget constraints and costs and effects of interventions

Relevant outcomes health focused

Important effects in wider public sector
- Public health programs (e.g. free school meals)
- Interventions for addictions

Budgets set independently of other sectors

Opportunity costs reflect budget constraints and costs and effects of interventions

Complex and varied objectives

Effects extending to wider economy
- High incidence treatments (for young)
- Policies to limit pandemics
- Air pollution amelioration

Patients' out of pocket costs

Effects on formal and informal productivity

Changes in health affect production & consumption

The analyst's decision on perspective

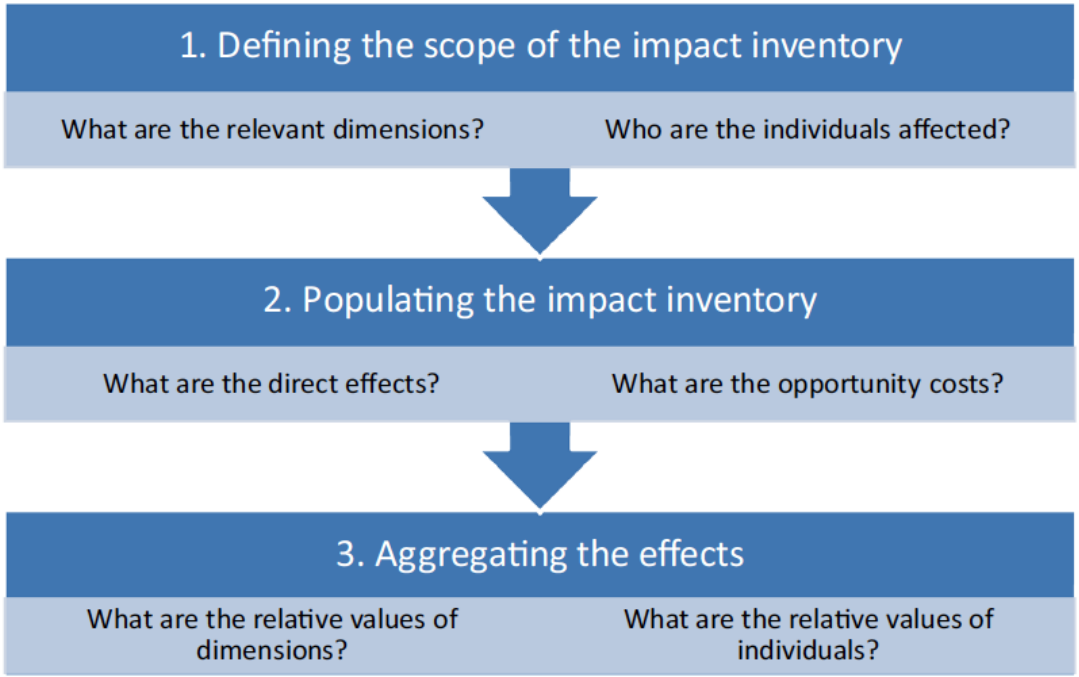
Analysis for a specified decision maker

- Likely to be a preferred perspective
- Primary (reference) analysis follows preference
- Try to give some focus to other costs and effects
- Qualitative vs quantitative vs aggregated
- Peer review should require this

Analysis without a specified decision maker

- Set out to capture all important effects
- Qualitative vs quantitative vs aggregated
- Primary analysis may still be from narrow perspective
- Strive for fuller aggregated analysis

The impact inventory



Walker *et al. Applied Health Economics and Health Policy*. 2019 17:577–90

Outcomes outside health

- Some project will have potentially significant effects outside health care
- Other parts of public sector (e.g. education, criminal justice)
  - Less work on a composite, generic outcome
  - Have to identify with decision-makers
- Wider economy
  - Quantify productivity and consumption effects

Summary

- Core principles apply to all studies
  - Recognize constraints on time and people
- Main methods selection issues relate to perspective and outcomes
- Many studies are constrained by funder, decision maker etc.
- Analyst should seek to reflect all important costs and effects
  - At least qualitatively
- Health is inevitably the focus of relevant outcomes in health care
  - Trade-offs contested
- Outside health care, less definition of outcomes
  - Needs close collaboration with relevant decision-makers

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Presentation 2: Planning and Conceptualising an Economic Evaluation
2.4: Conceptualisation

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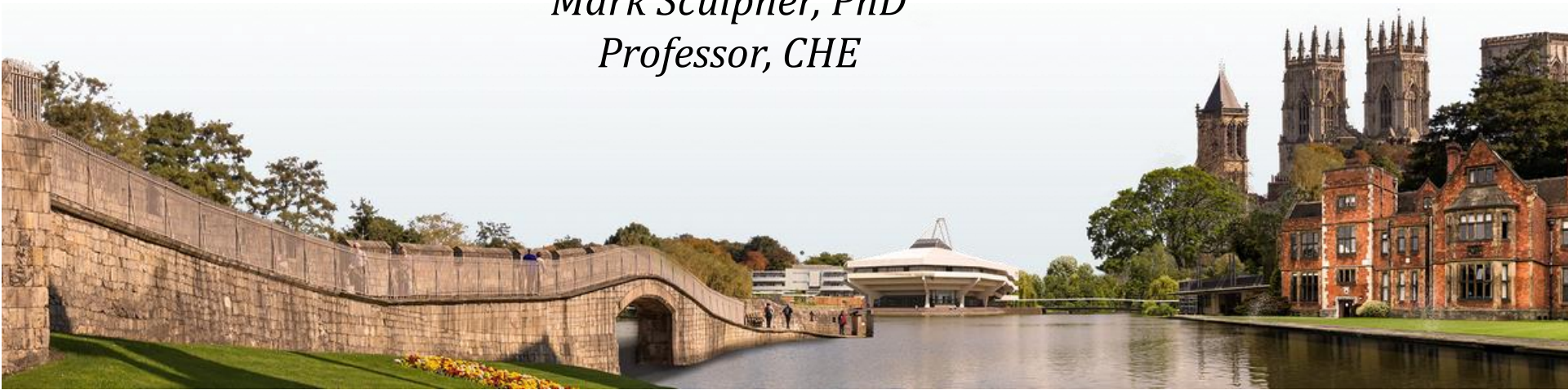


Objectives

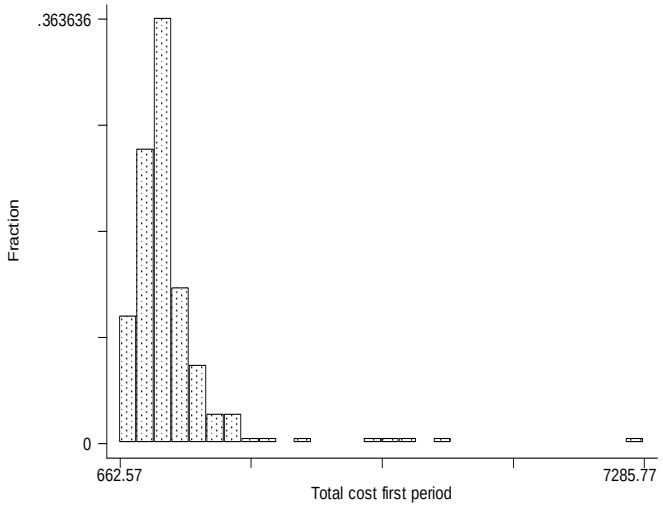
- Understand models and trials as vehicles for analysis
- Appreciate the importance of models
- Consider key concepts in conceptualisation
- Gain insights on conceptualisation with a case study

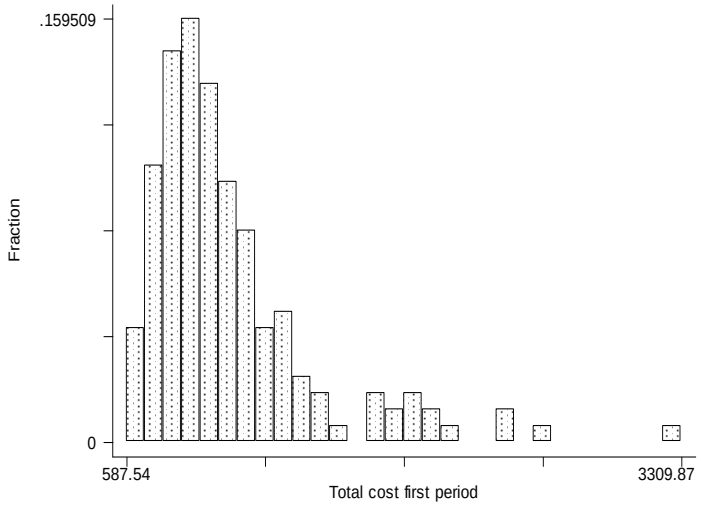
Vehicles for economic evaluation

- Trial-based
- Model-based

Trial-based economic evaluation: EVALUATE trial - costs

	Abdominal	Laparoscopic
Cost (6 weeks)		
mean (SD)	1,286 (611)	1,561 (1,107)
median	1,167	1,290
range	(662 – 7,286)	(587 – 3,309)





Sculpher *et al. BMJ* 2004; 328: 134.

Trial-based economic evaluation: EVALUATE Trial - QALYs

	Lap-assisted		Standard
Mean weights			
baseline	0.746		0.758
6 weeks	0.875		0.852
4 months	0.911		0.918
12 months	0.920		0.917
Mean QALYs over 1 year	0.899		0.897
Difference in QALYs*		0.0015	
(95% confidence interval)		(-0.015,0.018)	

* (Lap - standard)

Sculpher *et al. BMJ* 2004; 328: 134.

Trials, decisions and decision analysis

Needs of decisions	Decision modelling	Focus of clinical trials
Absolute change in net health benefit	Surrogacy	Relative effects in clinical outcomes
Consider full range of options	Synthesis	Subset of options (focus on the new)
Over relevant time-horizon	Extrapolation	Time until a clinically-relevant effect
Sub-groups with net health benefit	Heterogeneity	Positive average effect

Trials versus models

- To support decisions, models are generally necessary
- Trials remain key source of evidence for models
- Trial-based studies may be suitable:
  - Time horizon and follow-up the same
  - All options included
  - Trial and decision population the same
- Some jurisdictions support RCT-based studies
- Methods RCT-based analysis

What is conceptualization?

- Sometimes called ‘model conceptualization’ or developing a ‘conceptual model’
- Precedes decisions about type or structure of model (e.g. Markov, decision tree)
- How do we understand the impact of a disease on patients’ health and costs?
  - Over time
  - Using no intervention or standard of care (‘natural history’)
  - How does this vary between patients?
- How do we understand the effects of different options on that disease
  - Over time
  - Based on partial evidence
  - How does this vary between patients?
  - Intended and unintended effects

Common issues with conceptualization

Characterizing disease - events

- Distinct and rapid changes in clinical status
- Examples: stroke, exacerbations, death
- Can impact current health (QoL)
- Can impact costs
- Can impact future health (prognostic)
- Event likelihoods expressed as rates (which determine probabilities)
- Interventions can change event rates (treatment, prevention)

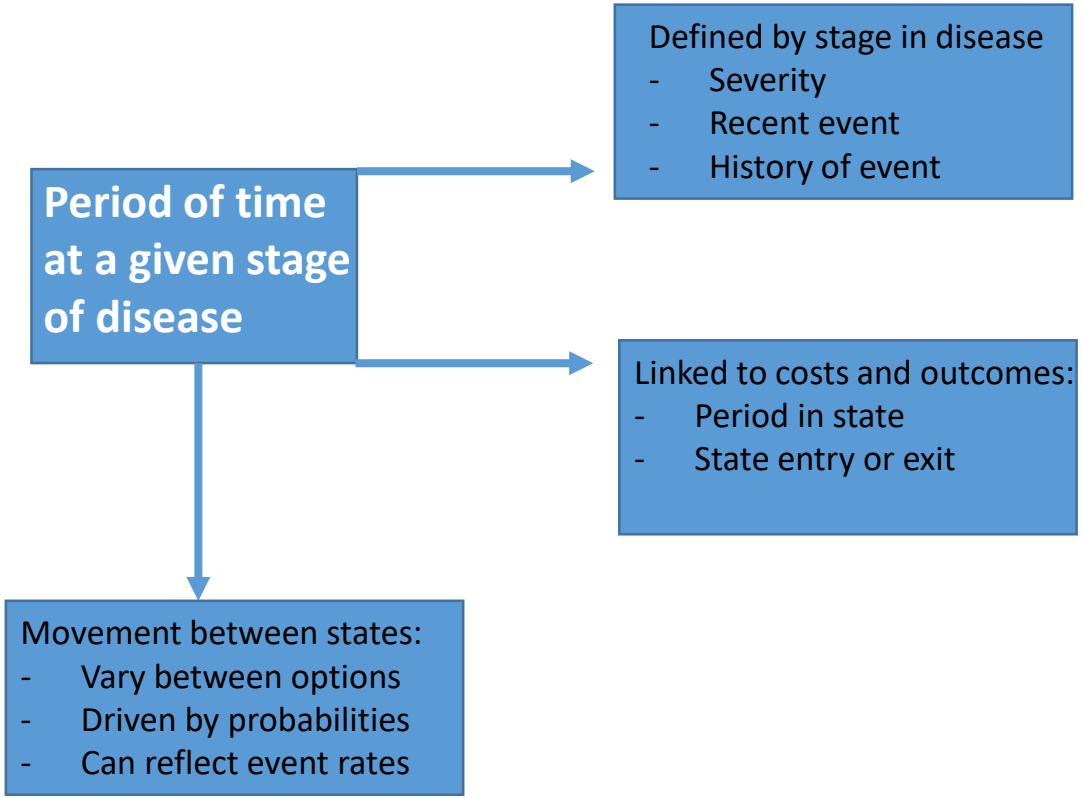
Common issues with conceptualization

Characterizing disease – continuous measures

- Clinical status reflected on a scale
- Examples:
  - Expanded Disability Status Scale (EDSS) in multiple sclerosis
  - Psoriasis Area and Severity Index (PASI)
- Change in measure can affect costs, health and prognosis
- Interventions can change level measure

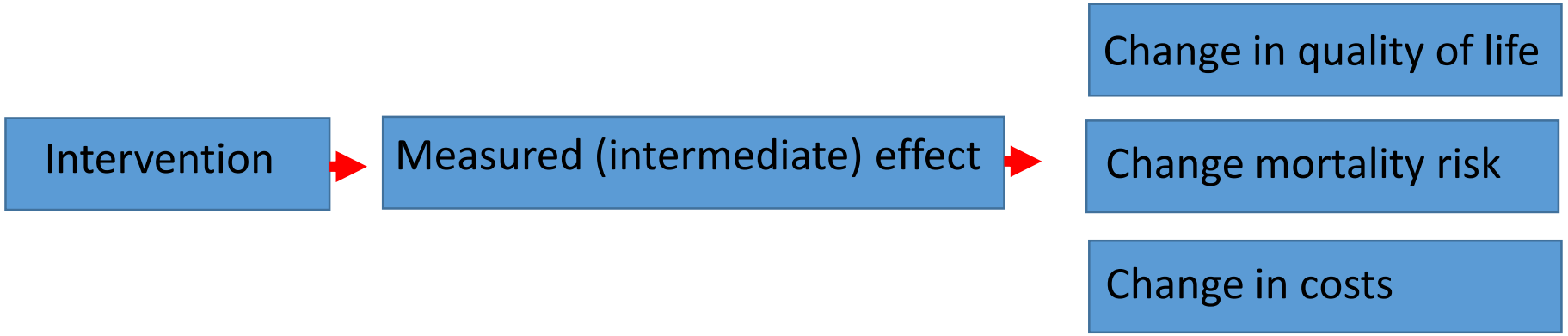
Common issues with conceptualization

Characterizing disease - disease states



Common issues with conceptualization

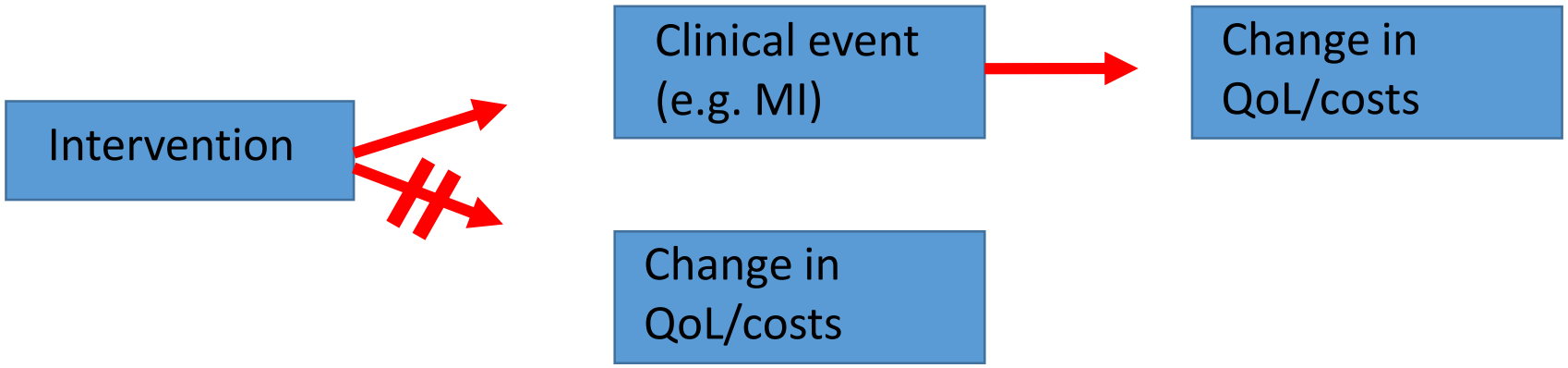
Intermediate effects



- Surrogate = a good intermediate effect
- Examples:
  - FEV1 in asthma
  - Cancer recurrence

Common issues with conceptualization

Conditional independence



Conceptualisation– case study

Ivabradine and outcomes in chronic heart failure (SHIFT): a randomised placebo-controlled study

*Karl Swedberg, Michel Komajda, Michael Böhm, Jeffrey S Borer, Ian Ford, Ariane Dubost-Brama, Guy Lerebours, Luigi Tavazzi, on behalf of the SHIFT Investigators**

Summary

Background Chronic heart failure is associated with high mortality and morbidity. Raised resting heart rate is a risk factor for adverse outcomes. We aimed to assess the effect of heart-rate reduction by the selective sinus-node inhibitor ivabradine on outcomes in heart failure.

Methods Patients were eligible for participation in this randomised, double-blind, placebo-controlled, parallel-group study if they had symptomatic heart failure and a left-ventricular ejection fraction of 35% or lower, were in sinus rhythm with heart rate 70 beats per min or higher, had been admitted to hospital for heart failure within the previous year, and were on stable background treatment including a β blocker if tolerated. Patients were randomly assigned by computer-generated allocation schedule to ivabradine titrated to a maximum of 7·5 mg twice daily or matching placebo. Patients and investigators were masked to treatment allocation. The primary endpoint was the composite of cardiovascular death or hospital admission for worsening heart failure. Analysis was by intention to treat. This trial is registered, number ISRCTN70429960.

Findings 6558 patients were randomly assigned to treatment groups (3268 ivabradine, 3290 placebo). Data were available for analysis for 3241 patients in the ivabradine group and 3264 patients allocated placebo. Median follow-up was 22·9 (IQR 18–28) months. 793 (24%) patients in the ivabradine group and 937 (29%) of those taking placebo had a primary endpoint event (HR 0·82, 95% CI 0·75–0·90, p<0·0001). The effects were driven mainly by hospital admissions for worsening heart failure (672 [21%] placebo *vs* 514 [16%] ivabradine; HR 0·74, 0·66–0·83; p<0·0001) and deaths due to heart failure (151 [5%] *vs* 113 [3%]; HR 0·74, 0·58–0·94, p=0·014). Fewer serious adverse events occurred in the ivabradine group (3388 events) than in the placebo group (3847; p=0·025). 150 (5%) of ivabradine patients had symptomatic bradycardia compared with 32 (1%) of the placebo group (p<0·0001). Visual side-effects (phosphenes) were reported by 89 (3%) of patients on ivabradine and 17 (1%) on placebo (p<0·0001).

Interpretation Our results support the importance of heart-rate reduction with ivabradine for improvement of clinical outcomes in heart failure and confirm the important role of heart rate in the pathophysiology of this disorder.

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Ivabradine – key conceptual considerations

Intervention for chronic disease	→	Duration of treatment effect Long-term time horizon
Cardiovascular	→	Possible heterogeneity in baseline risk
Outcomes trial measuring events	→	Unlikely need for surrogates Event driven costs and QoL effects
New therapy on top of ‘standard practice’	→	Limited scope for other comparators
Potential mortality effect	→	Long-term extrapolation
Composite endpoint	→	Likely need to separate out effects

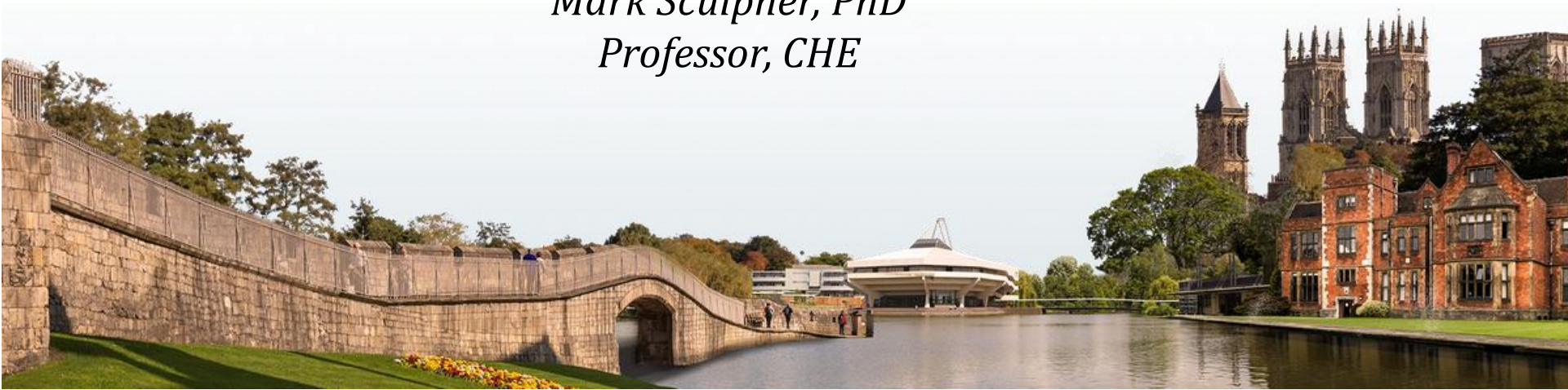
Summary

- RCTs (or other primary studies) can be appropriate vehicles for economic evaluation
- But decision models are more often appropriate to reflect the needs of decision making
- Conceptualisation is a key stage before selecting model type or structure
- Relates to how disease is characterized; key role for:
  - Events, scales and states
  - Considering changes over time

Online Advanced Methods for Cost-Effectiveness Analysis

Presentation 2: Planning and Conceptualising an Economic Evaluation
2.5: Conclusions

Mark Sculpher, PhD
Professor, CHE



Conclusions

- Defining the decision problem is a key element of planning
  - Population
  - Sub-populations
  - Options
- Many (core) key principles apply in all studies
  - Although inevitable variation in time and resources available
- Perspective and outcomes often require judgements
  - Need to balance ‘customers’ expressed needs against reflecting all major effects
- To support decisions, decision models often the main vehicle for economic evaluation
- Need to think about how a disease influences health and costs and how interventions change that impact

Further reading

Vehicles for economic evaluation

- Sculpher MJ, *et al.* Whither trial-based economic evaluation for health care decision making? *Health Economics.* 2006; 15: 677-687.

Perspective

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Conceptualisation

- Tappenden. *Conceptual Modelling For Health Economic Model Development.* HEDS Discussion Paper no. 12.05, 2012. http://eprints.whiterose.ac.uk/74464
- Roberts M, Russell LB, Paltiel AD *et al.* Conceptualizing a model: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-2. *Value in Health* 2012, 15:804-11