The current theory and practice of cost-effectiveness thresholds and the constraints and principles that might guide new decision rules for healthcare resource

UCL, 15th December 2014

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Overview

- Motivation for considering thresholds
- Methods for setting thresholds
- Thresholds in practice
- Theory of the budget exhaustion threshold
- Real world constraints on threshold design
- NICE’s proposed changes and consistency

Thresholds as Evidence

- The threshold provides a simple and transparent decision rule
- CEA aspires to be evidenced-based decision making
- Evidenced-based ICERs are only half the necessary evidence
- If we don't have appropriately empirically-informed thresholds we don't have evidenced-based policy recommendations

Thresholds and Ethics

- CEA involves difficult ethical choices
- Appropriate thresholds are fundamental to the ethical justification for CEA
- Popular concern is typically with *restrictive* thresholds and the implied rationing
- We need to be aware of the ethical problems of *over-generous* thresholds
  - Analogous concerns regarding investment incentives

Ways to Determine Thresholds

- Historical precedent
- Macroeconomic variables: GDP/capita
- Willingness to pay studies
- Ranking and budget exhaustion
  - All have problems

Ways to Determine Thresholds

- Historical precedent
  - Renal dialysis cited for US $50k [Ubel et al.]
  - Without theoretical foundation
  - No reason to assume efficient ($700k smear)
  - No way to reasonably update threshold

- Indication-specific precedent (IQWiG)

Ways to Determine Thresholds

- Macroeconomic variables: World Health Organisation's 1x 3x GDP per capita thresholds [Newall et al.]
  - Proposed for DALYs in developing world context
  - Widely cited in absence of explicit thresholds
  - Without theoretical foundation
  - No reason to assume efficient

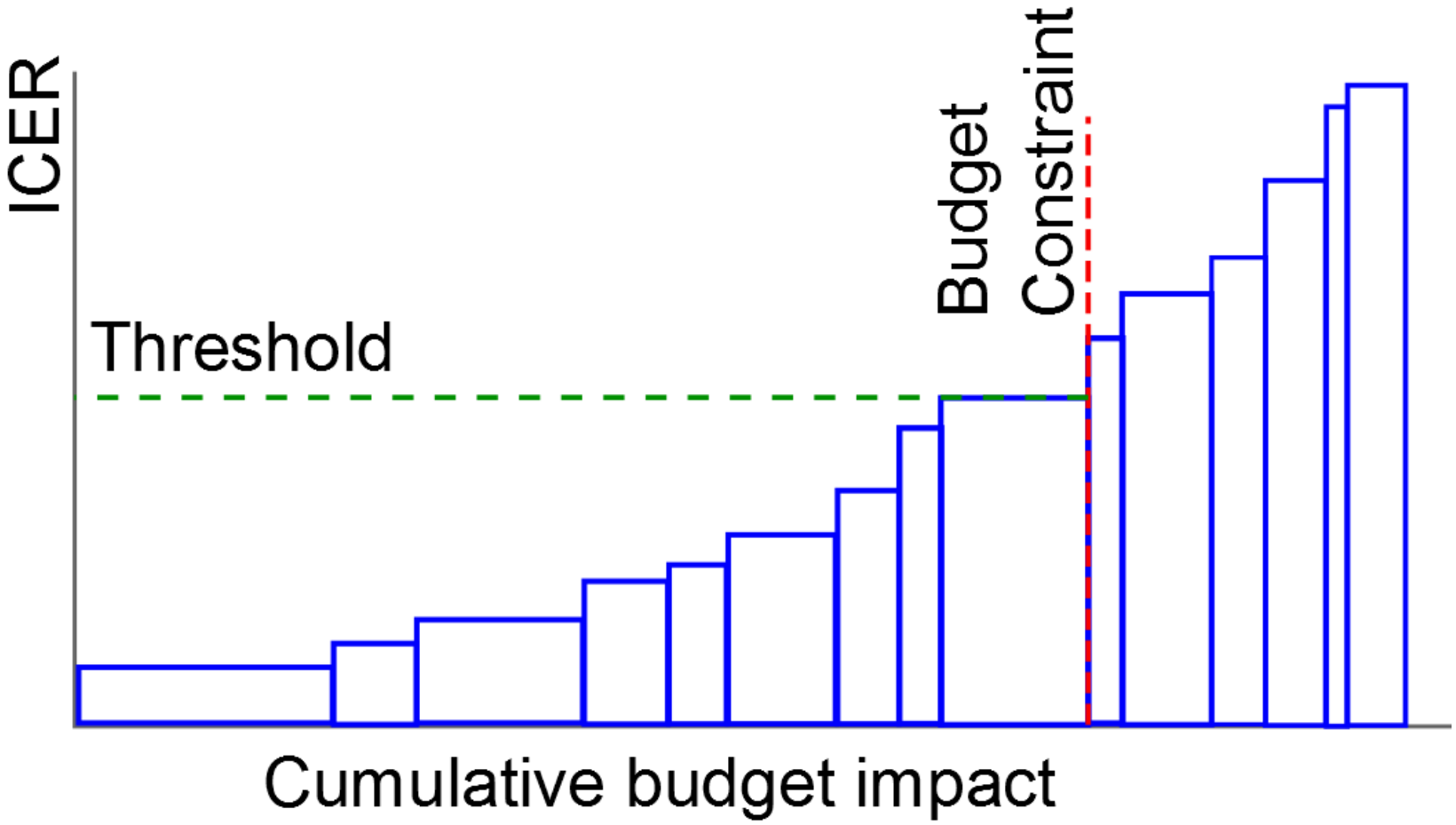
Ways to Determine Thresholds

- Willingness to pay: revealed or stated preference [Hirth et al]
  - Elicitation issues
  - WTP vs WTA asymmetries
  - Not necessarily directly reflective of WTP: market imperfections, heuristics, lives vs QALYs
  - Unrelated to costs of health provision
  - Potentially at odds with budgets setting processes

Budget Exhaustion Threshold

- Rank all interventions by cost-effectiveness until budget is exhausted [Weinstein & Zeckhauser]
- ICER of the last intervention adopted = threshold
- Broadly the approach suggested as suitable for NICE [Culyer et al]

Budget Exhaustion Threshold



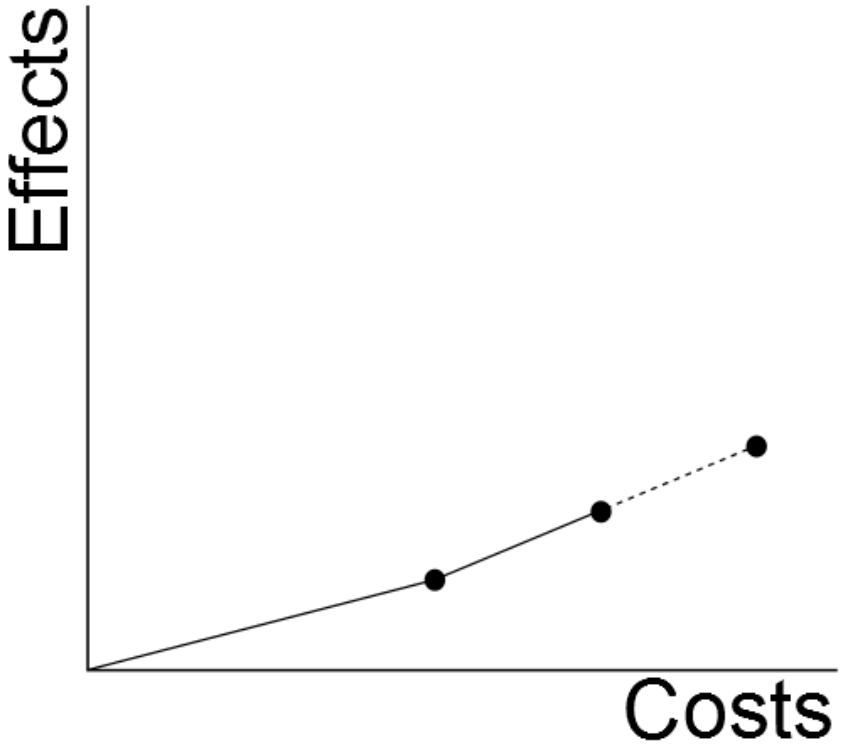
Thresholds in Practice: NL

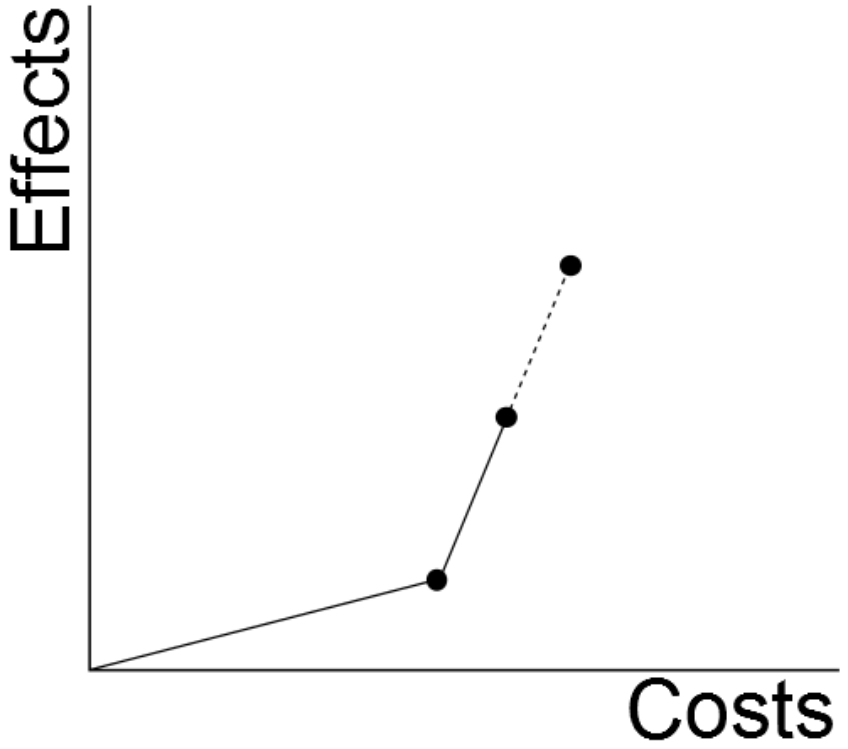
- Netherlands: Not explicit [Boersma]
  - Dual threshold of €20,000 and €80,000 depending on severity
  - Sometimes interpreted as:
    - €20,000 for preventative interventions
    - €80,000 for therapeutic interventions
  - Dual thresholds give rise to inefficiencies and potentially irrational allocations

Thresholds in Practice: Ireland

- Initially unofficial €45,000
- c. 2009 revised down to €20,000, still unofficial
- Nov 2012 Industry-govt. deal makes threshold explicit at €45,000 [IPHA DOE HSE], but:
  - Only applies to pharmaceuticals
  - Not binding, so serves as price floor not ceiling
  - No explicit upper bound

Thresholds in Practice: Germany [Sculpher et al]





Thresholds in Practice: UK

- Explicit threshold range of £20,000-30,000 [NICE]

- Expansion through range depends on:
  - Certainty of the ICER
  - Inadequate capture of health related quality of life
  - Innovative nature of the technology
  - Non-health objectives of the NHS

NICE Flexibility

- NICE keen to stress lack of rigid adherence to threshold [Rawlins et al], including wrt:
  - Disease severity
  - End of life
  - Stakeholder persuasion
  - Innovation
  - Disadvantaged populations
  - Children

Threshold range: good or bad?

- NICE operates a threshold range with an upper bound
- Explicitly cites factors that permit ICERs towards upper bound
- This allows NICE to exercise a degree of discretion
- Threshold range large relative to threshold itself

Threshold range: good or bad?

- Discretion implies we implicitly quantify what we did not explicitly quantify
  - Appears contrary to purpose of CEA
  - Discretion probably is a political necessity
- Concern that discretion will only be exercised in favour of identified candidate intervention
  - ie, discretion is only applied one way
- Upper bound does at least cap the “wiggle room”

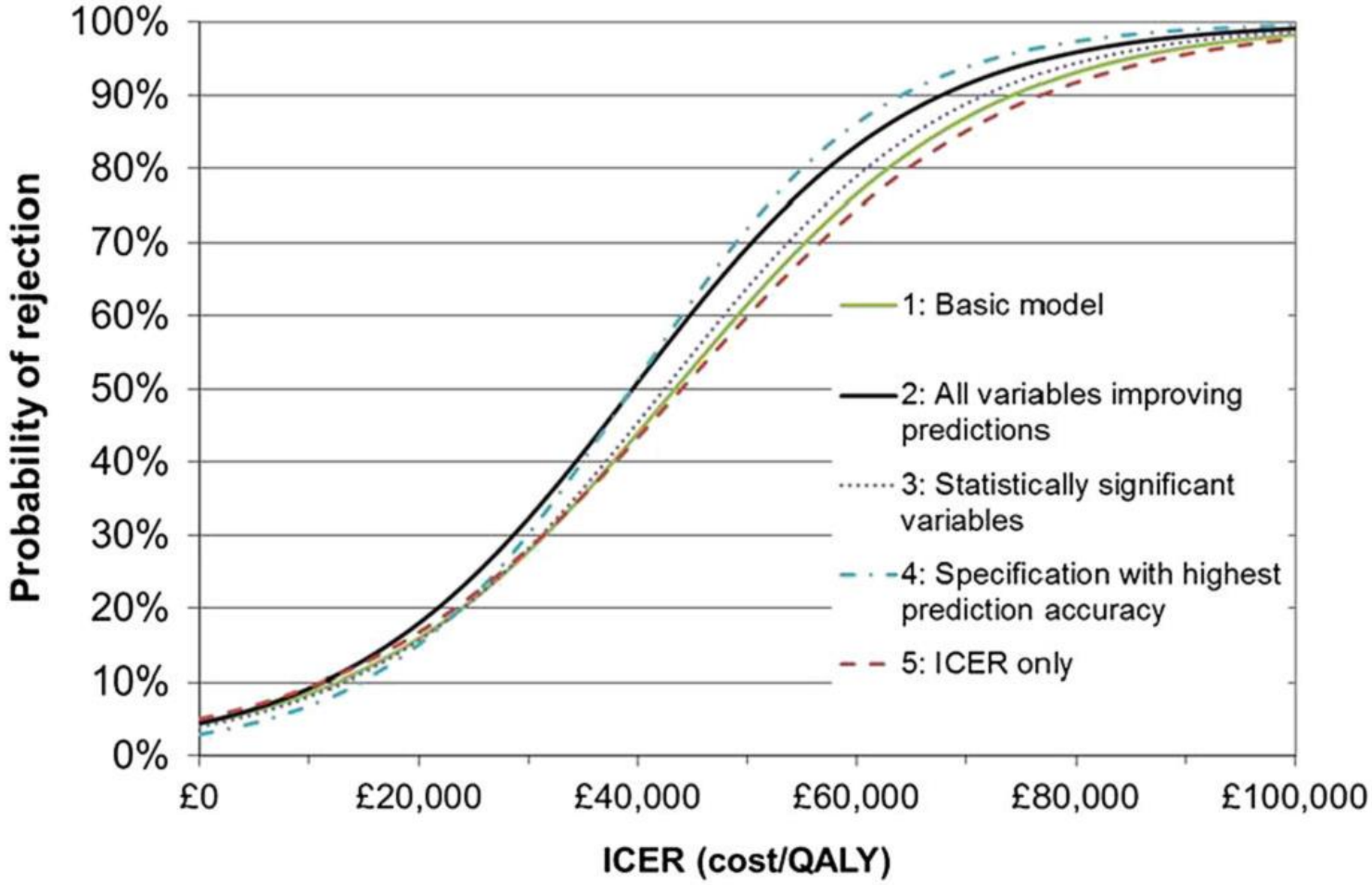
NICE Threshold in Practice

- Examination of NICE decisions finds higher threshold range centred around £40,000, [Devlin and Parkin, Dakin et al].

- Neither study finds time trends, implying no relaxation of threshold.

- Dakin et al. find an apparent cancer premium

NICE Threshold in Practice



Source: Figure 3, Dakin et al

Empirical Estimate of Opportunity Cost

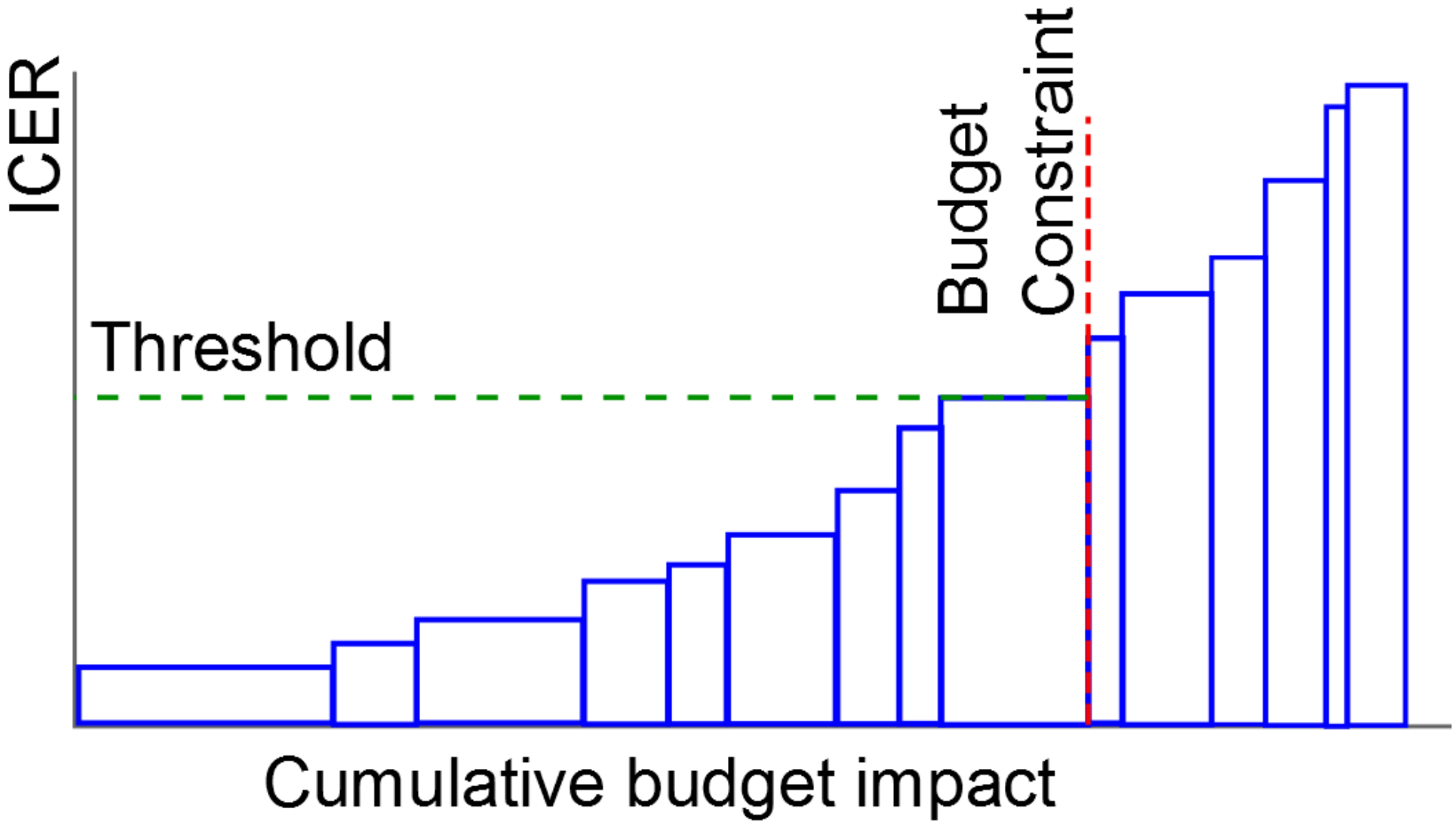
- Estimation of the cost-effectiveness of services actually displaced in the NHS [Claxton et al]
- Estimate of c. **£13,000/QALY**
- Large study relying on large number of assumptions [Raftery]
- Methods subject to criticism [Barnsley et al]
- Methods consistent with NICE's remit [Paulden et al 2014a], but not with true opportunity cost [Eckermann & Perkarsky]

Budget Exhaustion Threshold

- Threshold could change with:
  - Innovation
  - Productivity
  - Need
  - Budgets

- Exactly how depends on assumptions about disinvestment and reversibility of decisions

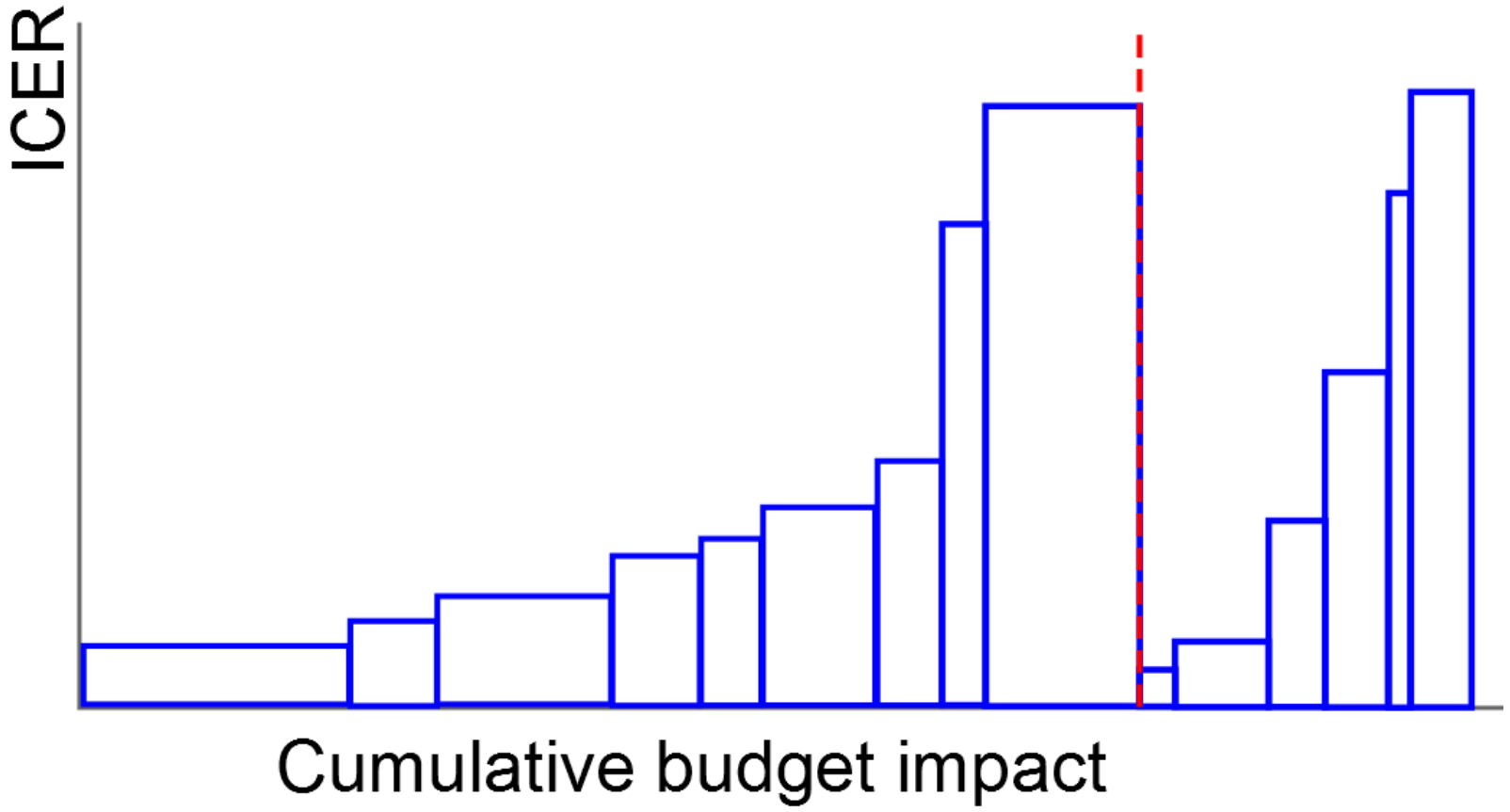
Budget Exhaustion Threshold



Exhaustion Threshold – Problems 1

- Large information requirement
- Circular logic of as a decision rule
  - Requires an optimal allocation to find the decision rule to find optimal allocations
- What if initial allocation is not optimal?

Budget Exhaustion Threshold



Exhaustion Threshold – Problems 2

- Requires:
  - Disinvestment
  - Interventions be divisible
  - The threshold to be updated iteratively

- Will fail to yield an optimal allocation if applied to a large set of new interventions without updating the threshold

Exhaustion Threshold – Problems 3

- Threshold does not account for actual opportunity cost [Gafni and Birch]
- Thus, an irresponsive threshold may fail to control health expenditure growth
- Threshold pricing leaves little scope for positive net health benefit [McCabe et al.]

Tension between Principles & Pragmatism

- The threshold should be dynamic
  - A static threshold is desirable from a planning and investment perspective
- The threshold should be responsive to budget impact
  - Permitting higher thresholds for smaller budget impact interventions raises concerns of a partial retreat from *value* to *affordability* as a decision criteria
- Single threshold for investment and disinvestment
  - A higher threshold for explicit disinvestment has been suggested

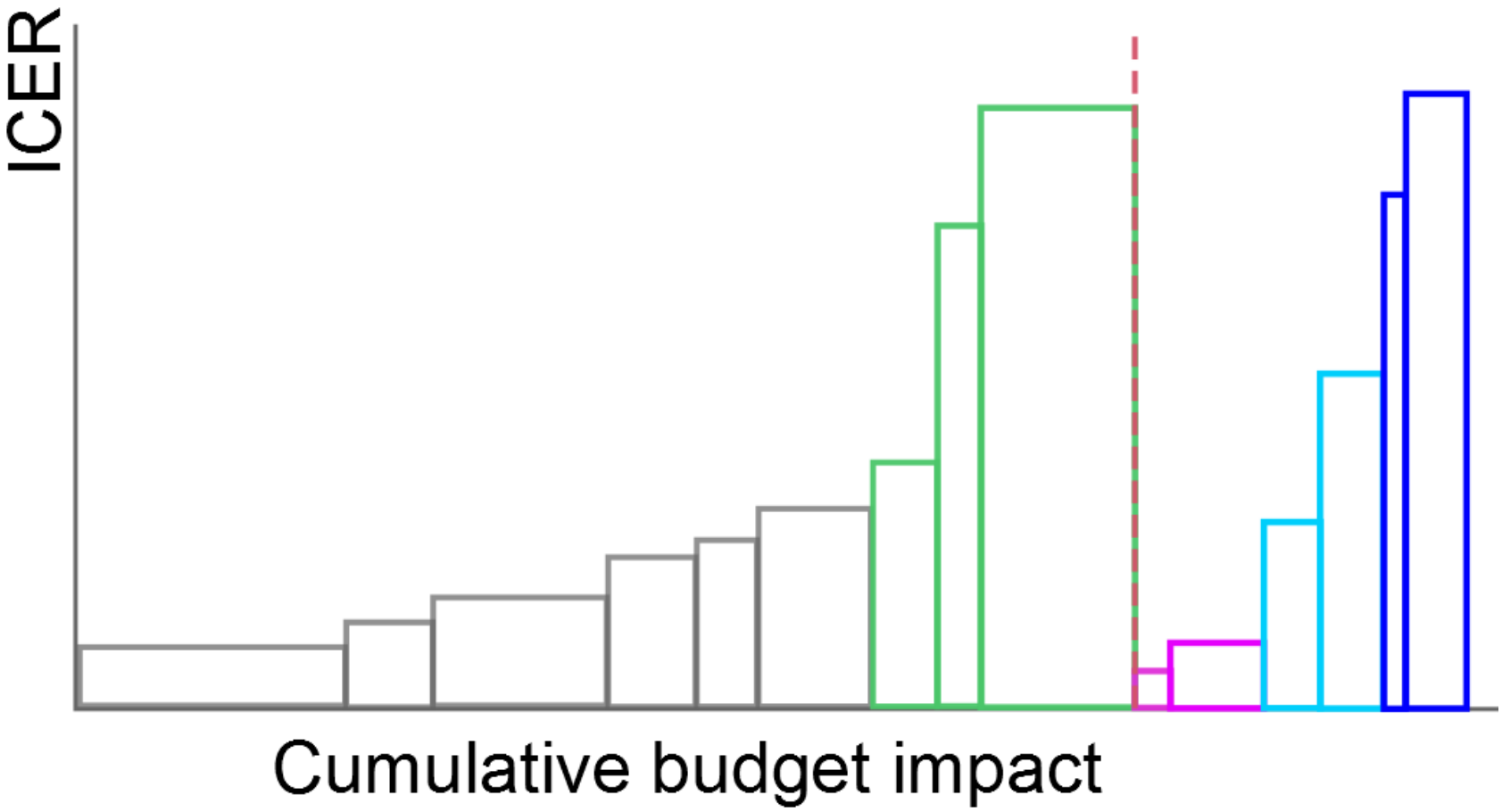
Prioritising Consideration of the non-Adopted set

- Claxton et al's estimates and Eckermann and Perkarsky's critique directs attention to interventions beyond the budget constraint

- This attention is welcome because:
  - Optimal explicit disinvestment is difficult to realise
  - Yet, inefficient displacement likely to persist
  - We are not yet at an efficient allocation

- How to target new expenditure at unimplemented interventions with greatest cost-effectiveness?

Prioritising Consideration of the non-Adopted set



Prioritising Consideration of the non-Adopted set - advantages

- The data requirements are less onerous
- Ensures explicit consideration of currently displaced interventions
  - Including interventions that lack backing of proprietorial interests or focused lobby support
- Gives incentives to manufacturers to bid to be part of new spending rounds

Prioritising Consideration of the non-Adopted set – disadvantages 1

- May require periodic spending rounds
  - eg, annual decision announcements
- Looses notion of single, predictable threshold
  - Reduces transparency
  - Problematic for NHB calculations
- May involve period of convergence with adjusting thresholds and considerable uncertainty

Prioritising Consideration of the non-Adopted set – disadvantages 2

- Pharmaceutical investment shackled to large budget impact spending challenges

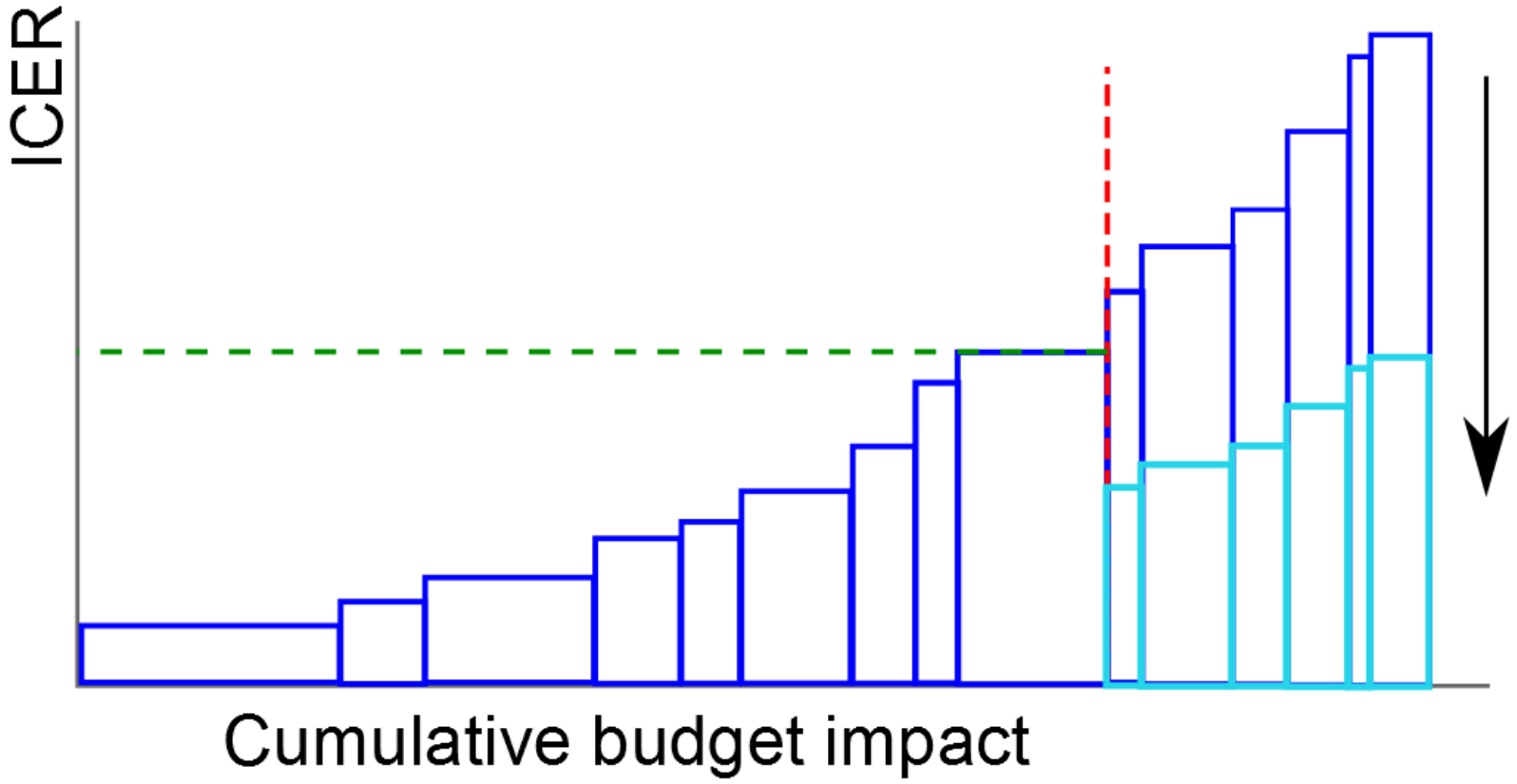
- Implies queue of interventions awaiting decisions
  - In tension with current pressure for rapid reimbursement
  - Not a problem in principle if only marginally beneficial

- Likely to be politically problematic

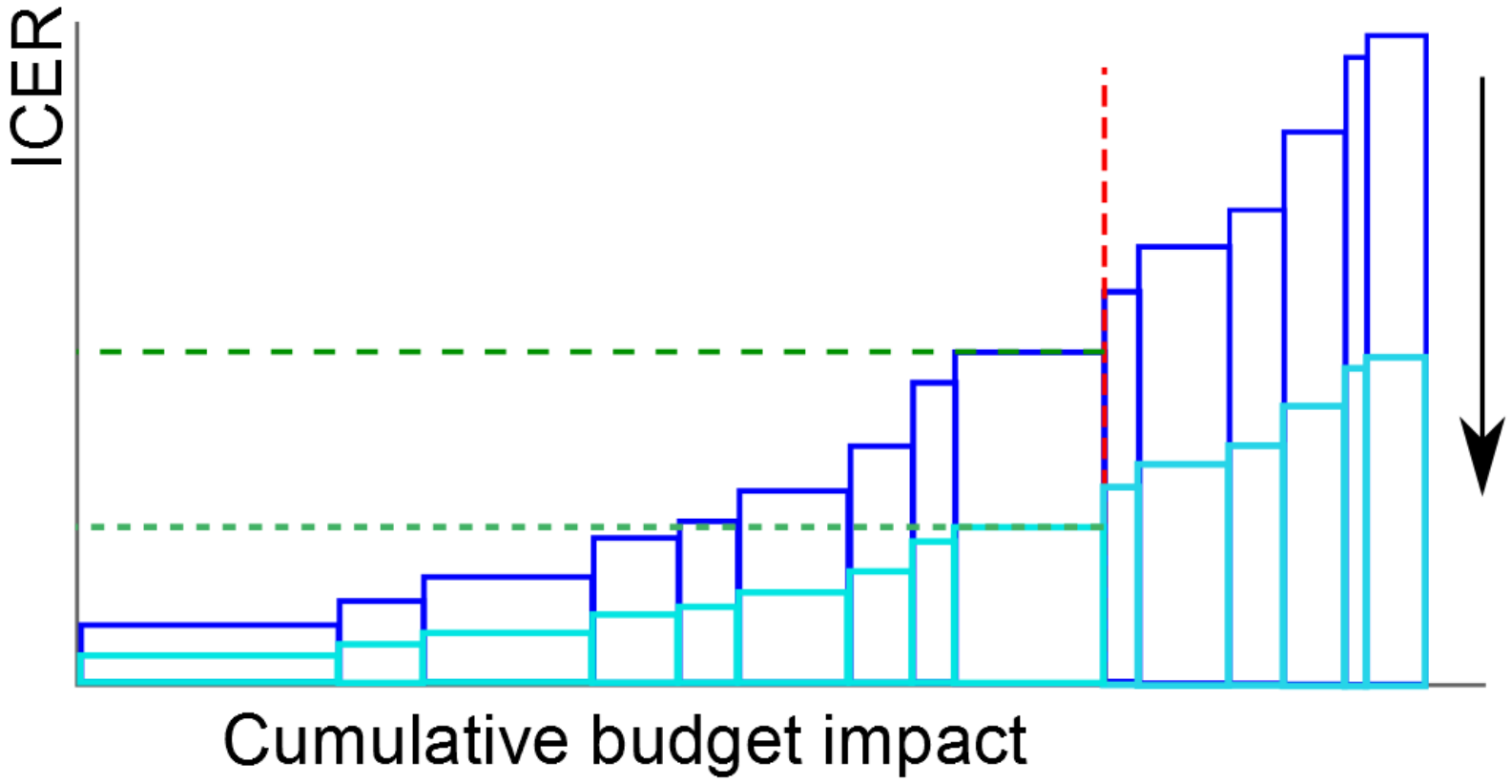
Changes to the Threshold

- NICE recently abandoned proposals to adjust its threshold
- The new threshold range was considerably wider at £20-50,000/QALY
- The range of interventions possibly qualifying for higher thresholds was potentially much greater
- Proposals were critiqued for being inconsistent in their application, leading to bias in favour of new interventions [Paulden et al 2014b]

Changes to the Threshold



Societal rather than Health Benefits



Sum up

- Thresholds bring consistency and transparency
- Simple and easy to understand decision rule
- CEA requires ethically defensible decision rules
- Budget exhaustion justification faces practical limitations
- Addressing interventions not adopted potentially a pragmatic solution
- Places adoption in context of implicit displacement
- Potentially inimical to new pharmaceutical products
- Could imply abandonment of thresholds

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