Rational Rationing

The Role of Research

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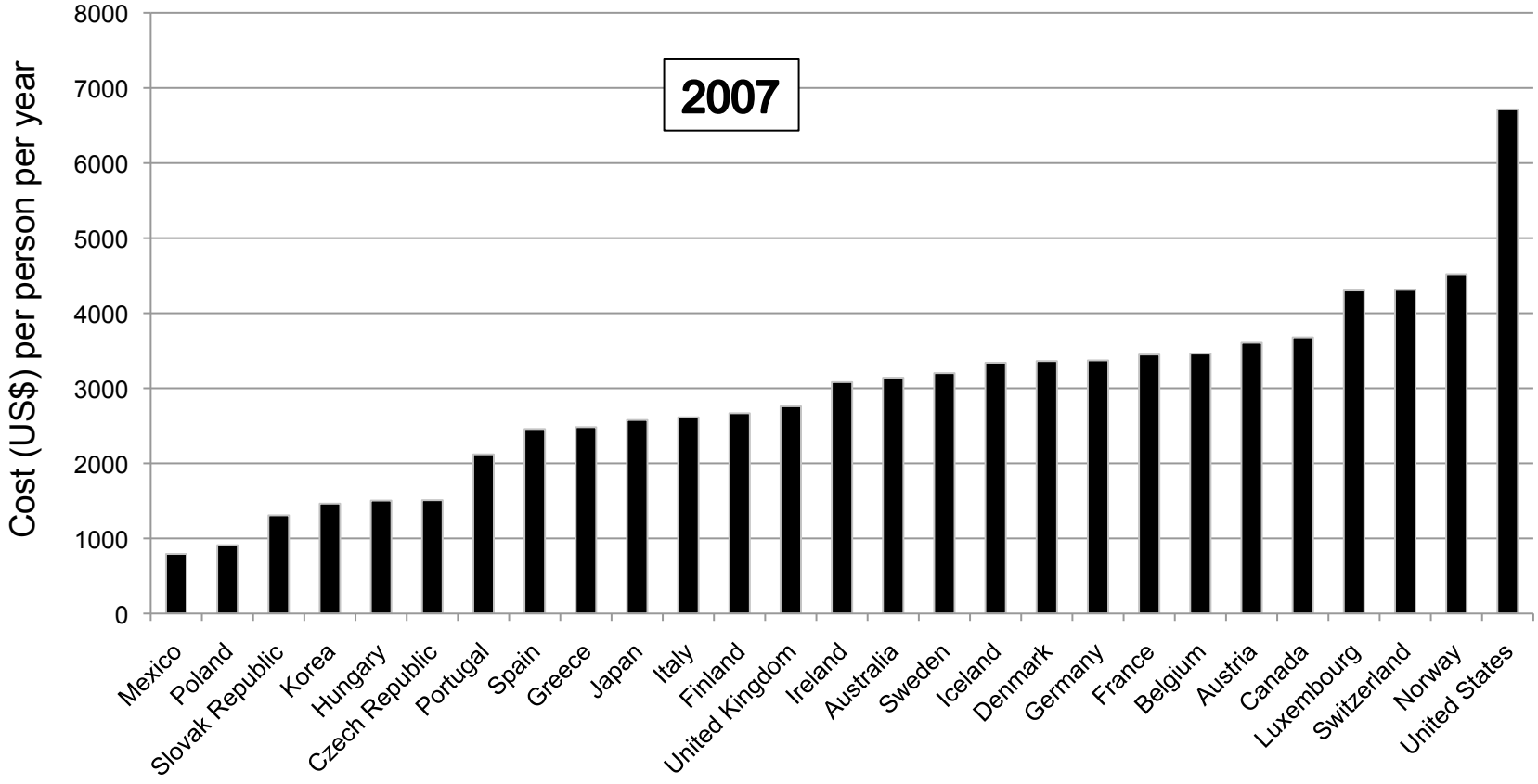
Resource Constraints

Healthcare Expenditure (US$ per person)



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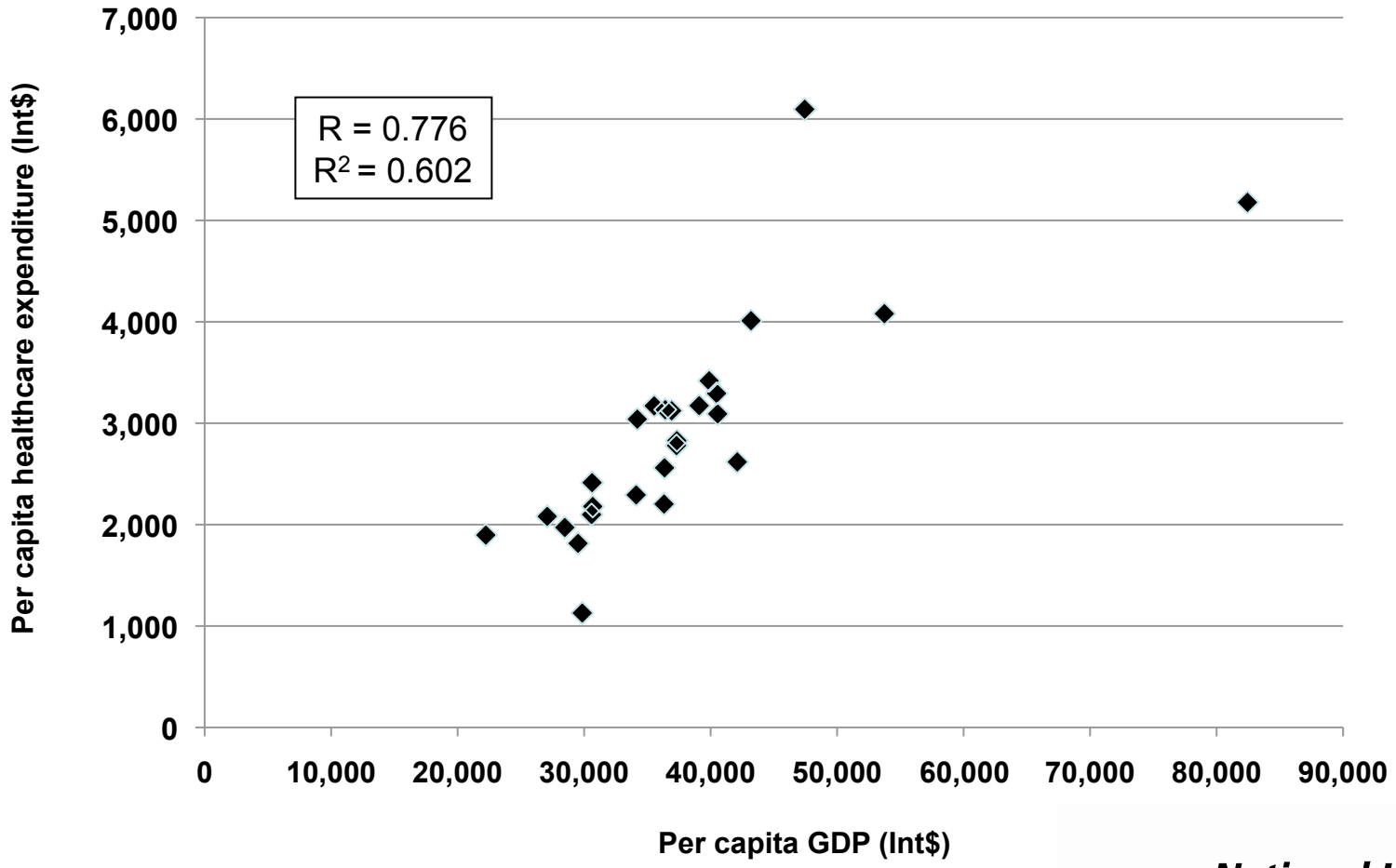
GDP and Healthcare Expenditure

2007



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NICE guidance

1. **Clinical:**
   - Technology appraisals
   - Clinical guidelines
   - Interventional procedures
   - Medical technologies
   - Diagnostics

2. **Public health**

3. **Quality standards and metrics**
   - Quality & Outcomes Framework
   - NICE Quality Standards

4. **NHS Evidence**

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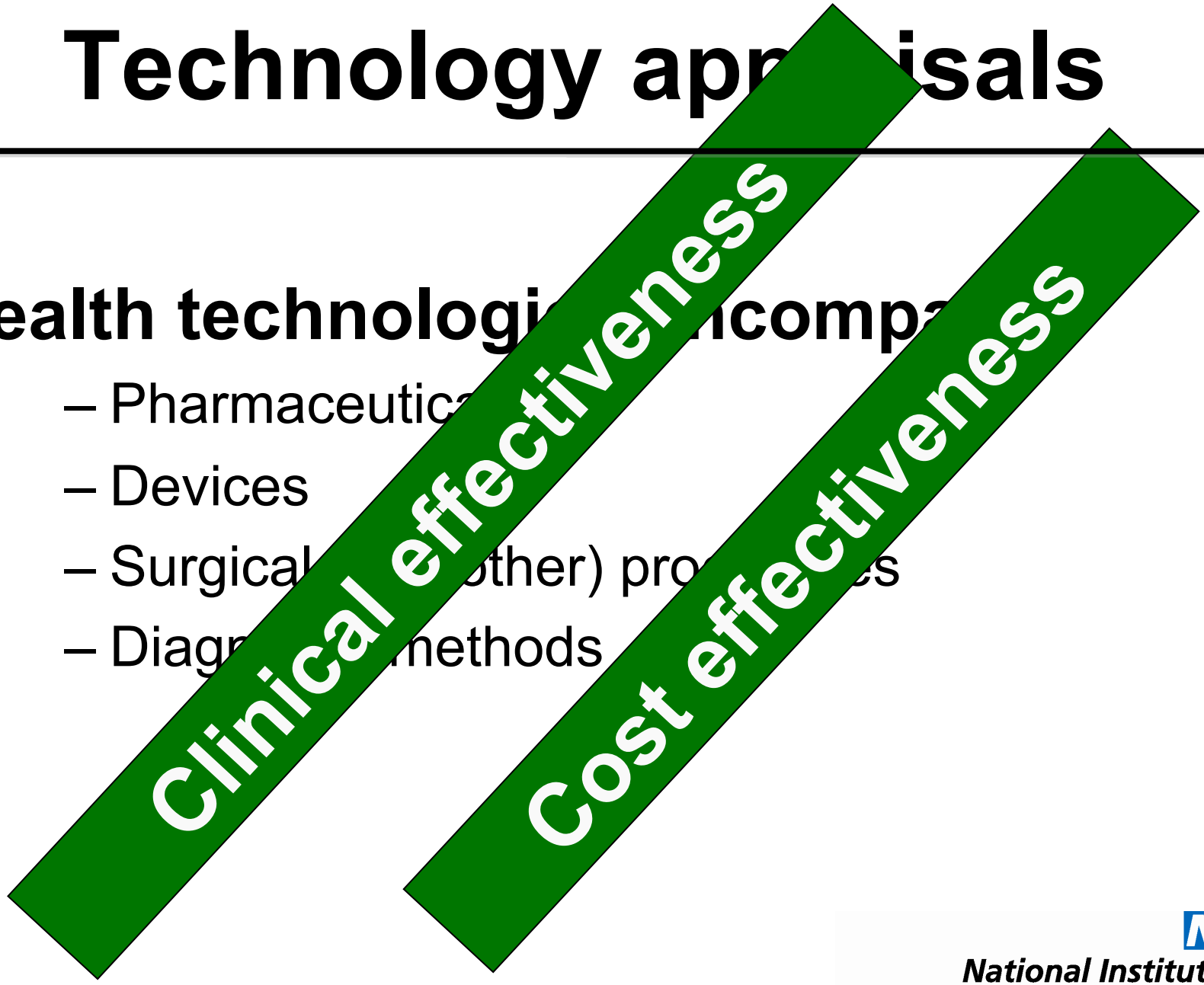
NICE guidance

Type	Published	In development
Technology appraisals	200	130
Clinical guidelines	130	52
Interventional procedures	322	30
Medical technologies	0	8
Diagnostics	0	3
Public health	27	33
Total	679	256

Technology appraisals

Health technologies encompass

- Pharmaceuticals
- Devices
- Surgical (and other) procedures
- Diagnostic methods



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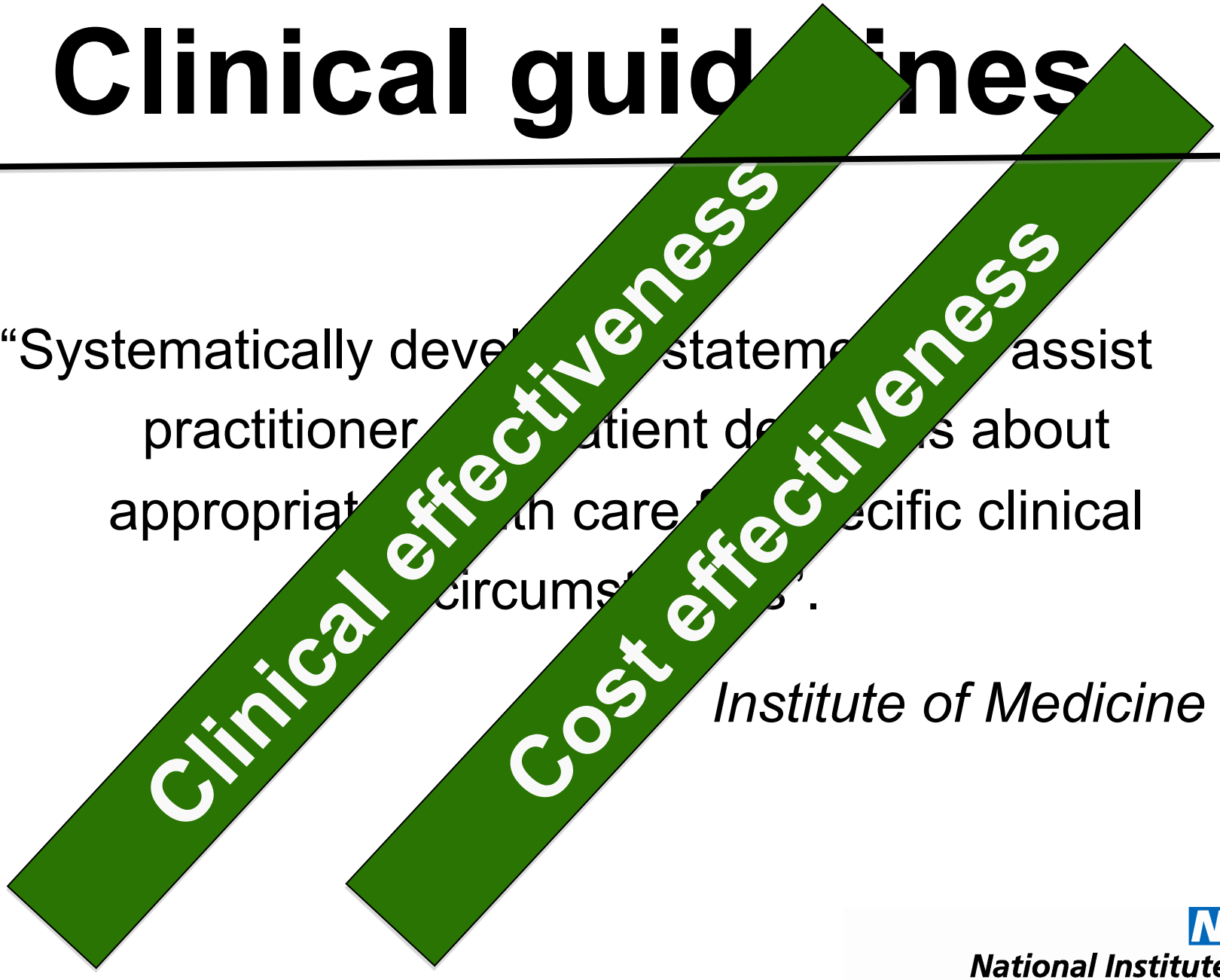
Clinical guidelines

"Systematically deve statem assist practitioner atient d s about appropriat th care ecific clinical circums ".

Institute of Medicine



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Principles

1. Robust
2. Inclusive
3. Transparent
4. Independent

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Clinical Evaluation

1. Randomised contro trials
2. Observati studies
3. S matic reviews

Avoiding "hierarchies" of evidence

Randomized controlled trials

advantages

1. Minimises bias
2. Minimises confounding
3. Minimises random error

Randomized controlled trials

disadvantages

1. The null hypothesis
2. P-values
3. Generalisability
4. Multiplicity
    - Stopping rules
    - Subgroup analyses
    - Safety analyses
5. Cost

Comparative effectiveness

1. **Direct comparisons**
    - A versus B
3. **Indirect comparisons**
    - A versus placebo
    - B versus placebo
    - Impute A versus B
3. **Mixed treatment comparisons**

Observational Studies

1. Historical controlled trials
2. Concurrent cohort studies
3. Case-control studies
4. Case series (registries)
5. Case reports

Systematic reviews

Efficacy:

- Good at synthesizing RCT evidence
- Weak at incorporating observational data

Safety:

- Good at synthesizing RCT evidence
- Very weak at synthesizing observational data

Cost effectiveness:

- Very poor

Economic Evaluation

Overarching principles:

1. **Economic perspective**
   - NHS and PSS
2. **Cost effectiveness**
   - Not affordability or budgetary impact
3. **Balance between:**
   - Efficiency (utilitarianism)
   - Fairness (egalitarianism)

Cost Utility Analysis

Costs (and savings)

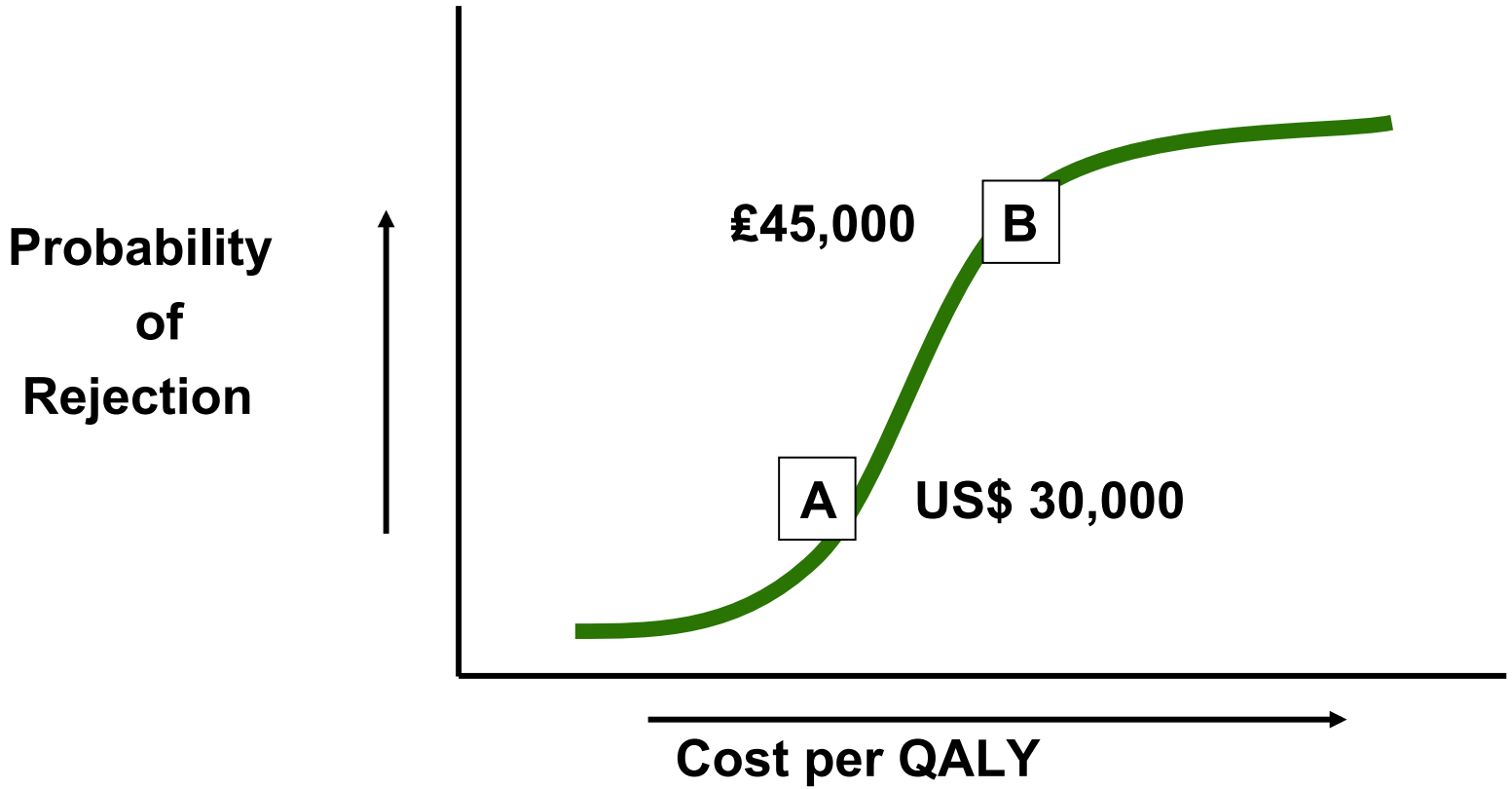
- direct
- indirect

Benefits

- in[illegible]ement (change) in HRQoL (utility)
- [illegible]e for which it is “enjoyed”

Incremental cost effectiveness ratio

Cost Ineffectiveness



Decision-making

1. Scientific judgements

- Reliability of the evidence-base
- Appropriateness of sub-groups
- Generalisablity
- Capture of quality of life
- Handling uncertainty

2. Social value judgements

- Severity of disease
- End of life interventions (“rule of rescue”)
- Age
- Health inequalities

Social Value Judgements

Citizens Council:

- 30 members
- Cross-section of England and Wales
- Serve for 3 years (one third retiring annually)
- Meet twice a year – for 3 days
- Deliberative process
- Reports directly to the Board

Culturally and context specific

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Case-by-Case Decisions

Factors taken into account include:

- severity of the underlying condition
- extensions to end of life
- stakeholder persuasiveness
- significant clinical innovation
- children
- disadvantaged populations
- corporate responsibility

Recommendations >£30,000 per QALY

Product	Condition	QALY (£)	Severity	Significant innovation
Riluzole	Amyotrophic lateral sclerosis	40,000	★	★
Trastuzumab	Early breast cancer	37,500	★	★
Imatinib	Chronic myeloid leukaemia	36,000 to 65,000	★	★
Pemetrexed	Mesothelioma	34,500	★	★
Sunitini	Advanced renal carcinoma	50,000	★	★
Lenalidomide	Multiple myeloma	43,000	★	★

Conclusions

1. Rationing can (and should) be rational
2. Research methodology needs improving
   - less resource-intensive approaches to RCTs
   - creative use of observational data
   - capture the potential of digital technology
3. Earn and retain the trust of all our stakeholders