Rational Rationing

The Role of Research

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Resource Constraints

Healthcare Expenditure (US$ per person)
GDP and Healthcare Expenditure
2007

R = 0.776
R² = 0.602
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
## NICE guidance

<table>
<thead>
<tr>
<th>Type</th>
<th>Published</th>
<th>In development</th>
</tr>
</thead>
<tbody>
<tr>
<td>Technology appraisals</td>
<td>200</td>
<td>130</td>
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<tr>
<td>Clinical guidelines</td>
<td>130</td>
<td>52</td>
</tr>
<tr>
<td>Interventional procedures</td>
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<td>30</td>
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<td>Diagnostics</td>
<td>0</td>
<td>3</td>
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<td>Public health</td>
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<td><strong>Total</strong></td>
<td><strong>679</strong></td>
<td><strong>256</strong></td>
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</table>
Technology appraisals

Health technologies encompass:

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

Clinical effectiveness  Cost effectiveness

National Institute for Health and Clinical Excellence
Clinical guidelines

“Systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances”.

Institute of Medicine
Principles

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

1. Randomised controlled trials
2. Observational studies
3. Systematic 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:
- improvement (change) in HRQoL (utility)
- time for which it is “enjoyed”
Cost Ineffectiveness

Cost per QALY

- A: US$ 30,000
- B: £45,000

Probability of Rejection
Decision-making

1. Scientific judgements
   - Reliability of the evidence-base
   - Appropriateness of sub-groups
   - Generalisability
   - 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
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

<table>
<thead>
<tr>
<th>Product</th>
<th>Condition</th>
<th>QALY (£)</th>
<th>Severity</th>
<th>Significant innovation</th>
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<tbody>
<tr>
<td>Riluzole</td>
<td>Amyotrophic lateral sclerosis</td>
<td>40,000</td>
<td>★</td>
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<td>Trastuzumab</td>
<td>Early breast cancer</td>
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<td>Imatinib</td>
<td>Chronic myeloid leukaemia</td>
<td>36,000 to 65,000</td>
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<tr>
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<td>★</td>
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<td>Sunitini</td>
<td>Advanced renal carcinoma</td>
<td>50,000</td>
<td>★</td>
<td>★</td>
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<tr>
<td>Lenalidomide</td>
<td>Multiple myeloma</td>
<td>43,000</td>
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<td>★</td>
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</table>
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