Outcomes-based healthcare: a health technology assessment (HTA) perspective

Dr Jacoline Bouvy
Science Policy and Research Programme
A brief history of NICE

• Originally set up in 1999 as the National Institute for Clinical Excellence (NICE), a special health authority, to reduce variation in the availability and quality of NHS treatments and care

• In April 2013 established as a non-departmental public body and took on responsibility for developing guidance and quality standards in social care – name changed to the National Institute for Health and Care Excellence
NICE’s overall aim

“To be the national point of reference for advice on safe, effective and cost effective practice in health and social care, providing guidance, advice and standards aligned to the needs of its users and the demands of a resource constrained system.”
Is NICE guidance mandatory?

**YES**
NHS organisations are *legally required* to provide *access to drugs* we have approved through our technology appraisal programme and highly specialised technologies programme.

**NO**
All other NICE guidance (clinical guidelines, public health, social care etc) is *advisory, not mandatory*. It is a summary of the evidence of what works, but it is not intended to replace clinical judgement.
Methods and decision-making

Clinical effectiveness → Cost effectiveness

Uncertainty
Non-health benefits
Fairness and equality
Social value judgements
Innovation

Robust Consistent Transparent
Clinical effectiveness

• Health benefit of a technology
  – Compared with established clinical practice

• Considering all available evidence (clinical trials, observational studies, submissions and experts testimony)

• Clinical outcomes and outcomes important to patients
Outcomes used by NICE

- NICE has to make decisions **across** different technologies and **disease areas**
- It is crucial that analyses adopt a consistent approach
- Fixed budgets and opportunity costs
- **QALY:**
  - Combines health-related quality of life & life expectancy in one measure
  - Not an endpoint in itself, but calculated/estimated using models
  - 1 QALY = 1 year in perfect health
Managing decision uncertainty

• What if a new drug might be cost-effective...
• ...but there is substantial uncertainty?
• Increases probability of making wrong decision

• Options for decision maker (*dependent on jurisdiction*):
  • No reimbursement
  • Lower price/discount/rebate to improve cost-effectiveness
  • Conditional reimbursement/managed access/coverage with evidence development/pay-for-performance
Current developments

• Products coming to the market earlier/less comprehensive evidence:
  • Conditional marketing authorisations
  • Accelerated assessment
  • Adaptive pathways
Current developments

- Increasing availability of real-world data:
  - electronic data beyond traditional clinical trials
- Big data/machine learning/AI
- Capacity to analyse and use such data is increasing
- Key challenge: regulators, HTAs and payers are not used to these data:
  - Dialogue and methods development needed
    - IMI GetReal
    - IMI BD4BO projects
Future?

• More collaborative approach between healthcare decision makers
  • Usefulness + acceptability of RWE and big data
  • Testing methods used in evidence synthesis
  • Testing robustness of data + methods