

Realising the Value from Health Data ~  
Improving Care and Research

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

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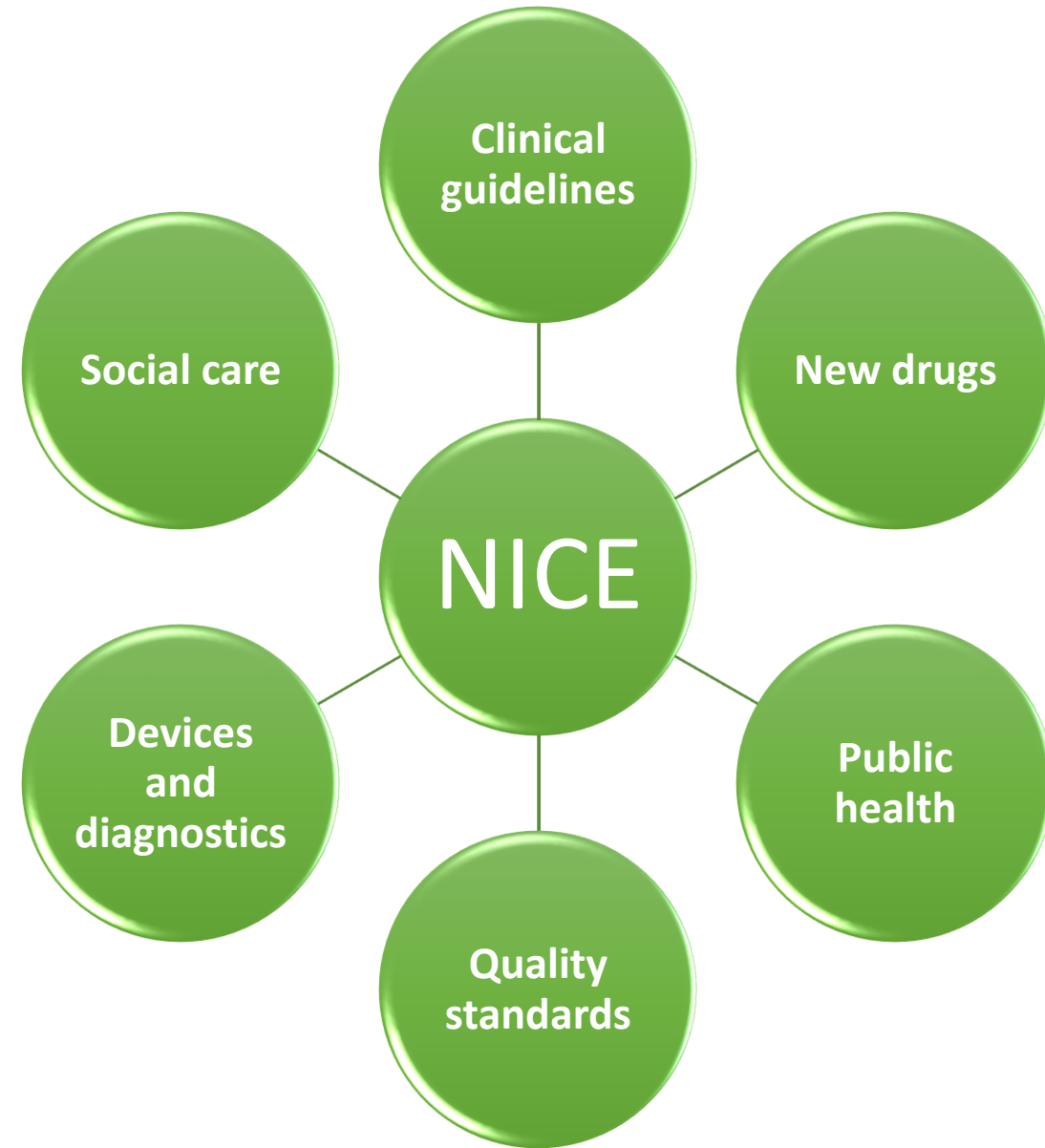
# A brief history of NICE



- 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?

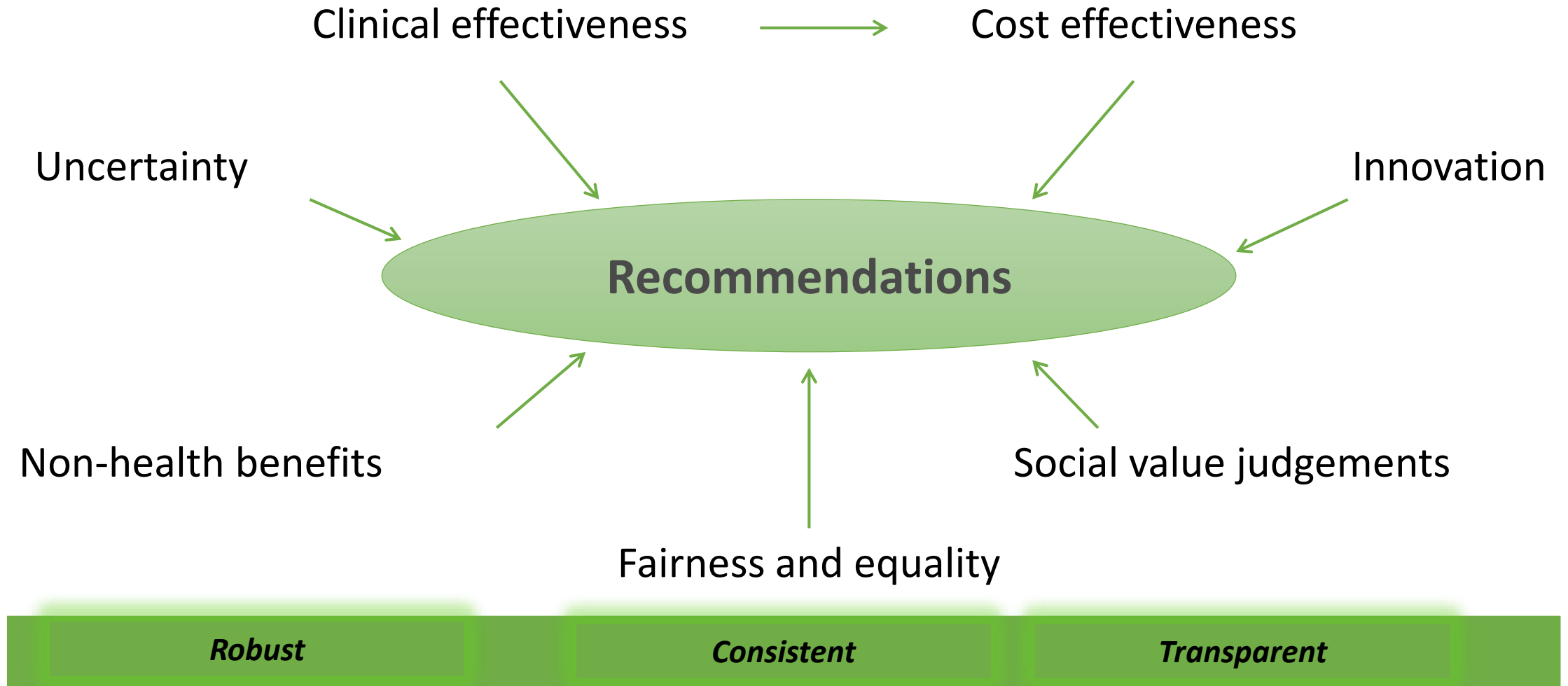
**YES**

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

**NO**

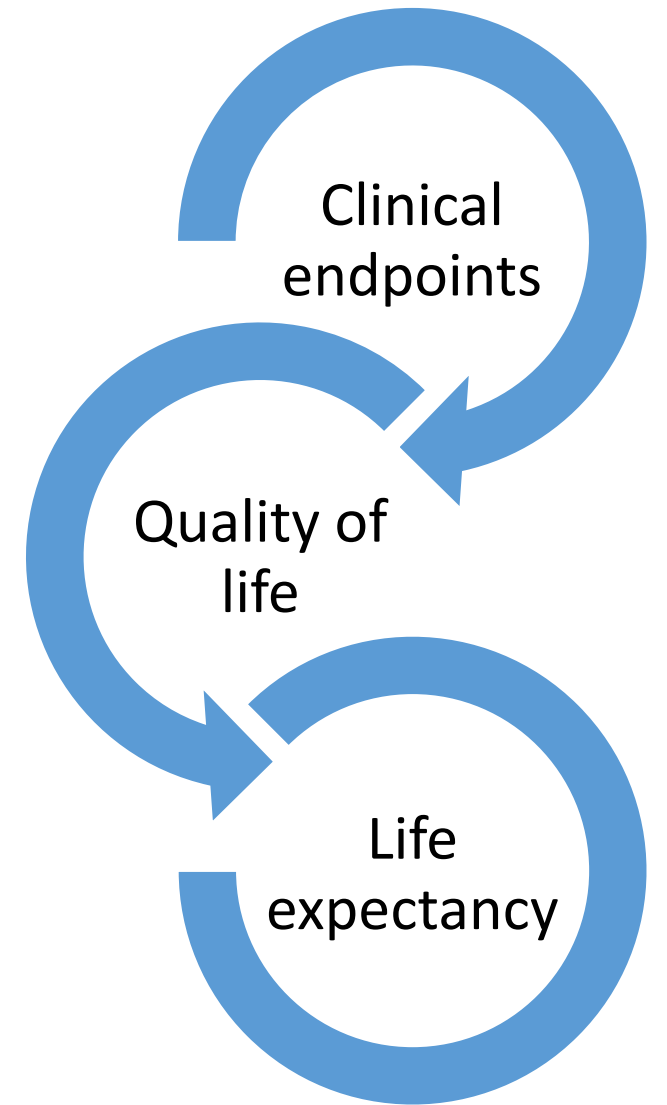
All other NICE guidance (clinical guidelines, public health, social care etc) is **advisory, not mandatory**. It is a summary of the evidence of what works, but it is not intended to replace **clinical judgement**.

# Methods and decision-making



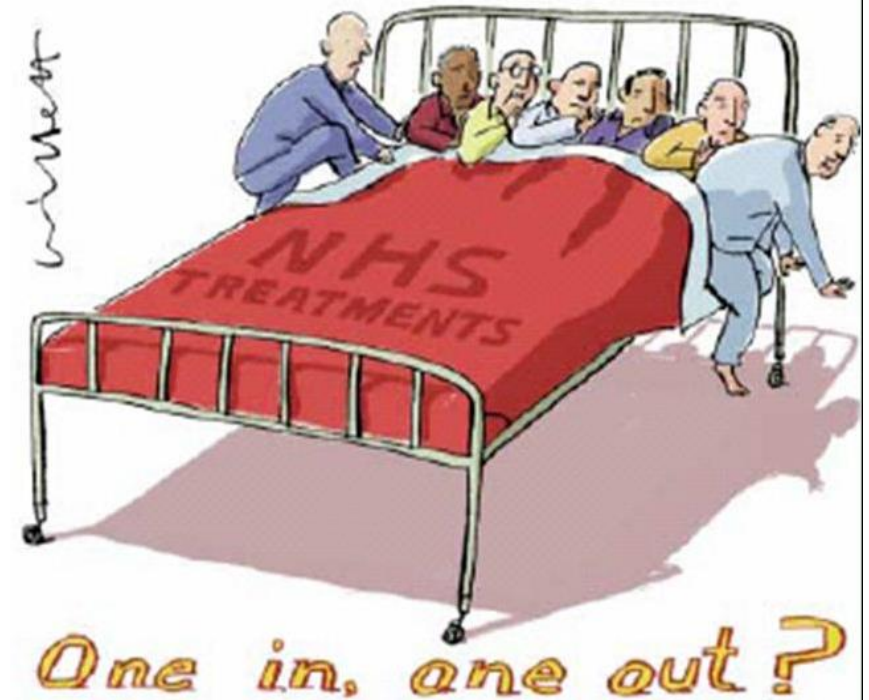
# Clinical effectiveness

- 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

