

# Medical advances and the changing role of real-world evidence

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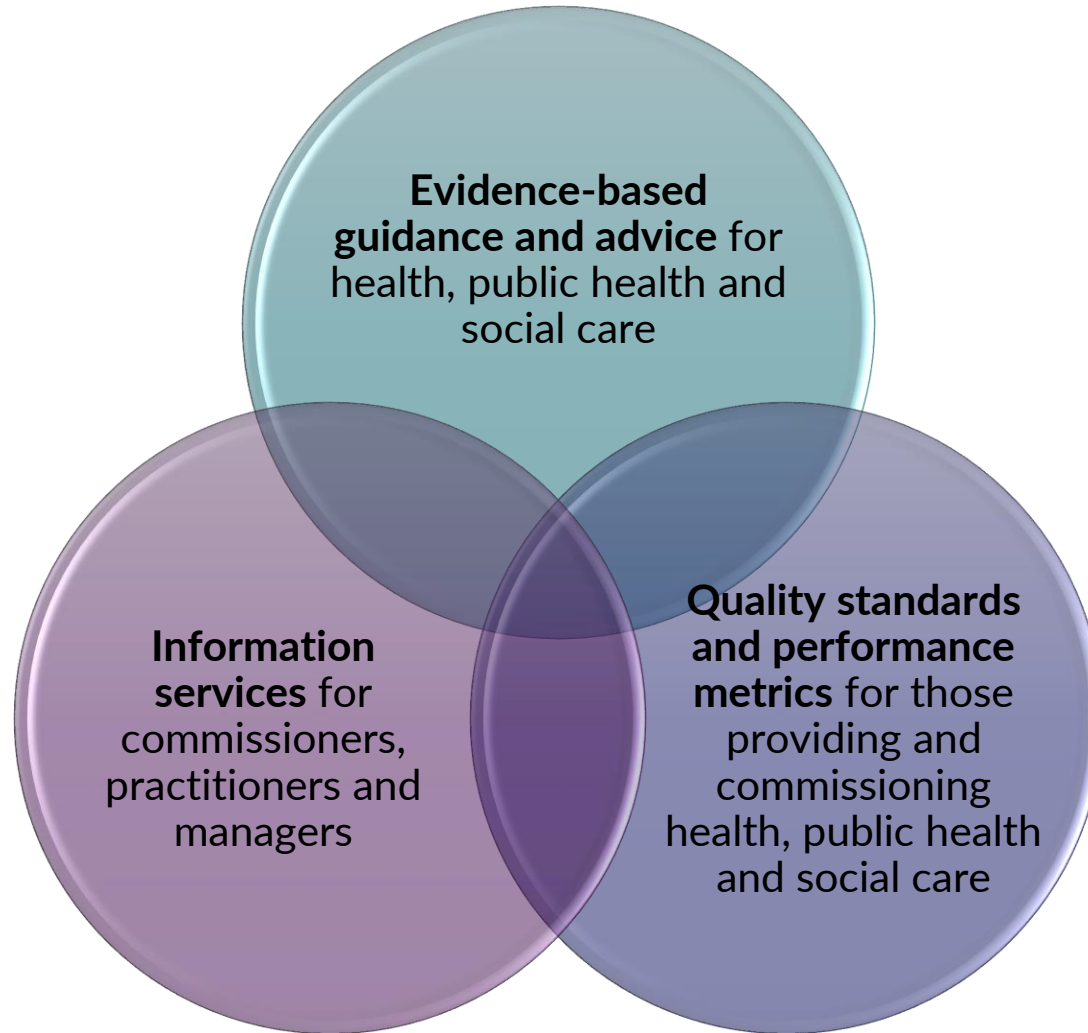
Science, Policy and Research Programme

# The background: why NICE was set up

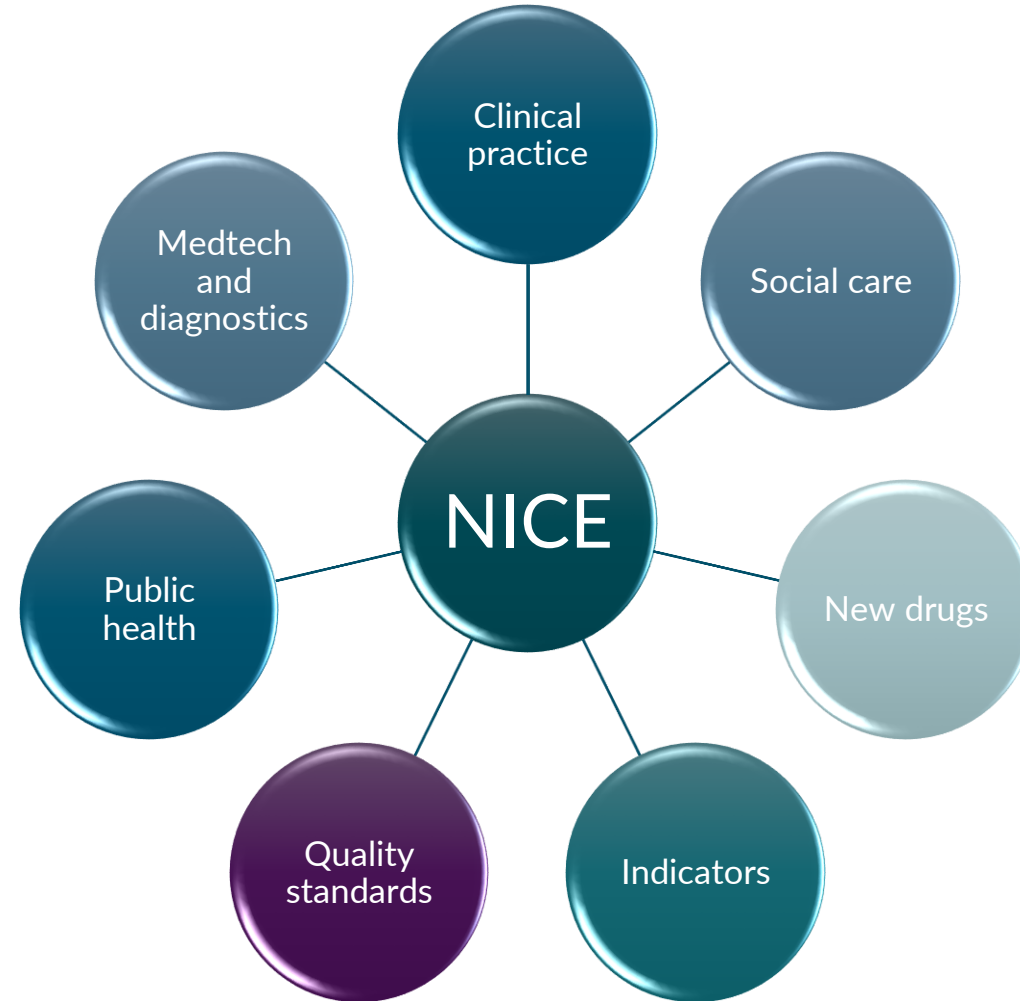
- Established in 1999
- Aim: to reduce variation in the availability and quality of treatments and care (the so called 'postcode lottery')
- To resolve uncertainty about which medicines and treatments work best and which represent best value for money for the NHS



# Improving outcomes for people



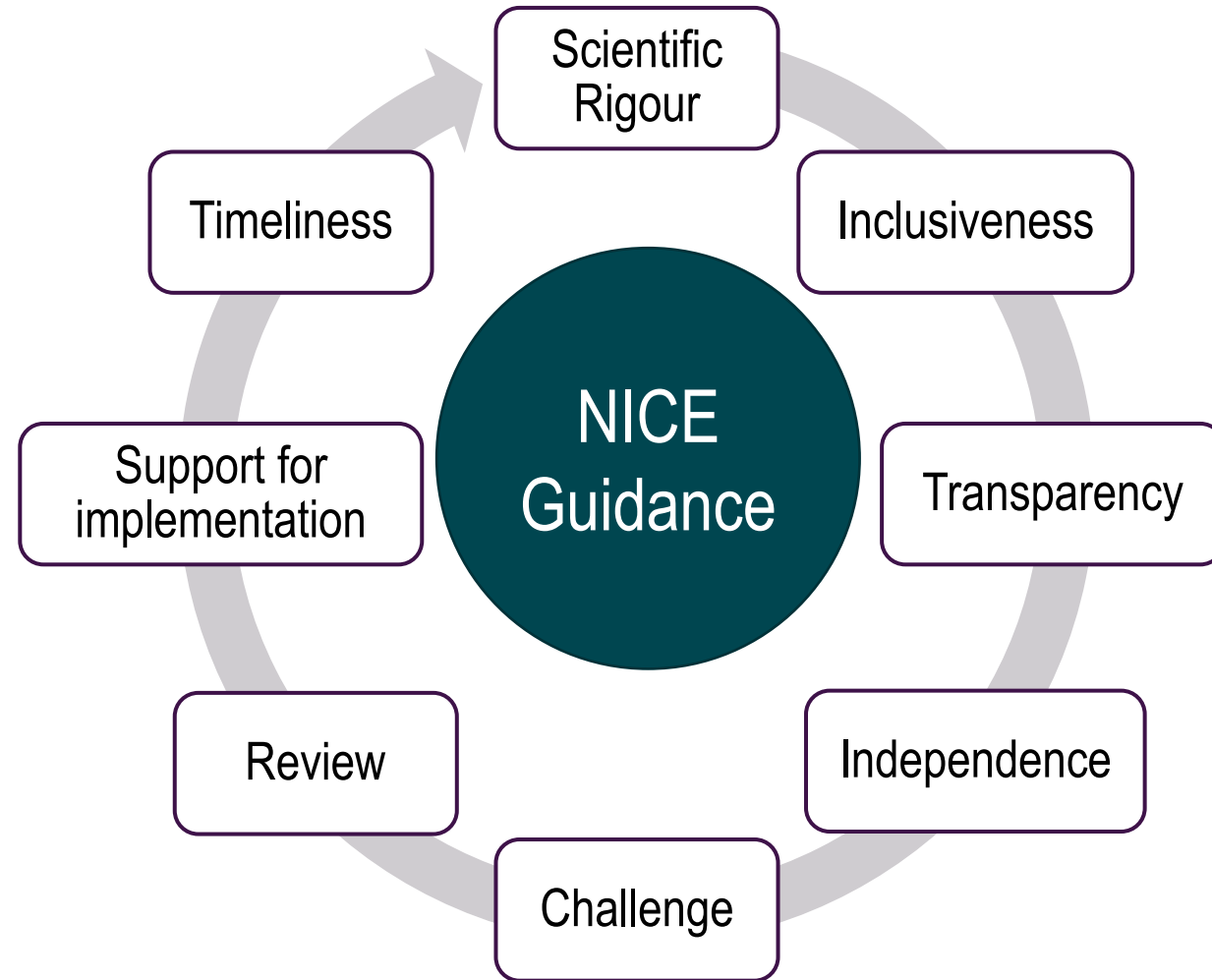
# The NICE portfolio in 2018



# Core principles of NICE's work

- Based on the best evidence available
- Expert input
- Patient and carer involvement
- Independent advisory committees
- Consultation
- Regular review
- Open and transparent process
- Social values and equity considerations

# Procedural principles for guidance development



# Our role: how does NICE develop recommendations?

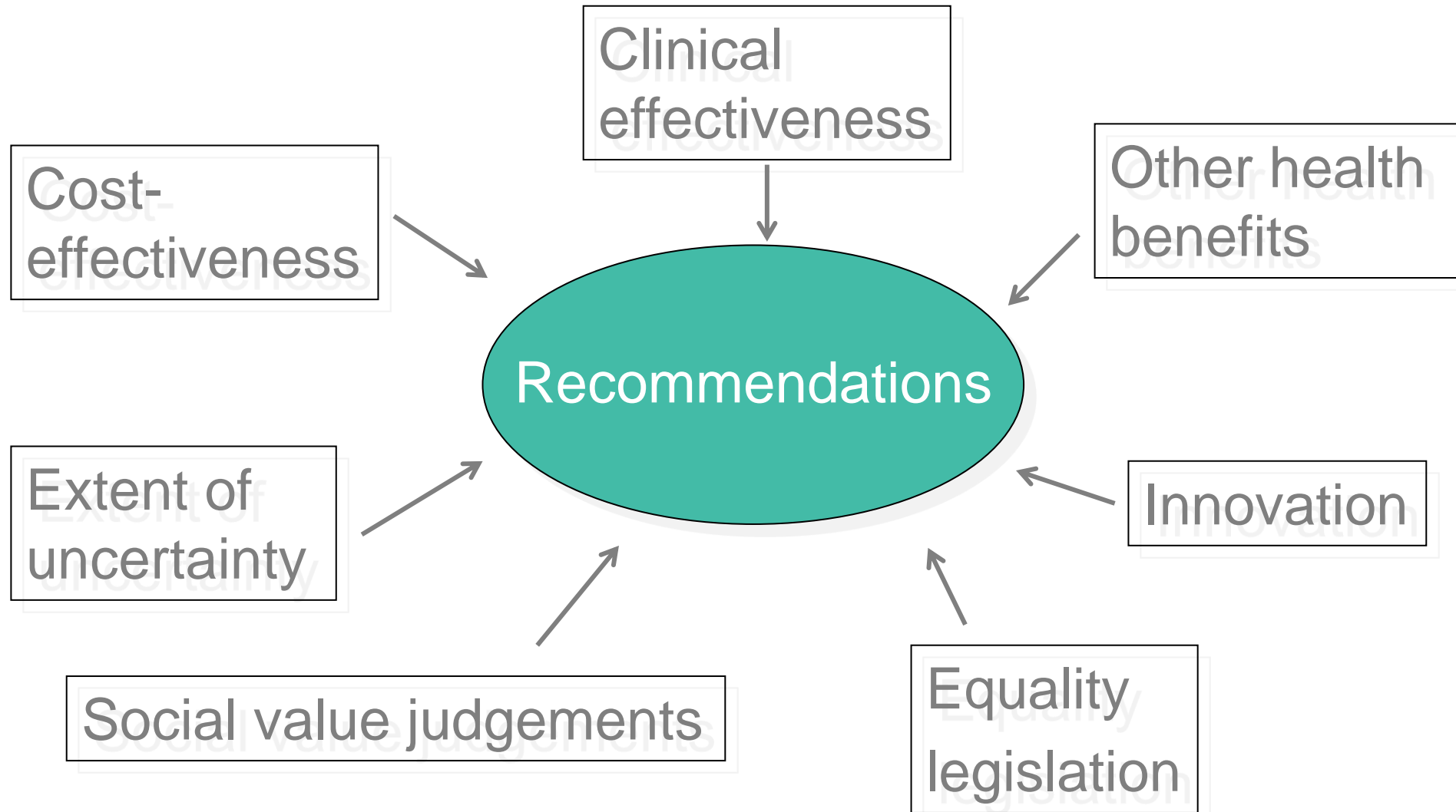
# What works? And how much does it cost?

NICE guidance takes into account both **clinical effectiveness** and **cost effectiveness**





# Committee decision making

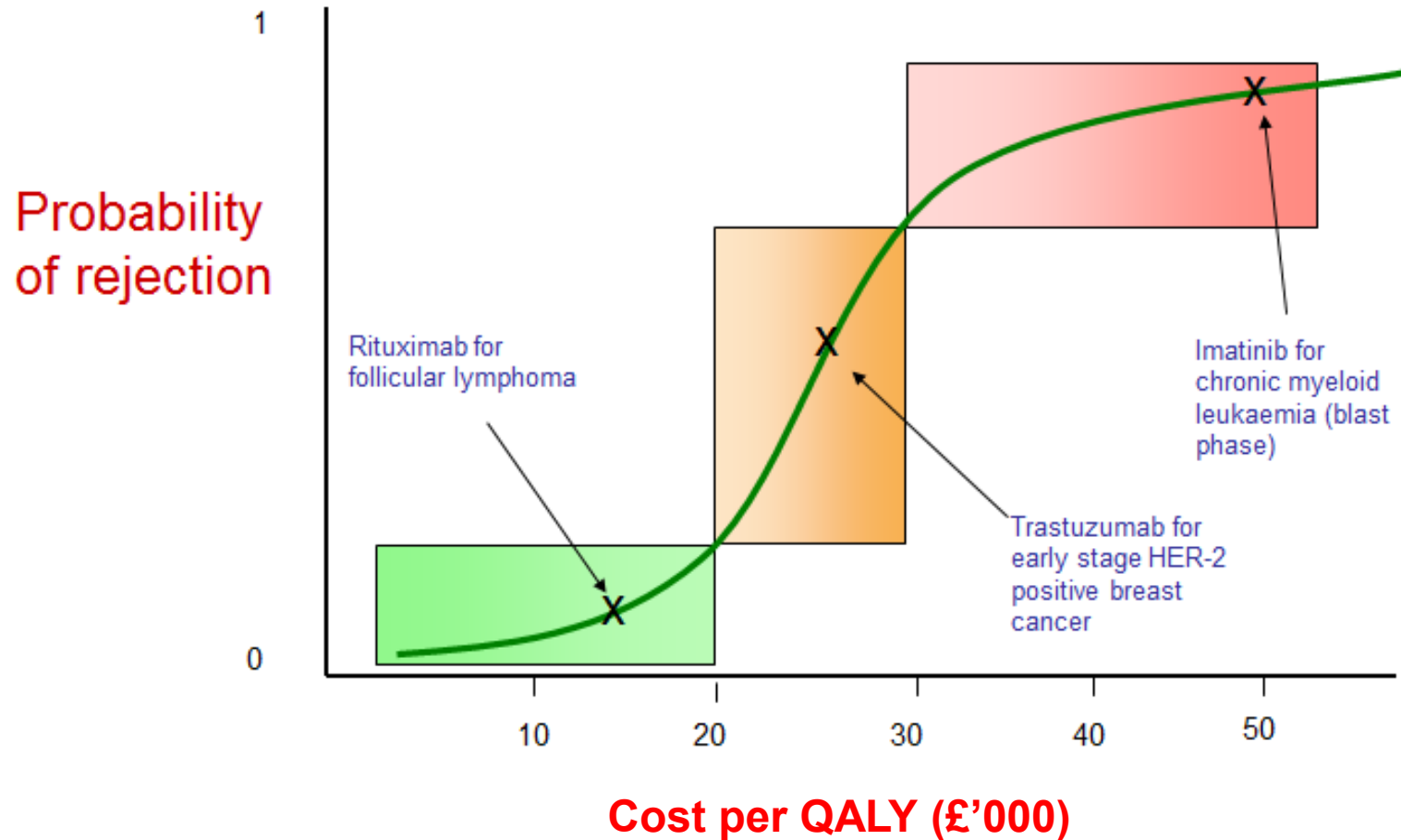


# Economic evaluation of new drugs/treatments

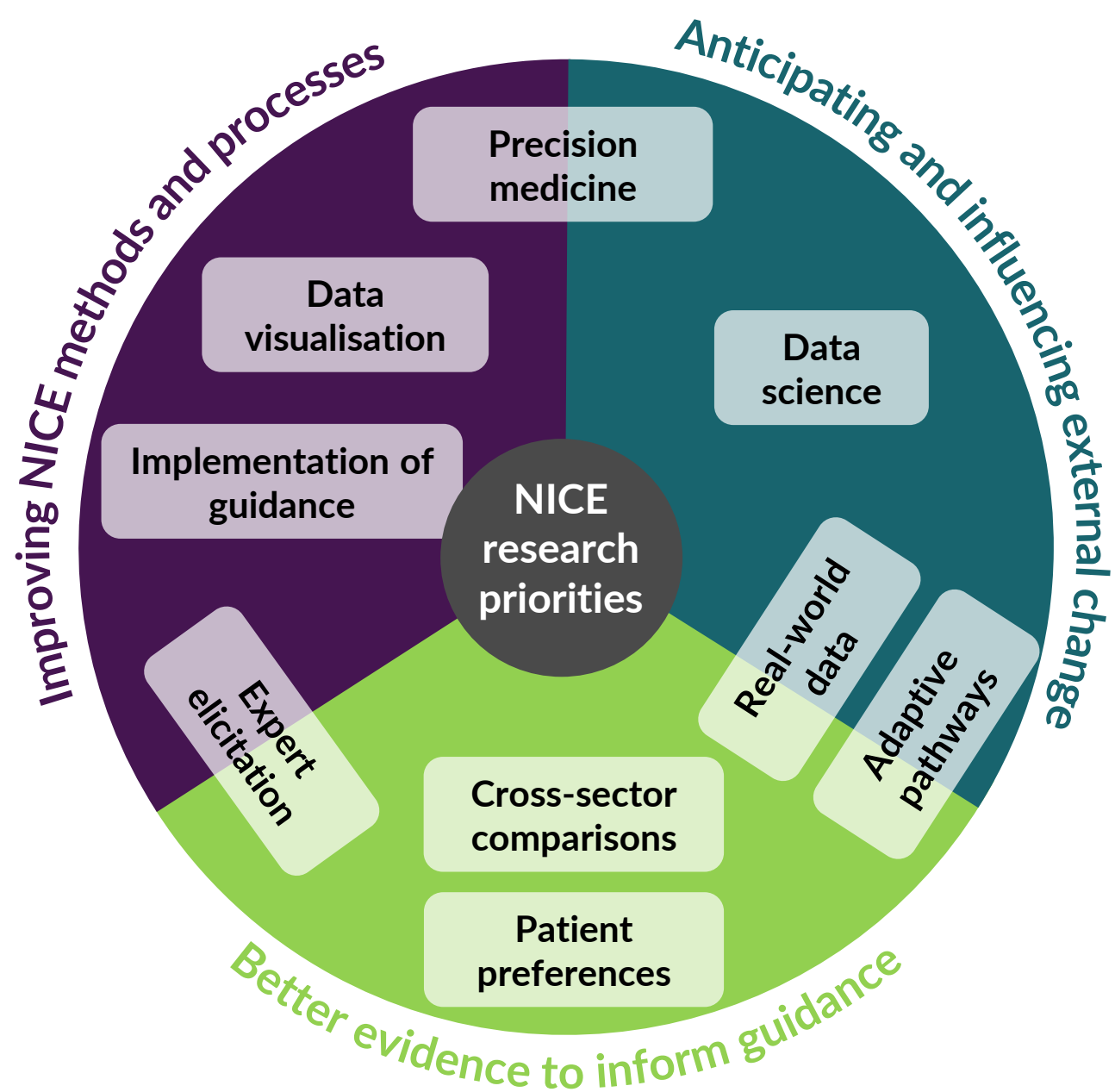
- How well does the drug/treatment work in relation to how much it costs compared to standard practice in the NHS ?
- Recognises the reality of fixed NHS resources
- Exposes the opportunity cost of new interventions, that is if you spend money on a new healthcare intervention, you have to take away the health care from someone else
- Enables consistency and fairness across all decisions



# Establishing value: cost effectiveness



# Research Priorities



Precision  
medicine

Data  
science

Real-  
world  
data

Adaptive  
pathways

**Anticipating and  
influencing  
external change**



## News and features

03 January 2018

### NICE final draft guidance recommends gene therapy for rare ‘bubble baby syndrome’ [Share](#)

Strimvelis, a treatment for an ultra-rare inherited immune deficiency condition that has been dubbed ‘bubble baby syndrome’ has been approved by NICE in final draft guidance.

🏠 > Business

### Leading US biotech Bluebird eyes UK launch for pioneering gene therapies



Andrew Obenshain, Bluebird’s head of Europe, said:  
“Europe will be the first market. We’re having very pro-active early discussions. We don’t plan to come and surprise governments.”  
*(Telegraph, 6 August 2017)*

## Cancer research

## NHS preparing to offer 'game-changing' cancer treatment

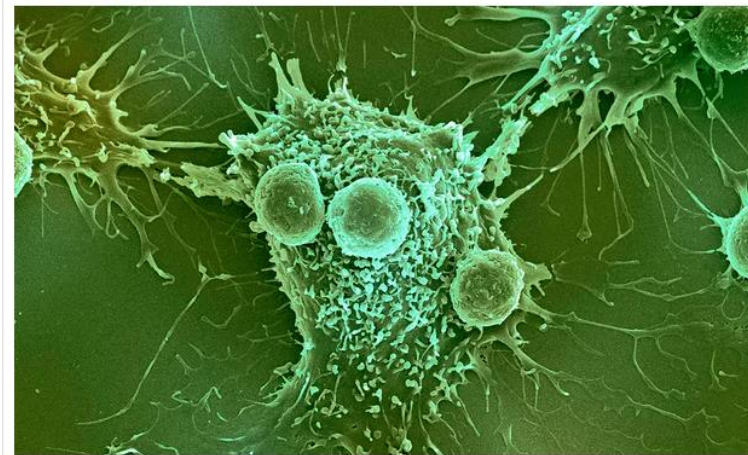
Health service chief calls for affordable access to CAR-T, which modifies immune system to destroy cancer cells

Sarah Boseley Health editor

Thu 26 Apr 2018 18.29 BST



3,954



▲ T lymphocytes and cancer cells. CAR-T therapy trials have put into remission patients who had only months to live. Photograph: Alamy

The NHS is preparing to fast-track a "game-changing" cancer treatment into hospitals, its chief executive has said, calling for the manufacturers to help by setting an affordable price.

# NEWS & ANALYSIS

Nature Reviews Drug Discovery | Published online 9 Mar 2018; doi:[10.1038/nrd.2018.6](https://doi.org/10.1038/nrd.2018.6)

## Tissue-agnostic cancer drug pipeline grows, despite doubts

The FDA could soon approve the first drug developed explicitly for a tissue-agnostic cancer indication, but biological complexity may limit this approach.



# Implications

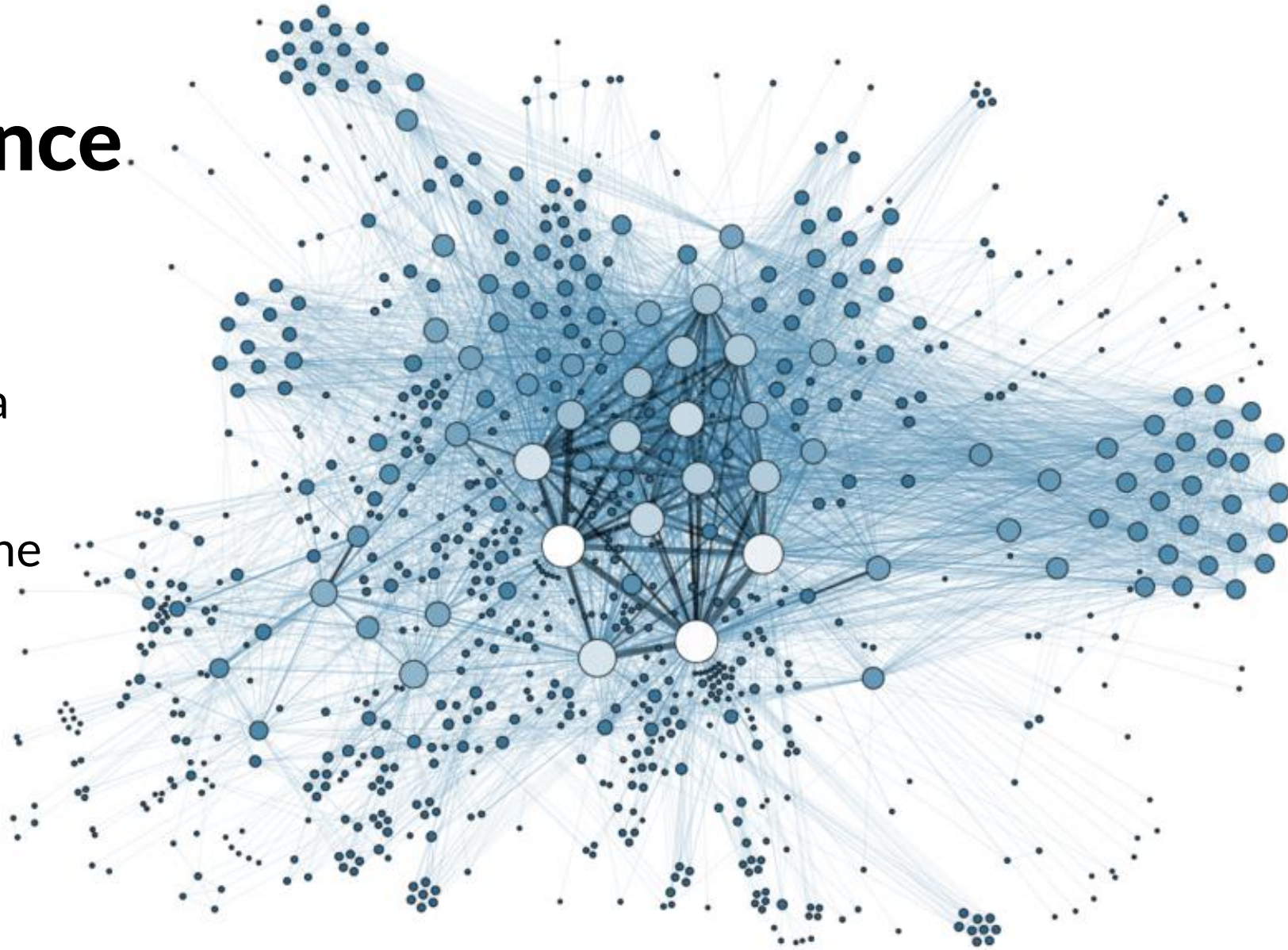


- Regulatory approvals based on:
  - Non-comparative studies
    - Single-arm trials
    - Basket trials
  - Small patient numbers
- Promise of highly innovative treatments...
- ...but with considerable uncertainty (esp long-term outcomes)
- ...and usually, (very) expensive



# Real-world evidence

- Increasing availability of data offers opportunities
- Advanced analytics & machine learning
- Need for quality standards



Grandjean, Martin (2014). "[La connaissance est un réseau](#)". *Les Cahiers du Numérique* 10 (3): 37-54.

# Conclusions

- The changing nature of medical innovations change the evidence available to inform NICE guidance...
- ...accelerating the need for better utilisation of real-world data and advanced analytics
- Quality standards are needed to ensure evidence generated using real-world data and advanced analytics to inform decisions is robust and valid

# Thank you

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