NICE National Institute for Health and Care Excellence

NICE Decision making

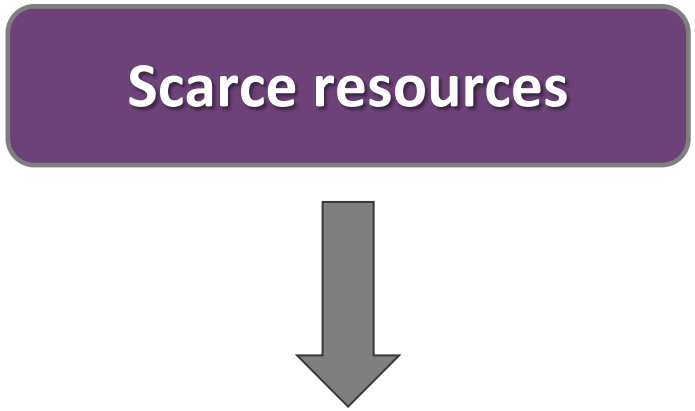
Introduction to health economics

18th October, 2019

Emily Crowe, Senior Scientific Adviser
NICE Scientific Advice programme

In partnership with: EMIG Ethical Medicines Industry Group

Health technology assessment (HTA)



DIFFICULT CHOICES FOR DECISON MAKERS

Need for tools to guide decisions on the most appropriate use of healthcare interventions

Why was NICE created?

To provide standards based on clinical and cost effectiveness

Encourage better use of healthcare resources

To minimise inappropriate variation in practice

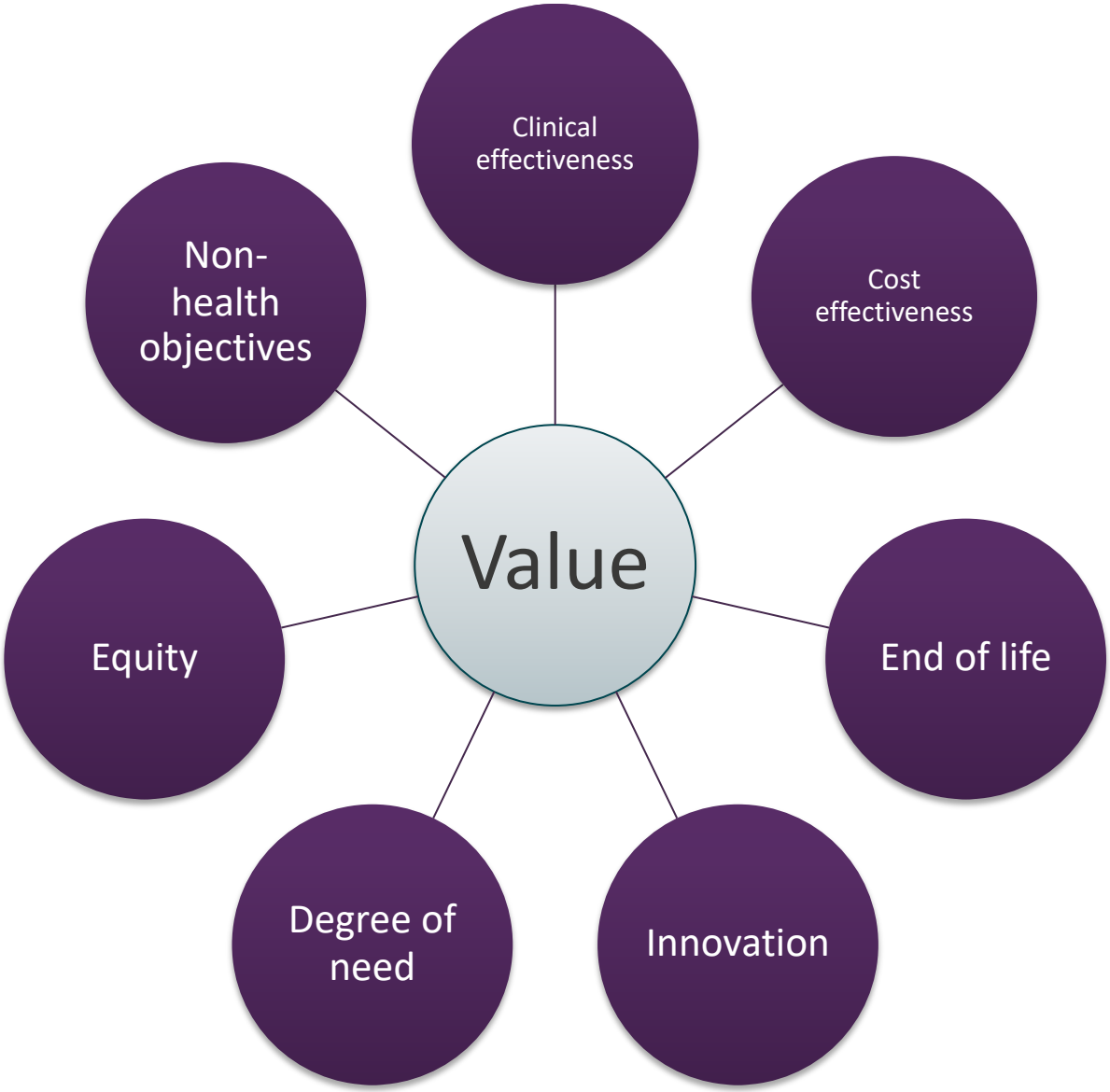
To deal with uncertainty

Speed uptake of interventions that are clinically and cost-effective

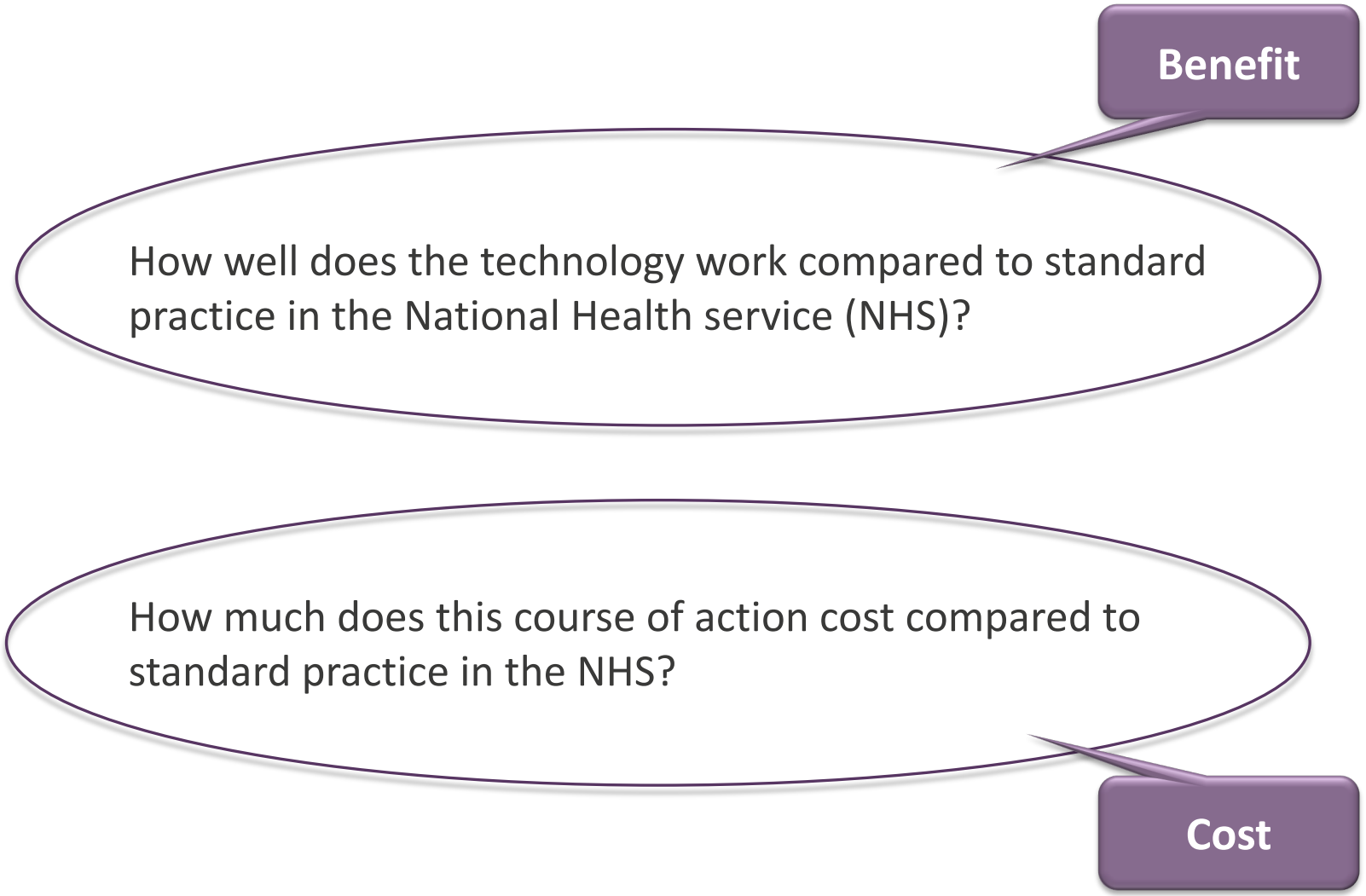
Since 1999
London | Manchester

Encourage more equitable access to healthcare

What does NICE value?



Two key questions asked by NICE



Measuring benefits: The quality adjusted life year (QALY)

- Health care should improve quality of your life and/or increase life expectancy

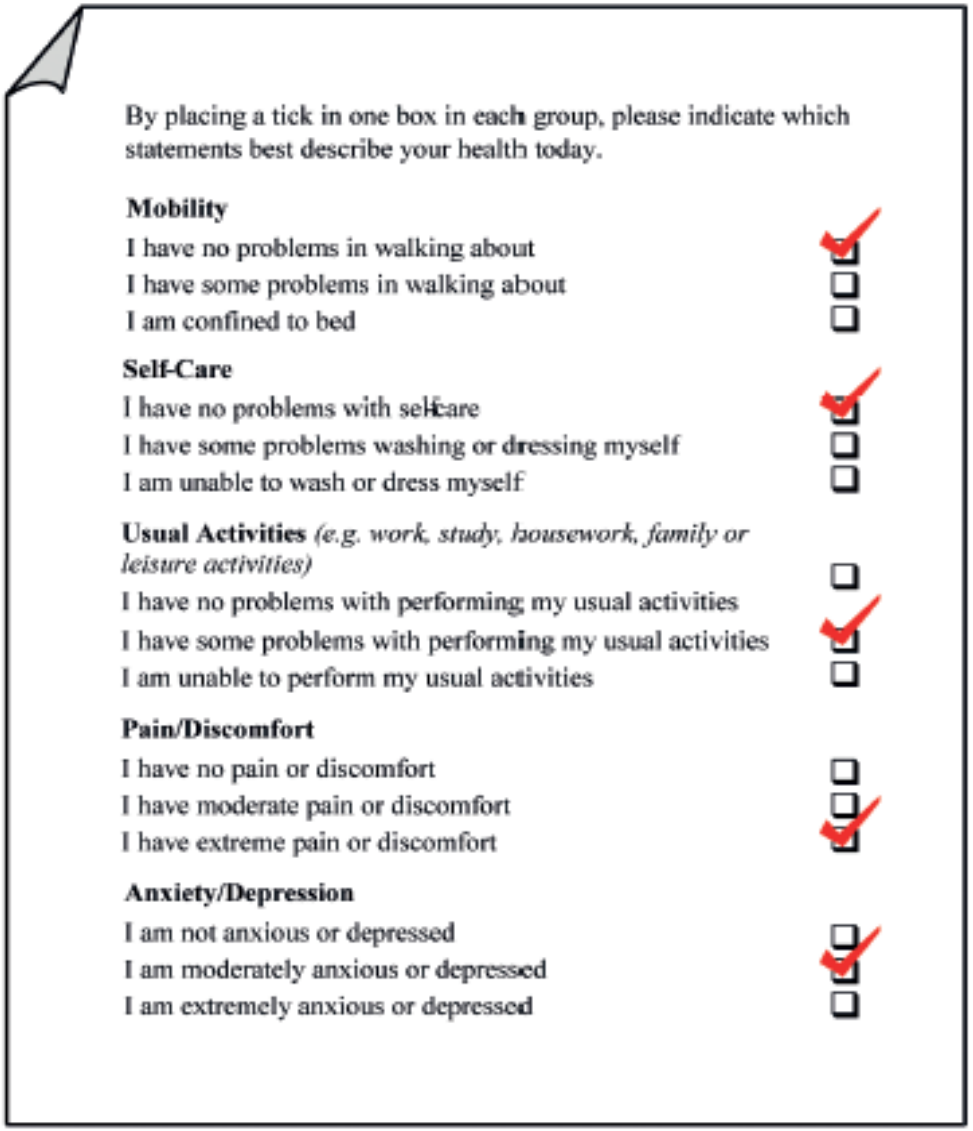
- An index which combines quality of life with life expectancy can be used to compare the benefit of all health care interventions

1 year in perfect health = 1 QALY

Measuring Quality of Life: EQ-5D

http://www.euroqol.org/

Measuring Quality of Life: EQ-5D



By placing a tick in one box in each group, please indicate which statements best describe your health today.

Mobility
- I have no problems in walking about ☑
- I have some problems in walking about ☐
- I am confined to bed ☐

Self-Care
- I have no problems with self-care ☑
- I have some problems washing or dressing myself ☐
- I am unable to wash or dress myself ☐

Usual Activities *(e.g. work, study, housework, family or leisure activities)*
- I have no problems with performing my usual activities ☐
- I have some problems with performing my usual activities ☑
- I am unable to perform my usual activities ☐

Pain/Discomfort
- I have no pain or discomfort ☐
- I have moderate pain or discomfort ☐
- I have extreme pain or discomfort ☑

Anxiety/Depression
- I am not anxious or depressed ☐
- I am moderately anxious or depressed ☑
- I am extremely anxious or depressed ☐

Levels of perceived problems are coded as follows:

☑ ☐ ☐ Level 1 is coded as a '1'

☐ ☑ ☐ Level 2 is coded as a '2'

☐ ☐ ☑ Level 3 is coded as a '3'

NB: There should be only one response for each dimension.

This example identifies the health state 11232

NICE

Measuring Quality of Life: EQ-5D

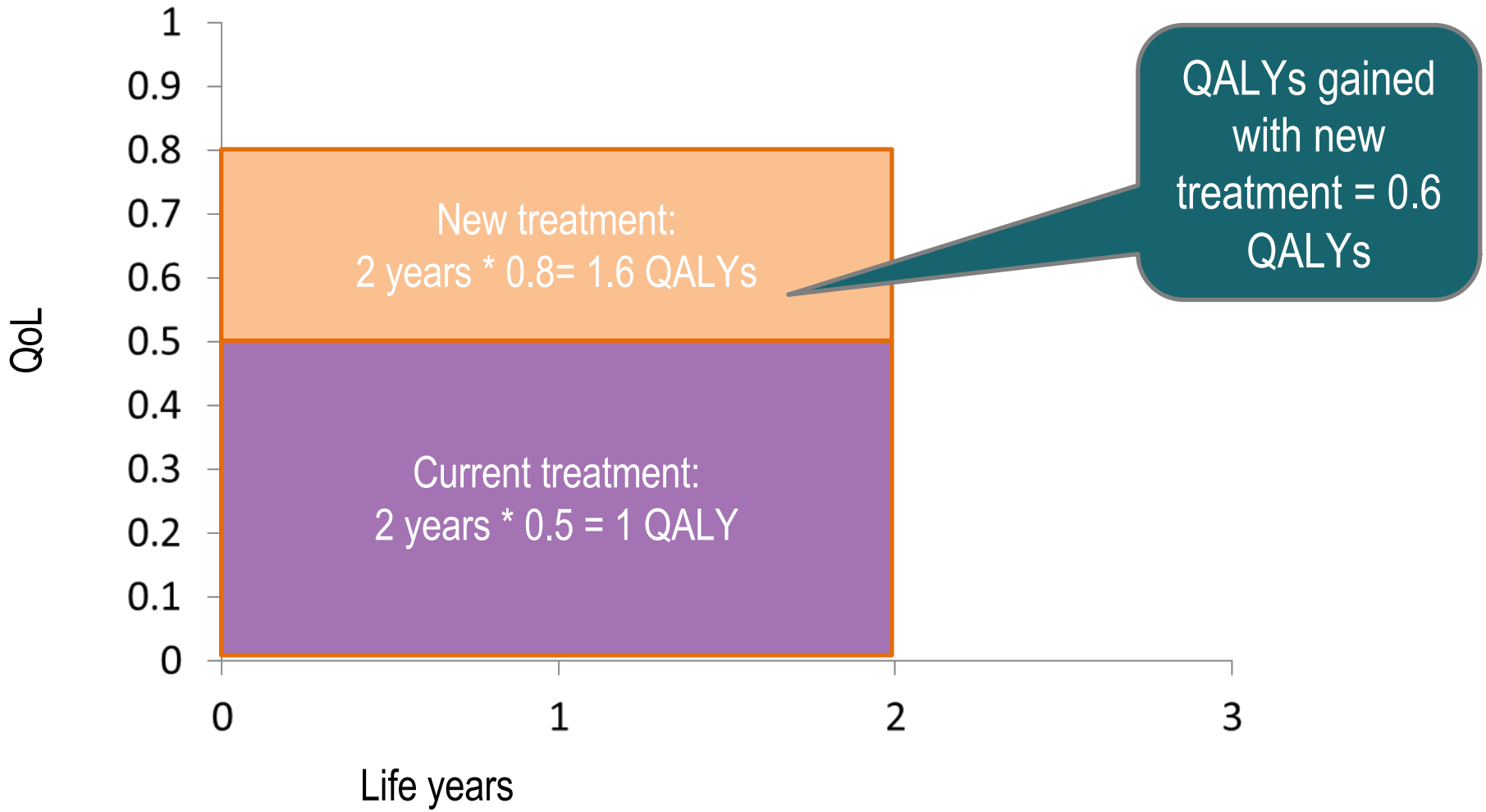
Utility is a number between 0 and 1 that is assigned to a state of health

Calculating QALYs

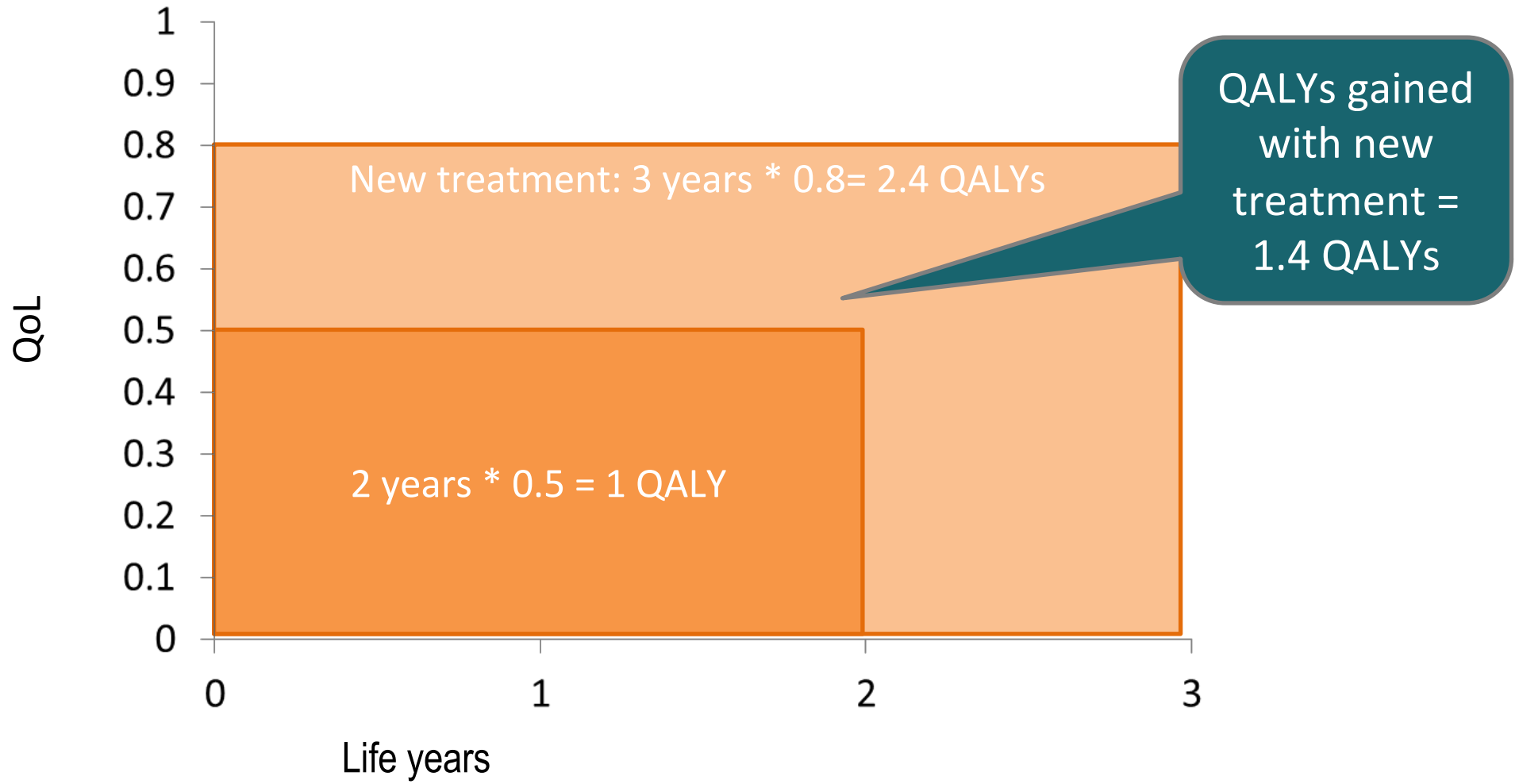
Drug	Years of life on treatment	Quality of Life on Treatment	QALYs
Drug A	10 years	0.5	5 QALYs
Drug B	5 years	1.0	5 QALYs
Device C	4 years	0.2	0.8 QALYs

QALYs enable comparison of products across different disease areas

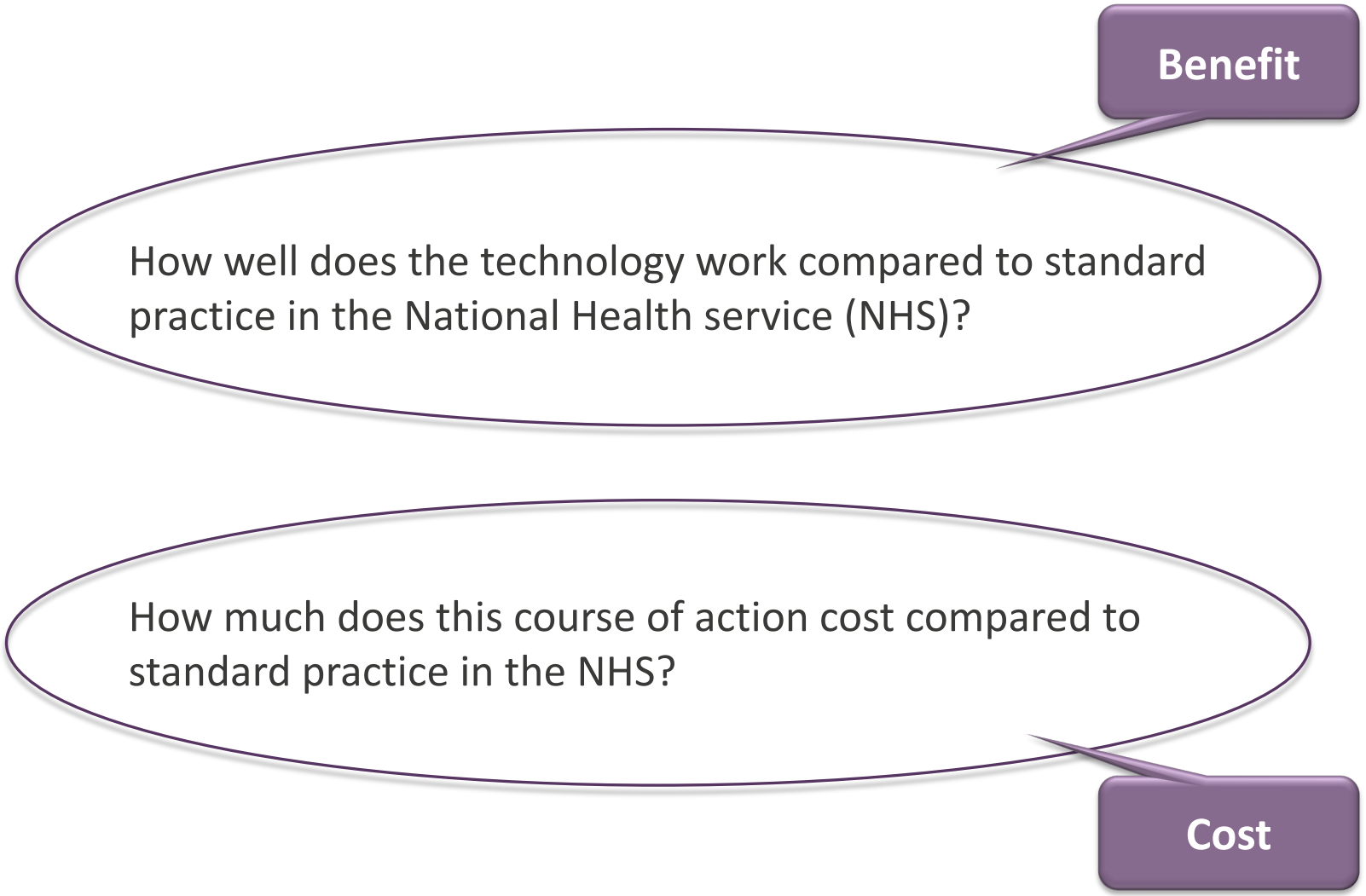
How does NICE measure health benefits?



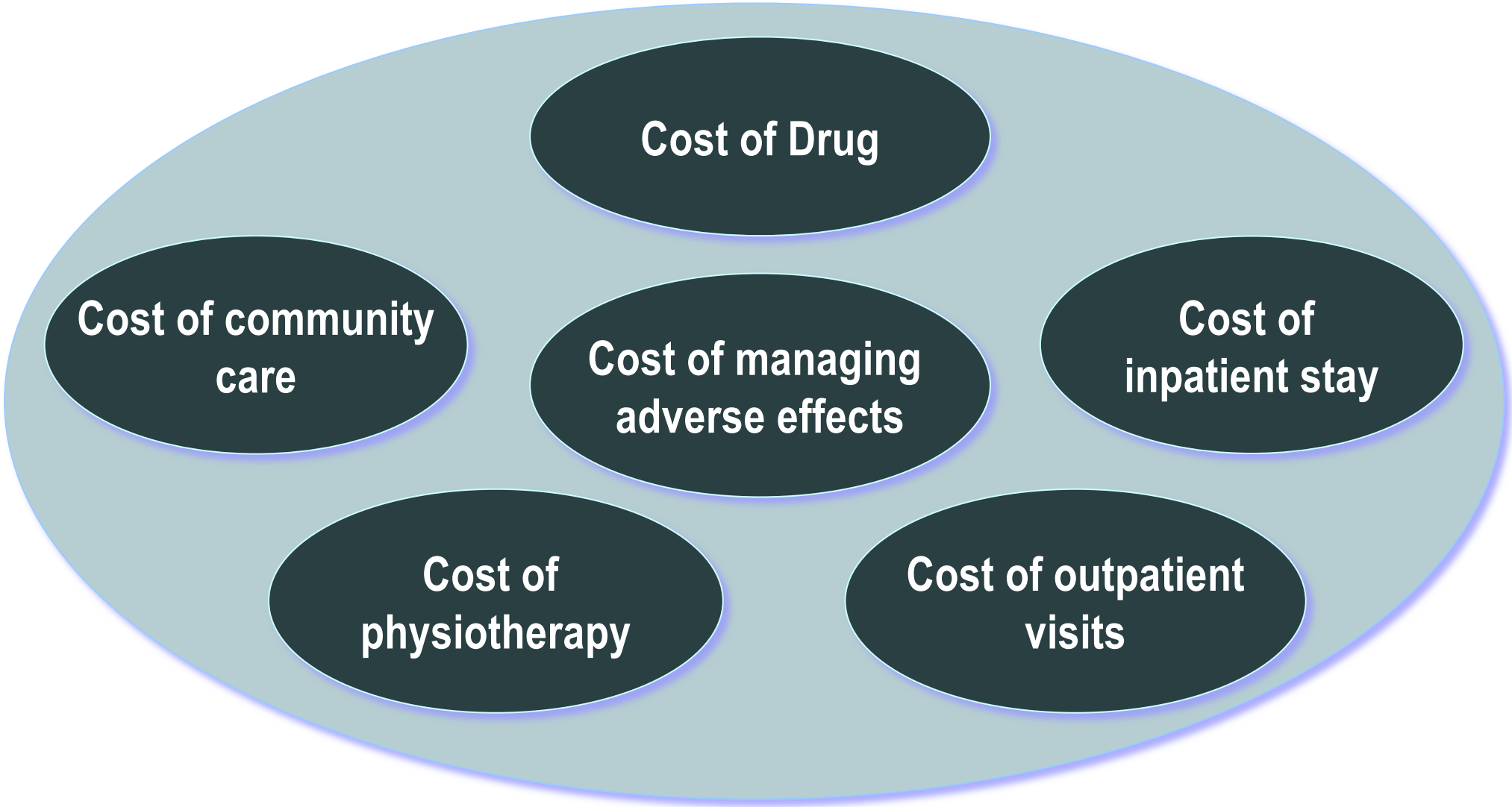
How does NICE measure health benefits?



Two key questions asked by NICE



Costs: ALL the costs associated with the pathway of care

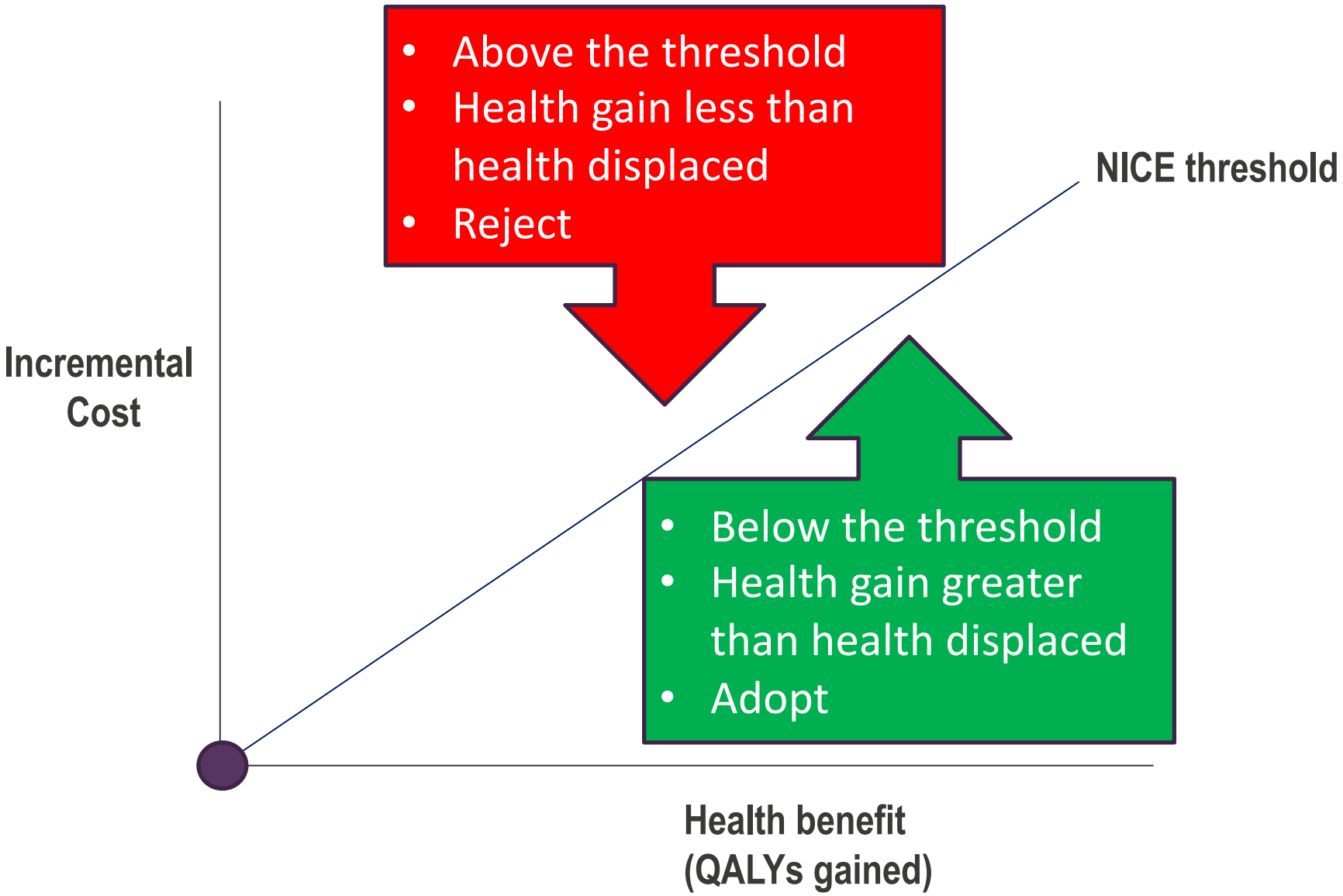


Combining costs and benefits: Incremental cost effectiveness ratio (ICER)

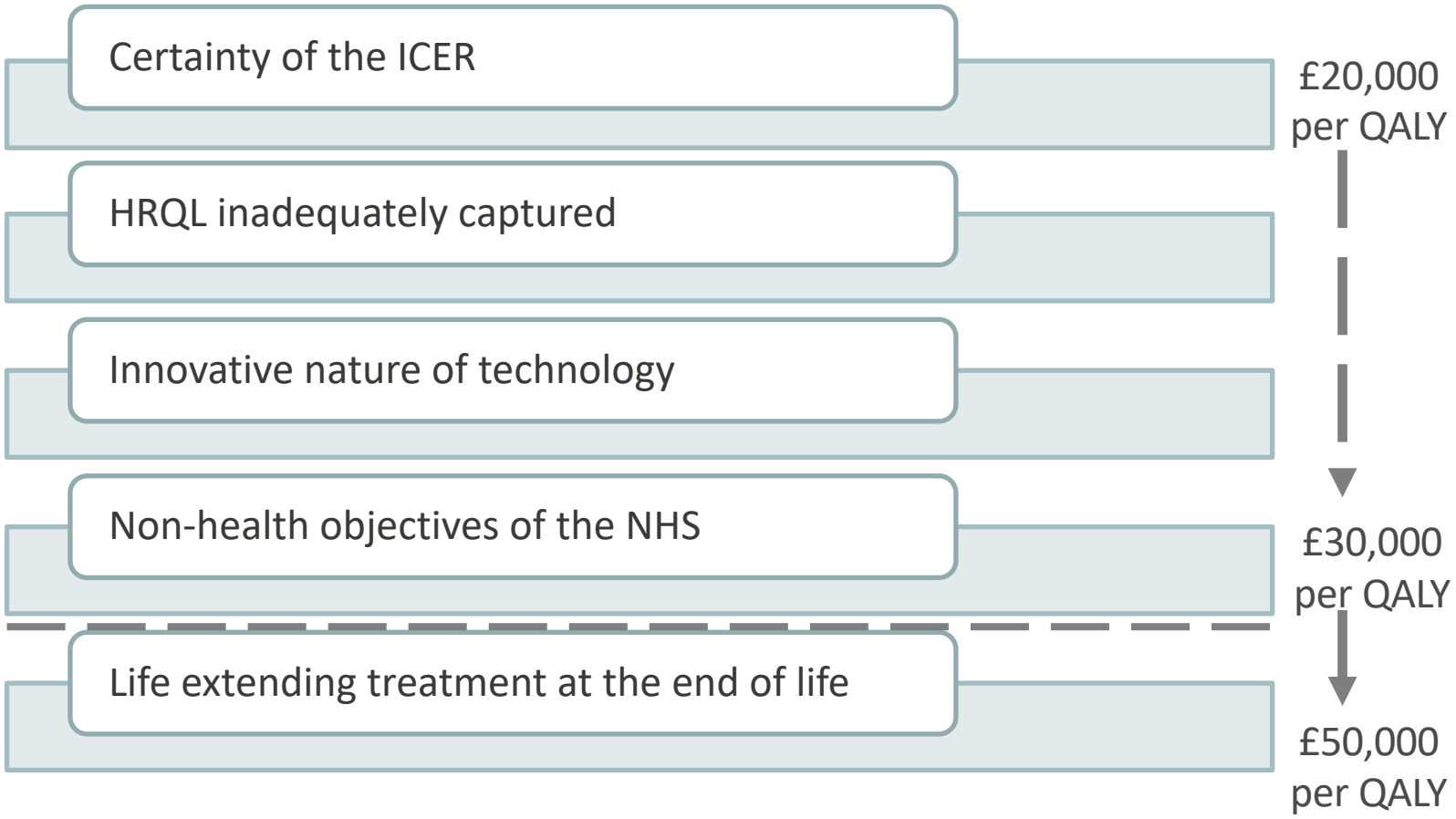
Incremental cost per additional QALY (quality-adjusted life year)

$$\text{ICER} = \frac{\text{New treatment costs} - \text{Current treatment costs}}{\text{QALYs with new treatment} - \text{QALYs with current treatment}} = \text{£/ QALY gained}$$

The ICER threshold



Flexible decision making

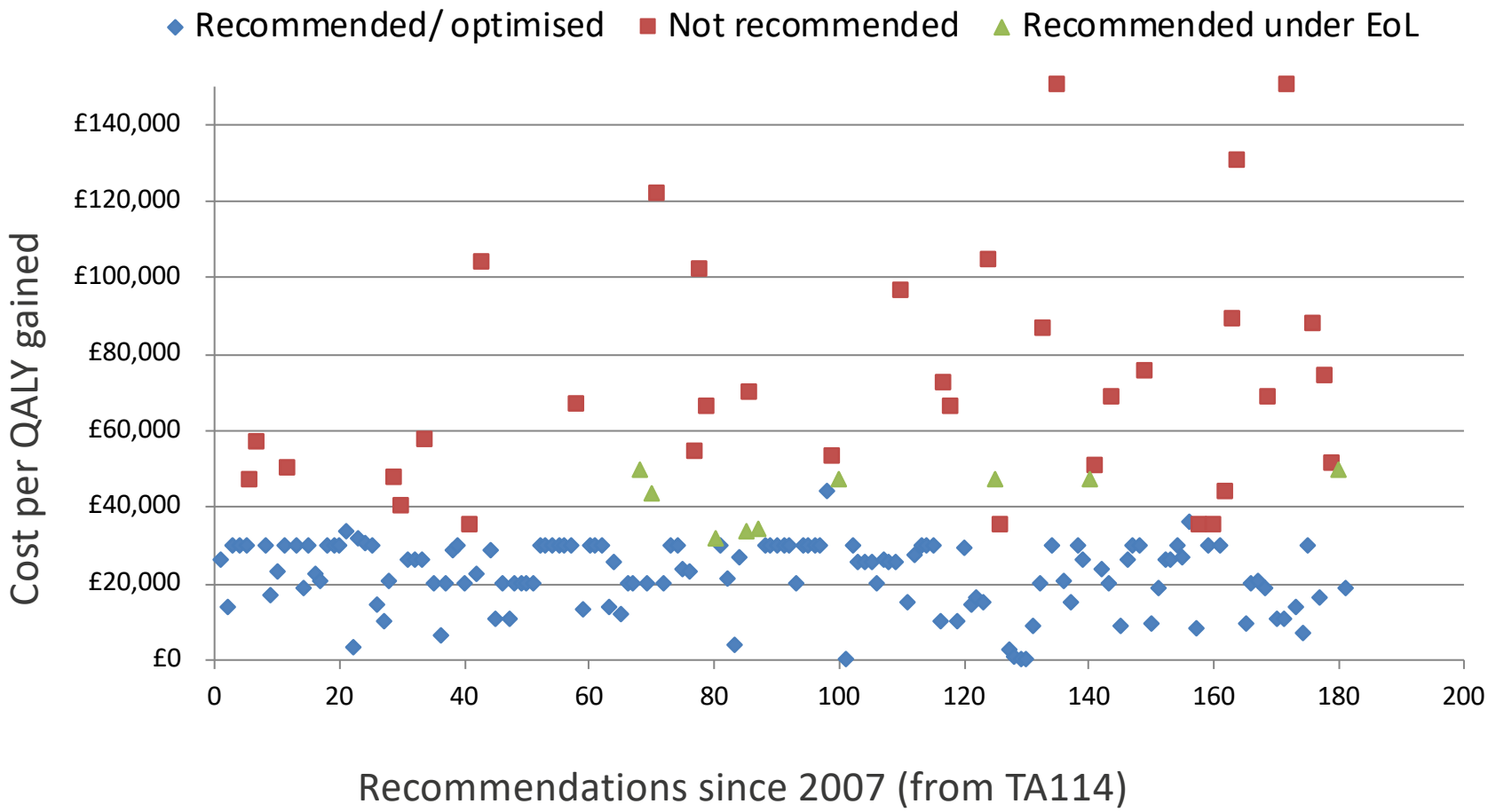


End of life (EOL) criteria

- ✓ The treatment is indicated for patients with a short life expectancy (<24 months)
- ✓ There is sufficient evidence to indicate that the treatment has the prospect of offering an extension to life, (>3 months) compared with current NHS treatment.
- ✓ The estimates of the extension to life are sufficiently robust
- ✓ The assumptions used in the reference case economic modelling are plausible, objective and robust.

ICER used in decision making

Most credible ICER presented for technologies appraised by NICE since 2007



NICE

Current challenges in evidence generation

Fewer comparative randomised clinical trials

- Increasing number of single arm trials and biased historical comparisons (matched case-control methods)
- Cross-over between arms
- Observational / real-world evidence / cancer registries

Conditional approval / exceptional circumstances

- Lack of long term phase 3 or phase 4 studies conducted on clinical outcomes (survival)
- Increased uncertainty

Novel clinical trial designs not specifically designed to inform medical practice

- Adaptive designs (platform trials, group sequential designs, seamless phase 2/3, etc.)
- Other master protocols (basket and umbrella trials)

TA556: Darvadstrocel for complex perianal fistulas in Crohn's disease

- A stem cell therapy indicated for adults with Crohn's disease with a complex perianal fistula who have an inadequate response to at least 1 conventional or biologic therapy

- 1 RCT compared darvadstrocel versus placebo (double-blind)

Limitations:

- No UK centres
- Limited follow-up (reliable up to 52 weeks)
- Lack of HRQL data collected – no EQ-5D

TA556: Clinical effectiveness

Outcome	Darvadstrocel	Placebo	
Remission at week 24	49.5%	34.3%	15.2% difference, p=0.024
Relapse at 1 year	50.8%	59.6%	P=0.0262
Remission at 1 year	27.1%	18.1%	

- The committee concluded that there was a benefit of darvadstrocel compared with placebo, but this is not large, and there are uncertainties about how long the benefit will be maintained.

TA556: Key drivers of cost effectiveness

Remission and relapse rates

- No reliable trial data beyond 2 years
- Small number of patients

Extrapolation of time to relapse and remission

- ICER was highly sensitive to choice of parametric curve
- £20,591/QALY (company's base-case)
- £143,131/QALY (ERG, scenario analysis using 2nd best fitting curve)
- Much uncertainty around the most plausible relapse rate beyond short trial duration

TA556: Recommendation

Darvadstrocel is not recommended, within its marketing authorisation, for previously treated complex perianal fistulas in adults with non-active or mildly active luminal Crohn's disease.

What's new and on the horizon at NICE?

Earlier engagement and resolution of technical issues with goal of single appraisal committee meeting

NICE to review all new drugs approved by regulators and most new indications of approved drugs

Charging for NICE technology appraisals and highly specialised technology (HST) appraisals

Commercial & Managed Access Programme (CMAP)

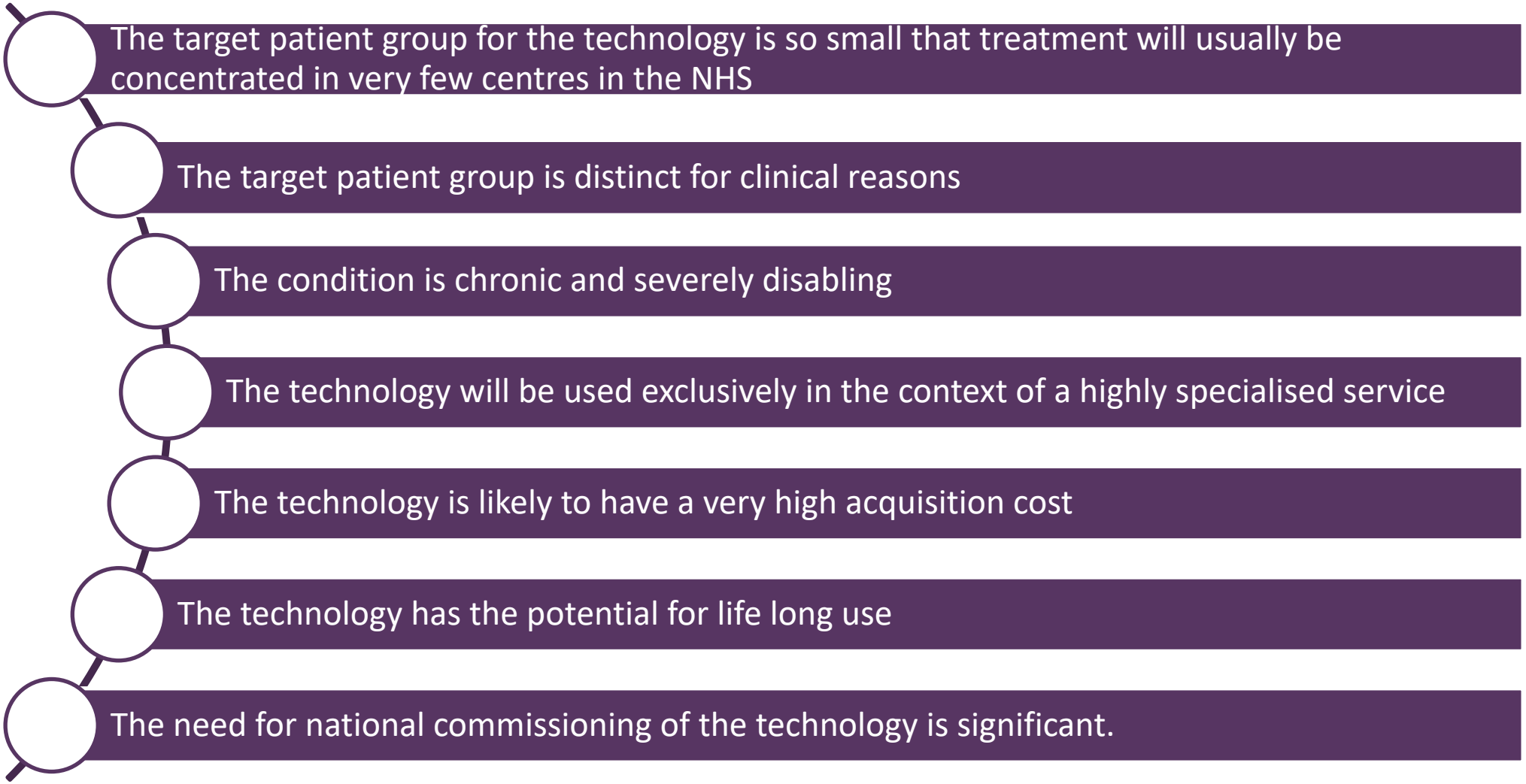
- Optimise NICE/NHS England interactions
- Align TA processes with NHS England Commercial Framework
- From CDF to Managed Access across all technology types

NICE methods review

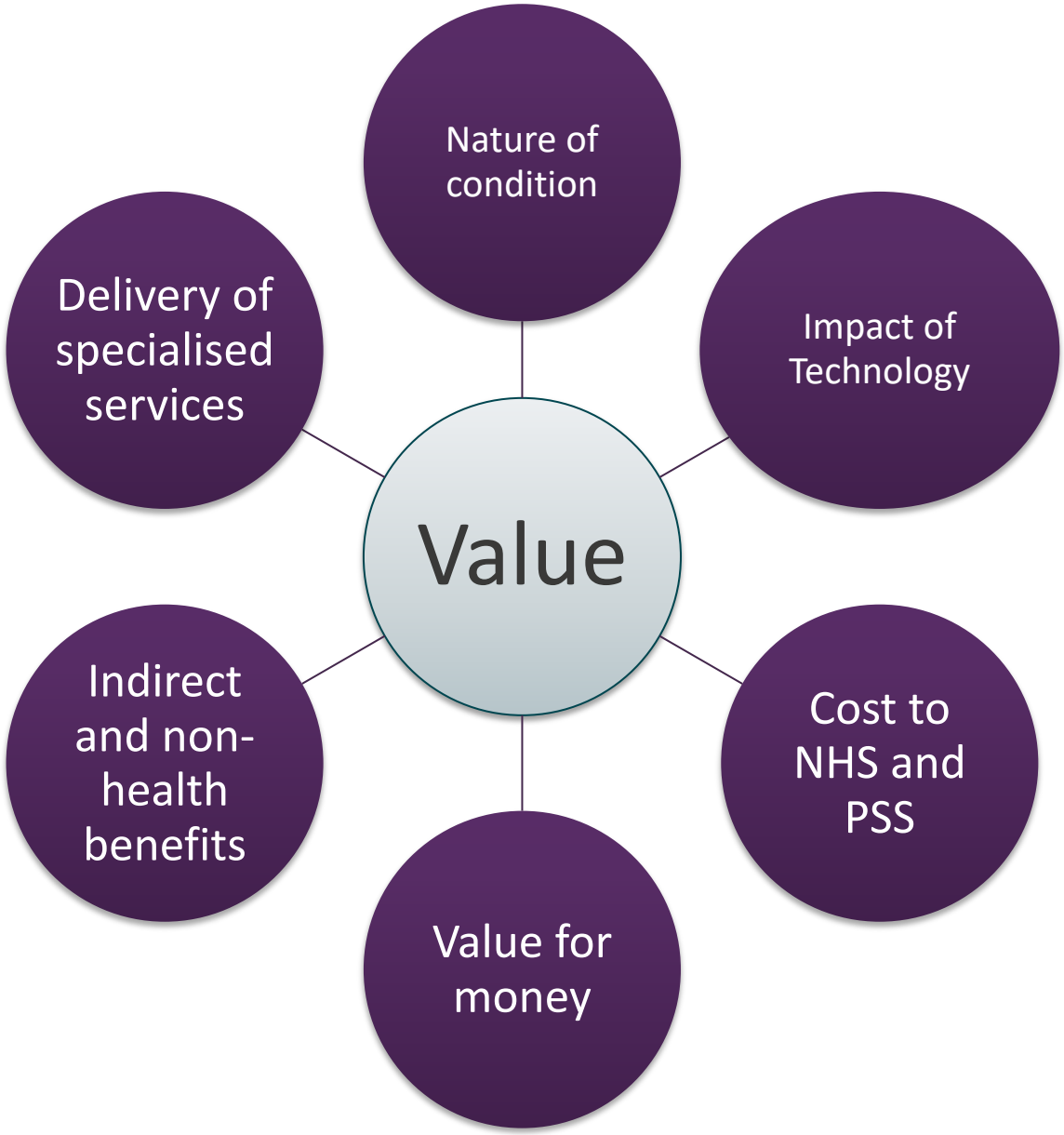
- Alignment of methods across NICE processes
- Development of methods to conduct appraisals under evidentiary challenges

What about rare conditions?

NICE Highly specialized technologies (HST) programme



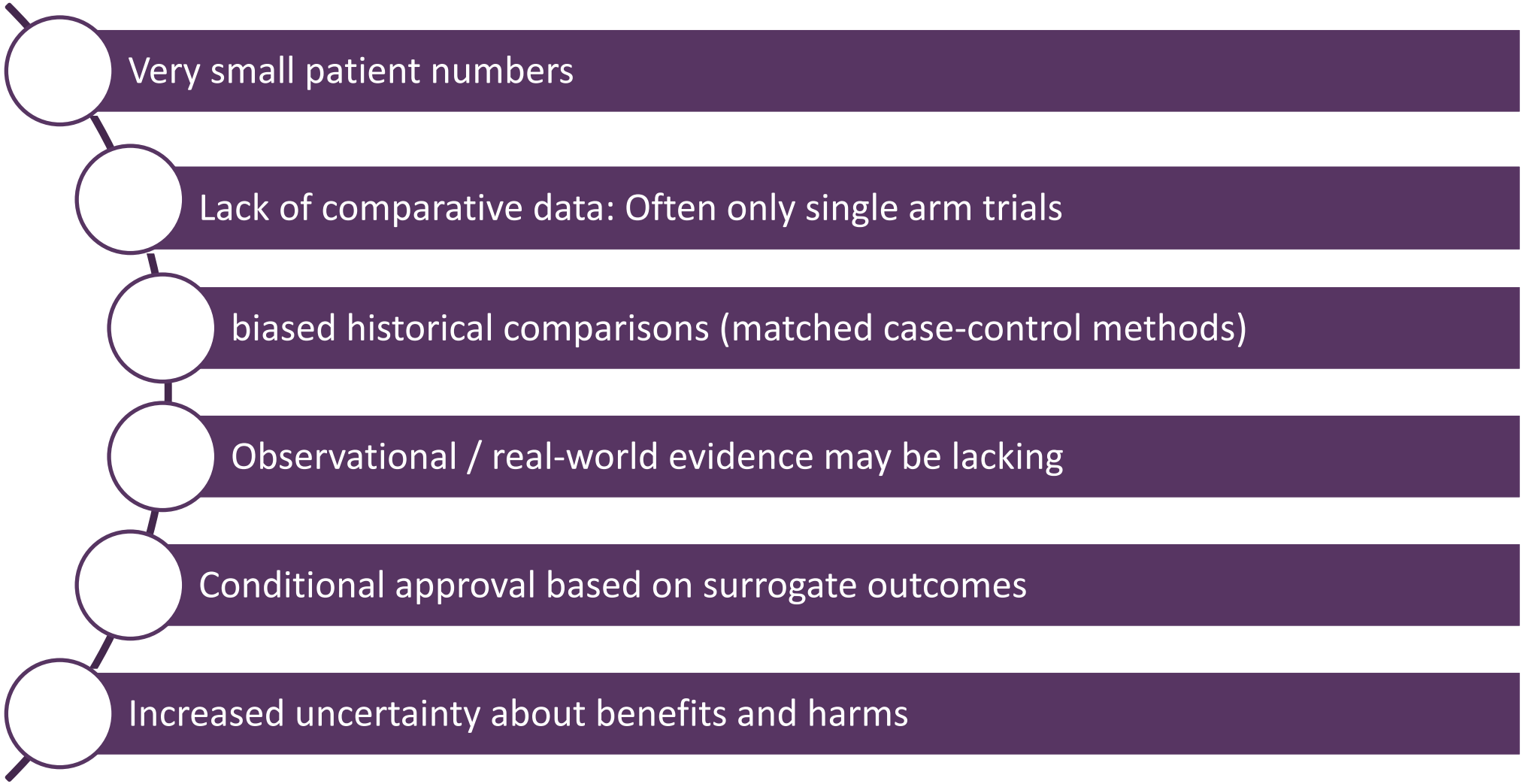
Perspective of a HST evaluation



HST

- Recommends ICERs up to £100K per QALY
- QALY weighting of between 1 and 3 depending on QALY gain
- Weighting requires compelling evidence that the treatment offers significant QALY gains

Current challenges in evidence generation for rare conditions



More information…

NICE Scientific Advice: www.nice.org.uk/scientificadvice

Technology Appraisals: www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance

HST: www.nice.org.uk/About/What-we-do/Our-Programmes/NICE-guidance/NICE-highly-specialised-technologies-guidance