What is HTA

Why patients should be involved

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Some key messages

1. Nowadays, **Health Technology Assessment** (HTA) is a systematic part of a global evaluation of (new and established) medicinal products
2. HTA evaluates **clinical benefits** of all new drugs, medical devices, therapeutic procedures, medical approaches, etc.
3. HTA is **comparative** by nature
4. **European Member States** have a key role in HTA and in pricing/reimbursement of drugs (no European harmonisation so far)

Some key messages

5. Affordability of new medicines requires increased use of limitations in reimbursement and « conditional coverage » (also called “risk sharing schemes”)
6. **Patient** representatives are considered a key actor by some HTA bodies but remain largely “underused” by many others
7. The question of a **centralised, EU-wide HTA** system is « on the table »

Why are patients needed in the HTA process?

Because

1. Evaluation of clinical benefits requires a patient-centred approach
2. HTA bodies are mandated to evaluate “real world” evidence, not only clinical trial data
3. If societal needs are taken into account (in addition to clinical benefits), the citizen voice is a must
4. Patients’ participation in HTA decisions may increase heterogeneity in the decision making body but there are ways to deal with that (e.g. MCDA = Multi-Criteria Decision Analysis)

Increasing hurdles for new medicines

RCTs

EMA

& quality of product

CLINICAL EFFICACY & SAFETY

Comparative data

ADDED THERAPEUTIC VALUE

Real world data & modelling

HTA

HEALTH SYSTEM VALUE

Various

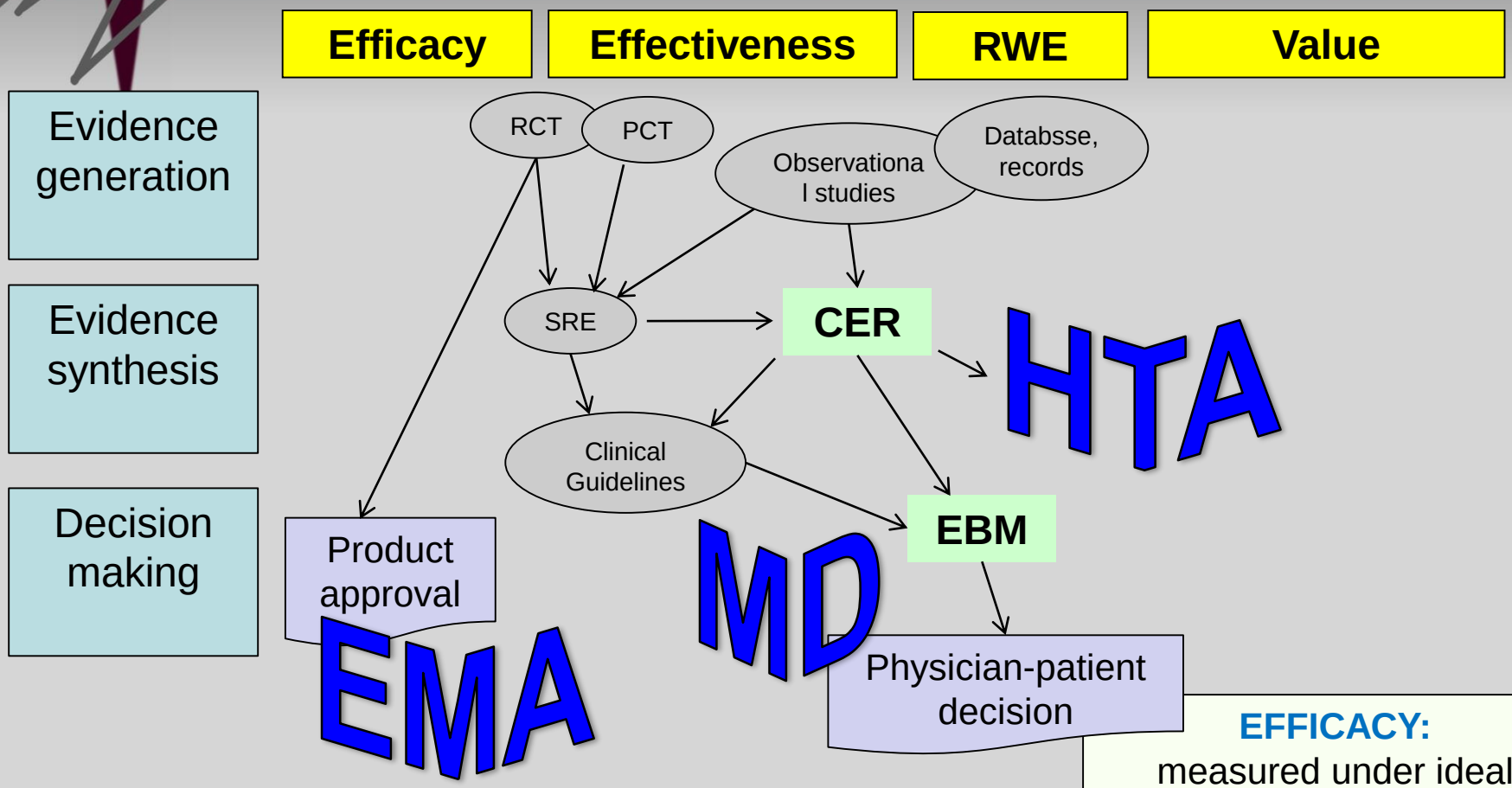
SOCIETAL VALUE

FDA

Complexity of data to demonstrate value

EMA/FDA	France	UK	Sweden
Budget N/A	Drug Budget	Healthcare Budget	Other Budgets (e.g. social security)

Building evidence for new medicines



EFFICACY:
measured under ideal circumstances (clinical trials)

EFFECTIVENESS:
usual circumstances of health care practice

RCT/PCT: randomised/practical clinical trial
SRE: systematic review of evidence
RWE: real world evidence
CER: comparative effectiveness research
EBM: evidence-based medicine

***Adapted from*: Luce et al., Milbank Quaterly 2010**

Real world evidence

"Real World" data is anything OTHER than RCT generated data…..data derived from:

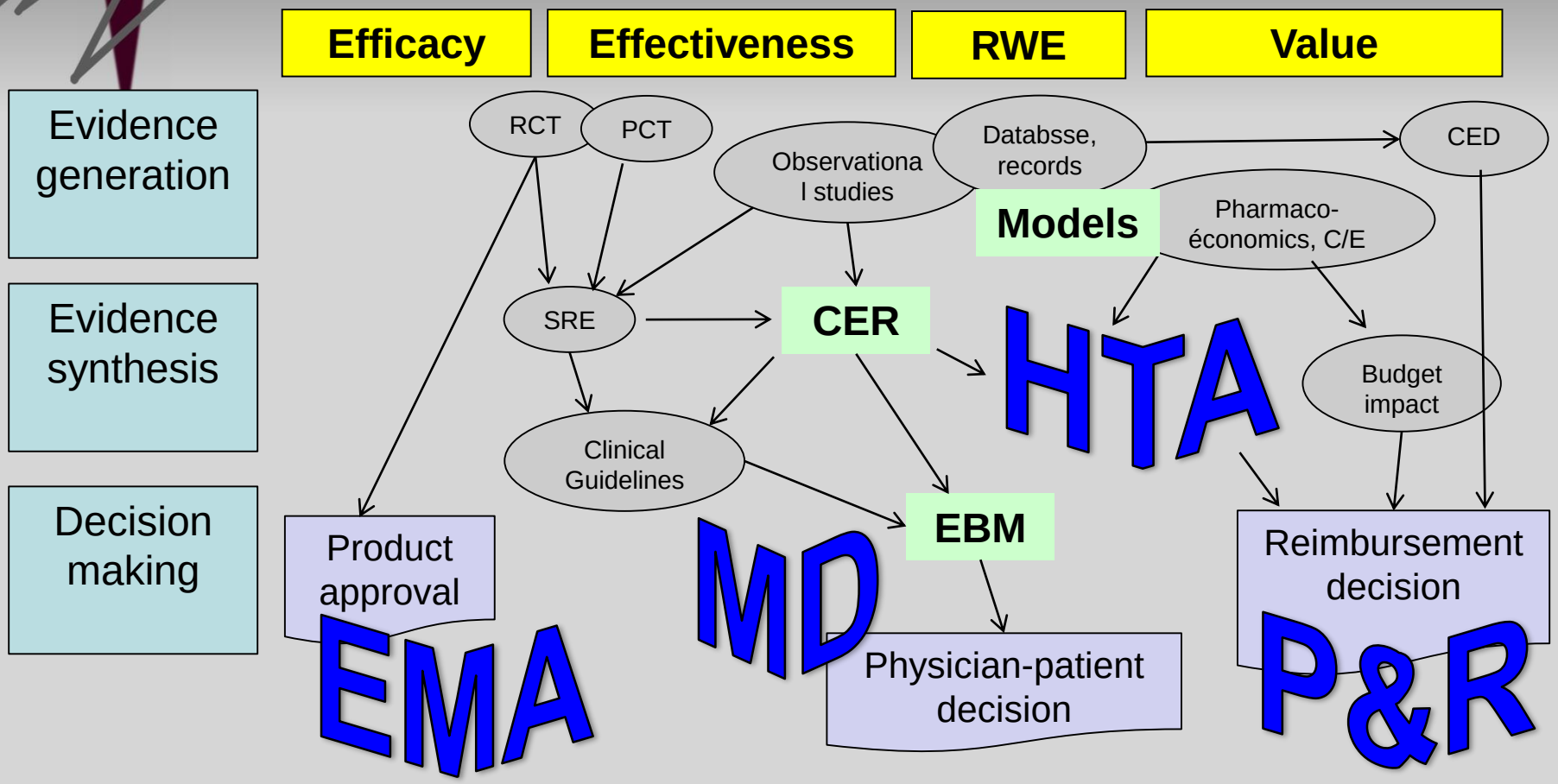
- Prospective observational studies
  - Non-interventional observations
- Database studies
  - Prospective registries create a database
  - Retrospective databases created for other reason
- Medical records
  - Data abstraction

Quality of data collection & analysis is key

In general, "real world data" are observations of effects based on what happens after a prescriptive (treatment) decision is made where the researcher does not or cannot control who gets what treatment and does not or cannot control the medical management of the patient beyond observing outcomes

Lou Garrison & Andreas Pleil,
ISPOR RWD Task Force, 2007

Building evidence for new medicines



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SRE: systematic review of evidence
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CER: comparative effectiveness research
EBM: evidence-based medicine
C/E: cost/effectiveness
CED: coverage with evidence development

Adapted from: Luce et al., Milbank Quaterly 2010

History of HTA in Europe

1970	Evaluation of medical technologies (EMT) in Sweden, UK, France, NL: use of "scientific standards"
1985	International Society on Technology Assessment in Health Care (ISTAHC) and its journal (Egon Jonsson & Stanley Reiser) – became HTAi in 2004
1987	First national HTA agencies (Sweden, NL, FR, Catalonia, CH…)
1993	International Network of Agencies for HTA (INAHTA)
1994-7	EUR-ASSESS, first large EC-funded collaboration project on HTA, includes harmonisation of HTA methodology, how to link the results of HTA to coverage, etc.
1995	ISPOR: International Society for Pharmacoeconomics & Outcomes Research
1999	NICE (UK): "guidance for best practice" based on findings from research (scientific excellence)
2004	IQWIG (DE): Institute for Quality and Efficiency in Healthcare HAS (FR): Haute Autorité de Santé
2005	EUNetHTA project co-funded by EC & participating partners organisations

Definition of HTA

- Multidisciplinary activity for…
- the systematic examination of…
- technical performance, safety, **clinical** efficacy and effectiveness, cost, cost-effectiveness, social consequences, legal and ethical considerations…
- …of any health technology (usually a drug, medical device, or clinical/surgical procedure)
- Health economics evaluation not always included (e.g. IQWIG in Germany)
- Provides policy makers with accessible, usable evidence-based information
- Most HTA agencies are advisory bodies, not regulatory authorities

From: Taylor & Taylor, 2009 (UK)

Regional fragmentation of HTA/reimbursement

Regulators

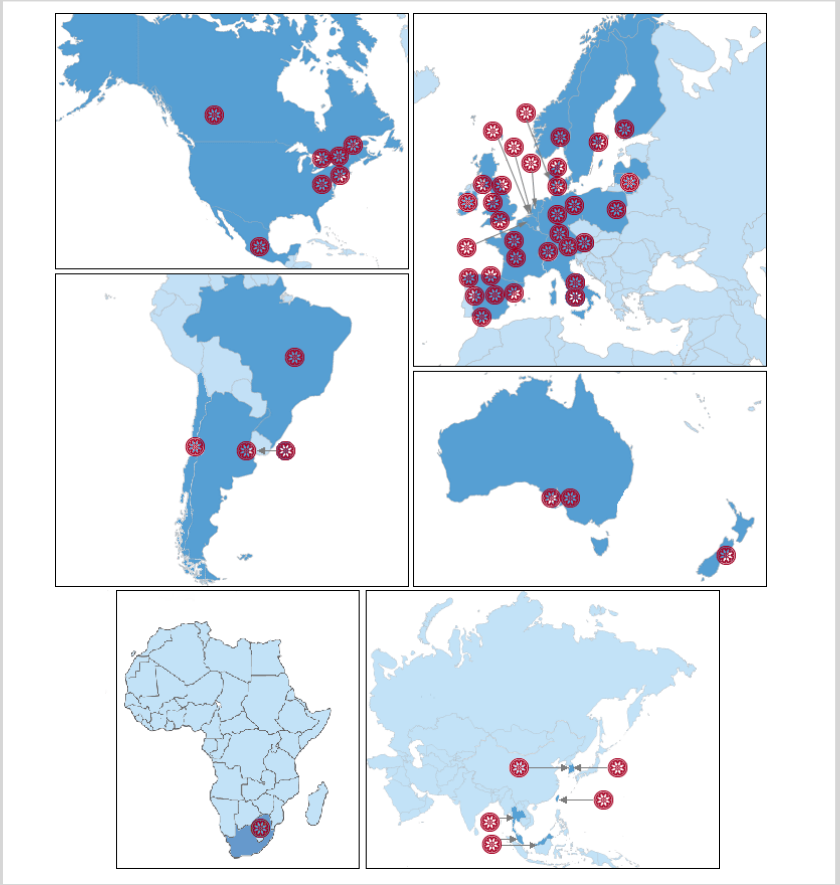
ICH
harmonisation for better health

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

FDA

Pmda
JAPAN

INAHTA Members



59 members from 23 countries

- In the 28 EU Member States, **the State** has the responsibility for providing health care and universal coverage (whether tax- or insurance-based)

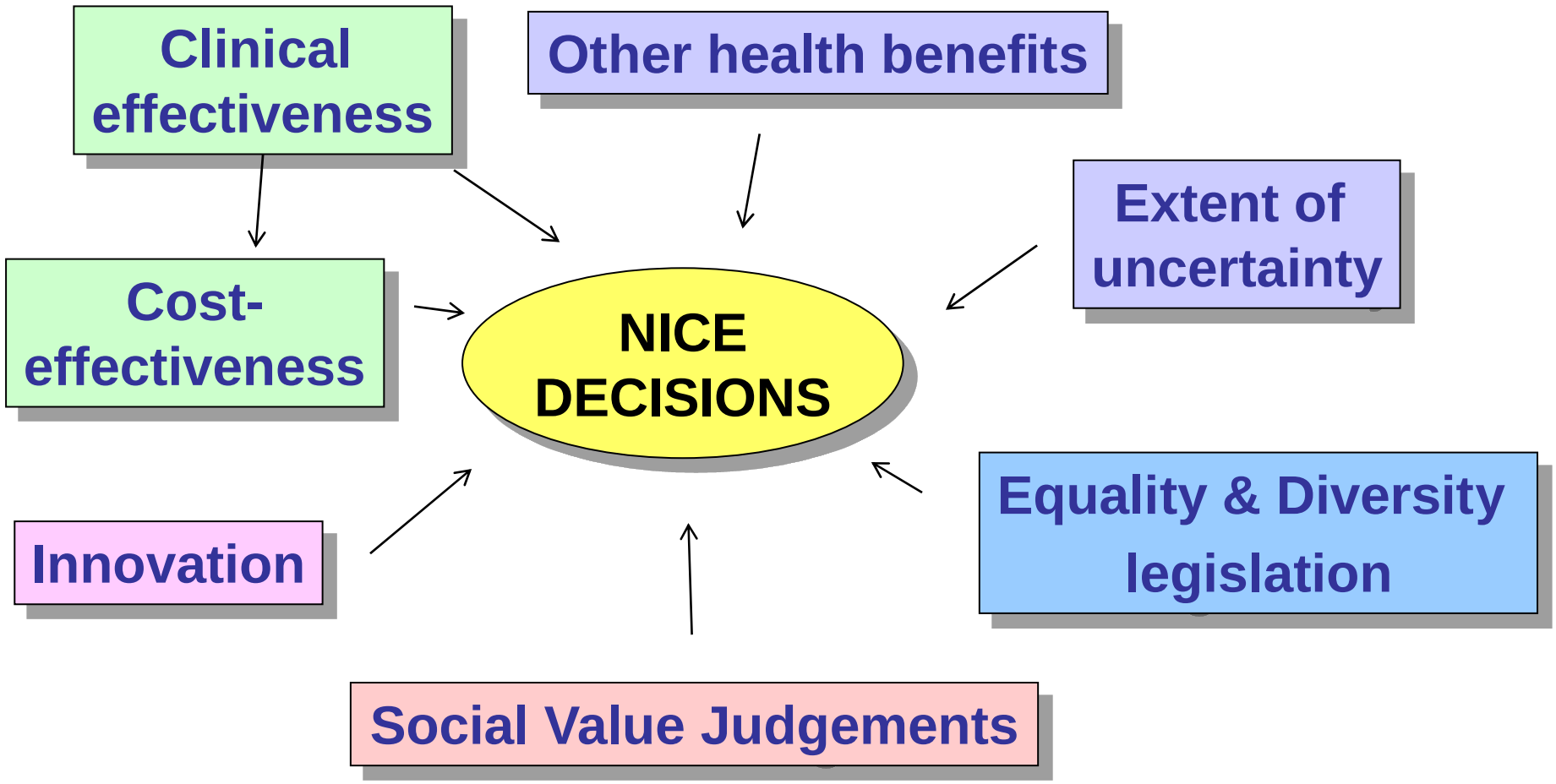
- Need to consider country-specific circumstances
  - structure of healthcare system
  - financing of healthcare (e.g. « Bismarck-type » = social health insurance {NL, DE} vs « Beveridge-type » = tax-funded {SE, UK})
  - demography
  - available resources, wealth

2 major questions addressed by HTA

- Clinical effectiveness
  - *How do health outcomes of the technology compare with available treatment alternatives ?*
  - *Health outcomes may be measured as QALYs (Quality-Adjusted Life Years)*

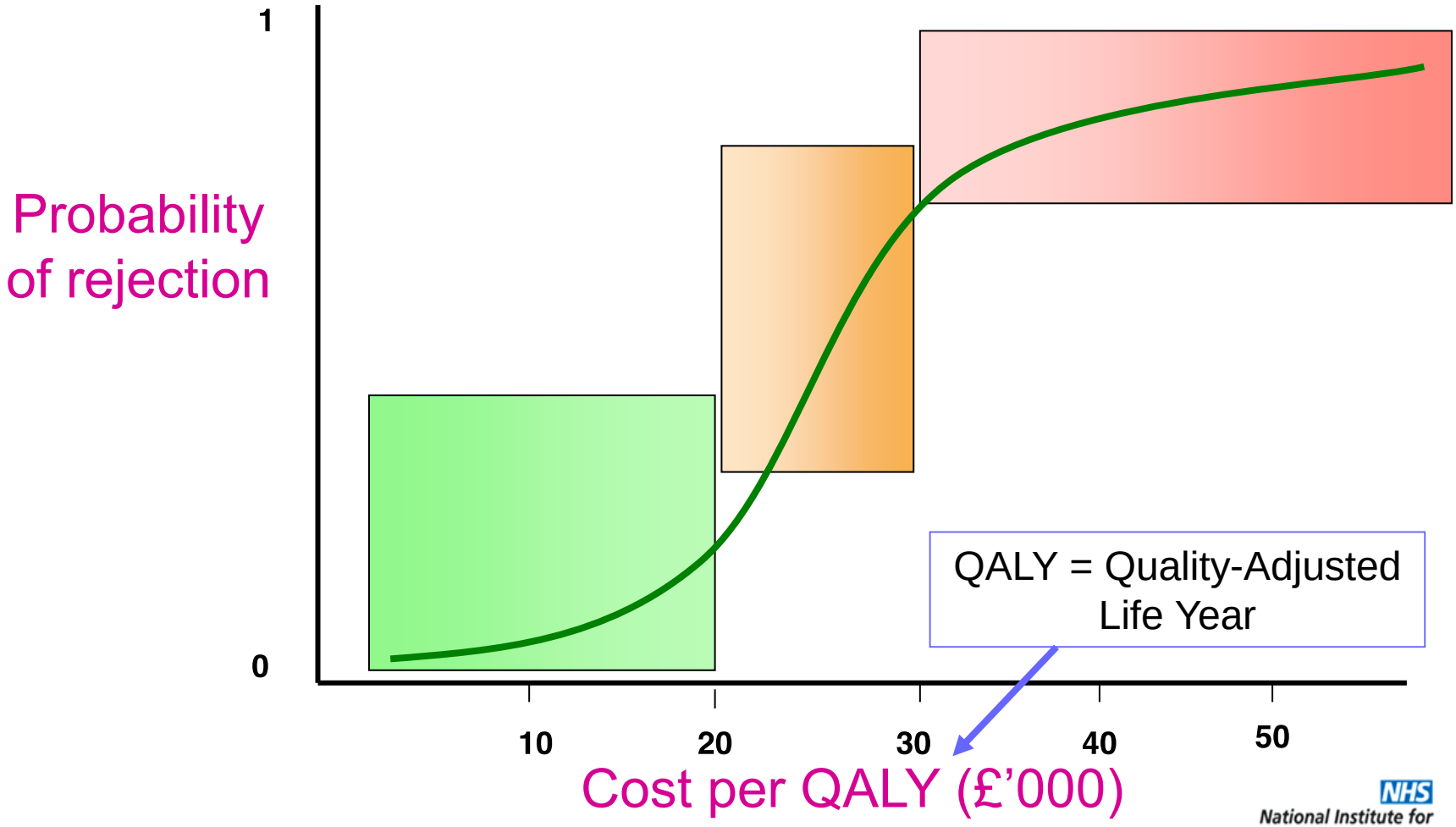
- Cost-effectiveness
  - *Are these improvements in health outcomes commensurate (matching in size, importance, quality) with the additional costs ?*

NICE (UK) criteria for decision making



NHS National Institute for Health and Clinical Excellence

NICE rejection rates based on cost-effectiveness



NHS
National Institute for Health and Clinical Excellence

Current challenges for HTA of new medicines

- Limited clinical trial data (surrogate endpoints, short follow-up, no active comparators)
- **Lack of** effectiveness data, real world evidence, patient oriented outcomes
- Need to compare with all existing therapies for the same indication
- What patient subgroups benefit the most?
- *Oncology:* complex treatment pathways, high medication costs
- Complex decision-making group: medical assessors, health economists (in many countries), clinical experts with low levels of conflicts of interest, + **ideally patients**
- Most (but not all) “payers” rely on HTA
- In the end, payers must reject some products (affordability)

Why are patients needed in the HTA process?

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Thank you !!