Health Technology Assessment: can Japan learn from England's successes and mistakes?

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Outline of today's lecture

- HTA and priority setting: defining the terms
- HTA: global momentum growing
- NICE in the UK: an overview
- Health service costs matter!
- NICE and drug pricing
- Vertical funds: more trouble than they are worth?
- HTA, strategic purchasing and quality improvement
- Reflections on Japan

DEFINING THE TERMS

What is Health Technology Assessment?

- HTA is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. HTA answers clinical questions of new, potential innovative, healthcare technologies such as: **How well does a new technology work compared with existing alternative health technologies?** For which population group does it work best? HTA can also answer economic questions like: **What costs are entailed for the health system?** It is therefore a considered key tool for decision makers to ensure the accessibility, quality and sustainability of healthcare.
-

  A **health technology** is defined as an intervention that may be used to promote health, to prevent, diagnose or treat acute or chronic disease, or for rehabilitation. Health technologies include pharmaceuticals, devices, procedures and organizational systems used in health care."
- *OR…*
- "**Health technology assessment (HTA) is a tool to review technologies and provide evidence of the value these technologies can deliver to patients and their families, health system stakeholders, and to society more broadly**."

Using HTA to inform priority setting

HTA can form an integral part of a *process* for considering *scientific evidence, economic evidence* and *social values*, to directly inform coverage and policy decisions relating to *healthcare interventions*

- drugs, devices, diagnostics, surgical interventions and services, both preventative and curative/palliative
- but also service delivery models, programmatic reforms, health and public policy interventions (e.g. smoking cessation).

Should include economic evaluation (EE)/ cost-effectiveness analysis (CEA); not just clinical effectiveness as waste costs lives

- **drawing comparisons**: compared to the status quo, what do we gain out of the new intervention, and at what extra cost?

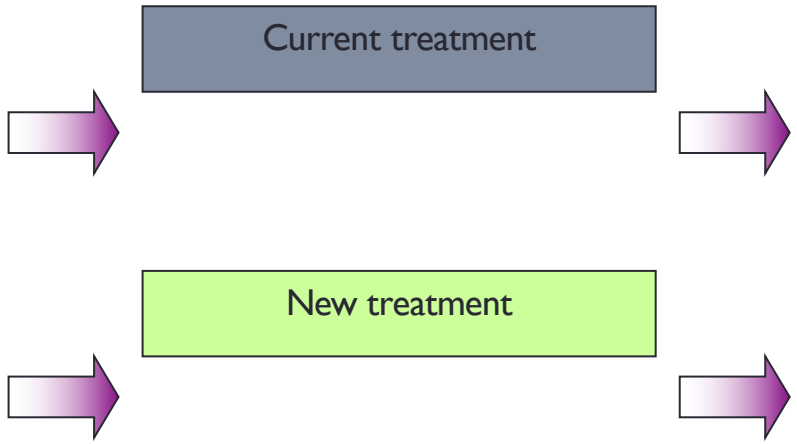
Not just a technical exercise: the process and social values are equally important

it must carry budgetary implications; ie it must have teeth to make a positive difference

Using Economic evaluation

Drummond, Stoddart & Torrance, 1987

"... the comparative analysis of alternative courses of action in terms of both their costs and consequences."



Costs
value of extra resources used (loss to other patients)

Consequences
value of
health gain for this patient group

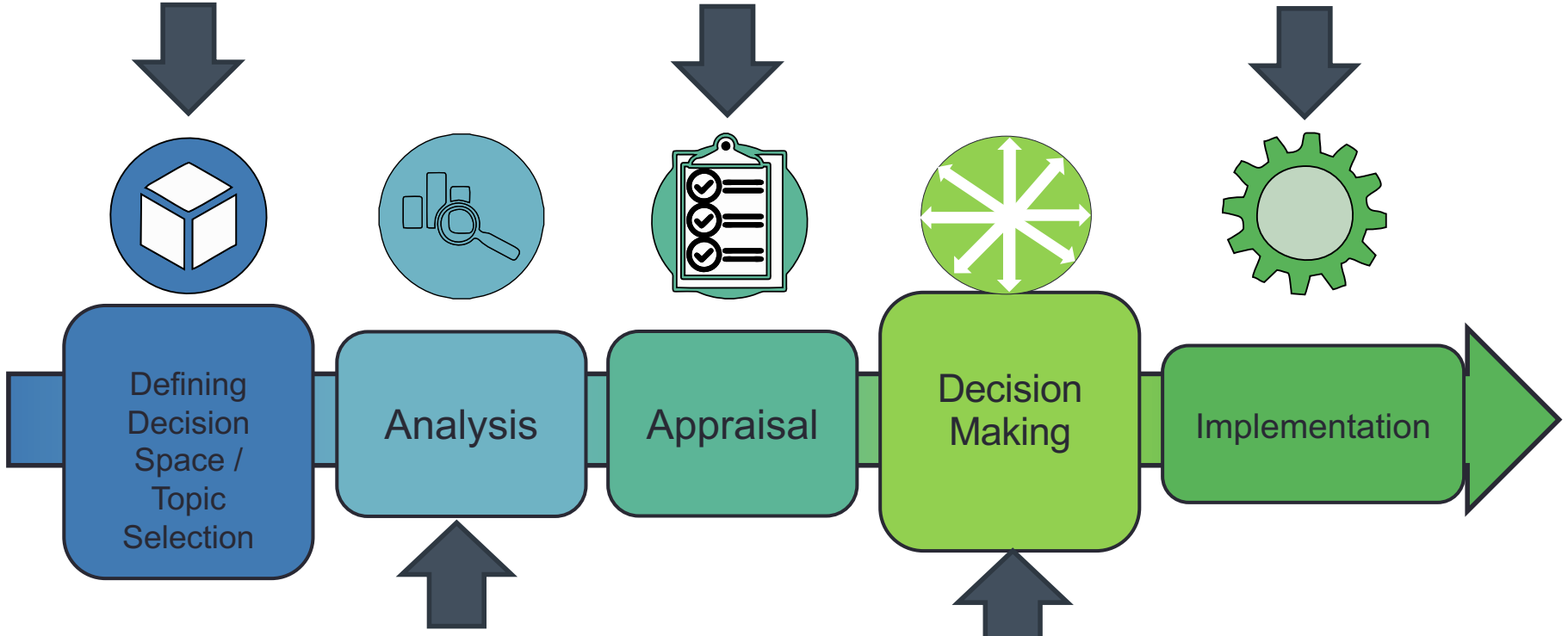
Analysis should be conducted separately for each subgroup of patients.

The HTA Process

What is the Decision problem? Topic identification and Prioritisation

How do we decide if the evidence is strong enough to support a decision? What are our recommendations?

How is the decision implemented and monitored?

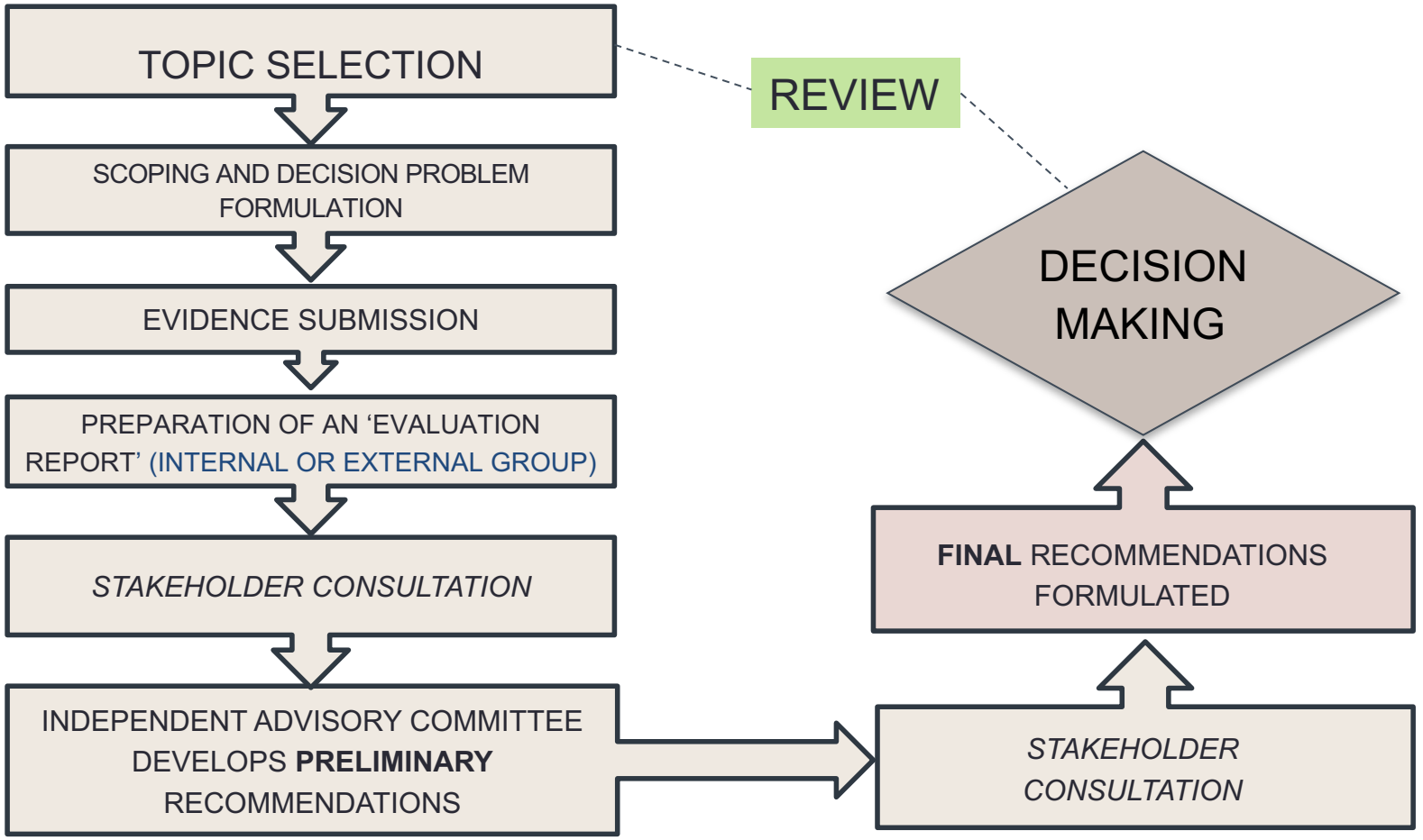


What is the required analysis needed to help answer the decision problem?

What is the decision to be taken?

Source: PRICELESS, South Africa

The HTA process in more detail…



HTA IS NOW A GLOBAL MOVEMENT…

World Health Organization

SIXTY-SEVENTH WORLD HEALTH ASSEMBLY
Provisional agenda item 15.7

A67/33
14 March 2014

Health intervention and technology assessment in support of universal health coverage

World Health Assembly resolution on Health Intervention and Technology Assessment, 2014

"to integrate health intervention and technology assessment concepts and principles into relevant strategies and areas…including, but not limited to, universal health coverage, health financing, access to and rational use of quality-assured medicines, vaccines and other health technologies, the prevention and management of non-communicable and communicable diseases, mother and child care, and the formulation of evidence-based health policy"

World Health Organization

Regional Office for South-East Asia

REGIONAL COMMITTEE

Provisional Agenda item 8.3

Seventieth Session
Maldives
6–10 September 2017

SEA/RC70/9

21 August 2017

Access to medicines

"Evidence helps when **negotiating price and rules on reimbursement**, which in turn affect access. Health technology assessment is a routine part of the decision-making process for adding medicines to the national benefit package in Thailand, and other countries such as Indonesia and India are introducing this approach."

HTA is becoming a major tool for priority setting and price negotiations for national governments in emerging markets...

National Health Insurance Act of 2013, Section 11- Excluded Personal Health Services

Philippines: "The Corporation shall not cover expenses for health services which the Corporation and the DOH consider cost-ineffective through health technology assessment…"

Indonesia: Minister of Health's Decree No. 71 /2013 Article 34

(5)Health Technology Assessment Committee **provide policy recommendation to the Minister on the feasibility of the health service** as referred to in paragraph (4) **to be included as benefit package of National Health Insurance**

national treasury
Department:
National Treasury
REPUBLIC OF SOUTH AFRICA

Service coverage (5.3):

South Africa "Detailed treatment guidelines, based on available evidence about cost-effective interventions, will be used to guide the delivery of comprehensive health entitlements. Treatment guidelines will be based on evidence regarding the most cost-effective interventions."

HTA unit budgeted @R368m in 2018 budget by country's Treasury

"the **India** Medical Technology Assessment Board for evaluation and appropriateness and cost effectiveness of the available and new Health Technologies in India…**standardized cost effective interventions that will reduce the cost and variations in care, expenditure on medical equipment…overall cost of treatment, reduction in out of pocket expenditure of patients…**'. Ref: MTAB, Ministry of Health & Family Welfare, Government of India

सत्यमेव जयते
MINISTRY OF HEALTH & FAMILY WELFARE

October 2018: China legislates HTA and launches National Centre of Medicine and Health Technology Assessment

FU Wei

Director-General, Research Fellow, China National Health Development Research Center, National Health Commission

FU Wei once served as Consultant, Director and Deputy Director of the former Division of Primary Health and Maternal and Child Care, the Department of Rural Health Management, the Department of Maternal and Child Health and Community Health, and the Medical Reform Office of the Ministry of Health, as well as Deputy Director of the Department of Healthcare Reform of former National Health and Family Planning Commission of the PRC (Medical Reform Office of the State Council). Other social posts includes: Vice President of the China Health Economics Association, Chairman of the Health Expenditure and Policy Committee, Chairman of the Application Evaluation and Protection Committee of Chinese Health Information and Big Data Association, Chairman of China Health Policy and Technology Assessment Research Network Committee, and Director of the Collaborative Center for Term Classifications and Standards of the World Health Organization.

4. Knowledge translation and Decision Making

- Pricing Negotiation for 18 Generic Cancer Drug
- Updating National Essential Drug List
- Comprehensive Drug Assessment
- Reviewing Public Health Service Package
- Setting Up the List of Appropriate Technologies in County Level Hospitals

"We have fully utilized HTA...to balance financially sustainability and access to new cancer drugs...up to 30% price reductions compared to nearby countries"
Director of Chinese Medical Insurance Bureau, Beijing, October 2018

中华人民共和国中央人民政府

关于发布2018年抗癌药医保准入专项谈判药品范围的通告

中华人民共和国国家卫生健康委员会
National Health Commission of the People's Republic of China

新闻发布

国务院办公厅关于完善国家基本药物制度的意见

国办发〔2018〕88号

各省、自治区、直辖市人民政府，国务院各部委、各直属机构：

国家基本药物制度是药品供应保障体系的基础，是医疗卫生领域基本公共服务的重要内容。新一轮医改以来，国家基本药物制度的建立和实施，对健全药品供应保障体系、保障群众基本用药、减轻患者用药负担发挥了重要作用。同时，也还存在不完全适应临床基本用药需求、缺乏使用激励机制、仿制品种与原研品种质量疗效存在差距、保障供应机制不健全等问题。为贯彻落实全国卫生与健康大会、《"健康中国2030"规划纲要》和深化医药卫生体制改革的部署要求，进一步完善国家基本药物

（二）完善目录调整管理机制。优化基本药物目录遴选调整程序，综合药品临床应用实践、药品标准变化、药品新上市情况等因素，对基本药物目录定期评估、动态调整，调整周期原则上不超过3年。对新审批上市、疗效较已上市药品有显著改善且价格合理的药品，可适时启动调入程序。坚持调入和调出并重，优先调入有效性和安全性证据明确、成本效益比显著的药品品种；重点调出已退市的，发生严重不良反应较多、经评估不宜再作为基本药物的，以及有风险效益比或成本效益比更优的品种替代的药品。原则上各地不增补药品，少数民族地区可增补少量民族药。

2018年全国药政工作会在京召开 明确加快短缺药品供应保障体系建设等7项重点

发布时间：2018-10-15

10月15日，2018年全国药政工作会议在京召开。明确近期我国药政工作将着力围绕加快短缺药品供应保障体系建设、全面实施国家基本药物制度新政策、全面落实药品采购"两票制"、提高药品供应保障能力、开展药品临床综合评价、推进国家药物政策体系和协调机制建设等7个方面重点展开。

…and in high income economies in the EU… (cont.)


EUROPEAN COMMISSION

Brussels, 31.1.2018
COM(2018) 51 final
2018/0018 (COD)

Proposal for a

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

on health technology assessment and amending Directive 2011/24/EU

(Text with EEA relevance)

{SWD(2018) 41 final} - {SWD(2018) 42 final}


The BeNeLuxA Initiative aims to ensure sustainable access to innovative medicine at affordable cost for our patients.

Positive outcome of joint reimbursement negotiations on Spinraza

Beneluxa Initiative partners Belgium and the Netherlands successfully negotiated the reimbursement of Spinraza Belgium and the Netherlands have reached an agreement on the pricing of Spinraza, a drug for Spinal Muscular Atrophy (SMA). Spinraza will be reimbursed for specific...

more

Ireland joins BeNeLuxA initiative

22 June 2018 Today, the Irish Minister for Health, Simon Harris signed an Agreement with his colleagues from Belgium, The Netherlands, Luxembourg and Austria to join the Beneluxa Initiative on Pharmaceutical Policy. The ceremony took place during the Employment, Social Policy,...

General update (January 2018)

The Steering Committee of the BeNeLuxA cooperation met in Luxembourg on 18 January 2018. Experiences with joint HTA reports and joint negotiations were assessed, and the planned activities for 2018 in the areas of HTA and pricing and reimbursement were discussed. Topics included...

more

"The outcome of HTA is used to inform decisions concerning the allocation of budgetary resources in the field of health, for example, in relation to establishing the pricing or reimbursement levels of health technologies. HTA can therefore assist Member States in creating and maintaining sustainable healthcare systems and to stimulate innovation that delivers better outcomes for patients"

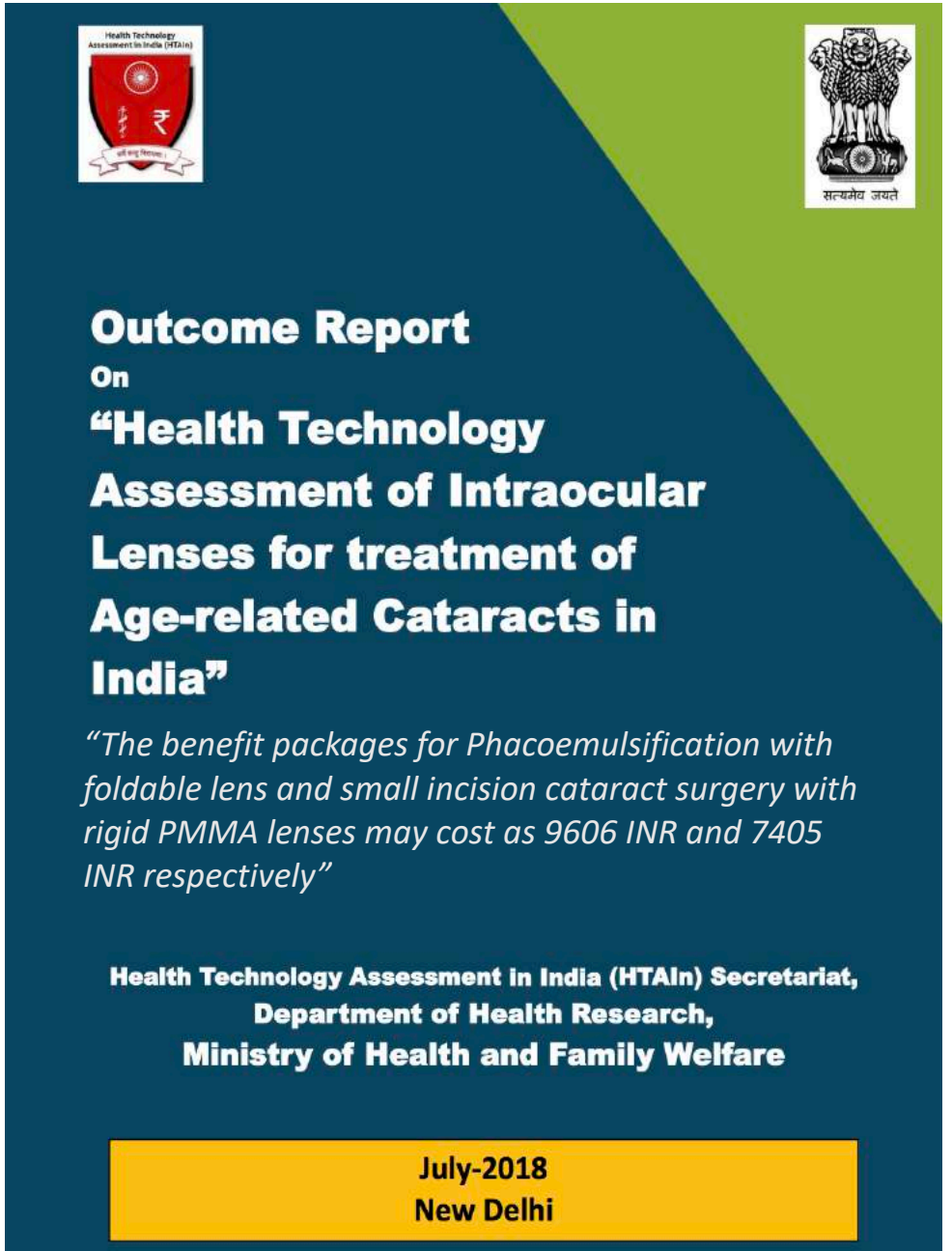
REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on health technology assessment and amending Directive 2011/24/EU

…who use HTA to decide listing and pricing of new technologies

Table 1. Summary of European Collaborations in Procurement of Health Innovations

Alliance	Member Countries	Initiation Date	Areas of cooperation
Valletta Declaration*	Malta, Cyprus, Greece, Italy, Spain, Portugal, Slovenia, Croatia, Ireland, Romania	May 2017	Information sharing on prices and markets, joint negotiation for purchasing to ensure affordability
Central Eastern European and South Eastern European Countries Initiative	Romania, Bulgaria, Croatia, Latvia, Poland, Sebia, Slovakia, Slovenia, Republic of Moldova, FYR Macedonia	November 2016	Price negotiation
Southerna European initiative	Greece, Bulgaria, Spain, Cyprus, Malta, Italy, Portugal	June 2016	Information sharing on prices and markets, and collaboration on R&D
Declaration of Sofia	Bulgaria, Croatia, Estonia, Hungary, Latvia, FYR Macedonia, Romania, Serbia, Slovakia, Slovenia	June 2016	Information sharing on prices and markets, with potential for joint purchasing in the future
Nordic Pharmaceuticals Forum	Denmark, Iceland, Norway, Sweden	June 2015	Horison scanning, information sharing on prices and markets
Romanian and Bulgarian Initiative	Romania, Bulgaria	June 2015	Joint negotiations in purchasing to get lower prices for pharmaceuticals and cross-border exchange of medicines in short supply to ensure continuity of access
Beneluxa Initiative on Pharmaceutical Policy	Belgium, Netherlands, Luxembourg, Austria, Ireland**	April 2015	HTA, horizon scanning, information sharing on prices and markets, joint negotiation for purchasing to ensure affordability
Baltic Partnership Agreement	Latvia, Lithuania, Estonia	May 2012	Centralized joint purchasing (tenders, negotiation, payment and distribution) to reduce expenditure and ensure continuity of access

* Michalopoulos, 2017, 2018; ** Ireland recently joined (An Roinn Slainte, 2018; Beneluxa, 2018a)



HTA informs pricing across EU

- "While some countries systematically apply HTA for all new



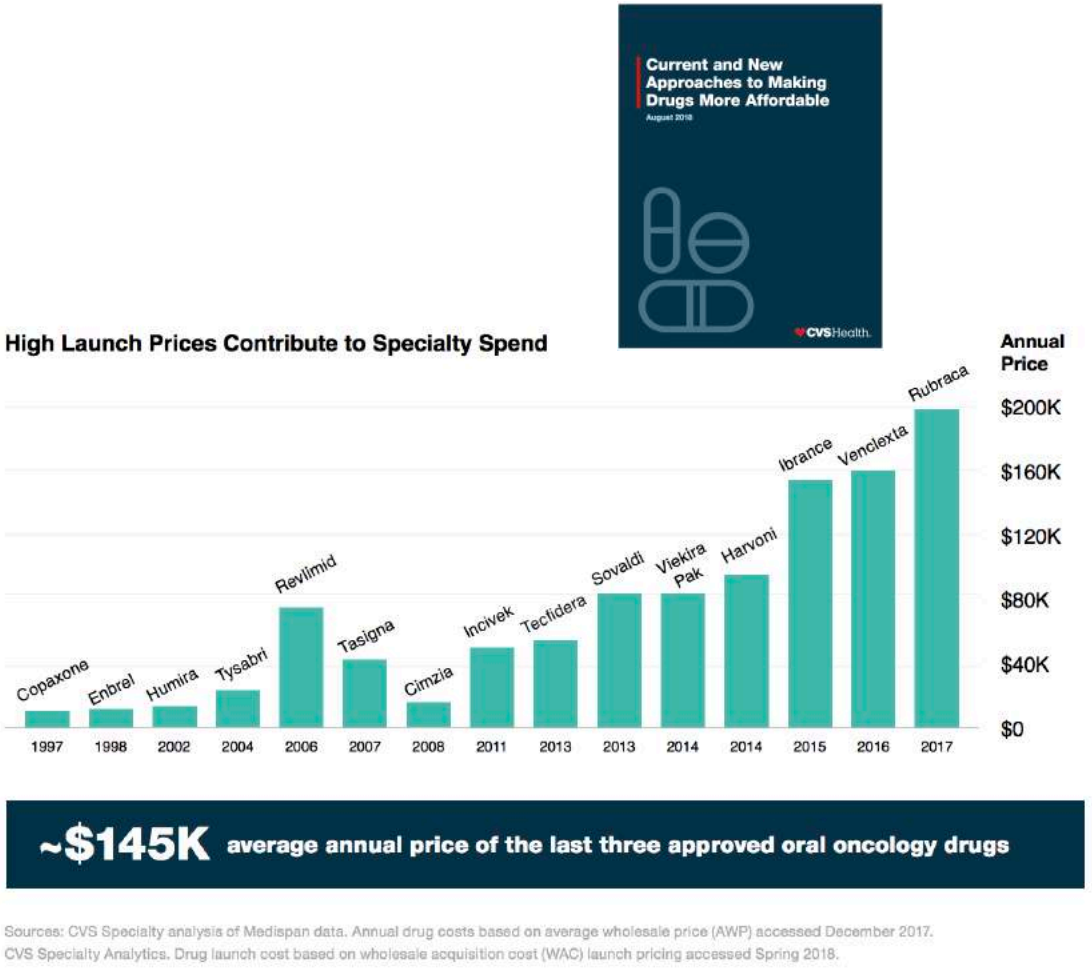
July 2018

Published outcomes

Branded Name	Company[2]	Therapeutic Area	Year	HTA Type
Lojuxta	Aegerion	Hyper-cholesterolemia	2015	Belgium re-used Ducth HTA work
Orkambi	Vertex	Cystic fibrosis	2016	First submission – Joint HTA (Belgium and Netherlands); external referee (Dutch Zorginstituut); Luxembourg used final report
Praluent	Sanofi	Dyslipidemias	2016	External referee (Dutch Zorginstituut for Belgium)
Orkambi	Vertex	Cystic fibrosis	2017	Second submission - Joint HTA (Belgium Netherlands); external referee (Dutch Zorginstituut); final report sent to Luxembourg and Austria
Vyndaqel	Pfizer	Amyloidosis	2017	External referee (Dutch Zorginstituut for Belgium); Luxembourg used final report
Ocaliva	Intercept	Primary biliary cholangitis	2018	Joint HTA (Belgium and Netherlands)
Spinraza	Biogen	Spinal Muscular Atrophy	2018	Joint HTA (Belgium and Netherlands)[3]

http://www.euro.who.int/__data/assets/pdf_file/0011/376625/pharmaceutical-reimbursement-eng.pdf?ua=1

And even in the USA private insurers adopt HTA…



- "CVS Caremark is initiating a program that allows clients to exclude any drug launched at a price of greater than $100,000 per QALY from their plan. The QALY ratio is determined based on publicly available analyses from the Institute for Clinical and Economic Review (ICER), an organization skilled in the development of comparative effectiveness analyses.
- Medications deemed "breakthrough" therapies by the U.S. Food and Drug Administration will be excluded from this program, which will focus on expensive, "me-too" medications that are not cost effective, helping put pressure on manufacturers to reduce launch prices to a reasonable level."

https://cvshealth.com/sites/default/files/cvs-health-current-and-new-approaches-to-making-drugs-more-affordable.pdf

NICE IN THE UK: PAST, PRESENT AND FUTURE

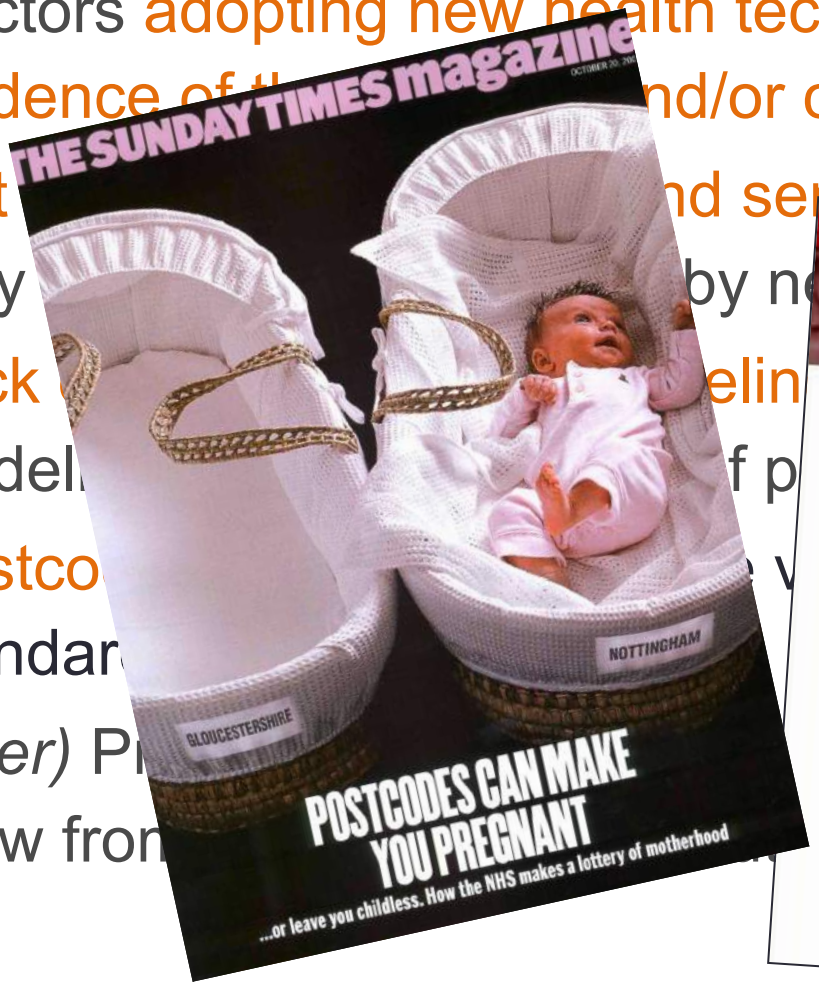
The role of NICE in the UK

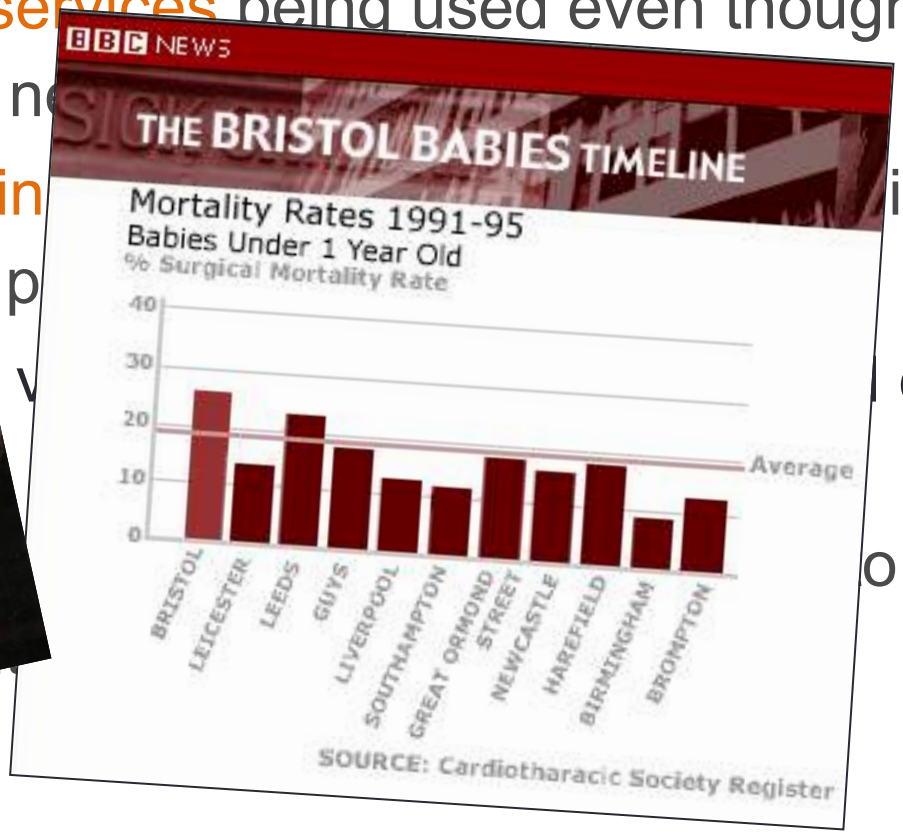
The National Institute for Health and Care Excellence (NICE) provides national guidance and advice to improve health and social care

- Produces ***evidence-based guidance*** and advice for health, public health and social care practitioners.
- Develops ***quality standards and performance metrics*** for those providing and commissioning health, public health and social care services;
- Provides a ***range of information services*** for commissioners, practitioners and managers across the spectrum of health and social care.

Background to the creation of NICE in the 90s…

- Doctors adopting new health technologies without adequate evidence of th nd/or cost effectiveness
- Out nd services being used even though they by n
- Lack elin inical guidel f p
- Postco care standar
- *(later)* Pr o grow fron





1997: A new Labour Government and 'The New NHS'

- "The Government is determined that the services and treatment that patients receive across the NHS should be based on the best evidence of what does and d[illegible] effec[illegible] of ev[illegible] nd cost-effectiveness. cation

  - 'A new National Institute for Clinical Excellence will be established to give new coherence and prominence to information about clinical and cost-effectiveness.'
  - '…membership will be drawn from the health professions, the NHS, academics, health economists and patient interests.'

- All too often in the past, the same problem has been partially solved in different areas. Best practice has not been shared as it should have been. As a result patients have not had fair access to the best the NHS has to offer."

1999: NICE is established: focus on professionals and quality, not drug prices

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  role and function of the National Institute as it gathers momentum and experience."

Clinical Governance:

"A framework through which NHS organisations are accountable for continually improving the quality of their services and safeguarding high standards of care by creating an environment in which excellence in clinical care will flourish."*

* G. Scally and L. J. Donaldson, Clinical governance and the drive for quality improvement in the new NHS in England BMJ (4 July 1998): 61-65

But quality comes at a cost: the Minister's Directions to NICE 1999/2005

"Subject to and in accordance with such directions as the Secretary of State may give, the Institute shall perform:
such functions in connection with the promotion of clinical excellence, and **the effective use of available resources in the health service**"
Article 3 (functions of the Institute) of the principal Order (1999/amended 2005)

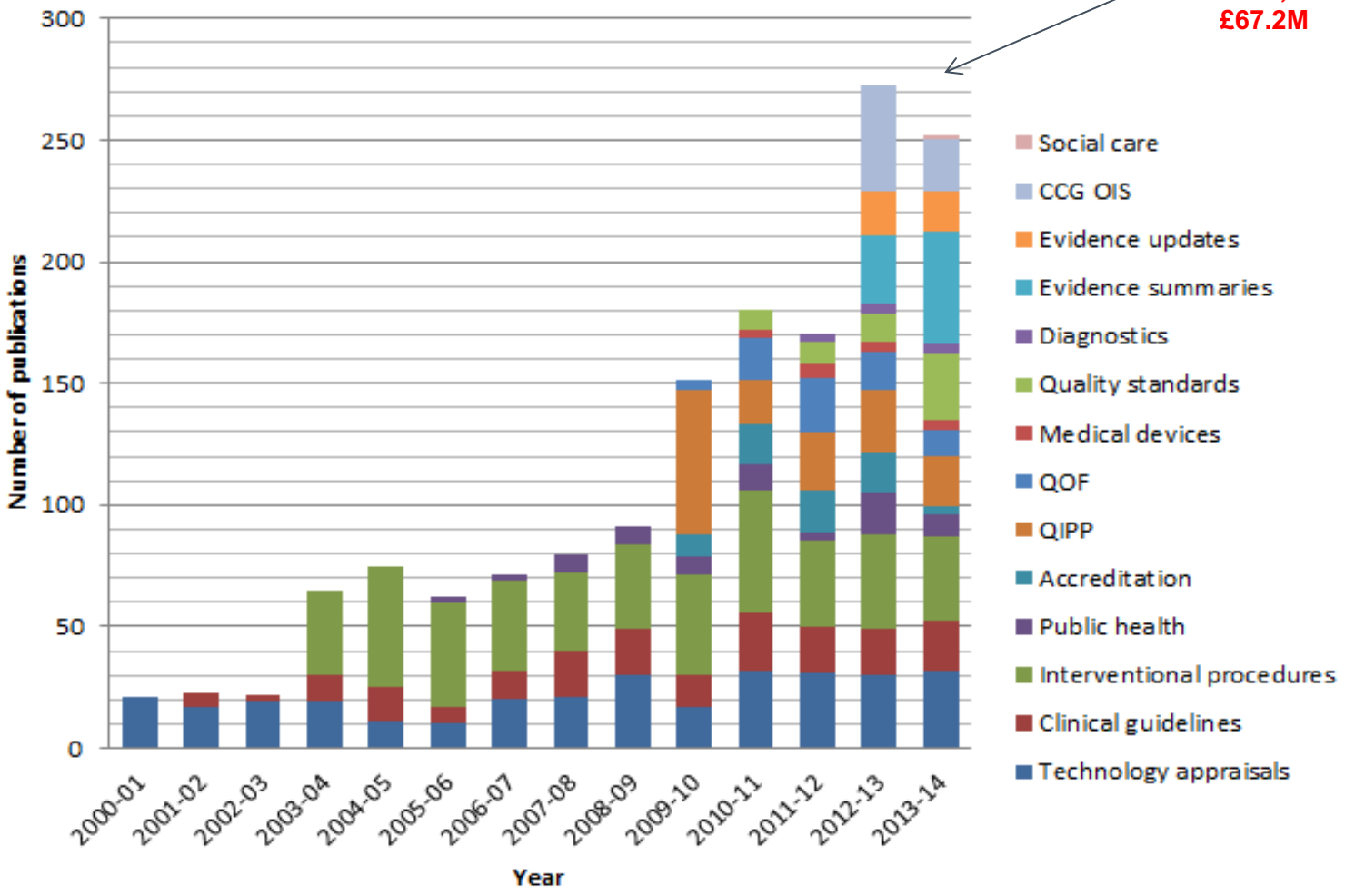
The Coalition government reforms, 2013: quality becomes the Law!

"The Secretary of State must…secure continuous improvement in the quality of services provided to individuals…In discharging the duty…the Secretary of State must have regard to the quality standards prepared by **NICE** under section 234 of the Health and Social Care Act 2012."

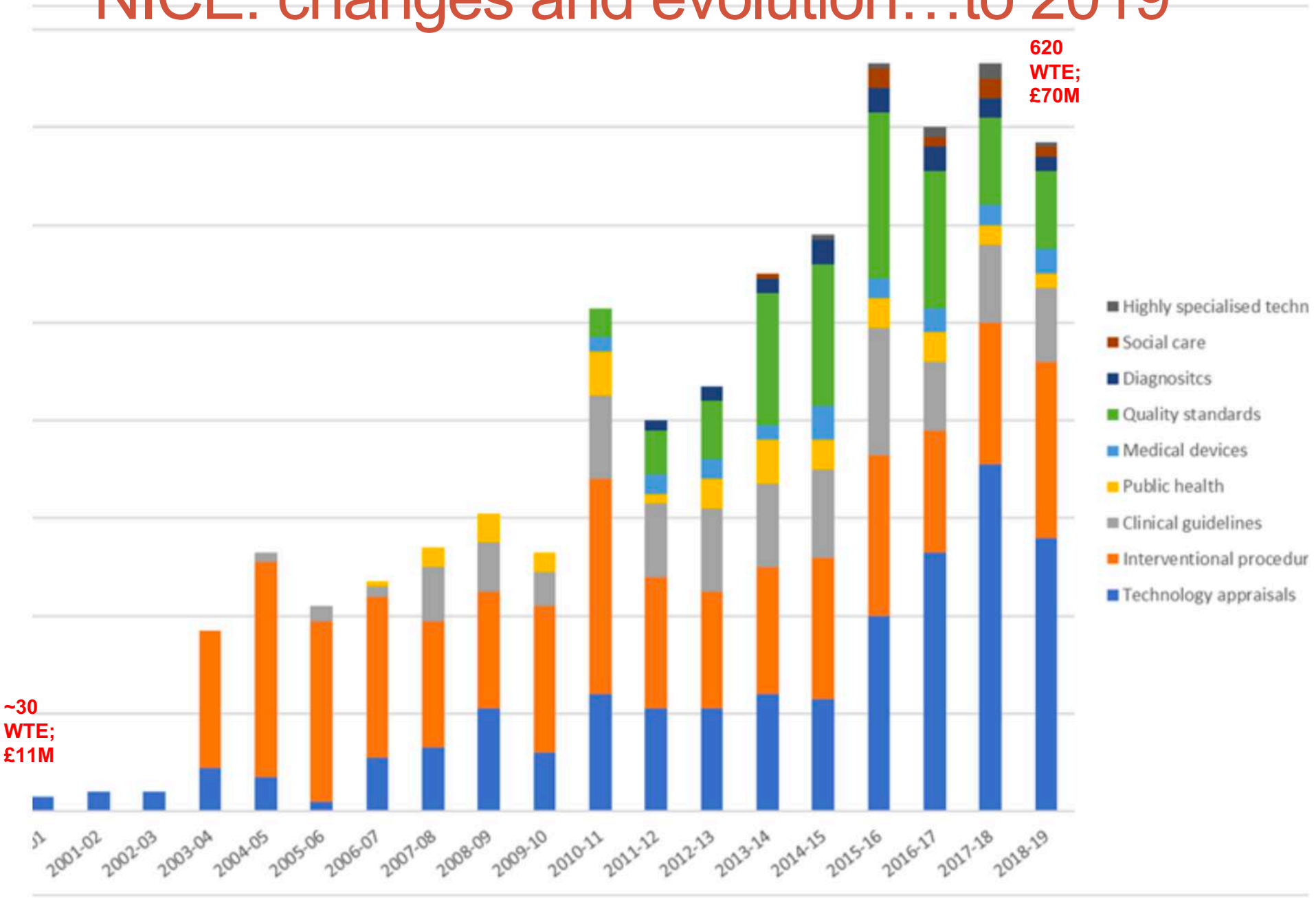
NICE: changes and evolution…to 2014

560
WTE;
£67.2M

~30
WTE;
£11M



NICE: changes and evolution…to 2019



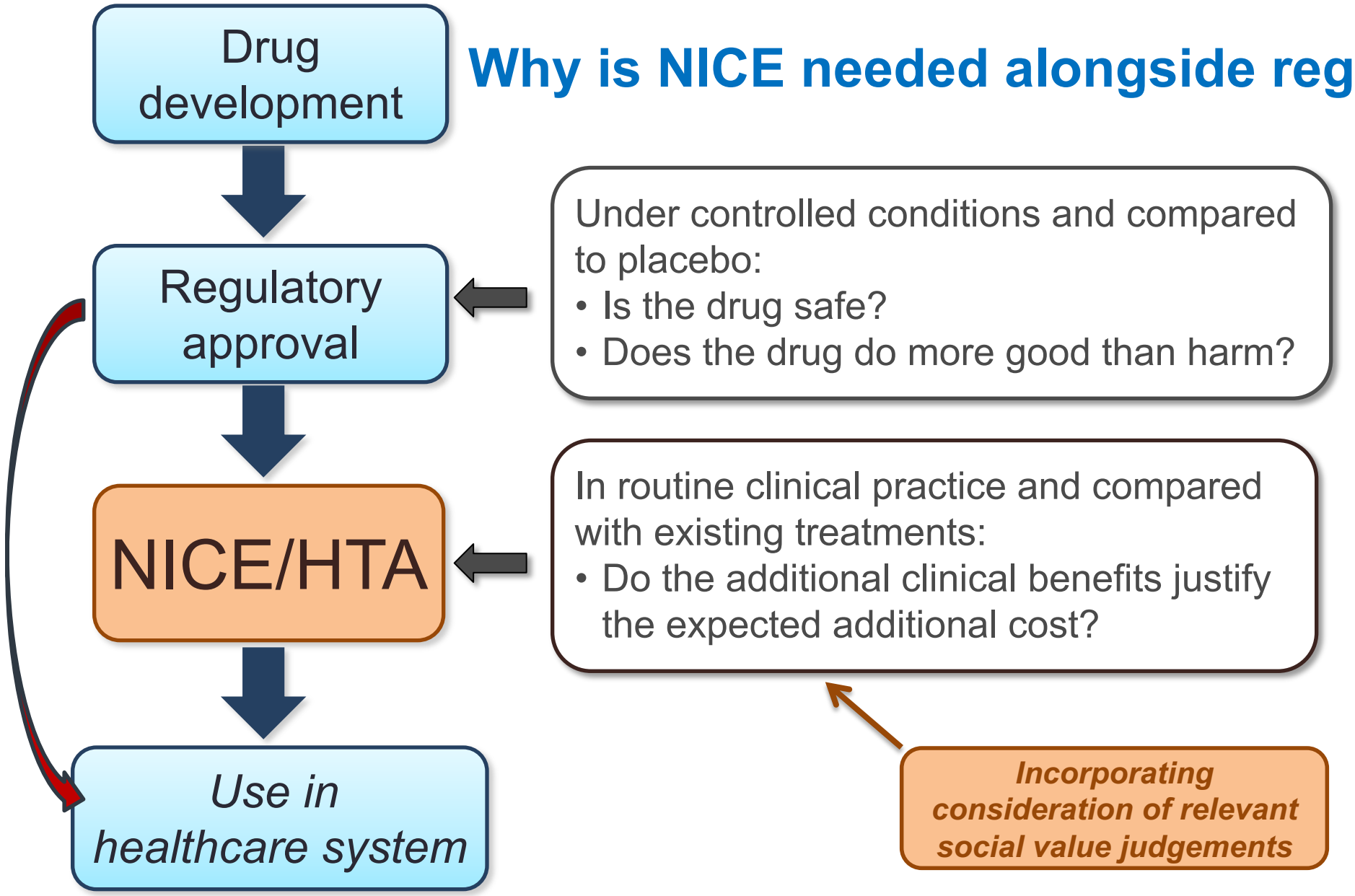
NICE - "Technology Appraisals"

- "The NICE **technology appraisal programme** assesses the clinical- and cost-effectiveness of new medicines, significant licence extensions and other health technologies….The NHS is ***legally obliged* to fund and resource medicines and treatments recommended by NICE's technology appraisals***. Since April 2016, it has been agreed that ***all new cancer medicines*** and significant new licenced indications will be appraised by NICE".

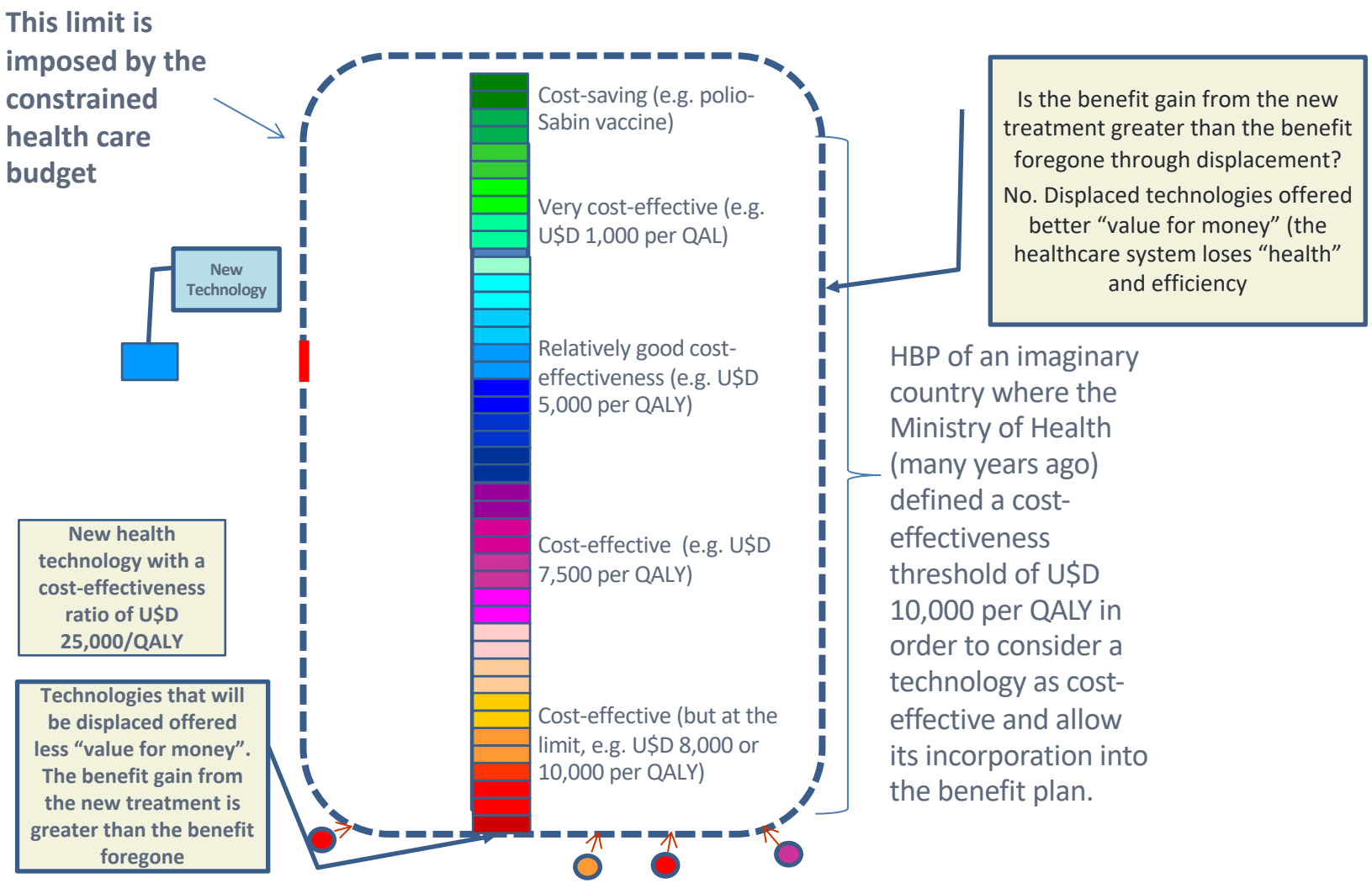
*When NICE recommends a treatment 'as an option', the NHS must make sure it is available within 3 months (unless otherwise specified) of the final guidance publication.

- Source: NICEimpact (2018) - *Cancer*

Why is NICE needed alongside regulation?

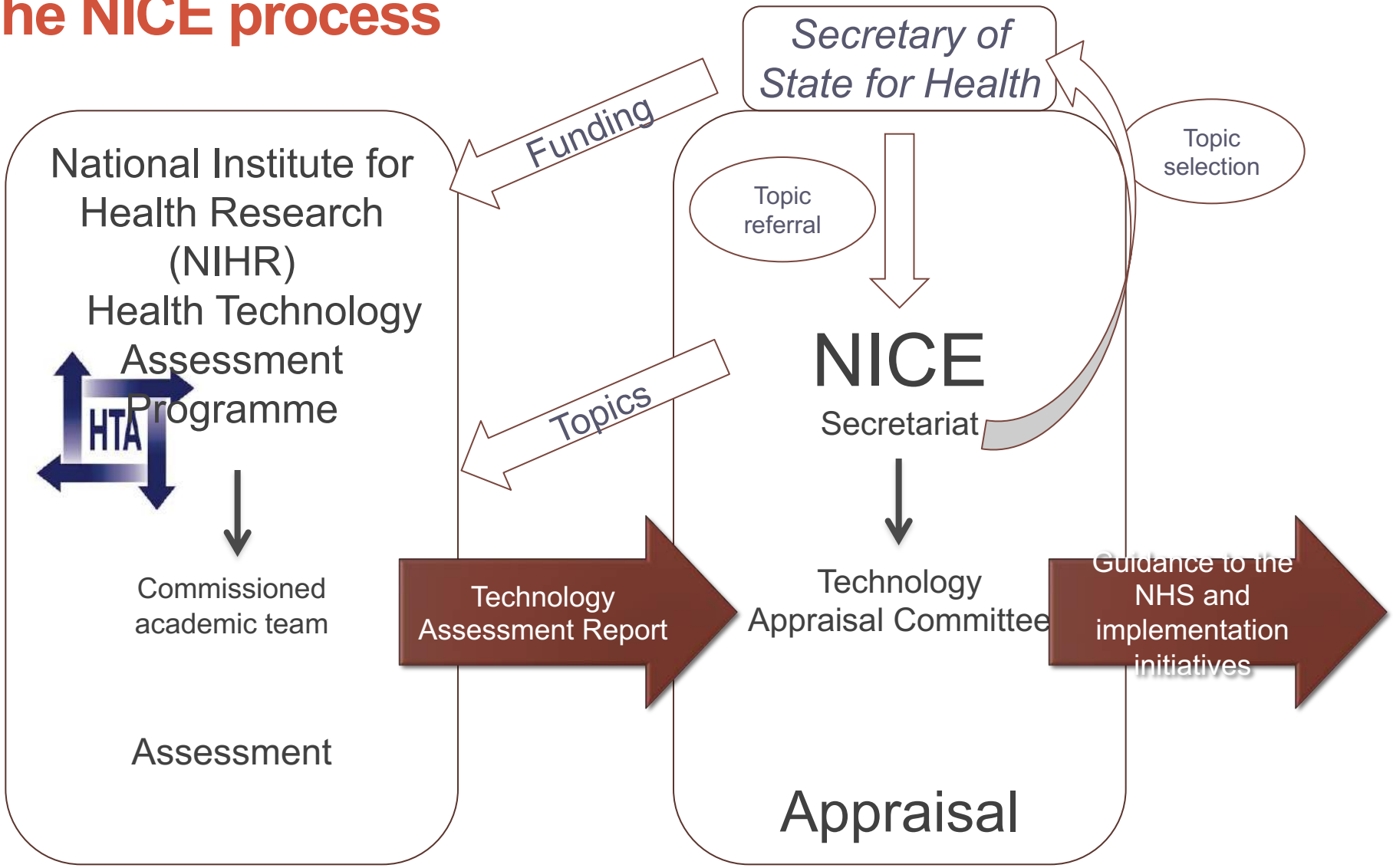


Opportunity costs matter!



Source: Andrés Pichon-Riviere , 2013. La aplicación de la evaluación de Tecnologías de Salud y las evaluaciones económicas en la definición de los Planes de Beneficios en Latinoamérica

The NICE process



Adapted from Walley, T. (2007) MJA; Overview of Health technology assessment in England: assessment and appraisal187: 283–285

Horizon scanning and topic selection
→working closely with industry for Technology Appraisals

Notify NICE on health technologies **3-5 years before UK licence** that may be suitable for NICE topic selection and ultimately NICE Technology appraisal or Highly Specialised Technologies evaluation.

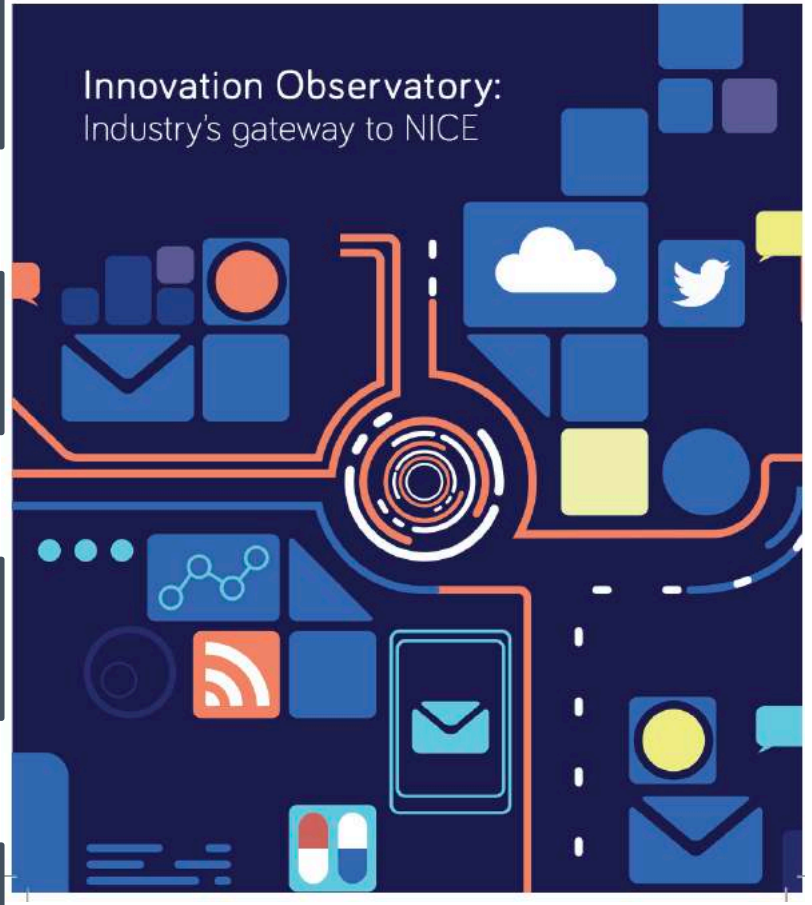
Companies cannot access the NICE TA process without contacting NIHRIO first.

Once a new or repurposed technology is **approximately 3 years prior to licence, the NICE topic selection team are notified**. Work with companies to ensure that the information passed to NICE is accurate and timely.

Rely on pharmaceutical companies providing us with regular updates on estimated regulatory and marketing authorisation plans. NIHRIO **respects confidential and commercially sensitive information.**

Innovation Observatory

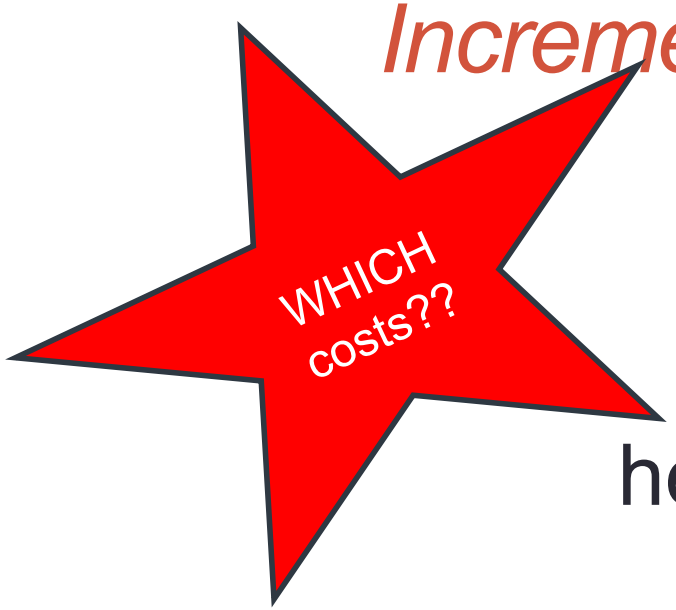
NHS National Institute for Health Research



- Notification of technology to NICE: 3 years to product licence
- NICE decision on whether to proceed returned to NIHRIO
- Evidence briefing produced by NIHRIO
- NIHRIO send briefing to company for comment
- NICE receive completed briefing from NIHRIO approximately 20-15 months before product licence and the TA process begins

http://www.io.nihr.ac.uk/wp-content/uploads/2017/05/NIHRIO-Innovation-Observatory-NICE-Leaflet-AW.pdf

Cost effectiveness –
Incremental cost-effectiveness ratio (ICER):



$$\frac{\text{cost}_{new} - \text{cost}_{current}}{\text{health gain}_{new} - \text{health gain}_{current}}$$

At NICE, health gain is expressed as quality adjusted life years (QALYs) which allows us to calculate the **cost per QALY** for any technology under consideration

COSTS MATTER!!!

NHS Reference Costs

- *”Reference costs are the average unit cost to the NHS of providing defined services to NHS patients in England in a given financial year. They show how NHS providers spend money to provide healthcare to patients.”*
  - In 2017/18 – 232 NHS providers spending £68 Billion delivering healthcare to patients
  - Reference costs collection is the **nationally mandated** collection of cost data from all NHS providers – began in 1997
  - It is NHS providers’ **responsibility** to improve their internal costing processes and systems
  - National bodies (Dept. Health, NHS England, NHS Improvement) have a responsibility to ensure the costs collected are useful – provide comprehensive and clear guidance on cost collection for providers

NHS Reference costs – some uses

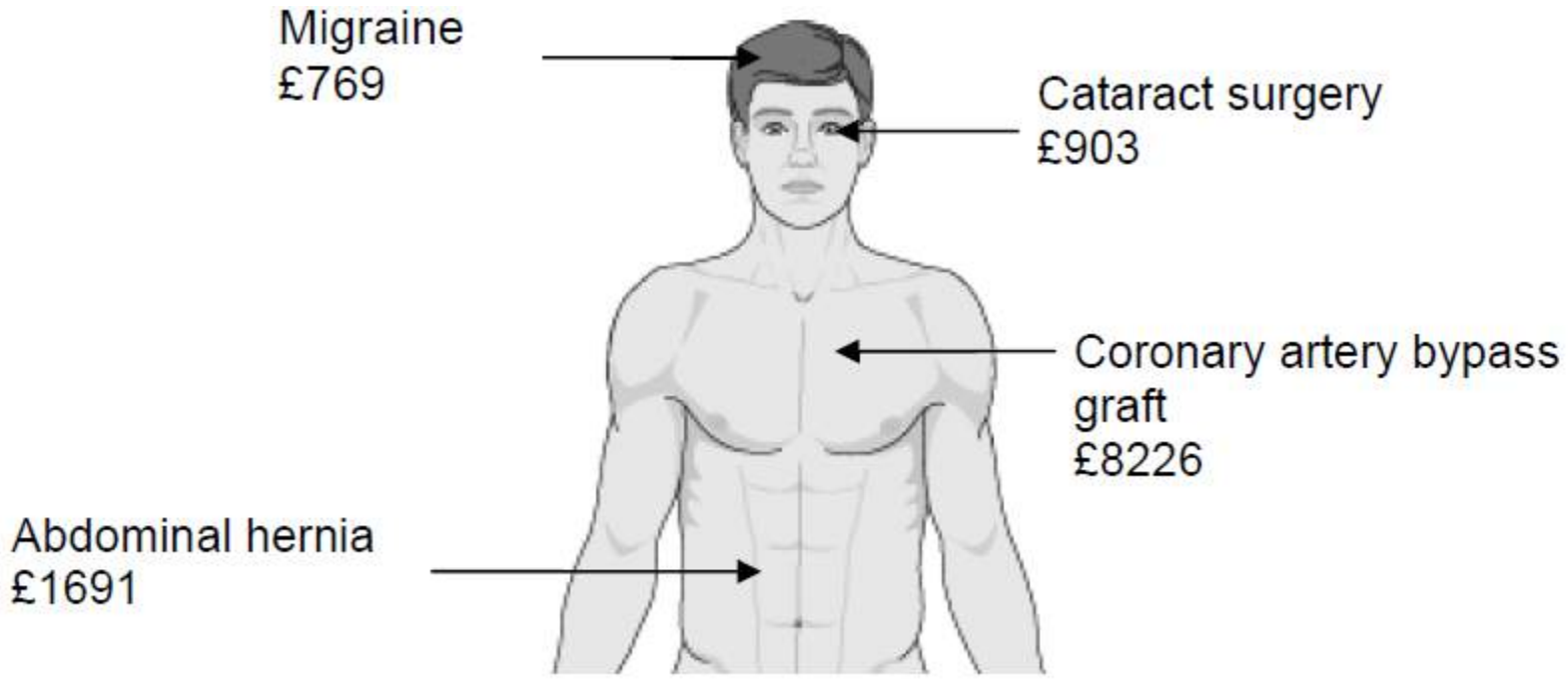
Helps NHS providers better understand the cost of their services

Improves accountability to government

Informs the national pricing of services --> National Tariff Payment System

Supports HTA by providing unit costs for cost-effectiveness calculations

The NHS National Tariff

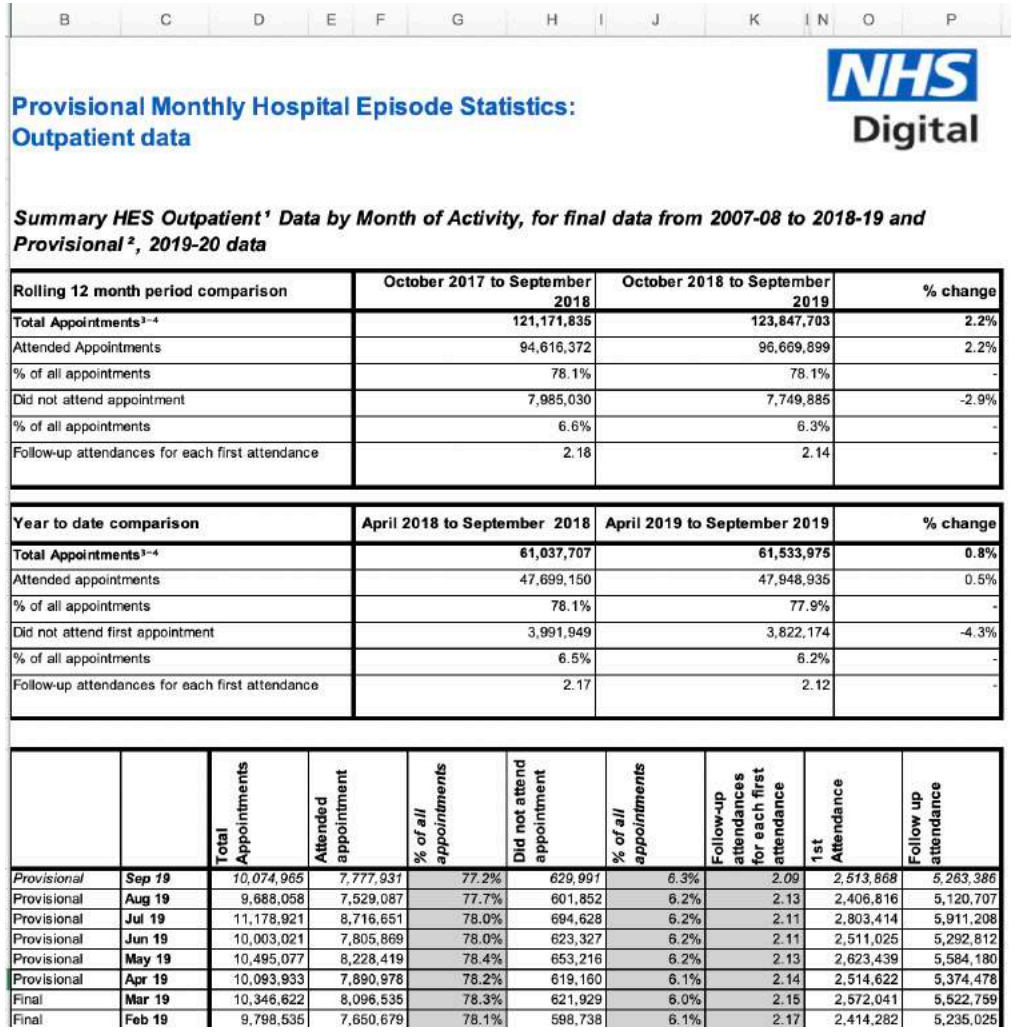


- Informed by average costs (NHS Reference Costs)
- Covers >60% of acute hospital income
- Driven by HRGs (the ”currency” - clinical grouping classification system)
- NICE guidance informs tariff

Source: DH (2011)

Hospital Episode Statistics (HES)

- Responsibility of NHS Digital (https://digital.nhs.uk/data-and-information/data-tools-and-services/data-services/hospital-episode-statistics)
  - HES is a data warehouse containing details of all admissions, outpatient appointments and Emergency attendances at NHS hospitals in England
  - Data are collected during a patient's time at hospital, submitted to NHS Digital for processing and returned to the providers → allows hospitals to be paid for the care they deliver
  - HES data also needed as an input into the National Tariff
  - Can also be used for research and planning health services



Provisional Monthly Hospital Episode Statistics: Outpatient data

NHS Digital

Summary HES Outpatient[1] Data by Month of Activity, for final data from 2007-08 to 2018-19 and Provisional[2], 2019-20 data

Rolling 12 month period comparison	October 2017 to September 2018	October 2018 to September 2019	% change
Total Appointments[3-4]	**121,171,835**	**123,847,703**	**2.2%**
Attended Appointments	94,616,372	96,669,899	2.2%
% of all appointments	78.1%	78.1%	-
Did not attend appointment	7,985,030	7,749,885	-2.9%
% of all appointments	6.6%	6.3%	-
Follow-up attendances for each first attendance	2.18	2.14	-

Year to date comparison	April 2018 to September 2018	April 2019 to September 2019	% change
Total Appointments[3-4]	**61,037,707**	**61,533,975**	**0.8%**
Attended appointments	47,699,150	47,948,935	0.5%
% of all appointments	78.1%	77.9%	-
Did not attend first appointment	3,991,949	3,822,174	-4.3%
% of all appointments	6.5%	6.2%	-
Follow-up attendances for each first attendance	2.17	2.12	-

		Total Appointments	Attended appointment	% of all appointments	Did not attend appointment	% of all appointments	Follow-up attendances for each first attendance	1st Attendance	Follow up attendance
Provisional	***Sep 19***	*10,074,965*	*7,777,931*	*77.2%*	*629,991*	*6.3%*	*2.09*	*2,513,868*	*5,263,386*
Provisional	**Aug 19**	9,688,058	7,529,087	77.7%	601,852	6.2%	2.13	2,406,816	5,120,707
Provisional	**Jul 19**	11,178,921	8,716,651	78.0%	694,628	6.2%	2.11	2,803,414	5,911,208
Provisional	**Jun 19**	10,003,021	7,805,869	78.0%	623,327	6.2%	2.11	2,511,025	5,292,812
Provisional	**May 19**	10,495,077	8,228,419	78.4%	653,216	6.2%	2.13	2,623,439	5,584,180
Provisional	**Apr 19**	10,093,933	7,890,978	78.2%	619,160	6.1%	2.14	2,514,622	5,374,478
Final	**Mar 19**	10,346,622	8,096,535	78.3%	621,929	6.0%	2.15	2,572,041	5,522,759
Final	**Feb 19**	9,798,535	7,650,679	78.1%	598,738	6.1%	2.17	2,414,282	5,235,025

Costs of staff time

- In the UK the Personal Social Services Research Unit (University of Kent) produce an annual "Unit Costs of Health and Social Care" (first produced in 1992) which provides detailed costing for staff time
  - E.g. provides national information on an hour c nurse's or doctor's time, in primary care or in hospital settings
  - Costings take it account education and qualifications, and even environment costs (carbon emissions)
  - Also have costs for on-line consultation system

See: https://www.pssru.ac.uk/project-pages/unit-costs/

5.1 Local authority own-provision care homes for adults requiring physical support (age 18-64, summary provided for 65+)

This table uses the ASC-FR data return (ASC-FR) for 2017/2018.[1]

Costs and unit estimation	2017/2018 value	Notes
Capital costs A. Buildings and oncosts	£154 per resident week	Based on the new-build and land requirements for local authority residential care establishments. These allow for 57.3 square metres per person.[2] Capital costs have been annuitised over 60 years at a discount rate of 3.5 per cent, declining to 3 per cent after 30 years.
B. Land costs	£26 per resident week	Based on Ministry of Housing, Communities & Local Government land estimates.[3] Land costs have been annuitised over 60 years at a discount rate of 3.5 per cent, declining to 3 per cent after 30 years.
C. Total local authority expenditure (minus capital) D. Overheads	£1,067 per resident week	The median revenue weekly cost estimate (£1,067) for adults requiring physical support in own-provision residential care. Capital costs relating to buildings and land have been deducted. The mean cost per client per week is reported as being £834 [using unique identifiers: 8710701 (numerator in thousands of pounds), 8710702 (denominator)]. Social services management and support services (SSMSS) costs are included in PSS EX1 expenditure figures so no additional overheads have been added.
Other costs E. Personal living expenses	£24.90 per week	The DWP personal allowance for people in residential care or a nursing home is £24.90.[4] This has been used as a proxy for personal consumption.
F. External services		No information is available.
Use of facility by client	365.25 days per year	
Occupancy	100 per cent	No statistics available, therefore 100 per cent occupancy assumed.
London multiplier	1.5 x A 3.83 x B 0.71 x C	Relative London costs are drawn from the same source as the base data for each cost element.[1,2,3]
Unit costs available 2017/2018		
Age 18-64 (using unique identifier 8710701; numerator in thousands of pounds, 8710702; denominator) £1,247 per resident week establishment costs (includes A to C); £1,272 per resident week (includes A to E). £178 per resident day establishment costs (includes A to C); £182 per resident day (includes A to E). **Age 65+ (using unique identifier 8713701; numerator in thousands of pounds, 8713702; denominator)** £963 (£930) median (mean) establishment cost per resident week. £138 (£133) median (mean) establishment cost per resident day.		

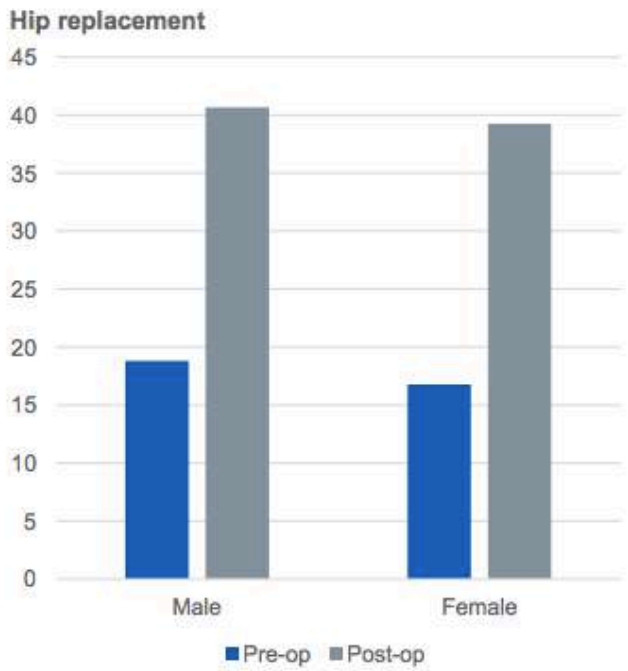
Patient Reported Outcome Measures

- NHS has recently started asking patients whether or not they feel better after certain operations: in 2009, England introduced the national PROMs programme
  - All patients having elective hip replacement, knee replacement, and up to September 2017, varicose vein and groin hernia surgery in England, have been asked to fill in standardised health questionnaires before they have surgery and once again some months afterwards.
  - Condition specific measures (Oxford Hip Score, Oxford Knee Score)
  - General health measures (EQ-5D)
  - Supports improvements in clinical practice, organisational benchmarking, research

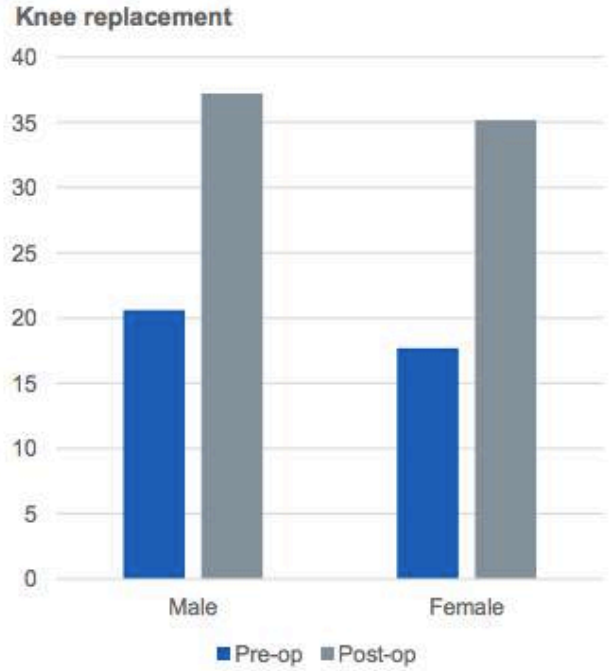
Latest PROMs data 2017/18

Key findings

- In 2017/18, patients undergoing hip replacements reported average health gains on the Oxford Hip Score of 21.8 for males and 22.5 for females. On the Oxford Knee Score, these were 16.6 for males and 17.5 for females.
- Almost all hip replacement patients (97.0%) showed an improvement on the Oxford Hip Score
- Of reported knee replacement patients 94.3% showed improvement on the Oxford Knee Score.



Oxford Hip Score



Oxford Knee Score

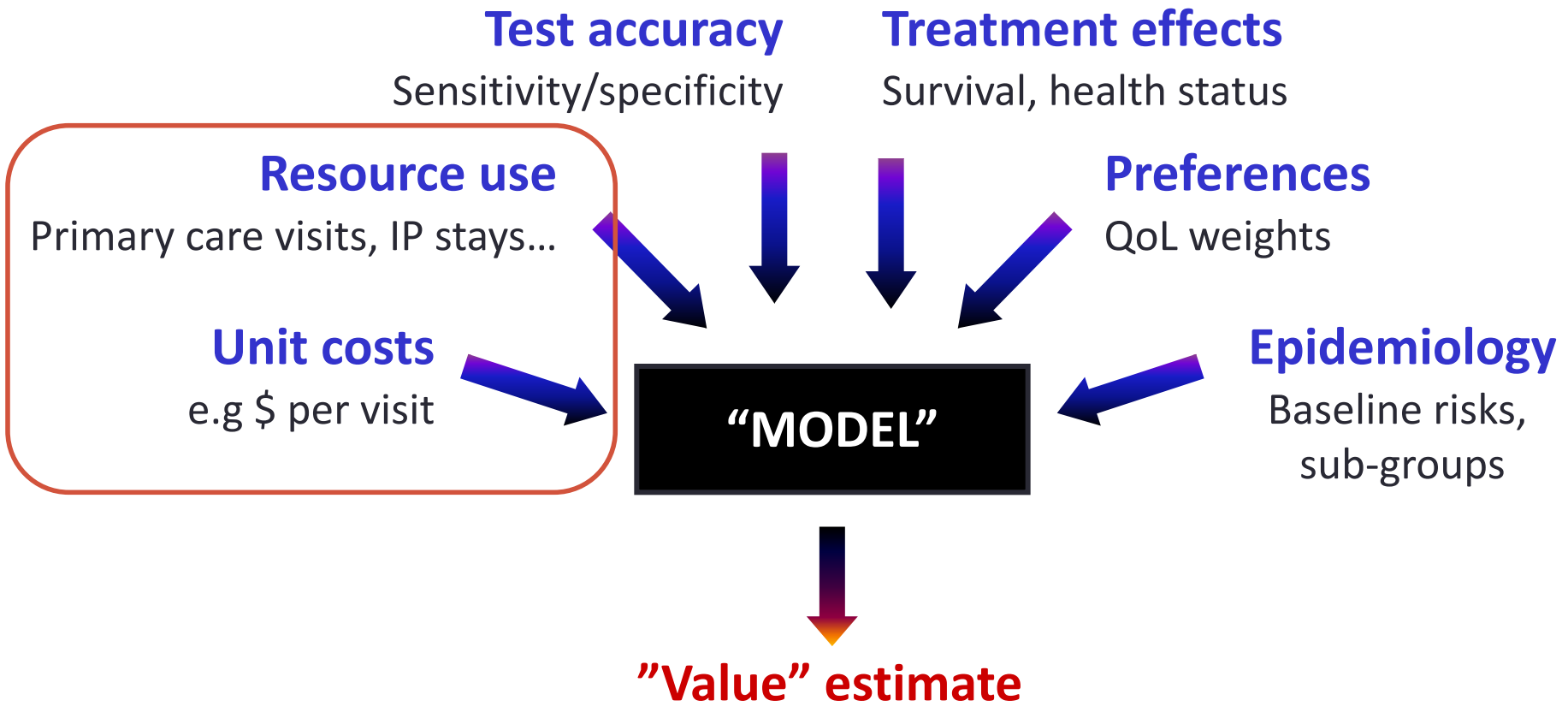
Key facts

Comparing pre- and post-operative 'EQ-5D Index' scores (a combination of five key criteria concerning patients' self-reported general health), an increase in general health was recorded for:

- 89.7 per cent of hip replacement respondents (88.8 per cent for 2016-17)

- 82.2 per cent of knee replacement respondents (81.0 per cent for 2016-17)

What data do we need?



Economic Evaluation and Value for Money in HTA

Kalipso Chalkidou, *MD, PhD*
Professor of Practice in Global Health, Imperial College London
Director of Global Health Policy and Senior Fellow, Center for Global Development
Director, international Decision Support Initiative

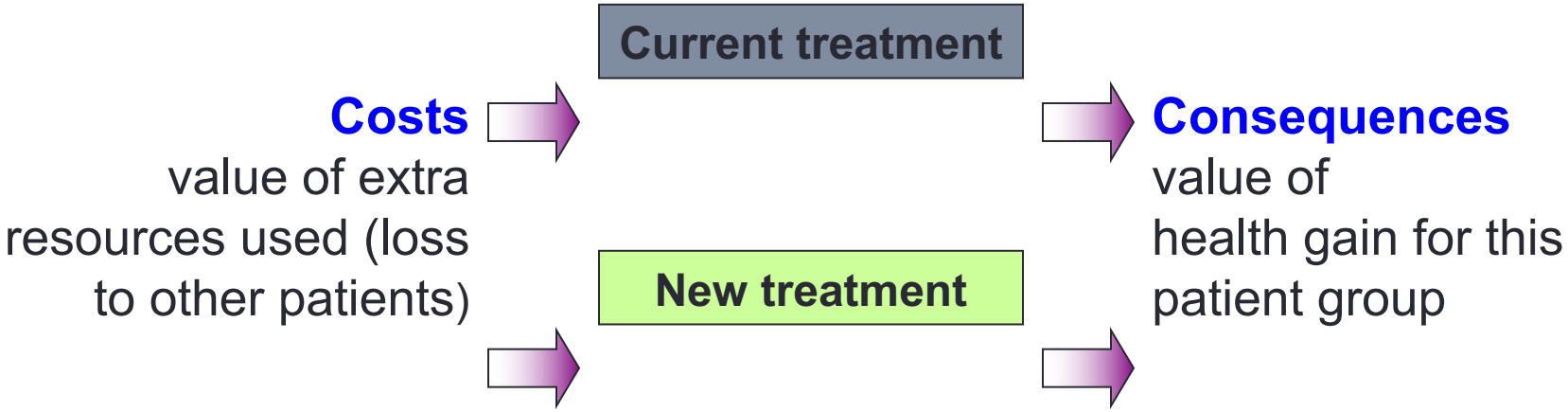
What type of analyses can inform HTA?

Type of analysis	Where it is used
Cost-of-illness analysis	A determination of the **economic impact of an illness** or condition (typically on a given population, region, or country) e.g., of smoking, arthritis, or diabetes, including associated treatment costs
Cost-Effectiveness Analysis	A **comparison of costs in monetary units with outcomes in quantitative non-monetary units such as Quality Adjusted Life Years (QALYs) or averted Disability Adjusted Life Years (DALYs),** reduced mortality or morbidity. This is often termed "cost-utility analysis" (CUA) and you should give thought to whether your preferred outcome measure should be some indicator of health gain or loss or some indicator of the utility of such gains or losses. An advantage of the health gain/loss approach is that it is more readily understandable by clinicians and the public and easier to validate.
Budget Impact Analysis	Can be conducted in addition to a CEA **to determine the impact of implementing or adopting a particular technology** or technology-related policy **on a designated budget**, e.g., for a drug formulary or health plan.
Cost-Consequence analysis	A form of cost-effectiveness analysis that presents **costs and outcomes in discrete categories**, without aggregating or weighting them
Cost-Minimisation analysis	A form of analysis that **assumes that the effects of two interventions are the same, but the costs differ**. The analysis **compares costs to identify the least costly**
Cost-Benefit analysis	compares costs and benefits, both of which are **quantified in common monetary units**

Economic evaluation in HTA

"... the comparative analysis of alternative courses of action in terms of both their costs and consequences."

Drummond, Stoddart & Torrance, 1987



Analysis should be conducted separately for each subgroup of patients.

Cost effectiveness – *Incremental cost-effectiveness ratio (ICER):*

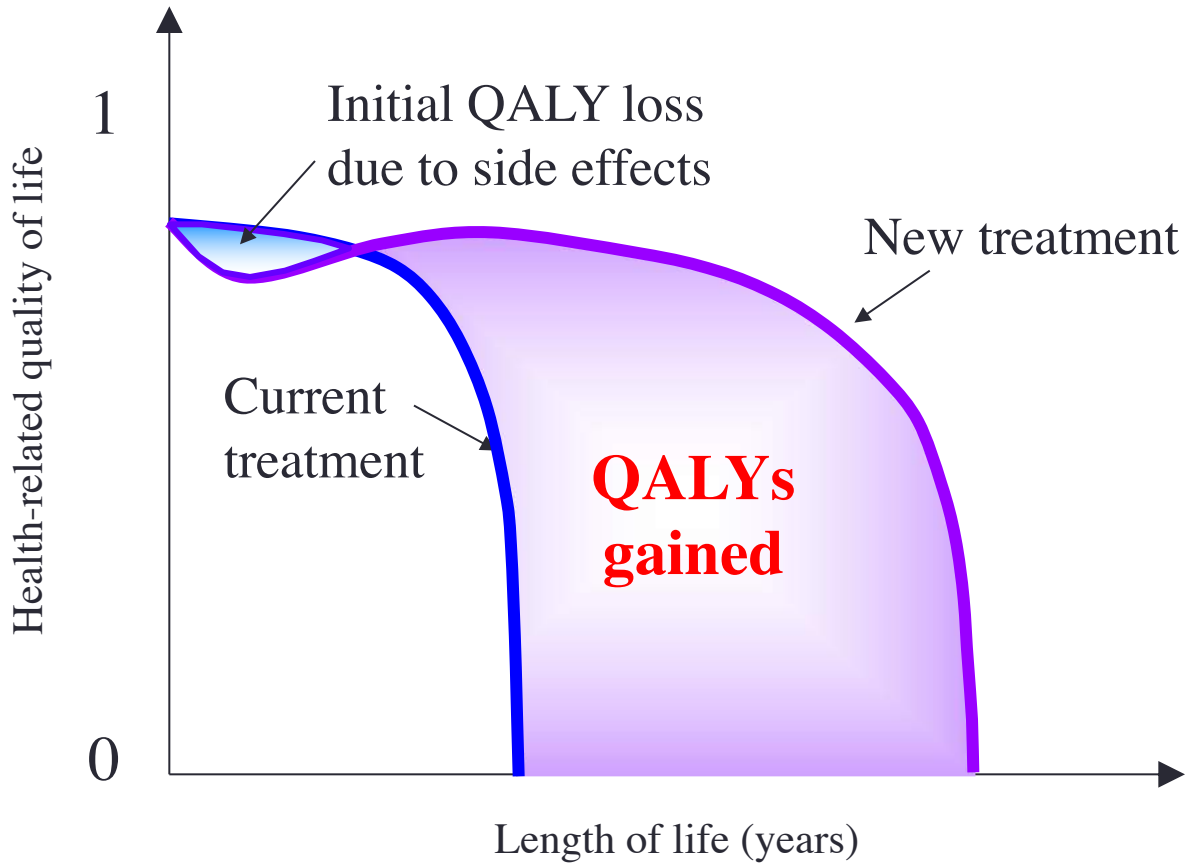
$$\frac{\text{cost}_{new} - \text{cost}_{current}}{\text{health gain}_{new} - \text{health gain}_{current}}$$

How do you express “health gain”?

A generalizable health outcome: **Q**uality **A**djusted **L**ife **Y**ears

- **What is a QALY?**
  - A QALY combines both quantity and health-related quality of life (QoL) into a single measure of health gain
  - The amount of time spent in a health state is weighted by the QoL score attached to that health state
  - QoL scores should reflect peoples' preferences over health
  - QoL is usually scored with 'perfect health'=1 and death=0
- **Why use QALYs?**
  - Can weigh up net effect of treatment for patients
    - Survival vs. QoL (e.g. for cancer chemotherapy)
    - Long-term QoL for chronic & recurrent conditions (e.g. arthritis)
    - Benefits vs. harms (e.g. COX II inhibitors)
  - Allows broader comparisons between patient groups

The **Q**uality **A**djusted **L**ife **Y**ear



“A QALY is a QALY is a QALY”

Usual value judgements used to calculate QALYs:

1 QALY = one year of ‘perfectly healthy’ life for one person

= two years of life with QoL of 0.5 for one person

= one year of life with QoL of 0.5 each for two people

Alternatives to the QALY

- Using QALYs may not always be possible
  - Because of assumptions underpinning the QALY and other factors (such as adequate data availability)
- Alternatives? Single indicators (e.g. weight loss in kg; or deaths averted; or or life years gained, and so on) – but lose benefits of using a generalizable measure
  - May be unavoidable however – use must be justified
- Disability-Adjusted Life Year: one lost year of "healthy" life
  - *But like QALYs also associated with important assumptions and simplifications*

Going beyond the QALY

Accounting for 'fairness'

- Equity-adjustment (See Principle 11)
  - Weight QALY gains to different individuals according to their age, health status, socio-economic status…?
  - Research is progressing, but no usable methods yet (?)

- Deliberative approach
  - Provide decision-making panels with descriptive information about the distribution of QALYs
  - They discuss and make qualitative judgements about trade-offs
  - *Current NICE method* (Tony Culyer)

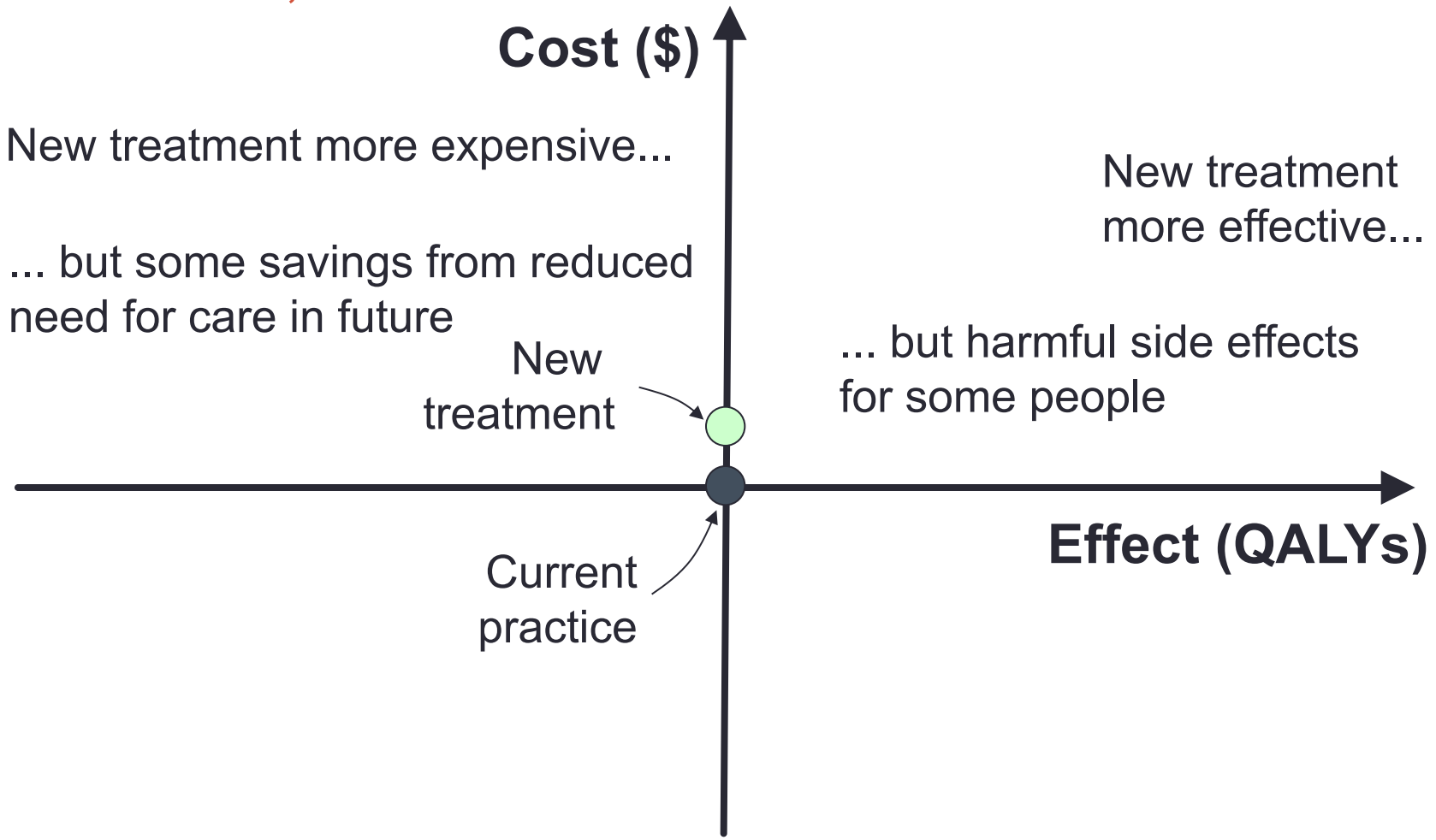
- Multicriteria Decision Analysis (MCDA)…

Using HTA and CEA to make decisions

- What's important (to you…)?
  - Clinical effectiveness
  - Uncertainty
  - Disease severity
  - Special populations (e.g. children, people with cancer…)
  - "End-of-Life"
  - Legal constraints
  - Implementation issues
  - 'Fairness'
  - Supporting 'innovation' by industry
  - Cost effectiveness and 'opportunity cost'…
  - All of the above? And more?
- Cost-effectiveness thresholds (implicit/explicit)….

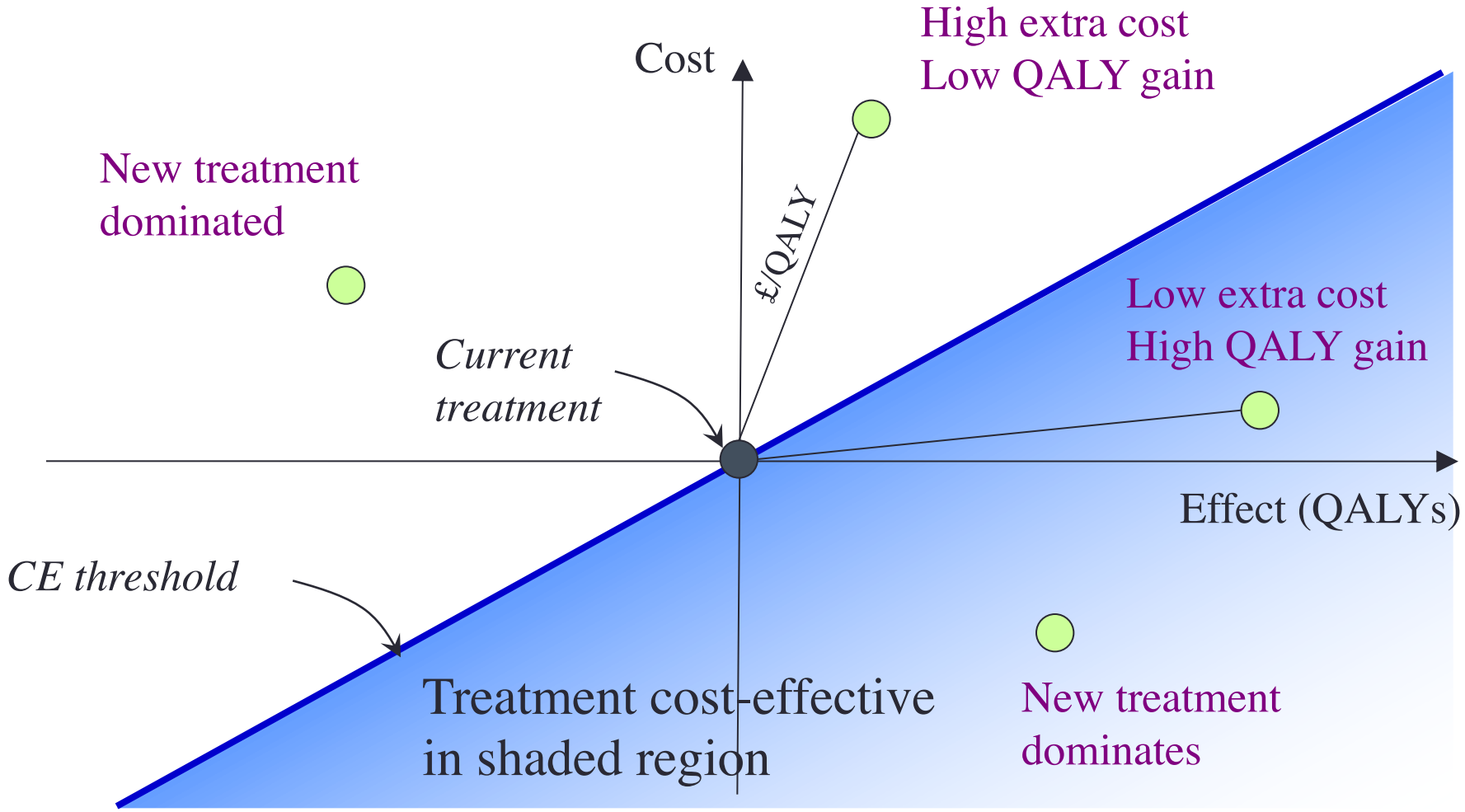
Assessing cost effectiveness

Weighing up the benefits, harms and costs



Assessing cost effectiveness

"Value for money"



Thresholds – implicit and explicit

Explicit	Implied/not stated
NICE – UK (NB not Scotland) • £20 – 30,000 per QALY; £50,000 + per QALY ("End of Life" etc)	Pharmaceuticals Benefits Advisory Committee (PBAC) – Australia • Technologies with ICERs greater than $75,000/QALY rarely recommended (OECD)
National Centre for Pharmacoeconomics - Ireland (NCPE) • €45,000/QALY	Pharmaceutical Management Agency (PHARMAC) – New Zealand • They "fund medicines within a fixed budget, and as CE is only one of its nine decision criteria used to inform decisions, thresholds cannot be inferred or calculated"
Health Intervention and Technology Assessment Program (HITAP) – Thailand • 160,000 Baht per QALY (approx. 1.2 x GNI per capita) – "demand side" threshold	Canadian Agency for Drugs and Technologies in Health (CADTH) – Canada • Use a "supply side threshold" but not stated

Source: Thokala et al, 2018

What is your "threshold"?

If the concern is to improve ***population health***, need to consider ***opportunity costs*** – that is comparing the health benefits gained from an intervention with the health that is likely to be lost as a consequence of additional investments

Sources of thresholds: The perils of a threshold not linked to WTP = ‘Cost Effective’ and Unaffordable

World Health Organization

“In low and middle income countries, the World Health Organization (WHO) has recommended thresholds of **1 to 3 times gross domestic product** (GDP) per capita – seemingly on the basis of recommendations from the “Commission on Macroeconomics and Health” report from 2001.”(1)

NICE
National Institute for Health and Care Excellence

“For instance, values of **GB£20-30,000** and **US$50,000** per QALY have commonly been applied in the United Kingdom and United States, respectively; without clear rational but with some sense they reflect the consumption value of health.”(1)


Value in Health
Volume 21, Issue 3, March 2018, Pages 266-275
Resolving the “Cost-Effective but Unaffordable” Paradox: Estimating the Health Opportunity Costs of Nonmarginal Budget Impacts

“To say that an alternative is cost-effective but not affordable must mean that the (implicit or explicit) “threshold” used to judge cost-effectiveness **does not reflect the opportunity costs** incurred given the scale of the impact on health expenditure” (Lomas et al 2018)

New Intervention is cost-effective if it falls below a CE Threshold determined by Willingness to Pay

1) Cost-Effectiveness Thresholds iDSI working group final report

NICE decision options

NICE can:

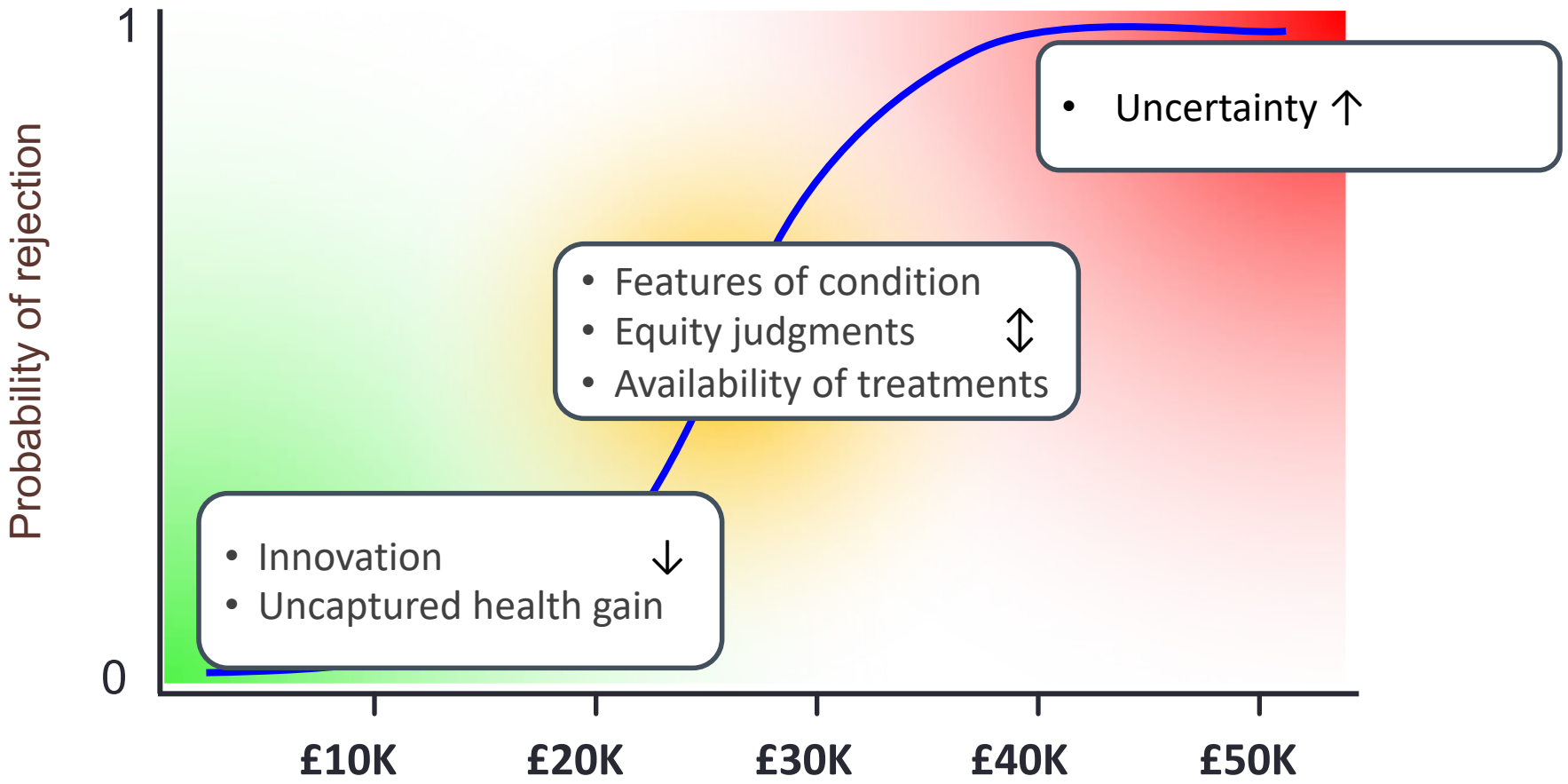
- Recommend for routine commissioning (either in line with marketing authorisation or “optimised”)
- Not recommend for routine commissioning
- Recommend for inclusion in the Cancer Drugs Fund or other managed access

Health professionals are expected to take NICE guidance fully into account when exercising their clinical judgment; though guidance cannot override professional autonomy

The NHS (in England) is obliged to provide funding & resources for medicines & treatments recommended by NICE –within 3 months ***[this has now been revised]***

All NICE guidance is reviewed within 3 years and may or may not be updated

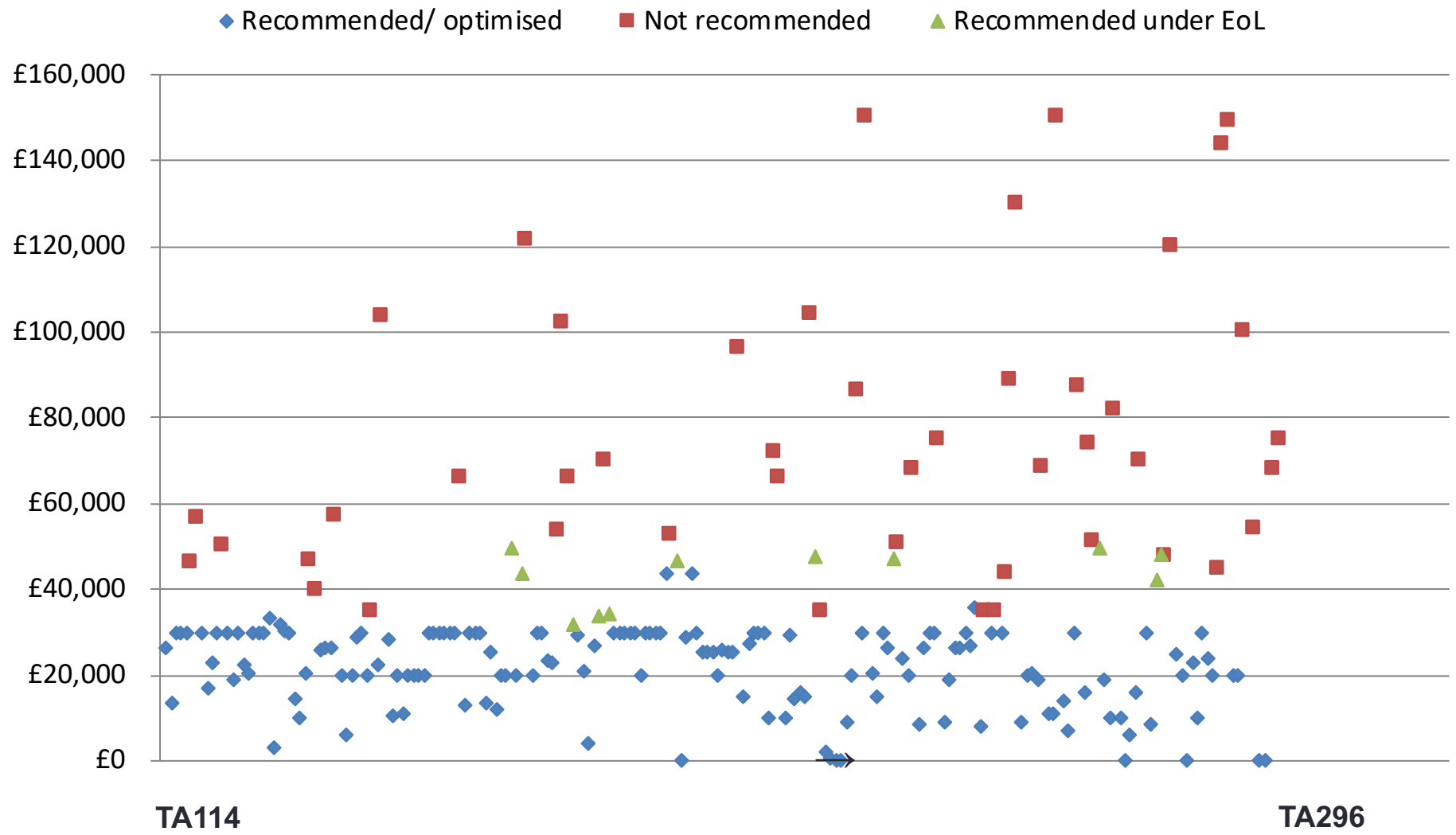
NICE's decision making



The Committee will want to be increasingly certain of the cost-effectiveness of a technology as the impact of the adoption of the technology on NHS resources increases

(Para 6.2.14 Guide to Methods of Technology Appraisal, NICE 2013)

NICE decisions based on value for money: 2007 – 2013: the ICER matters



NICE's threhold

- NICE "does not use a precise maximum acceptable ICER above which a technology would automatically be defined as not cost effective or below which it would".*
- £20,000 to £30,000 per QALY gained range
- Below £20,000 will recommend treatment, above £20,000 a case can be made e.g. the change in HRQL has been inadequately captured, or distinctive benefits not adequately captured in the QALY measure
- "Above a most plausible ICER of £30,000 per QALY gained … need to identify an increasingly stronger case"
- £50,000 for life-extending end-of-life treatments

*Guide to the Methods of Technology Appraisal, April 2013

An inherently political and social engagement process

- "Those developing *clinical guidelines, technology appraisals or public health guidance* must take into account the relative costs and benefits of interventions (their 'cost effectiveness') when deciding whether or not to recommend them." (Principle 2, SVJ, NICE 2008)

BUT

- "Decisions about whether to recommend interventions should *not be based on evidence of their relative costs and benefits alone*. NICE must consider other factors when developing its guidance, including the need to distribute health resources in the fairest way within society as a whole." (Principle 3)
- See: http://www.nice.org.uk/media/C18/30/SVJ2PUBLICATION2008.pdf

Application of 'special circumstances'

Table 1

Application of 'special circumstances' in the appraisal of some products with incremental cost-effectiveness above £30 000 per quality adjusted life year

Topic	ICER ('000s)	Severity	End of life*	Stakeholder persuasion	Significant innovation	Disadvantaged population	Children
Riluzole (motor neurone disease)	38–42	✓	✓	✓			
Trastuzumab (advanced breast cancer)	37.5	✓			✓		
Imatinib (chronic myeloid leukaemia)	36–65	✓			✓		
Imatinib (gastrointestinal stromal tumour)		✓	✓		✓		
Pemetrexed (malignant mesothelioma)	34.5	✓	✓			✓	
Ranizumab (age-related macular degeneration)	>>30			✓	✓		
Omalizumab (severe asthma)	>30	✓		✓	✓		
Sunitinib (advanced renal cancer)	50	✓	✓	✓	✓		
Lenalidomide (multiple myeloma)	43	✓	✓		✓		
Somatotropin (growth hormone deficiency)	n/a			✓	✓		✓
Chronic subcutaneous insulin infusion (childhood Type 1 diabetes)	n/a			✓			✓

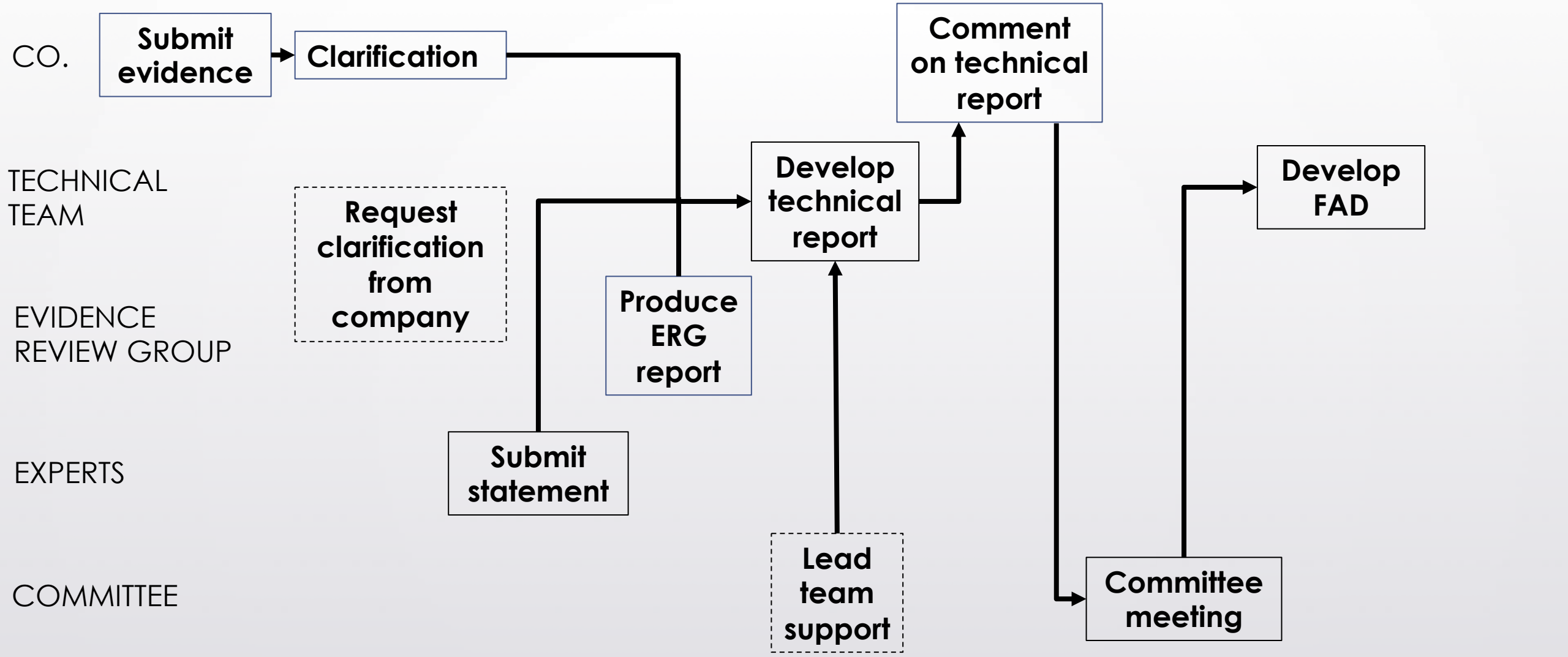
*End-of-life considerations have only been explicitly taken into account since January 2009 on the basis of supplementary advice from the Institute to the Appraisals Committee.
ICER, incremental cost-effectiveness ratio (£ per quality-adjusted life year).

Rawlins, Barnett, Stevens Br J Clin Pharmacol 2010

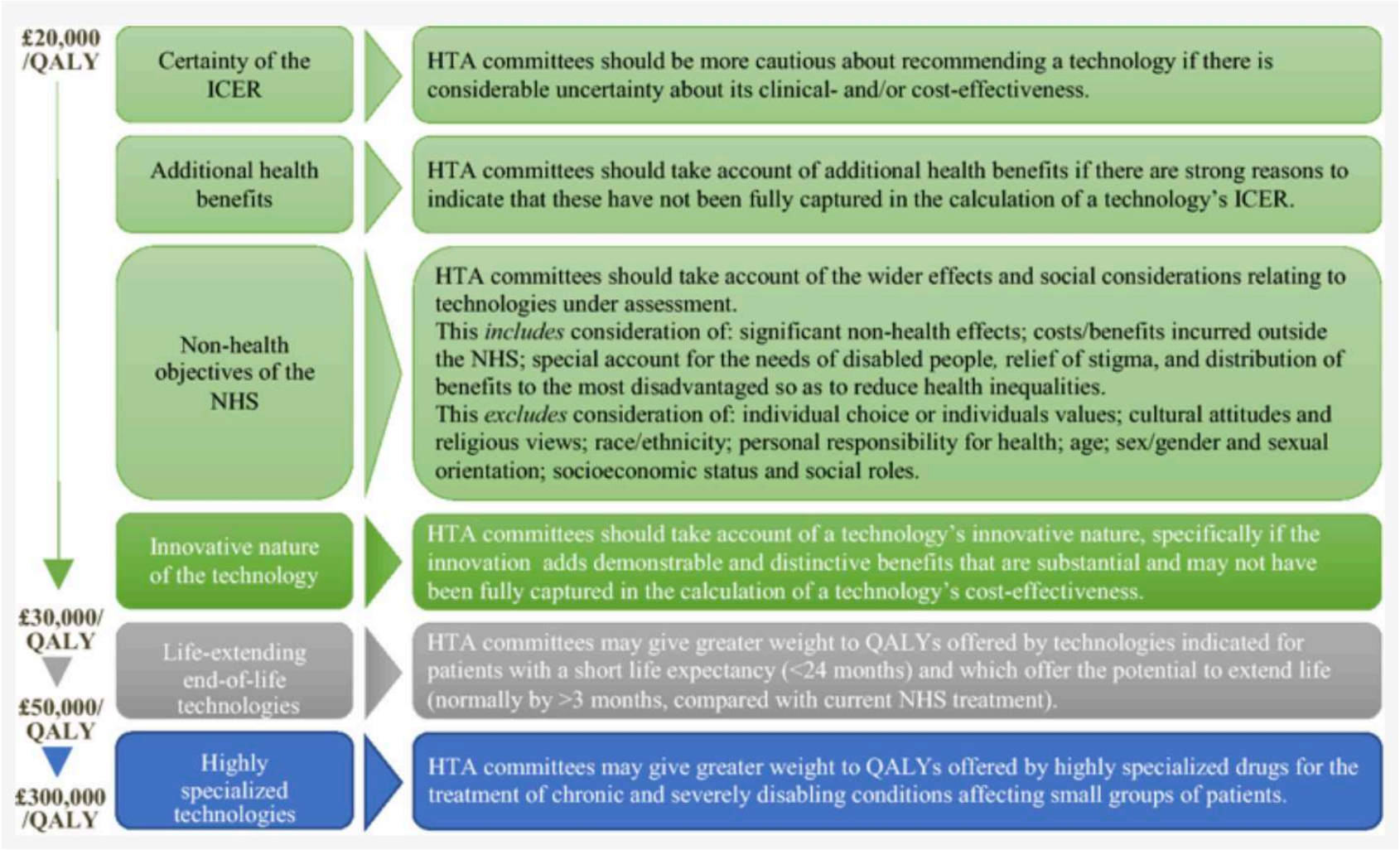
NICE processes

- Multiple Technology Appraisal (MTA)
  - First appraisal completed Apr 2000
  - Normally covers more than one technology, or one technology for more than one indication
  - An extensive review of the evidence (planned to be completed in 62 weeks)
- Single Technology Appraisal (STA)
  - "rapid" review process first guidance issued Aug 2006
  - STA can only cover a single technology for a single indication
  - New STA process since 2018
- Fast Track Appraisal (FTA) from 1 Apr 2017
  - "those technologies that NICE can be confident would fall below £10,000 per QALY"
  - e.g. aflibercept for treating myopic choroidal neovascularisation

Reproduced : Professor John Cairns, LSHTM


CO.
Submit evidence
Clarification
Comment on technical report
TECHNICAL TEAM
Develop technical report
Develop FAD
Request clarification from company
EVIDENCE REVIEW GROUP
Produce ERG report
EXPERTS
Submit statement
Lead team support
COMMITTEE
Committee meeting
EVIDENCE SUBMISSION
ERG REPORT
TECHNICAL CONSULTATION
AC MEETING
FAD
FINAL GUIDANCE
Day
60
80
90
120
150
210
245
290

But increasingly NICE's threshold has been going up…



https://link.springer.com/article/10.1007/s11211-019-00333-9

£100,000 threshold for highly specialised technologies*

- * Chronic & severely disabling condition, where the technology has the potential for life long use, usually in very few centres in the NHS & likely to have a very high acquisition cost.
- Introduced QALY weights (proportional to the incremental QALYs gained) up to a maximum of 3

Incremental QALYs gained (per patient, using lifetime horizon)	Weight versus £100,000/QALY
Less than or equal to 10	1
11-29	Between 1 and 3 (using equal increments)
Greater than or equal to 30	3

Reproduced : Professor John Cairns, LSHTM

Highly Specialised Technologies

- Technology appraisals can be for fairly rare conditions, e.g. nusinersen for treating spinal muscular atrophy [between 1,200 & 2,500 children & adults in UK]
- Criteria to be met to be highly specialised technology
  - The **target patient group** in its licensed indication is so small that treatment will usually be concentrated in very few centres in the NHS
  - The **condition** is chronic and severely disabling
  - The **technology** is expected to be used exclusively in the context of a highly specialised service; is likely to have a very high acquisition cost & has the potential for life long use.

Reproduced : Professor John Cairns, LSHTM

NICE in the future

A number of areas of strategic interest reflecting the growing importance and potential opportunities offered by digitalisation, ‘big data’, machine learning….

1. Maintaining recommendations up to date
2. Rapid sequencing of new drugs and technologies
3. Integrating recommendations into IT systems
4. Improving the accessibility of NICE recommendations (putting advice and guidance into a single “integrated product” on NICE website)

…..”more and faster appraisals” (The 2019 Voluntary Scheme for Branded Medicines Pricing and Access)

NICE announces details of health technology evaluation methods review

Following approval at its recent Board meeting NICE has confirmed the details of its review of the methods it uses to develop guidance on drugs, medical devices and diagnostics.

22 July 2019 Share

The purpose of the review is to optimise NICE's evaluation methods to support the ambition of the NHS to provide high quality care that offers good value to patients and to the NHS. For medicines, the review is linked to the commitments in the 2019 Voluntary Scheme for Branded Medicines Pricing and Access.

> This update is part of the regular review and refresh of our methods to ensure that they are robust and up-to-date.

Engagement with key stakeholders has resulted in a short-list of topics that will be

Department of Health & Social Care

abpi

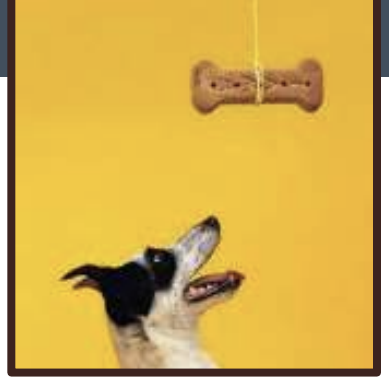
The 2019 Voluntary Scheme for Branded Medicines Pricing and Access - Chapters and Glossary

1.4 The National Institute for Health and Care Excellence (NICE) also supports the Voluntary Scheme and will have a central role in its operation.

3.20 The standard cost effectiveness threshold used by NICE will be retained at the current range (£20,000 - £30,000 per QALY) and not changed for the duration of the Voluntary Scheme.

NICE AND DRUG PRICING

The drugs' market in the UK



the person who consumes the drug (the patient) neither decides nor, in most cases, pays

the person who decides which drug should be used (the prescribing doctor) neither pays nor consumes, and

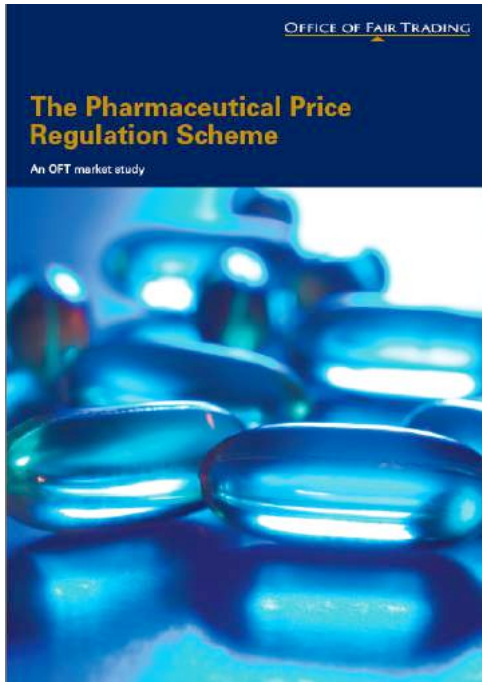
the institution that pays for the drug (the NHS / Government) neither consumes nor decides.

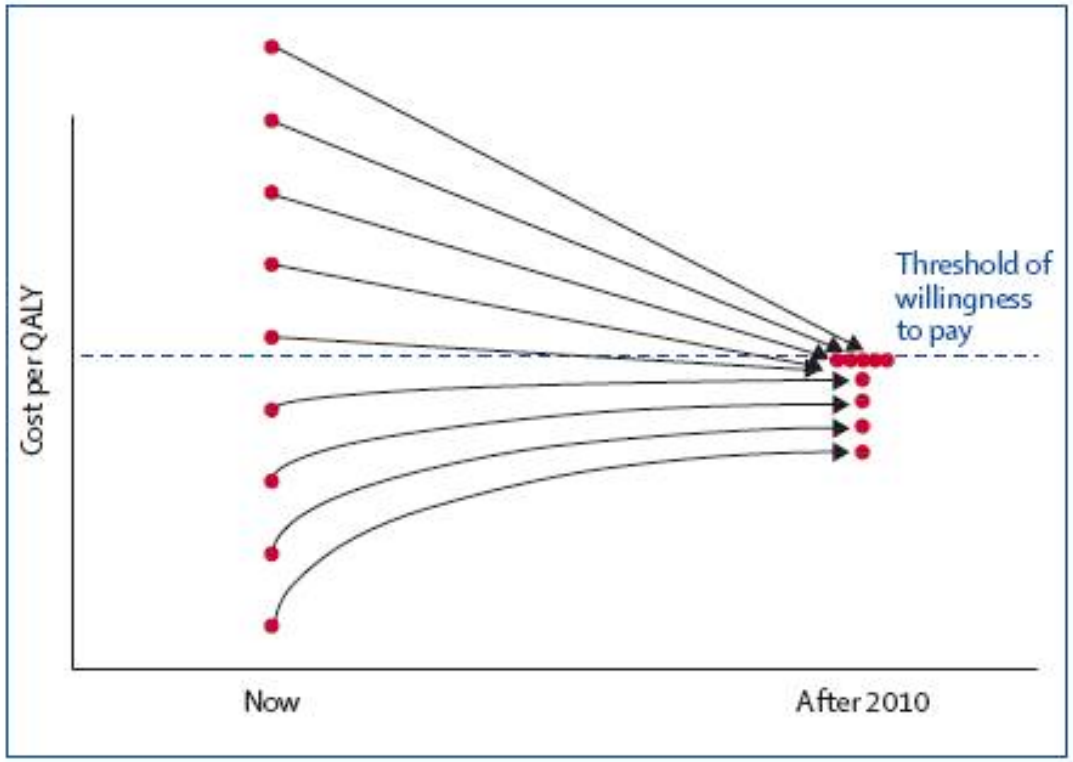
Misaligned incentives mean market forces alone cannot fix the problem: government regulation is necessary!

Office for Fair Trading called for pricing reform but…

- **"We recommend that Government reform the PPRS replacing current profit and price controls with a value based approach to pricing** to ensure the price of drugs reflect their clinical and therapeutic value to patients and the broader NHS."

OFT, February 2007





Webb and Walker, Lancet 2007

…HTA can be inflationary!

Monday 15 August 2016 Campaigns and policy

International comparisons of Health Technology Assessment

A report from Breast Cancer Now and Prostate Cancer UK

"…**more flexibility** [should] be brought into the system **to allow price negotiation**, as happens in other countries."

A new report by leading charities Breast Cancer Now and Prostate Cancer UK shows NHS cancer patients in the UK are missing out on innovative treatments being made available in some comparable countries of similar wealth.

NICE does not do pricing but…NICE can:

01

Signal that it requires a price reduction to offer a positive recommendation and ultimately reject

02

Negotiate confidential price discounts (increasingly done by NHS England) in the context of a managed entry agreement

03

Recommend a cancer drugs enters the Cancer Drugs Fund for a limited period and re-evaluate

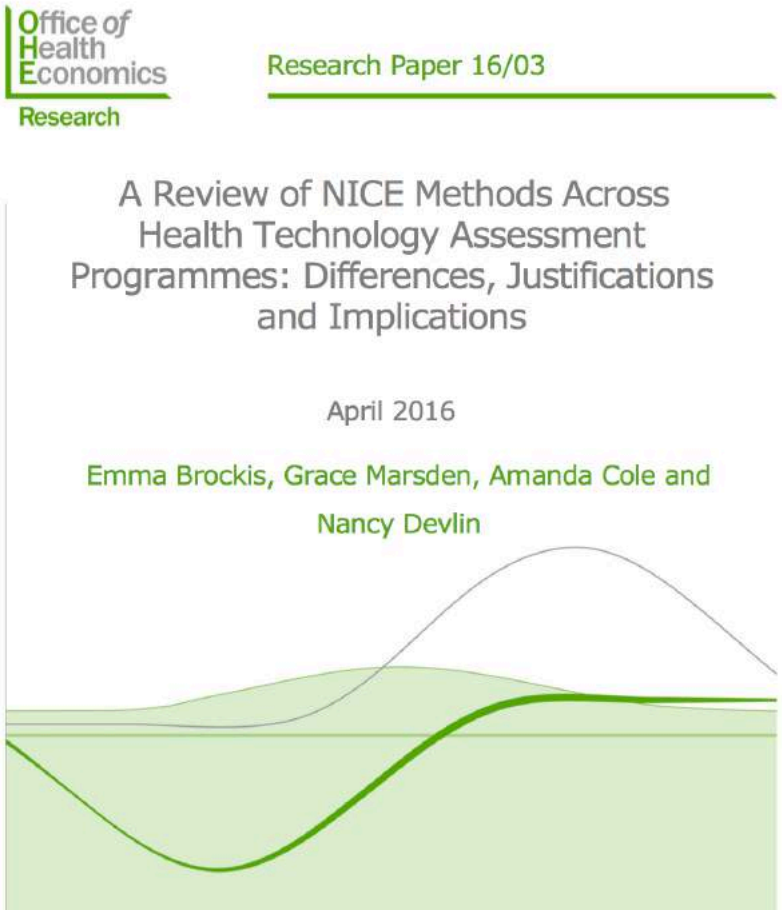


Table 1: Remit and Scope of each NICE HTA programme

	Technology Appraisal Programme	Medical Technologies Guidance	Diagnostics Assessment Programme	Highly Specialised Technology Programme	Clinical Guidelines
What is appraised?	Medicines, medical devices, diagnostics, surgical procedures, therapeutic technologies, systems of care, screening tools.	Medical devices (active, active implantable, in vitro), genetic tests.	Diagnostic technologies/ tests, genetic tests.	Drugs for very rare conditions.	Condition specific care and services.
Referral	Primarily HSRIC; Formal referral required from Secretary of State for Health.	Primarily product sponsors; Also HSRIC.	Product sponsors, national clinical directors, medical royal colleges, professional bodies, national expert bodies, or HSRIC.	Primarily HSRIC; Formal referral required from DH.	Topic oversight group.
Selection/ routing	Must have been granted, or be soon to receive, marketing authorisation; Significant benefit to patients; new formulation at lower price; appropriate evidence available.	Have CE mark (or expected within 1 year); New or innovative technology; Cost saving or cost neutral technology.	CE marking (before publication); Potential to improve health outcomes, but at an increased cost to the NHS.	Criteria same as those used by AGNSS; Process similar to TAP.	Priority topics and those where existing NICE guidance does not cover the whole topic.
Prioritisation criteria	Significant health benefit; Significant impact on NHS resources and other government policies; Inappropriate variation in the use across the country.	Provide most benefit to patients and the NHS; Scoring system.	Particular urgency to the NHS.	Not stated.	Discussion between NHS England, DH and Public Health England.

Source: NICE (2011a), NICE(2011b), NICE(2011c), NICE(2011d†), NICE(2011e†), NICE (2013a), NICE (2013b), NICE (2013c), NICE (2015a), NICE (2015b), NICE (2015c).
Abbreviations: AGNSS: Advisory Group for National Specialised Services; CE mark: European Conformity mark; DH: Department of Health; HSRIC: Horizon Scanning Research & Intelligence Centre.

And its methods getting even more complex!

Its processes getting more complex

Figure 4 Summary of the appraisal process

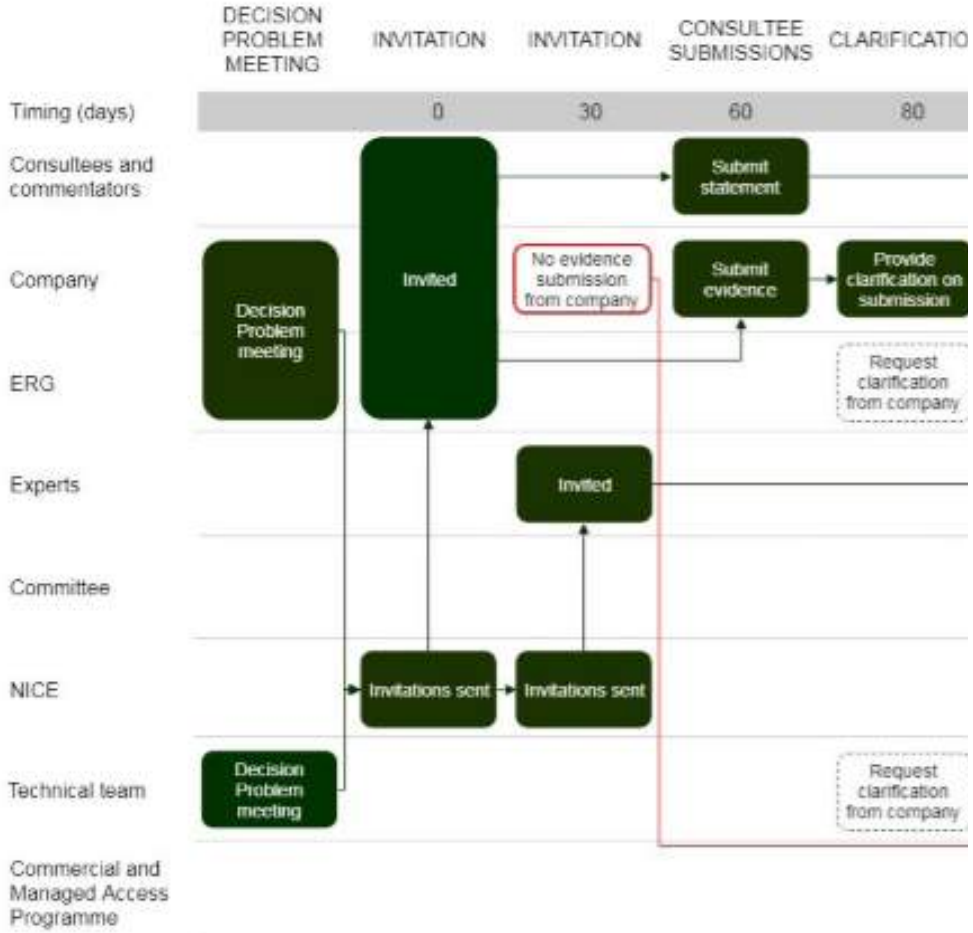
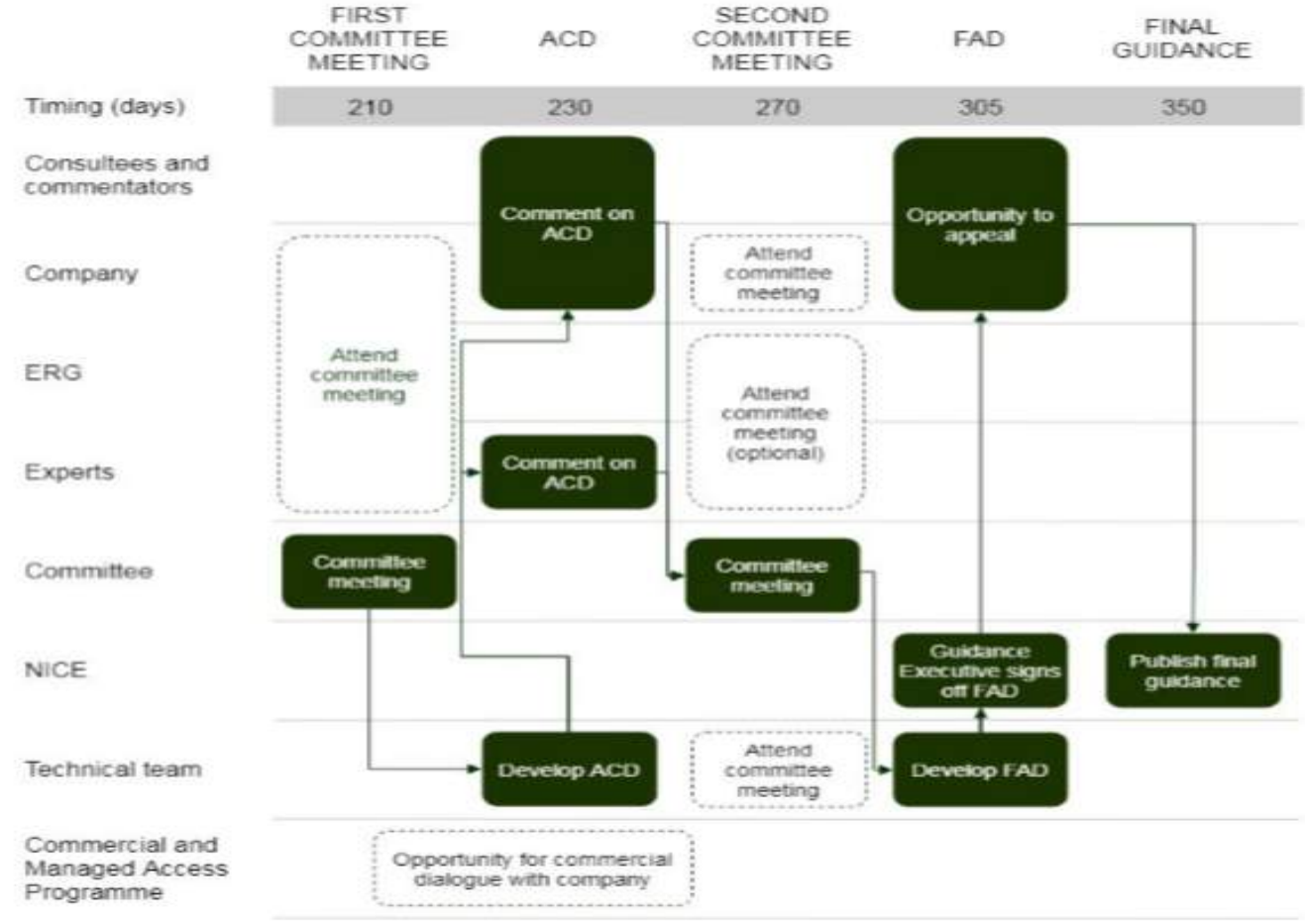


Figure 5 Summary of the appraisal process when an ACD is produced



***Further details*:**
https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/technology-appraisal-processes-guide-apr-2018.pdf

Drug prices

- Current practice: cost-effectiveness of new drugs assessed at the price set by the company
- If NICE looks like saying NO at the initially proposed price companies increasingly offer a **Patient Access Scheme**.
- Also all drugs entering the 2016 CDF have a commercial arrangement
- These schemes involve pricing agreements designed to improve cost effectiveness & facilitate patient access to specific drugs.

Reproduced : Professor John Cairns, LSHTM

Pricing arrangements 2007-2018

- About 4 per year 2007-2011 [42% discounts on list price]
- Over 12 per year 2012-2015 [94% discounts on list price]
- About 40 per year 2016-2018 [92% discounts on list price]

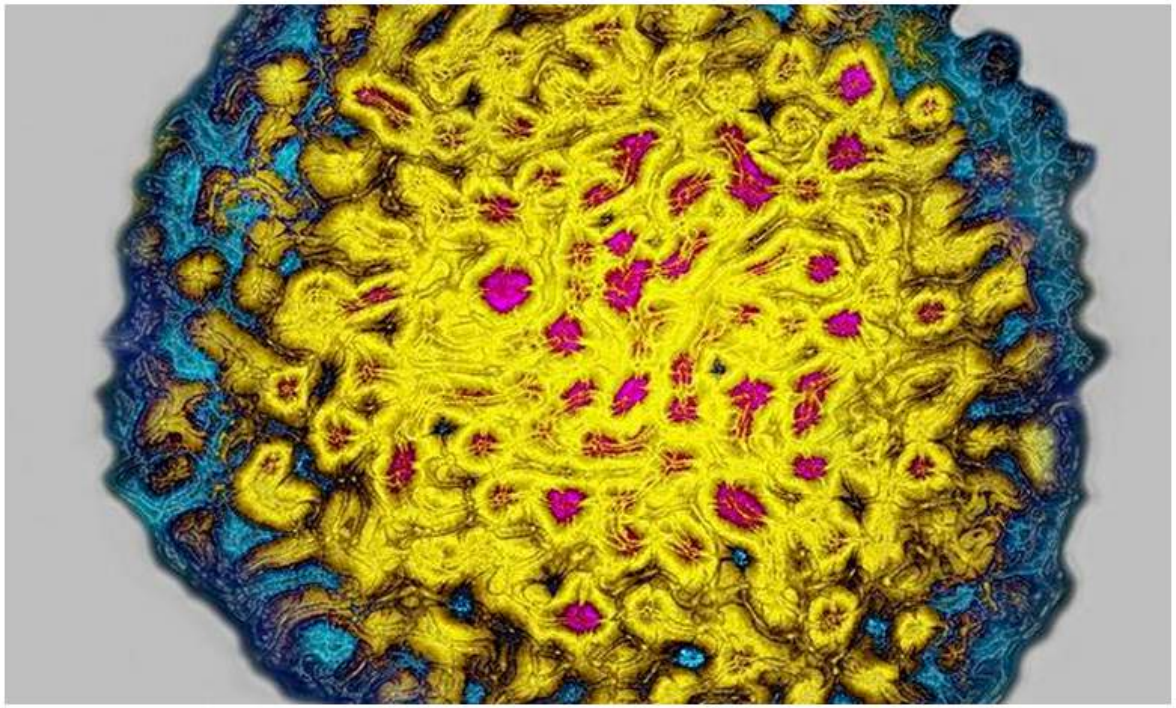
Outcome based	Patient level	***1***	Refund for patients who do not reach agreed target
Non-outcome based	Patient level	***21***	Free stock for initial limited period or after a dose cap
		1	Fixed cost per patient
	Population level	***164***	Discount on list price

Reproduced : Professor John Cairns, LSHTM

Cancer not the main/only problem…
Sovaldi: "Cost-effective" but unaffordable?

Hepatitis C drug delayed by NHS due to high cost

NHS England balks at bill for dispensing sofosbuvir: £1bn for every 20,000 people treated



The price offered by Gilead in the UK is almost £35,000 for a 12-week course. Many patients will need a 24-week course, costing £70,000. In its final draft guidance on sofosbuvir, Nice said it was allowing NHS England to postpone implementation for four months, until the end of July instead the beginning of April. NHS England failed to comment.

When budgets don't follow recommendations

Feature

A pill too hard to swallow: how access to high priced drugs

A joint investigation by *The BMJ* and Cambridge a
how NHS England tried to limit access to expensiv
Jonathan Gornall, Amanda Hoey, and Piotr Ozierar

1 response

Analysis: Betting on hepatitis C: how financial specula
access to medicines

Editorial: High cost of new drugs

February 19, 2015 12:00 am

Expensive drugs cost lives, claims report

Andrew Ward, Pharmaceuticals Correspondent

The adoption of expensive new drugs by the NHS is doing patients more harm than good, according to a study that urges a sharp reduction in the price pharmaceuticals companies are paid for their products.

Research by the University of York found that lives were being lost and quality of life diminished because spending on overpriced drugs was diverting resources from other kinds of healthcare that would produce more benefit.

Runaway threshold? Putting a break on NICE…

- Introduce a 'fast track' NICE technology appraisal process for the most promising new technologies, which fall below an incremental cost-effectiveness ratio of £10,000 per QALY (quality adjusted life year), to get these treatments to patients more quickly.
- Operate a 'budget impact threshold' of £20 million, set by NHS England, to signal the need for a dialogue with companies to agree special arrangements to better manage the introduction of new technologies recommended by NICE. This would apply to a small number of technologies that, once determined as cost effective by NICE, would have a significant impact on the NHS budget.
- Vary the timescale for the funding requirement when the budget impact threshold is reached or exceeded, and there is therefore a compelling case that the introduction of the new technology would risk disruption to the funding of other services.
- Automatically fund, from routine commissioning budgets, treatments for very rare conditions (highly specialised technologies) up to £100,000 per QALY (5 times greater than the lower end of NICE's standard threshold range), and provide the opportunity for treatments above this range to be considered through NHS England's process for prioritising other highly specialised technologies.

Capping NICE's spending

Budget impact

- NICE has historically not considered budget impact when making recommendations
- However, since 1 April 2017 it is routinely asked whether a positive recommendation will increase spending by more than £20 million in any of the next 3 years
- If it is likely to do so, NHS England can delay implementation from 3 months (current rule) to up to 3 years
- This increases their opportunity to bargain with manufacturers (developed partly in response to the challenges of paying for NICE's positive HCV recommendations).

Reproduced : Professor John Cairns, LSHTM

The new PPRS: capping growth—industry reimburses the NHS

Table 1: forecasts and profile of annual payment percentages

	2014	2015	2016	2017	2018

As the unadjusted

Period	Aggregate net sales covered by the PPRS payment Column 1	Resulting aggregate PPRS payments Column 2
2013	£7,901M	N/A
2014	£8,340M	£311M
2015	£8,179M	£847M
2016	£8,062M	£628M
2017	£8,147M	£387M
2018 Q1	£2,003M	£156M
2018 Q2	£2,013M	£157M
2018 Q3	£1,968M	£153M
2018 Q4	£1,903M	£148M

https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/668673/PPRS_Payment_percentage_2018.pdf
https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/761834/voluntary-scheme-for-branded-medicines-pricing-and-access-chapters-and-glossary.pdf

The new drug pricing landscape

NHS

The NHS Long Term Plan

Published 7th January 2019

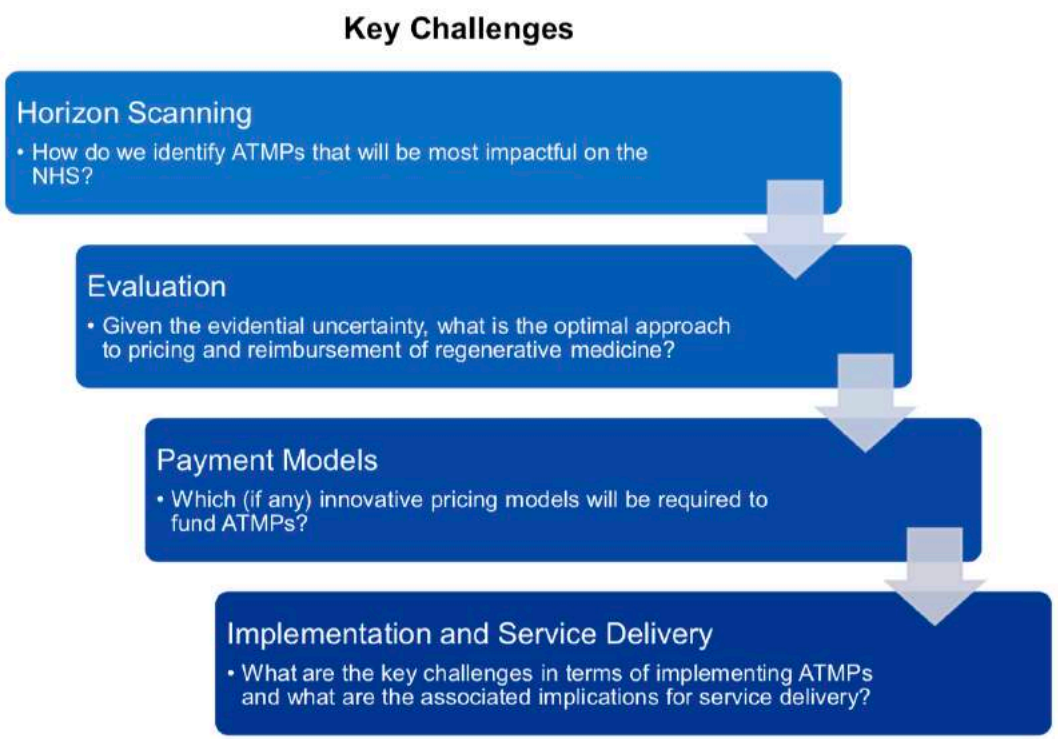
Department of Health & Social Care

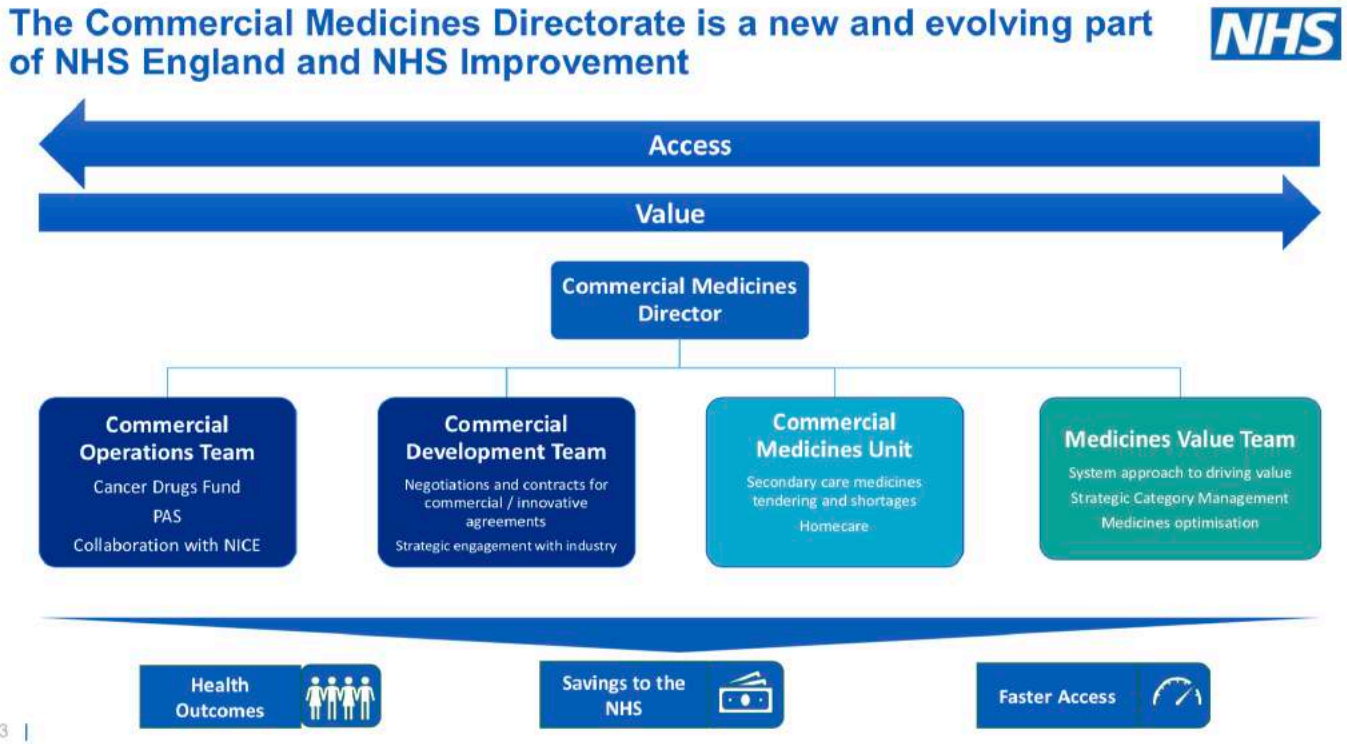
abpi

The 2019 Voluntary Scheme for Branded Medicines Pricing and Access - Chapters and Glossary

Published 5th December 2018

The NHS is now in charge:
→gene therapies: the new frontier





ENGLAND'S VERTICAL FUND FOR CANCER DRUGS: A CAUTIONARY TALE

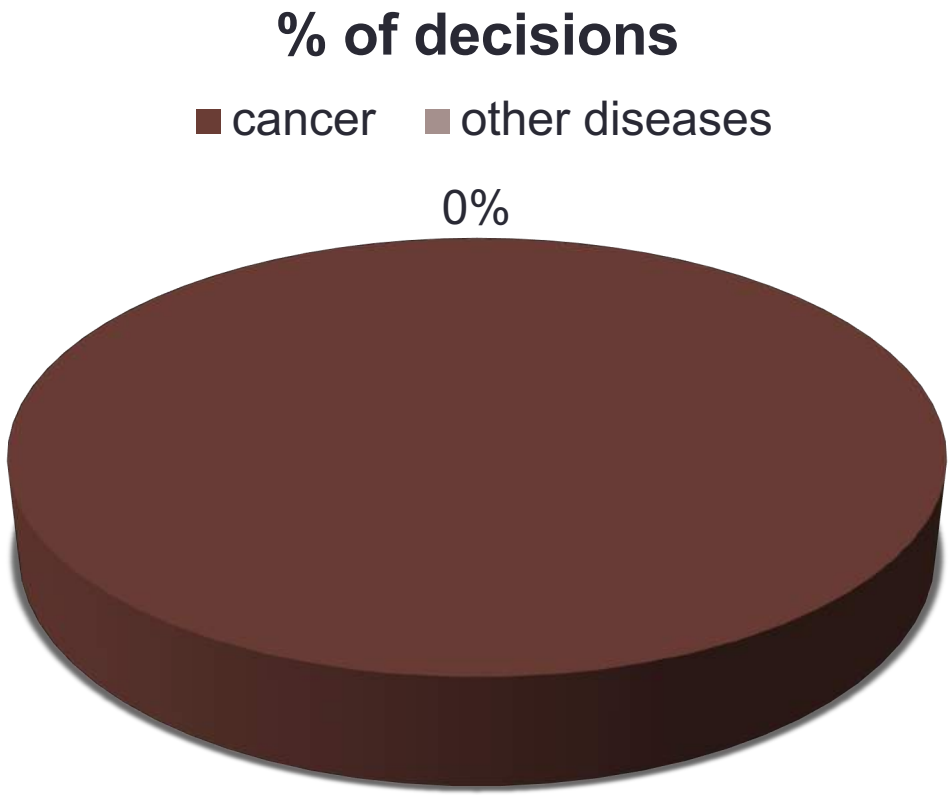
Policies for improving timely access to new cancer drugs

- Single Technology Appraisal (2005)
- NICE End-of-Life policy (2008/9)
- Cancer Drugs Fund (2010/11)
- Orphan drugs evaluation (2013/14)
- Value Based Assessment (2014/closed down)
- CDF as part of NICE (2016)

The launch of NICE's End-of-Life policy (2009)

- "A QALY is a QALY is a QALY" NICE Methods Manual 1999-2009
- NICE is asking that its advisory committees "consider recommending seemingly cost-ineffective treatments which are life-extending for patients with short life expectancy, and which are licensed for indications affecting small numbers of patients with incurable illnesses." NICE Supplementary Guidance to its Advisory Committees – January 2009

End of Life decisions as of May 2014



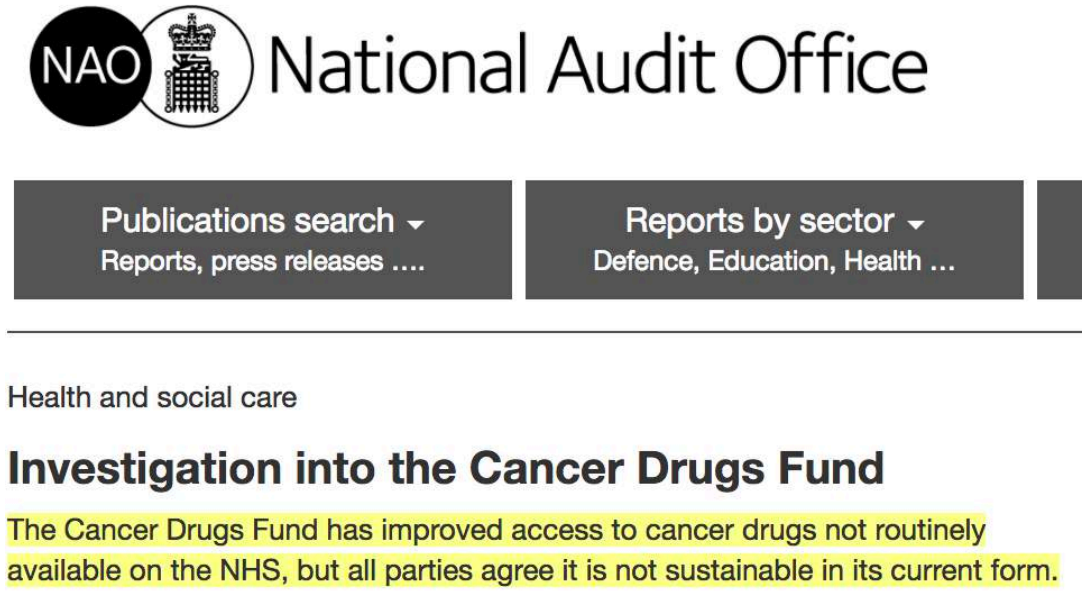
But NICE's committees still find some cancer drugs not to be good value for money or clinically effective

An election promise

Cancer Drugs Fund in pre-election manifesto

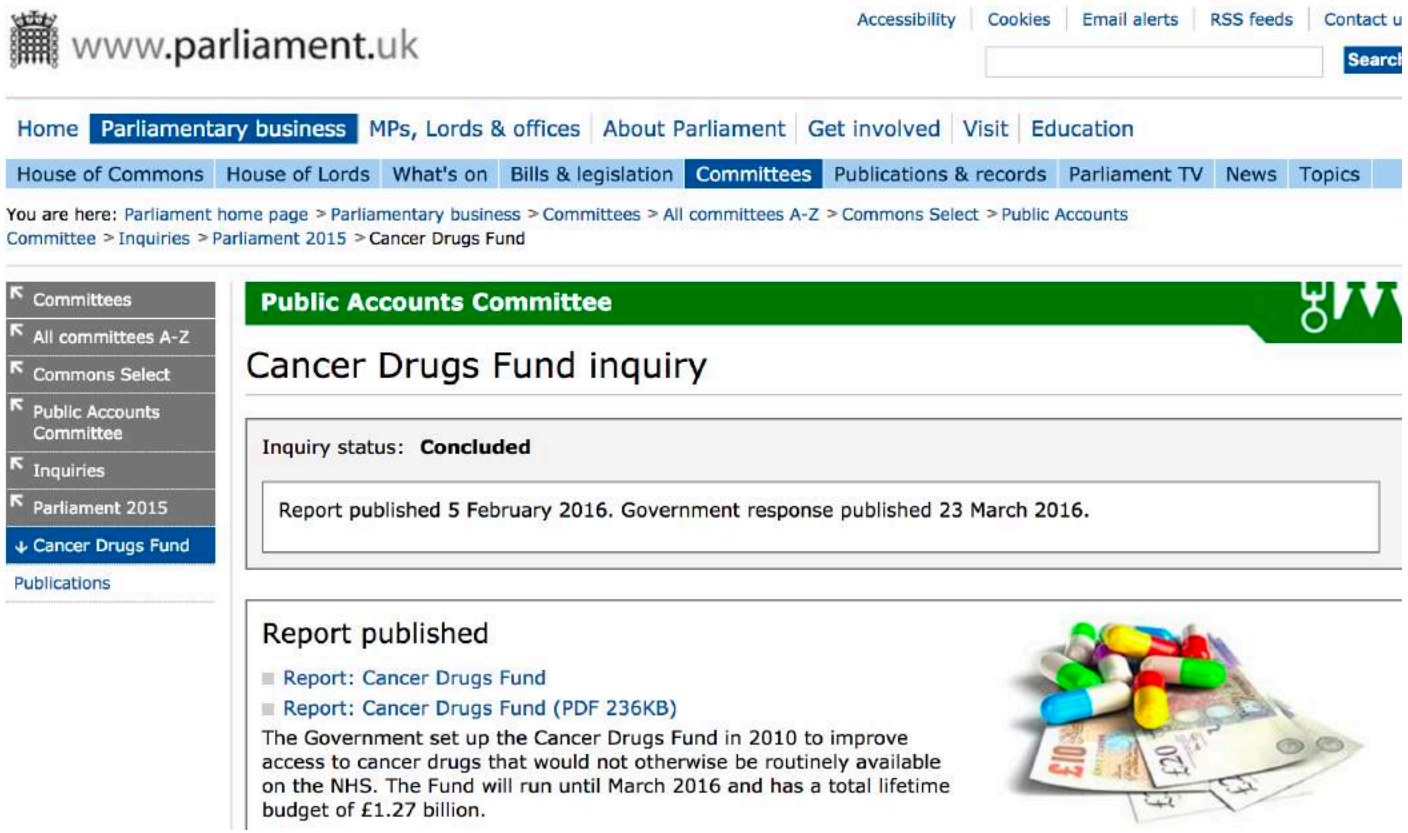
"We will create a Cancer Drugs Fund to enable patients to access the cancer drugs their doctors think will help

Sep 2015: the country's National Audit Office investigates



- **"Did it improve outcomes?** Due to a lack of data, it is not possible to evaluate the impact that the Fund has had on patient outcomes, such as survival.
- **What impact did it have on prices?** The cost of the Fund from 2010 to 2015 was £968 million, slightly above the allocated budget. In the early years [it] was underspent. However, taking 2013-14 and 2014-15 together…the cost of the Fund rose by £241 million – an increase of 138%. Over half of the rise was because of an increase in the average cost of treatment per patient…"

February 2016: The country's Parliament investigates



- "There is no assurance that the Department and NHS England are using their buying power effectively to pay **a fair price** for cancer drugs, including drugs paid for through the Fund.
- It is unacceptable that the Department and NHS England still **do not have data to evaluate the impact of the Fund on outcomes** for patients five years after the Fund was set up."

The press

"a populist gesture that gives the impression of benefiting patients, but in fact rewards poor quality drugs while benefiting a handful of pharmaceutical companies at the expense of the taxpayer and the full range of NHS patients" Dec 2014

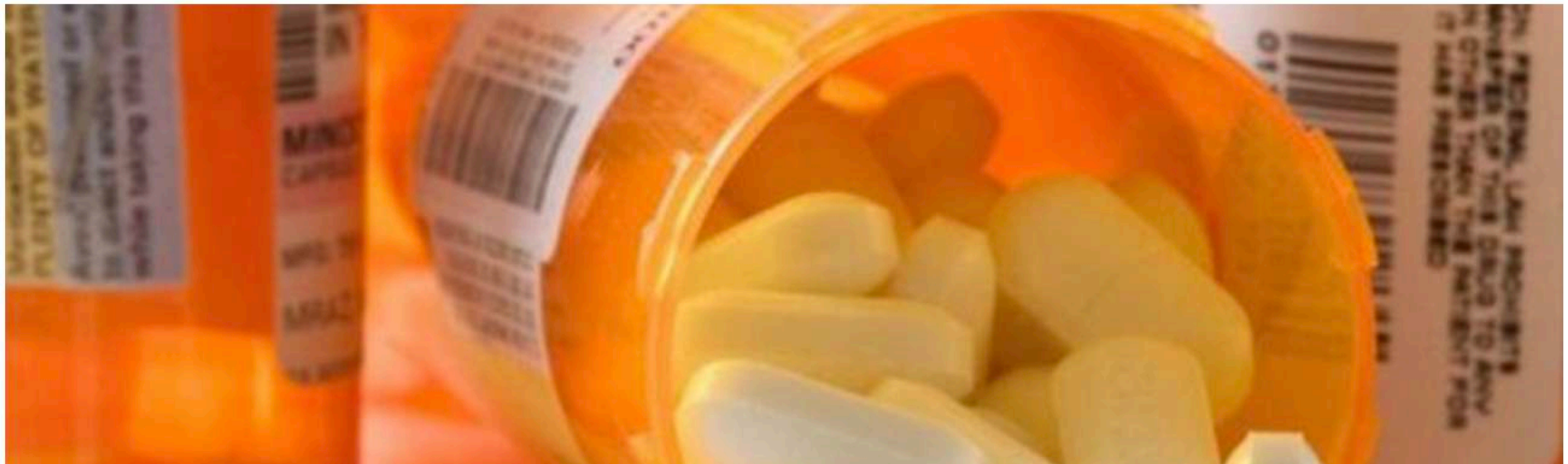
"This mechanism for diverting taxpayers' money to enhance, to little or no purpose, the profits of Big Pharma might be more aptly named "the Drug Company Fund"" Dec 2014

Health

Cancer Drugs Fund 'huge waste of money'

By Nick Triggle
Health correspondent

28 April 2017



The Payer takes back control: NHS England

- Access to promising new treatments, via managed access arrangement, while further evidence is collected to address clinical uncertainty.
- Interim funding for all newly recommended cancer drugs, giving patients access to these treatments many months earlier than before.
- **The expenditure control mechanism ensures that the CDF will not overspend.**

Appraisal and Funding of Cancer Drugs from July 2016 (including the new Cancer Drugs Fund)

A new deal for patients, taxpayers and industry

The new arrangements cap the total, set up companies and products to compete against one another and make the whole idea of the CDF “unappealing”

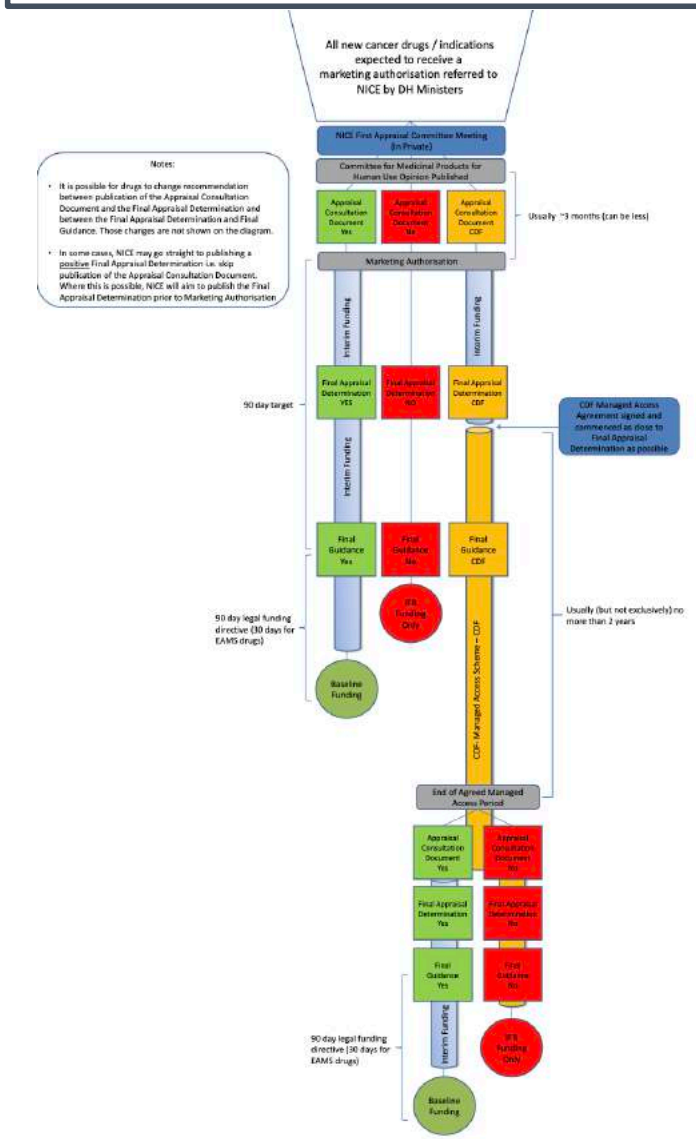
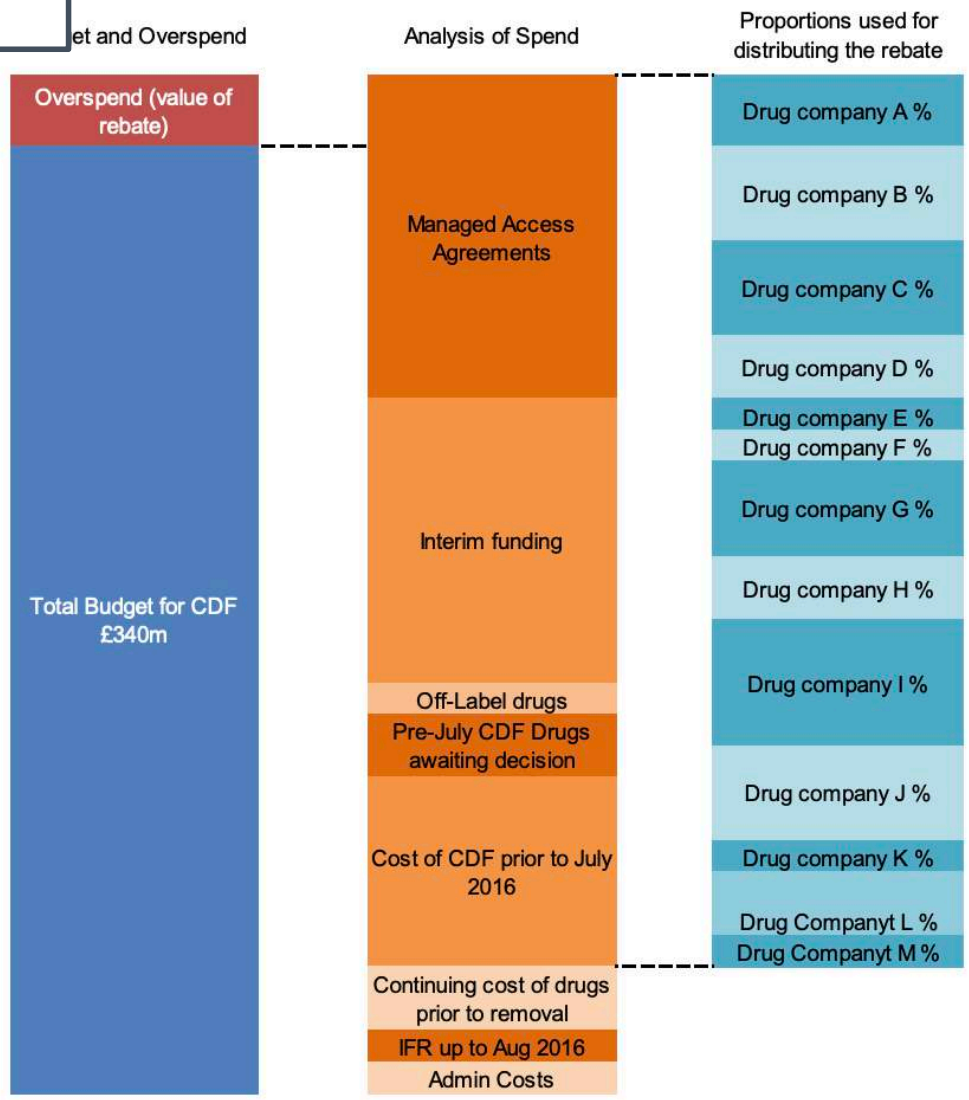
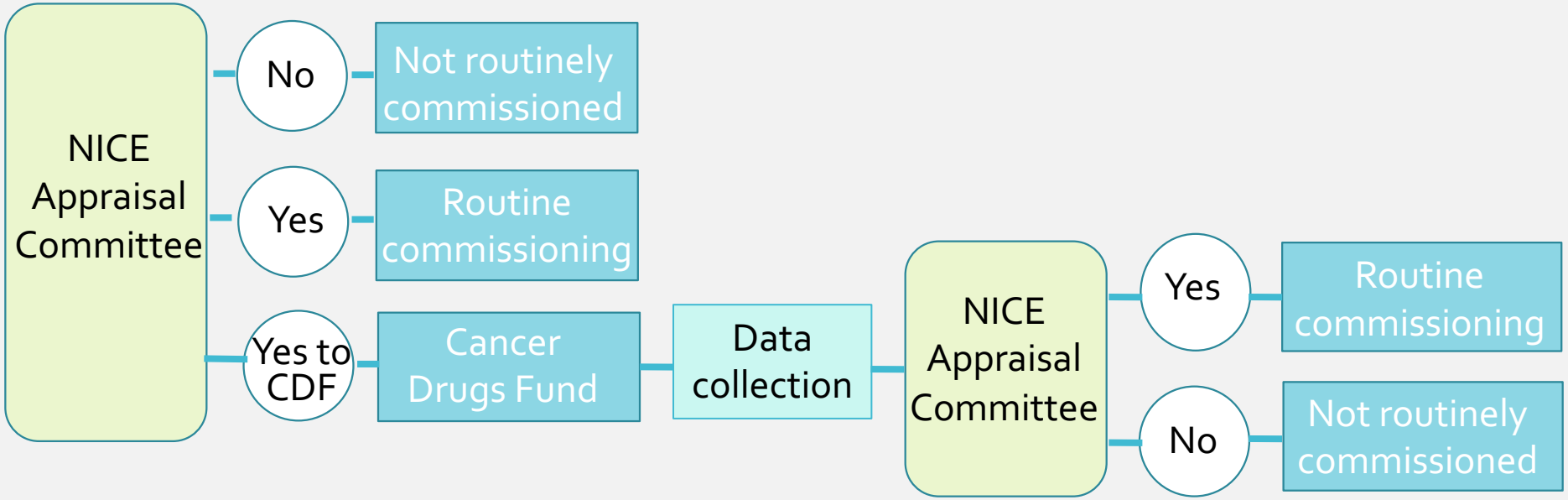


Diagram Showing Methodology for the Calculation of the Retrospective Rebate



Size of the blocks is for illustrative purposes only and does not represent a forecast.

Cancer Drugs Fund 2016



Based on NHS England Board Paper PB.25.02.2016/04 Appendix 2

Reproduced : Professor John Cairns, LSHTM

2016 CDF criteria

Proceed down if answer to each question is yes

Starting point: drug not recommended for routine use due to clinical uncertainty

1. Is the model structurally robust for decision making (omitting the clinical uncertainty)?

2. Does the drug have plausible potential to be cost-effective at the offered price, taking into account end-of-life criteria?

3. Could further data collection reduce uncertainty?

4. Will ongoing studies provide useful data?

and

5. Is CDF data collection via SACT relevant and feasible?

Consider recommending entry into the CDF (invite company to submit CDF proposal)

Reproduced : Professor John Cairns, LSHTM

Did the CDF deliver value for the English society?

The evidence:

- Of the 47 CDF approved indications, only 18 (38%) reported a statistically significant OS benefit, with an overall median survival of 3.1 months
- When assessed according to clinical benefit scales, only 23 (48%) and 9 (18%) of the 47 drug indications met ASCO and ESMO criteria, respectively.
- NICE had previously rejected 26 (55%) of the CDF approved indications because they did not meet cost-effectiveness thresholds.
- Four drugs—bevacizumab, cetuximab, everolimus and lapatinib—represented the bulk of CDF applications and were approved for a total of 18 separate indications. 13 of these w… …ical b…
- T… …efit.
- £…
- N… …nt w… …ide- e…

Conclusions

We conclude the CDF has not delivered meaningful value to patients or society. There is no empirical evidence to support a ‘drug only’ ring fenced cancer fund relative to concomitant investments in other cancer domains such as surgery and radiotherapy, or other noncancer medicines. Reimbursement decisions for all drugs and interventions within cancer care should be made through appropriate health technology appraisal processes.

Cancer Drugs Fund: “a difficult legacy”

“But the real change to help get these drugs into the market in the UK will not come from siloed funds, but rather from these drugs costing less in the first place.

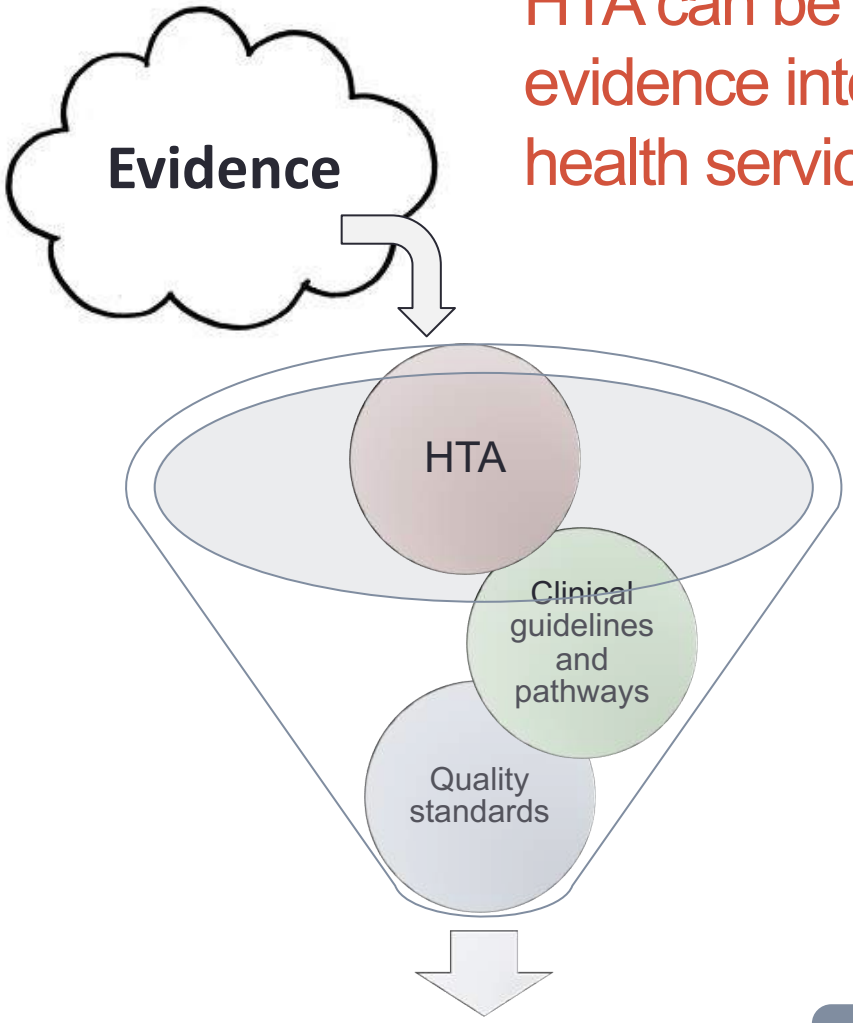
Both the government and pharma play on the fear surrounding cancer for their own ends, but pricing a cancer drug artificially high simply because it treats a feared disease does not seem fair to the NHS or, more pertinently, to patients”

PharmaFile, Feb 2014; http://www.pharmafile.com/news/182224/cancer-drugs-fund-assessing-difficult-legacy

LOOKING BEYOND THE ASSESSMENT OF INDIVIDUAL “TECHNOLOGIES” – WIDER HTA

Clinical guidelines and quality improvement

HTA can be part of a stepwise process getting evidence into policy and improving the quality of health services…



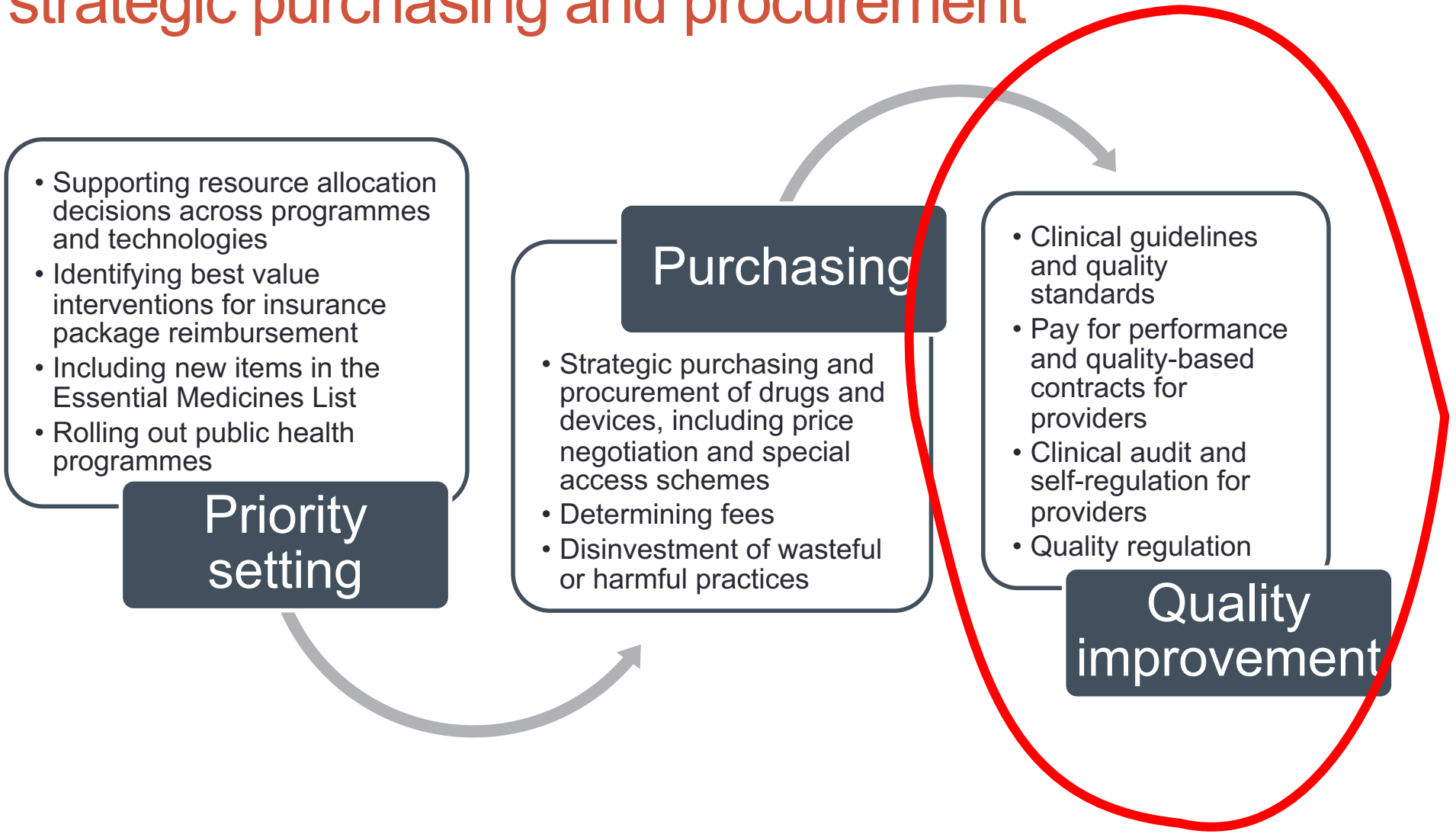
Health technology assessment (HTA) to compare clinical and cost-effectiveness of different interventions

Clinical guidelines (STGs) and pathways distilled from HTA and other evidence

Quality standards and indicators from evidence-based guidelines

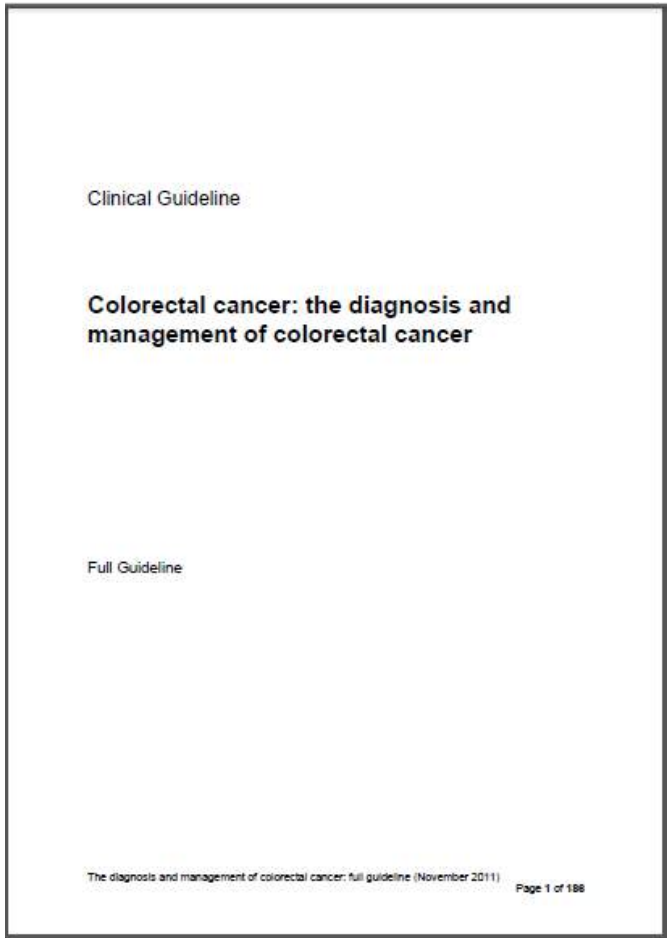
Health benefits plans (HBPs), pay-for-performance, other levers (regulation, accreditation, education…)

…informing both quality improvement and strategic purchasing and procurement



Clinical guidelines - what are they?

- Broad guidance covering all or specific aspects of the management of a particular condition (the pathway) [development time for guidelines is usually between 12 and 27 months (from the start of scoping to publication)]
- Incorporates technology appraisals, interventional procedures and other related NICE guidance where appropriate
- Recommendations advisory only (but can be used to develop *quality standards* to assess clinical practice and inform payment)



Clinical Guideline

Colorectal cancer: the diagnosis and management of colorectal cancer

Full Guideline

From evidence to setting standards and improving quality

Example: Quality standard for diabetes prevention developed from NICE guideline

Clinical and/or cost-effectiveness evidence (NICE PH38, 2017)

Lifestyle-change programmes are cost-effective for all people at high risk of diabetes, particularly for people with higher HbA1c or fasting plasma glucose levels

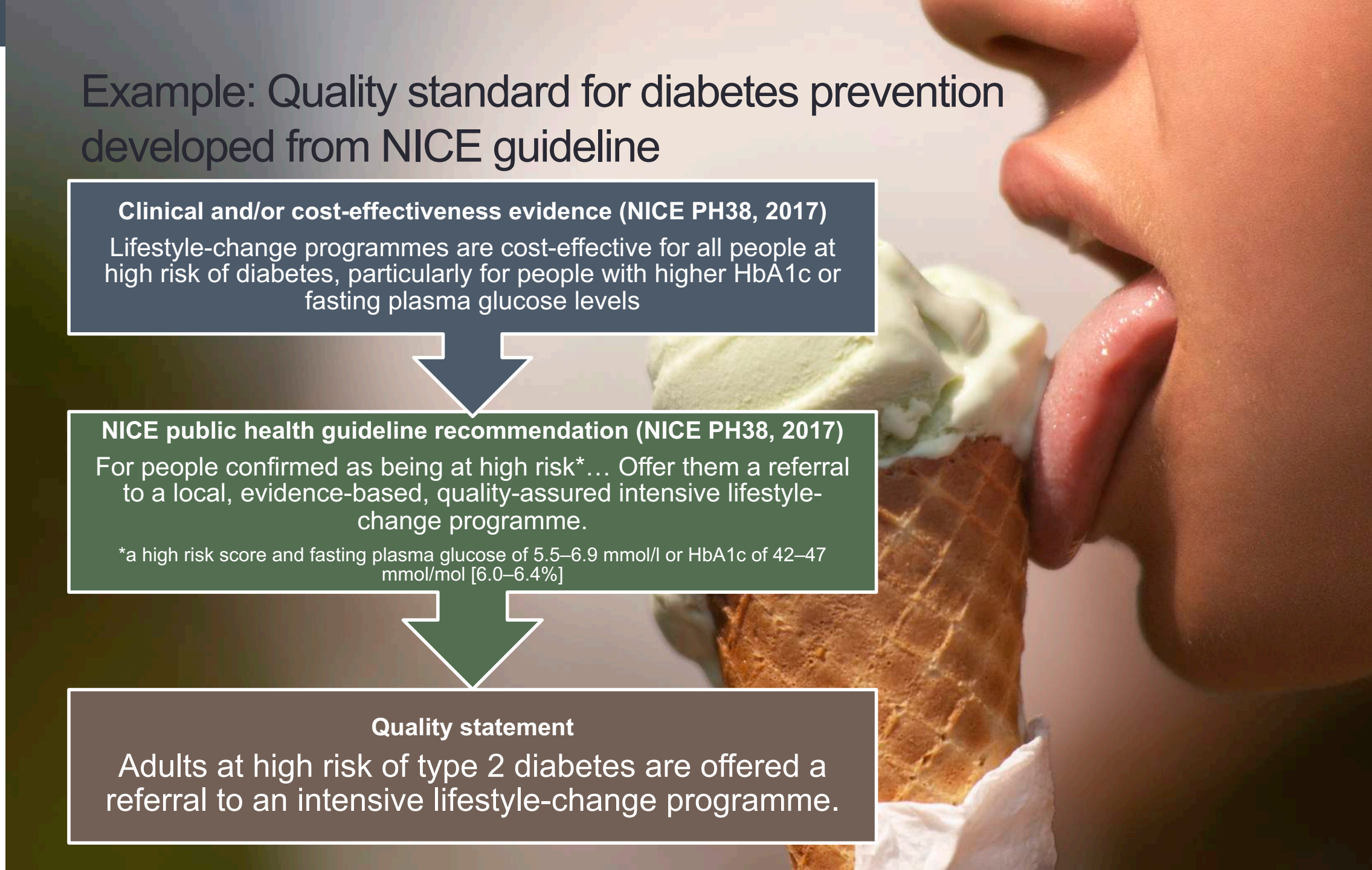
NICE public health guideline recommendation (NICE PH38, 2017)

For people confirmed as being at high risk*… Offer them a referral to a local, evidence-based, quality-assured intensive lifestyle-change programme.

*a high risk score and fasting plasma glucose of 5.5–6.9 mmol/l or HbA1c of 42–47 mmol/mol [6.0–6.4%]

Quality statement

Adults at high risk of type 2 diabetes are offered a referral to an intensive lifestyle-change programme.



Quality measure: Process

What amount of "quality care" is being provided?

Quality measure
Proportion of adults at high risk of type 2 diabetes who are offered a referral to an intensive lifestyle-change programme

=

$$\frac{\text{No. of adults at high risk of type 2 diabetes who are offered a referral to an intensive lifestyle-change programme } \textit{(numerator)}}{\text{All adults at high risk of type 2 diabetes } \textit{(denominator)}}$$

Implementation

Health outcomes
Weight loss of participants in lifestyle-change programmes
Incidence of type 2 diabetes

Case study: importance of clear institutional roles – indicator development in the UK

Role	*Description*
Priority setter	**Establish priority** areas for indicator development, based on epidemiological and healthcare use data.
Evidence generator	**Synthesise evidence** on best clinical practice, used to develop indicators.
Standard setter	Determine the best wording and **content** for each indicator, and what the **targets for success** should be.
Information collector	Collect and analyse data on healthcare use and performance against indicators.
Health service employer	'Owners' (adopters) of indicators in **everyday use**; responsible for ensuring reporting against indicators, and data quality.

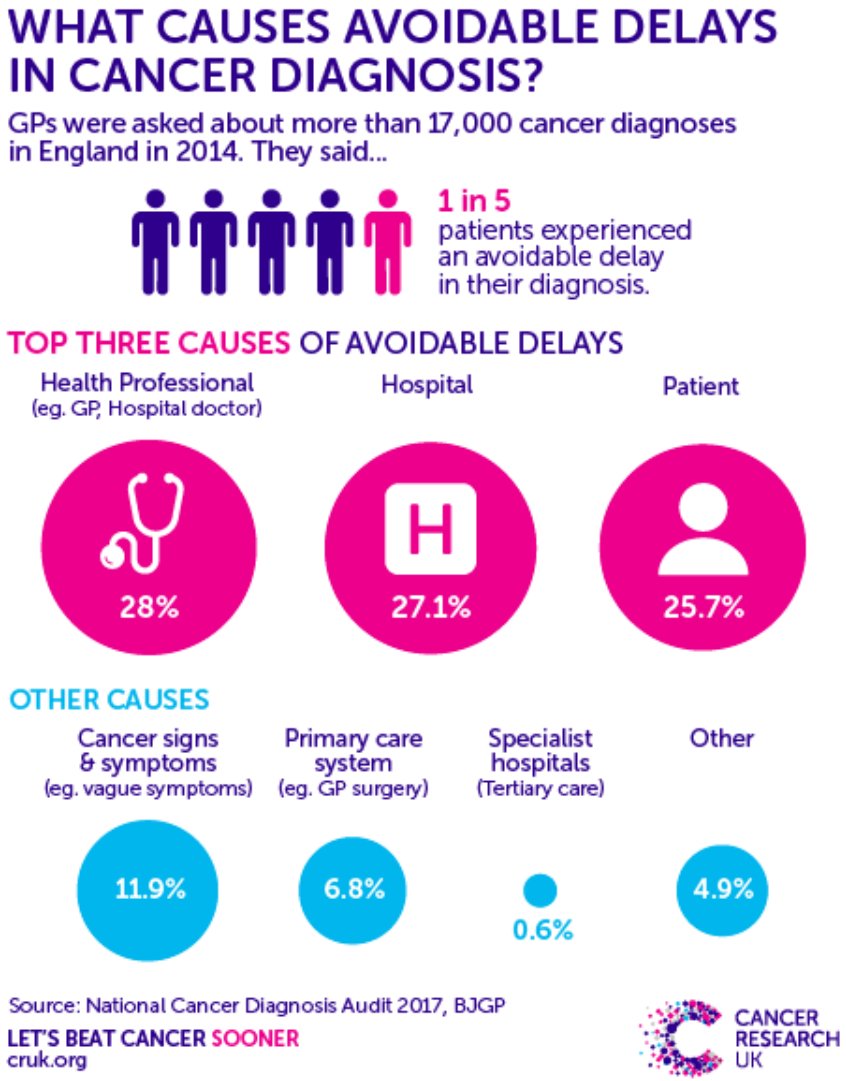
Case study: importance of clear institutional roles – indicator development in the UK

Role	*Description*	
Priority setter	**Establish priority** areas for indicator development, based on epidemiological and healthcare use data.	***NHS England***; *Devolved administrations; Public Health England*
Evidence generator	**Synthesise evidence** on best clinical practice, used to develop indicators.	***NICE***
Standard setter	Determine the best wording and **content** for each indicator, and what the **targets for success** should be.	***NICE*** *(incl. collaborating centres)*
Information collector	Collect and analyse data on healthcare use and performance against indicators.	***NHS Digital***
Health service employer	'Owners' (adopters) of indicators in **everyday use**; responsible for ensuring reporting against indicators, and data quality.	***NHS England*** *(incl. NHS Employers)*

Audit and benchmarking

- Monitoring and Evaluation can help provide evidence on the value of HTA and its impact on decision-making, and importantly inform learning and future quality improvement.
- At the most simple level, monitoring seeks to address the following questions:
  - Do we observe changes in health technology utilisation after the decision?
  - Which patients receive it? And in what settings?
- Surveys and clinical audits can be used to investigate the impact of an HTA driven decision at the level of provision of care, but those require specific data collection
- In the UK, the professional associations fund and conduct national audit programmes, independently from the government, which healthcare providers and clinicians voluntarily participate in
  - Often include questions and key performance indicators drawn from NICE guidelines and quality standards

National Cancer Diagnosis Audit (2017)

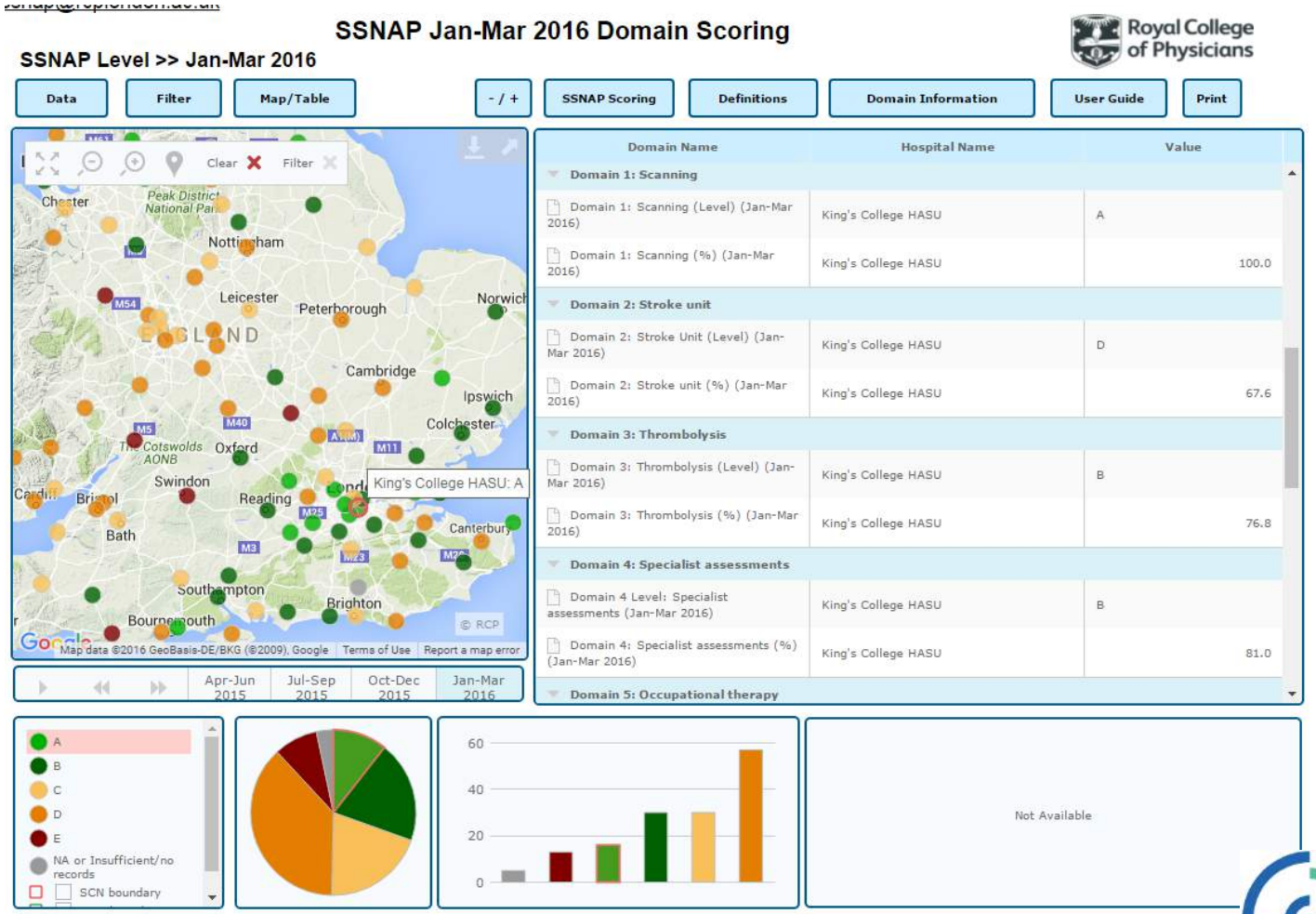


"The findings highlight examples of good practice, identify areas for quality improvement with the aim to help health professionals to diagnose cancer earlier.

The data also provide a baseline for future audit of the impact of 2015 NICE guidance on management and referral of suspected cancer."

CRUK (2017)
http://www.cancerresearchuk.org/health-professional/diagnosis/national-cancer-diagnosis-audit

Auditing and benchmarking of providers against national standards for stroke care



Source: Sentinal Stroke National Audit Programme (SSNAP)

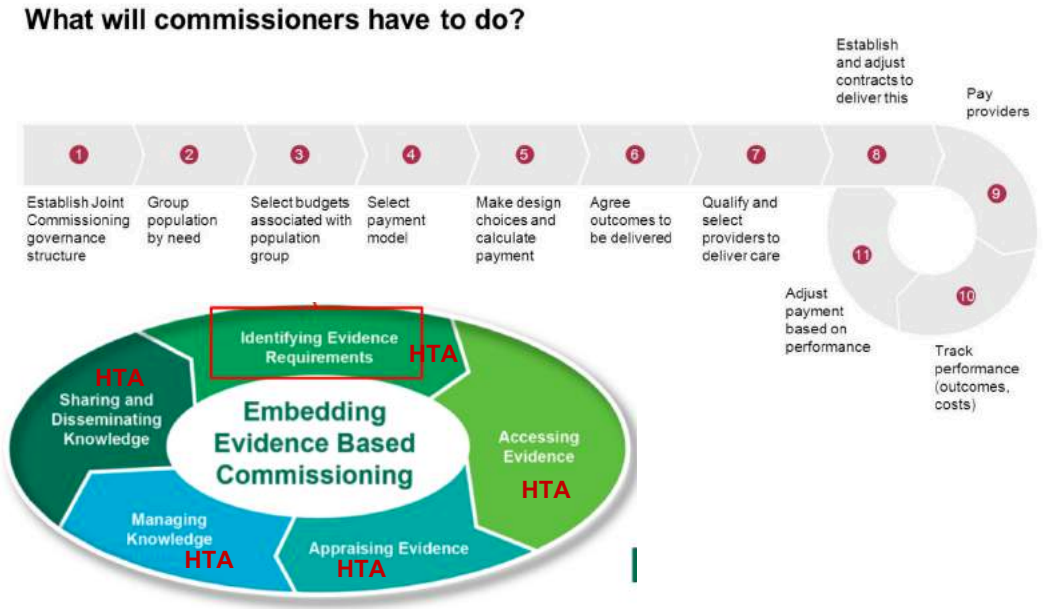
Last but not least…Strategic Purchasing:

Using HTA Determining what to buy, from whom, how (and for how much):

- Identify comparative value of alternatives and determine a “value-based price”
- Design outcome/quality-based indicators and performance manage

The strategic purchasing cycle

Courtesy of The NHS Information Centre for health and social care. Full diagram available at: www.ic.nhs.uk/commissioning



THOUGHTS ABOUT THE JAPANESE SYSTEM

UK-Japan comparison…

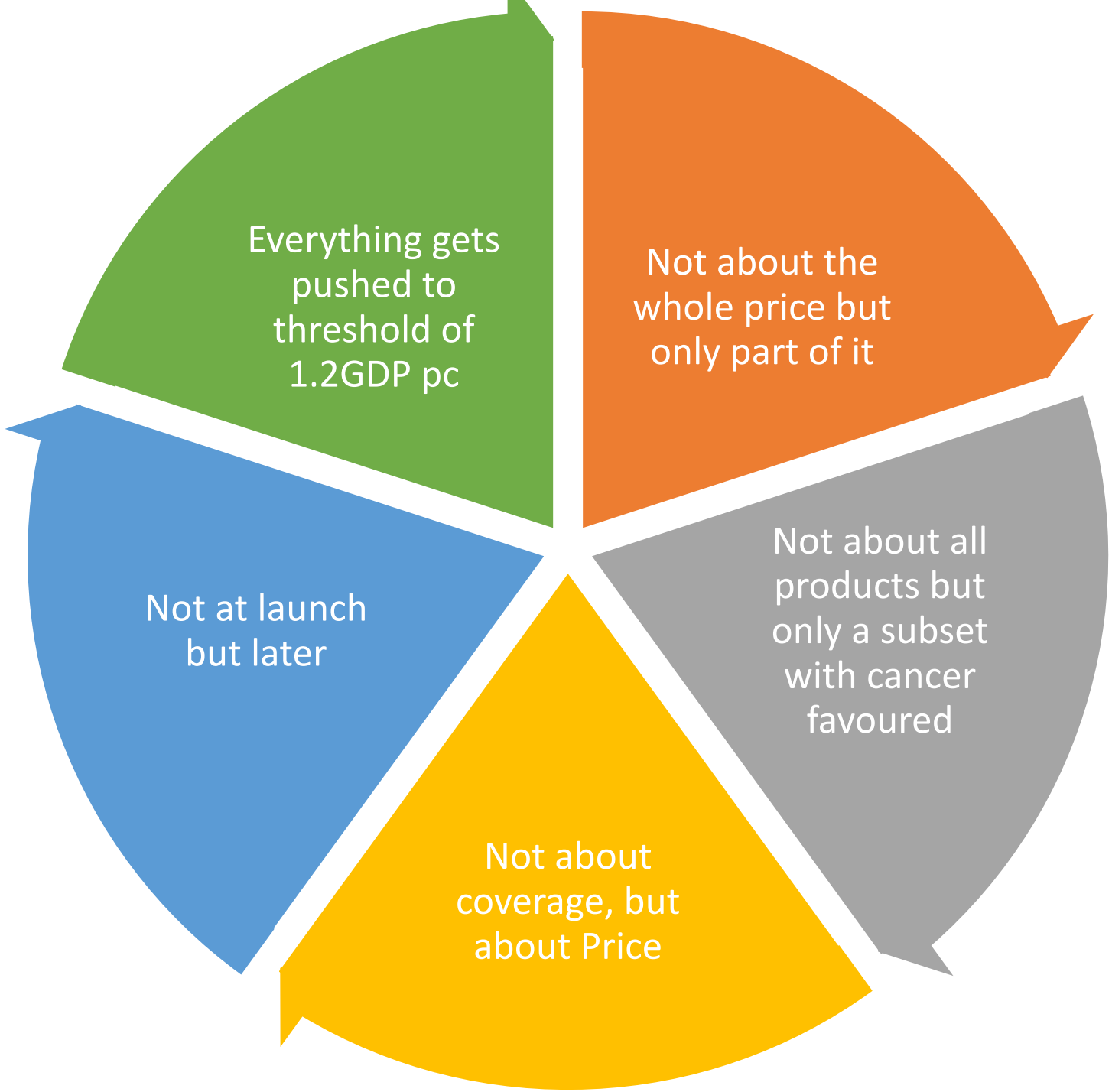
UK and Japanese systems: difference and similarities (i)

System Characteristic	Japan	UK
Drug price control mechanism	Sophisticated and complex regulation and market based controls; stable over time • **HTA** plays a role in price adjustment on only part of the product price • Complex formula for defining price for on-patent products via similar efficacy comparison and cost calculation	Complex combination of market competition and controlled margins for generics; NICE and PPRS for branded. • NICE/HTA has played an increasing role on pricing using **HTA** (2007-2017); • Now NHS England/payer gaining more power using **budget impact** criteria (2018-present)
Purpose of using HTA	To adjust a proportion of price premium; complements current pricing rules	• To manage ”listing”; NICE recommendation encourages (but no longer guarantees) NHS coverage • Only indirect link to pricing
Indication pricing?	Weighted mean of ICERs during pilot; now revised to weighted mean of price adjustment	No; NICE looks at one price put forward by manufacturer; flexible pricing means this can change but hardly ever used; system can be gamed and launch sequence matters

UK and Japanese systems: difference and similarities (ii)

System Characteristic	Japan	UK
HTA timing	Post launch and after companies enter system with a given price	Prelaunch; starts before marketing authorization and runs alongside regulatory approval process
Target products	• Only high budget impact products • Not for paediatric products or orphan products	• Increasingly universal; all new indications • Includes orphan but with special rules/threshold
Special considerations	Cancer, rare diseases, paediatrics	End of life rule and Cancer Drugs Fund favour cancer though this is reversed Higher threshold for rare drugs
Threshold	JPY 5m (1.2xGDP pc) *but*… Threshold used as a cut off to determine whether HTA will be used to adjust fraction of price If price after adjustment is less than threshold then it is adjusted upwards…	£20-30k (0.65-1xGDP pc) but higher for certain diseases

Some possible problems…



Suggestions…

Consider HTA at launch

- Align with regulatory approval process, not with reimbursement process
- If already on market, hard to revise price
- Shorten process; consider most multinationals have health econ models ready to adapt to Japanese setting!

Use HTA as a carrot and a stick

- Reconsider price raising measures as they may inflate budget
- Apply threshold to whole price, not a fraction
- Great to reward transparency (disclosure rate) but ultimately impact on health is what matters

Consider a threshold linked to budgetary constraint

- Particularly important if prices are raised to meet this threshold
- Carry out BIA and monitor trends in expenditure

Beware of inefficient comparators

- As HTA rule applied to fraction of price, pricing based on similar products which may however be not cost effective, can set negative precedent (eg hep C drugs)

Evaluate impact on spend and readjust process

To support decision making…

- Need to look at the entire body of the 'best available' evidence
- Evidence is never complete
- Judgement is unavoidable
- Uncertainty matters – and it should be fully explored
- ….and always make important information part of ***routine*** data collection…


ありがとう！