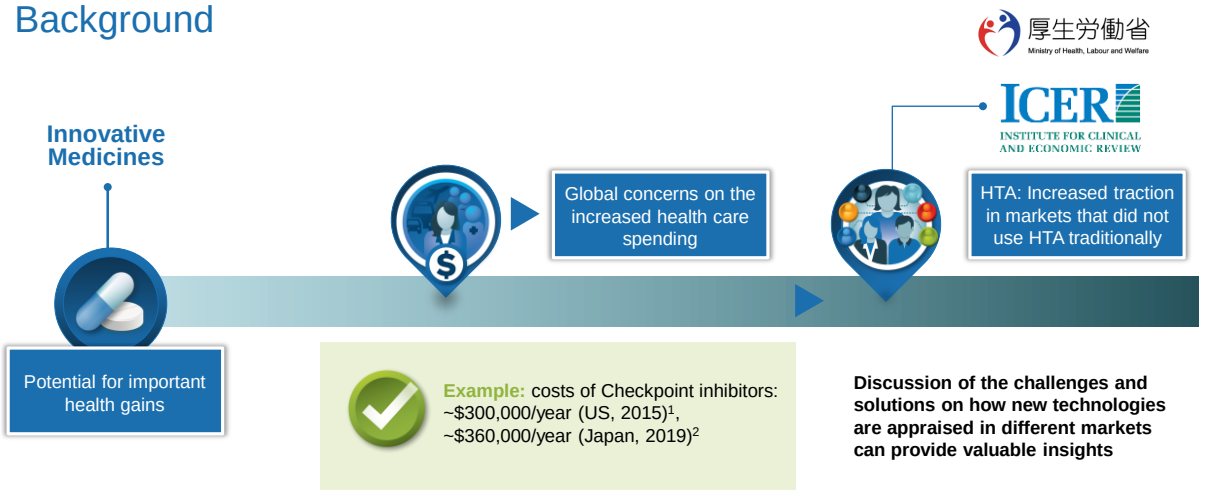
Do Innovative Technologies Require Innovative Appraisal Techniques?

Case Studies From Recent HTAs in the UK, US, and Japan

4 November 2019

Background



Example: costs of Checkpoint inhibitors: ~$300,000/year (US, 2015)[1], ~$360,000/year (Japan, 2019)[2]

Discussion of the challenges and solutions on how new technologies are appraised in different markets can provide valuable insights

HTA = health technology assessment; US = United States.

1. Andrews, 2015.
2. Cancer Treatment Cost.com, 2019.

Workshop Agenda

Overview

Relationship between HTAs and pricing and market access in the UK, Japan, and US

Case Studies

Innovative appraisal techniques

Applications

UK = United Kingdom.

Speakers

Ataru Igarashi, PhD

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Director of NICE Scientific Advice

National Institute for Clinical Excellence

London, UK

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RTI Health Solutions

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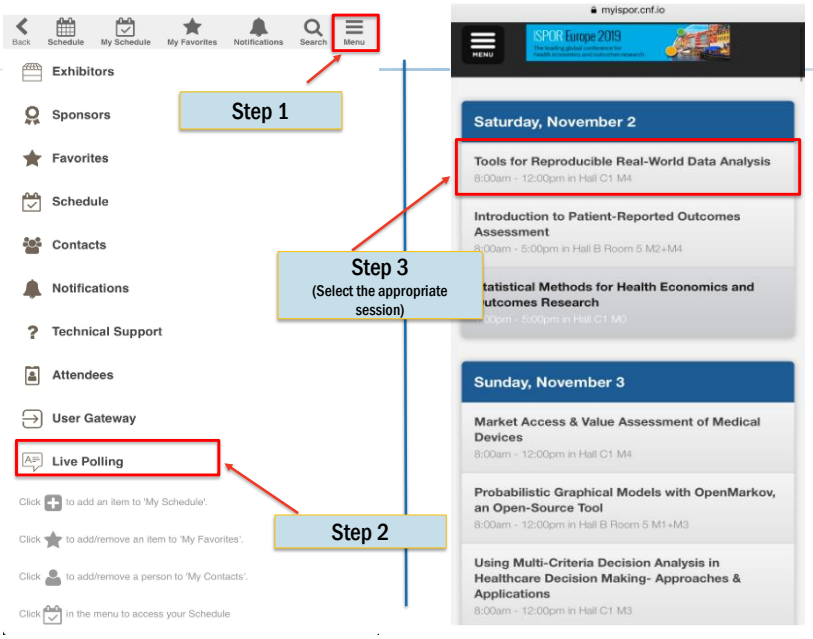
NICE = National Institute for Health and Care Excellence; USA = United States of America.

How To Participate in Polling Using the ISPOR Mobile App



ISPOR = International Society for Pharmacoeconomics and Outcomes Research.

Get to Know Participants!

Which research section are you representing?

- ☐ 1) Academia
- ☐ 2) HTA/regulatory bodies
- ☐ 3) Industry
- ☐ 4) Consultancy
- ☐ 5) Others

Which geographic area does your organization belong to?

- ☐ 1) Europe
- ☐ 2) Canada
- ☐ 3) USA
- ☐ 4) Latin America
- ☐ 5) Asia
- ☐ 6) Australasia
- ☐ 7) Others

In your opinion, which aspect of HTA innovation is most important?

- ☐ 1) Fair ways to link reimbursement to the product's value
- ☐ 2) Results that stimulate innovation
- ☐ 3) Results that ensure patients' access to treatment
- ☐ 4) Others

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Poll: Which research section are you representing?

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Poll: Which geographic area does your organization belong to?

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Poll: In your opinion, which aspect of HTA innovation is most important?

RTI (h)(s) Health Solutions

4 November 2019

United Kingdom Overview

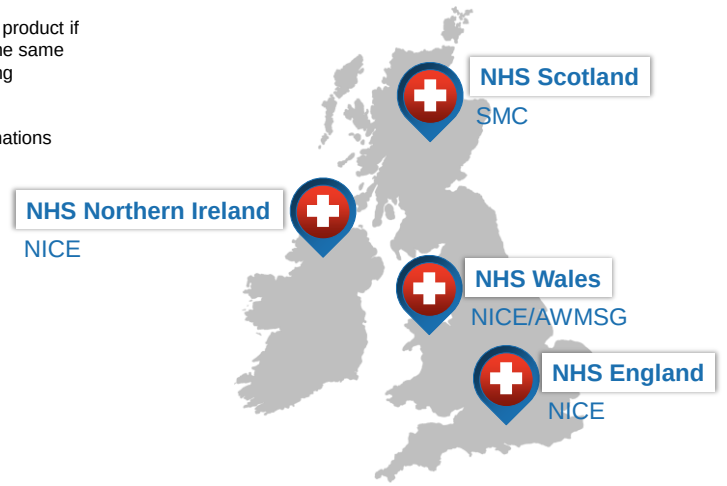
Isobel Pearson, DPhil; Director, Health Economics, HTA; RTI Health Solutions; Manchester, UK

The power of knowledge. The value of understanding.

UK HTA Authorities

RTI (h)(s) Health Solutions

- AWMSG will not normally consider appraising a product if NICE intends to publish their final appraisal of the same product within 12 months of the date of marketing authorisation
- Northern Ireland adapts as appropriate determinations made by NICE



AWMSG = All Wales Medicines Strategy Group; NHS = National Health Service; SMC = Scottish Medicines Consortium.
Source: Adapted from Toumi, 2018; AWMSG, 2019a; O'Neill et al., 2012.

The power of knowledge. The value of understanding.

NICE Technology Appraisal Processes

- NICE uses 3 different methodologies to assess health technologies

HTA Methodology	Assessed Technologies	Approximate Timeline
Single technology appraisal	Single technology for a single indication	41-50 weeks
Multiple technology appraisal	Several technologies used for one condition or a single technology for multiple indications	47-60 weeks
Fast-track appraisal	Single technology for a single indication for technologies that offer exceptional value for money	32 weeks

Note: In addition, there is a highly specialised technology process that assesses select ultra-orphan products.
Sources: NICE, 2019a; Stevenson et al., 2018.

Cancer Drugs Fund in England

HTA Methodology	Assessed Technologies	Approximate Timeline
All new cancer drugs, and significant new licensed indications for cancer drugs, are referred to NICE for appraisal	• Recommended for routine commissioning – 'yes' • Not recommended for routine commissioning – 'no' • Recommended for use within the Cancer Drugs Fund	• Draft guidance prior to a receiving its marketing authorisation • Final guidance within 90 days of marketing authorisation wherever possible

Sources: NHS England, 2016; NICE, 2019b.

Special Consideration for Rare Diseases

The NICE HST process considers only drugs for very rare conditions

NICE has introduced a budget-impact test for technologies appraised within both the Technology Appraisal and the HST programmes
If the budget impact exceeds £20 million, in any of the first 3 years, NHS England may engage in commercial discussions with the company

- The SMC PACE process allows patient groups and clinicians a stronger voice in decision making for products to treat both end-of-life and very rare conditions
- The SMC has also introduced a revised assessment process for ultra-orphan medicines
- The AWMSG has introduced an additional process to further assess the benefits of a rare disease medicine from the perspective of clinicians and patients through a CAPIG meeting

CAPIG = Clinician and Patient Involvement Group; HST = highly specialised technology; PACE = Patient and Clinician Engagement.
Sources: AWMSG, 2019b; NICE, 2017a; 2017b; 2019c; SMC, 2016; 2019.

Additional Information on UK HTA Processes and Drug Pricing

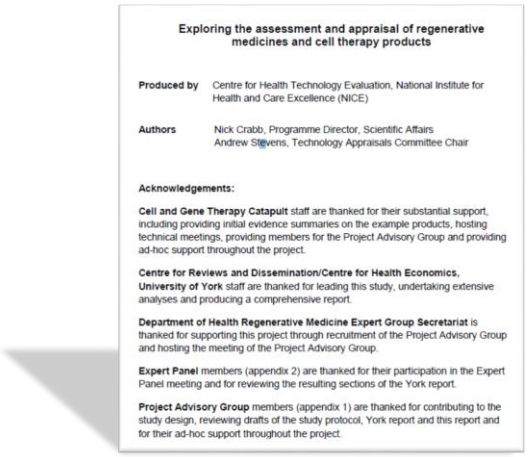
See the handout for additional information on NICE technology assessments, HSTs, the Cancer Drugs Fund, and pricing of branded and generic medicines

NICE National Institute for Health and Care Excellence

Case Studies From the UK

Jeanette Kusel
Director, NICE Scientific Advice

NICE Regenerative Medicines Study (2016)



Exploring the assessment and appraisal of regenerative medicines and cell therapy products

Produced by Centre for Health Technology Evaluation, National Institute for Health and Care Excellence (NICE)

Authors Nick Crabb, Programme Director, Scientific Affairs
Andrew Stevens, Technology Appraisals Committee Chair

Acknowledgements:

Cell and Gene Therapy Catapult staff are thanked for their substantial support, including providing initial evidence summaries on the example products, hosting technical meetings, providing members for the Project Advisory Group and providing ad-hoc support throughout the project.

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Department of Health Regenerative Medicine Expert Group Secretariat is thanked for supporting this project through recruitment of the Project Advisory Group and hosting the meeting of the Project Advisory Group.

Expert Panel members (appendix 2) are thanked for their participation in the Expert Panel meeting and for reviewing the resulting sections of the York report.

Project Advisory Group members (appendix 1) are thanked for contributing to the study design, reviewing drafts of the study protocol, York report and this report and for their ad-hoc support throughout the project.

- Prompted by a recommendation from the Department of Health Regenerative Medicine Expert Group
- Included a broad exploration of the applicability of NICE technology appraisal methods to regenerative medicines
- Hypothetical example product based on early clinical data for related real products, supplemented with hypothetical evidence

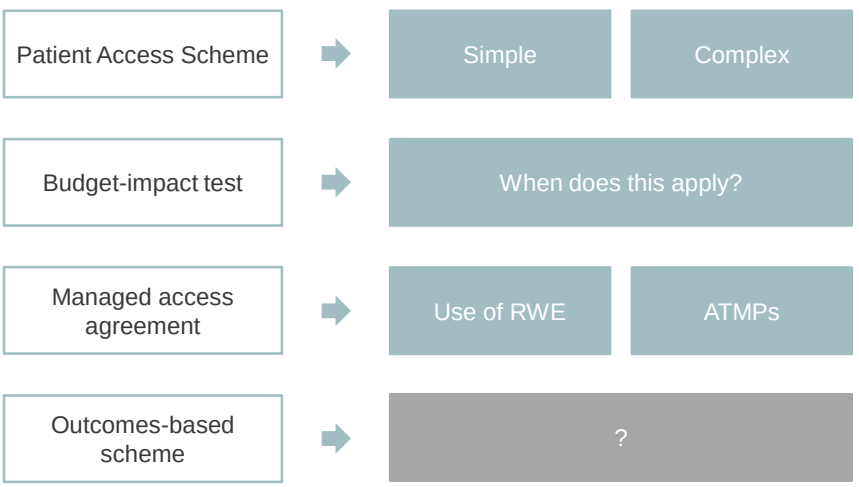
NICE Source: NICE, 2016.

NICE Regenerative Medicines Study (2016)

- ✓ NICE appraisal methods and decision frameworks applicable
- ✓ Key to quantify and present clinical outcome and decision uncertainty

NICE

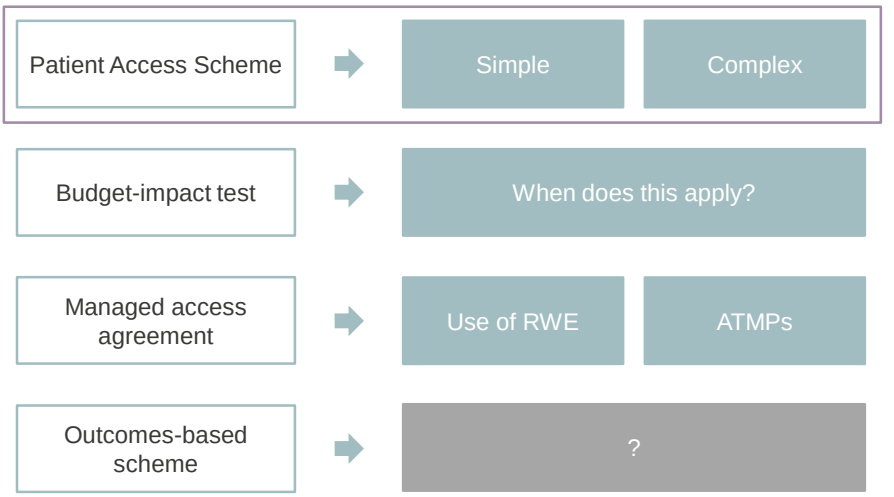
Innovative Pricing Methods in England



NICE

ATMP = advanced therapy medicinal product; RWE = real-world evidence.

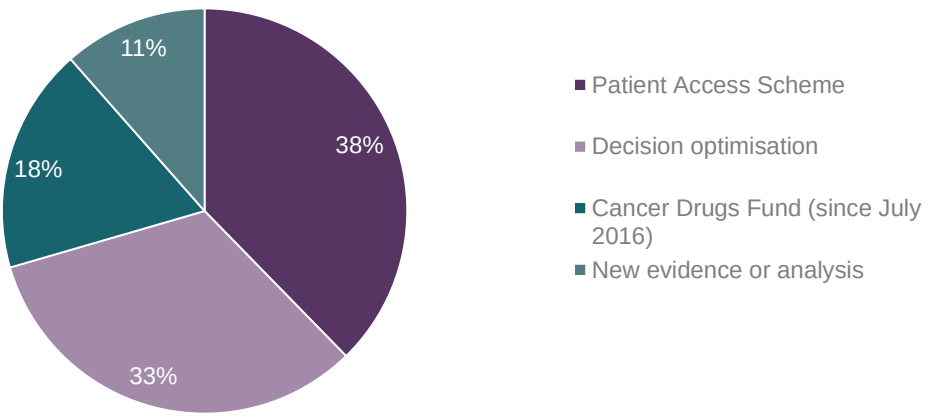
Innovative Pricing Methods in England



NICE

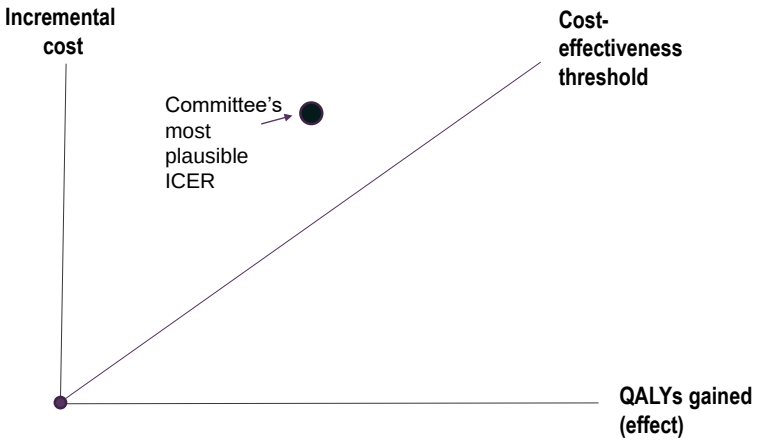
Patient Access Schemes

Primary reason for reversal of a negative preliminary decision from NICE



NICE Source: Walton et al., 2019.

Patient Access schemes: Simple Discount



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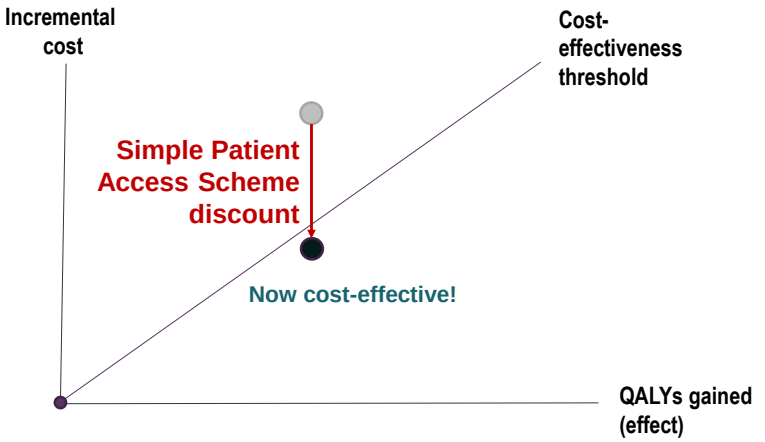
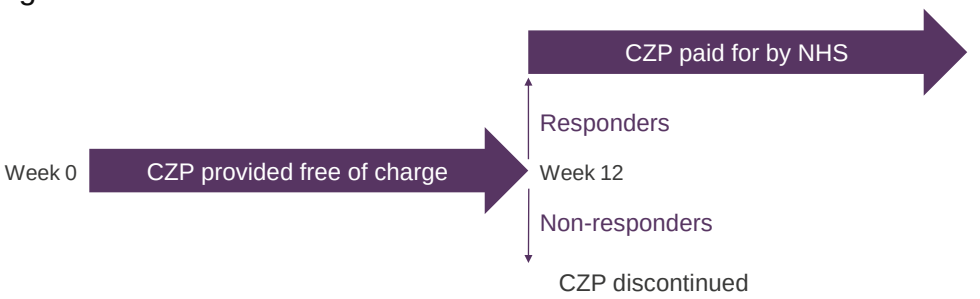
Patient Access Schemes: Simple Discount



NICE

Complex PAS: Certolizumab Pegol in Rheumatoid Arthritis

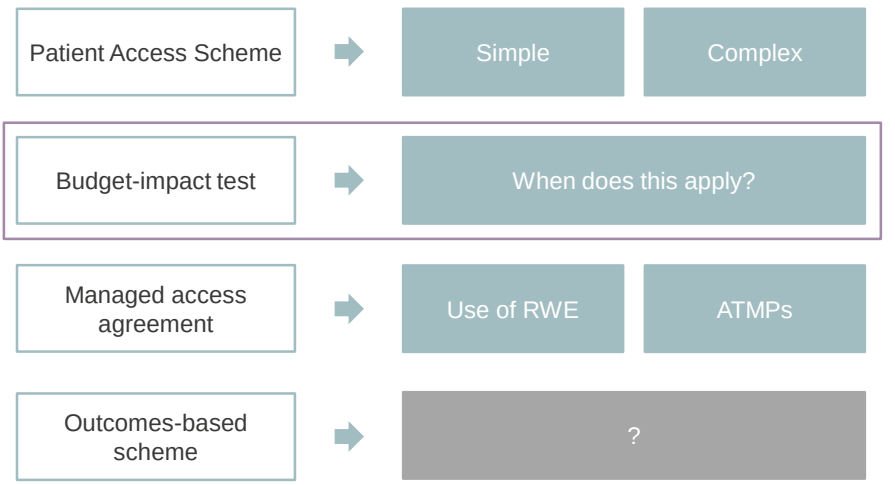
Free-stock scheme: the first 12 weeks of therapy are provided free of charge



NICE

CZP = certolizumab pegol; PAS = Patient Access Scheme.

Innovative Pricing Methods in England



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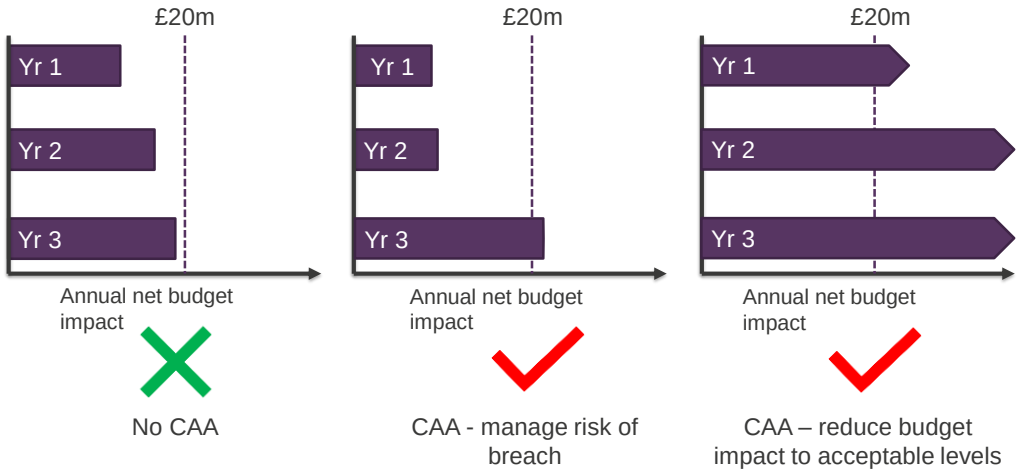
Budget-Impact Test



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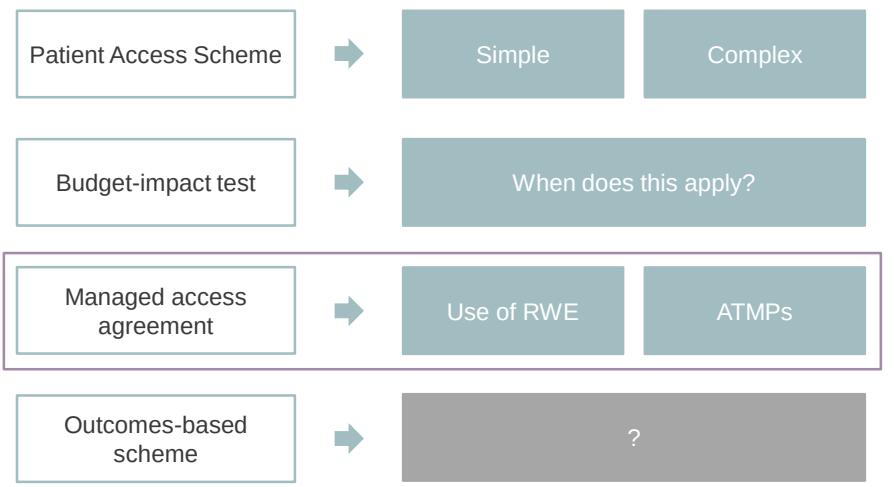
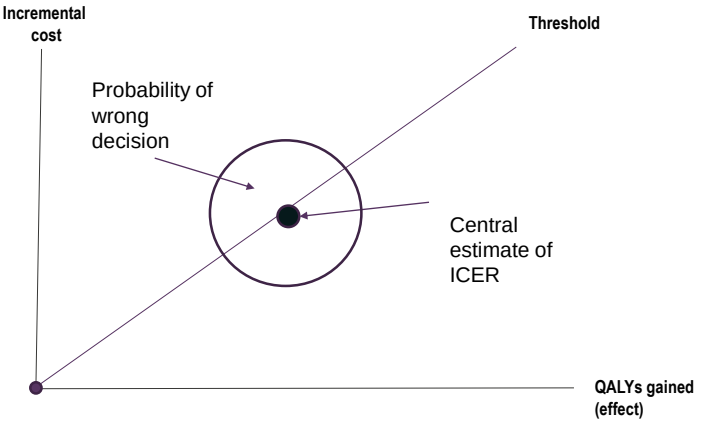
CAA = commercial access arrangement; yr = year.

Innovative Pricing Methods in England



NICE

Managed Access: Illustrative Example



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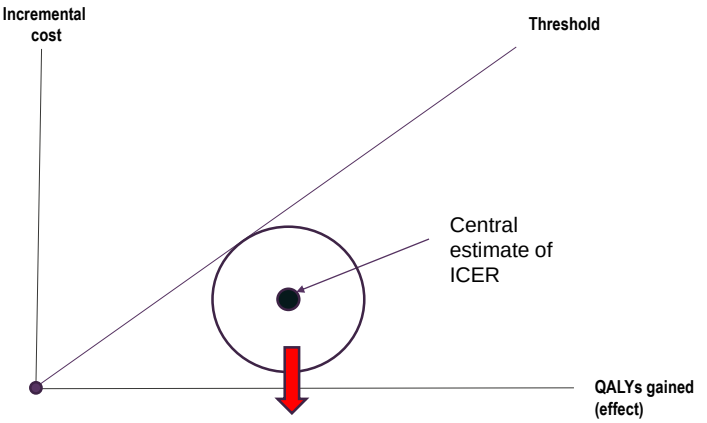
Managed Access: Illustrative Example



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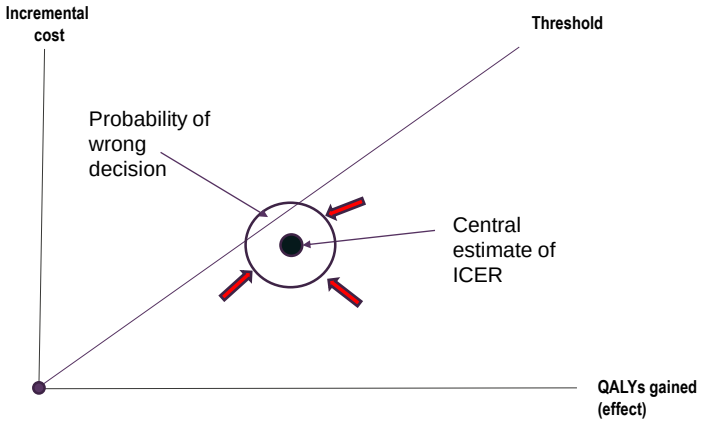
Managed Access: Illustrative Example



NICE

Managed Access Case Study: Brentuximab Vedotin

Technology Appraisal 446
2016
Brentuximab vedotin for treating CD30-positive Hodgkin lymphoma

Issues in original appraisal: uncertainty over whether the drug would improve rates of stem-cell therapy

RWE data collection within CDF

Technology Appraisal 524
2018
Brentuximab vedotin for treating CD30-positive Hodgkin lymphoma

Data collected in CDF on rates of stem-cell transplant after the drug showed improvements compared to chemotherapy

ICER < £20k

NICE

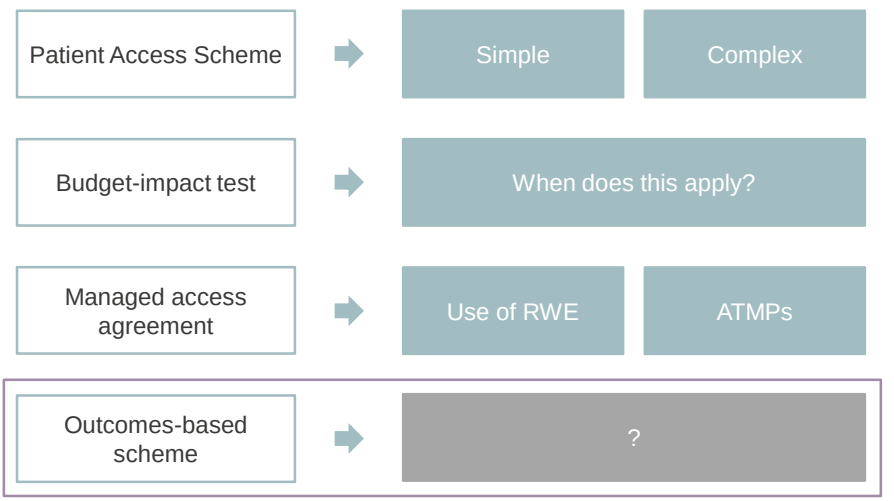
CAR-T NICE Appraisal Outcomes

Axicabtagene ciloleucel (Yescarta; Kite-Gilead) for treating diffuse large B-cell lymphoma and primary mediastinal B-cell lymphoma after 2 or more systemic therapies	• TA559: recommended for use within the CDF
Tisagenlecleucel- T (Kymriah; Novartis) for treating relapsed or refractory diffuse large B-cell lymphoma after 2 or more systemic therapies	• TA567: recommended for use within the CDF
Tisagenlecleucel-T (Kymriah; Novartis) for treating relapsed or refractory B-cell acute lymphoblastic leukaemia in people aged 3-25 years	• TA554: recommended for use within the CDF

NICE

CAR-T = chimeric antigen receptor T; TA = technology appraisal.

Innovative Pricing Methods in England



NICE

Summary

- The NICE methods are suitable for evaluating the cost-effectiveness of innovative technologies
- As for all technologies, Patient Access Schemes might be needed to ensure that the technology is cost-effective at the appropriate ICER threshold
- Due to the large upfront costs associated with some ATMPs, separate commercial agreements may be needed with NHS England to ensure affordability for the UK health care system
- Due to the long-term uncertainty around the clinical benefits, managed access arrangements may be used—but are they sustainable?

Japan

Ataru Igarashi, PhD, Associate Professor of Health Economics and Outcomes Research, University of Tokyo, Japan

Overview of Japanese Healthcare system

- All people are covered by Public Health Insurance (NHI) System since 1961

Name	# of Insurers	# of Insured	characteristic	Co-payment
Employees' HI	1,400	65Mil.	Employees under 74y	30% for ordinal 20% for 70-74y
National HI	1,900 (each city/town)	38Mil.	Others under 74y	
Mutual aid association	90	9Mil.	Civil servants under 74y	
HI for Aged population	47 (each pref.)	15Mil.	All persons >=75y	10%

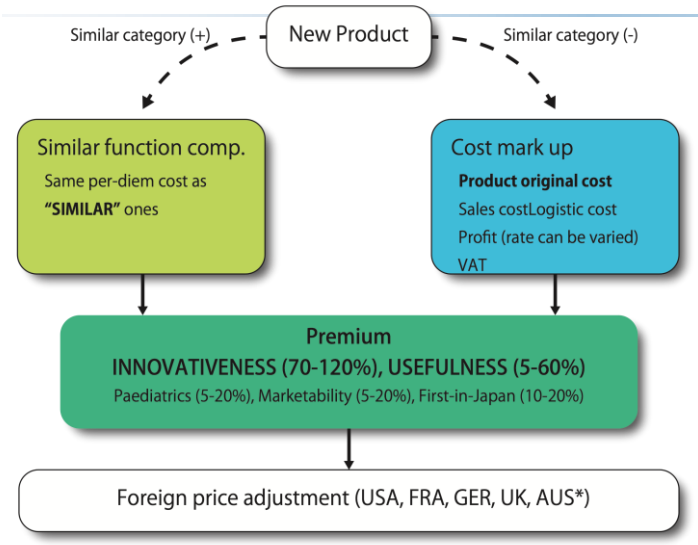
Basic package are almost the same throughout every insurers

Japanese UHC system (from 1961)

Funding Source	Taxation, Insurance premium, mixed
Service to be covered	All medical care activities (Drugs), Positive list, Negative list
Patient co-pay	Co-payment system (Fixed amount/Fixed proportion), Entirely free,
Special co-pay reduction for vulnerable	Co-pay reduction for elderly, infants, poverty, and/or those who suffered from severe illness
Payment system	Fee-for-service system, Prospective Payment System (fixed fee for 1 day/fixed fee for 1 hospitalization)

Almost all (99%) drugs are covered with UHC in Japan

Pricing System for New Drug



NEW drugs will be covered by NHI system with fixed price within 60-90 days after NDA

Very FEW products get INNOVATIVENESS premium (60-120%), SOME products get USEFULLNESS premium (5-40%)

Price changing system for existing drugs

- Two systems are available

Name	Timing	Eligible	Purpose/Effect
Revision	Biannual (annual)	ALL	To minimize margins between wholesale/reimbursement price (generally <10%)
Re-calculation	Biannual (4 times/y)	few	Applied in case sales amounts of particular products are exploded (up to 50%)

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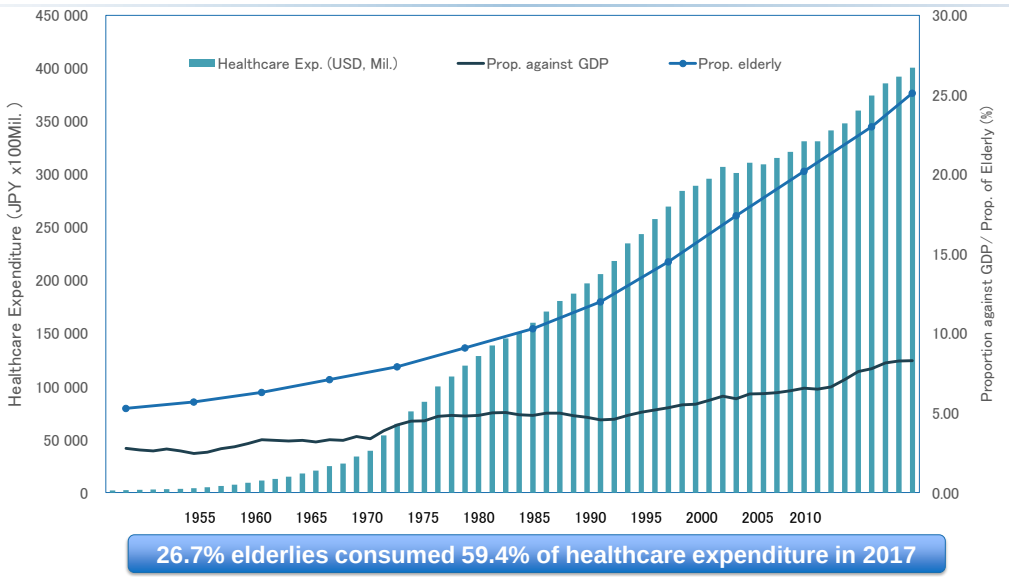
Options for price recalcuration system

- Price recalculation system for…

Name	Eligible drugs which…
Change of indication	Main indication was changed Similar drug is available for new main indication
Change of dosage	Dosage for main indication was changed (e.g. per diem dose x2 -> price cut for 50%)
Market expansion	Huge sales amount

Health Expenditure, prop.of GDP and Aging proportion in Japan (1954-2014)



26.7% elderlies consumed 59.4% of healthcare expenditure in 2017

Three KUROFUNEs into Japanese market

drugs for	Costs per month	Impacts for healthcare budget (1Y)
Hep. C	**JPY1.0Mil – 1.6Mil. (Duration: 3month)**	**300-400Bil. (Maximum)**
Cancer (PD-L1)	**JPY2.6Mil (Duration: Unknown)**	**3Bil (Melanoma) 100-1,000Bil.??(Lung Cancer)**
Hyper-lipidemia (PCSK-9)	**JPY40,000 (Duration: Unknown)**	**50Bil. (3% of patients with hyper lipidemia)**

Physicians and General public, as well as insurers, claimed that SOME system to check the eligibility of UHC should be implemented

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Source: MHLW 2016

Why medical care expenditure kept increasing? (2003-2015, %)

	04	05	06	07	08	09	10	11	12	13	14	15
Fee revise	-1.0		-3.2		-0.8		0.2		0.0		-1.2	
Pop.	0.1	0.1	0.0	0.0	-0.1	-0.1	0.0	-0.2	-0.2	-0.2	-0.2	-0.1
Aging	1.5	1.8	1.3	1.5	1.3	1.4	1.6	1.2	1.4	1.3	1.2	1.2
Tax											1.4	
Others	1.2	1.3	1.8*	1.5	1.5*	2.2	2.1	2.1	0.4	1.1	0.6*	2.7
Tot.	1.8	3.2	0.0	3.0	2.0	3.4	3.9	3.1	1.6	2.2	1.8	3.8

*Co-payment rate was changed for certain people

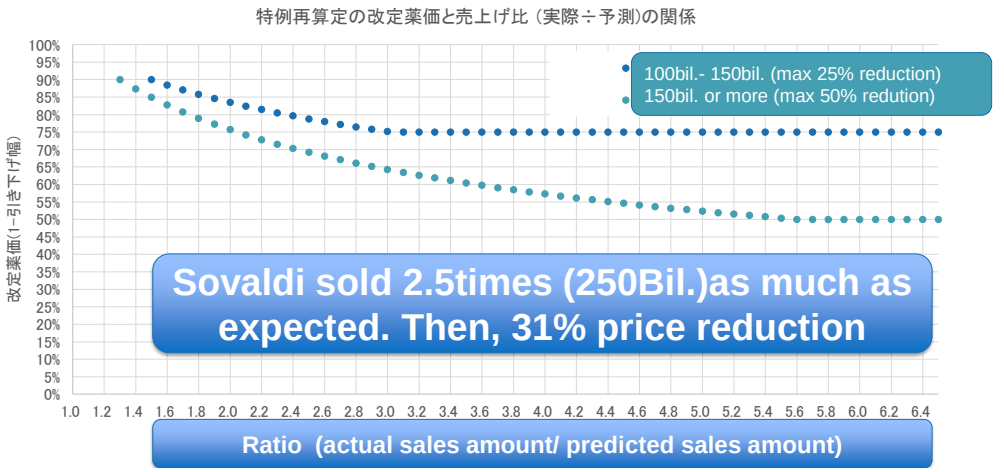
Aging is not the ONLY reason for budget explosion

What's happened for High-cost medication?

Price recalculation (not revision) system for market expansion		
name	**condition**	**percentage**
Ordinal rule	**based on sales amount**	**up to -25% (markup) up to -15% (similar)**
Special rule established after introduction of Sovaldi/Harvoni		
Special rule (from 2016)	**only sales amount**	**up to -25% (100-150bil.) up to -50% (150bil.-)**

Framework for "Special price recalculation"

- **Special rule was set up (only for them?)**



Targeted product for special price reduction (from 2016)

Name	Amount	Previous price	Revised Price
Sovaldi (Hep. C)	Sold >1.5Bil.	JPY62,000	JPY42,000
Harvoni (Hep. C)	Sold >1.5Bil.	JPY80,000	JPY55,000
Opdivo (Cancer)	Sold 100-150Bil.	JPY730,000	JPY365,000
Avastin (Cancer)	Sold 100-150Bil.	JPY180,000	JPY160,000
Pravix (Cardio)	Sold 100-150Bil.	JPY280	JPY200

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Characteristics of JP-HTA
(Pilot: 2016.4 - 2019.3 Entire: 2019-)

1	Eligible products are chosen from drugs ALREADY REIMBURSED
2	Results are used for PRICE REVISION, not for COVERAGE DECISION
3	HTA result will be applied only to PREMIUM portion
4	ICER values are compared with the threshold value to determine if it is cost-effective (UK NICE – like system)
5	Things other than Cost-Effecitiveness will be taken into account at the appraisal process (UK NICE – like system)
6	Drugs with multiple indications are evaluated via weighted-mean of revised price for eligible subgroup

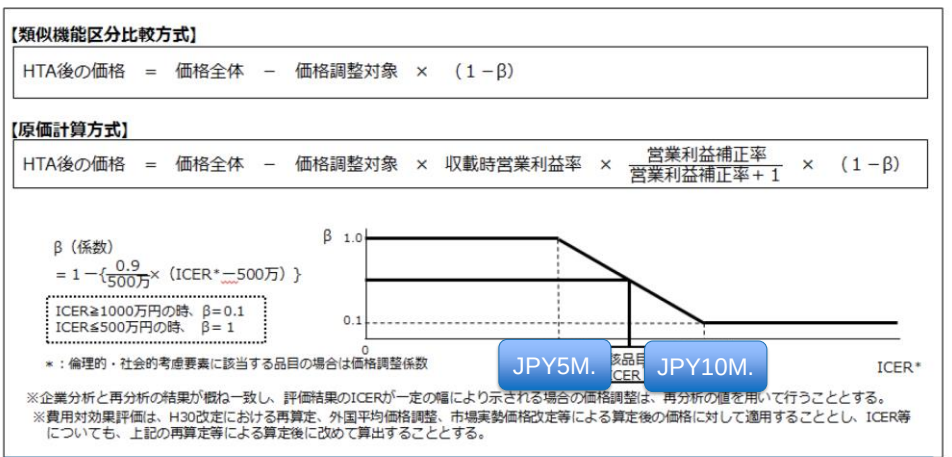
Why “step-by-step” implementation?

- To minimize the criticism before the opdivo-ERA

”Access limitation!!”	Oh, we would use HTA for price negotiation, not for coverage decision
”Access delay!”	Oh, we would use HTA for CURRENT treatments, not for NEW ones

Japan-specific way how to reflect results into price revision rate (provisional implementation, slope-like)

（図4）価格調整方法



The ICER value is directly reflected to the price revision rate

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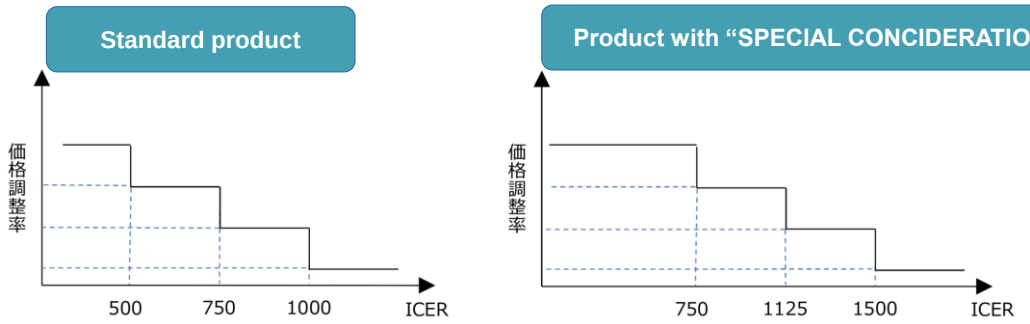
Japan-specific way how to reflect results into price revision rate (Entire implementation, step-like)



Examples should be categorized to “SPECIAL” at the appraisal process	
Anti cancer drug	Drugs for rare diseases
Drugs for pediatric diseases	

The ICER value is Still directly reflected to the price revision rate

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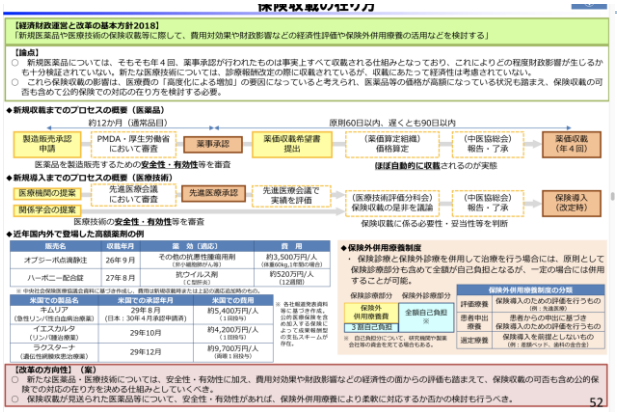
No additional factor needs to be considered in the appraisal process???

- Role of appraisal is very limited under current system

Viewpoint	Role	Importance
Practical	**Simply minimize price reduction rate**	**Less** important Drugs with poor ICER and extra priority of other factors **need not to** get higher price
Conceptual	To compensate the limitation of CEA/ICER	More important Other factors should be seriously considered, as no flexibility is allowed for CEA/ICER part

“Extra value” other than CEA/ICER is difficult to be incorporated to one-dimensional scale

What MOF and Payers are thinking about?



MOF seek to expand HTA to coverage decisioin

Payers' association claimed that mild medication should be kicked out from insurance coverage

CHRONOLOGY of the perception of NHI system

-2015	**PAX JAPANA (pre-opdivo era)**	ALL drug should be covered with same condition, as **Japan has UHC**
2015-19	**POST-opdivo era**	Some system should be implemented for products with huge budget impact, to maintain our system
2019-	**POST-Kymriah era**	Products which are "ATTRACTIVE" from financial perspective should be assessed Coverage range should? be limited???
2020-	**POST-Zolgensma, Aducanumab era**	???

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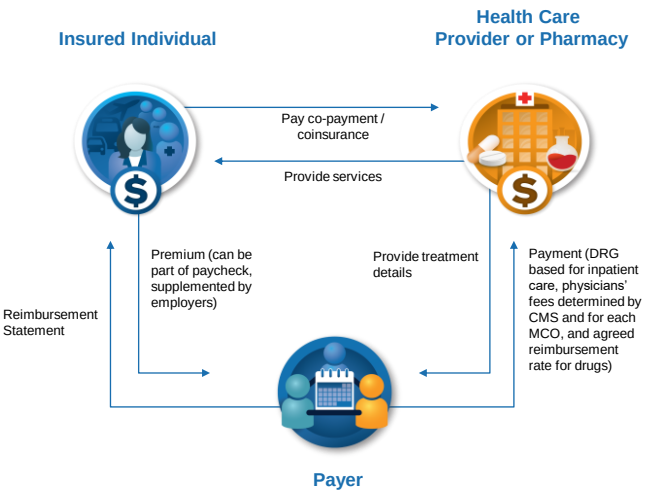
United States

Naoko Ronquest, PhD, Senior Director, US Health Economics, RTI Health Solutions, Research Triangle Park, North Carolina, United States

Health Insurance Reimbursement Process in the US[1]

- 91.2% of US population covered by health insurance (2017)[2]
  - Most insured individuals: private, commercial insurance plans (57% employment based, 10% other)
  - Approximately 40% of insured individuals: public plans (e.g., Medicare, Medicaid, CHIP, military health care)
- Premium, copay, and coinsurance rates: set by each plan
- Coverage and reimbursement rates for drugs and health care resources negotiated for each payer



CHIP = Children's Health Insurance Program; DRG = diagnosis-related group.

1. ISPOR, 2015.
2. Berchick et al., 2018.

Typical US Drug Price Setting Process and Role of HTA [1,2]

Manufacturer to set prices freely

- Payers (both CMS and private payers) do not regulate the price of a pharmaceutical product
- Payers set different reimbursement price/rates for each drug

New drug coverage and pricing decisions

- P&T committee in each private plan, pharmacy benefit manager, hospital, and public program reviews the evidence dossier and make decisions on formulary inclusion
- Some private payers and PBM conduct their own clinical and economic reviews (e.g., budget-impact model)
- Reimbursement varies across health plans
- Negotiated drug prices are proprietary information for each payer

No Federal HTA Requirement

- A survey of US payers in 2018:
  - ~30% of interviewees use reports from Institute for Clinical and Economic Review in their P&T reviews
  - ~40% reported they are likely to use the economic evaluation results to request rebates from manufacturers
  - ~80% reported they are likely to favour products found to be more cost-effective in their PA/Step edits requirements

CMS = Centers for Medicare and Medicaid Services; HTA = health technology assessment; P&T = pharmacy and therapeutic; PA = prior authorisation; PBM = pharmacy benefit management.

1. ISPOR, 2015.
2. White et al., 2018.

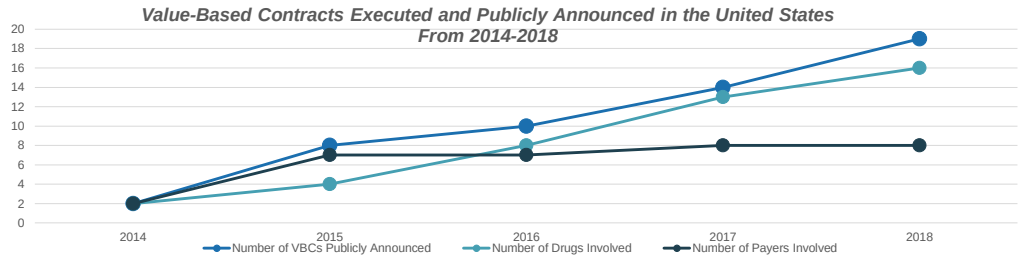
Organisations That Perform Public Health Technology Assessments

Organisations	Year Formed
Blue Cross Blue Shield Technology Evaluation Center	1985
Agency for Healthcare Research and Quality	1984
Evaluation of Genomic Applications in Practice and Prevention	2004
Institute for Clinical and Economic Review	2006

- In a 2009 survey of 11 payers,[1] none of the 11 payers reported they would use outcomes of cost-effectiveness assessments for their formulary decision making
- In another study in 2018, 40% of 22 payers who were interviewed reported they would use economic evaluation results from the Institute for Clinical and Economic Review to request rebates from manufacturers[2]

1. Trosman et al., 2011.
2. White et al., 2018.

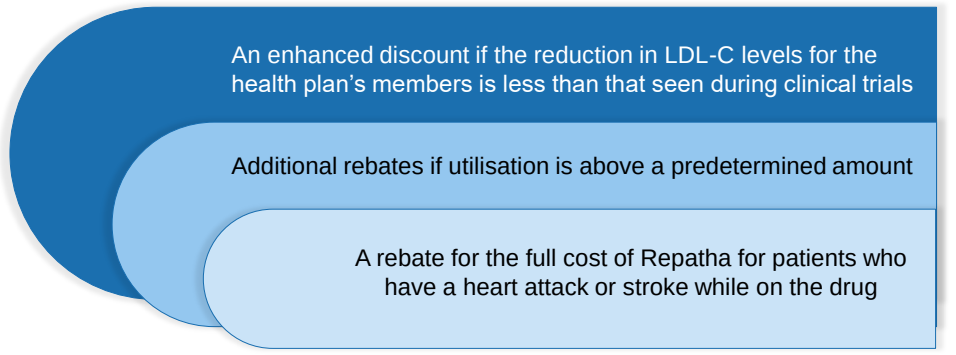
Value Based Contracts Are Becoming More Common in the United States[1]



- Contracts typically are not tied to value-based pricing but rather are tied to other measures such as achieving outcomes shown in the product labels
- Some payers have reported they are likely to use the threshold prices reported in reports from the Institute for Clinical and Economic Review to request rebates from manufacturers, but using reports for outcomes-based contracts may be difficult because the reports (as of 2019) do not list threshold pricing for different efficacy/safety scenarios

1. Seeley and Kesselheim, 2017.

Example: Repatha (Evolocumab, a Treatment of Hyperlipidemia) (Amgen and Harvard Pilgrim, 2015)[1,2,3]



- A report by the Institute for Clinical and Economic Review (2016) suggested the threshold value-based price to be $3,000 to $7,000 vs. $14,100 (listing price) per year
- In 2018, Amgen announced a 60% discount to the listed price ($5,850 per year)

LDL-C = low-density lipoprotein cholesterol.

1. Barlas, 2016.
2. Amgen, 2018a.
3. Amgen, 2018b.

Innovative Outcomes Based Contracts: Case Studies From 2018

Most US outcomes-based scheme	Not tied to value-based pricing
Praluent (alirocumab)	Collaboration with the Institute for Clinical and Economic Review to match the price to the institute's threshold value-based pricing An arrangement to pass the rebates to patients
Orbative (oritavancin)	Rebates based on the outcomes that fails the cost-offset threshold
Luxturna (voretigene neparvovec)	Rebates based on both short-term and long-term outcomes Long-term payment structure (multiple instalments)

Praluent (Alirocumab) for Hypercholesterolemia[1,2]

Collaboration between ICER and manufactures, in establishing a value-based price

- Manufacturers provided early access to data from a trial, to enable a revised assessment
- Based on ICER's new analysis results, the manufacturers offered net price within a cost-effective range to high-risk patients

Payment mechanism

- Payer will pass a portion of the rebates it received directly to patients

Coverage

- Simplified prior authorisation requirement
- A simplified attestation form confirming that Praluent is appropriate for the patient, based on the label and on patient history

ICER = Institute for Clinical and Economic Review.

1. Regeneron, 2018.
2. Sanofi, 2018.

Orbative (Oritavancin) for Acute Bacterial Skin Infections[1]

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A Medicaid contract based on the cost-offset argument

Manufacturer's value hypothesis

- Oritavancin will save costs by avoiding hospitalisation
- Oher drugs require the patient to be hospitalised while being treated

Contract

- Drug listed as a first-line treatment (vs. needing to fail another treatment)
- If the state does incur higher costs from oritavancin despite the avoided hospitalisations, the manufacturer will cover the costs through additional rebates to the state

1. National Academy for Health State Policy, 2018.

Luxturna (voretigene neparvovec, a gene therapy) for Inherited Retinal Disease[1]

RTI (h)(s) Health Solutions

Rebates if patient outcomes fail to meet

- Threshold short-term efficacy (30-90 days) measures
- Threshold longer-term durability (30 months) measures
- Outcomes are based on full-field light sensitivity threshold testing scores

An alternative to the traditional "buy and bill" model

- To sell directly to payers or payers' specialty pharmacy (vs. treatment centres)

Innovative payment model (proposal):

- Payers to pay in installments over several years vs. a single, up-front payment
- Challenges: current government requirement (the threshold) for payments and outcomes-based rebates.
- A proposal to CMS, in progress, to conduct a project that enables commercial and government payers an installment payment option, as well as greater rebates tied to clinical outcomes

1. Spark Therapeutics, 2018.

Potential Presentation of HTA Results That Can Be Translated to Outcomes-Based Contract Design

RTI (h)(s) Health Solutions

Step 1: Identify key cost-effectiveness drivers

Step 2: For a variety of scenarios for key model drivers, present threshold prices associated with a range of willingness-to-pay threshold ICER

Step 3: If key drivers are surrogate endpoints, suggest mapping between them and readily measurable outcomes

Presentation for each WTP threshold	Short-term response (in 6 months)	Shot-term key AE event (in 6 months)	Long-term (in 2 years) response	VBP / month associated with a WTP threshold
Base case	Base case	Base case	Estimated for base case	$1,000
Scenario 1	Worst-case scenario	Base case	Base case	$600
Scenario 2	Best-case scenario	Base case	Base case	$1,600
Scenario 3	Base case	Worst case scenario	Base case	$800
Scenario 4	Base case	Best case scenario	Base case	$1,100
Scenario 5	Base case	Base case	Best case	$1,500
Scenario 6	Base case	Base case	Worst case	$500

	Potential contract for a product with a manufacturer's price, $1,500 a month
Initial pricing	$500 discount (base-case analysis)
At 6 months, assess the average response rate	Worse short-term efficacy: manufacturer rebate up to $400 per month
	Better short-term efficacy: payer to pay back up to $600 per month
	Worse short-term safety: manufacturer rebate up to $200 per month
	Worse short-term safety: payer to pay back up to $100 per month
At 2 years, assess the average response rate	Better long-term efficacy: payers to pay back up to $500 per month
	Worse long-term efficacy: manufacturer rebate up to $500/month

AE – adverse event; VBP = value-based pricing; WTP = willingness-to-pay.

Outcome-Based Contracts: Implementation Hurdles

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Key value-based pricing drivers (e.g., response to treatment): not readily observable by payers (claims, health records)

Typical commercial plan members stay in the same program for the average of 2-3 years: payers unlikely to recoup the cost-offsets from long-term outcomes

A proposal to eliminate safe harbor protection for drug rebates under the anti-kickback statute in January 2019 (The Office of Inspector General of the Department of Health and Human Services): withdrawn in July 2019

- The Institute for Clinical and Economic Review recently published a white paper on alternative models for pharmaceutical rebates[1]
- The elimination of rebates may undermine progress towards meaningful outcomes-based contracts
- Whether the rebates should be paid to plan sponsors vs. patients directly is under discussion

1. Institute for Clinical and Economic Review, 2019.

Summary

	UK	US	Japan
Patient access schemes	Y	N	N
Managed access schemes	Y	N	N
Outcome-based contracts	Y	Y	N
Indirect use of ICER per QALY gained for pricing decision	N	Y	Y
Price adjustment based on budget-impact testing	Y	I	Y
Long-term payment structure	N	Y	N

- In all 3 systems reviewed, innovative pricing and reimbursement methods have been used to overcome challenges in rising health care costs
- Further collaboration among HTA bodies, payers, and manufactures is deemed necessary to establish sustainable value-based payment schemes

I = informal (no formal methods); N = no; QALY = quality-adjusted life-year; Y = yes.

Polls

Were there methods that are not used in your country that could be considered in the future	☐ Yes ☐ No

Which one (list presented methods)	What do you think is the biggest roadblock?
☐ 1) Patient access schemes	☐ 1) Difference in system
☐ 2) Managed access schemes	☐ 2) Timeline to implement
☐ 3) Outcome-based contracts	☐ 3) Gaining consensus
☐ 4) Indirect use of ICER per QALY gained for pricing decision	☐ 4) Others
☐ 6) Price adjustment based on budget-impact testing	

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Were there methods that are not used in your country that could be considered in the future?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Which method would you like to consider for your country's HTA?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: What do you think is the biggest roadblock?

Questions?

Thank You!

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RTI (h)(s)
Health Solutions

Supplemental Materials

NICE Methodologies for Technology Appraisal and Highly Specialised Technologies

RTI (h)(s) Health Solutions

Technology Appraisal

Topic selection	Identified by NIHRIO; selection by NICE, DoHSC, and NHS England; referral by DoHSC or routed via MTEP process (devices/diagnostics only)
Type of technology(s) assessed	Single (**STA**) or multiple (**MTA**) technologies, including: • Pharmaceutical products • Medical devices • Diagnostics • Surgical procedures • Health promotion activities or a single technology for multiple indications (**MTA**)
HTA method(s) used and thresholds	**MTA/STA: CEA; ICER < £20,000-30,000 per QALY** (additional weighting for EOL treatments up to £50,000) **Fast track (FTA): CEA; ICER < £10,000 per QALY or cost comparison** shows similar or greater health benefits at similar or lower cost

Highly Specialised Technology

Evidence submission	**FTA/STA:** by company, critiqued by ERG **MTA:** by companies and ERG
Recommendations	5 options: • Recommended • Optimized • Only in research • Not recommended • Recommended in the CDF (full/optimized)
Approximate timeline[a] (published)	**MTA:** 47-60 weeks **STA:** 41-50 weeks **FTA:** 32 weeks
Funding mandate for positive guidance	Yes: for **MTA/STA** after 3 months (or 9 months if budget impact exceeds £20 million per year); for **FTA** after 30 days

CDF = Cancer Drugs Fund; CEA = cost-effectiveness analysis; DoHSC = Department of Health and Social Care; EOL = end-of-life; ERG = Evidence Review Group; FTA = fast-track appraisal; ICER = incremental cost-effectiveness ratio; MTA = multiple technology appraisal; MTEP = Medical Technologies Evaluation Programme; NIHRIO = National Institute for Health Research Innovation Observatory; QALY = quality-adjusted life-year; STA = single technology appraisal.
[a] Timings are approximate from preparation of draft scope (week 0) to final guidance publication and are subject to change.
Sources: Adapted from Stevenson et al., 2018; NICE, 2019d.

NICE Methodologies for Technology Appraisal and Highly Specialised Technologies

RTI (h)(s) Health Solutions

Technology Appraisal

Topic selection	As technology appraisal
Type of technology(s) assessed	Single technology for a single indication for very rare conditions. All of the following must apply • Small target patient group treated in very few NHS centers • Clinically distinct patient group • Chronic and severely disabling condition • Expected use exclusively in highly specialized services • Very high acquisition cost • Potential for lifelong use • A significant need for national commissioning
HTA method(s) used and thresholds	**CEA: maximum threshold < £300,000 per QALY**

Highly Specialised Technology

Evidence submission	Company evidence submission, critiqued by ERG
Recommendations	4 options • Recommended • Optimised • Only in research • Not recommended
Approximate timeline[a] (published)	25-35 weeks
Funding mandate for positive guidance	Yes: as MTA/STA

[a] Timings are approximate from preparation of draft scope (week 0) to final guidance publication and are subject to change.
Source: Adapted from Stevenson et al., 2018.

The power of knowledge. The value of understanding.

Cancer Drugs Fund in England

Selection	**All new cancer drugs, and significant new licensed indications for cancer drugs, are referred to NICE for appraisal** **A drug/indication can be identified for entry into the CDF at several points during a technical appraisal** • **At submission of evidence by the pharmaceutical company when the submission dossier includes a proposal for data collection** • **At the assessment phase when the ERG or NICE identifies that the drug could be a candidate for the CDF** • **At the appraisal committee meeting**
HTA method(s) thresholds	< £20,000-30,000 per QALY (additional weighting for EOL treatments up to £50,000)
Recommendations	3 options • Recommended for routine commissioning – 'yes' • Not recommended for routine commissioning – 'no' • Recommended for use within the CDF
Managed Access Agreement	A managed access agreement will need to be agreed upon between the pharmaceutical company and NHS England to resolve significant clinical uncertainty after consideration by NICE The managed access agreement consists of • Data collection agreement – presents the outcomes that need to be collected to resolve key areas of clinical uncertainty • Commercial agreement – determines the cost of the drug during the duration of the managed access agreement
Approximate timeline (published)	Draft guidance prior to a receiving its marketing authorisation Final guidance within 90 days of marketing authorisation, wherever possible
Funding mandate for positive guidance	Yes, funded from the very first positive recommendations from NICE, usually the Final Appraisal Determination Usually, but not exclusively, funding is for no more 2 years Pharmaceutical companies have the option of accessing interim funding from marketing authorisation for drugs that have received either a draft recommendation for routine commissioning – 'yes' or a draft recommendation for use within the CDF

[a] Two key criteria have been met: 1) the treatment is indicated for patients with a short life expectancy, normally less than 24 months; and 2) there is sufficient evidence to indicate that the treatment has the prospect of offering an extension to life, normally of a mean value of at least an additional 3 months, compared with current NHS treatment.
Sources: NHS England, 2016; NICE, 2019b.

Pricing of Branded and Generic Medicines

Branded Medicines		Generic Medicines
2019 Voluntary Scheme for Branded Medicines Pricing and Access	**Statutory Scheme**	
Replaces the 2014 Pharmaceutical Price Regulation Scheme Parties involved include • Department of Health and Social Care, acting on behalf of the UK Government and the governments of Scotland, Wales and Northern Ireland • NHS England • Association of the British Pharmaceutical Industry • Manufacturers or suppliers of Branded Health Service Medicines that have joined the Voluntary Scheme NICE will have a central role in the operation of the 2019 Voluntary Scheme Growth in NHS spending on new drugs will be capped at 2% a year for the next 5 years	• Any company that is not a member of the 2019 Voluntary Scheme is automatically subject to the Statutory Scheme • Works in a similar way to the 2019 Voluntary Scheme • The Voluntary Scheme is renegotiated every 5 years, whereas statutory regulations may change at any time, subject to approval by Parliament	Generic medicines are covered by the Drug Tariff • Produced each month by the Pharmaceutical Directorate of the NHS Business Services Authority Free pricing NHS has relatively limited influence over how much generic medicines cost

Source: Department of Health & Social Care, 2018; House of Commons Committee of Public Accounts, 2019; Thomson Reuters Practical Law, 2019.