The evolving approach to medicines value assessment in European markets

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HTA Conference, Athens, 22 January 2019

THE LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE

Overview

- 12 issues to address spanning from regulatory developments to value assessment and funding schemes

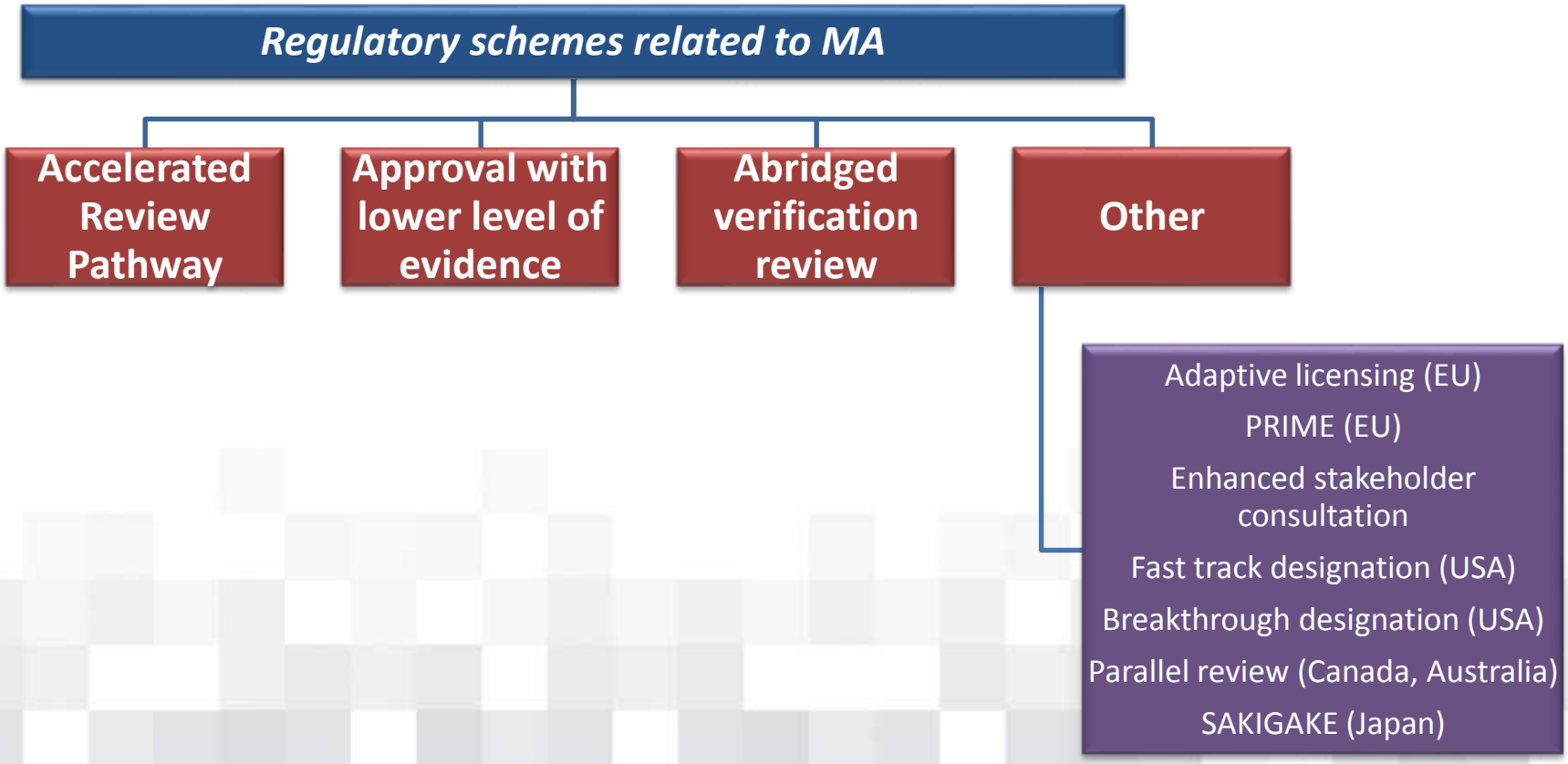
1. The Regulatory Landscape and how it will change in the future

- No fundamental changes to the way drugs are granted MA; but the regulatory landscape will change in 4 ways, which will have momentous implications for evidence collection, RWE and the way regulators and HTAs assess such evidence

1. Respond to increased transparency calls
2. Increase efforts to ensure earlier market access
3. Greater use of other forms of MA
4. Newer/emerging study designs challenging current approaches by HTAs

2. Early Market Access schemes and pathways

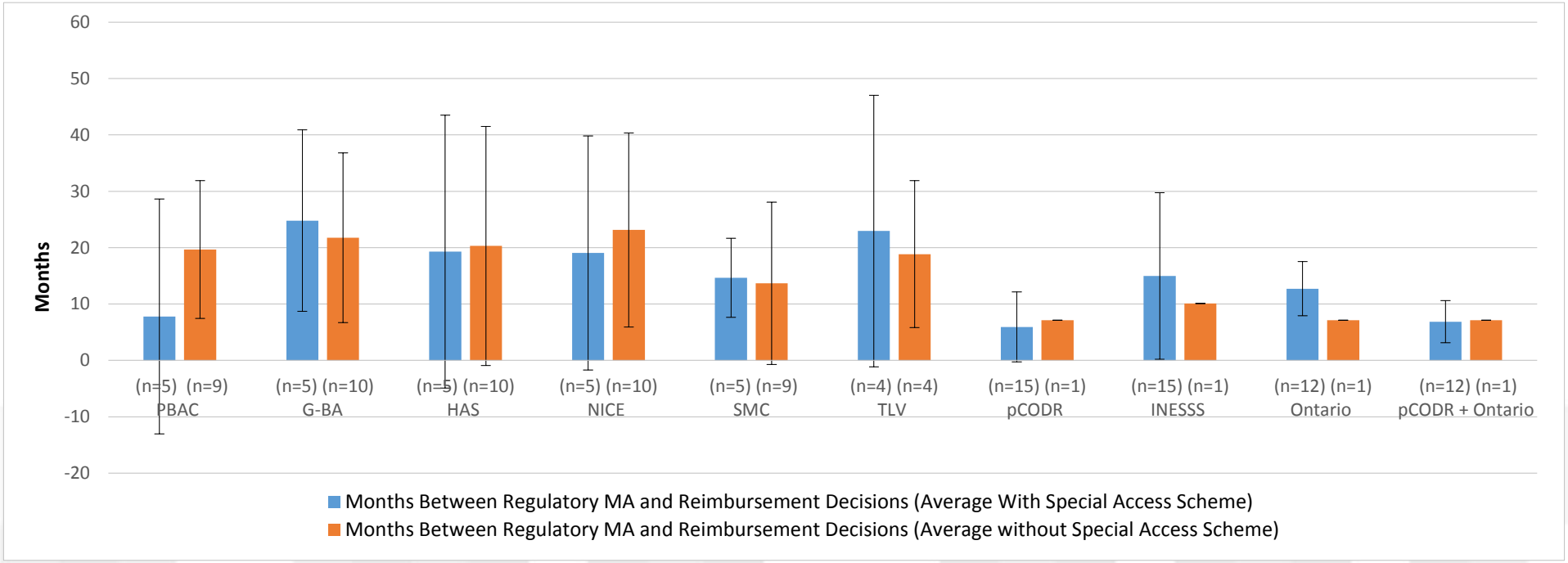
Taxonomy of early regulatory approval schemes



Source: LSE, 2017.

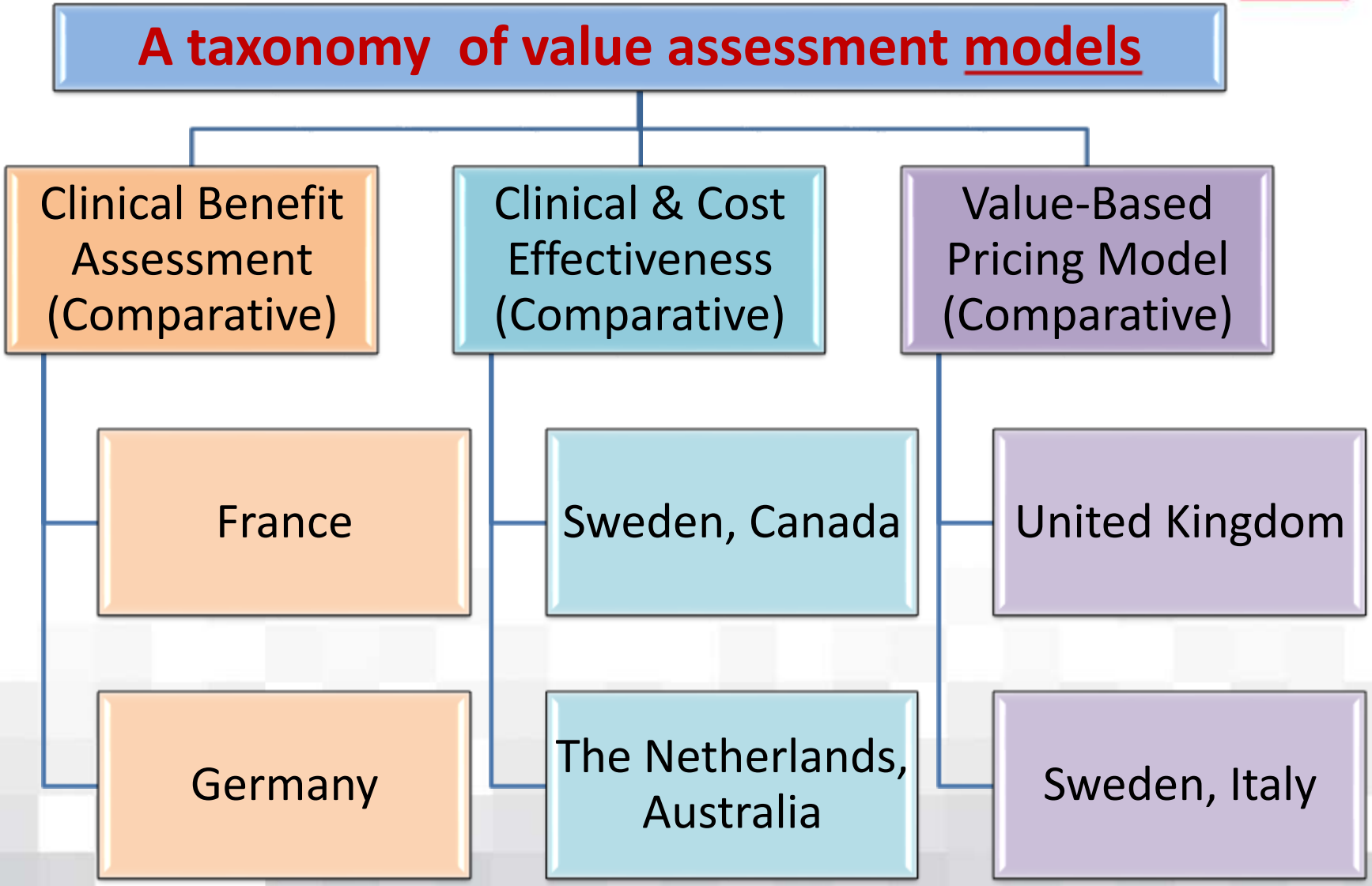
2. Early Approval schemes have little impact at HTA level

Marketing authorization vs HTA/funding decision in cancer medicines



Kanavos et al, 2019 forthcoming

3. Models of value assessment and examples of how they relate to different settings: more countries doing VA & HTA



4. Multiple dimensions of value shape coverage decisions

		France	*Germany*	*Sweden*	*England*	*Italy*	*Netherlands*	*Poland*	*Spain*
Burden of disease									
	Severity	***	**	**	**	*	**	**	**
	Availability	***	*	*	***	*	**	*	**
	Prevalence	*	**	*	*	**	**	**	**
Therapeutic									
	Direct endpoints	***	***	***	***	***	***	***	***
	Surrogate endpoints	**	**	**	**	**	**	**	**
Safety									
	Adverse events	***	***	***	***	***	***	***	***
	Tolerability	**	**	**	**	**	**	**	**
	Contraindications	**	**	**	**	**	**	**	**
Innovation									
	Clinical novelty	***	*	*	*	**	**	***	**
	Nature of treatment	***	*	*	**	x	*	***	**
	Ease of use & comfort	*	*	**	*	x	*	x	*
Socioeconomic									
	Public health	**	**	*	**	*	***	***	*
	Budget impact	*	***	**	***	**	**	***	**
	Social productivity	*	**	***	**	*	**	*	**

***	mandatory/ formal/explicit/ planned/ directly/ grading system
**	"considered", e.g. recommended, informal/implicit but planned, formal/explicit but ad-hoc/indirectly, etc.
*	optional/ informal/implicit/ad-hoc/ indirectly/ no grading system
x	not considered in any way

Source: Angelis and Kanavos, *Social Science & Medicine* 2017

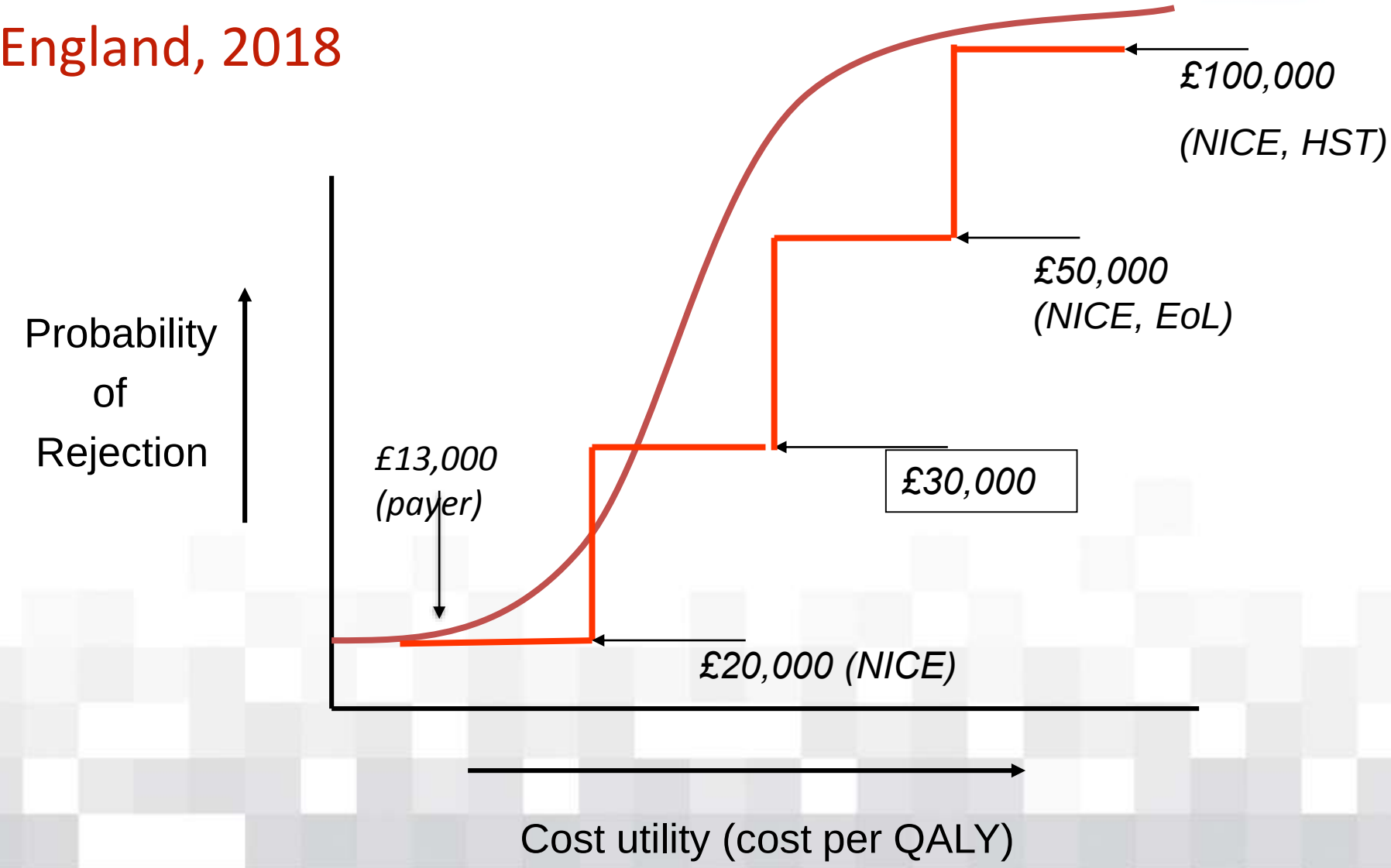
5. Multiple WTP thresholds depending on product type

- Overarching principles:

1. Economic perspective vs societal perspective
   - Health system and social services/care
2. Cost effectiveness
   - Not affordability or budgetary impact
3. **Balance between:**
   - **Efficiency (utilitarianism – someone loses out)**
   - **Fairness (egalitarianism – equity prevails and no one loses out)**
   - **Subscribing to the fairness principle means that additional criteria are considered**

>>> What is our Willingness to Pay (WTP) threshold?

5. Multiple WTP Thresholds and how NICE works it out – England, 2018



6. Value-based procurement

- May imply multiple thresholds with 'payer' WTP being lowest, often lower than HTA
- Strategic procurement involving negotiation or tendering in in-patent medicines
- UK, Nordics, Baltics

The Council of Medicine: Denmark

'Added value':

'The extra value a pharmaceutical offers compared to existing treatment in terms of prolonging life, adverse effects and quality of life.'

Danish Regions 2016

Six categories:

1. Major added value
2. Important added value
3. Minor added value
4. No added value
5. Negative added value
6. Non-demonstrable added value.

Amgros

Economic assessment:

1. Added treatment costs compared to existing treatment alternatives
2. Budget implications.

Definition of range for 'acceptable price level'

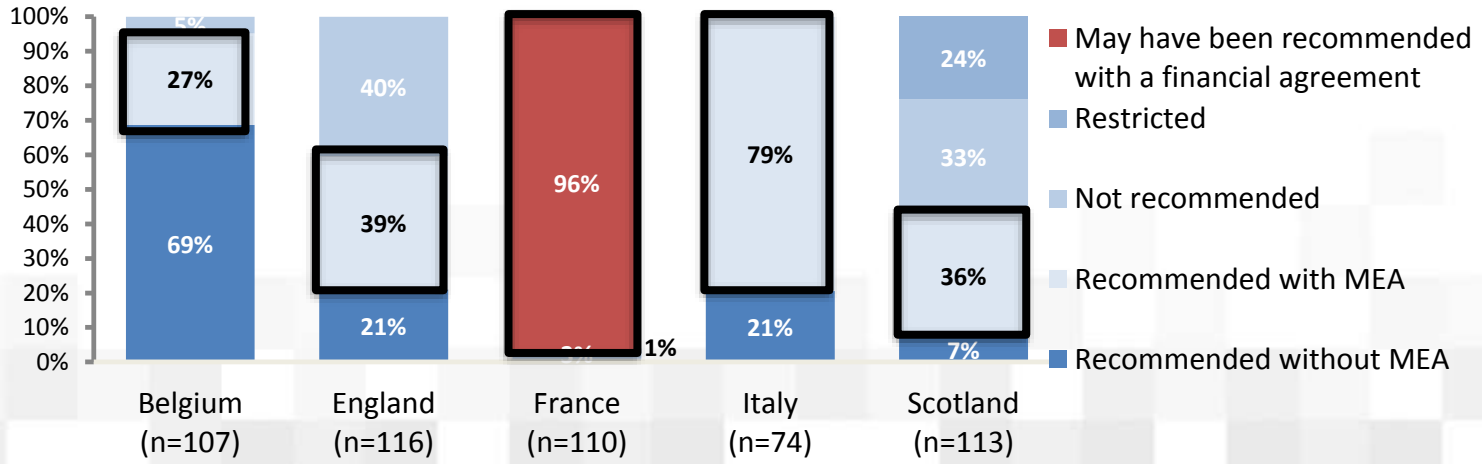
Price negotiation

Treatment recommendation

7. Uncertainties over evidence generate higher prevalence of managed entry agreements

- HTA agencies increasingly express doubts over clinical and economic impacts of new drugs, relying on managed entry agreements (e.g., financial, outcomes-based, or combination) to protect national budgets and share risk with biopharmaceutical companies
- Financial arrangements (e.g., price-volume, price discount, cap) account for the majority of MEAs

Most HTA agencies recommend fewer drugs without restrictions or managed entry agreements



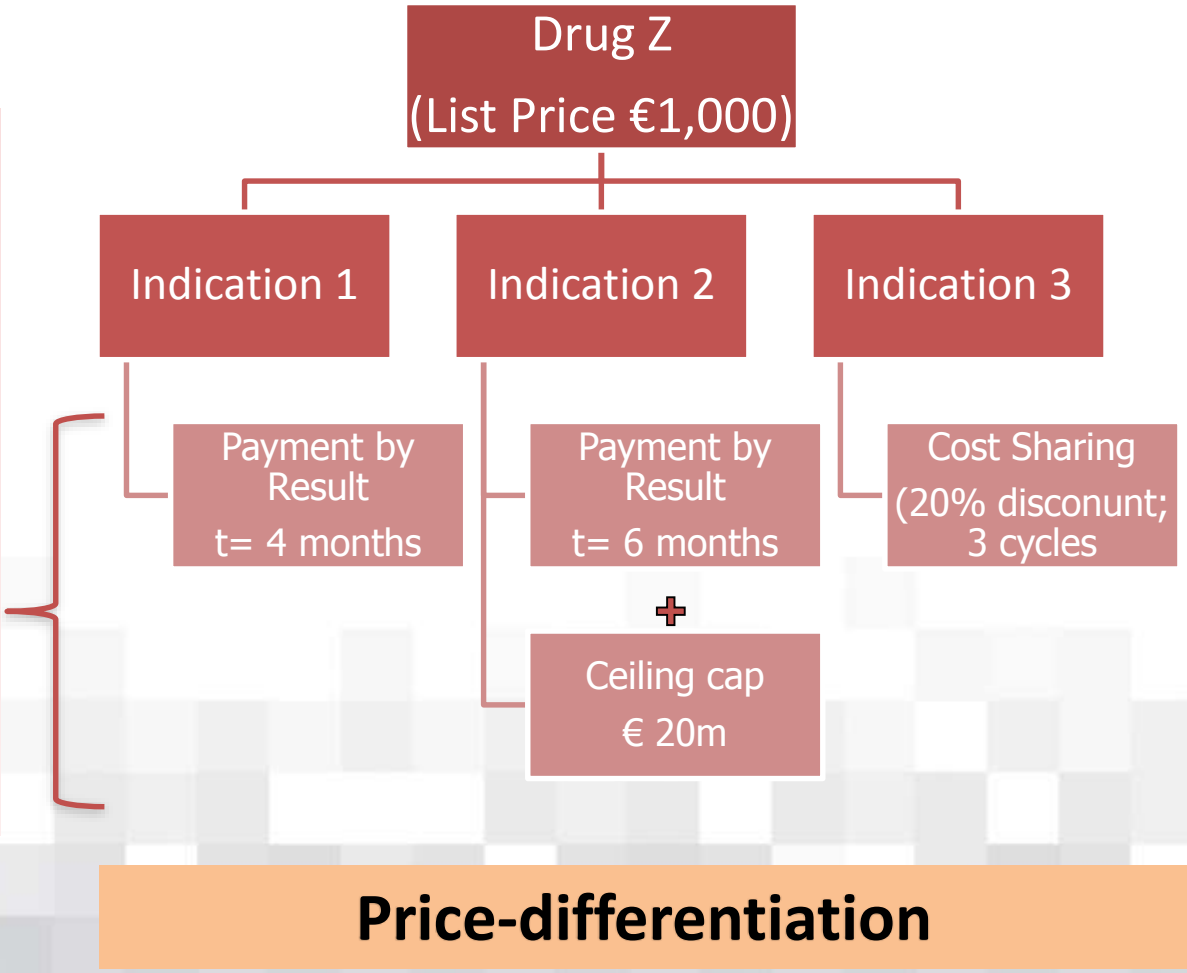
Abbreviation: MEA, managed entry agreement.
Note: France keeps evidence of financial MEAs confidential.
n=total number of drug-indication pairs studied 2012-2016.
Source: LSE, March 2017.

8. Multiple indications: Drive for indication-based reimbursement

Same list price, value-based cost – example: Italy

Drugs with multiple indications may be reimbursed under different schemes or using the same scheme with different features.

Requirement: monitoring registries



9. Decision-making (appraisal)

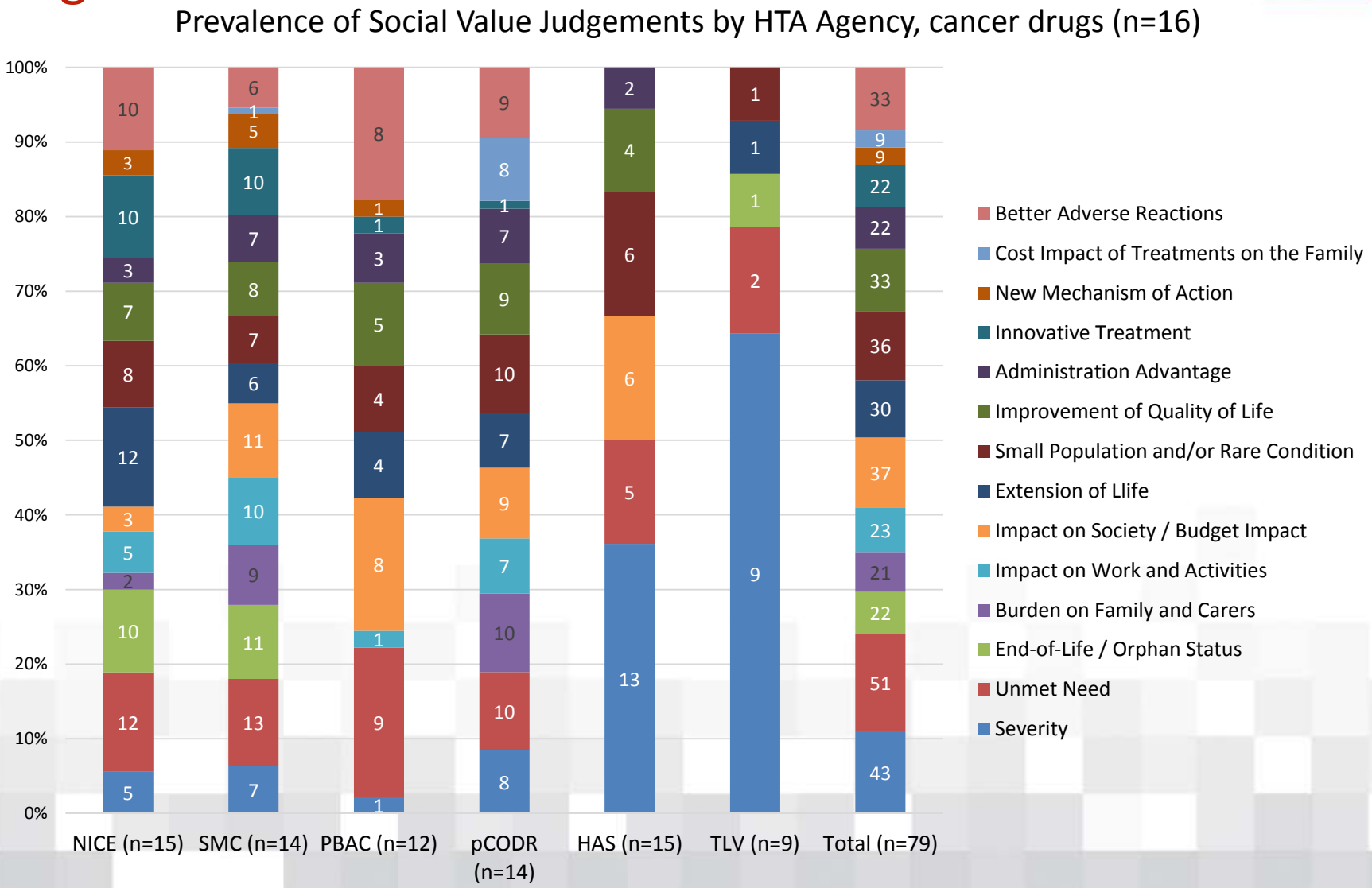
- What kind of judgements are we making (irrespective of the model of HTA)?

- Scientific judgements
  - Reliability/Quality of the evidence-base
  - Appropriateness of sub-groups and the associated analysis
  - Generalisability in population
  - Capturing quality of life adequately
  - Handling uncertainty

- Social value judgements
  - Severity of disease
  - End of life interventions ("rule of rescue")
  - Age
  - Health inequalities

  - SVJs are taken into account, but there is lack of appropriate metrics
  - SVJs can be 'revealed' (e.g. 'rarity' or 'end of life interventions') but can also be 'implicit' judgements based on treatment characteristics or the disease profile

9. Social value judgements across 7 HTA agencies



10. Special funding arrangements

- Mandate**:** To avoid that a patient suffering from a very serious medical condition, must forgo treatment because the essential health service they need is not reimbursed and it is particularly expensive
- Conditions covered: rare indications, rare diseases, innovative medical devices or medical procedures, for children with chronic diseases, for care abroad, other 'expensive' medicines
- Key issue: budget expansion and ever increasing cost
- Examples:
  - Special solidarity Fund (Belgium)
  - Add-ons policy (the Netherlands)
  - Cancer Drugs Fund (England)
  - Orphan Drugs Risk Share (Scotland)

11. Cross border collaboration initiatives and their impact: Spectrum of activities

Four areas where cross-border collaboration could have an impact

Joint **Horizon Scanning**

- Countries use common methodology to horizon scan for new technology
- Exchange information on horizon scanning
- Enables HTA bodies and reimbursement agencies to be prepared when a new technology is launched
- Minimal threat for industry

Joint **HTA**

- Countries agree on principles to conduct joint HTA
- Determine common stance on value of new medical technologies to be assessed
- Results used to inform coverage decisions at national level
- Viable but technical details not yet developed

Joint **Negotiation**

- Requires close collaboration and coordination of activities to achieve tangible benefits and agreement on key endpoints
- Larger market with stronger negotiating power
- One country will have to lead
- Pharma will benefit as large market guarantee and only one reimbursement dossier required

Joint **Procurement**

- Combined purchasing of goods by two or more contracting member states with one tender published on behalf of all countries in collaboration
- Joint HTA and negotiation pre-requisites
- Requires close collaboration and agreement on willingness to pay, price and budget availability

Most stakeholders believe that chances of agreeing on any pan-EU pricing and reimbursement systems are remote as healthcare is generally seen as a national issue. However, it is likely that there will be developments on EU-wide HTA issues in the next 3-5 years, following the draft regulation proposal by the EU Commission on HTA.

11. Cross border collaboration initiatives and their impact

Groups of countries collaborate to form clusters aiming to improve medicines supply

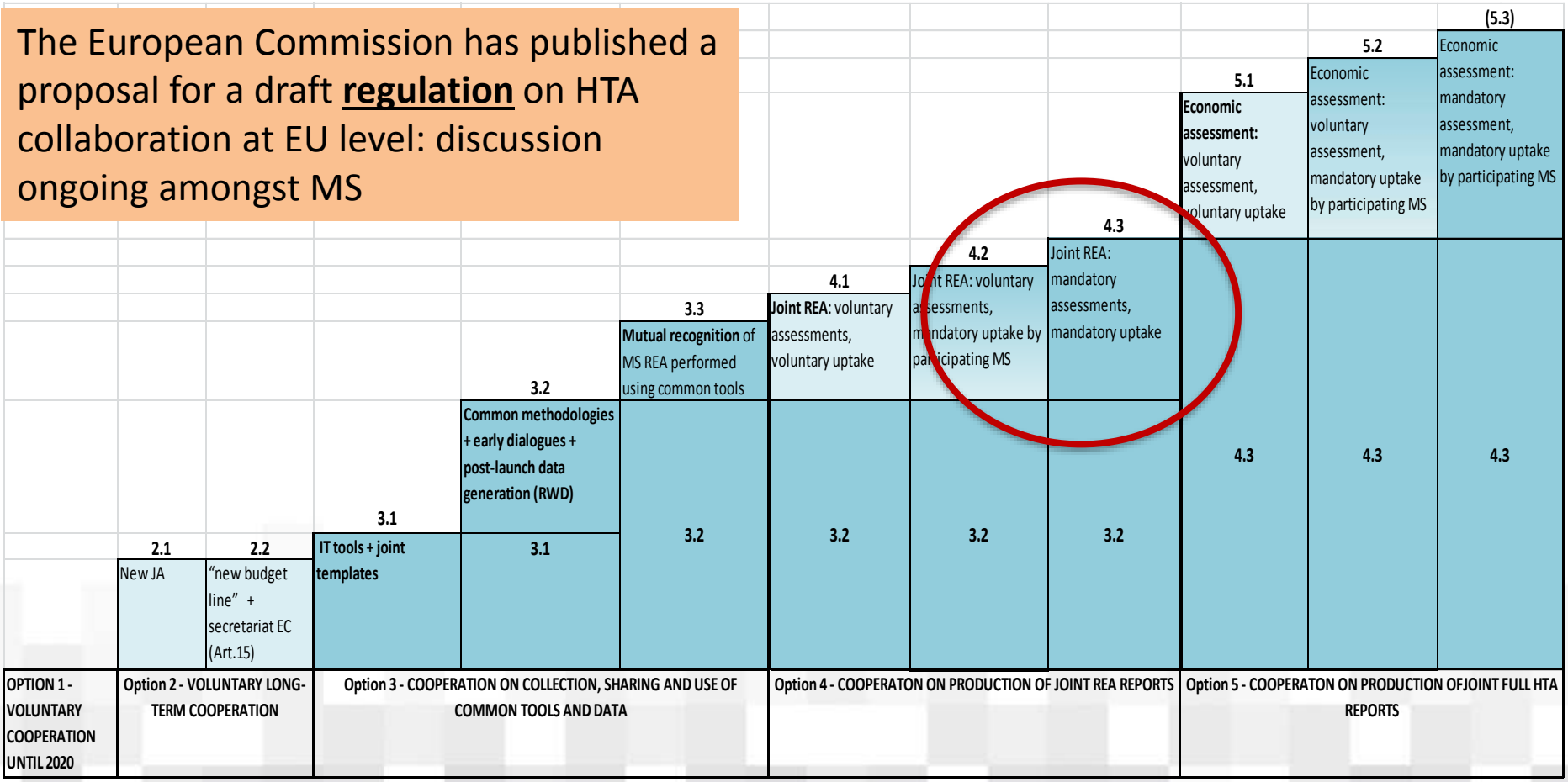
↑ Market size, bargaining power and security of supply

BENELUXA	CEEC	"Nordic Forum"	Valetta Declaration
• Belgium • The Netherlands • Luxembourg • Austria	• Central and Eastern European Countries	• Denmark • Norway • Sweden • Finland • Iceland	• Portugal • Spain • Italy • Greece • Malta • Cyprus • Ireland • Others

- **2016**: Belgium, Luxembourg, The Netherlands and Austria (BENELUXA) started working together to increase their bargaining power; collaboration spans across the spectrum of activities, ranging from joint horizon scanning, joint HTA, joint negotiation and joint procurement, with pilots on Horizon Scanning and P&R
- **February 2017**: Announcement of Joint Horizon Scanning model by BENELUXA
- **2016**: The Nordic Forum countries (SWE, NOR, DEN, ISL) have announced their intent to collaborate further in strategic procurement and also conduct joint horizon scanning activities

12. Supra-national initiatives: EU HTA collaboration

The European Commission has published a proposal for a draft **regulation** on HTA collaboration at EU level: discussion ongoing amongst MS



Link: http://ec.europa.eu/smart-regulation/roadmaps/docs/2016_sante_144_health_technology_assessments_en.pdf

Concluding remarks

- Different methods of assessing value of medical technologies – none perfect
- HTA not tantamount to economic evaluation
- Special funding arrangements
- Irrespective of the prevailing model a broader selection of criteria are used – attempts at addressing overall value on a case-by-case basis
- There are always (significant) exceptions to the benchmark model in use
- Multiple thresholds, strategic procurement

Thank you!

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