



The exciting new world of the 'Estimand'

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Why is what is good enough for approval not good enough for reimbursement?

Healthcare Technology Assessment (HTA)

- is the *systematic evaluation* of the *properties, effects*, and/or *impacts* of health technology.

Purpose- to address the direct, indirect, intended, and unintended benefits and consequences of the adoption of healthcare technology .

-Hailey, Babidge, Cameron, & Davignon 2010

We use both kinds of data already...

- RCT



Efficacy

Does it work in experimental setting

Population selected

Placebo or a selected comparator



- RWE



Effectiveness

How does it work in medical practice

Patients as they come

Many alternative treatments

How to do things differently.....



Cost-
effectiveness

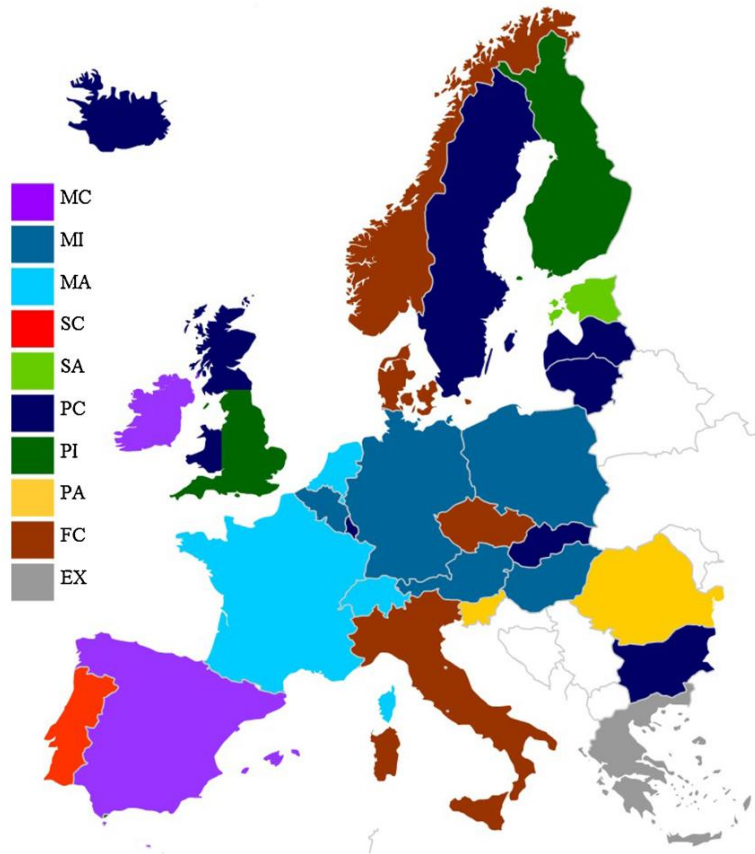
Added
clinical
Benefit

Budget
optimisation

...and highly complicated

310

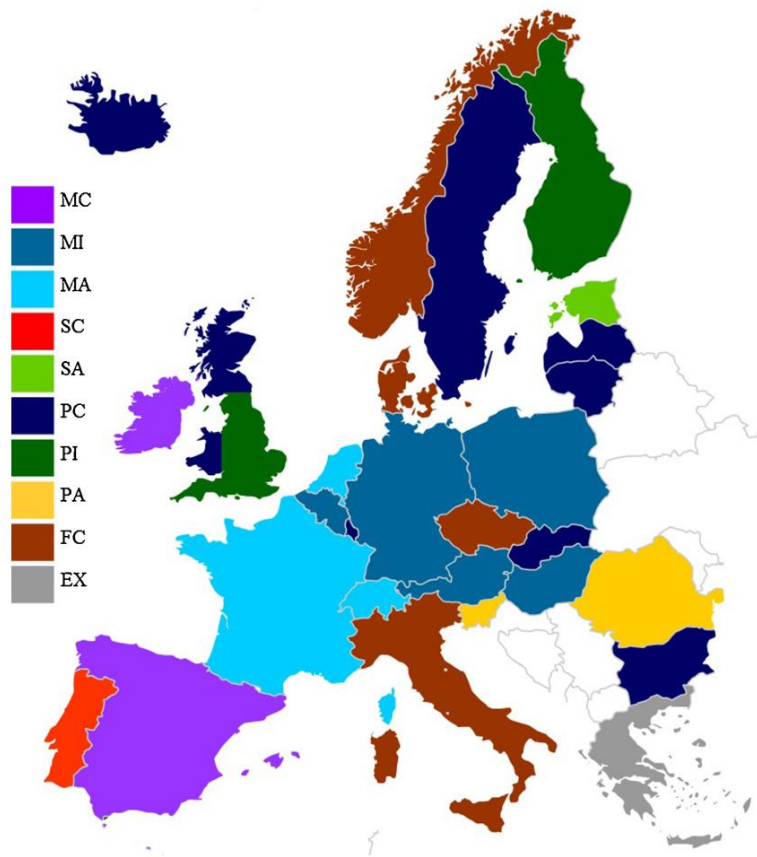
N. Allen et al. / Health Policy 113 (2013) 305–312



...and highly complicated

310

N. Allen et al. / Health Policy 113 (2013) 305–312



Development of archetypes for non-ranking classification and comparison of European National Health Technology Assessment systems

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System Process Archetypes

	M	S	P	F	E
C		DC (CYP) NCPE (IRE) DGFPs (SPA) MOH DTC (MAL)	INFARMED (POR)	AWMG (WAL) PDL (BUL) IMPRC (ICE) CHE (LAT) LRC (LIT) MSS (LUX) CC (SVK) SMC (SCO) TLV (SWE)	SUKL (CZE) DKMA (DEN) NOMA (NOR) AIFA (ITA)
I		HEK (AUS) INAMI (BEL) IQWIG (GER) OHTA (HUN) AHTAPol (POL)		NICE (ENG) HILA (FIN)	
A		HAS (FRA) CFH (NET) FDC (SWZ)	SAM (EST)	MoH (ROM) ZZS (SVN)	
X					GREECE LIECHTENSTEIN

Archetype Key
MC
MI
MA
SC
SA
PC
PI
PA
FC
EX

Models to 'predict' the future

- All models are wrong; some models are useful

George E. P. Box; Norman R. Draper (1987)

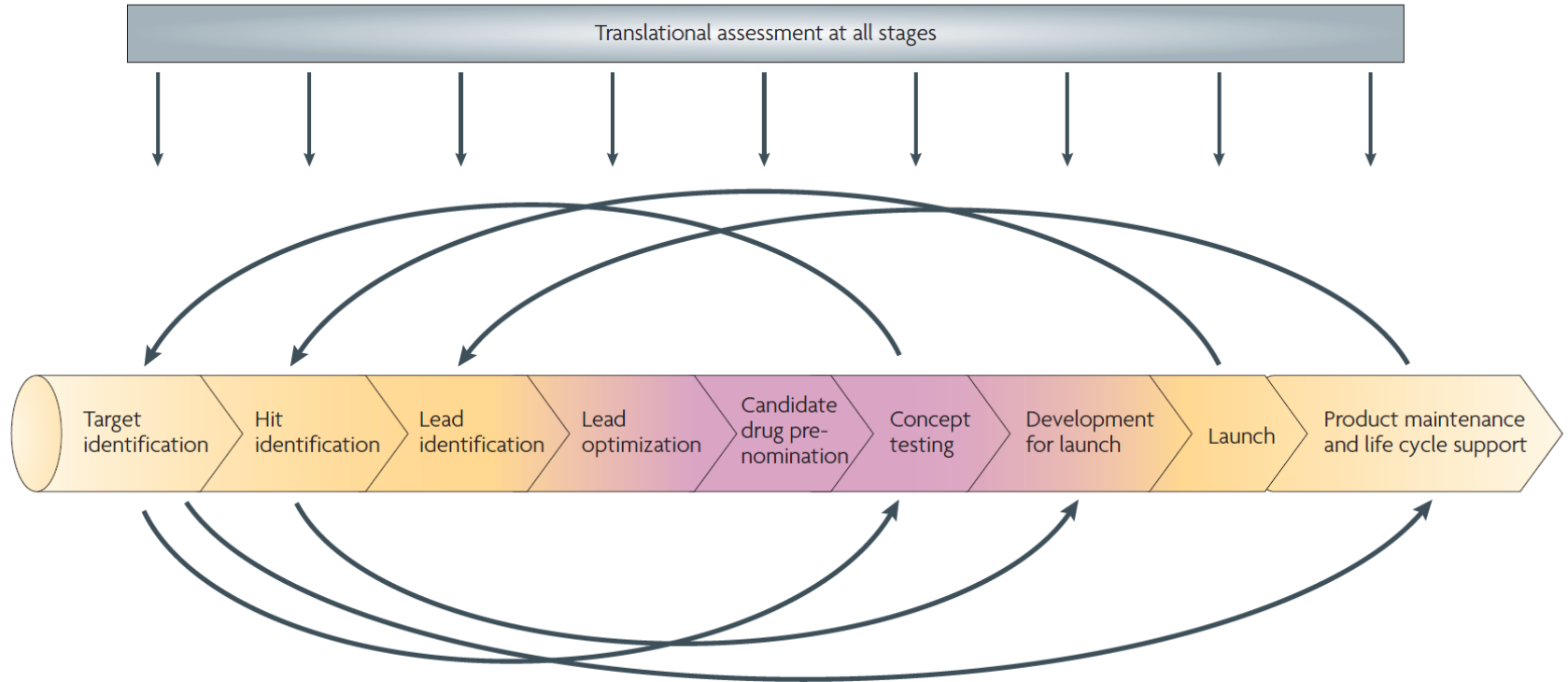
- Health economic models predict the future based on available data from different sources

Regulatory

HTA



The process might be linear



The thinking shouldn't

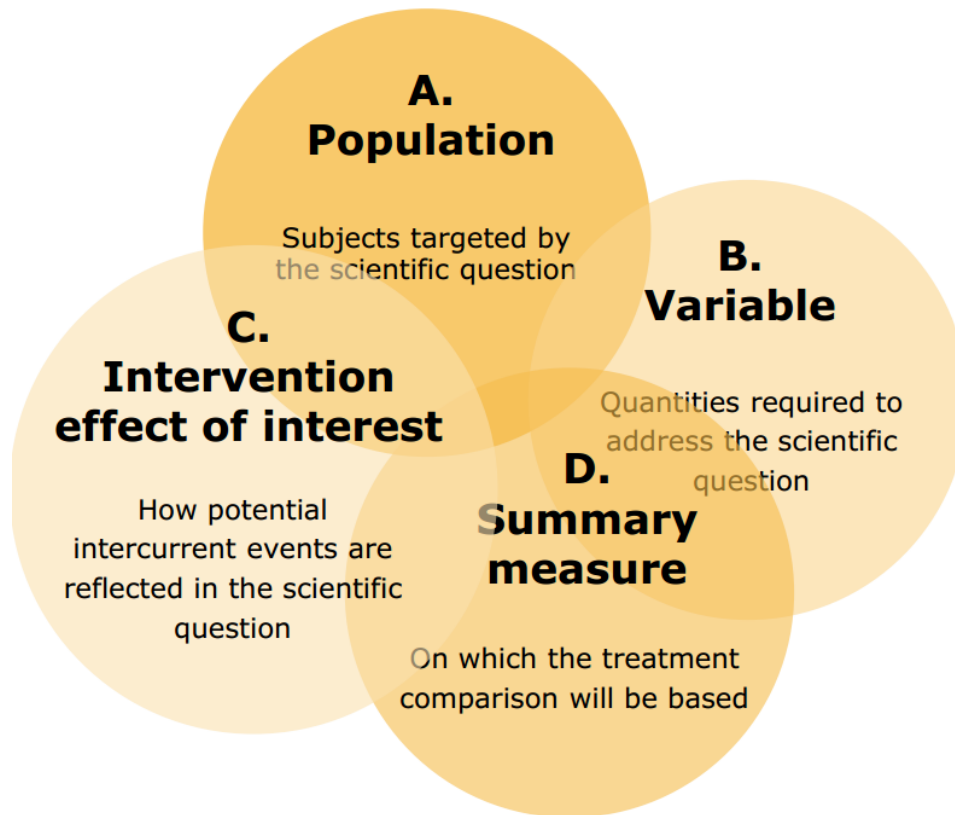


Figure 1: Description of an Estimand.

Source: Estimands and Sensitivity Analyses: What's in the new ICH E9 Addendum?

Chrissie Fletcher, 1 Day PSI Meeting on Estimands, 27th Sept 2017

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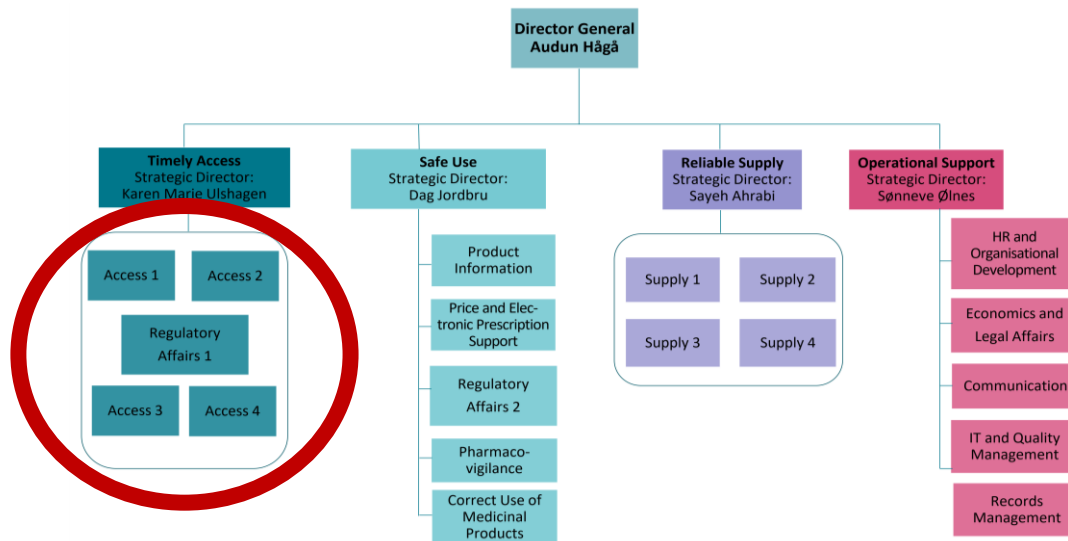
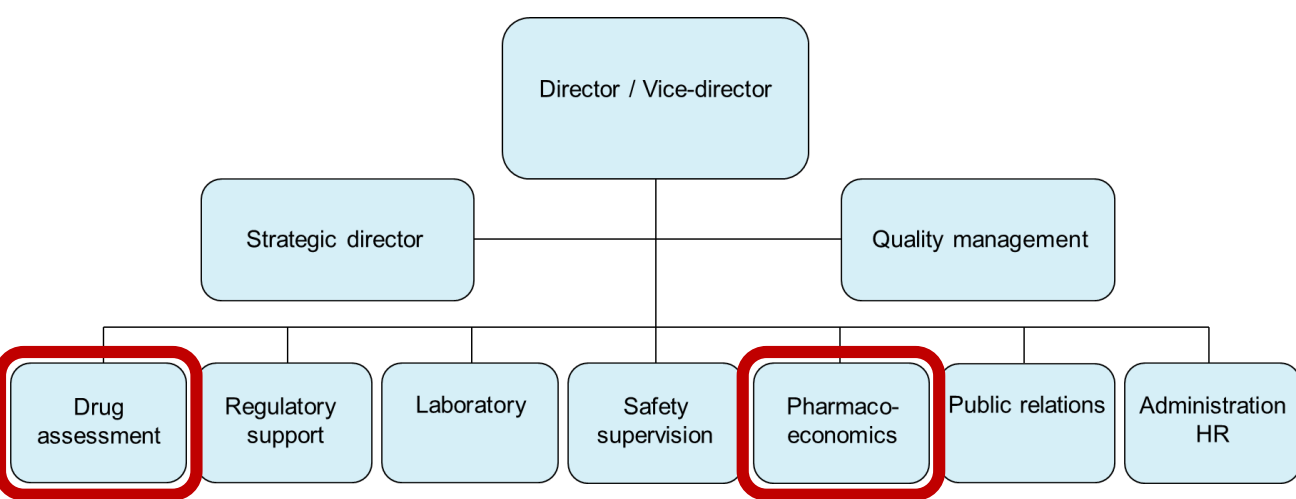


Effectiveness

How does it work in medical practice

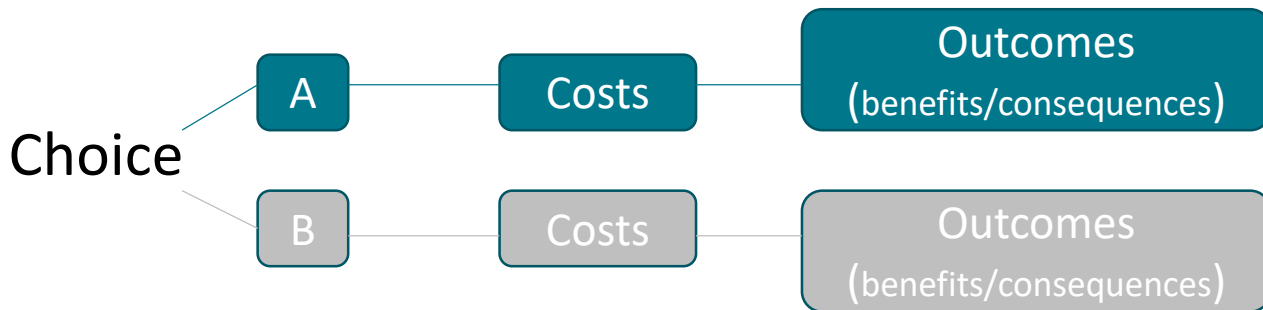
Patients as they come

Many alternative treatments



HTA: the basics

- The aim is to maximize the health of the total population within the given budget
- HTAs want value for money!



Economic evaluation

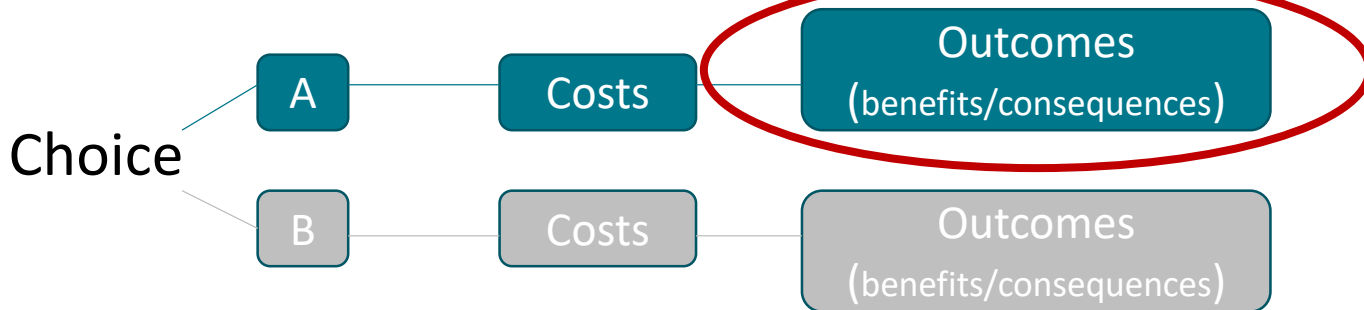
‘the **comparative analysis** of alternative courses of action in terms of both their **costs** and **consequences**’

(Drummond McGuire, 2001)

$$\text{ICER} = \frac{\text{Incremental costs (A-B)}}{\text{Incremental benefit (A-B)}}$$

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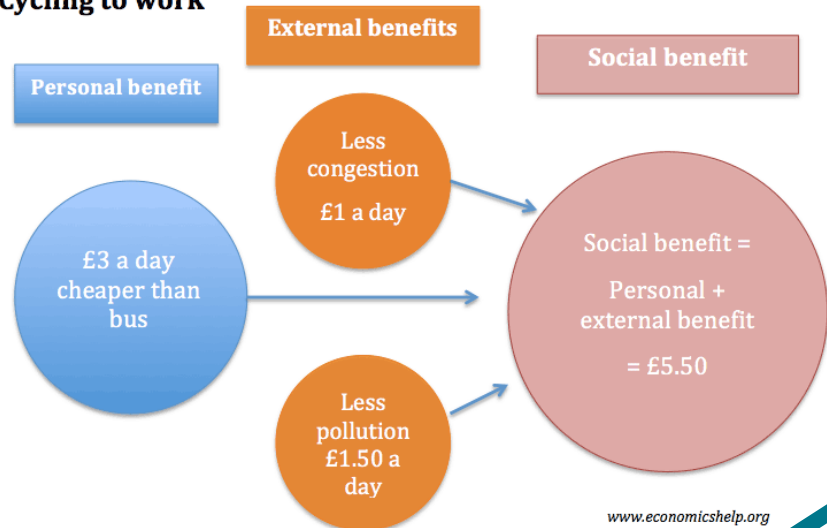
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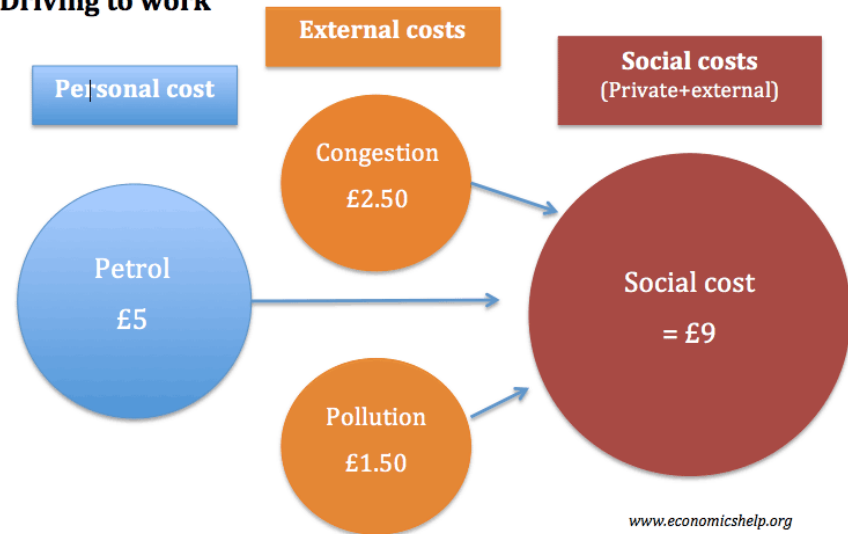
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Cycling to work



Driving to work



The Estimand is taking a different approach

- It focuses the discussion on the 'true value' of a new drug
- It requires a multidisciplinary discussion and that includes the guys from the sales department!
- We no longer focus on one analysis but rather a set of analyses, some of which might be far more relevant to different stakeholders
- Could we see different study designs and development programs on a better understanding?
- We finally don't want to simplify but make it more complex

The future might finally require more complex analyses than just linear regression!

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