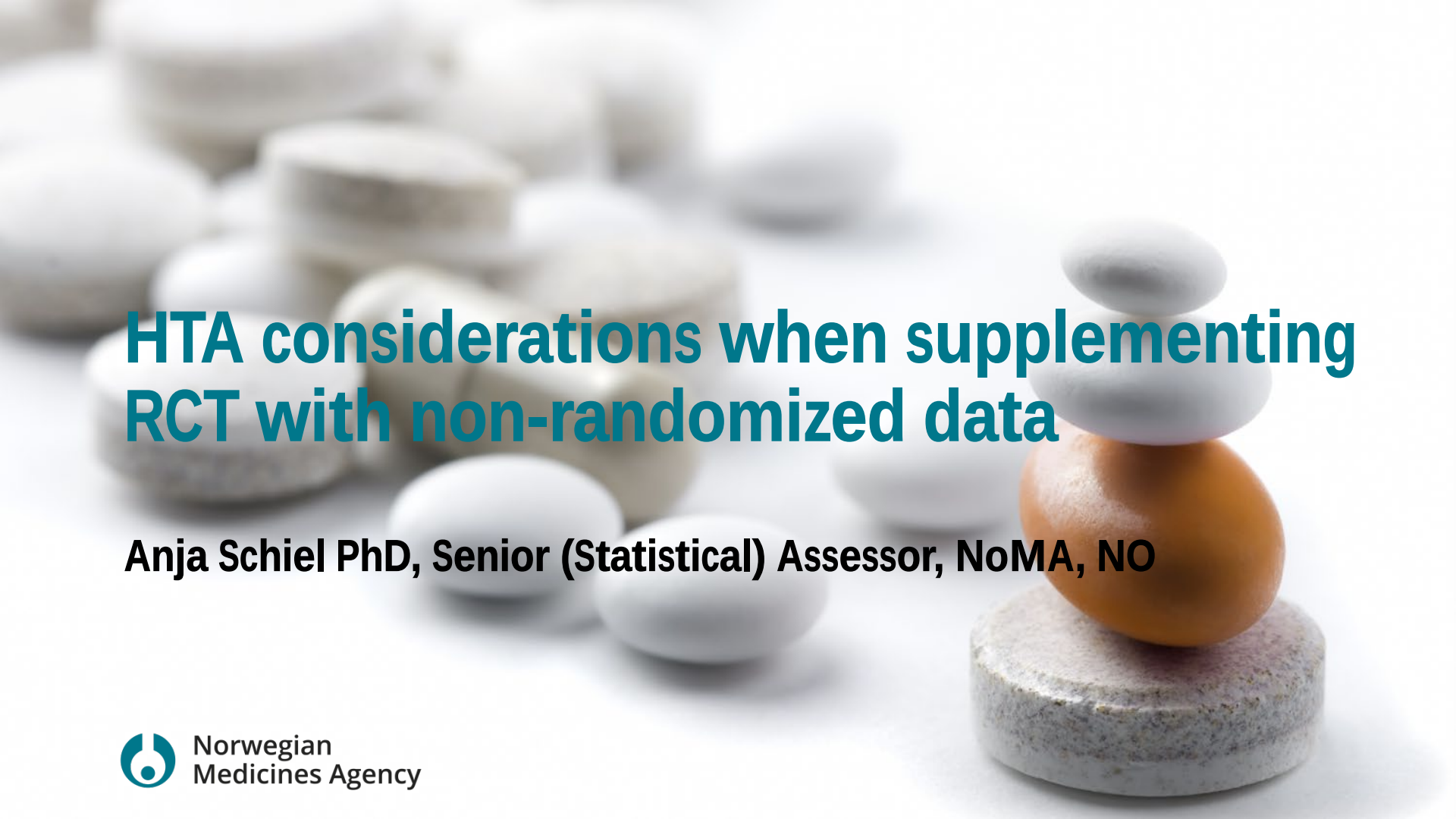

HTA considerations when supplementing RCT with non-randomized data

Anja Schiel PhD, Senior (Statistical) Assessor, NoMA, NO

Norwegian Medicines Agency

Disclaimer

The views presented here are my own and should not be considered the views of NoMA, EMA or all HTAs in general

RWE, the new magic bullet

- Not in the HTA world
- Data sources other than RCTs have always been used.

RWE, the new magic bullet

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- A health technology assessment has to:

  - Ø Incorporate all appropriate evidence into the analysis
  - Ø Compare the new intervention with the full range of relevant alternatives
  - Ø Reflect uncertainties in the conclusions of the analysis

HTA models have always relied on the use of f.e.

- Utility values have routinely been derived from large observational studies and surveys
  - If one has a Societal perspective this is unavoidable
  - NoMA has a mixed, societal/payer perspective, yet we tend to accept observational utilities over trial derived utilities (the patients perspective)
- Expected number of patients are often based on sources such as the Norwegian prescription database
- Non-drug related costs, related to national clinical practice are variables often derived from registries/expert opinion

Is RWE always acceptable?

- No but it can be:
  - Small populations
  - ATMPs
  - Orphan drugs (COMP)
  - Personalized medicine
  - Histology independent (agnostic)

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How do you want it - the crystal mumbo jumbo or statistical probability?

The problem

- RCT

Efficacy

Does it work in experimental setting

Population selected

Placebo or a selected comparator

- Real world

Effectiveness

How does it work in medical practice

Patients as they come

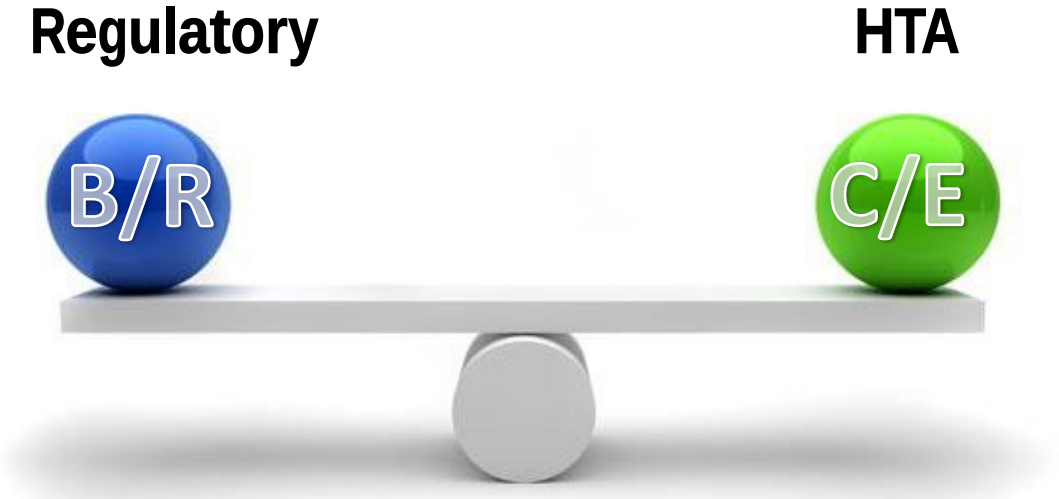
Many alternative treatments

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



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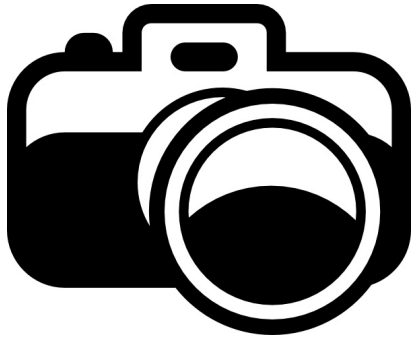
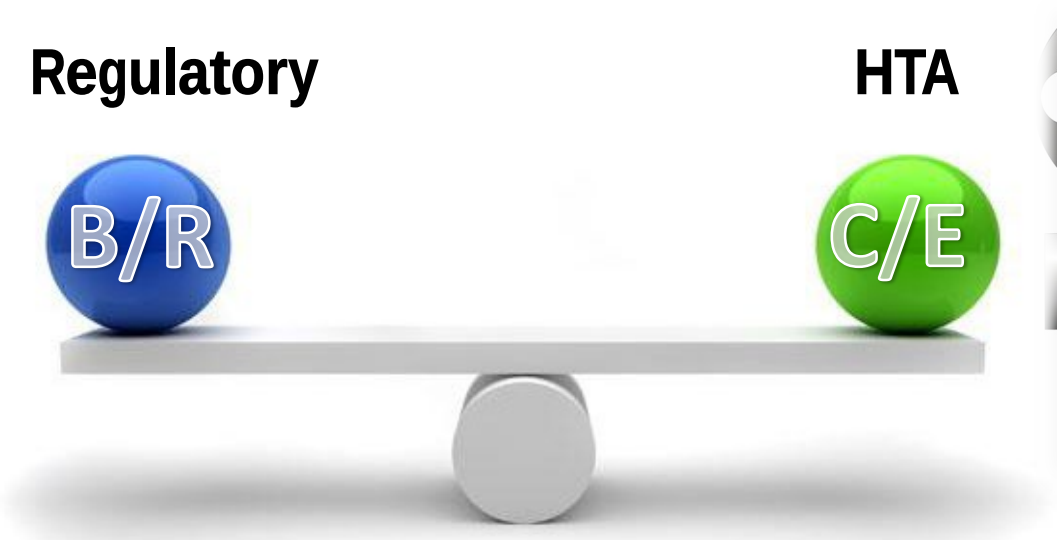
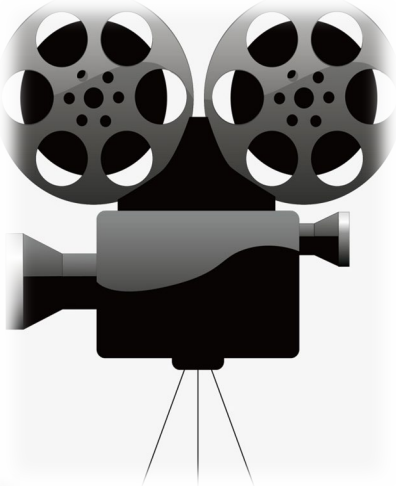
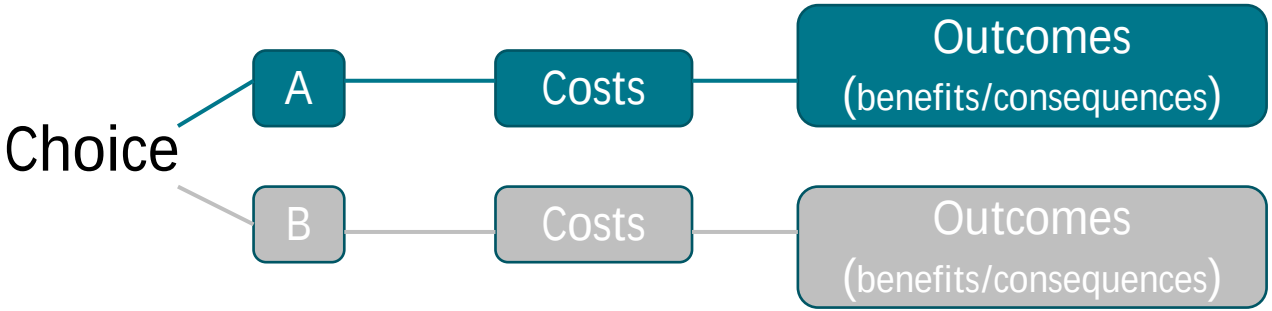






HTA: the basics

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



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

Economic evaluation
'the **comparative analysis** of alternative courses of action in terms of both their **costs** and **consequences**'
(Drummond McGuire, 2001)

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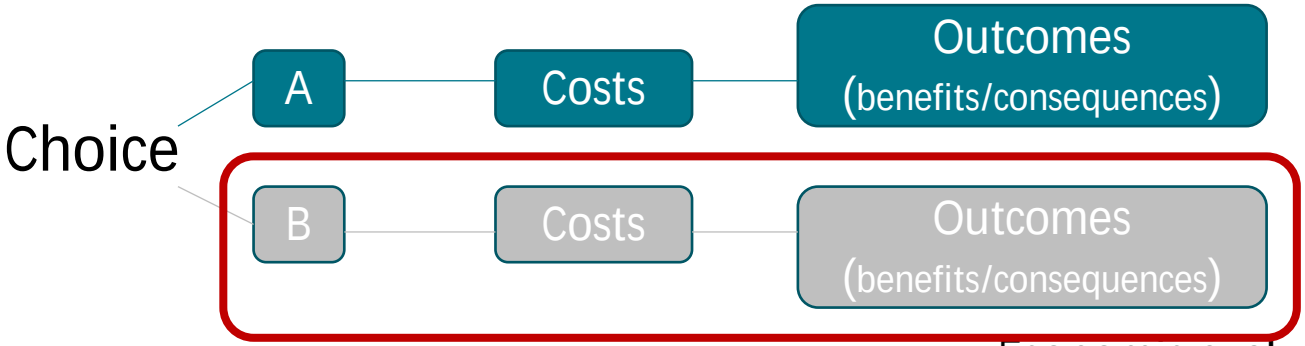

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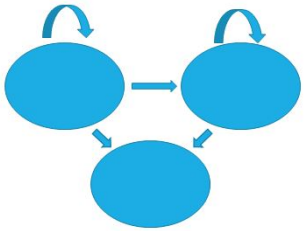
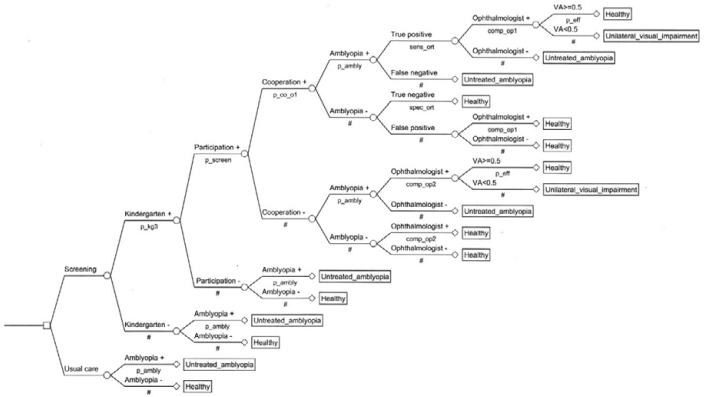
Data, we need data.....

- All HTA agencies need robust comparative (randomized) data
- Cost utility analyses (CUA) require even better data
  - To run a lifetime horizon model extrapolations is almost always required
  - Transition probabilities between health states must be informed by enough data





RWE Intensifying Across Product Lifecycle

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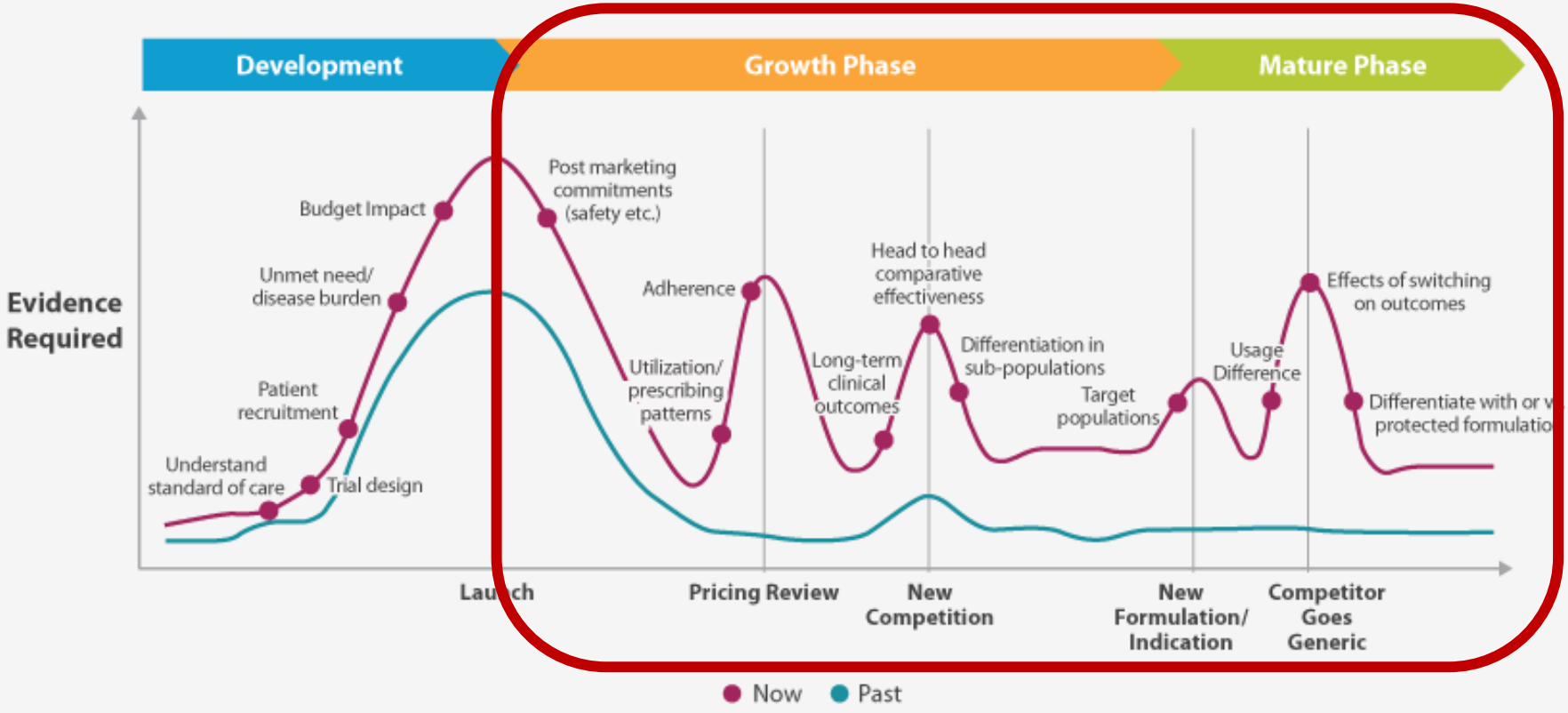

Guidance

- NICE, TSD17 (The use of observational data to inform estimates of treatment effectiveness in technology appraisal: Methods for comparative individual patient data)
- Institute of Health economics Alberta
- FDA
  - Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices; Guidance for Industry and Food and Drug Administration Staff.
  - Use of Electronic Health Record Data in Clinical Investigations Guidance for Industry
- National HTA agencies

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