On market approval and market access: breaking the linear thinking on how to innovate in a crowded space?

Discussant - Karen Facey

k.facey@btinternet.com

BBS 15 July 2022

Disclaimer

Healthcare Improvement Scotland | SHTG Advice on health technologies

Technoleg Iechyd Cymru Health Technology Wales

RWE4Decisions REAL WORLD EVIDENCE

IMPACT HTA

WP10 Appraisal Framework Suitable for Rare Disease Treatments

https://www.impact-hta.eu/work-package-10

All views my own

1. How can we break the linear thinking relating to regulatory approval and market access?

Hurdles of Authorisation and Access

European Marketing Authorisation

Health Technology Assessment

Health Technology Assessment (HTA)

HTA is a multidisciplinary process* that uses explicit methods to determine the **value** of using a health technology at different points in its lifecycle.

The purpose is to inform decision-making to promote an equitable, efficient and high-quality health system.

*The process is formal, systematic, and transparent,
and uses state-of-the-art methods to consider the best available evidence.

O'Rourke B, Oortwijn W, Schuller T, the International Joint Task Group (2020). The new definition of health technology assessment: A milestone in international collaboration. International Journal of Technology Assessment in Health Care 1–4.

Value in HTA

The dimensions of value for a health technology may be assessed by examining the intended and unintended consequences of using a health technology compared to existing alternatives.

These dimensions often include

- **clinical effectiveness and safety**
- ***costs and economic implications***
- *ethical, social, cultural and legal issues*
- ***organisational and environmental aspects***
- ***as well as wider implications for the patient, relatives caregivers & the population.***

The overall value may vary depending on the perspective taken, the stakeholders involved, and the decision context.

O'Rourke B, Oortwijn W, Schuller T, the International Joint Task Group (2020). The new definition of health technology assessment: A milestone in international collaboration. International Journal of Technology Assessment in Health Care 1–4.

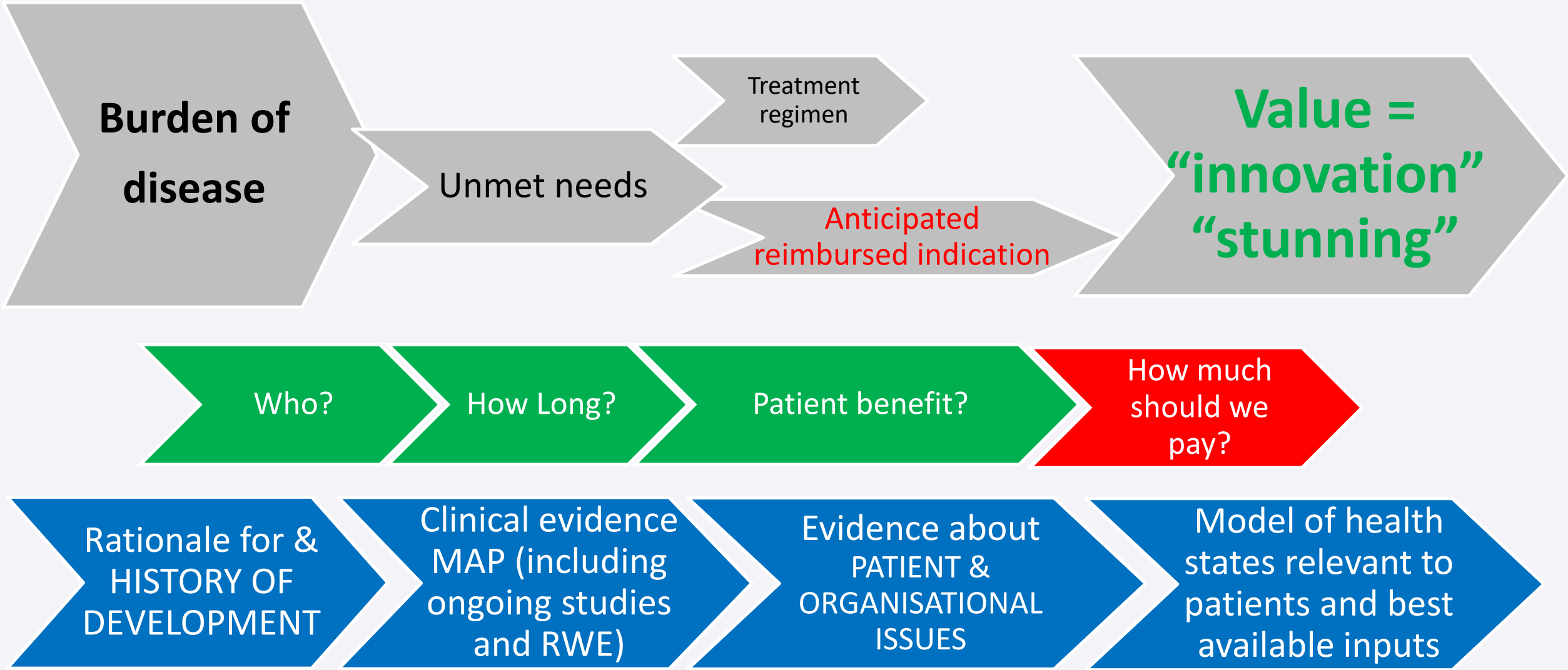
HTA Submissions

- Health condition (epi… …ression and care pathway)
- The technology (ratio… …egimen, reimbursed indication)
- Clinical effectiveness
  - Dir… …
  - Indirect
- Cost utility
  - Budget impact

Population
Intervention
Comparator
Outcomes

- *Expert inputs to provide national context and discuss best assumptions*
- *Appraisal deliberation*

Global Value Dossiers – Siloes not linear exposition of value



1. How can we break the linear thinking relating to regulatory approval and market access?

Upfront planning of evidence generation to demonstrate value

- Clinical trials that take account of the needs of different markets ("the sweet spot of the PICOs")
- Early development of construct of economic model and discussion at scoping meetings (or before)
- Iterative revision of evidence generation plan to demonstrate all aspects of value
  - scientific advancements (e.g. for endpoints)
  - results from previous trials and competitors
  - deliberately planned and well conducted RWE studies that address potential HTA/appraisal "uncertainties" (not investigator interests)
- Pre-competitive collaborations in disease areas to develop outcomes, inputs and models suitable for HTA

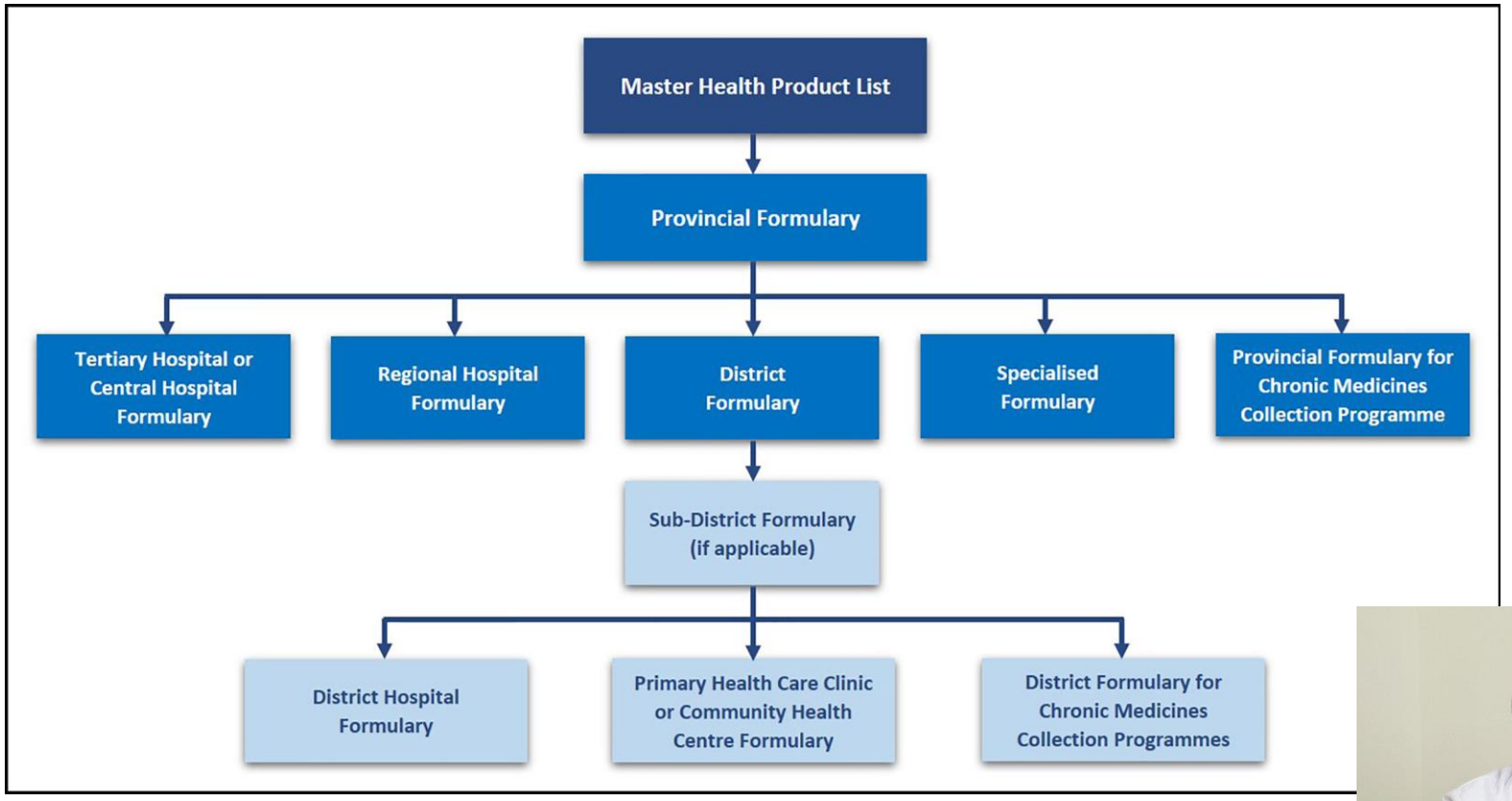
HERCULES

Hurdles? of Authorisation and Access

European Marketing Authorisation

Health Technology Assessment

But access depends on…

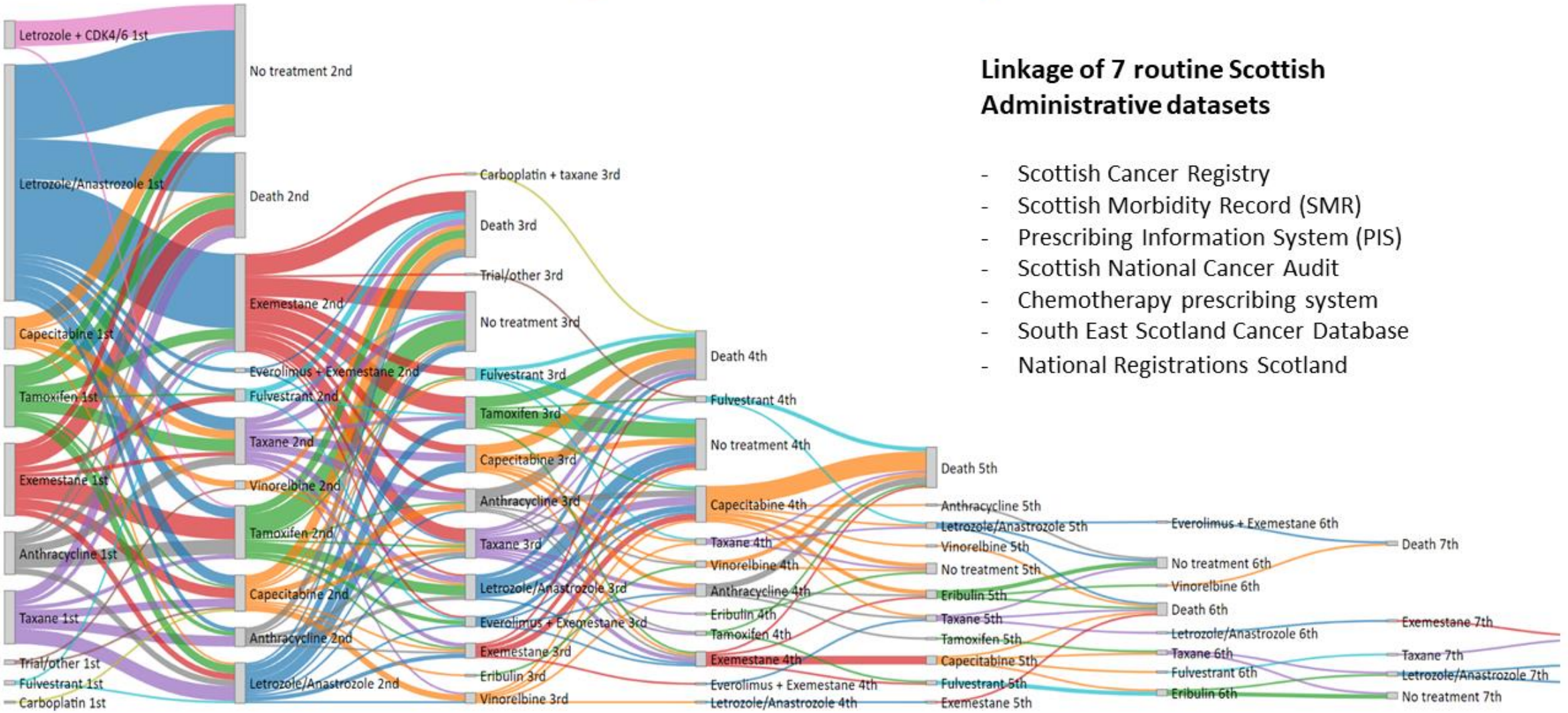


2. How can we further innovate in a space crowded with many drugs?

Real World Treatment Sequencing Patterns in Secondary Breast Cancer (ER+ HER2–)

Pathway Visualisation Using National Datasets

Edinburgh Cancer Informatics Programme

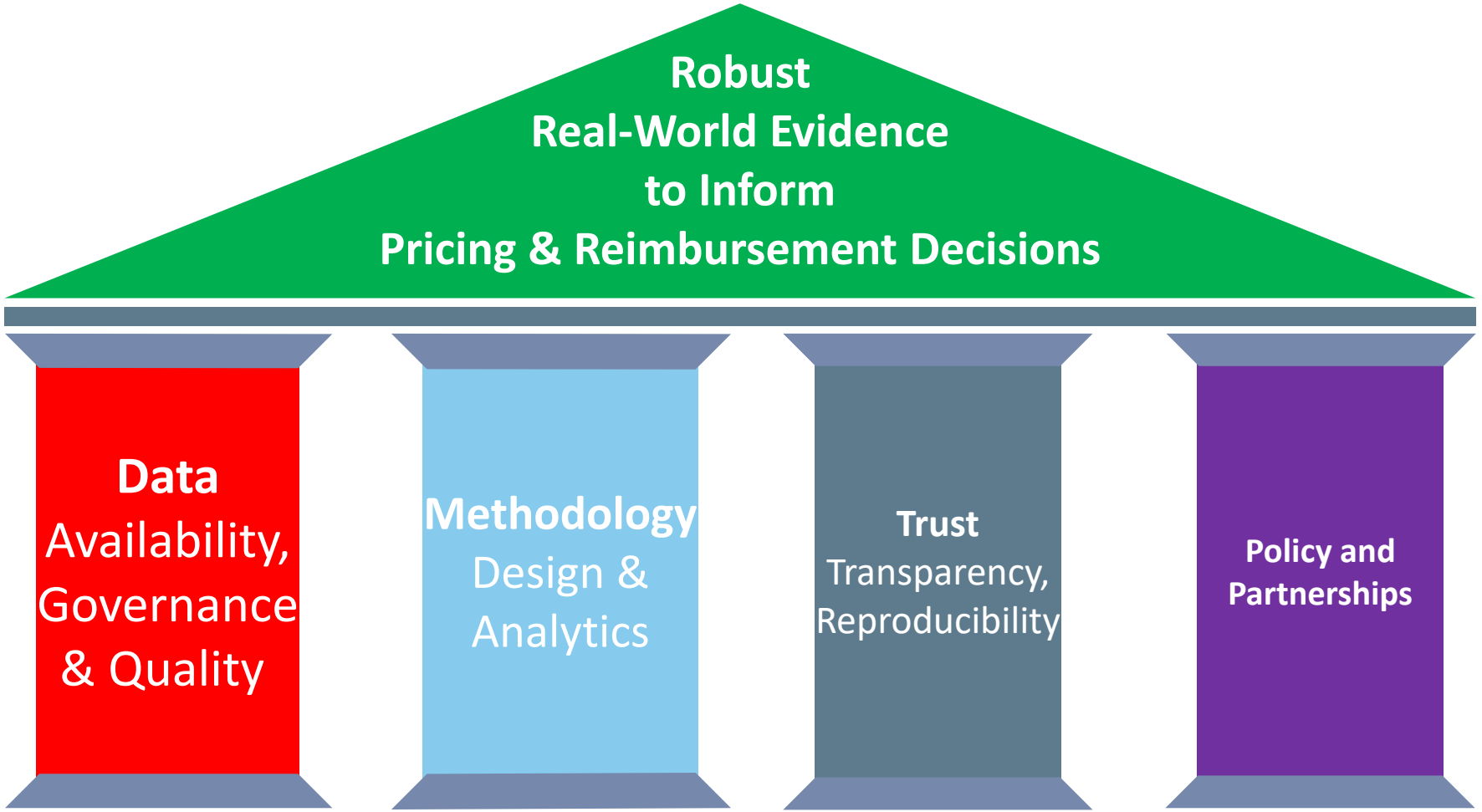


Linkage of 7 routine Scottish Administrative datasets

- Scottish Cancer Registry
- Scottish Morbidity Record (SMR)
- Prescribing Information System (PIS)
- Scottish National Cancer Audit
- Chemotherapy prescribing system
- South East Scotland Cancer Database
- National Registrations Scotland

Horne A et al. ESMO Poster, 2019 https://blogs.ed.ac.uk/cancer-data/2019/10/26/treatment-sequencing-in-metastatic-breast-cancer/

Review of reviews - RWE uptake by HTA/Payers in EU and N. America
4 Pillars to support development of robust RWE for decision-makers
Capkun et al. Submitted to Int J Tech Assess Health Care 2022



+ Management support to provide resources and upskill

NOVARTIS

RWE4Decisions

HTA Body Initiatives
(ATMPs, rare diseases, personalised medicine potential for limited use of outcomes-based agreements)

Zorginstituut Nederlands
- Unambiguous and reliable information sharing (interoperability, data strategy, innovation)
- Review of 4 clinical registries for collection of longer term effectiveness data

National Institute for Health and Care Excellence, England
- RWE Framework
- More stringent evaluation of feasibility of Managed Access Agreements
- Partnership with NHS Digital for collection of data from wide range of NHS sources

CADTH, Canada
- Academic expertise in clinical registries – linking to HTA and treatment optimization?

2. How can we further innovate in a space crowded with many drugs?

- Focus on areas of unmet need!
- Develop partnerships to develop RWE that helps determine treatment optimization that go beyond outcomes-based agreements

Thus supporting health system sustainability