

Evidence for decision making: An update from NICE

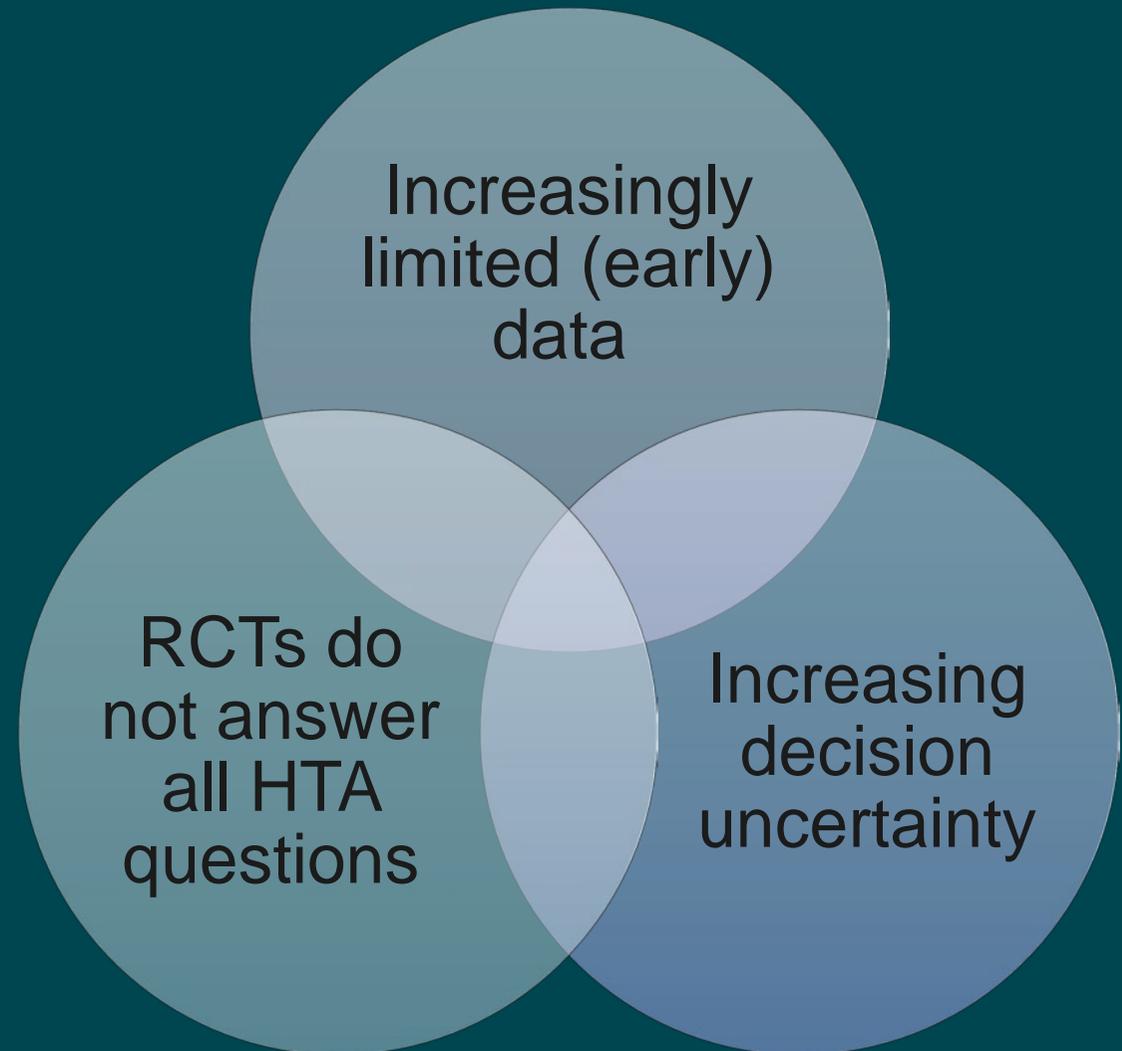
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National Academies of Sciences, Engineering, and Medicine, 17 July 2018

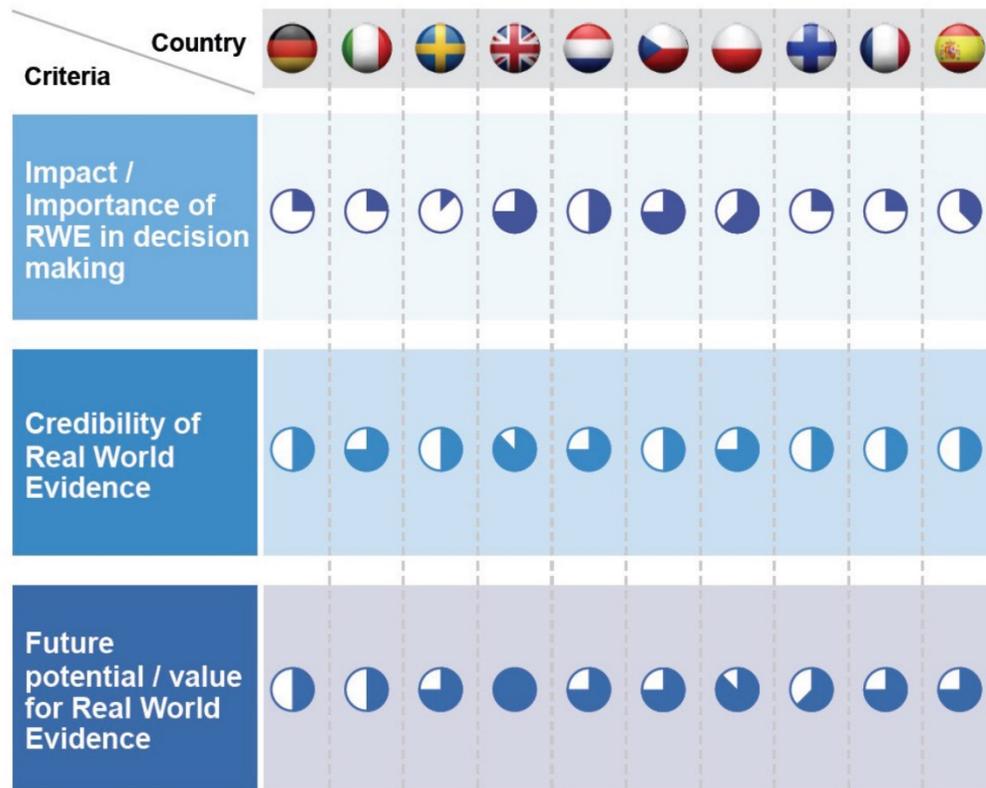
Health technology assessment (HTA) has a problem!



RWE has a role, but issues with data quality, robustness of study results and limited/varied acceptance by different HTA bodies.



Acceptability of RWE varies across Europe



Gill, J. *et al.* 2016. "The use of Real World Evidence in the European context". DOI: 10.21953/LSE.68442

Low ○ - ● High

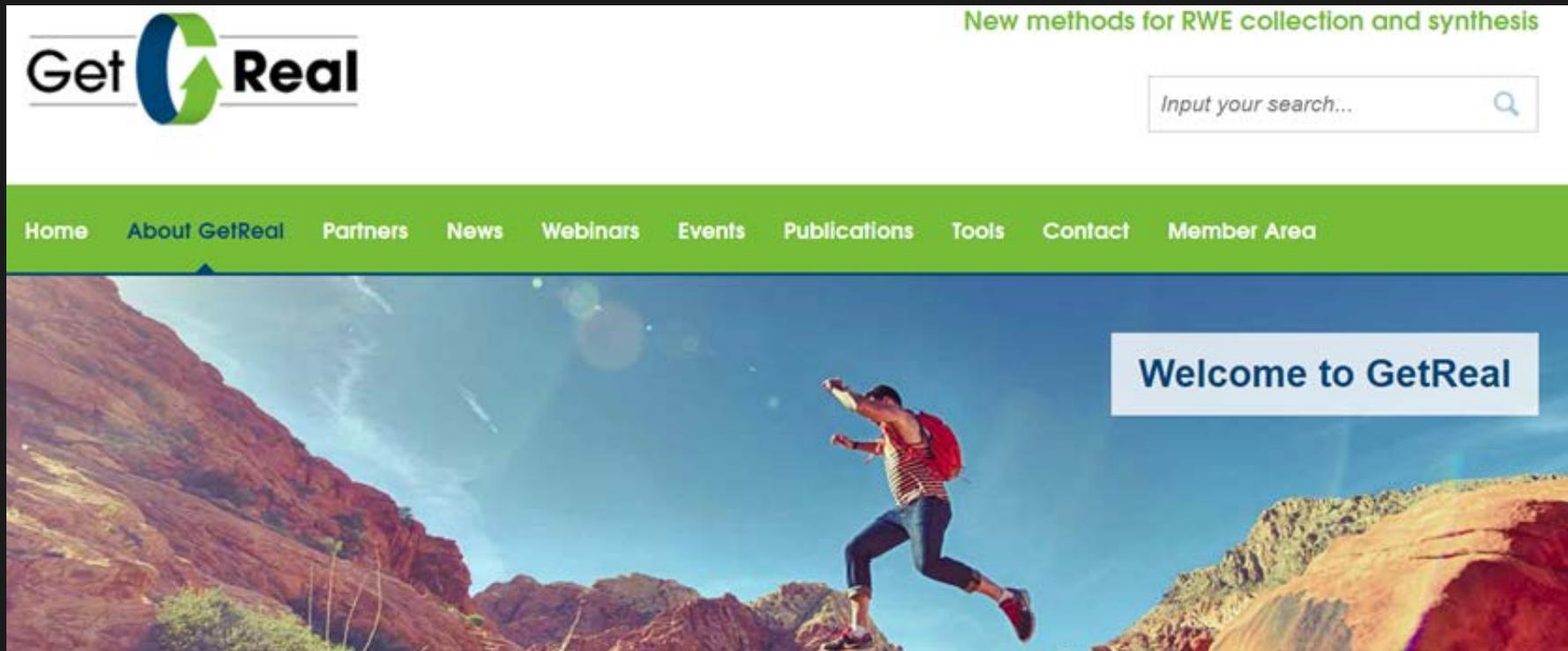
Some disadvantages of RWD in HTA

- Limited availability of RWD at time of assessment
- Potential for bias (e.g. selection, information, confounding bias)
- Poor quality (e.g. incomplete or missing data)
- Data sources not established for research purposes (e.g. EHR, claims databases)

Makady A, Goettsch W. *et al.* 2015 "Review of Policies And Perspectives on Real-World Data for Drug Development and Assessment" IMI GetReal Deliverable. Available at www.imi-getreal.eu.

IMI GetReal: RWE in effectiveness research and decision making

www.imi-getreal.eu rwe-navigator.eu



GetReal's aim was to help demonstrate how new methods of RWE collection and synthesis could be developed and considered for adoption earlier in pharmaceutical R&D and the healthcare decision making process.

NICE It brought together stakeholders to work together to identify opportunities for tackling issues with current evidence generation (e.g. cost, timeliness, fitness-for-purpose...)

GetReal policy recommendations



- 1) **Integrity, quality, access and privacy** protection of RWD sources
- 2) Guidance on RWE study **design, evidence synthesis and interpretation** in decision making
- 4) RWE **training and education**
- 5) Broader **involvement** of stakeholders in RWE generation and use of RWD
- 6) Emphasis on a **joint scientific advice** process (regulatory/HTA/ payer)
- 7) Construction of a RWE **forum** and **linking** with ongoing initiatives

The new IMI GetReal Initiative



- ❖ Research community
- ❖ Think tank
- ❖ Task forces
- ❖ Tools (NMA, pragmatic trials, methods, RWE Navigator)
- ❖ Education and training
- ❖ Dissemination (webinar, conferences, publications)

A self-funding entity that will:

Continue to drive international consensus and use of RWE in decision making

Continue to provide the tools that are required to deliver high quality RWE

Continue to provide the education and training required to generate and use RWE

Think tank: vision

“The Think Tank will consist of international thought leaders and will discuss, assesses and give recommendations on the opportunities and barriers to the generation, use and acceptability of RWE. They will act as ambassadors for the use of RWE during the project and beyond, engaging with key stakeholder groups to drive policy debate and facilitate the uptake of the outputs of IMI GetReal and the GetReal Initiative.”



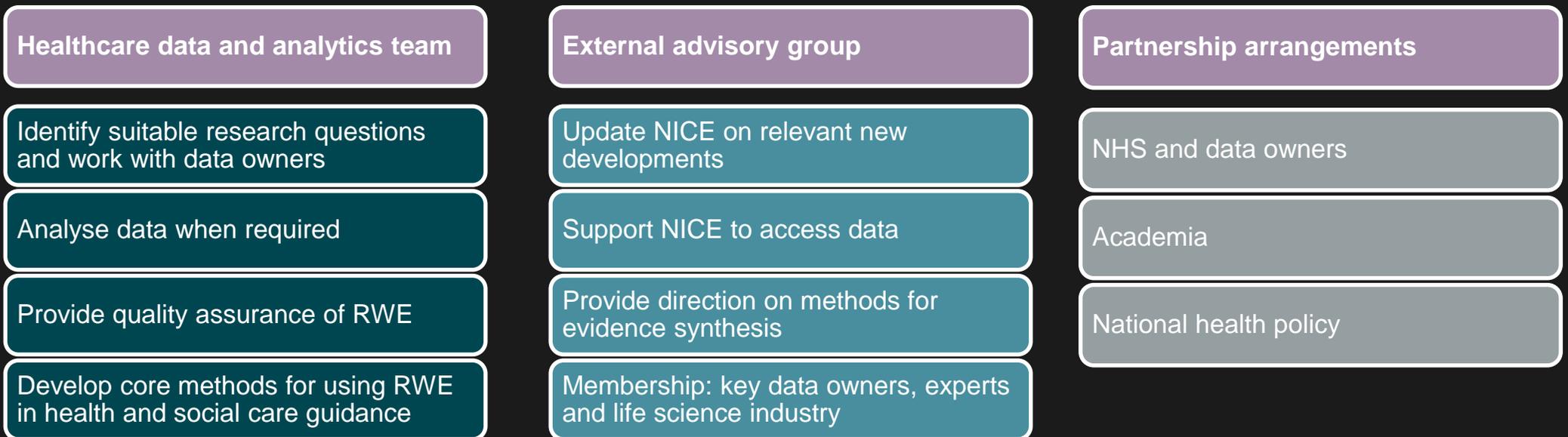
RWE and NICE

How is the journey so far influencing our work?

The NICE portfolio in 2018



Example 1: Future cross-NICE RWE functions and capabilities



Example 2: Updated guidelines manual*

Learnings from the GetReal project are being incorporated in an updated version**

Describes a range of possible situations in which RWD can be used in the context of NICE guidelines

Is not intended to be exhaustive, but to encourage guideline developers to consider whether analysis of RWD could be used to support decision making.

Consider RWE:

- To address efficacy/effectiveness gap
- To demonstrate comparative effectiveness
- To extrapolate treatment effect beyond duration of clinical trials
- For impact analysis

Example 3: Focus on methods development

NICE's **Science Policy and Research** is prioritising areas for methods development

Working with **national research funding bodies** to help fund academic work:

- Medical Research Council's 'highlight notice' on RWE for decision making

Engaging in **research projects directly** with partners in selected areas, such as:

- Develop best practice for applying adjustment methods to RWD to control for confounding (e.g. propensity score methods, instrumental variables etc.)
- Outcome definition in RWD and big data
- Use of 'big data' in healthcare decision making
- Use of advanced analytics, including AI in guideline development



Strategic vision for methods development: **to maintain our status as world-leader in the development of high-quality evidence based guidance**