NICE methodologies and real-world data

Use of real-world data and evidence in the context of the 2020 NICE methods review

Topics for today

- Decision makers' views on real-world evidence in assessments
- NICE and real-world evidence
- What the 2020 review says

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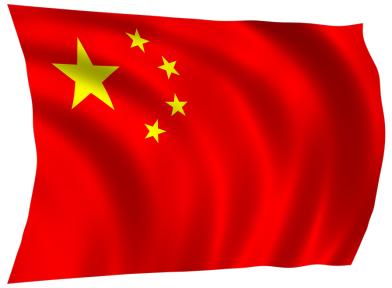
Decision makers' views on real-world evidence have become more open over time



Interest in RWE growing around the world:

- In 2018, the FDA announced it would investigate:
  - "…the potential use of RWE to support changes to labelling about drug product effectiveness"
- 2019 ICER value assessment framework update:
  - "we reaffirm our ongoing commitment to use existing real-world evidence (RWE) and commit to exploring new collaborative relationships with organizations to generate RWE that can complement published data sources"
- In 2019 Health Canada announced guidelines for RWE:
  - "The use of RWE in regulatory decision making continues to be of importance to Health Canada… High quality data collection should be present, agnostic of the study design."
- China's Centre for Drug Evaluation have launched a consultation into RWE

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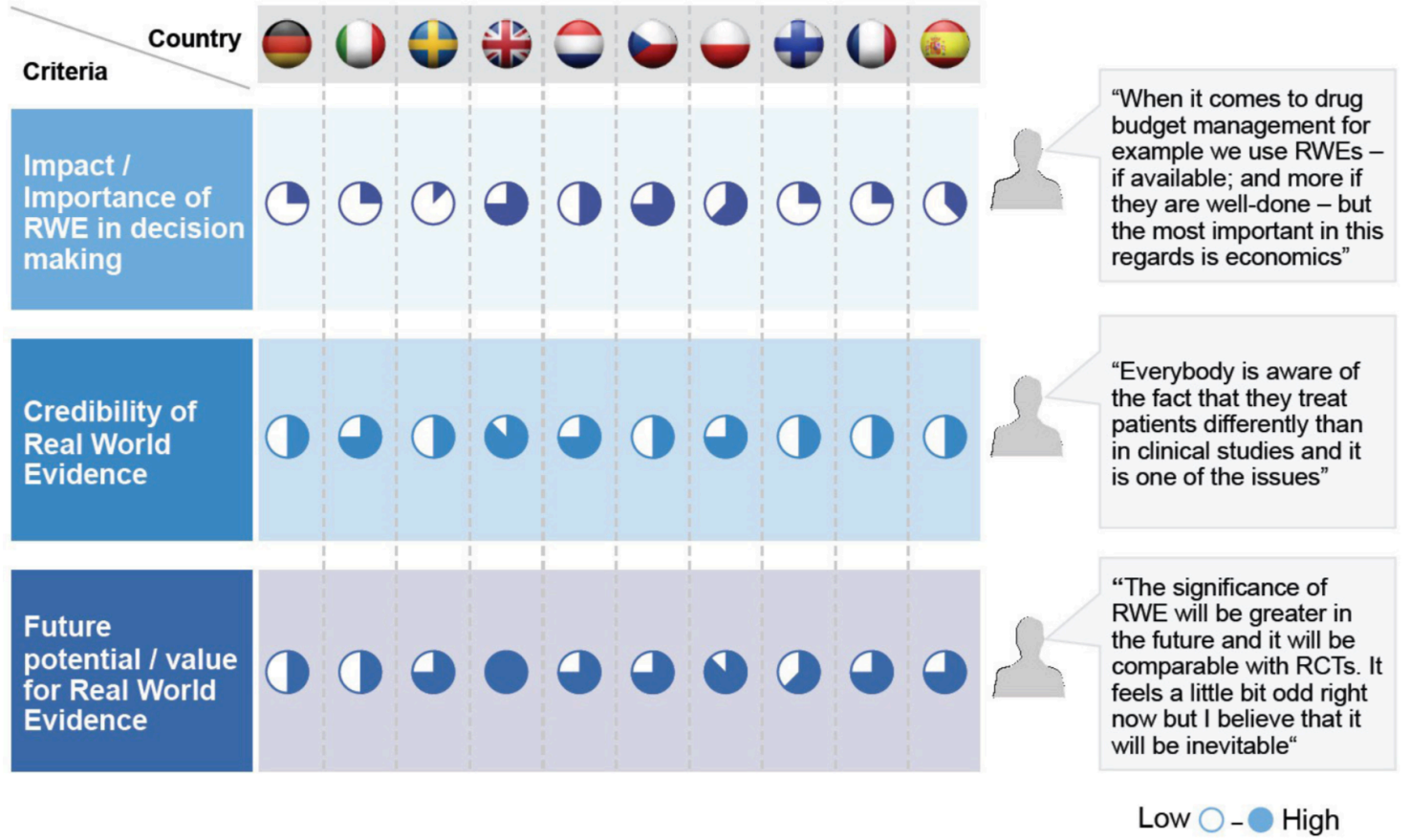
Expert opinion points towards increasing use and benefit of RWE over time

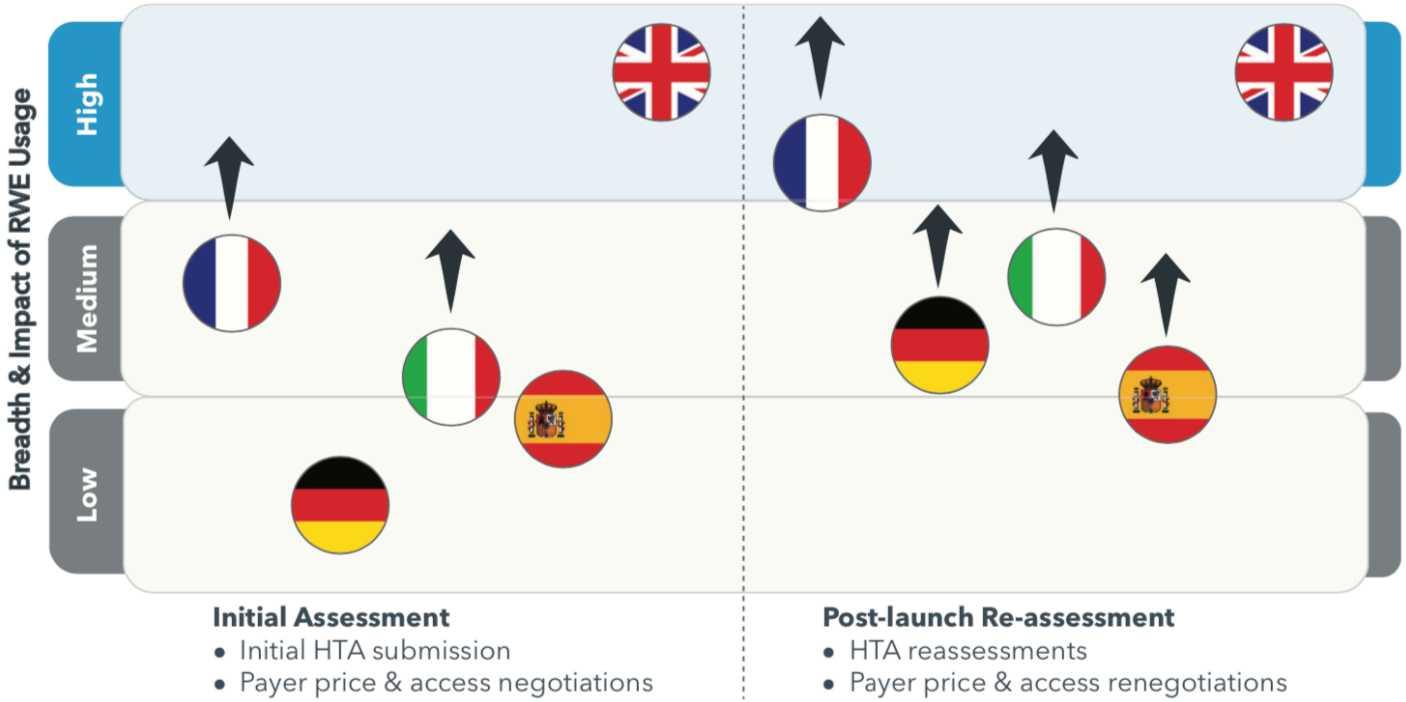
- "The benefit of RWE is becoming more prominent, but the landscape is currently fragmented"



Gill *et al.*, 2016, The use of Real World Evidence in the European context: An analysis of key expert opinion

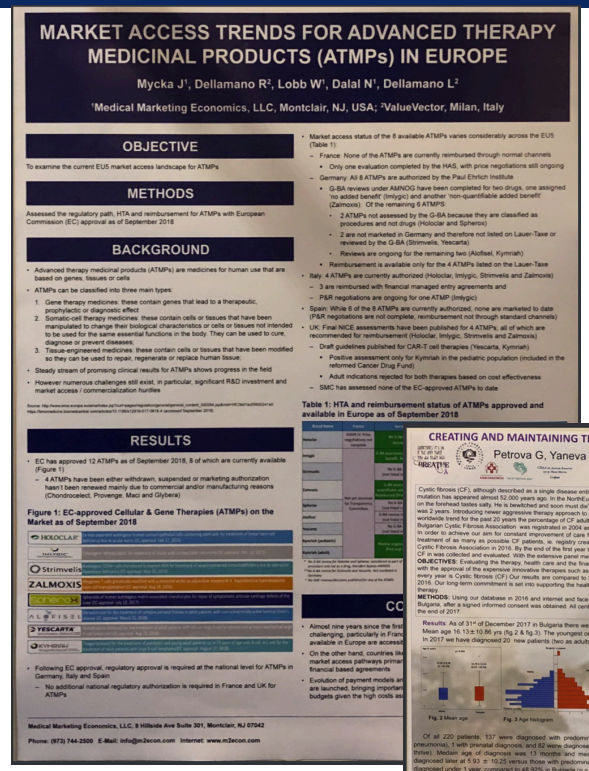
Expert opinion points towards increasing use and benefit of RWE over time (2)

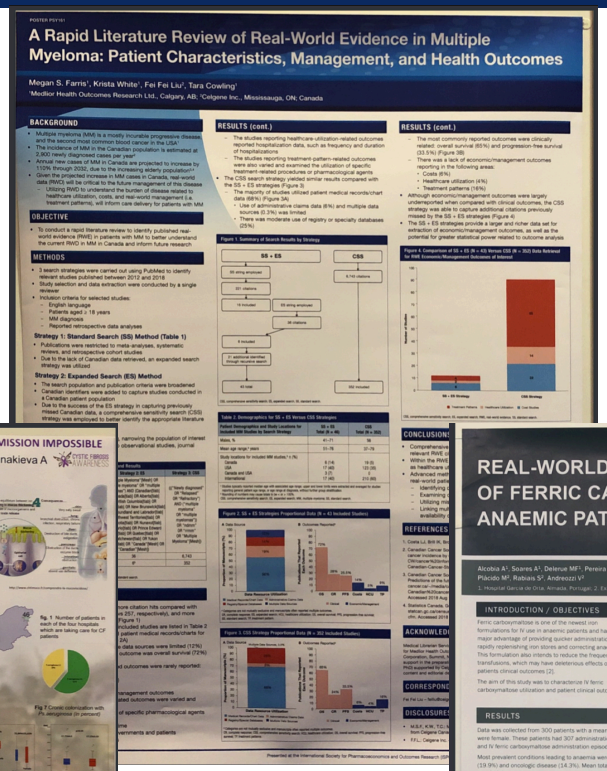
- "In 2017, only 12% of initial submissions to NICE that included RWE received a negative recommendation vs. 23% for those without."

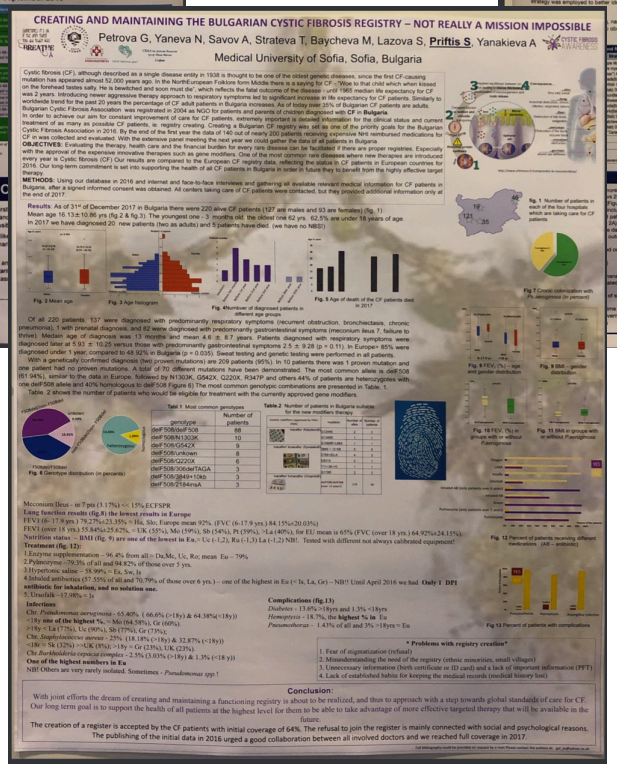


IQVIA's 2018 white paper *RWE: FROM "NICE TO HAVE" TO "MUST HAVE"*

Registry-based and observational studies are well used by industry already







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NICE has been discussing RWE for years, and has engaged widely to explore it as an option

2015	2016	2017	2018-19
Commissioning through Evaluation – promising products needing more evidence	EMA Adaptive Pathways pilot	Using RWE in decision-making	Aug 2017 – Aug 2018, 15.8% with MEAs (n=101)
	Public discussions of pros and cons of RWE: *"can be a useful addition"*	• Decision-making framework, case-studies, trial design	Aug 2018 – Aug 2019, 22.0% with MEAs (n=59) **All** recommended, CDF, or optimised

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RWE has been highlighted in two specific topics of the NICE methods review

- Topic 2: Exploring uncertainty
- Topic 3: Types of evidence (sources and synthesis)

From: https://www.nice.org.uk/news/article/nice-announces-details-of-health-technology-evaluation-methods-review

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Topic 2 poses questions RWE could answer, given the right system

Topic 2 asks: how should NICE quantify and compare uncertainty?

- Uncertainty arises from:
  - **Extrapolation** when making long-term claims from short-term data
  - Choice of/assumptions in analytical methods
  - Justifying the use of **real-world evidence**
- MEAs, CDF: collection of RWE to mitigate uncertainty **under current rules**
- If uncertainty is quantified, would this become mandatory at some threshold?

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Topic 3 may answer some key industry requirements around acceptable evidence

Topic 3 will explicitly determine if randomised, non-randomised, and real-world evidence (RWE) types are considered appropriately.

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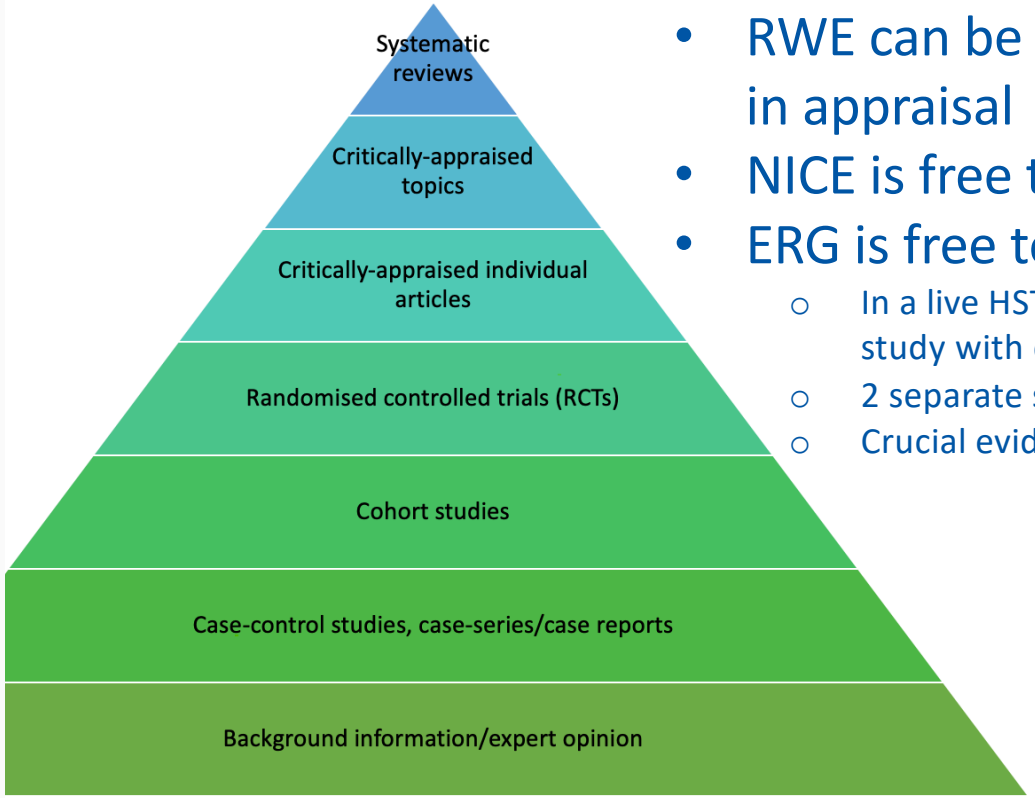

- Not all evidence is objectively equal
- RWE can be the deciding evidence in appraisal
- NICE is free to make a judgment
- ERG is free to make a judgment
  - In a live HST, ERG has replaced pivotal Ph III study with other data in the model
  - 2 separate single-arm trials
  - Crucial evidence is RWE from US-based study

Q&A

Nermina Ferizović

Consultant Health Economics and Outcomes Research and Real-world Evidence

Nermina is a health economist specialising in the generation of evidence to highlight both the health benefits and the wider impact of health interventions. Nermina's role focusses on two key areas of health economics and outcomes research: the assessment of clinical and cost-effectiveness of interventions and real-world evidence generation using worldwide secondary real-world data sources. Nermina designs pharmacoepidemiologic and pharmacoeconomic studies and conducts patient-level data and statistical analyses in R and STATA. In addition, Nermina develops economic models in a wide range of disease areas meaning that a range of modelling techniques are applied including decision trees, Markov models, patient simulation models and epidemiological models. Further, Nermina contributes to the authorship of health technology assessment submissions for worldwide regulatory bodies.

Nermina holds an MSc in Health Economics and Health Policy from The University of Birmingham and is a member of the Royal Economic Society.

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EMIG Market Access Group 5th September 2019

EMIG Ethical Medicines Industry Group