

External challenges for the acceptance of RWE

Dr Richard White
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Efficacy (RCT) vs effectiveness (RWE): An analogy



Standing quarter mile
12.5 seconds



Standing quarter mile
> 12.5 seconds!

External challenges for RWE – trust in the methodology, and trust in the pharma industry

Editorial

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Building trust in real-world evidence and comparative effectiveness research: the need for transparency

“The demand for real-world evidence will continue to increase, but work needs to be done to break down the current barriers to acceptance of real-world evidence in influencing healthcare decision-making.”

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Keywords: observational • patient outcomes • policy • real-world data

Real-world evidence (RWE) – defined by the International Society for Pharmacoeconomics and Outcomes Research as “data used for clinical, coverage and payment decision-making that are not collected in conventional randomized controlled trials (RCTs)” [1] – is increasingly required by regulators, payers

and CER studies in particular, are therefore at risk of significant bias if outcomes with different treatment options are compared without adjustment for confounders such as patient demographics, disease severity, comorbidities and concomitant medications. Selection bias is almost inevitable in RWE,



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Challenge 1 – ‘You haven’t randomized, so you’re not comparing apples with apples’



Standing quarter mile
16.2 seconds



Standing quarter mile
21.6 seconds



Standing quarter mile
12.5 seconds

Challenge 2 – ‘Industry designs their studies to give them the results they want to see’



Standing quarter mile
19.5 seconds



Standing quarter mile
21.6 seconds

Challenge 3 – ‘Industry does lots of studies but only publishes the ones that give the best results’



External challenges for RWE: *Prescrire* comments on the HMA-EMA Joint Big Data Taskforce (1/2)



Paris, 9 April 2019

Response to a public consultation

Prescrire comments

on the core recommendations of the summary report of the HMA-EMA Joint Big Data Taskforce

We would like to thank the HMA-EMA Joint Big Data Taskforce for having opened a public consultation on their summary report outlining a set of recommendations and associated actions on what needs to be addressed.

External challenges for RWE: *Prescrire* comments on the HMA-EMA Joint Big Data Taskforce (2/2)

‘We strongly invite EMA and HMA to consider **very carefully the source and quality** of “big data” and their internal validity and utility...’

‘They should **address the major methodological challenge of bias** in real life data, that historically led to the development of RCT...’

‘We urge European and national regulators **not to weaken marketing authorization requirements** by shifting the provision of evidence to real world data...’

‘Recommendations should be based on robust evidence generated through **independent rigorous evaluation free from vested financial conflicts of interests**’

Building trust in RWE: A task for all of us

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‘With access to potentially life-enhancing new treatments at stake, we owe it to patients to act together to build trust in RWE through greater transparency.’

Thank you!