

THE VALUE OF REAL-WORLD EVIDENCE

RWD and **RWE**

Real-world data (RWD) are information relating to patient health status and/or the delivery of healthcare collected outside of randomized clinical trials (RCTs)^{1,2}



Real-world evidence (RWE) is the clinical evidence regarding usage of a medical product derived from analyzing RWD¹

RWE complements and supplements RCT data^{2,3} RCTs and RWE answer different questions



RCTs: Can the drug work?

RCTs provide information on the efficacy of a drug in a controlled setting

- Designed to show causality
- Patients are randomly assigned to treatment or comparator
- Selected patient population with strict inclusion and exclusion criteria
- Protocol-driven treatment; highly monitored, controlled environment
- Data derived from prespecified, protocol-defined, uniformly assessed endpoints

RWE: Does the drug work?

RWE provides information on the effectiveness of the drug in the everyday clinic setting

- Unable to determine causality
- Patients are not randomized
- Diverse and unselected patient populations that can result in varied outcomes
- Routine clinical practice
- Data derived from assessments of clinical judgment, with variability in patient care and adherence

RWE complements and supplements RCT data³⁻⁵ What information do they provide?

Information provided	Controlled setting	Real-world practice
Clinical outcomes	(efficacy)	<pre>(effectiveness)</pre>
Safety	\checkmark	\checkmark
Quality of life (QoL) and patient-reported outcomes	\checkmark	\checkmark
Practice patterns and treatment adherence		\checkmark
Information on broader patient populations		\checkmark
Healthcare resource utilization (HRU)		\checkmark

Combination of data derived from RCTs and RWE provides a more complete picture of a therapeutic intervention



Improved

- Clinical practice
- Healthcare decisions
- Patient management and outcomes



Increased acceptance of RWE in the healthcare field

Healthcare stakeholders require an increasing amount of evidence to address their key needs. This information can be generated from RWD, leading to increased prominence and acceptance of RWE in the healthcare field⁵⁻¹¹

	NEE	DS	RWE CONSIDERED
Regu agend	latory • Asse cies • Post	ess risk-benefit ratio -market surveillance	 Effectiveness Safety QoL
Payer Techr Asses	rs / Health nology • Cost asse	:-effectiveness essment	 Effectiveness Safety QoL HRU
Healt provi	 Optin Stra Stra Subp Undeprefer 	mize care protocol tify outcomes by oopulations erstand patient erences	 Effectiveness Safety QoL and patient preference HRU
Patie	Optin Rece pers or ne	mize personal health eive care in line with onal preference eed	 Effectiveness Safety QoL and patient preference

1. FDA. https://www.fda.gov/media/99447/download [Last accessed 13 July 2020]; 2. Khozin S et al. *J Natl Cancer Inst.* 2017;109(11). doi:1.1093/jnci/djx187; 3. Singal AG, et al. *Clin Transl Gastroenterol.* 2014;5.e45. doi:10.1038/ctg.2013.13; 4. The Association of the British Pharmaceutical Industry. https://www.abpi.org.uk/media/1591/2011-06-13-abpi-guidance-demonstrating-value-with-real-world-data.pdf; 5. Berger M et al. *Value Health* 2017;20:1003-1008; 6. Nordon C et al. *Value Health* 2016;19:75-81; 7. Pietri G & Masoura P. *Value Health* 2014;17:A450-451; 8. Pobiruchin M et al. *J Biomed Inform* 2016;60:385-394; 9. Khozin S et al. *J Att Cancer Inst.* 2017;109:10: 10. Shore C et al. eds. Examining the Impact of Real-World Evidence on Medicat Product Development: Proceedings of a Workshop Series. Washington (DC): National Academies Press (US); 2019. Available from: https://www.ncbi.nlm.nih.gov/books/NBK540105/ [Last accessed 3 July 2020]; 11. Khosla S et al. F1000Res 2018;7:111.



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