Takeda | ONCOLOGY

WHAT IS REAL-WORLD EVIDENCE?

Analysis of real-world data (RWD) produces real-world evidence (RWE). RWE is obtained from analyses conducted outside the context of randomized controlled trials (RCTs). It provides insights into how patients respond to treatments in their everyday lives.

RCTs are the gold standard for evaluating the safety and efficacy of a treatment component of a treatment's path to regulatory approval.[1] By capturing information from a wider range of patients in different settings, RWE can generate additional data, including critical information on patients excluded from RCTs, to help optimize patient care and provide complementary insight into the impact of treatments.

WHAT IS RWE AND HOW IS IT USED

RWE is used in various ways by different stakeholders in the healthcare community:

- Regulatory bodies use RWE to monitor post-market safety and adverse events
- Payors use RWE to support coverage decisions
- Physicians and patients use RWE to inform treatment decisions
- Medical product developers use RWE to inform clinical trial designs

BENEFITS AND LIMITATIONS OF RWE

RWE is complex and understanding its benefits and limitations is critical to furthering our knowledge of diseases, treatments and the patient experience.

BENEFITS

Patient Inclusion: The freedom to enroll a broader patient population for RWE studies - not just those that are healthy or local enough to participate in RCTs, but also those with comorbidities and frailties not typically reflected in RCTs - allows for generation of data in a more representative population.

Patient-Focused Findings: RWE captures the reality of how a treatment can impact everyday lives, allowing patients to be involved in their treatment and provide direct input.

Timely Results: RWE studies can be completed relatively promptly for broad patient populations, which allow data to be available within a shorter timeline compared to RCTs.

LIMITATIONS

Lack of Centralized Methodology: Although regulatory agencies, such as the FDA, have developed a framework for use of RWE as well as a variety of other related guidances, there is currently no centralized standard methodology for RWE.[2]

Increased Variability: RWE can come from a range of sources, which may broaden the variety of study designs and available data, making it difficult to obtain consistent results.

Association, Not Causality: RWE studies often point to an association between variables, not a causal relationship, which may make it difficult to determine the effect of a treatment.[3]

RWE AND MULTIPLE MYELOMA

RWE plays an important role in understanding treatments for multiple myeloma.

13% increase

It is expected that there will be approximately a 13% increase in multiple myeloma cases in the U.S. from 2020-2025.[4]

22% to **72%**

Approximately 22% to 72% of people living with multiple myeloma are ineligible for RCTs due to factors such as age, frailty, comorbidities, organ dysfunction or may lack accessibility to enroll.[5]

RWE can provide greater insight into improving outcomes for multiple myeloma patients by capturing data on underrepresented patient populations.

Takeda is committed to embracing new methods of collecting and analyzing data through a robust RWE program, including the global INSIGHT MM study – the largest prospective, observational study in multiple myeloma to date[*] – as we continue to provide transformative solutions for multiple myeloma patients around the globe.

[*]As of August 2021

References

1. Hariton, E., & Locascio, J. J. (2018). Randomised controlled trials—the gold standard for effectiveness research. *BJOG: An International Journal of Obstetrics and Gynaecology*, 125(13), 1716. Accessed March 5, 2021.
2. Commissioner, O. of the. (2018). FDA Budget Matters: A Cross-Cutting Data Enterprise for Real World Evidence. FDA Voices. https://www.fda.gov/news-events/fda-voices/fda-budget-matters-cross-cutting-data-enterprise-real-world-evidence. Accessed March 5, 2021.
3. Mann CJ. Observational research methods. Research design II: cohort, cross sectional, and case-control studies. *Emergency Medicine Journal*. 20 (2003): 54-60. Accessed March 5, 2021.
4. Richardson, P.G., San Miguel, J.F., Moreau, P. et al. Interpreting clinical trial data in multiple myeloma: translating findings to the real-world setting. Blood Cancer Journal. 8, 109 (2018). Accessed March 5, 2021.
5. Terpos, E., Mikhael, J., Hajek, R. et al. Management of patients with multiple myeloma beyond the clinical-trial setting: understanding the balance between efficacy, safety and tolerability, and quality of life. Blood Cancer J. 11, 40. 2021. 2021. 11(2): 40 Accessed July 22, 2021