Public Policy Statement | July 2026

Real-World Data and Evidence

Real-world evidence (RWE), evidence derived from the analysis of real-world data (RWD) reflecting routine clinical practice[a], has become an established component of the evidence landscape supporting healthcare decision making. Merck believes in the use of RWD and RWE to inform understanding of the clinical and economic value and impact of new health technologies on healthcare systems and the patients they serve. Consistent with this view, Merck supports transparent and responsible use of rigorous, high-quality RWD and RWE as part of the totality of scientific evidence used in regulatory, reimbursement, and healthcare policy decision making. National and professional standards for RWE generation, including study design, methodology, reporting, and the reliability and relevance of real-world data, should be aligned across healthcare authorities and applied consistently to RWE generated by both public and private organizations, informed by the experience and expertise of all stakeholders.

Background

While randomized clinical trials remain the gold standard of scientific evidence regarding the safety and efficacy (performed under controlled circumstances) of new technologies, healthcare decision makers around the world are increasingly utilizing RWE to understand the potential clinical, economic and humanistic impacts of health technologies on patients, and health systems. [a,b,c] Real-world data and real-world evidence (RWD/E) can complement information from traditional clinical trials by providing vital contextual information such as disease burden, natural history of disease, healthcare resource utilization, and in some cases can provide insight into the potential clinical benefits of new treatments. After regulatory approval, RWD may be used to provide information about real-world safety, the clinical and economic impact of new technologies and to assess patient experience data. Further, RWD/E supports a diverse and broad approach to scientific research by helping to characterize unmet medical needs, informing study design, and providing information about patient sub-populations.[b,c]

[a] U.S. Food and Drug Administration. (2018). *Framework for FDA's real-world evidence program*. https://www.fda.gov/media/120060/download

[b] VO TT, Kim S, Jaksa A, Bates B, Zhou W, Stewart MD, Burcu M. Diversity by Design: Real-World Data to Enhance Representation in Clinical Cancer Research. Clin Cancer Res. 2025 Nov 3;31 (21):4396-4044. Doi:10.1158/1078-0432.CCR-25-1255. PMID: 40834138

[c] Segal JB, Varadhan R, Groenwold RHH, Li X, Nomura K, Kaplan S, Ardeshirrouhanifard S, Heyward J, Nyberg F, Burcu M. Assessing Heterogeneity of Treatment Effect in Real-World Data. Ann Intern Med. 2023 Apr;176(4):536-544. doi: 10.7326/M22-1510. Epub 2023 Mar 21. Erratum in: Ann Intern Med. 2023 Jun;176(6):882. doi: 10.7326/L23-0162. PMID: 36940440; PMCID: PMC10273137.

Global momentum around the appropriate use of RWD/E is reflected in guidance issued by various health authorities and health technology assessment bodies (e.g., the U.S. Food and Drug Administration, European Medicines Agency, UK Medicines and Healthcare products Regulatory Agency, UK National Institute for Health and Care Excellence, France's Haute Autorité de Santé, Canada's Drug and Health Technology Agency, and the International Council for Harmonization (ICH)). However, differences in scope, purpose, and evidentiary standards—along with limited transparency regarding the assessment of individual studies—create uncertainty for health technology developers. As access to RWD expands and use of RWE increases, well-defined and consistently applied standards are increasingly important to support trust, transparency, and predictable decision making.[d]

Principles

Merck believes that regulatory, reimbursement, and other healthcare policy decisions should be informed by evidence of the highest possible quality that is relevant to the scientific question being addressed. When appropriate, RWD/E can be a trusted and valuable component of the totality of scientific evidence considered by decision makers.

To support a stable and reliable healthcare ecosystem in which decisions are informed by evidence generated from scientifically rigorous methods and practices, Merck believes that:

- **Common and harmonized standards** across stakeholders are needed to ensure the quality, reliability, and interoperability of RWD sources, as well as the appropriate design and conduct of RWE studies.
- **Fitness for purpose and fitness for use** are primary determinants of study suitability and require that the relevance and reliability of the data, together with methodological rigor sufficient to support credible and interpretable evidence, are appropriate to address a clearly defined scientific question and to inform the intended decision context.[e]
- **Consistent application of standards** for RWD/E, including study design, methodology, data quality, and data sources, should apply equally to evidence generated by public and private organizations and be informed by the experience and expertise of all stakeholders.
- **Reproducibility and auditability** of RWE should be supported by transparent documentation (e.g. study design, data sources, analytical methods and code, assumptions and limitations) that enable appropriate review, consistent with the decision context.
- **Reporting and interpretation** of RWE should be accurate and complete, with findings and conclusions appropriately bound by the study design, including clear communication of

[d] Orsini LS, Berger M, Crown W, Daniel G, Eichler HG, Goettsch W, Graff J, Guerino J, Jonsson P, Lederer NM, Monz B, Mullins CD, Schneeweiss S, Brunt DV, Wang SV, Willke RJ. Improving Transparency to Build Trust in Real-World Secondary Data Studies for Hypothesis Testing-Why, What, and How: Recommendations and a Road Map from the Real-World Evidence Transparency Initiative. Value Health. 2020 Sep;23(9):1128-1136. doi: 10.1016/j.jval.2020.04.002. PMID: 32940229.

[e] U.S. Food and Drug Administration. (2023). *Considerations for the use of real-world data and real-world evidence to support regulatory decision-making for drug and biological products: Guidance for industry*. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-real-world-data-and-real-world-evidence-support-regulatory-decision-making-drug

uncertainty, potential bias, and contextual limitations relevant to the decision being informed.

Merck remains committed to the design and conduct of RWE studies that meet established professional best practice research standards and support the objective of informed decision-making.