Policy Statement | May 2024

# Real-World Data and Evidence

Real-world data[a] (RWD) and real-world evidence[b,1] (RWE) hold potential to help decision-makers understand the clinical and economic value and impact of new health technologies for healthcare systems and the patients they serve. Health authorities (HAs) and health technology assessment (HTA) decision-makers globally are building decision-making frameworks that include RWD/E studies to support a range of decisions across the product lifecycle, including enabling data-driven approaches that can help optimize care delivery. Consistent and globally standardized approaches to methods, quality standards, and guidelines are needed to leverage the potential of RWD/E, fully and appropriately.

Merck supports the consideration and use of rigorous, high-quality RWD/E as part of the totality of scientific evidence in regulatory, reimbursement, and healthcare policy decision-making. We are committed to use of RWD/E in a transparent and responsible way with the aim of improving the evidence base for our innovations to address the needs of patients, healthcare providers, and healthcare systems. We believe that stakeholder standards for RWD/E, including for study design, methodology, data quality and sources should be established and consistently applied to RWE generated by public and private organizations, and informed by the experience and expertise of all stakeholders.

## Background

The growing availability of RWD holds exciting potential for understanding the holistic clinical and economic impact of innovative therapies and vaccines, and their effect on patients, healthcare quality, and healthcare system efficiency and costs. While randomized clinical trials (RCTs) remain the gold standard of scientific evidence regarding the safety and efficacy (performed under controlled circumstances[2]) of therapies and vaccines, healthcare decision-makers around the world are increasingly interested in the potential uses of RWE. RWD/E complements and can augment information from traditional clinical trials, characterizing natural history of disease, rare outcomes, or real-world product experience. Further, RWD/E supports a diverse and broad approach to scientific research, e.g., by informing study design, providing information about sub-populations, and supporting ongoing information gathering about product safety and effectiveness.

[a] Real-world data (RWD) refers to data relating to patient health status and/or the delivery of health care that is routinely collected from a variety of sources, primarily outside the typical research (e.g., randomized control trial) setting. Examples include insurance claims data, electronic medical records, prescription refill data, lab reports, physician notes, biomarkers and genomics data, patient registries, patient surveys, data from patient wearable devices, and social media data.

[b] Real-world evidence (RWE) refers to clinical or economic evidence regarding the use and/or potential benefits or risks of a particular health technology based on the analysis of RWD. RWE is usually generated in the form of a study using RWD to develop insights and inform healthcare decision making.

Global momentum around appropriate use of RWD/E continues, as reflected in various HA and HTA guidance documents (e.g., from the US Food and Drug Administration, the European Medicines Agency, the UK Medicines and Healthcare products Regulatory Agency, the UK National Institute for Health and Care Excellence, France's Haute Autorité de Santé, and Canada's Drug and Health Technology Agency).[3] Industry has used RWD/E in various forms over several decades. Developments in the healthcare environment, including the interest in rapid evidence generation during the COVID-19 pandemic, highlighted the opportunity for RWE to provide valuable, timely insights to policymakers, payers, and providers, as well as the need for global harmonization of RWD/E standards. To more fully realize the potential of RWD/E, there is a need to define and advocate for evidence-based, globally standardized approaches to RWD/E methods, quality standards, and guidelines and their consistent application across the public and private sectors. Harmonized standards are important enablers of stakeholder trust and of a transparent review environment that facilitates industry planning.

## 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 posed. RWD/E should be a trusted and valuable part of the holistic portfolio of scientific evidence considered by decision-makers. Towards this aim, we believe:

- Common and harmonized standards are needed across stakeholders for quality and interoperability of RWD sources, as well as RWE study design and conduct.
- Fitness for purpose – whether data are valid and appropriate to answer the specific question in the given context – should be the critical determinant for assessing appropriateness of a study.
- Standards for RWD/E, including for study design, methodology, data quality and sources should be established and consistently applied to RWE generated by both public and private organizations and informed by the experience and expertise of all stakeholders.

Merck remains committed to the design and development of RWE studies for decision-making that meet best practice research standards.[4]

[1] Framework for FDA's Real-World Evidence Program. December 2018. https://www.fda.gov/media/120060/download
[2] Last JM, ed. A Dictionary of Epidemiology, 4th Edition. New York: Oxford University Press, Dec 2000. ISBN-13: 978-0195141696
[3] Framework for FDA's real-World Evidence Program (2018). MHRA guidance on the use of real-world data in clinical studies to support regulatory decisions (2021). Real-world evidence framework to support EU regulatory decision-making (2023). International Harmonisation of Real-World Evidence Terminology and Convergence of General Principles Regarding Planning and Reporting of Studies Using Real-World Data, with a Focus on Effectiveness of Medicines (draft September 2023).
[4] Acha V, Barefoot B, Juarez Garcia A, Lehner V, Monno R, Sandler S, Spooner A, and Verpillat P. Principles for Good Practice in the Conduct of Non-interventional Studies: The View of Industry Researchers. 2023. Therapeutic Innovation & Regulatory Science **57**(6): 1199-1208.