European Federation of Pharmaceutical Industries and Associations

Harnessing RWE to transform Healthcare Decision-Making in Europe

EFPIA vision on the role and use of RWE in decision-making

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Introduction: Healthcare decision-making is evolving with the integration of Real-World Evidence (RWE). While traditional clinical trials remain crucial, RWE offers **valuable insights from everyday clinical practice. This supports the discovery and development of innovative new medicines, helping to understand how medicines work in the real world**. RWE complements clinical trial data offering a holistic understanding of treatment effectiveness, safety and value in diverse, real-world populations. The availability of digital health tools, electronic health records (EHRs), and advances in data analytics have enabled the large-scale collection and analysis of Real-World Data (RWD). These developments provide opportunities to bridge the gap between research and routine care.

The vision for the future: To fully integrate RWE into healthcare decision-making, **EFPIA envisions a collaborative environment where industry, regulators, healthcare providers, patients, academic researchers, HTA bodies and others work together.**

What are RWD and RWE?

- RWD: Information on patient characteristics, clinical outcomes, and health interventions collected through routine clinical practice. Sources of RWD include registries, claims data, digital health applications, patient-reported outcomes, and social determinants of health, among others. These diverse sources offer a broader context to traditional clinical trial data.
- RWE: Clinical evidence derived by analysing RWD, offering insights into the benefits, risks and value of medicines.

Significance of RWE: RWE has proven its role in accelerating patient access to treatments and vaccines, in a broad range of conditions, from rare diseases to COVID-19 vaccines and more. It has significantly improved understanding of treatment effectiveness and safety so that clinicians prescribe the right medicine to the right patient at the right time.

Opportunities and challenges:

1. Opportunities:
   - Enhanced decision-making: RWE can generate insights and complement clinical trials and product development. It offers novel outcome measures, such as digital endpoints, that fill gaps in knowledge and reduce uncertainties about a product's real-world use and value. For instance, treatments for Spinal Muscular Atrophy (SMA), a severe neuromuscular disorder, relied on RWE for regulatory approvals and reimbursement decisions, bringing life-saving therapies to patients faster.
   - Patient-centric care: As seen during the COVID-19 pandemic, RWE can help tailor patient care by providing insights into long-term effectiveness, safety and patient experiences.

- Innovation in research: It can inform R&D strategies as well as support the development of new therapies, particularly in areas where traditional clinical trials are challenging or unethical e.g., when treating children, pregnant women or patients with rare diseases.

2. Challenges:

- Establishing trust: Ensuring the generation of consistent, relevant and reliable RWE to gain trust from regulators, healthcare providers, HTA bodies and patients.
- Data access and technical infrastructure: Currently, national laws hinder international research and activities. It is anticipated that the implementation of the European Health Data Space could help in addressing some of these challenges by driving greater consistency in the interpretation of the General Data Protection Regulation, greater standardisation of EHR systems, improving healthcare information infrastructure and streamlining data access models.
- Data quality and interoperability: RWD are usually generated and collected for purposes other than research and may be of variable quality. The data must be sufficiently curated, validated and reliable, and its fitness-for-purpose should be assessed for a particular study. In addition, RWD data are stored in many databases cannot readily communicate with each other and in formats that are incompatible. This variety can complicate the analysis of data from multiple healthcare systems and sources.
- Predictability and acceptability: Ensuring consistent acceptance of RWE by regulatory and HTA bodies to streamline its use in decision-making.

<u>Key actions to unlock RWE</u>:

- Enhance data quality: Implement harmonised guidelines for better data quality at source, ensuring that RWE is robust and reliable.
- Foster collaboration: Encourage partnerships across stakeholders to facilitate data access for research purposes, address data quality and interoperability, advance methodologies, and share insights and knowledge.
- Build trust and transparency: Develop clear guidelines for the acceptance of RWE in regulatory and HTA decision making, ensuring transparency in its generation and application.
- International harmonisation: Align RWE approaches globally to streamline regulatory processes, avoid unnecessary additional studies, support development of innovative treatments, and increase usability and acceptability of RWE.

These key actions can be jointly addressed by stakeholders involved in the use of RWD, and in the generation and assessment of RWE for healthcare decision making. Joint initiatives and collaborative efforts such as the Innovative Health Initiative, European Health Data and Evidence Network (EHDEN) Foundation, GetReal Institute, and many others, can provide forums for engagement. Collaboration will foster the development of an improved health information infrastructure. Together, stakeholders can develop a regulatory and HTA framework that facilitates iterative scientific dialogue on evidence generation plans for faster, better decision making.

Conclusion:

RWE is a powerful tool that, when used effectively, can transform healthcare and improve delivery of treatments that are safe, effective and aligned with patients' needs in real-world settings. The successful integration of RWE into decision-making processes will depend on sustained investment in data infrastructure, regulatory adaptability, and ongoing engagement with all stakeholders. This collaborative approach will ensure that Europe remains at the forefront of healthcare innovation. By fostering a collaborative and trusted ecosystem, EFPIA members aim to leverage RWE to improve patient outcomes and advance medical innovation across Europe. **Together, we can create a future where healthcare decisions are informed by comprehensive integrated evidence, leading to better health for all Europeans.**