Real-world data and evidence: A European regulatory perspective

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Abstract

The European Medicines Agency (EMA) has increasingly recognised the value of real-world data (RWD) and real-world evidence (RWE) in this ever-changing regulatory landscape. Regulatory decisions have been traditionally based primarily on data from randomised controlled trials. However, RWD – information collected from everyday clinical settings such as patient registries, health records, and insurance claims – has emerged as a complementary resource. The EMA views RWE as a key tool for enhancing drug development, supporting adaptive licensing, and providing a more accurate assessment of the safety and effectiveness of medicines once they are on the market. The agency emphasises a structured approach to incorporating RWD through frameworks, pilot programmes, and data standardisation initiatives. Through various initiatives, including the DARWIN EU® initiative and its Big Data Steering Group, the EMA is working to ensure the scientific validity and regulatory acceptability of RWE across the medicinal product lifecycle.

Image: Freepik

Introduction

The European Medicines Agency (EMA) has been increasingly incorporating real-world evidence (RWE) into its regulatory framework to support decision-making across the lifecycle of medicines. This integration reflects the growing recognition of RWE's potential to complement traditional clinical trial data, particularly in addressing evidence gaps, monitoring safety, and informing post-approval assessments.[1] EMA's approach to RWE is structured around several key foundations, including the generation of fit-for-purpose data, collaboration with stakeholders, and the development of robust methodologies to ensure the quality and reliability of RWE.[2]

In their report "EMA Regulatory Science to 2025: Strategic reflection," EMA proposed "Driving collaborative RWE generation to improve the scientific quality of evaluations" as one of the five strategic goals in human medicine.[3] EMA's evolving strategy emphasises the need for continuous dialogue with stakeholders to optimise the use of RWE in regulatory evaluations.[4] This shift aims to enhance the overall benefit-risk assessment of medicinal products. Moreover, EMA's commitment to integrating RWE signifies a paradigm shift in regulatory science, fostering a more comprehensive understanding of medicines' real-world performance and safety profiles.[4] This progressive approach acknowledges some limitations of traditional randomised controlled trials (e.g., small and highly selected sample size) and seeks to leverage the growing availability of digital health data for more effective regulatory decisions.[5] This article explores the technical methods, practical applications, and specific case studies of RWE integration into EMA's regulatory framework, highlighting the challenges and future directions in this evolving field.

Table 1. OPTIMAL (OPerational-TechnIcal-MethodologicAL) framework for real-world evidence[a]

Pillar	Focus area	Key components
Operational	Concerns the **quality and governance** of real-world data and data sources	• Data provenance, access, and governance structures • Completeness, consistency, accuracy, traceability, and sustainability of data collection • Data sharing agreements aligned with the General Data Protection • Regulation and national legislation; use of ENCePP Code of Conduct • Transparent documentation of data source policies and collaboration models
Technical	Relates to **data structure, interoperability, and management systems**	• Use of standardised terminologies and coding systems • Interoperability across systems and potential for data linkage (e.g., registries, electronic health records, claims) • Mapping to Common Data Models, including validation • Quality assurance/control procedures, including internal/external audits • Benchmarking against external data sources • Capture of critical time elements and consistent recording practices • EMA qualification procedure for data source
Methodological	Focuses on the **scientific rigour** of study design and analysis of real-world evidence	• Use of appropriate, validated study designs for regulatory purposes • Identification and control of confounding factors and biases • Documentation of feasibility analysis • Study protocol registration and transparent reporting of results • Use of best practices in epidemiology/statistics • Seeking EMA Scientific Advice for study protocol evaluation

[a]Adapted table from *Cave et al. (2019)*[6]

Abbreviations: EMA, European Medicines Agency; ENCePP, European Network for Centres of Pharmacoepidemiology and Pharmacovigilance.

EMA's regulatory framework and actions on RWE

EMA has taken a proactive approach to integrating RWE into its regulatory decision-making framework. This includes strategic initiatives, infrastructure development, pilot programmes, and stakeholder collaboration aimed at fostering trust in the utility of RWD and RWE.

Key pillars of the RWE framework

EMA's approach to RWE is structured around three central pillars – operational, technical, and methodological (Table 1), forming the OPTIMAL framework,[6] which could collectively aim to maximise the potential of RWE in regulatory decision-making.

EMA has identified three major pathways for generating RWE: the Data Analysis and Real World Interrogation Network (DARWIN EU®), in-house electronic health databases, and studies commissioned via EMA framework contracts.[2] Building on these and the key pillars, EMA has introduced several initiatives aimed at bolstering its commitment to RWE.

The Big Data Steering Group

In 2020, EMA and the Heads of Medicines Agencies established the Big Data Steering Group (BDSG) to enhance the capacity of the European regulatory network to use big data, including RWD. This group provides strategic direction on priorities, such as data quality, analytics capabilities, and RWE acceptability. They aim to advance the integration of big data into the regulatory framework to improve public health outcomes.[7] BDSG is currently exploring the use of mobile health (mHealth) data, generated from devices like smartphones and wearables, to provide detailed patient information (e.g., heart rate and sleep quality) for regulatory decisions. While mHealth data shows promise, challenges such as data quality and privacy must be addressed.[8]

DARWIN EU®

Launched in 2022, DARWIN EU® is a key initiative enabling the EMA to access and analyse RWD from across Europe. By establishing a network of data partners across the EU, DARWIN EU® facilitates timely and robust analyses, enabling regulators to assess the safety and effectiveness of medicinal products more efficiently.[9] This network aims for approximately 40 partners by February 2026 and currently provides access to anonymised health data from around 180 million patients across Europe.[10]

DARWIN EU® assists in:

- Supporting regulatory assessments, such as risk-benefit evaluations, safety signals, and post-marketing surveillance.
- Enhancing the understanding of disease epidemiology and treatment patterns.
- Contributing to research and innovation by enabling high-quality studies at scale.

Guidance documents and scientific advice

The EMA provides specific guidance on the design, conduct, and reporting of RWE studies. For example, the "Guideline on Registry-Based Studies"[11] and the "Guideline on Good Pharmacovigilance Practices" outline expectations for the quality and transparency of RWE.[12]

Additionally, developers can seek scientific advice from the EMA on planned RWE studies.[2] This helps align study designs with regulatory

expectations early in the process, thereby increasing the likelihood that RWE will be considered acceptable during regulatory review.

Pilot programmes and case studies

The EMA has conducted various pilots to test the regulatory acceptability of RWE. For instance, a pilot programme involving post-authorisation safety studies demonstrated how RWE could supplement traditional data sources in assessing long-term safety.[13] Another pilot programme used patient registries to evaluate treatment effectiveness in rare diseases, supporting product label extensions.[14,15]

Through these pilots, EMA has demonstrated that well-conducted RWE studies can provide reliable and actionable evidence for regulatory purposes.

Technical methods for RWE integration

EMA has developed and refined various technical methods to incorporate RWE into its regulatory processes. These methods are designed to address the complexities of RWD, ensuring its quality, reliability, and relevance for decision-making.

Study design and data sources

RWE studies often involve retrospective cohort studies, case-control studies, and observational research. EMA emphasises the importance of using fit-for-purpose data sources, such as electronic health records, claims databases, and patient registries.[16] For instance, EMA's pilot programme identified 61 research topics for RWE generation, with a focus on medicine safety, clinical trial design, drug utilisation, and disease epidemiology, and each necessitates fit-for-purpose data sources relevant to the regulatory research question.[17] Additionally, EMA has stressed the importance of early interactions with stakeholders, such as marketing authorisation holders, to better understand research questions and optimise RWE generation.[17,18] Similarly, the use of external controls derived from RWD has been explored to contextualise outcomes from uncontrolled trials, though this requires careful consideration of eligibility criteria, temporality, and population representation.[19]

Analytical methods

The use of advanced analytical techniques, such as propensity score matching and instrumental variable analysis, can help address confounding and bias in RWE studies.[20] EMA also advocates for transparency in study design and analysis, with a focus on reproducibility and robustness.[21] EMA recommends sensitivity analyses to be considered to assess the reliability of findings in the context of uncertainty and potential bias.[2,19,22,23]

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Data quality and standardisation

Ensuring the quality of RWD is a critical challenge. EMA has emphasised the need for standardised data collection and reporting practices. Initiatives such as the European Health Data and Evidence Network (EHDEN)[24] and the GetReal Institute[25] aim to improve data interoperability and facilitate the use of RWE in regulatory and Health Technology Assessment (HTA) processes.[26,27]

Practical applications of RWE in the EU regulatory framework

Practical applications of RWE have emerged across various stages of the medicinal product lifecycle, from pre-approval to post-marketing surveillance.

Regulatory decision-making

RWE is increasingly used to support regulatory decisions, particularly in cases where clinical trial data is limited. For example, RWE has been instrumental in evaluating the safety and effectiveness of orphan medicinal products to overcome the unique challenges of rare disease drug development.[28] The EMA has also used RWE to inform decisions on drug utilisation and clinical management, particularly in the context of the COVID-19 pandemic.[17]

In regulatory submissions, RWE has been used in the context of marketing authorisation applications (MAAs) and extensions of indication. A review of MAAs submitted to the EMA in 2018 and 2019 revealed that 40% of these applications included RWE, primarily derived from registries and hospital data, to support safety and efficacy assessments.[29] Similarly, RWE has been used to inform post-marketing safety evaluations, particularly for rare adverse events and subgroup analyses.[30] EMA has also recognised the value of RWE in evaluating the effectiveness of risk minimisation measures and understanding product usage and misuse.[30]

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Health technology assessment

RWE plays a pivotal role in HTA processes, particularly in evaluating the real-world effectiveness and cost-effectiveness of treatments. HTA bodies such as the National Institute for Health and Care Excellence (NICE) and the Haute Autorité de Santé (HAS) have increasingly accepted RWE to inform reimbursement decisions, though challenges remain in terms of data quality and generalisability.[22,31] Moreover, the collaborative work between EMA and EUnetHTA (European Network for Health Technology Assessment) highlights the importance of using extrapolation (inferring data from adults to children, aiding regulatory decision-making) in paediatric drug development to address ethical concerns and support robust evidence generation, ultimately aiding in benefit/risk considerations for regulatory authorities and HTA bodies.[32]

Post-approval surveillance

RWE is widely used for post-marketing surveillance, enabling the identification of safety signals and adverse events in real-world settings. The EMA's DARWIN EU® initiative, a network for RWD analysis, has been instrumental in monitoring the safety of approved medicinal products.[27]

Healthcare resource use

RWE is increasingly used to assess the economic impact of healthcare interventions. By providing insights into treatment patterns, resource utilisation, and long-term outcomes, RWE supports value-based decision-making and resource allocation.[22,33]

Image: Freepik

Case studies: RWE in action across the EU

Several case studies illustrate the successful integration of RWE into EMA's regulatory framework.

Orphan medicinal products

RWE has been particularly valuable in the approval of orphan medicinal products, where clinical trial data is often limited. For example, the approval of abaloparatide for osteoporosis relied on RWE to address gaps in clinical trial data, demonstrating the effectiveness of RWE in supporting regulatory decisions for rare diseases.[34]

Oncology medicines

In oncology, RWE has been used to evaluate the real-world effectiveness of cancer treatments. A review of oncology-targeted therapies approved between 2018 and 2022 indicated that RWE contributed to regulatory decisions in 21% of cases, demonstrating its role in addressing evidence gaps and enhancing understanding of treatment outcomes.[35] A case study on the approval of a novel oncology medicine highlighted the role of RWE in bridging the gap between clinical trial results and real-world outcomes, facilitating regulatory and HTA decisions.[36]

Rare diseases and registry data

The SATURN initiative demonstrates the feasibility of using existing registries to collect RWE for rare diseases. By leveraging data from the Registry of Osteogenesis Imperfecta, SATURN has provided valuable insights into treatment practices and outcomes, supporting regulatory and HTA decision-making.[37]

Challenges in RWE integration

Despite its potential, integrating RWE into the EMA's regulatory framework faces several challenges.

Data heterogeneity and quality

The heterogeneity of RWD sources across EU member states poses significant challenges. Differences in healthcare systems, data collection practices, and privacy regulations complicate the aggregation and analysis of RWD.[6,33]

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Regulatory harmonisation

The lack of harmonised guidelines for RWE use across EU member states and HTA bodies remains a barrier. While the EMA has made progress in developing RWE-specific guidance, inconsistencies in terminology and methodological preferences persist.[26,38]

Ethical and privacy considerations

The use of RWD raises ethical and privacy concerns, particularly concerning patient confidentiality and data protection. The implementation of the General Data Protection Regulation in the EU has introduced additional complexities in RWD utilisation.[22,33]

Image: Freepik

Future directions for RWE in the EU

To fully realise the potential of RWE, the EMA and other stakeholders must address existing challenges and invest in initiatives that enhance the quality, accessibility, and harmonisation of RWD.

Harmonising RWE guidelines

The development of harmonised guidelines for RWE use across EU member states and HTA bodies is essential. A public-private partnership, the Integration of heterogeneous Data and Evidence towards Regulatory & HTA Acceptance (IDERHA) project,[39] aims to align RWE requirements and reduce fragmentation in regulatory and HTA processes.[38,40] IDERHA, launched in April 2023, aims to apply artificial intelligence (AI) and machine learning (ML) to link and analyse diverse health data for early detection of lung cancer and improved quality of life for those affected.[39]

Enhancing data infrastructure

Investing in robust data infrastructure is critical to overcoming the limitations of RWD. Under the IDERHA project, initiatives such as the European Health Data Space (EHDS) aim to improve data interoperability and facilitate cross-border data sharing.[6,41] EHDS is designed to enable individuals to access and manage their health data across the EU. This initiative includes the primary use of data (EHDS1; MyHealth@EU) for healthcare delivery and decision-making, and the secondary use of data (EHDS2; HealthData@EU) for research, innovation, policy-making, and regulatory purposes.[42]

Promoting innovation and collaboration

The integration of advanced technologies, such as AI and ML, holds promise for enhancing RWE generation and analysis. Collaboration among regulators, industry stakeholders, and academia will be key to driving innovation and addressing methodological challenges.[22,43]

Building on existing initiatives

The EMA's Network Strategy to 2025 aims to leverage RWE further in regulatory processes, addressing critical research questions and enhancing medicinal product evaluations.[44] Building on that, EMA's Network Strategy 2028 plans to further leverage RWE with a strengthening contribution from digital transformation and AI.[45]

Conclusion

EMA is at the forefront of integrating RWE into the regulatory framework. Through strategic initiatives like DARWIN EU®, robust guidance documents, and collaborative stakeholder engagement, EMA is enabling the generation and use of high-quality, regulatory-grade RWE. Despite challenges in data quality, methodology, and interoperability, the outlook is promising. Continued investment in data infrastructure, methodological innovation, and regulatory alignment will be key to unlocking the full potential of RWE. As RWE becomes increasingly embedded in decision-making processes, it promises to enhance the evaluation of medicinal products, improve patient outcomes, and support more dynamic and responsive regulatory practices.

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The opinions expressed in this article are the author's own and not necessarily shared by his employer, the EMWA, or the EMA.

Disclosures and conflicts of interest

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