Infosheet

Review: real-world data studies

Experience gained in conducting studies and providing real-world evidence (RWE) to support EMA regulatory decision making

Sustainable framework to support scientific evaluations in the EU

By complementing existing evidence, use of **real-world data (RWD)** and **real-world evidence (RWE)** can speed up medicine development and support post marketing safety and effectiveness monitoring.

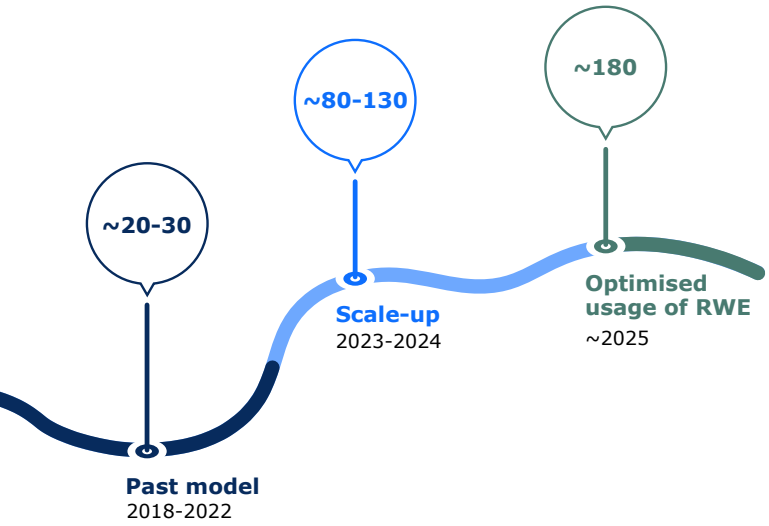
Overseen by the Big Data Steering Group (BDSG), EMA and the European medicines regulatory network (EMRN) are working to **establish a sustainable framework enabling better integration of RWD/RWE into regulatory decisions**. Pilots with EMA scientific committees and working parties (COMP, PDCO, SAWP, CHMP and CAT) are ongoing. EMA has reviewed the experience gained so far in conducting studies with RWD and the challenges and opportunities of providing RWE to support regulatory decision making.

Transformational journey to fully integrate RWE in EU regulatory decision making

By 2025 the use of real-world evidence will have been enabled and the value will have been established across the spectrum of regulatory use cases to:

- support the planning and validity of studies performed/submitted by applicants;
- understand the clinical context;
- investigate associations and impact of regulatory decisions.

Number of RWD studies per year:



EMA's 3 main pathways for RWE generation

RWD can come from marketing authorisation applicants/holders, academia or national competent authorities. EMA can access RWD as follows:

EMA studies
Conducted by EMA's RWD analysts in collaboration with requester through direct access to 6 European primary healthcare data sources.

Framework contracts
Studies commissioned to research organisations and consortia with access to specialised data and expertise.

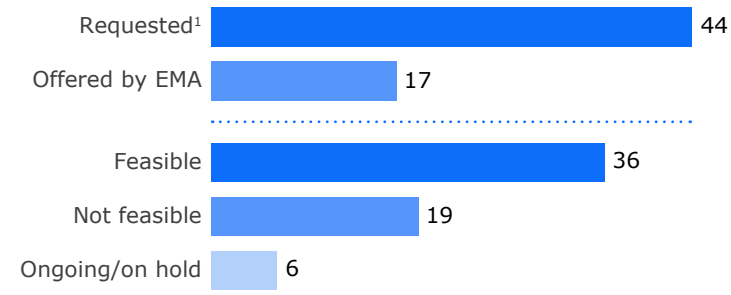
DARWIN EU®
Studies conducted via a federated network of data, expertise and comprehensive services with access to data partners and sets of analyses.

The pathways differ in terms of processes, speed of analysis, capacity and characteristics of data sources.

EMA-led RWD studies

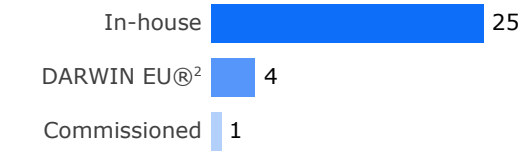
From September 2021 to February 2023:

61 **Research topics identified**, of which:



[1] Requested by decision-maker.

30 **Studies initiated**, of which:



[2] Studies were limited to 4 in the first year of its establishment.

Additional studies (not included in the review):

11 COVID-19 studies

7 Impact studies

The studies mainly addressed research needs of the PRAC, PDCO, COMP, and SAWP regarding safety signals, periodic safety update report single assessments, applications for paediatric investigation plans and waivers, orphan designations and scientific advice. Two out of three study requesters considered the results support their assessment.

Main learnings

- RWD studies addressed various research questions and supported decision making for a variety of regulatory contexts.
- All 3 RWE generation pathways are important with their strengths and limitations.
- Main reason for unfeasible studies in 2022: conditions not diagnosed or medicines not used in primary care setting.
- Collaboration with study requesters is paramount for implementation of the RWE framework.
- Information on data source characteristics needed.

Recommendations for enabling the use of RWE

The report provides a set of recommendations to address identified opportunities and challenges.

Access to data sources
Wider access to more diverse and complementary data sources

Accelerate
Strategies to further accelerate RWE generation

Regulatory context
Anticipate RWE needs of decision-makers by identifying research questions earlier

Capacity and capability
Develop educational and knowledge management sharing tools

Collaboration
Close collaboration with decision-makers and other stakeholders

The learnings and recommendations arising from the review will feed into the work of the Big Data Steering Group and further inform the establishment of DARWIN EU®.

The report

The report Real-world evidence framework to support EU regulatory decision making takes stock of the experience gained from 1 September 2021 until 7 February 2023 in conducting RWD studies and evaluates the **opportunities and challenges in supporting regulatory decision making with RWE**.

The report is available on the EMA website.

Contact us

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