

BIG DATA HIGHLIGHTS

Quarterly update on implementation activities of the
HMA-EMA Big Data Steering Group workplan

An agency of the European Union



Editorial

Patients' perspective on real-world evidence in regulatory decision making

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Digitalisation has increasingly been embedded in our everyday life. Digital technologies have begun to change the practice of medicine, hence research and innovation. The availability of a broad scope of tools, whether electronic health records, mobile-health tools, wearables, registries or others, have increased tremendously the generation of health data.

Digitalisation is steering medicine to bottom-up data management that involves real-time data extraction and analysis of various data sources. This offers the opportunity to improve the generation of evidence, including the design, speed and efficacy of randomised controlled trials for the development of new medicines without compromising patient safety.

Patients are generally supportive of sharing their data for research.



They perceive that data sharing could lead to innovative medicines that meet their real needs and improve health outcomes, and most notably their quality of life. Public and patient motivations and willingness to share data relate to their perception of contributing to the 'common good'. Nonetheless, they expect transparency and require reassurance and mechanisms to mitigate risks so that their health data will be protected.

Meaningful patient involvement in both research for novel products and in medicines regulatory processes is key factor for trust in future innovations. Patient needs and expected outcomes are key to factor-in from the outset to ensure relevance, value, efficacy and adherence. Robust collection of high-quality data coming from patient reported outcomes is increasingly important to better evaluate the added value of a medicine.

Patients' perspective on real-world evidence in regulatory decision making (continued)



The use of real world data and evidence in the development, authorisation and monitoring of medicines is increasing. Interoperability, data standards and quality controls are key to foster reliability and trustworthiness of RWE generation. Therefore, the regulatory framework must recognise and enable RWE use in the whole lifecycle of medicines.

As the EU is in the process of reviewing its pharmaceutical legislation, politicians and regulators have the opportunity to enable public and patient engagement in RWD and RWE generation and strengthen the possibility of patient and public interaction with their data.

To maximize patient uptake and the availability and use of RWE, governments must invest in improving digital health literacy and must address any remaining concerns over data protection. Digital health literacy is important to facilitate secondary use of health data and to build up trust in the potential of RWE use in the lifecycle of medicines and medical devices.

Finally, shortages of medicinal products are a direct threat to people's good health and as such, it is essential to capture people's experience with shortages to better understand the health and societal impact. Direct reporting from patients can help competent authorities to track shortages and identify potential problems with alternative treatments that have been prescribed.

Featured topics

Big Data Priority recommendations

Data quality and representativeness

Data Quality Workshop

One of the priority recommendations of the HMA-EMA joint Big Data Task Force was to establish an EU framework for data quality that will support the trust of patients and healthcare professionals in the decisions reached by regulators when various types of data underpin those decisions. In this context EMA and HMA in collaboration with TEHDAS (Joint Action 'Towards a European Health Data Space') held a [Data Quality Framework workshop on 7 April 2022](#).

The workshop aimed to share the current progress on building a data quality framework for medicines regulation and solicit feedback from subject matter experts to help shape the further drafting of the framework. Breakout sessions focused on particular use cases in the clinical and non-clinical spaces.

The input from the workshop will be taken into consideration when drafting the general scope of the Data Quality Framework during 2022 that will also be complemented by a written stakeholder consultation. The workshop report will be published in the coming weeks on the [Big Data page](#) of the EMA website.

EU Network processes

Real-world evidence integration into medicines regulatory decision making

EMA scientific committees are using RWE to support decision making in medicines regulatory procedures. Since October 2021 this evidence is generated more systematically through a multi-phased pilot. The first phase of the pilot resulted in routine support on RWE to the PRAC and will shortly extend it to SAWP and COMP.

The second phase of the pilot is dedicated to RWE use cases of the CAT and PDCO while preliminary pilot discussions with CHMP and CMDh are ongoing. The RWE pilot with the EMA scientific committees has several aims:

- To refine the use cases and the processes of the committees;
- To understand which types of questions can be answered based on currently accessible data, the capacity for questions to be processed and the timelines needed; and
- To raise awareness of the challenges and opportunities of RWE.

Between October 2021 and March 2022, 25 requests for studies or analyses were initiated through the pilot, of which 44% were feasible, 24% not feasible, with 32% still under evaluation. The median time to obtain the results was 9 days. From the finalised studies, 62.5% of the results were used during the evaluation procedures to support the decision making process.

The following use cases are illustrative examples of RWE requests received during the pilot phase:

- Update on disease prevalence in case of rare diseases, especially when the submitted evidence is outdated or it is believed that the new clinical standards extend survival and might modify the established prevalence;
- Use of RWE in paediatric procedures to provide information on drug use and prevalence of the disease in younger populations to contextualize the need for a randomised controlled trial;
- Safety evaluation, either at very early stages (safety signal validation, drug utilisation data or background incidence of adverse drug reactions), during referral procedures (contribution to the causality assessment) or in the assessment of the effectiveness of risk minimisation measures.

Validity, transparency and reproducibility of RWE are given high priority. There are ongoing discussions on process improvement to ensure that RWE analyses can provide timely decision-ready evidence on the effectiveness and safety of treatments.

The pilot with the EMA scientific committees will continue throughout 2022 and will be followed by routine implementation of RWE services into the committees' decision making.

Network capability to analyse

Advanced Analytics for interrogation of unstructured data

One of the approaches to address the Big Data priority recommendation on building the EU Network capability to analyse data is to develop a search tool that will allow members of the EMRN to interrogate unstructured data from selected types of documents using selected metadata terms. The potential benefits of this approach include improved:

- Efficiency of regulators' advice and assessment through faster identification of prior procedures with similar characteristics to the set of information that needs to be assessed;
- Cross-procedure knowledge through easier and more accurate identification of patterns across procedures that enable research and support generation of guidance;
- Characterisation of key features of submitted dossiers based on pre-specified metadata that will help identify required expertise for assessment.

In March 2022, a technology pilot was successfully completed that focused on interrogating unstructured information from the final scientific advice letters issued by CHMP having received advice from SAWP. The next step is to further develop the search tool based on the pilot findings before progressing with other use cases concerning other types of documents used by different EMA committees.

The ultimate goal of this development is to continuously enhance informed decision making for faster access to innovative, effective and safe medicinal products.

International initiatives

Clinical Trial Standardisation Development

In line with the recommendations of its Data Standardisation Strategy, the EMRN is actively involved with international partners in the work of [ICH M11](#) - Clinical Electronic Structured Harmonised Protocol. This includes contribution to the ICH guideline drafting with the aim to:

- Establish an internationally harmonised standard template for the format and content of clinical trial protocols to support consistency across sponsors; and
- Develop a technical specification that uses an open, non-proprietary standard to enable electronic exchange of clinical protocol information

These outputs will help to overcome inefficiencies and difficulties encountered in reviewing and assessing clinical protocols by involved stakeholders such as medicines regulators, clinical trials sponsors and ethical oversight bodies.

A public consultation on the draft ICH Guideline, Protocol Template and Technical Specification is expected to be launched in the third quarter of 2022.



Veterinary recommendations

European Veterinary Big Data strategy 2021 – 2027

On 5 May 2022, HMA adopted the European Veterinary Big Data strategy for 2021-2027.

The entry into force of [Regulation \(EU\) 2019/06](#) in January 2022 presented an opportunity to investigate the convergence of the traditional regulatory systems development with the advancement offered by emerging digital technologies. In line with the [European Medicines Regulatory Network strategy to 2025](#) and building upon the recommendations defined by the HMA/EMA Big Data Task Force, the European Veterinary Big Data strategy provides the Veterinary EU Regulatory Network with a data framework on how veterinary data resulting from newly availability solutions should be managed to support key regulatory activities. The strategy focuses on increasing interoperability across regulatory systems that shall be operated by an optimised set of scientific resources to reduce administrative and economic burden and enhance consistency, transparency and responsiveness within the Agency. The primary objectives are oriented at the implementation of sustainable processes and infrastructures powering re-usability of the data across key regulatory domains such as pharmacovigilance, antimicrobial resistance, environmental risk assessment, regulatory submission, innovation of veterinary medicinal products development, demonstration of efficacy/effectiveness and to deliver accurate and reliable information to promote public and animal health. The methods to develop data culture and to address and anticipate future data analytics rely on the establishment of the following pillars:



Moreover, the strategy proposes the following phased implementation steps:

- Short term phase, namely the **collection** of key underlying data and identification of additional data sources to fulfil regulatory activities in the subsequent phases (until 2023);
- Medium term, namely the **integration** of key data in targeted regulatory processes and the **inception of key analytics solutions** (2023-2025);
- Long term, namely **connection** of data to power information sharing and dissemination and to **expand analytic capabilities** (2024-2027).

The key tasks and initiatives outlined in the adopted EU Veterinary Big Data Strategy will be reflected in the HMA/EMA Veterinary Big Data work plan and will be driving the discussion at the 2nd Vet Big Data stakeholder forum planned for October 2022. The link to the EU Veterinary Big Data Strategy will be available on EMA and HMA websites.

EU Network skills

Big Data curriculum tender

EMA has launched a call for tender to select contractors able to develop training courses on Big Data. These trainings will increase the capability of the EU Regulatory network to understand, analyse and interpret Big Data, which will ultimately benefit the regulatory decision making process and therefore public health.

The Big Data Curriculum tender is divided into three lots:

- **Lot 1** - Development and Delivery of Trainings for the Data Science Curriculum
- **Lot 2** - Development and Delivery of Trainings for the Pharmacoepidemiology and Real-world Evidence Curriculum
- **Lot 3** - Development and Delivery of Trainings for the Biostatistics and Clinical Trial Methodology Curriculum and Development and Delivery of Modules in the Field of Clinical Trials.

[Call for tender's details](#)

Getting involved

Upcoming events



First Big Data Steering Group meeting with industry stakeholders

30 May 2022

ICMRA workshop on real-world evidence

29-30 June 2022

Public consultation on the Data Quality Framework

Q3 2022

Launch of pilot for raw data processing to support regulatory decision making

Q3 2022

Recent events

Multi-stakeholder information webinar on DARWIN EU

24 February 2022



Multi-stakeholder
webinar on
DARWIN EU®

24 February 2022
10:30-12:00 CET

Data quality framework for medicines regulation workshop

7 April 2022



Online workshop on
Data quality
framework for
medicines regulation

7 April 2022
13:00 – 17:15 CET

Read the [previous issue](#) of the newsletter

List of acronyms

CAT: Committee for Advanced Therapies
CHMd: Coordination Group for Mutual Recognition and Decentralised Procedures - Human
CHMP: Committee for Medicinal Products for Human Use
COMP: Committee for Orphan Medicinal Products
EMRN: European medicines regulatory network
HMA: Heads of Medicines Agencies
ICH: International Council for Harmonisation of Technical Requirements Registration Pharmaceuticals Human Use
ICMRA: International Coalition of Medicines Regulatory Authorities
PDCO: Paediatric Committee
PRAC: Pharmacovigilance Risk Assessment Committee
RWD: Real-world data
RWE: Real-world evidence
SAWP: Scientific Advice Working Party
TEHDAS: Towards the European Health Data Space

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