

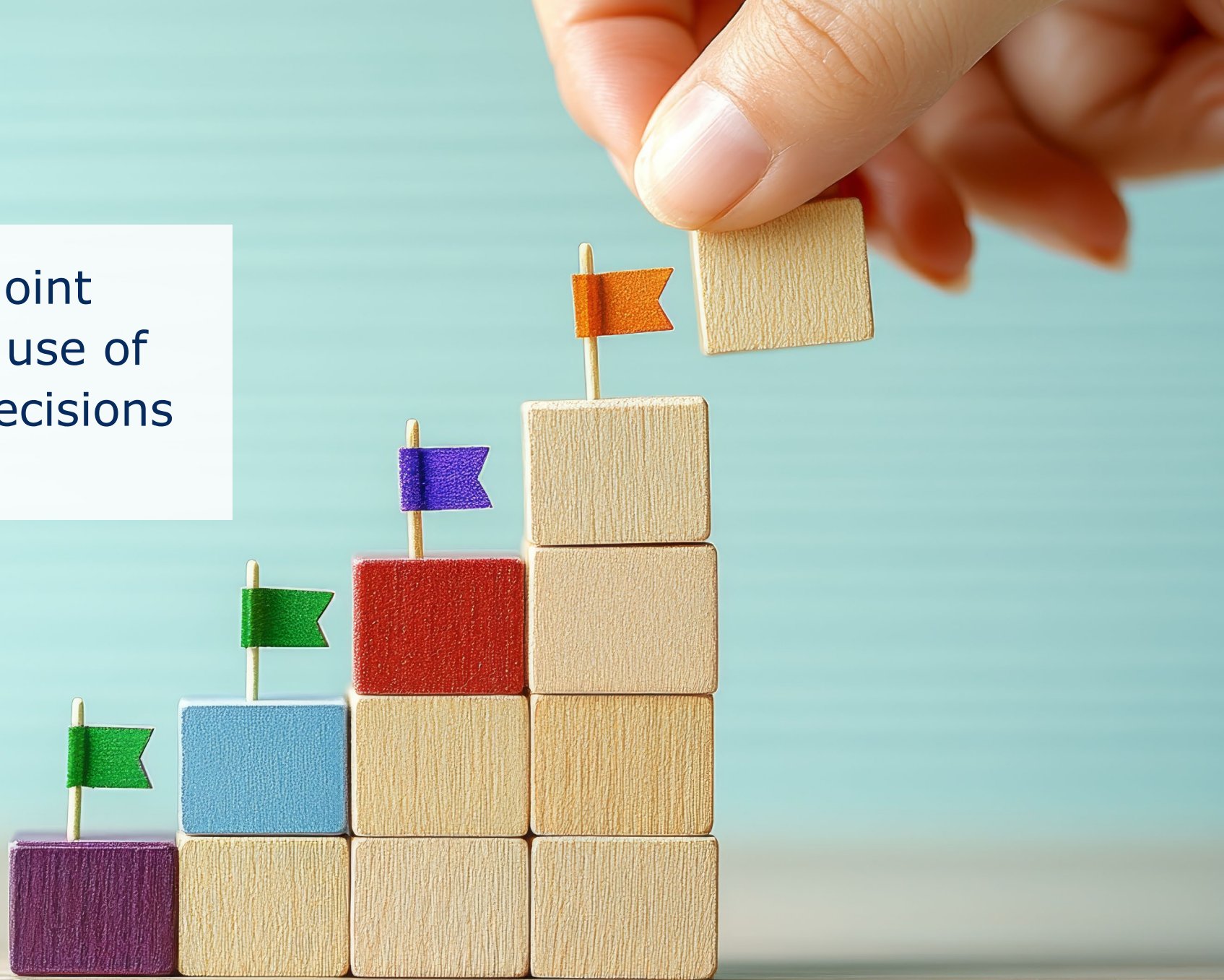
HMA-EMA Big Data Stakeholder Forum 2024

5th year into our journey to data
driven medicines regulation

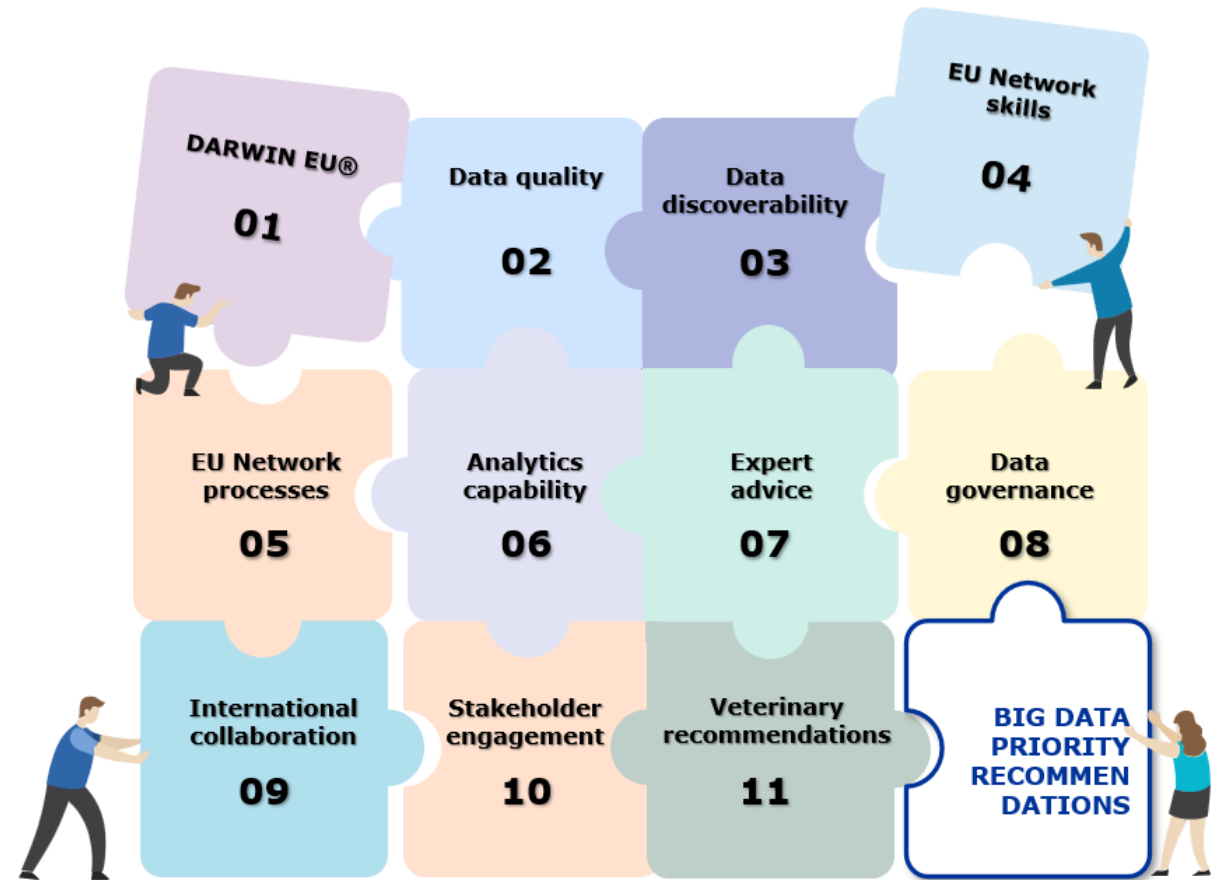
Peter Arlett, co-chair of the Big Data Steering Group

28 November 2024

Five years into our joint
endeavor for better use of
data in medicines decisions



Where we came from: 11 priorities set the stage for data-driven regulation transformation



Did we reach our goals?




In 2025... transformation to data-driven regulation



- **The use of RWE will have been enabled and its value will have been established** across the spectrum of regulatory use cases.
- **Clinical trial raw data analysis** will support regulatory decision-making.
- **DARWIN EU network** will support better decision-making.
- **Through public searchable catalogues data will be discoverable** and of known quality and representativeness allowing choice of optimal data source, enabling regulators to guide development and expertly assess study results.
- **EU Network will have knowledge and experience** in data science, methods and analytics to advise companies developing products and to expertly assess application dossiers.
- **EU and international guidelines and standards available** will help industry and regulators develop and supervise medicines.
- Continued full **compliance with data protection and ethics** of data sharing.
- **Guided by patients and working with stakeholders** to deliver data transformation to support the development and use of better medicines for patients.

"At the core of a successful MA dossier is excellent clinical evidence"

A composite image featuring a blue gradient background on the right and a medical-themed collage on the left. The collage includes a laptop, a stethoscope, a tablet displaying a line and bar chart, and an ECG strip.

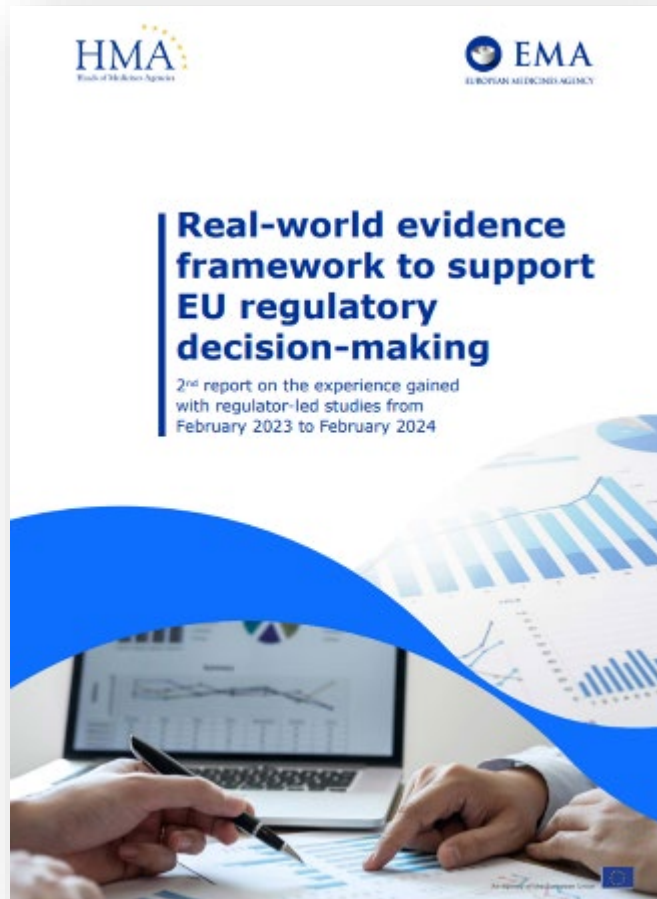
Real-world evidence integration into medicines regulatory decisions

“

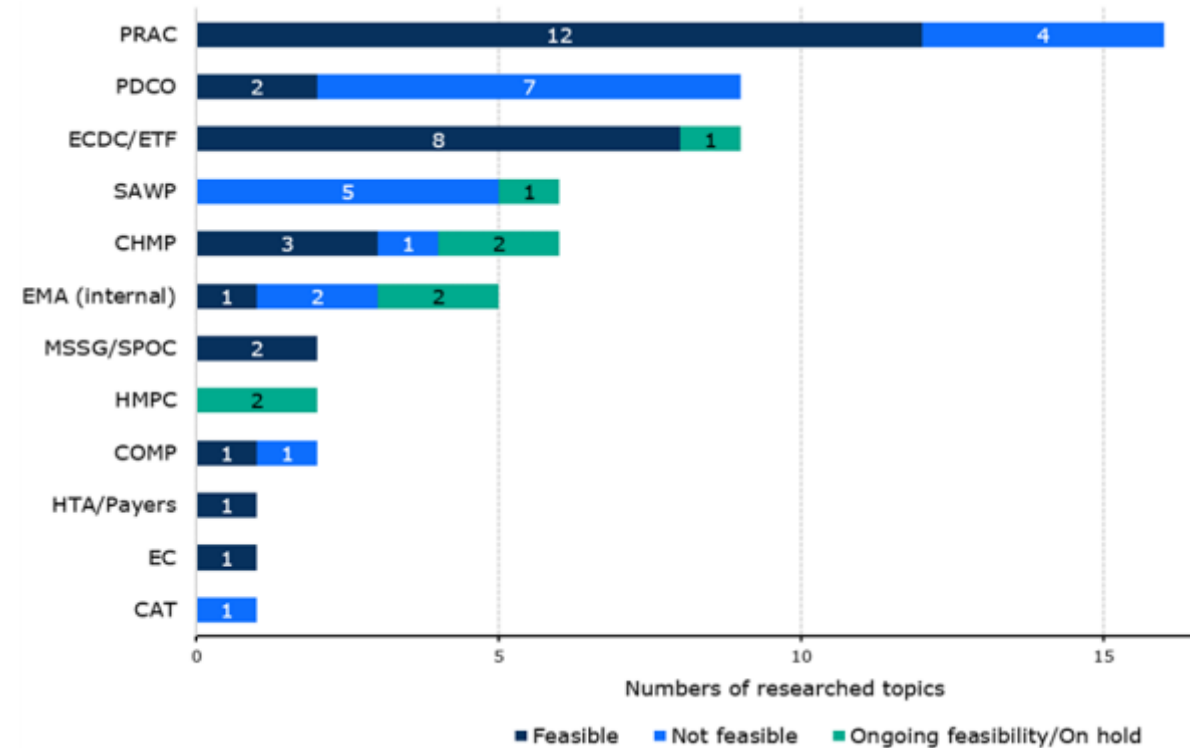
Vision: by 2025 the use of real-world evidence will have been enabled and the value will have been established across the spectrum of regulatory use cases.

Clinical Pharmacology and Therapeutics: 2022 January. Vol 111; No 1: 21 – 23.

RWE is used across the medicinal product lifecycle



In 12-months, 60 requests by:



DARWIN EU® is now fully operationaland continues to grow



Primary pathway

for generating real-world
evidence



20 data partners

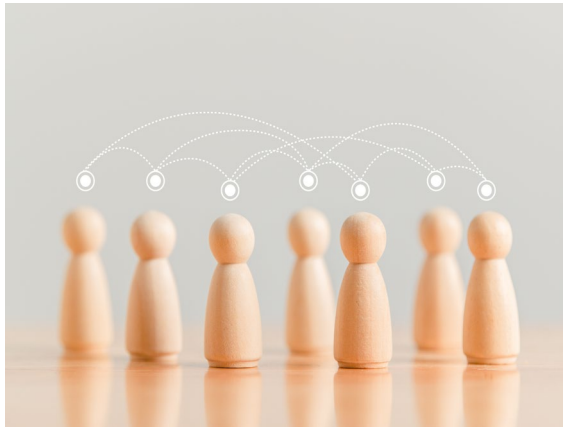
from 13 European countries
have need onboarded



Access to data from

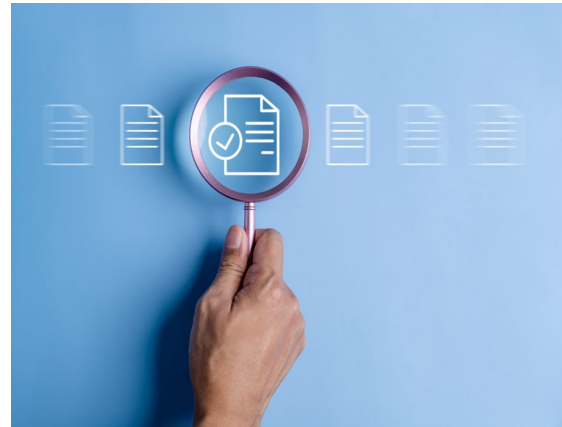
~ 130 million patients in
Europe (2024)

International collaboration on the use of real-world data established



International regulatory collaboration on RWE

ICMRA Working Group on Real-World Evidence for Public Health Emergencies



ICH Reflection Paper

For convergence on RWE terminology, format of study protocols and reports, and study transparency



ICH M14 guideline

On non-interventional pharmacoepidemiological studies for safety assessment of medicines



Data discoverability,
quality and transparency in
observational research

Catalogues of RWD sources and studies made data more discoverable, while fostering observational research



3,000

real-world data studies



235

real-world data sources



Data Quality Framework for medicines regulation

- set the standard for health data
- foundation to inform EHDS



Leveraging AI and data analytics

Enabling safe and responsible use of AI



Guidance and policy

- Reflection paper on use of AI
- Guiding principles on the use of LLMs



Tools and technologies

- Roll-out of knowledge mining tools
- Use cases to build Network knowledge mining roadmap



Collaboration & change management

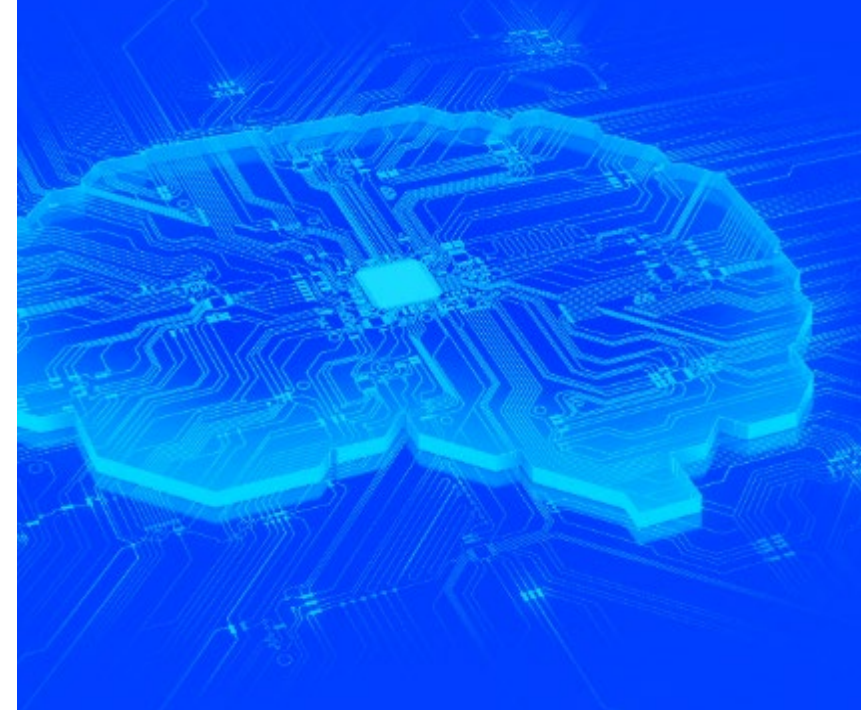
- Public AI workshops
- Network masterclasses on AI



Experimentation

- Network AI technical deep dives
- Network AI experimentation

Multi-annual AI workplan 2023-2028



Practical learnings from the raw data pilot



Potential for earlier authorisation of medicines



Better understanding of data drives better opinions



Industry requests for pilot participation welcome





Veterinary recommendations

Veterinary big data strategy: from vision to action

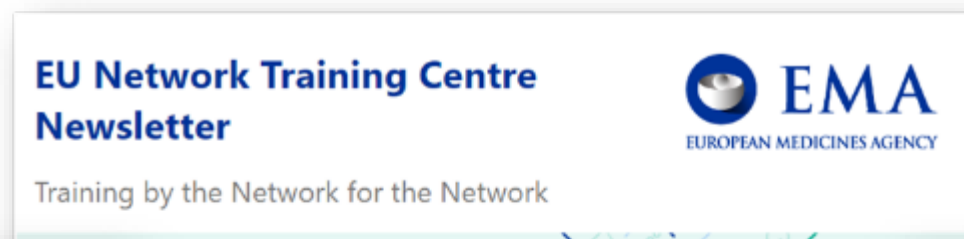


Veterinary Big Data Workplan 2022-2025



Network skills

Empowered the network through digital and big data training offers



BIG DATA, AI

First modules of the Data Science curriculum now available

The Data Science curriculum is a series of trainings on data science that have been specifically designed for the European Medicines Regulatory Network (EMRN) to enhance knowledge and develop skills in this field.

Data science curriculum

ARTIFICIAL INTELLIGENCE, PHARMACOVIGILANCE

Challenges and future role of artificial intelligence and machine learning in pharmacovigilance

This is a recording of a webinar held on 26 July 2024 on the challenges and future role of artificial intelligence and machine learning in pharmacovigilance. The webinar was delivered by Dr Dirk Mentzer, Head of Safety of Biomedicines and Diagnostics Division, Paul Ehrlich Institut.



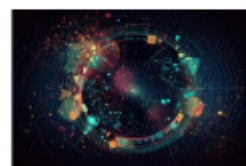
more

DIGITAL, DATA, DATA PROTECTION

Digital Academy intro modules and collections on Data Literacy and Data Protection

Increase your data literacy and data protection skills with the new Digital Academy interactive e-learning modules and accompanying learning resource collections

more



DIGITAL

Digital Academy intro module and collection on Design Thinking

In this new Digital Academy 30-minute interactive e-learning module and accompanying learning resources collection, you will learn about Design Thinking.

more



BIG DATA, RWD, RWE

RWD in support of regulatory work in paediatrics – example of the RWD extrapolation framework with a pilot study in dermatomyositis and polymyositis - Real World Academy session 4

This 4th session of the Real World Academy focuses on the use of RWD to inform and support regulatory work in paediatrics.



BIG DATA

DARWIN EU Autumn School: Population-level disease epidemiology and patient-level characterisation

This is the second webinar in a series to increase the understanding of different types of data and their use through the DARWIN



04/11/2024 - 05/11/2024



Stakeholder collaboration and engagement

Leveraged your insights and expertise for better data outcomes



Methodology Working Party

- European specialised expert community (ESEC)
- Special interest areas (SIAs) including on AI and RWE



Pilots and focus groups

- Dedicated to real-world data, clinical trials raw data
- Stakeholder representation at the Big Data Steering Group and DARWIN EU



Events and public consultations

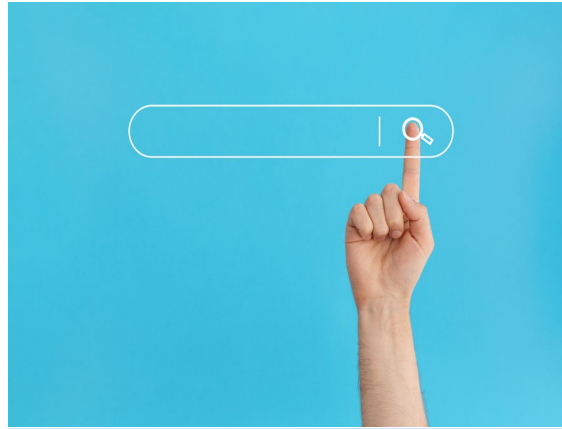
- Input sought on data quality framework, reflection papers on RWE and AI, network data strategy
- Workshops included patient registries, RWE methods, AI and pharmacogenomics

Stakeholders kept informed



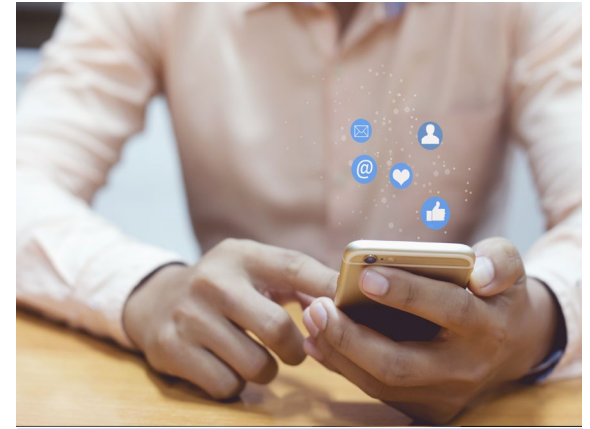
In writing

Big Data Highlights
Targeted stakeholder
mailing



Online

Big data webpage
EMA website: news and
events



Social media

LinkedIn
X



Future horizons

Seizing opportunities in a changing medicines landscape



EU medicines legislative changes

- Reform of pharmaceutical legislation
- European Health Data Space
- AI Act



EU medicines regulatory network strategy 2028

- Theme 2: leverage data, digitalisation and AI
- Maximise generation, interoperability, use and exchange of data
- Realise the network vision on AI across all EMANS focus areas



Strengthening network data governance

- New Network Data Steering Group
- Scope: data governance, interoperability, data analytics and AI
- Network data strategy – **public consultation** until 31 Dec 2024

Diverse types of data,
new approaches to
analysis – unlock
potential to support
medicines regulation



A close-up photograph of two hands, one slightly darker in skin tone than the other, clasped together in a firm grip. The hands are positioned centrally, with fingers interlaced. The background is a solid, light blue color.

Collaboration is key

Thank you.



ema.europa.eu/bigdata



[@EMA News](#)



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