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

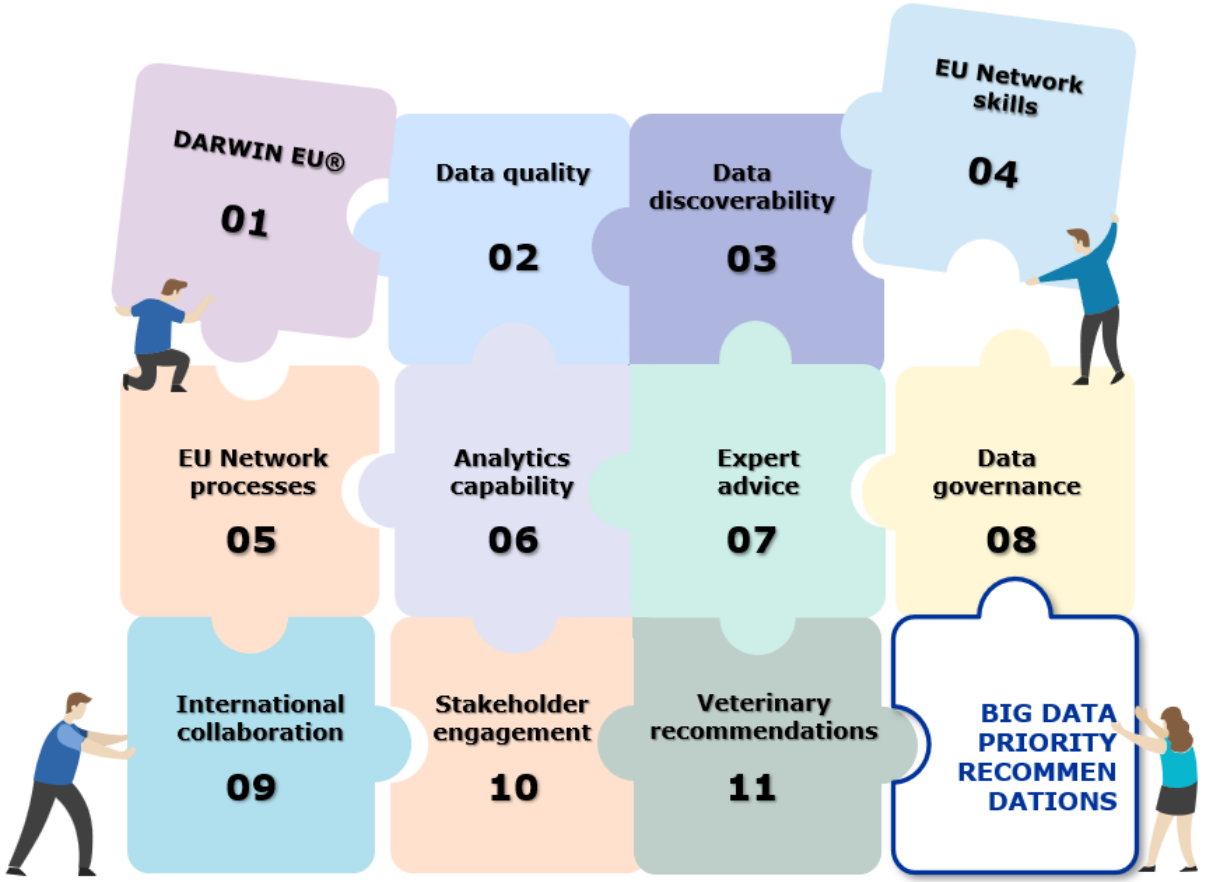

HMA

EUROPEAN MEDICINES AGENCY

HMA-EMA Joint Big Data Taskforce
Phase II report:
'Evolving Data-Driven Regulation'

[1] Corr. The links in the Annex IV and Annex VII have been updated.

See websites for contact details
Heads of Medicines Agencies www.hma.eu
European Medicines Agency www.ema.europa.eu



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"

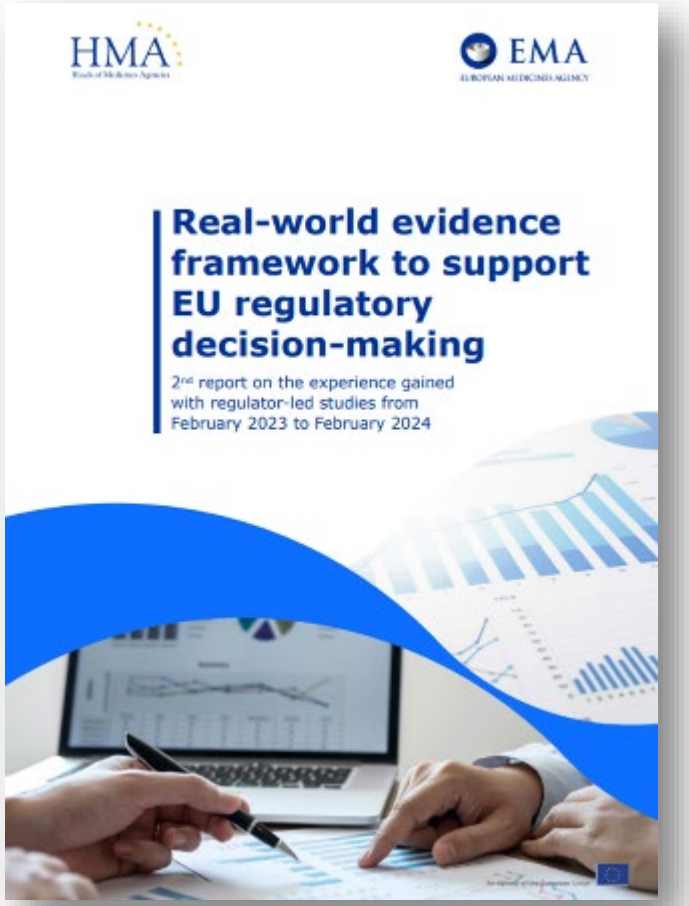
Real-world evidence integration into medicines regulatory decisions

“

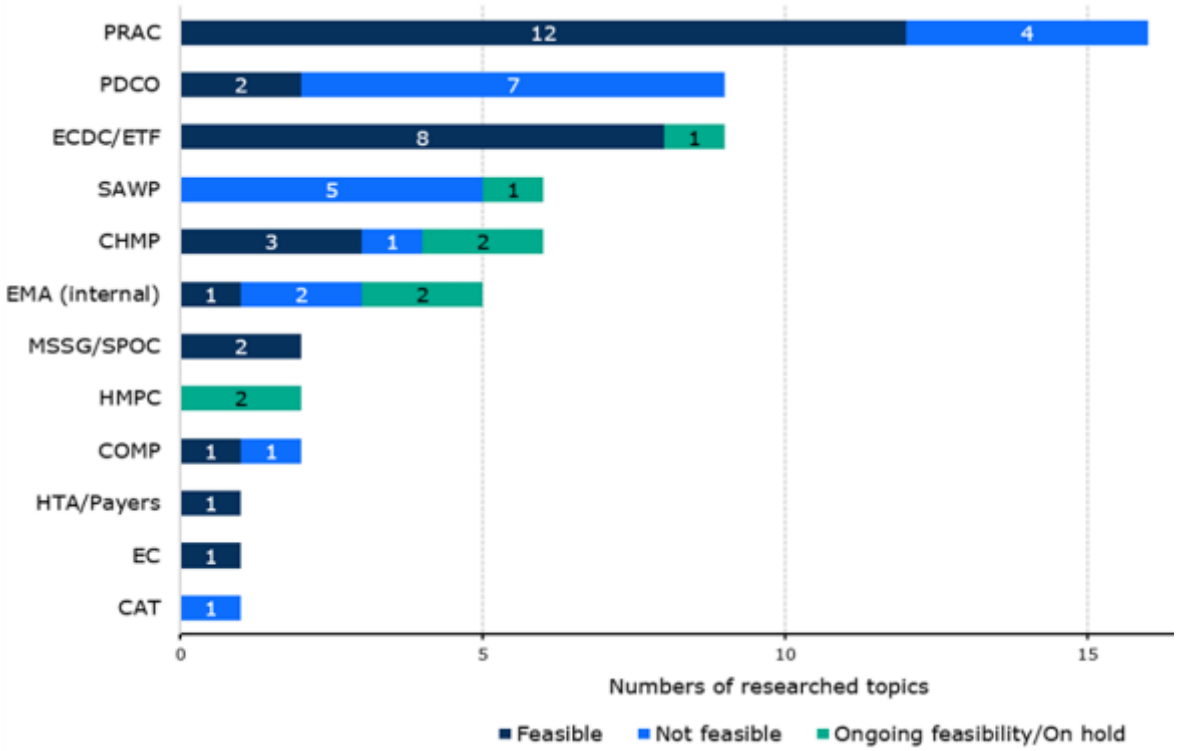
Vision: by 2025 the use of real-world evidence will have been enabled and the value will have been established across the spectrum of regulatory use cases.

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 operational ....and continues to grow

Primary pathway

for generating real-world evidence

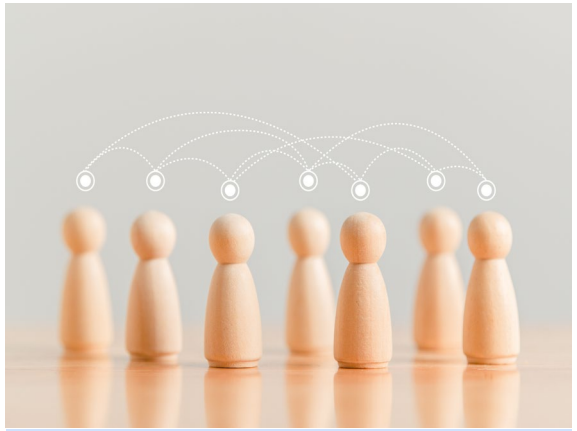
20 data partners

from 13 European countries have need onboarded

Access to data from

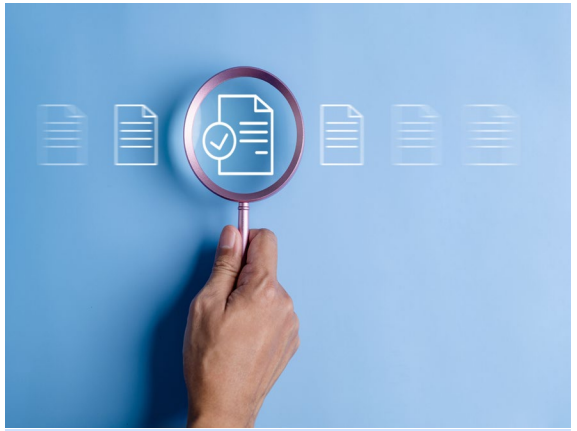
~ 130 million patients in Europe (2024)

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

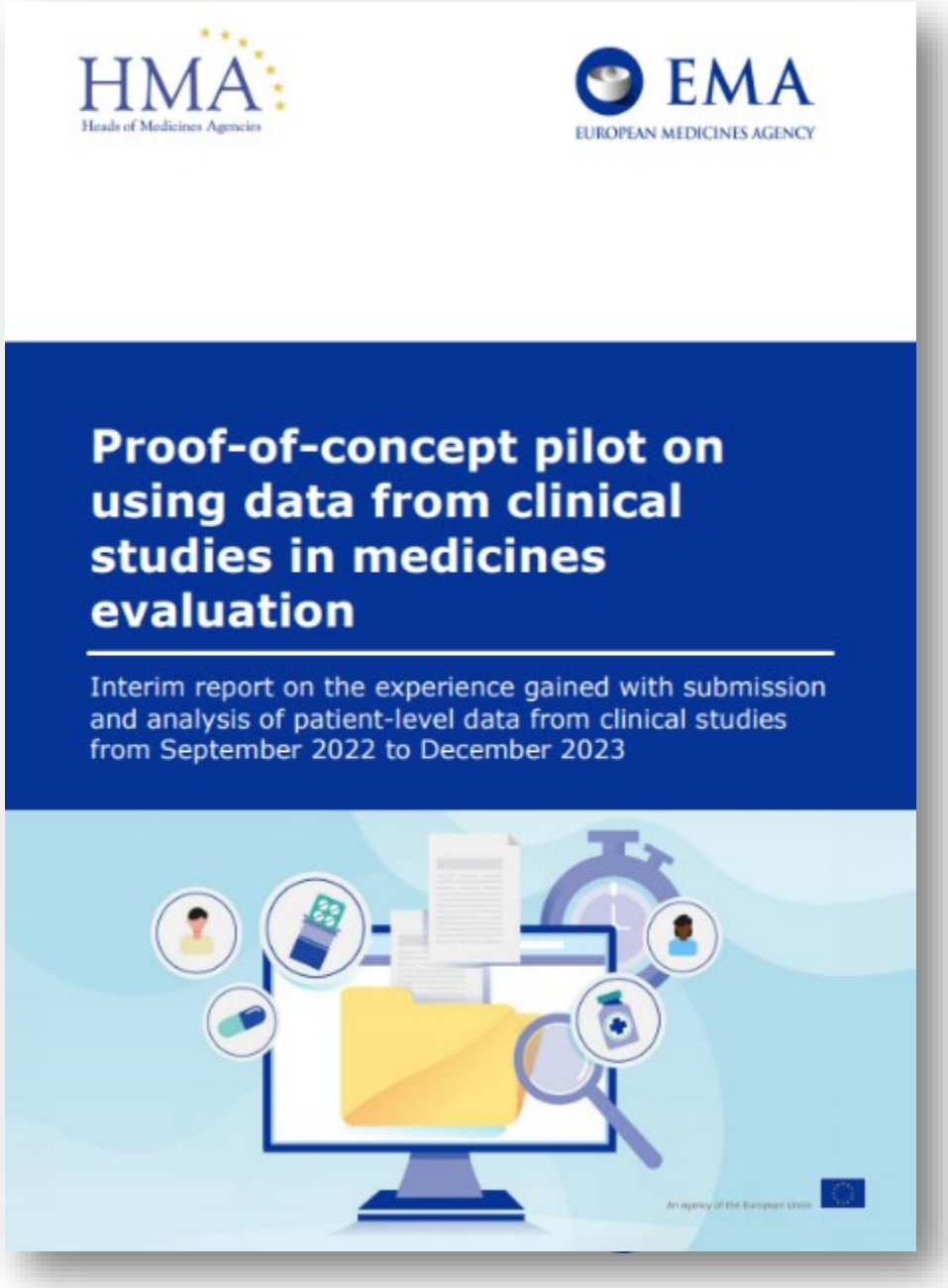
- Network AI technical deep dives
- Network AI experimentation

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

EMA
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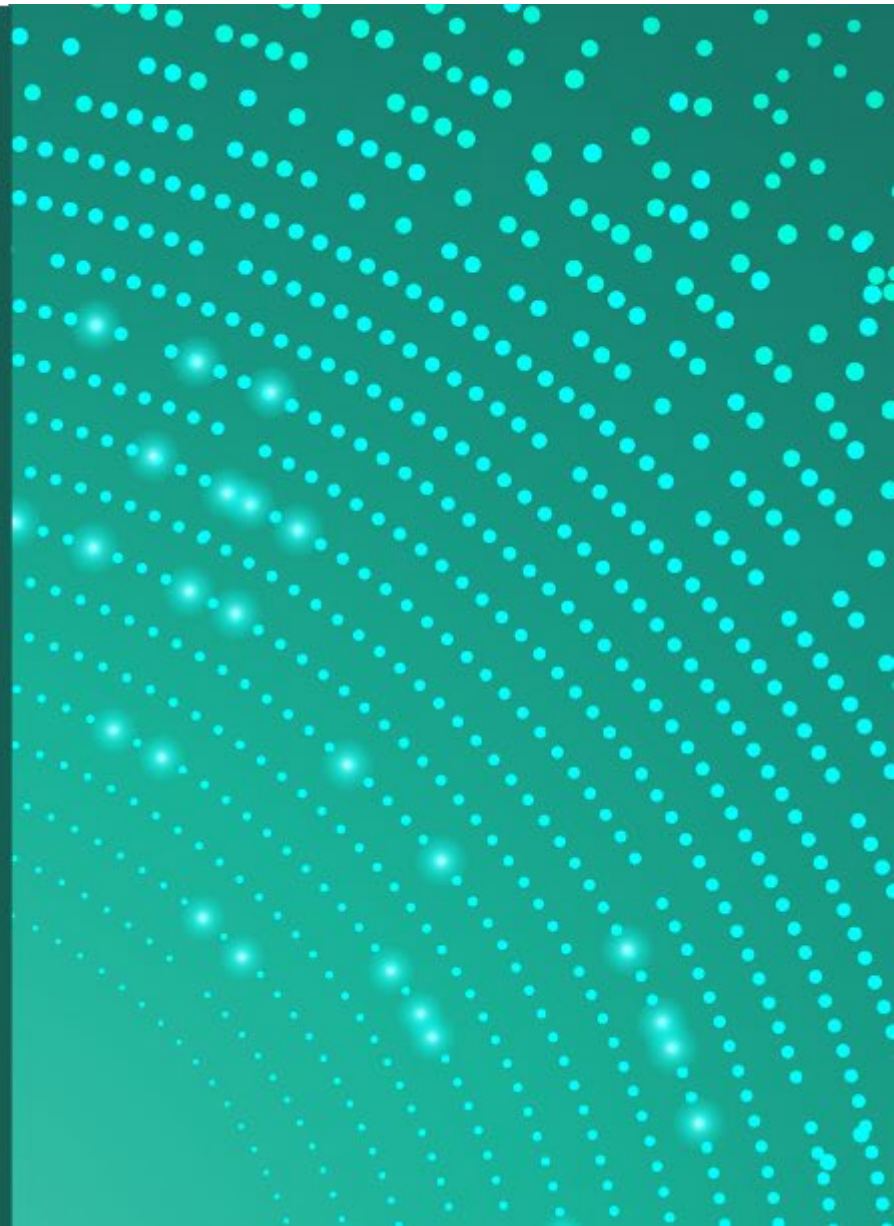
Heads of Medicines Agencies

Veterinary Big Data Workplan 2022-2025



Network skills

Empowered the network through digital and big data training offers

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

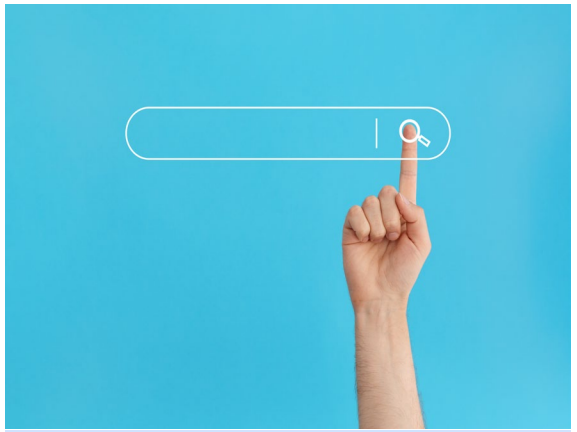
- Input sought on data quality framework, reflection papers on RWE and AI, network data strategy
- Workshops included patient registries, RWE methods, AI and pharmacogenomics

Stakeholders kept informed

In writing

Big Data Highlights

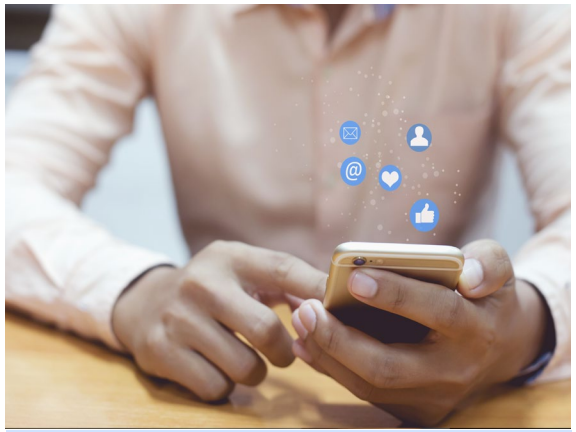
Targeted stakeholder mailing



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

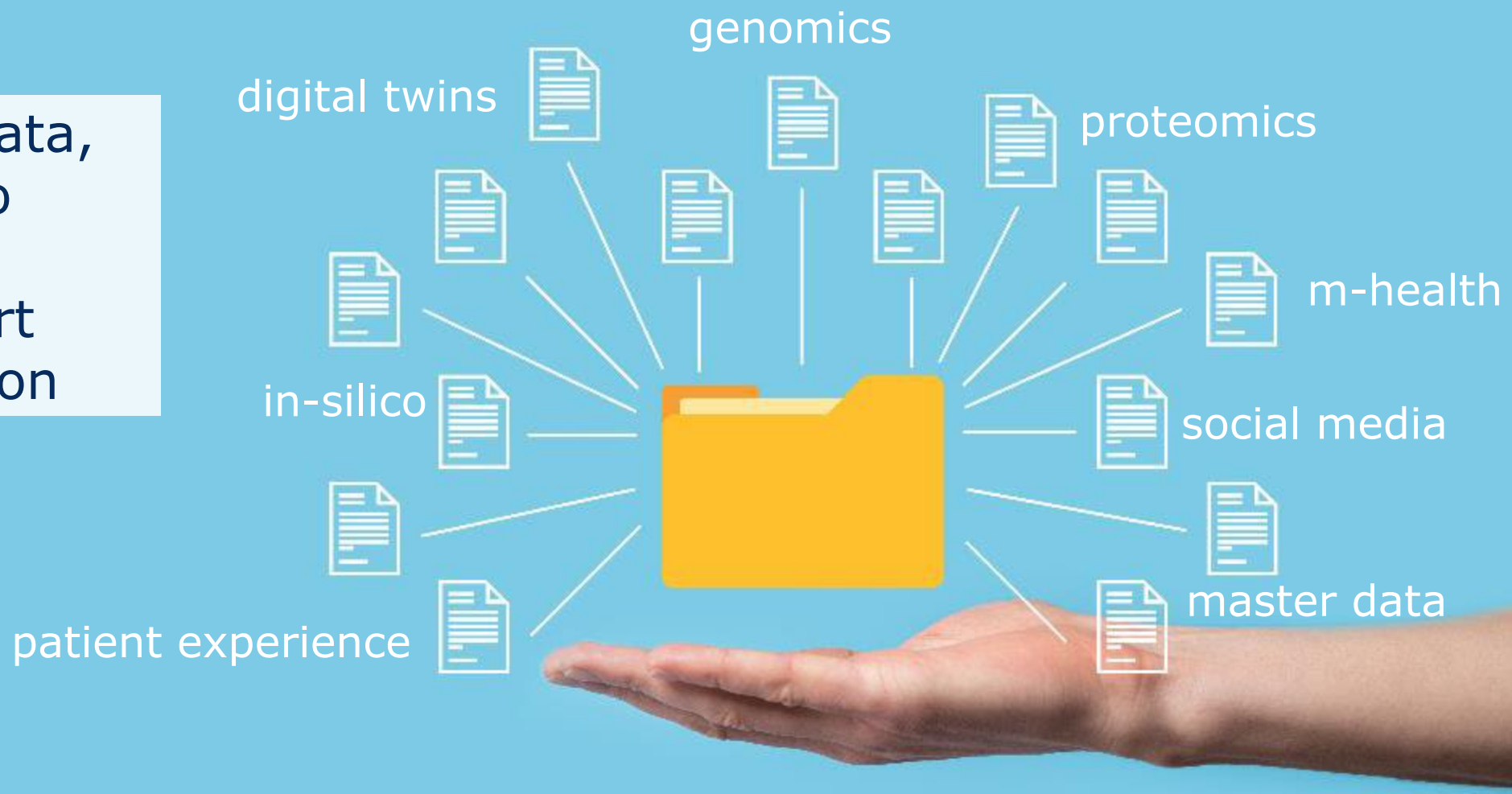
EU medicines regulatory network strategy 2028

- 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



Collaboration is key

Thank you.

 ema.europa.eu/bigdata

 @EMA_News

 European Medicines Agency

 Send a question via our website