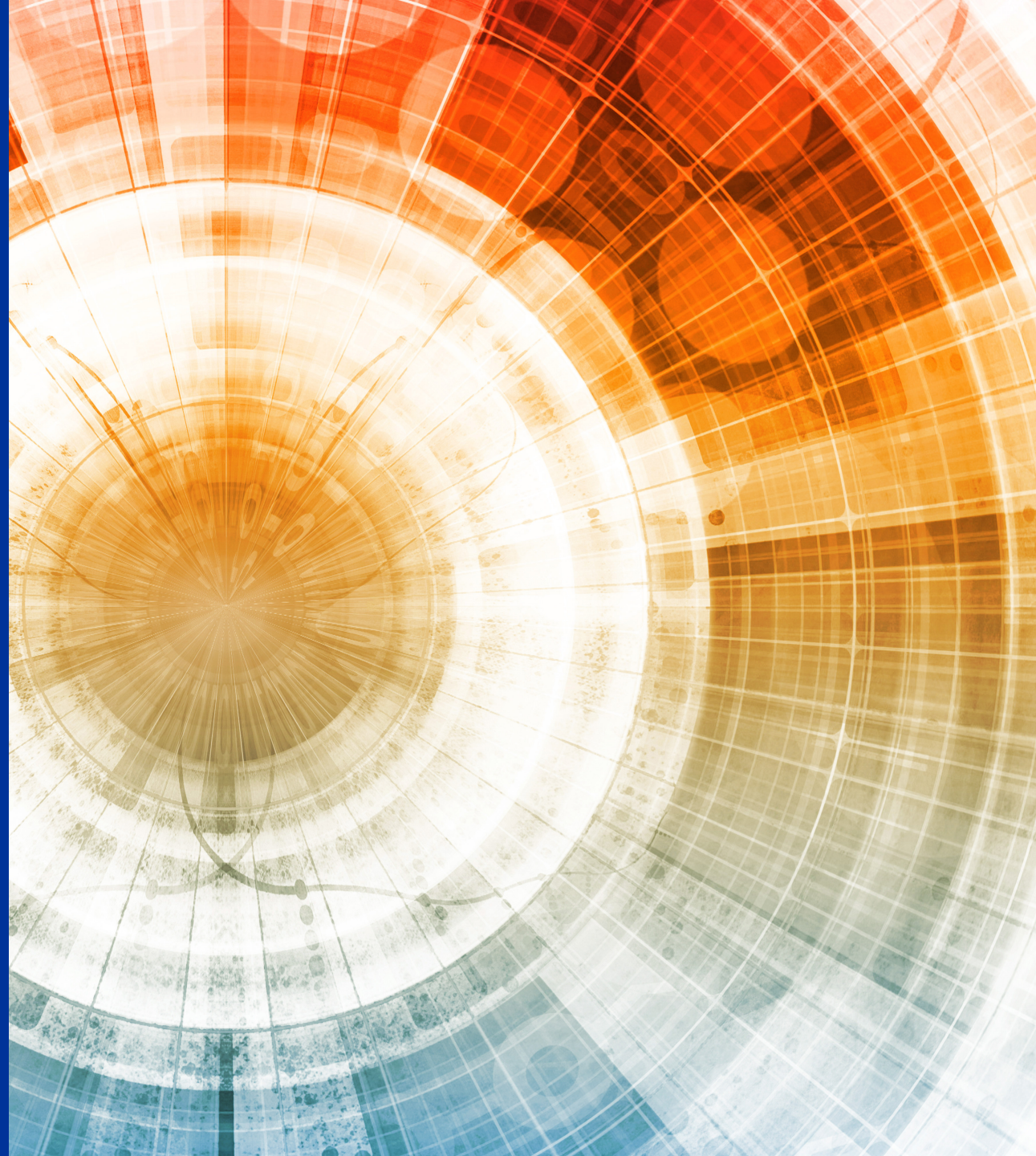
EMA
EUROPEAN MEDICINES AGENCY

European Landscape of Clinical Research and Health Care – an EMA update

CDISC EU Interchange 2025
May 14, 2025

Presented by Eftychia-Eirini Psarelli

Data Analytics and Methods Task force, European Medicines Agency



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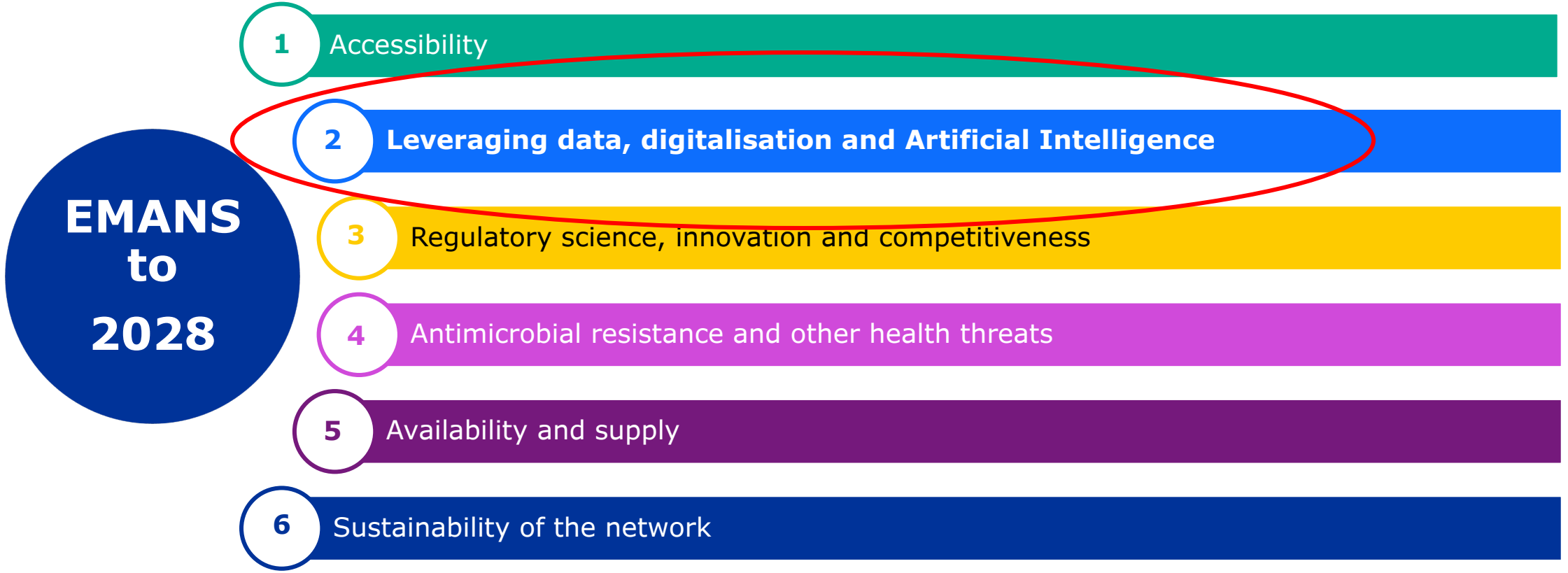
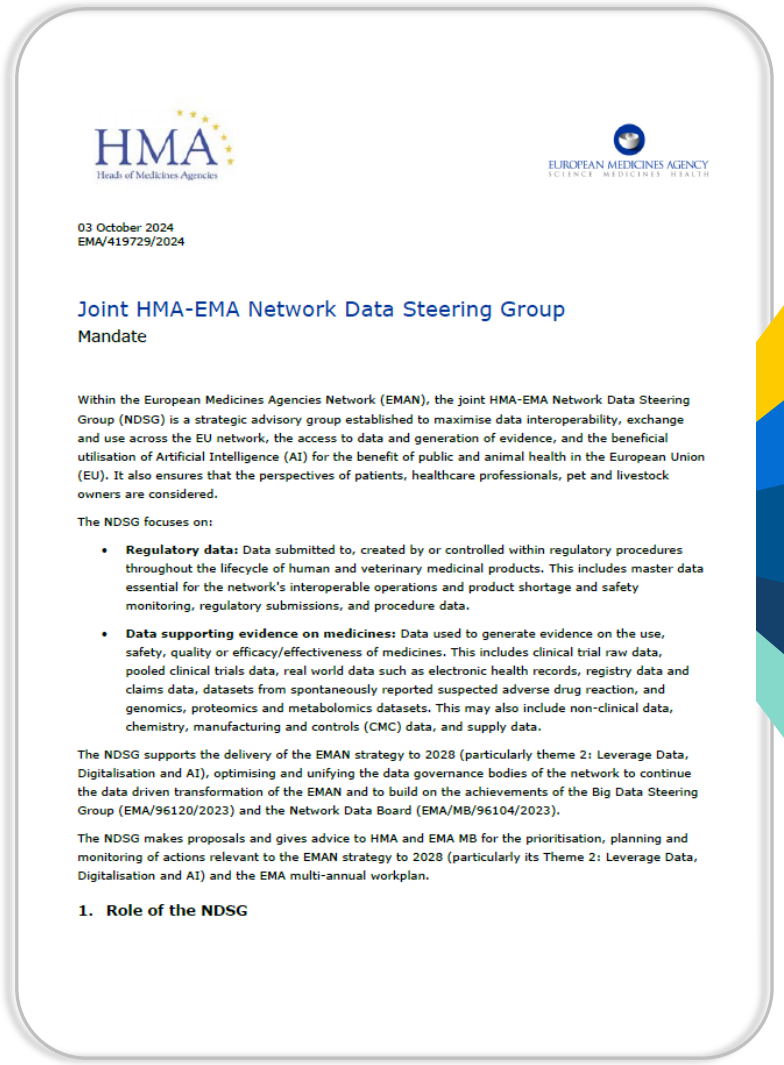


HMA-EMA Network Data Steering Group mandate (est. 2025)



HMA
Heads of Medicines Agencies

EUROPEAN MEDICINES AGENCY
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03 October 2024
EMA/419729/2024

Joint HMA-EMA Network Data Steering Group
Mandate

Within the European Medicines Agencies Network (EMAN), the joint HMA-EMA Network Data Steering Group (NDSG) is a strategic advisory group established to maximise data interoperability, exchange and use across the EU network, the access to data and generation of evidence, and the beneficial utilisation of Artificial Intelligence (AI) for the benefit of public and animal health in the European Union (EU). It also ensures that the perspectives of patients, healthcare professionals, pet and livestock owners are considered.

The NDSG focuses on:

- **Regulatory data:** Data submitted to, created by or controlled within regulatory procedures throughout the lifecycle of human and veterinary medicinal products. This includes master data essential for the network's interoperable operations and product shortage and safety monitoring, regulatory submissions, and procedure data.
- **Data supporting evidence on medicines:** Data used to generate evidence on the use, safety, quality or efficacy/effectiveness of medicines. This includes clinical trial raw data, pooled clinical trials data, real world data such as electronic health records, registry data and claims data, datasets from spontaneously reported suspected adverse drug reaction, and genomics, proteomics and metabolomics datasets. This may also include non-clinical data, chemistry, manufacturing and controls (CMC) data, and supply data.

The NDSG supports the delivery of the EMAN strategy to 2028 (particularly theme 2: Leverage Data, Digitalisation and AI), optimising and unifying the data governance bodies of the network to continue the data driven transformation of the EMAN and to build on the achievements of the Big Data Steering Group (EMA/96120/2023) and the Network Data Board (EMA/MB/96104/2023).

The NDSG makes proposals and gives advice to HMA and EMA MB for the prioritisation, planning and monitoring of actions relevant to the EMAN strategy to 2028 (particularly its Theme 2: Leverage Data, Digitalisation and AI) and the EMA multi-annual workplan.

1. Role of the NDSG

Strategy & Data Governance
Advisory reference point for data related matters; Ensuring proportionate data governance for managing EMAN data assets; Proposing and agreeing EMAN strategies and related implementation plans, recommendations, positions; Contributing to implementation of relevant EU legislative initiatives; Fostering international collaboration, alignment and harmonization; Support Network IT Portfolio; Horizon Scanning

Interoperability
Enabling effective management and use of high quality data; Enabling high levels of interoperability and exchange of data through the use of standards, terminologies and master data

Data analytics
Maximising evidence generation via access to data/analysis of RWD (DARWIN EU), CT study data, other type of data (i.e. patient experience data, genomic data…); Piloting new analytical approaches

Artificial Intelligence
Overseeing the work to realise the EMAN vision to harness AI capabilities for personal productivity, process automation and systems efficiency, data insights and strengthened decision-support.

Change management, guidance and international initiatives
Network guidance, reflection papers, Big data training, Stakeholders' events (workshops, forum, webinars, masterclass, deep dive…), learnings and integration with business processes

DARWIN EU®

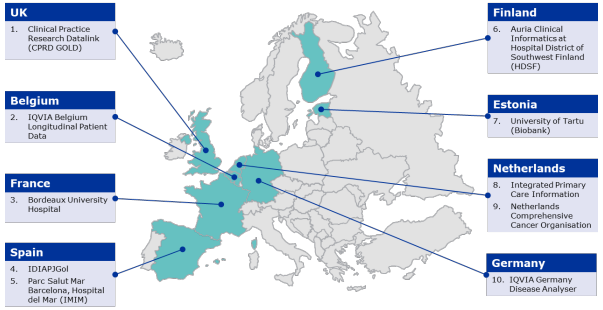
RWE use throughout medicinal product lifecycle

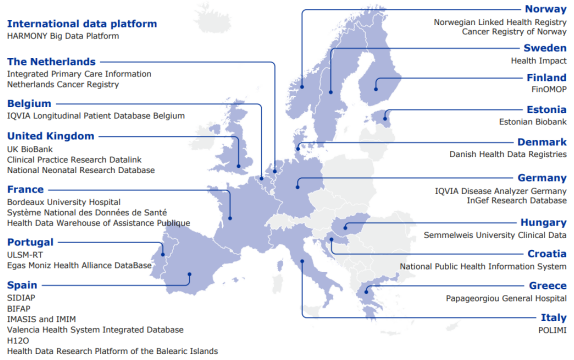
DARWIN EU® vision

The vision that the EMA had when DARWIN EU® was designed is really now in action to support core medicine regulation, as well as public health in the EU

For this, we will continue to optimise the way we generate and deliver evidence to the EMA, its committees, the network and other stakeholders, to make it even **better, faster and smarter** and with more and more data from the majority of the European countries

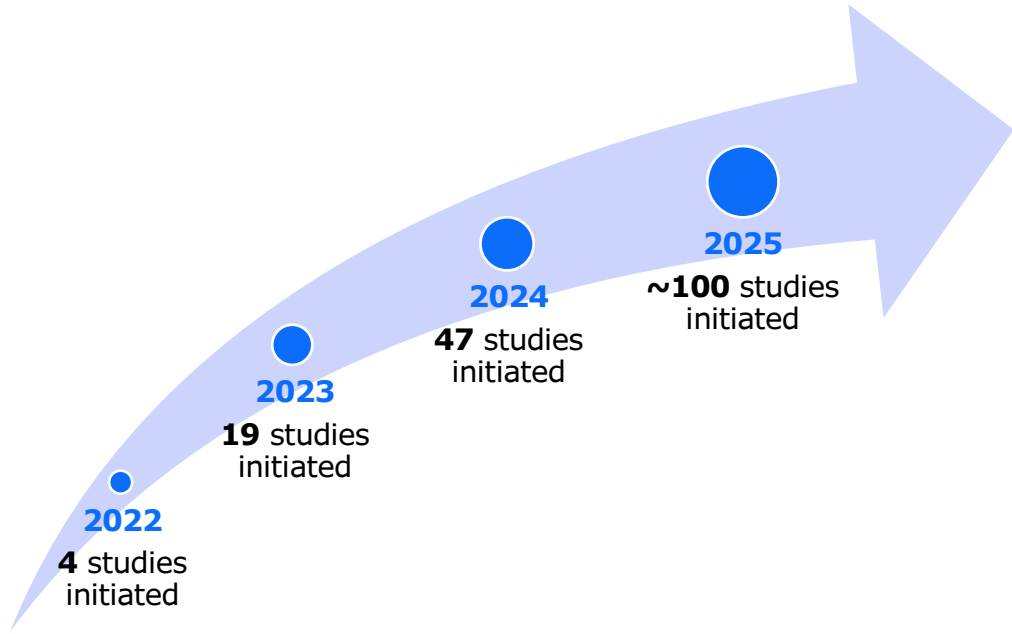
End of 2022



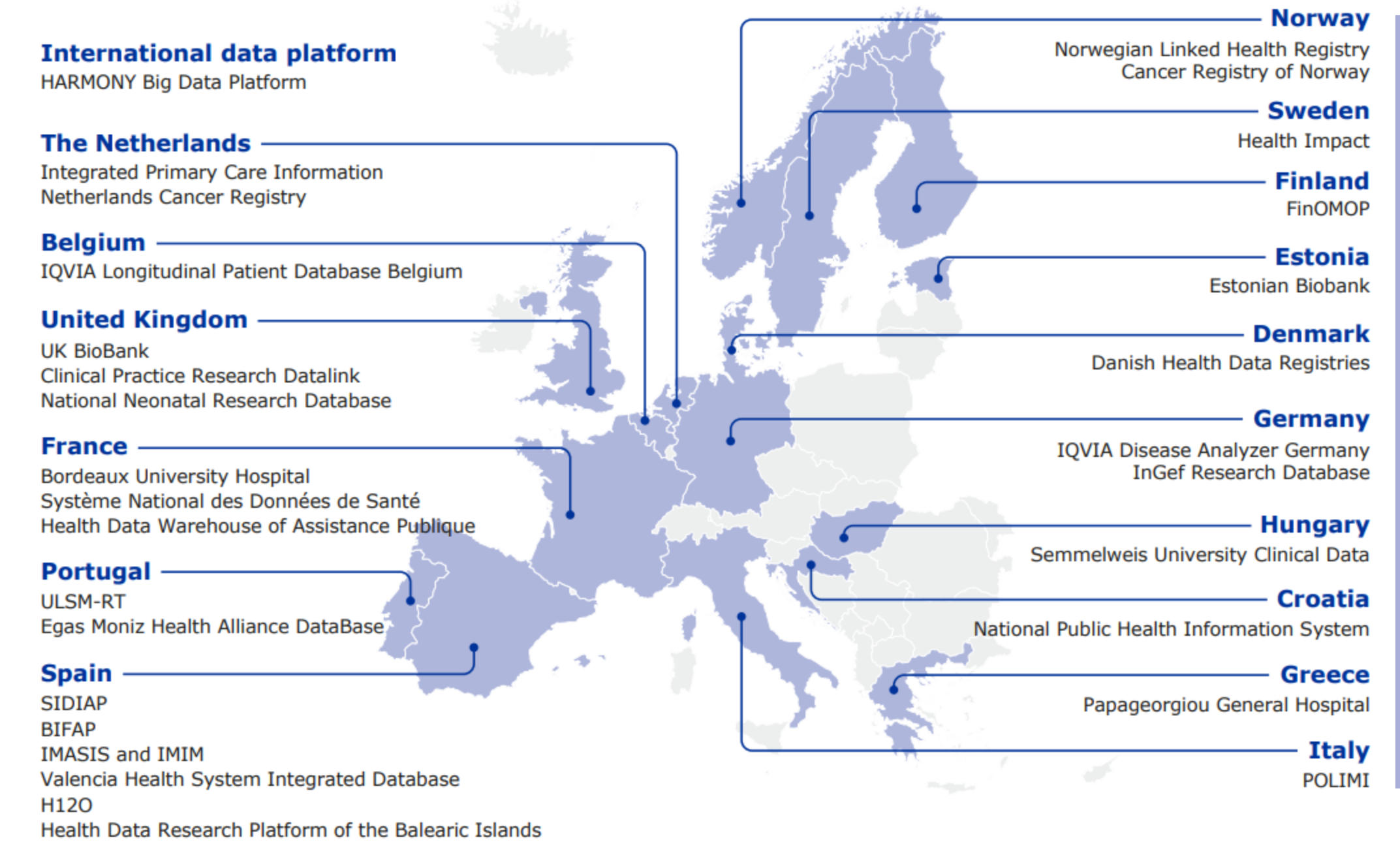


End of 2024

From **~26** to **~180** million active patients



DARWIN EU Network of Data Partners



International data platform
HARMONY Big Data Platform

The Netherlands
Integrated Primary Care Information
Netherlands Cancer Registry

Belgium
IQVIA Longitudinal Patient Database Belgium

United Kingdom
UK BioBank
Clinical Practice Research Datalink
National Neonatal Research Database

France
Bordeaux University Hospital
Système National des Données de Santé
Health Data Warehouse of Assistance Publique

Portugal
ULSM-RT
Egas Moniz Health Alliance DataBase

Spain
SIDIAP
BIFAP
IMASIS and IMIM
Valencia Health System Integrated Database
H12O
Health Data Research Platform of the Balearic Islands

Norway
Norwegian Linked Health Registry
Cancer Registry of Norway

Sweden
Health Impact

Finland
FinOMOP

Estonia
Estonian Biobank

Denmark
Danish Health Data Registries

Germany
IQVIA Disease Analyzer Germany
InGef Research Database

Hungary
Semmelweis University Clinical Data

Croatia
National Public Health Information System

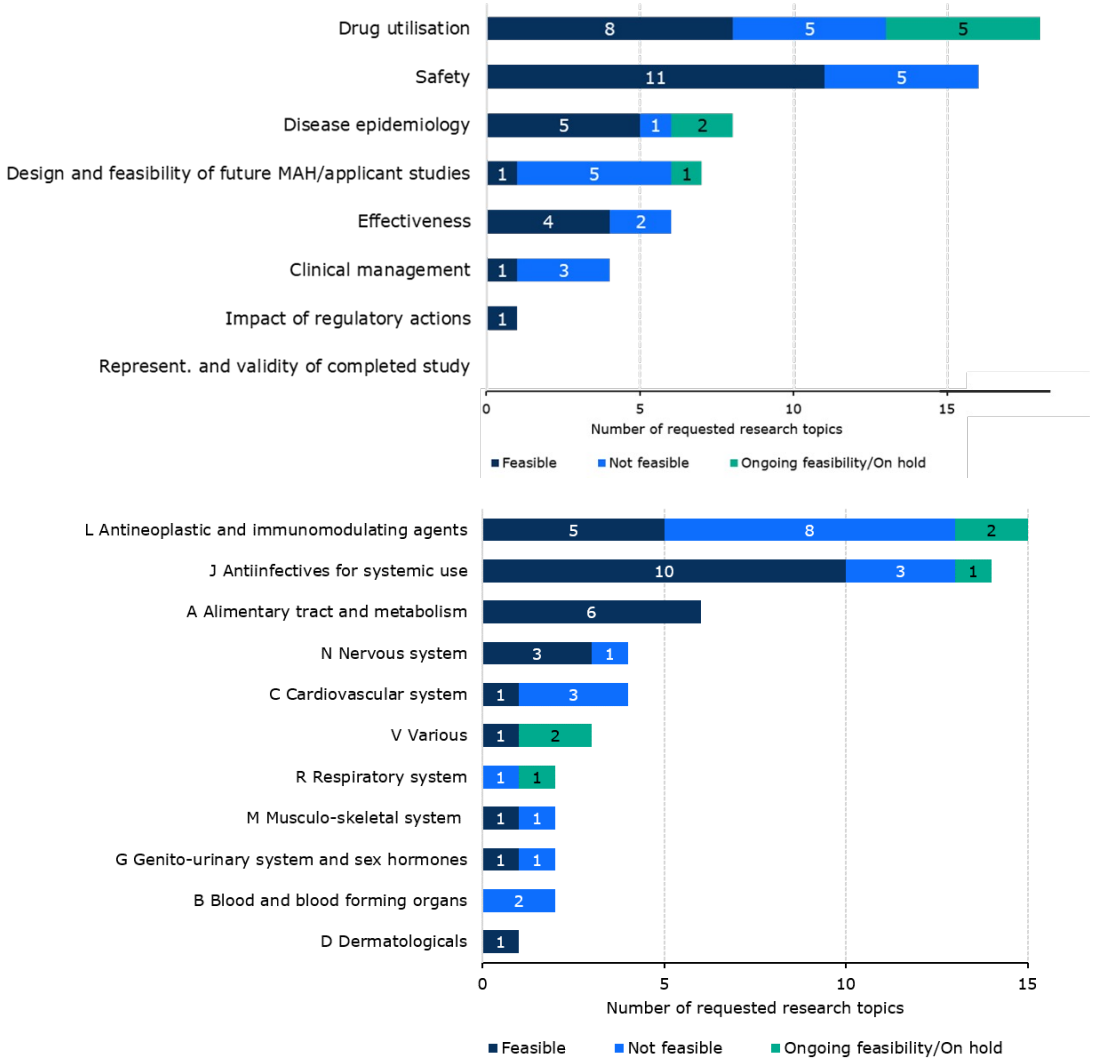
Greece
Papageorgiou General Hospital

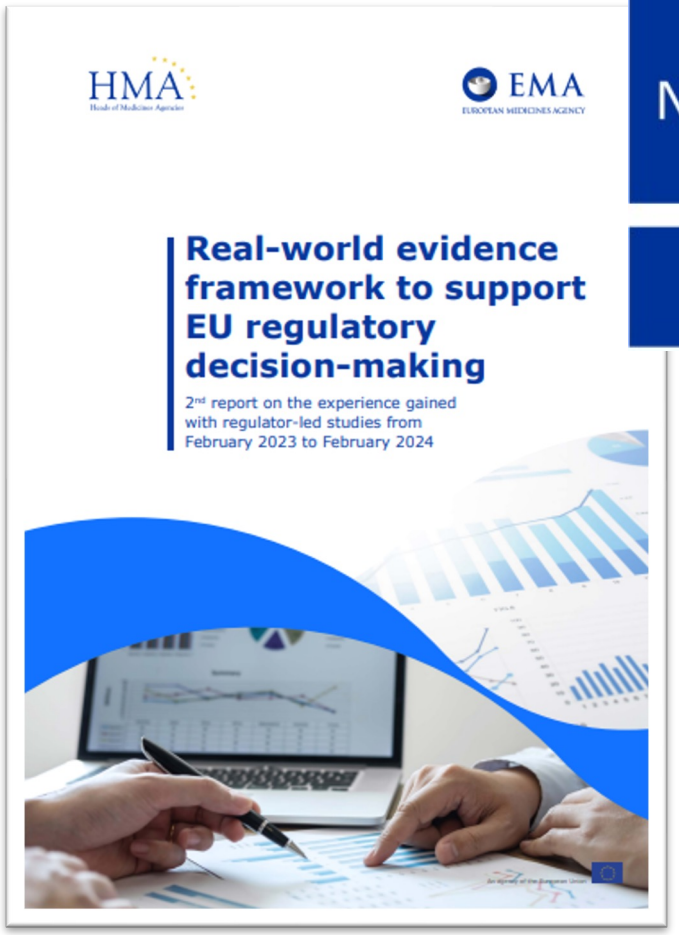
Italy
POLIMI

30 Data Partners as of Feb 2025 in **16 European countries** (~ +10 by end of Feb 2026)

EMA

Reports on RWE experience





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NEW research topics

(Feb '23 – Feb '24)

38	16	6
DARWIN EU	In-house	FWC

Published in July 2024

Period covered: **Feb 2023 to Feb 2024**

Clinical study data submission

A proof-of-concept pilot, launched in 2022

Expected benefits of clinical study access and analysis

Purpose of the pilot

Determine the **benefits of early clinical study data access** (at time of submission) **and analysis** to support the scientific assessment of medicinal products; identify the **target operating model***, **capacity and capability requirements**, and **technical requirements** for receiving, validating, storing, managing and analysing clinical study data.

Expected benefits of the clinical studies' data analysis for selected key stakeholders

EU patients

- Faster access to innovative, safe and effective medicines
- Improved confidence in regulatory decision-making
- Refined product labelling/targeting of subgroups within the recommended indications
- Facilitation of cross-product analyses

Network / EU health agencies

- Improved understanding of clinical study results to inform regulatory decision making
- Reduced need to put questions of data interpretation to the applicant/MAH
- Facilitation of cross-product analyses
- Optimised use of inspections

Applicants/MAHs

- Workload reduction due to fewer complex questions
- Shorter clock-stops
- Earlier authorisation

EMA-CHMP Clinical study data pilot when it was initially designed

- Timeline: Approx. **10 regulatory procedures over 2-3 years** from September 2022 (interim report after half-way point).
- Scope: **Initial marketing authorisation applications** and **post-authorisation applications** (e.g., variations or extensions). No restrictions for clinical characteristics of dossier.
- Participation: Procedures are based on **voluntary participation of CHMP Rapporteur teams** and **applicants/marketing authorisation holders (MAHs).**
- Usage: **Three analysis objectives** including Clinical Efficacy & Safety, Pharmacokinetic-Pharmacodynamic (PK-PD) and Good Clinical Practice (GCP) site selection.
- Resources: Three **resourcing scenarios for data analysis** are explored: (1) the Rapporteurs' assessment team, (2) EMA or (3) EMA contractor.

Procedures included in the pilot

Pilot's half-way point *reached in December 2023…*

Procedure number	Therapeutic Area	Type of procedure
1	*Neurology*	*iMAA-Full MAA*
2	*Endocrinology*	*iMAA-Biosimilar*
3	*Oncology*	*Post auth. Type II variation*
4	*Dermatology*	*Post auth. Type II variation*
5	*Gastroenterology*	*iMAA-Full MAA*

...up to now, May 2025

Procedure number	Therapeutic Area	Type of procedure
6	*Oncology*	*iMAA-Biosimilar*
7	*Gastroenterology*	*iMAA-Full MAA*
8	*Oncology*	*iMAA-Full MAA*
9	*Immunology*	*iMAA-Full MAA*
10	*Oncology*	*iMAA-Full MAA*

→ Pilot interim results based on **feedback collected from pilot participants** (e.g. Rapporteur teams, applicants/MAHs, etc.) published in October 2024

Key preliminary learnings as reported by pilot participants

Added value for assessment and decision making

- **Fewer questions to the applicant/MAH;** resolved with clinical study data analysis [potential to reduce overall assessment time]
- Improved **understanding of the information submitted** in the MAA dossier [potential for better opinion on indications, CIs and warnings]
- **Consensus** on methodological issues **amongst Rapporteurs** [potential to reduce outstanding issues during decision-making discussion]
- Potential to **optimise** the use of limited **inspection resources** [shorter time needed to plan and conduct inspections]

Capacity and capability

- **Additional EMRN expertise needed** in the field of statistical programming, PK-PD modelling, biostatistics, and **clinical trial data standards** [training]
- Conduct of tasks on clinical study data still **allowed assessment to be performed** according to timelines

Governance and processes

- **Most resourcing scenarios** tested successfully for analyses supporting the clinical efficacy and GCP routine inspection
- **Non-Rapporteur NCAs appear engaged,** with provision of Member State comments based on clinical study data analyses
- **CDISC data package requirements (SDTM, ADaM)** by other international regulators deemed suitable [no additional work for applicants]

Technical aspects

- **Data receipt, storage and analytics infrastructure** for EMRN will require optimisation to upscale
- **Choice of software under investigation** for all areas: clinical efficacy, PK-PD modelling, GCP [established off-the-shelf options available]

Clinical study data pilot phases and timelines

Pilot's initial timeline

September 2022

Start of the pilot

Q3-Q4 2024

Interim pilot report

Q4 2025

Final pilot report

Pilot extension

New

2025 onwards

Pilot extension and preparatory work for potential implementation

New

Q4 2026

Follow-up pilot report

2028+

New EU Pharmaceutical Legislation comes into force (estimate)

Pilot's extension focus areas

Register your interest to participate in the pilot with a specific procedure via **rawdatapilot@ema.europa.eu**

Intensify exploration of systematic use of clinical study data in support of regulatory assessment and decision-making

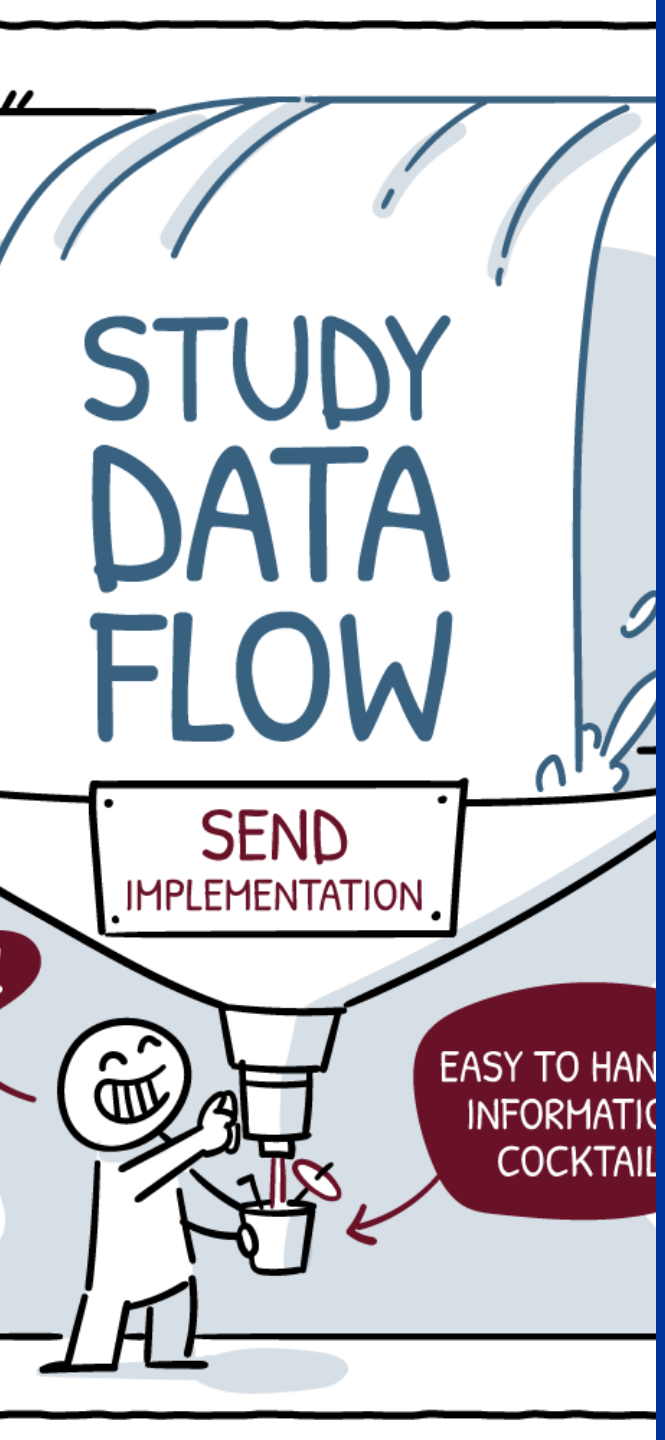
- Extent of **data interrogation** will vary – e.g. depending on dossier's quality
- Extent of **statistical analysis** will vary - e.g. for selected complex dossiers, or dossiers where applicant/MAH is not cooperating

Explore **IT solutions** in 2025/2026 (building on existing platforms):

- Clinical study data **receipt, storage and analytics infrastructure** for EMRN
- **Automated processes** for systematic data package characterisation incl. data validation
- Explore submission of **JSON** transport file format

Change management activities to intensify:

- Awareness of stakeholders and foster **knowledge sharing** (leverage EMRN fora, workshops)
- Production/update of **process and data guidance**
- **Training**



Submission of non-clinical data in SEND format

A proof-of-concept study, initiated in 2024, continuing in 2025

Background

Goal

Proof-of-concept study to evaluate if non-clinical information in SEND format will improve **quality, consistency** and **efficiency** of assessments

Expected benefits

- Improved and more consistent assessment **quality**
- More **science driven** & less **data driven** questions to Applicant
- **Fewer non-clinical rounds** and/or **faster completion** of the non-clinical dossier's review

SEND requirements by international regulators

Use	Data exchange standard	Regulatory agency	Date support start	Date requirement start
Animal study datasets	3.1	CDER	Aug 2017	March 2019
		CBER	March 2021	March 2023
		EMA	Jan 2024	N/A - **voluntary**

What is happening in 2025

Since January 2024 applicants are encouraged to submit their (already available) non-clinical SEND data packages as part of their eCTD MAA submission (64 iMAAs)

Since 2025 applicants that did not submit SEND with their (initial) MAA submission are contacted with a request to submit in a subsequent submission (15 iMAAs)

- 5/64 (8%) of applicants submitted their SEND data package as part of their initial eCTD MAA submission
- 8/15 (53%) of applicants submitted a SEND data package as a follow-up submission
- 7/15 that did not submit had no SEND data (biological, vaccines and radioligand)

For additional information you can reach out to: SEND@ema.europa.eu

‘‘Trusted medicines by unlocking the value of data’’

Joint HMA-EMA Network Data Steering group’s vision

Appendix

Useful links

- The European medicines agencies network strategy 2028
- Data in regulation: Big data and other sources | European Medicines Agency (EMA)
- Data Analysis and Real World Interrogation Network | European Medicines Agency (EMA)
- Use of clinical study data in medicine evaluation | European Medicines Agency (EMA)

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Thank you

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