Big Data Steering Group ongoing activities

20 November 2024

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Key progress

BDSG progress update: selected highlights

DARWIN EU®	• DARWIN EU is operational and has access to data from approximately **130 million patients from 13 European countries.** • The Industry focus group on RWE was established and had a kick-off meeting in Sept 2024.
DATA QUALITY & REPRESENTATIVENESS	• The network consultation of the **Real-world data quality** chapter for medicine regulation was completed in May. A public consultation will be launched before end of 2024. FOR YOUR **INPUT**
DATA DISCOVERABILITY	• EMA-HMA catalogues of real-world data sources & non interventional studies (real-world studies) were launched in February 2024. • **A review of utility of mobile health and social media data was conducted and reports are being published.**
EU NETWORK SKILLS	• Five modules of the **Data Science curriculum** (Big Data essentials, AI, Data Management, Data Visualisation, Introduction to Data Science) were **released via EU NTC in September** 2024.
EU NETWORK PROCESSES	• BDSG has published its **second report on the experience gained in conducting studies with real-world data (RWD).** The report covers period from February 2023 to February 2024.
NETWORK CAPABILITY TO ANALYSE	• The **EMRN computing capabilities to analyse data survey** was completed in May 2024. • The 1st AI-enabled Scientific Explorer knowledge mining tool for EU regulators was launched in March 2024.

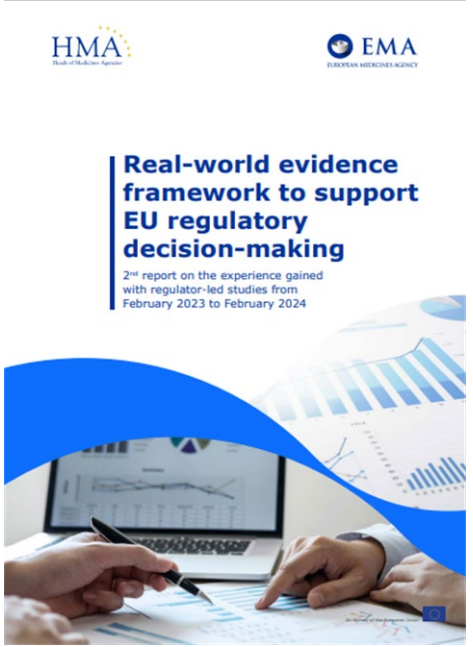
BDSG progress update: selected highlights

DELIVERY OF EXPERT ADVICE	• A **public consultation of a draft RWE reflection paper** conducted between May-August 2024. • **RWE and AI Special Interest Area** of the Methodology Working Party have been **established**.
GOVERNANCE FRAMEWORK	• The Network data governance review initiated. The BDSG and EU NDB will be unified into 1 data governance body, the Network Data Steering Group (NDSG). • **The draft EU Network data strategy will be released for public consultation in October** 2024, aiming to have the document finalised in early 2025. FOR YOUR **INPUT**
INTERNATIONAL INITIATIVES	• **ICMRA RWE for public health emergency group was established in July 2024**. • The **ICH reflection paper on RWE was adopted in June 2024**.
STAKEHOLDER ENGAGEMENT	• **Five workshops were delivered** under the joint HMA-EMA Big Data Steering Group workplan. • The change management activities overview was published in July. • **A BDSG survey on stakeholder communication and engagement was launched** on 19 September. (JointHMA-EMABDSGworkplansurvey – open until end of this year). FOR YOUR **INPUT**
VETERINARY RECOMMENDATIONS	• Fourth **veterinary big data stakeholder forum will take place on 14 October 2024**.

Selected highlights

RWE learnings publication – second report published

- BDSG has published its second report on the experience gained in conducting studies with real-world data (RWD).
- The report covers the period from February 2023 to February 2024.
- A total of 41 studies were conducted (completed or ongoing), supporting an extended range of decision-makers.
- The report includes a progress update on the previous recommendations, lessons learned and proposals for further actions.



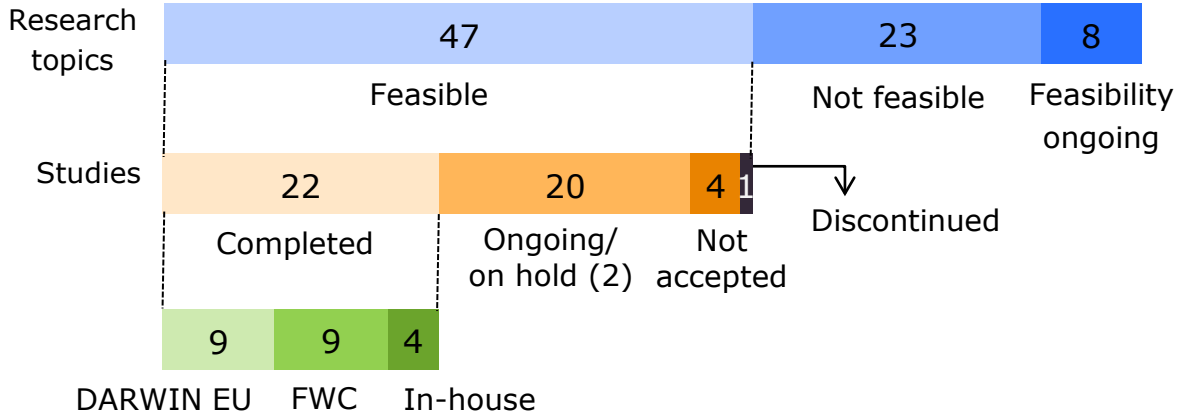
Second report – Number of study requests

60

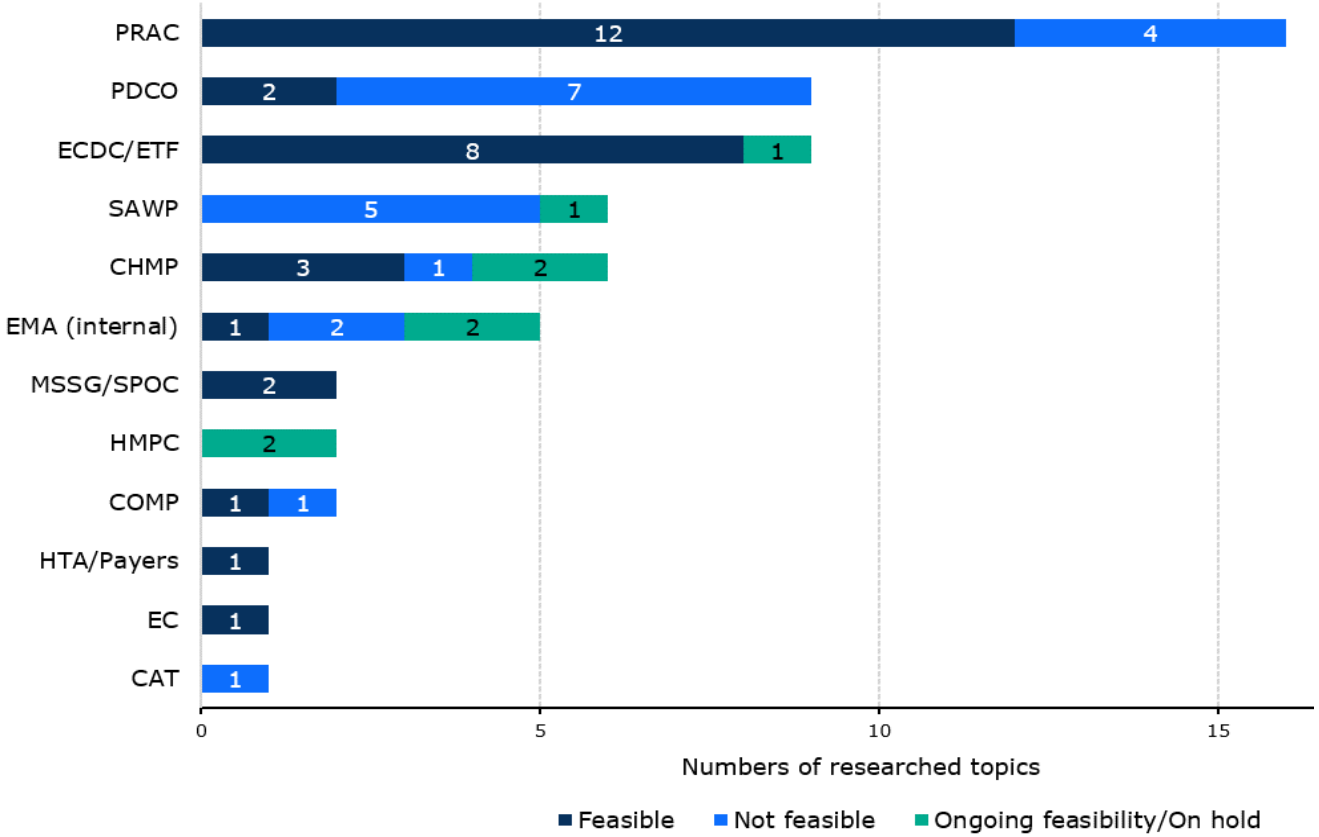
NEW research topics
(Feb ‘23 – Feb ‘24)

+18
Prior research topics

38	16	6
DARWIN EU	In-house	FWC



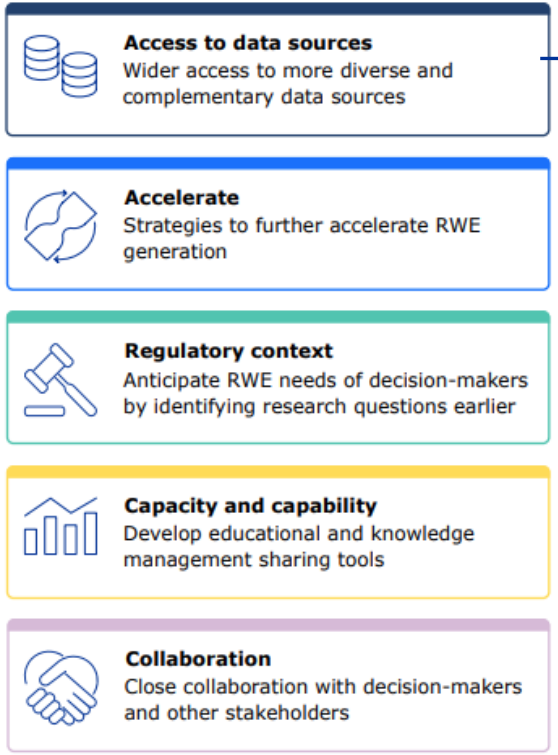
NEW study topics (n=60) by 'decision-maker'



Highlights

- **DARWIN EU 2nd year of establishment** completed
  - 20 data partners → 130 million patients from 13 European countries
  - Main RWE generation pathway for studies to support regulatory decisions
- **40 studies** completed (22) or ongoing (18), including 13 studies to inform vaccine safety and effectiveness, and public health emergencies
- For the first time, studies conducted
  - to support monitoring of the **demand and stock levels of critical human medicines**
  - on **herbal** substances
  - for **HTA and payer** organisations
  - to support EMA's **geriatric medicines** strategy

Recommendations – progress report (**example**)



Implementation status:

- DARWIN EU network of data sources increased from 10 to **20 data partners**, covering now
  - **15 European countries** and over **130 million patients**

Further actions:

- **Continue the growth of DARWIN EU**, focusing on more **specialised data sources** including data in special populations, rare diseases and oncology
- Strengthen the **outreach to data/registry owners** to facilitate access to data for secondary use

Use of clinical study data in medicine evaluation: Interim report

- The clinical trial raw data analysis interim report reviews the current experience with analysis of CT raw data and provides preliminary recommendations for end of pilot and post-pilot activities.
- A survey to gather feedback from all pilot participants was conducted in December 2023: 60 participants, 64% response rate, **very positive feedback on feasibility and utility of raw data analysis.**
- Interim report is published on EMA website.
- EMA and HMA extended the duration of the pilot to continue to gather valuable insights.

Raw data pilot overview

Timelines: Approx. **10 regulatory procedures over 2 to 3 years** from September 2022.

Procedure scope: **Initial marketing authorisation applications** and **post-authorisation applications**.

Participation: Procedures are based on **voluntary participation of CHMP Rapporteur teams** and **applicants/Marketing authorisation holders**.

Analysis scope: **Three analysis objectives** including Clinical Efficacy & Safety, Pharmacokinetic-Pharmacodynamic (PK-PD) and Good Clinical Practice (GCP) site selection.

Resourcing scenarios: **Three resourcing scenarios** for data analysis being explored: the CHMP Rapporteur teams, EMA staff and/or EMA contractors (DKMA).

** CHMP – Committee for Medicinal Products for Human Use*

The Big Data Steering Group will evolve in 2025: Joint HMA/EMA Network Data Steering Group

- **Key drivers:**
  - Review of the EMAN Strategy to 2028 (Theme 2: Leverage **data**, digitalisation and **AI**)
  - Drafting of EMRN Data Strategy: Need for one governance group to oversee the implementation data governance artefacts
- **Role**:
  - 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 AI.
- **Scope**:
  - Regulatory data: Data submitted to, created by or controlled within regulatory procedures throughout the lifecycle of human and veterinary medicinal products, e.g. 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, e.g. clinical trial raw data, real world data such as electronic health records, registry data and claims data, genomics….

Joint HMA/EMA Network Data Steering Group – mandate

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 raw data, other data type (i.e. PED, genomic…); Piloting innovative design of clinical studies and 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.

Stakeholders' engagement and network capability/capacity

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

Joint HMA/EMA Network Data Steering Group – membership

- Membership continues to include representatives from EU patient association (1) and EU Healthcare professional association (1) – Call for membership open
- Competencies:
  - Methodological expertise including biostatistics, modelling and simulation, data science, AI/ML, and specifically with a focus on applications in design and analysis of clinical studies, as well as business support
  - Data management, standardisation and interoperability with insight in data needs of the business and the technological constraints and opportunities of the existing IT infrastructure and applications
  - Familiarity with data protection, data security, EU data legislation and policies
- Will be appointed until end of 2028, in line with EMANS to 2028. Aim for kick off face-to-face meeting January 2025.

AI workplan delivery: key highlights

- BDSG continues oversee implementation of the BDSG Multi-annual AI workplan 2023-2028.
- Key progress:
  - Final AI Reflection Paper - published on 30 Sept 2024.
    - Topics selected for guidance development will be announced as part of publication of the updated MWP Consolidated 3-year rolling work plan
  - Preparation started to support the implementation of the AI Act: liaison with DG SANTE
  - Guiding principles on Use of large language models (LLMs) - published on 5 Sept 2024.

Expert reports on mHealth data and social media data

- mHealth data report:
  - Acknowledging challenges in terms of data quality and protection of patient privacy, mHealth tools such as smartphones, health applications, smartwatches and other wearables can generate a large variety of detailed patient data like heart rate and body temperature.
  - Found to be useful as a complementary source of data for EU medicine regulation in three domains: 1) to support planning and validity of applicant studies, 2) to support the understanding of clinical context, and 3) to investigate associations of products on safety and efficacy outcomes and impacts of regulatory actions.
- Social Media data report:
  - Despite challenges related to access, quality and ethical use of social media data, various patient data can be extracted from social media, including demographic data, patient experience data (PED), and data related to drug use and disease factors.
  - Potential utility of certain social media data as a complementary source of data to generate evidence, especially for some niche cases like abuse/misuse, misinformation.
  - We will continue to monitor progress in this area, under the umbrella on the work that the Network is doing on Patient Experience Data and support relevant research initiatives.
- The reports propose a set of points for consideration for future actions to increase the utility of mHealth data and social media data in regulatory decision-making. Might be used as reference/starting point for new work, might be considered in the future workplan 2025-2028, in consultation with NDSG and PCWP/HCPWP.

Fifth EMA/HMA Big Data Stakeholder Forum

28 November 2024

- Agenda: Fifth EMA/HMA Big Data Stakeholder Forum | European Medicines Agency (EMA)
- Key sessions on:
  - Session 1: Implementation of the HMA/EMA Big Data Task Force priority recommendations
  - Session 2: Evidence generation to advance regulatory excellence, here and now: **RWD** and **clinical study data** pilot
  - Session 3: Evidence generation to advance regulatory excellence, preparing for tomorrow: **genomic** data, **mHealth** data, **social media** data
  - Session 4: Empower evidence generation with data strategy: Building a unified **Network data strategy** for operational excellence
- Register now for online participation: open until **26 November**

Stay up to date

- Big Data webpage
- Big Data Highlights - **resubscribe** here
- EMA events
- Social media **@EMA_News**

Comments from Patient representative (George Paliouras) to BDSG:

CONTEXT:

- Patients and citizens in general have a vested interest in the use of data by EMA. Most importantly, they are the beneficiaries of the desired outcome; namely more efficient delivery of more effective treatments. Additionally, they are the main source of the actual data.
- In a world that is changing fast, BDSG is doing a tremendous effort to help EMA adapt and make the most of the new digital environments.
- The role of patients is critical in this effort, and we are trying to be present and make this dynamic process as relevant to the patients and society as possible.

ON THE BDSG ACTIVITIES:

- We are promoting the increased use of RWD, which will eventually provide a better picture of the situation of the patient. RWD have started complementing "experimental" CT data and we want it do more so.
- We are pushing for the sharing of data, especially where data is sparse and fragmented, e.g. rare diseases.
- We contribute to the shaping of quality criteria for data. In this context, we are also voicing concerns about the use of data that may introduce bias or noise, e.g. some social media.
- We are emphasising the importance of patient experience data and more generally data provided by patients. Such data should be collected in ways that do not add burden to patients and should be used effectively.
- We are seeking to increase awareness of patients and the society in general about the importance of data, the efforts of EMA, the impact of new technologies like AI, and the key role of patients.
- We disseminate patient-driven efforts to collect, organise and manage data. Such efforts could be combined with initiatives of the EMA.

FOR THE FUTURE:

- Patients can be involved more actively in RWD efforts, like DARWIN-EU, aiming to make decisions more relevant to their needs.
- The BDSG/NDSG can improve the communication of interesting efforts and results to society. This requires special effort.

Comments from HCP representative (Ioana Agache) to BDSG:

Background:

- Through the specific knowledge and expertise to offer healthcare professionals (HCPs) are important stakeholders in all EMAs activities. EMA plays an increasingly integral role as an agency not just dedicated to ensuring safe and effective products, but also to promote public health and participate more actively in the scientific research enterprise directed towards new treatments and interventions.
- The use of big data is necessary to improve the assessment of safety, efficacy, quality, and performance of EMA-regulated products and incorporate new digital environments into regulatory science.
- The partnership between EMA, HCPs, patients, and citizens is vital in analyzing complex data for practical and reliable systems.

As a HCP member of the BDSG:

- We contributed our expertise on the analysis of the multiple forms of healthcare data such as biomedical signals, genomic data, sensing data, biomedical images, and social media
- As healthcare activities generate large amounts of data, we shared our views on analytical procedures used in our field to derive actionable judgments from data management technologies.
- We helped increase awareness of HCPs and Academia about the efforts of EMA BDSG on assessing when and how to have confidence in novel technologies and the evidence generated from big data
- We contributed to the creation of data quality and representativeness framework providing specific advice on the accessibility, timeliness, relevance, and accuracy as a robust foundation for informed decision-making, strategic agility, and sustained success. The correct data must be collected at the right time and context for an effective data discovery process.

Upcoming challenges:

- Big data analytics has held the gap between structured and unstructured data. However, we need to overcome the shift to an integrated data environment. Data sharing, real-time processing, data privacy and security, and heterogeneous data analysis are yet to be tackled.
- The translation of the work of BDGS into intelligent healthcare and predictive analytics helping HCPs to select people who get the most benefit from pharmaceutical interventions and understand more about the side effects and risks of the medications in a model combining pharmacovigilance, phenotyping, and illness detection.