EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3.3 Data Analysis and Real World Interrogation Network (DARWIN-EU®)

PCWP/HCPWP joint meeting, 2 March 2022

Presented by Andrej Segec, EMA TDA-DAT

DARWIN EU

EUROPEAN MEDICINES AGENCY

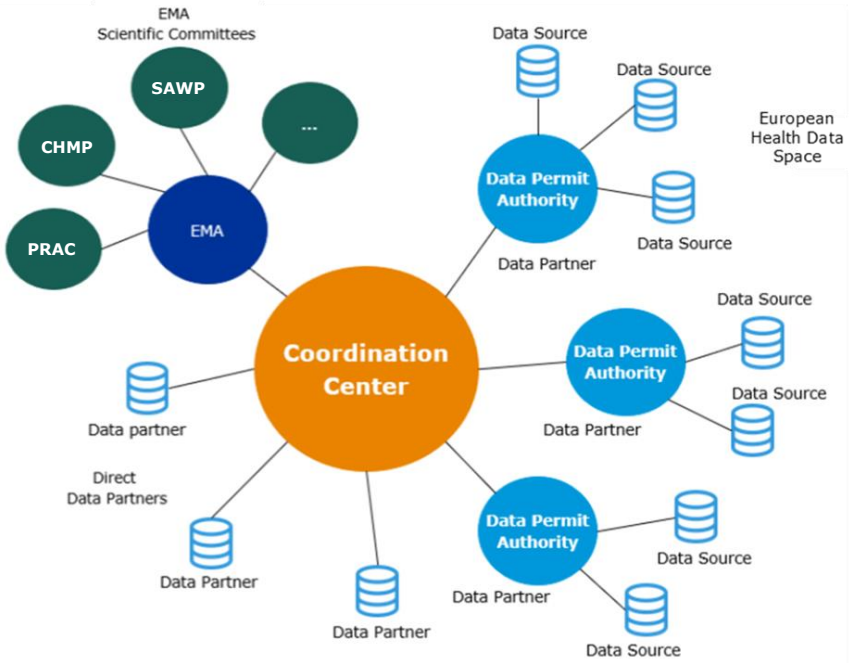
By 2025 the use of Real-World Evidence will have been enabled and the value will have been established across the spectrum of regulatory use cases

- European Medicines Regulatory Network (EMRN) strategy to 2025 -

DARWIN EU® is a federated **network** of **data**, **expertise** and **services** that supports better decision-making throughout the product lifecycle by generating reliable **evidence from real world healthcare data**

Coordination center:

- Erasmus University Medical Center Rotterdam perform the CC services
- Coordinates and maintains the network, data partners and performs studies



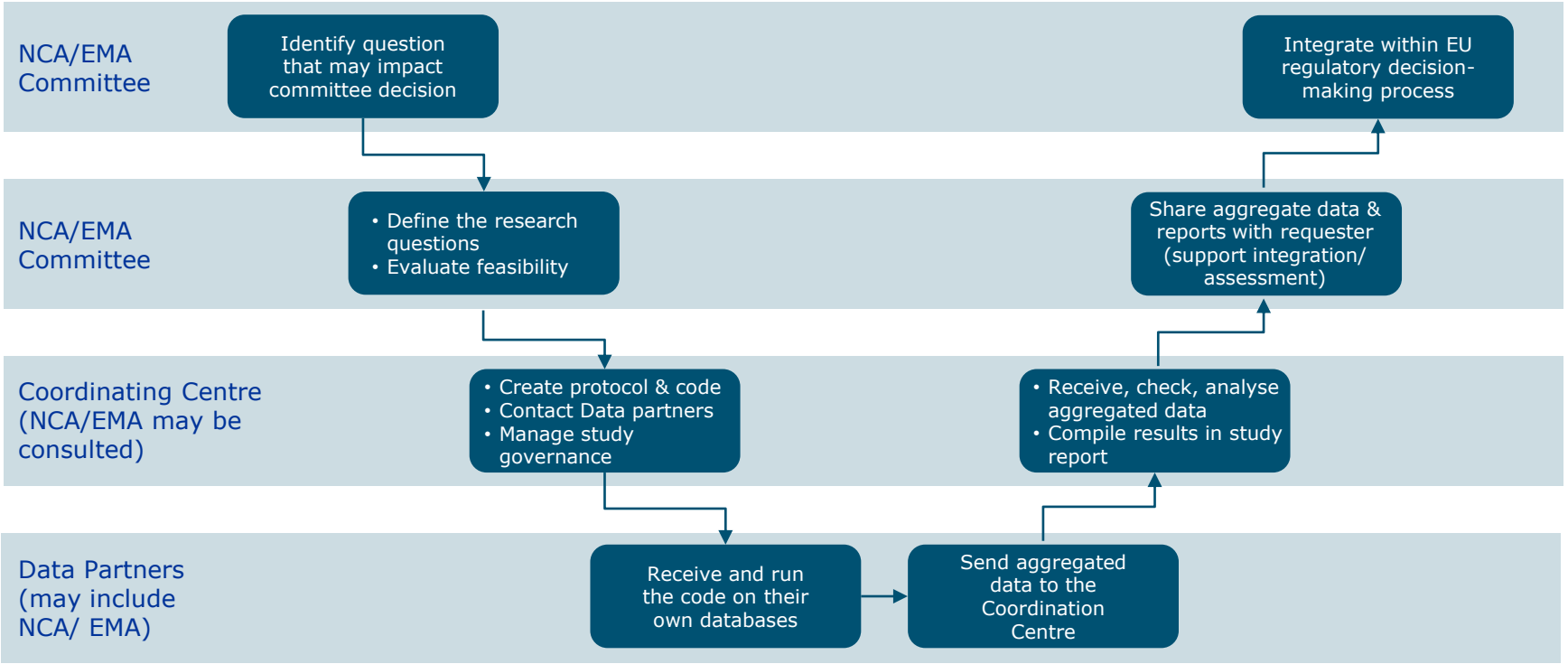
FEDERATED NETWORK PRINCIPLES

- Data stays **local**
- **Use of Common Data Model** (where applicable) to perform studies in a timely manner and increase consistency of results

What will DARWIN EU® do?

- Provide scientific expertise in formulating and executing studies and analyses
- Maintain a catalogue of known, relevant data holders, continually ensuring the discoverability & quality of data held by data holders
- Maintain & expand the federated network of data partners, assisting new data holders in conforming with required standards for usage in regulatory context
- Conduct scientific studies and analyses on behalf of the EMRN and EMA scientific committees
- Deliver training, governance, support of business services
- Enable the EMRN, EMA and the scientific committees to make use of the EHDS in the context of medicines regulation, acting as EHDS ‘pathfinder’

What is the DARWIN EU® process for conducting studies?



Who will benefit from DARWIN EU®?

EU medicines regulators

- Drug development – disease epidemiology, unmet need, historical controls, planning
- Authorisation – contribution to benefit-risk, controls, extrapolation to general and/or special populations
- Post-authorisation – benefit-risk monitoring, extension of indication, risk minimisation measures

DARWIN EU® will **increase the capacity** of the EMRN to undertake high-quality observational studies based on RWD and **reduce the time** per study

EU patients and healthcare professionals
Faster access to innovative medicines and safe and effective use

European Commission
Key use case for the European Health Data Space

National competent authorities
Support health policy and delivery of healthcare systems

HTA bodies and payers
Support better quality decisions on cost-effectiveness

EU and international health agencies
Use cases specific for other EU Agencies such as ECDC

Academia and research organisations
Increase use of RWE, methodology development, and better data quality

Industry
Enable better evidence supporting decision-making, increase receptiveness for RWE in MA submissions, and reduce time & cost of drug development

Implementation roadmap

Phase I - 2022

- Start running pilot studies to support EMA committees – **first benefits delivered**
  - Coordination Centre set-up
  - Data Protection Impact Assessment
  - Start recruiting and onboarding data partners
  - Pilot with the EHDS model and existing Data Permit Authorities
- Consultation of stakeholders

Phase II - 2023

- Support the majority of Committees in their decision-making with reliable RWE by 2023

Phase III - 2024

Up scale delivery and capacity to routinely support the scientific evaluation work of EMA's scientific committees and NCAs by delivering studies and maintaining data sources.

Operation - 2025/2026

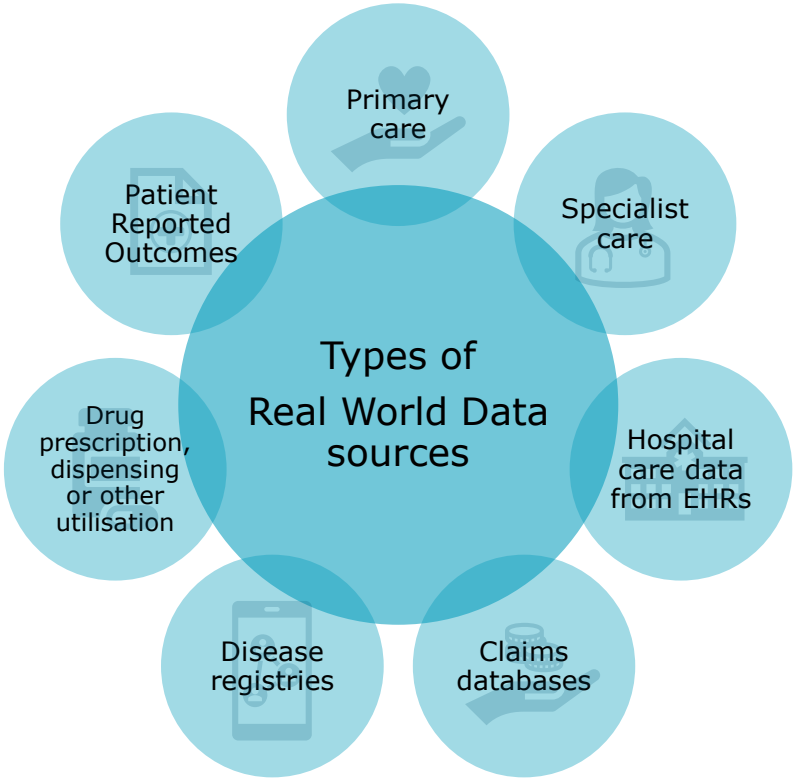
- DARWIN EU® to be fully operational and yearly evolves to meet the needs from the EU Regulatory Network
- **Integration with the EHDS**

DARWIN EU® - Coordination Centre immediate next steps

- **Formation** of the coordination centre: governance team, technology operations team, governance & boards
- **Project management** (e.g. project plan, risks management, reporting)

- **Strengthening** of the coordination centre:
  - Requirements & solution design
  - Conflict of Interest management process
  - Mandate and composition of the Scientific Panel
  - Change management plan
- **Strategic oversight** of the coordination centre:
  - Management plan and Business plan
- **On-Boarding of data sources templates**:
  - On-boarding specifications, data use agreement
- **Execution of studies templates**:
  - Feasibility assessment form, study outline/protocol/report, Agreement for Study Participation

Looking ahead at 2022: onboarding of data partners and first studies



First pilot studies in 2022 for a number of use cases across the medicine lifecycle

Over 5 years, ~380 studies will be conducted

Why can RWD analyses generated by DARWIN EU® be useful?

Ultimate goal: better informed and more efficient regulatory decision-making

To help fill knowledge gaps

- Providing additional information needed for decision-making such as more recent data or additional sensitivity analyses, or access to more and different databases (e.g. those established and maintained by public health authorities)

Transparent and tailored analyses

- Transparent and trusted sources of RWD
- Tailored to the Committee's questions, with involvement of the Committee/requester at every step

Faster evidence generation, avoiding the procedural steps for imposing and supervising MAH sponsored studies

Ability to study multiple substances of the same class avoiding unnecessary duplication and inefficiency that might be feature of studies done by industry

Three main areas for which RWD analyses can support committees' decision-making

1 Support the planning and validity of applicant studies

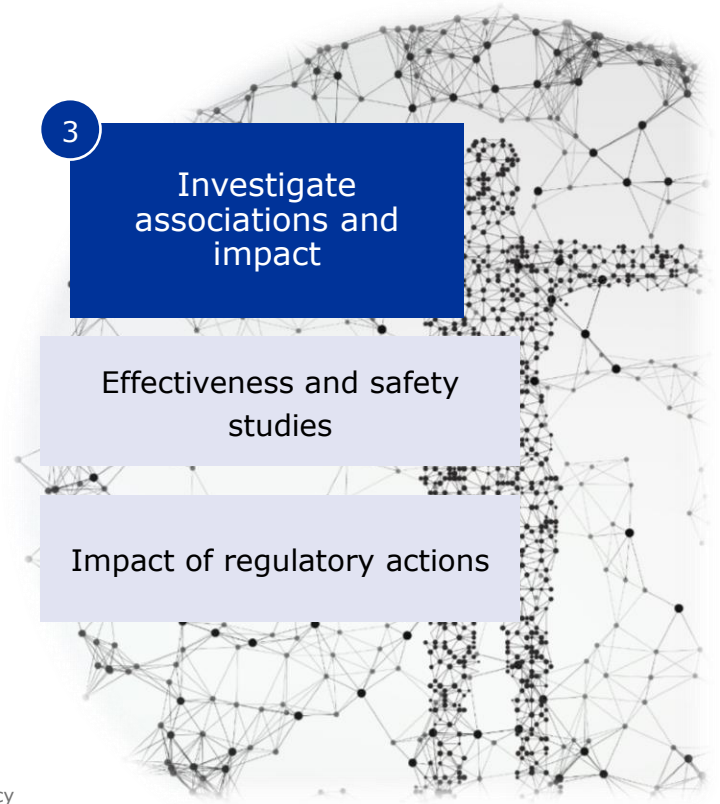
- Design and feasibility of planned studies
- Representativeness and validity of completed studies

2 Understand the clinical context

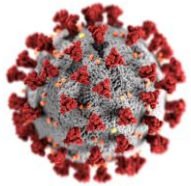
- Disease epidemiology
- Clinical management
- Drug utilisation

3 Investigate associations and impact

- Effectiveness and safety studies
- Impact of regulatory actions

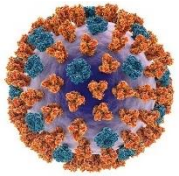


DARWIN EU® as central pillar for health crisis planning & response



Possible use cases include

- Monitoring the use of medicines to predict demand and shortages
- Understanding the disease natural history to support development of vaccines and therapeutics
- Provide evidence for repurposing existing medicines
- Monitor the safety and effectiveness of vaccines and therapeutics post-authorisation



DARWIN EU® will support future crisis responses with an operational infrastructure for conducting studies

More Information

[Data Analysis and Real World Interrogation Network (DARWIN EU) | European Medicines Agency (europa.eu)]

Coordination Centre website – coming soon in 2022!

- For questions to the Coordination Centre, please contact: enquiries@darwin-eu.org

For regular updates on DARWIN EU® Subscribe to the Big Data Highlights newsletter by sending an email to: bigdata@ema.europa.eu

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HMA Heads of Medicines Agencies

EMA EUROPEAN MEDICINES AGENCY

BIG DATA

HIGHLIGHTS

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

An agency of the European Union

Editorial

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INSIDE THIS ISSUE

Welcome to the first edition of the newsletter on Big Data which reports on the implementation of the HMA-EMA Big Data Steering Group workplan 2021-2023 and the data and digital pillar of the Network Strategy 2025.

The BDSG was established in May 2020 with the mandate to take forward and advise on implementation of the priority recommendations set out in the Big Data Task Force final report (phase two). The vision, set out by the Big Data Taskforce that guides delivery of the workplan is that:

by delivering a regulatory system able to integrate Big Data into its assessment and decision making, we can support the development of innovative treatments more quickly and optimise the safe and effective use of medicines.

Big Data Task Force Final Report, Dec 2019

Already in 2021, we have seen an impressive array of activities and outputs that support transformation to data-driven medicines regulation. These include the finalisation of the network's data standardisation strategy and a series of technical workshops covering standardisation, real-world data, artificial intelligence, veterinary data and real-world evidence. More details on the 2021 deliverables are included later in this newsletter.

Moving forward, we are excited to be able to share with you more regular updates on big data and the implementation of the BDSG workplan via this newsletter. We look forward to collaborating and leveraging the work of stakeholders to increase the utility of big data in medicines regulation.

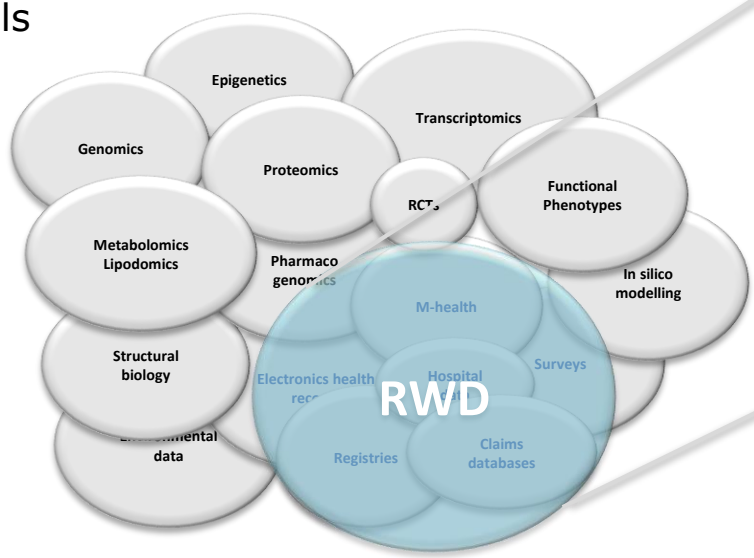
The Big Data Steering Group welcomes your feedback and questions you may have by email at bigdata@ema.europa.eu .

We hope you find the Big Data Highlights informative!

Backup slides

What is real-world data and evidence?

Real-World Data (RWD): routinely collected data relating to patient health status or the delivery of health care from a variety of sources other than traditional clinical trials



Real-World Evidence (RWE): information derived from analysis of real-world data

What analyses and studies will DARWIN EU® deliver?

Category of observational analyses and studies	Description
Routine repeated analyses	Routine analyses based on a generic study protocol • Periodical estimation of drug utilisation • Safety monitoring of a medicinal product • Estimation of the incidence of a series of adverse events
Off-the-shelf studies	Studies for which a generic protocol is adapted to a research question • Estimate the prevalence, incidence or characteristics of exposures • Health outcomes • Describe population characteristics
Complex Studies	Studies requiring development or customisation of specific study designs, protocols and Statistical Analysis Plans (SAPs), with extensive collection or extraction of data • Etiological study measuring the strength and determinants of an association between an exposure and the occurrence of a health outcome considering sources of bias, potential confounding factors and effect modifiers
Very Complex Studies	Studies which cannot rely only on electronic health care databases, or which would require complex methodological work • Studies where it may be necessary to combine a diagnosis code with other data such as results of laboratory investigations, or studies requiring additional data collection