EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EU regulators collaboration and alignment in RD innovative & needs-led research and development

2nd ERICA general assembly, Bologna

Presented by Kristina Larsson on 21 June 2022
Head of office Orhan Medicines, European Medicines Agency

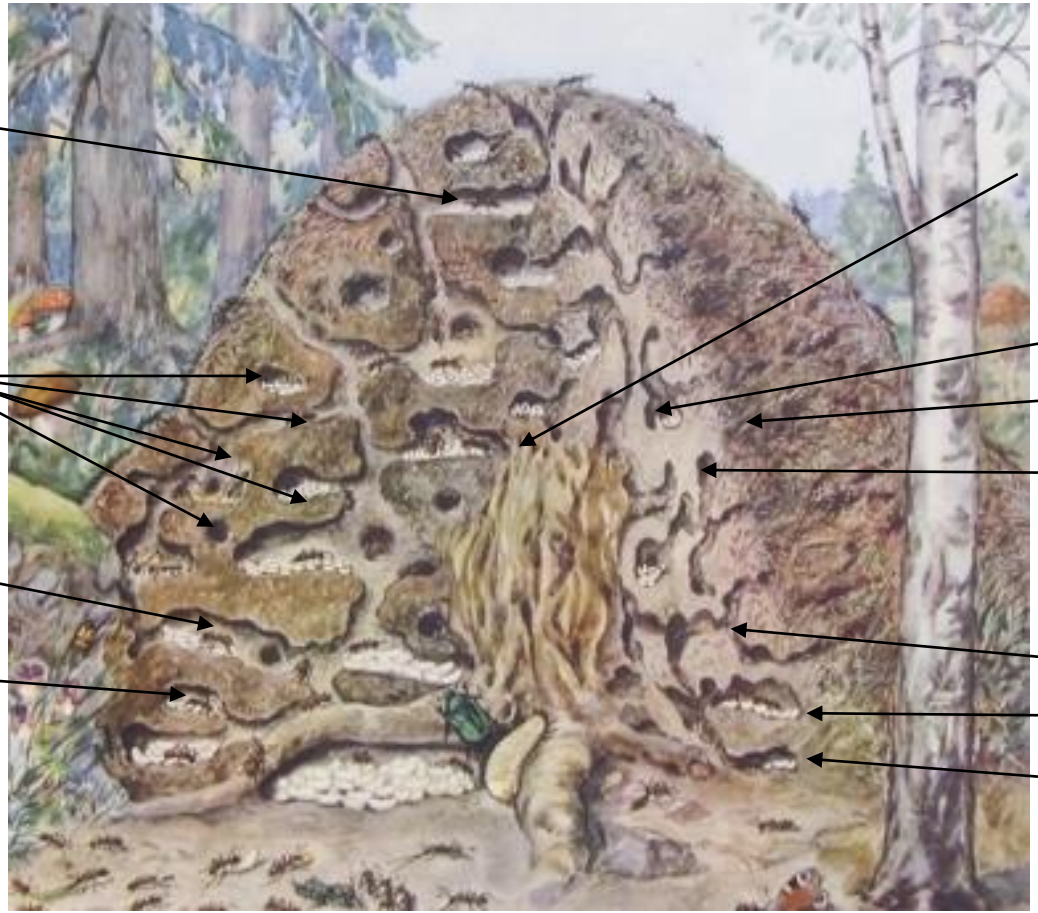
An agency of the European Union

RWE
methodology
clinical trials

Therapeutic
areas

translational
science

quality



Committees
Working parties

academia

patients

SME

scientific advice

paediatrics

orphans

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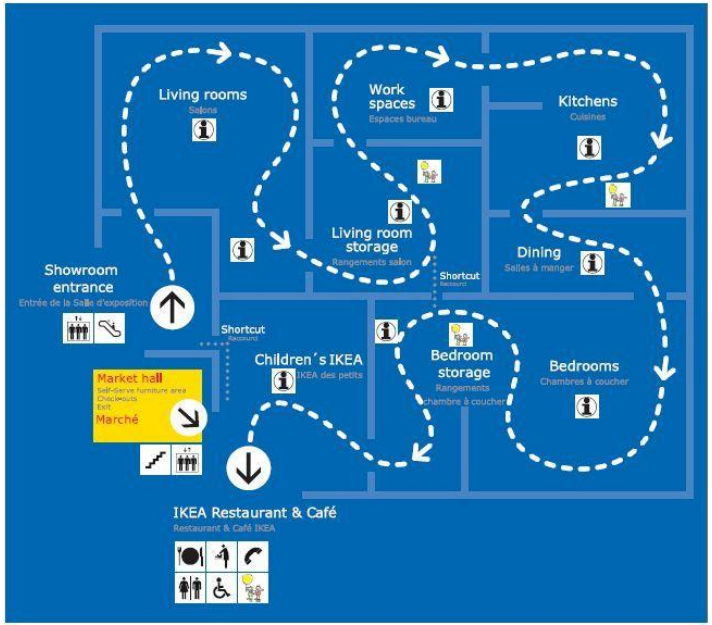
The map (presentation outline)



- Contacts
- Procedures
- Projects
- Interactions

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Contact points

- Healthcare Professionals Liaison

Ivana Silva
ERN contact

- Academia Liaison
- SME office

Healthcare professionals | European Medicines Agency (europa.eu)

Academia | European Medicines Agency (europa.eu)

Support to SMEs | European Medicines Agency (europa.eu)

Home · IRIS (europa.eu)

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Procedures

PRIME

Innovation Task Force

Orphan designation

Paediatric development

Scientific advice and Protocol assistance

Qualification advice and opinion

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Accelerating Clinical Trials in the EU (ACT EU)

ACT EU is an initiative to **transform the EU clinical research environment** in support of medical innovation and better patient outcomes.

- 10 priority actions with a focus on:
  - **enabling clinical trials** (in particular multinational trials)
  - **innovative trial methods**
  - **GCP modernisation**
  - **engaging all stakeholders**
- Read the press release and paper

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ACT EU priority action 8: Methodologies guidance

Estimands - ICH E9(R1)

- Guidance to align trial objectives with suitable trial design and statistical tools for estimation and hypothesis testing
- Ensures trials generate robust results

Complex trials

- Guidance to trial sponsors on the planning and conduct of complex clinical trials and the design of master protocols
- Complex trial Q&A published June 2022

Upcoming focus area: **Decentralised trials**

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How to get involved

The **ACT EU Multi-Stakeholder Platform** will:

- Bring the clinical trials community together, including patients, HCPs, sponsors, assessors and inspectors to discuss key topics
- Help clinical trial stakeholders identify and respond to new developments in tools and methods

Workshops and events to be announced on the EMA website later in 2022 and 2023.

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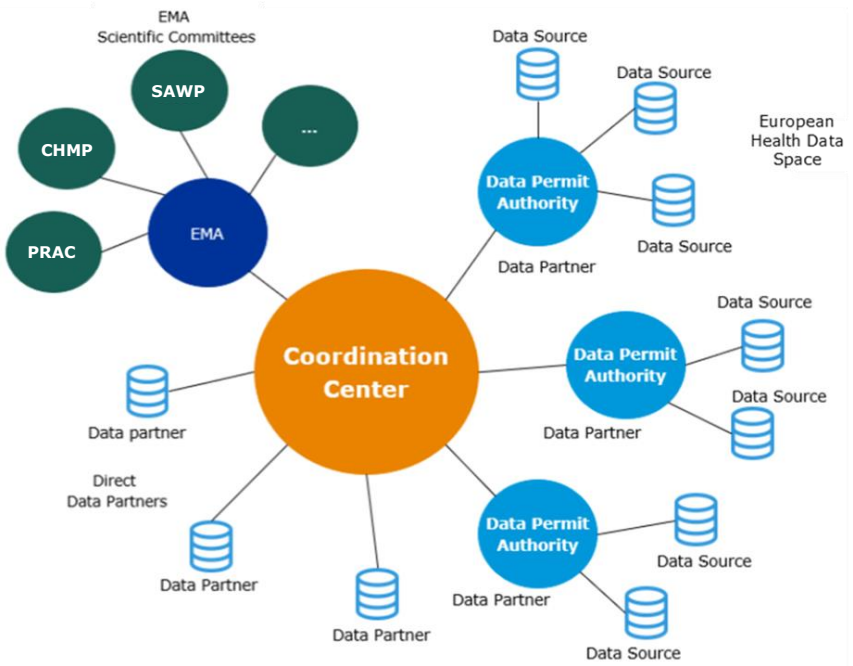
The **Data Analytics and Real World Interrogation Network** (DARWIN EU®) is a federated **network** of **data**, **expertise** and **services** that supports better decision-making throughout the product lifecycle by generating reliable **evidence from real world healthcare data**

Coordination Center:

- Coordinates and maintains the network, data partners and performs studies
- **Erasmus University Medical Center Rotterdam** selected via tender to perform CC services



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

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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'

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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**

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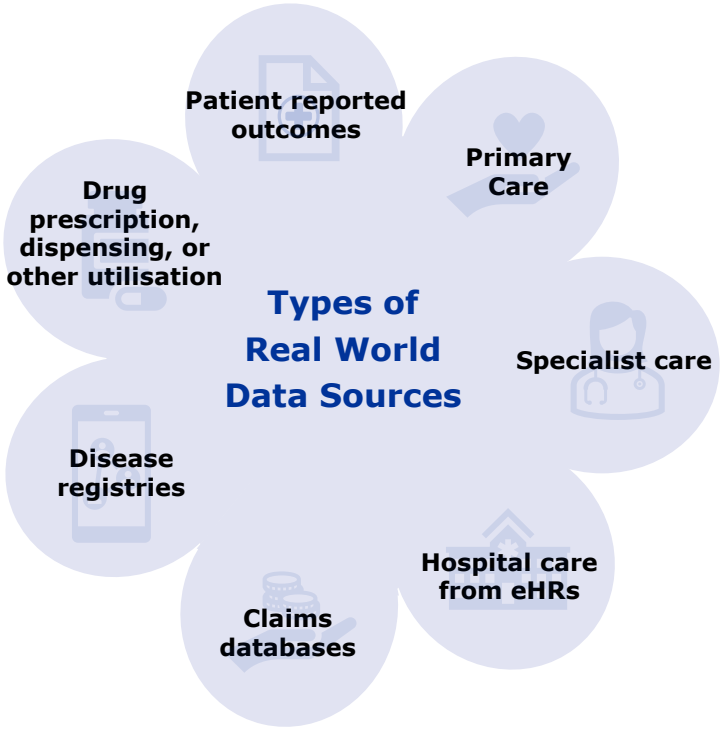
Looking ahead to 2022 | Onboarding of data partners and first studies



Initially 10 data partners to be onboarded

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

By 2025, > 100 studies per year will be conducted

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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 • Estimate the prevalence, incidence or characteristics of 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

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We like collaborations!

- RD-ACTION / EMA / DG SANTE workshop: how European Reference Networks can add value to clinical research | European Medicines Agency (europa.eu)
- Expert consultation on Inherited Retinal Dystrophies 17 June 2022.
- Experts for Scientific advisory group and ad hoc expert group meetings
- Experts for procedures

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Any questions?

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