



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Real World Data analyses to support Rare diseases

International Conference on Rare Diseases - Greek Chapter
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European Medicines Agency

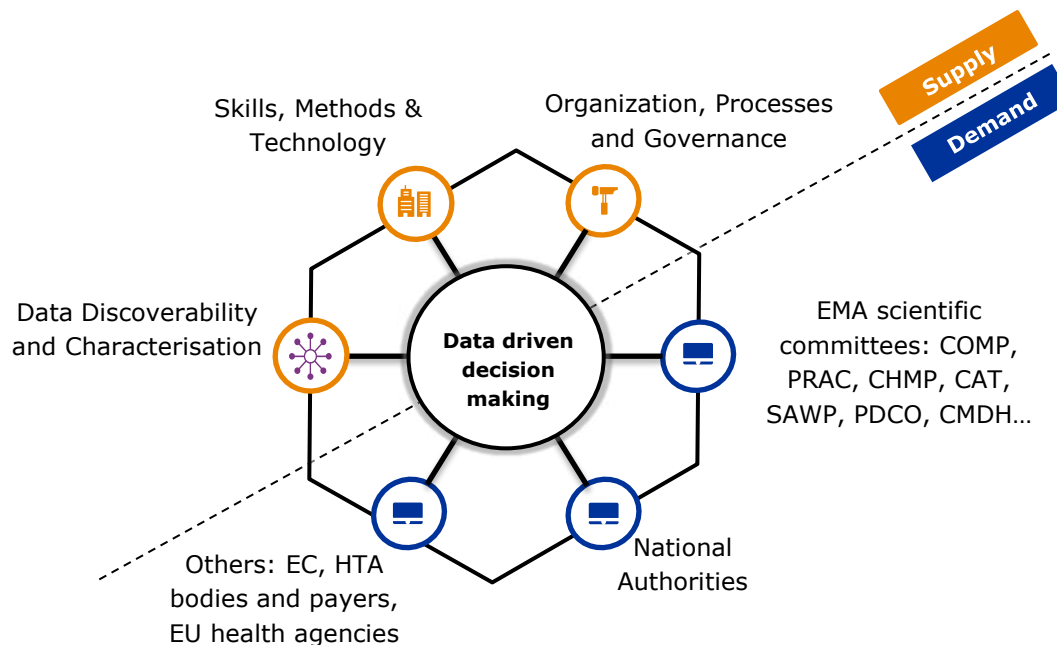
An agency of the European Union





How to increase the generation and use of RWE provided by the European Network to improve decision-making on medicines

International regulators have been establishing systems for generating the evidence needed, e.g. FDA Sentinel system, Health Canada CNODES, PMDA MID-NET,...



DARWIN EU®



A federated network of data, expertise and services

EU Medicines Regulatory Network

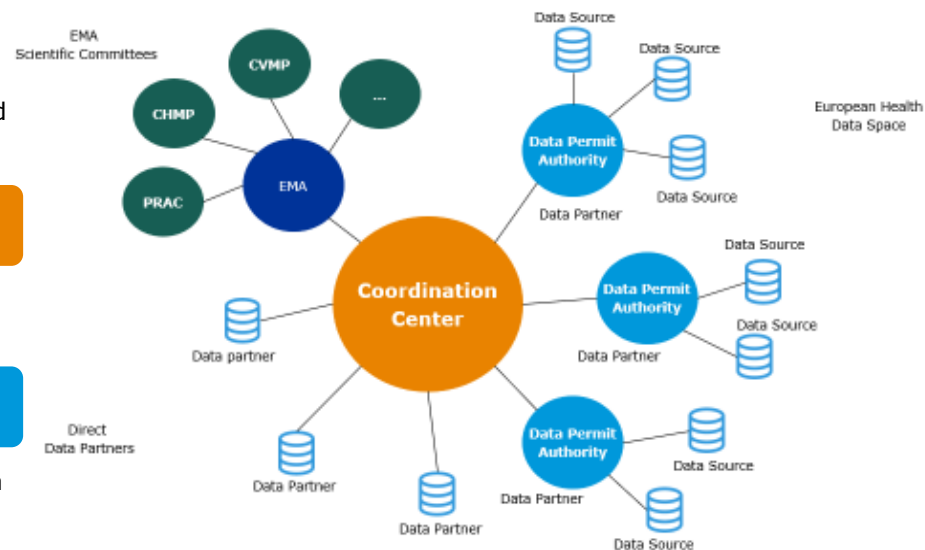
- **EMA** - provides leadership, setting standards, contracting studies, **overseeing**
- **European Medicines Regulatory Network** - including EMA scientific committees and working parties, national competent authorities (NCAs) and the European Commission: **request studies** via EMA

The Coordination Centre

- **Establishes** and **maintains the network** (including onboard/maintain data sources), manage the **execution of scientific studies**

Data Partners, incl. Data Permit Authorities

- **Partners** who have access to data, or who may request analyses in a data source and provide results to the Coordination Centre
- This includes **Data Permit Authorities** (DPAs), already existing or to be created as part for the European Health Data Space (EHDS)





Characteristics of DARWIN EU®



Federated network



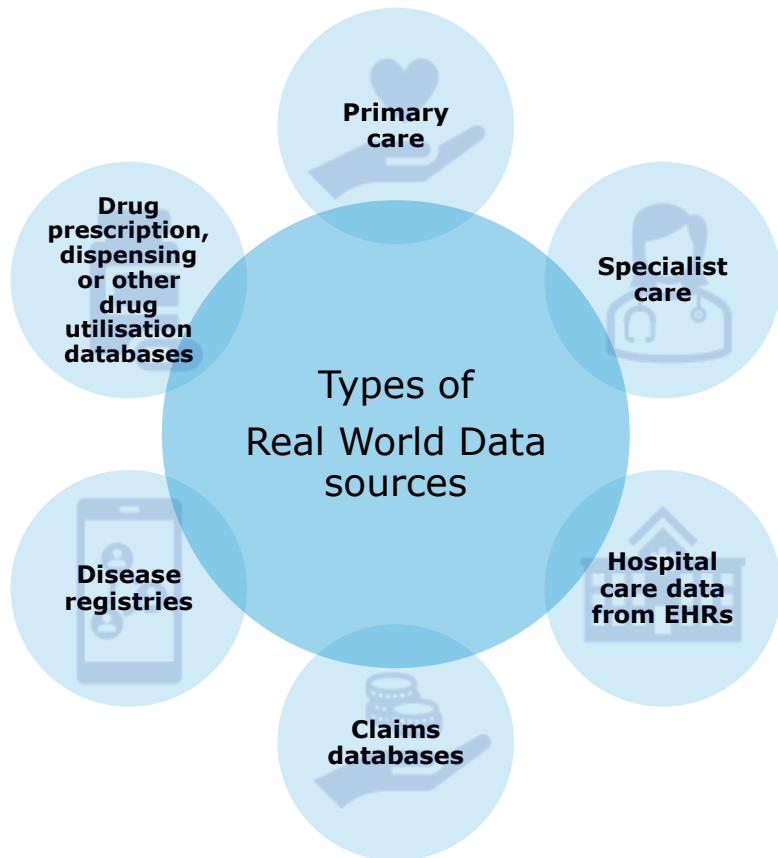
Data stays local and is queried remotely



Common data model



Anonymous data



The selected databases to represent:

- Geographical settings of the databases available in Europe
- Different healthcare settings
- Different formats
- Different complexity

Where can RWD analysis be useful? ...specifically for rare diseases

Challenge

- Small numbers, small numbers, small numbers... →
- Diseases with different clinical presentations →
- Treatment patterns differ across countries →

RWE solution

- Multi-databases with pooled patient data or where meta-analysis can be applied
- Multiple case definitions can be explored
- Multi-database studies with geographic diversity

Any questions?

Further information

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