SETTING THE SCENE:

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EUROPEAN ALLIANCE
FOR PERSONALIZED MEDICINE

VISION EUROPE 2030

DISRUPTIVETECHNOLOGIES,DEMOCRATIZEDTRIALS& NEXT-GEN TREATMENT PARADIGMS

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Evolving regulatory landscape for RWE

Dr. Daniel Morales

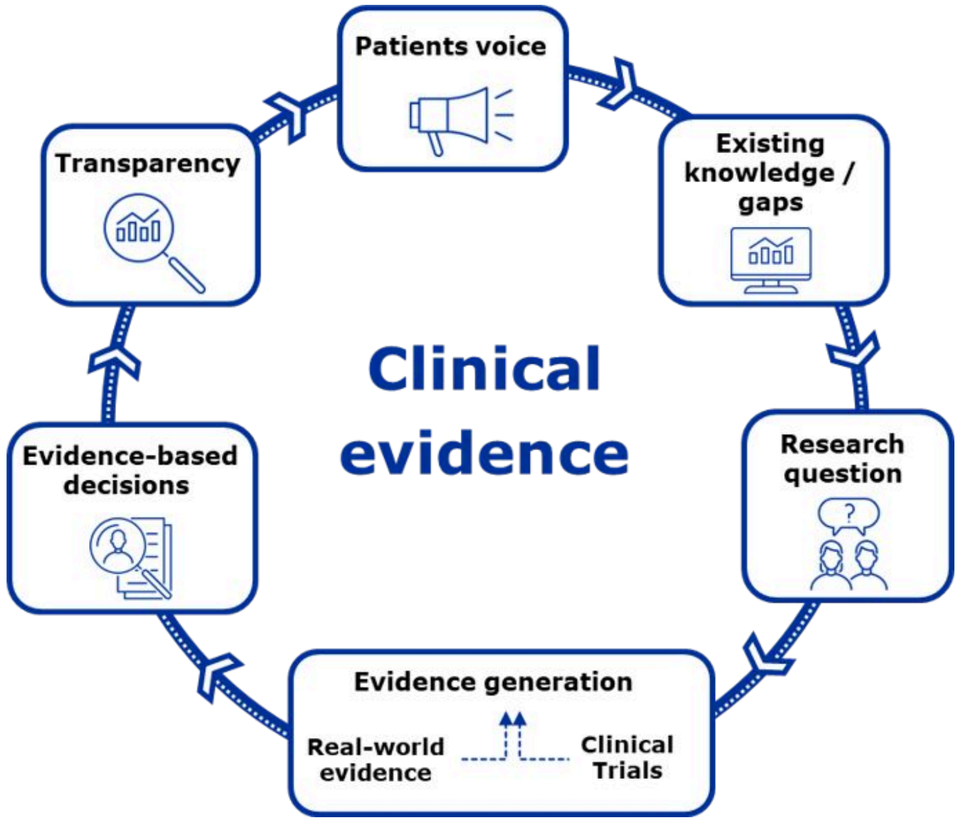
RWE – EMA

3rd Sept 2025

Disclaimer

The presenter does not have any conflict of interests.

Generating clinical evidence
Shared vision towards 2030



- **Patient** voice guides every step of the way
- Evidence generation is **planned** and guided by purpose, data, knowledge and expertise
- Research question **drives** evidence choice and embraces spectrum of data and methods
- Clinicaltrials remain core but should be **better, faster** and **optimised**
- Real world evidence is **enabled**, and its value is **established**
- High **transparency** level underpins societal trust

Clinical Evidence 2030. Arlett P, Umuhire D, Verpillat P, Foggi P, Wändel Liminga U, Sepodes B, Lunzer M, Aylward B, Vamvakas S, Roes K, Pétavy F, Thirstrup S, Lamas M, Cooke E, Broich K. ***Clin Pharmacol Ther***. 2025 Feb 14. doi: 10.1002/cpt.3596.

In the EU, generation of RWE for regulatory purpose is already in action!

Three main areas where RWD analyses support decision-making

1 Understand the clinical context

- Disease epidemiology
- Clinical management
- Drug utilisation

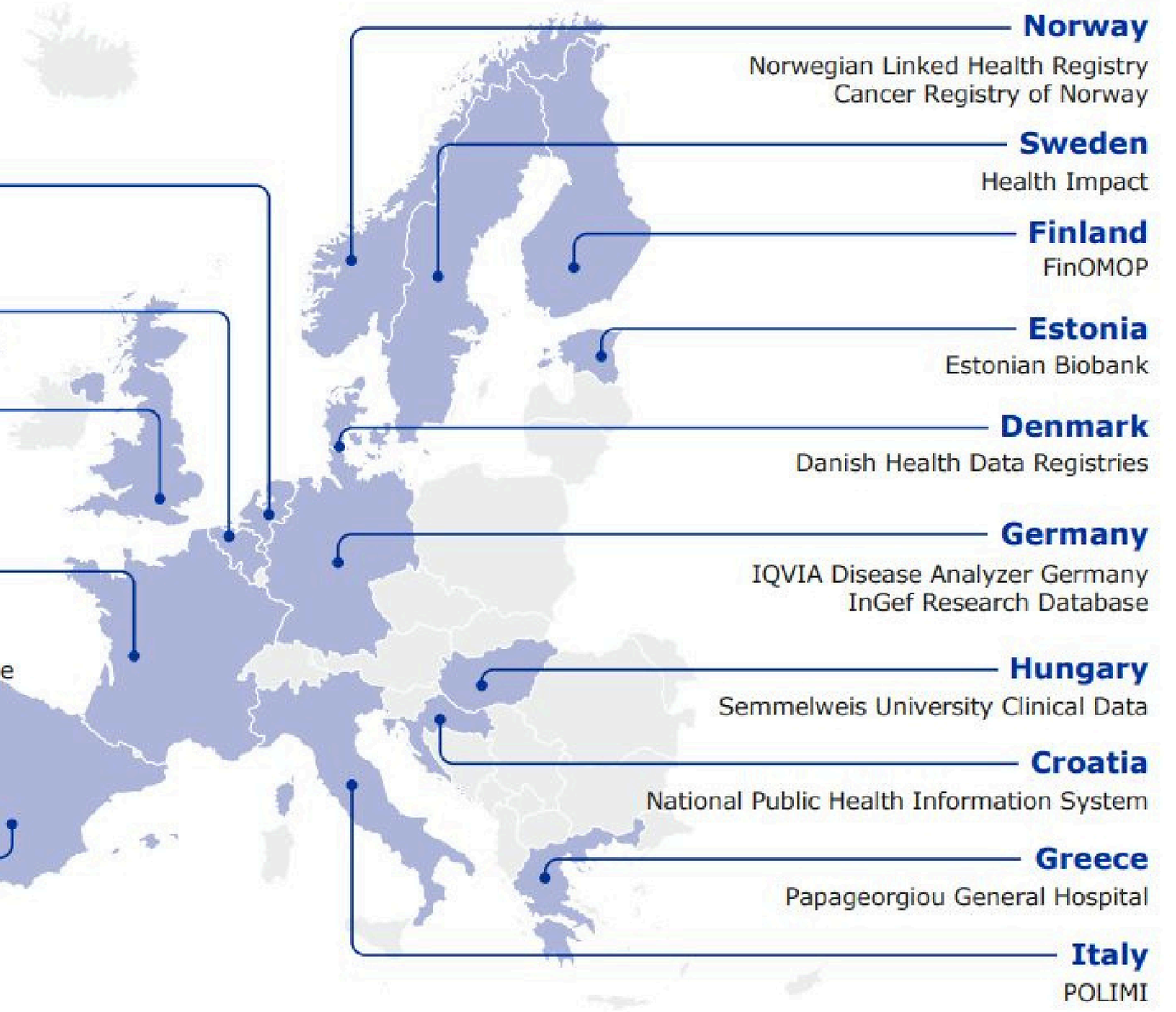
2 Support the planning and validity

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

3 Investigate associations and impact

- (Comparative) Effectiveness and safety studies
- Impact of regulatory actions

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 (~ +10 by end of Feb 2026)in **16 European countries**

~100 studies per year from 2025 onwards

HMA
Heads of Medicines Agencies

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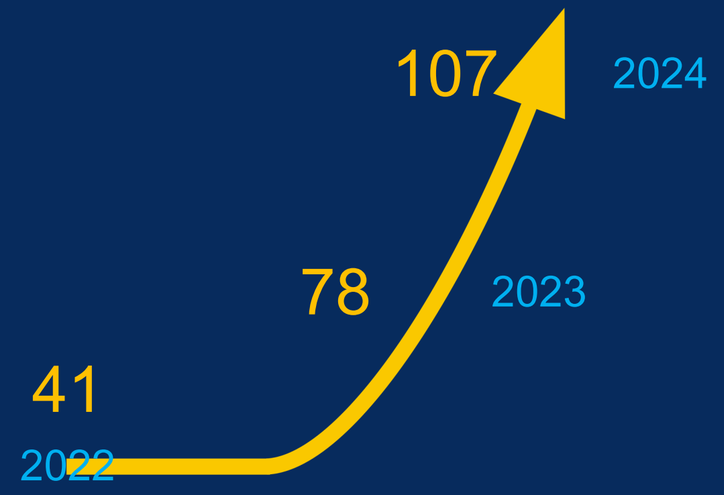
Real-world evidence framework to support EU regulatory decision-making

2nd report on the experience gained with regulator-led studies from February 2023 to February 2024

Number of research questions addressed per year



From 60% to78% feasible

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Examples of studies

Doxycycline and association with risk of **suicidality** (PRAC request to facilitate signal assessment)

Safety signal on “suicidality” raised based on cases reported to the Finnish national competent authority and EudraVigilance

Currently available evidence not supporting link between this antibiotic drugand riskofsuicidality
—>**No update to doxycycline product information warranted**

Pharmacovigilance Risk Assessment Committee

Juvenile polymyositis (JPM) and dermato-myositis (JDM) anddisease natural historyin paediatricpopulation (PDCO requestto better understand thediseasecontext)

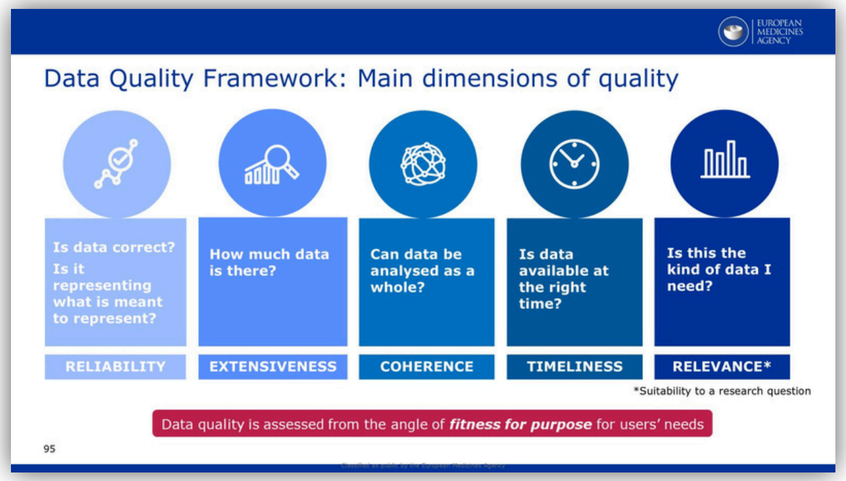
Largest European JDM & JPM study showing increased prevalence over time, clinical manifestations and treatments in line with clinical recommendations

Used by PDCO in a PIP as results suggested sufficient patients available to perform acontrolled clinical trial —> **Obligation placed on the applicant**

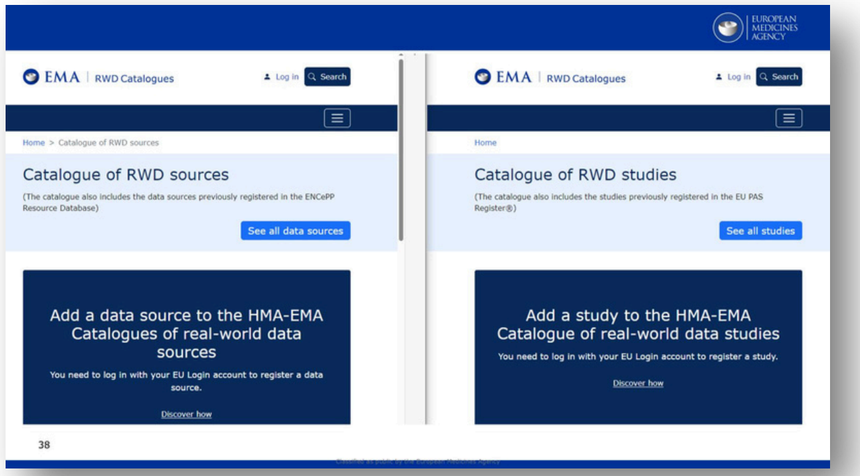
Paediatric Committee

Additional tools to ensure relevant and reliable RWE

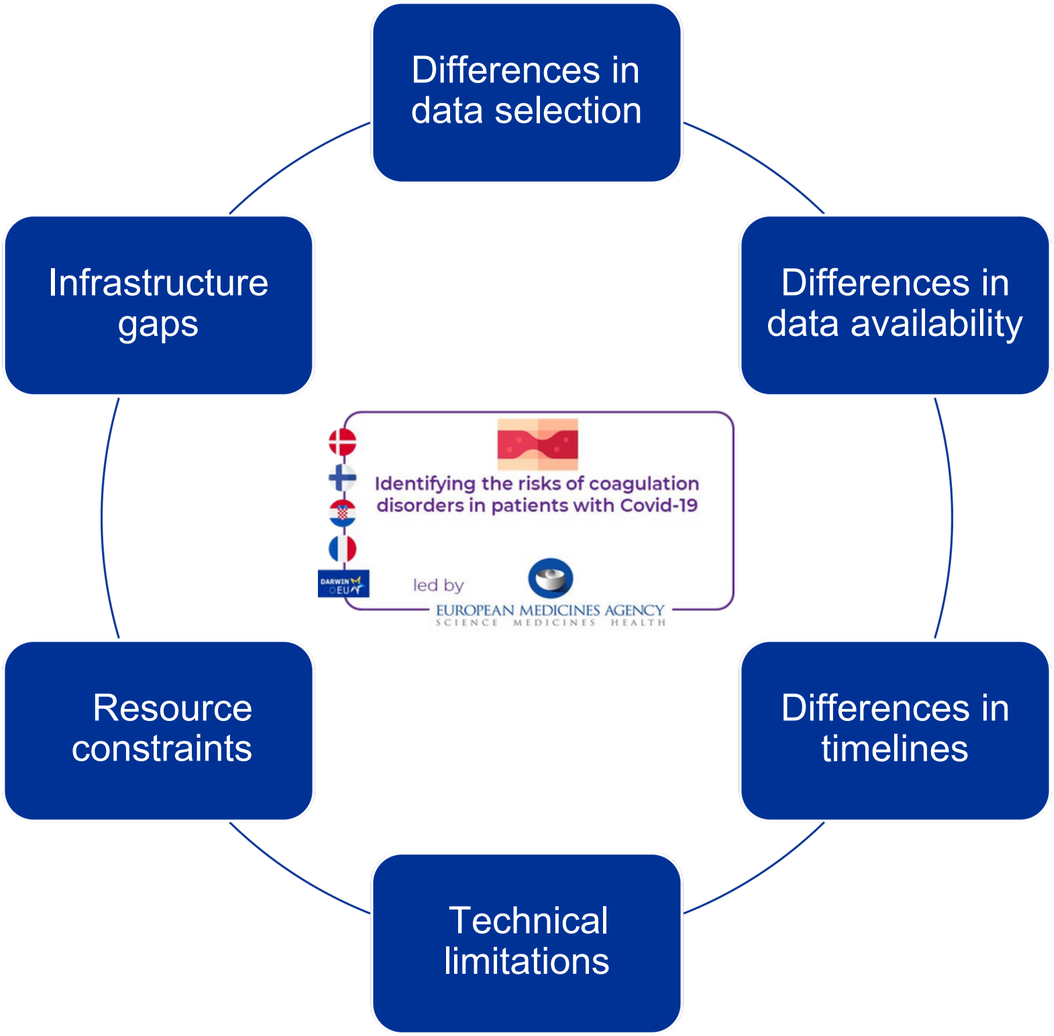
Data Quality Framework for EU medicines regulation and its RWD chapter



HMA-EMA Real-World Data Catalogues

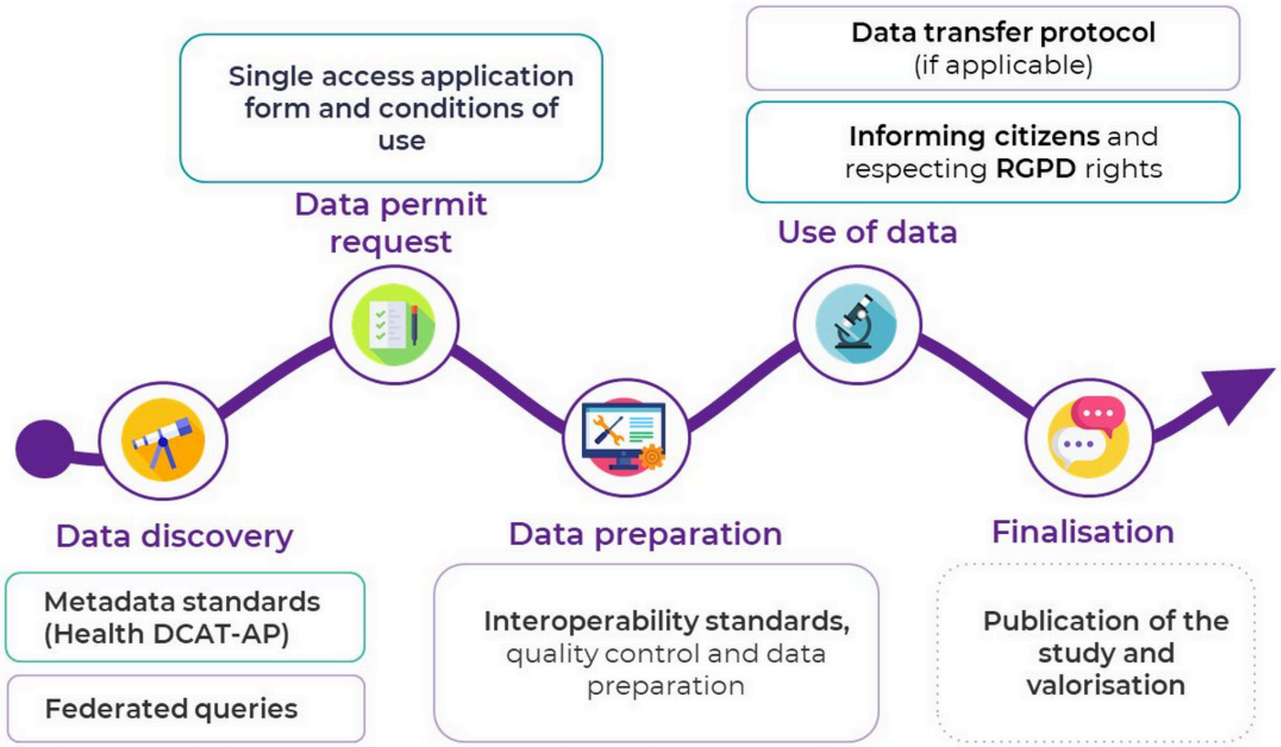


Challenges



Opportunities

European Health Data Space



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Thank you

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