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EUROPEAN MEDICINES AGENCY

Real World Evidence update

20th Industry Stakeholder Platform

13 November 2025

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The presenter does not have any conflict of interests.

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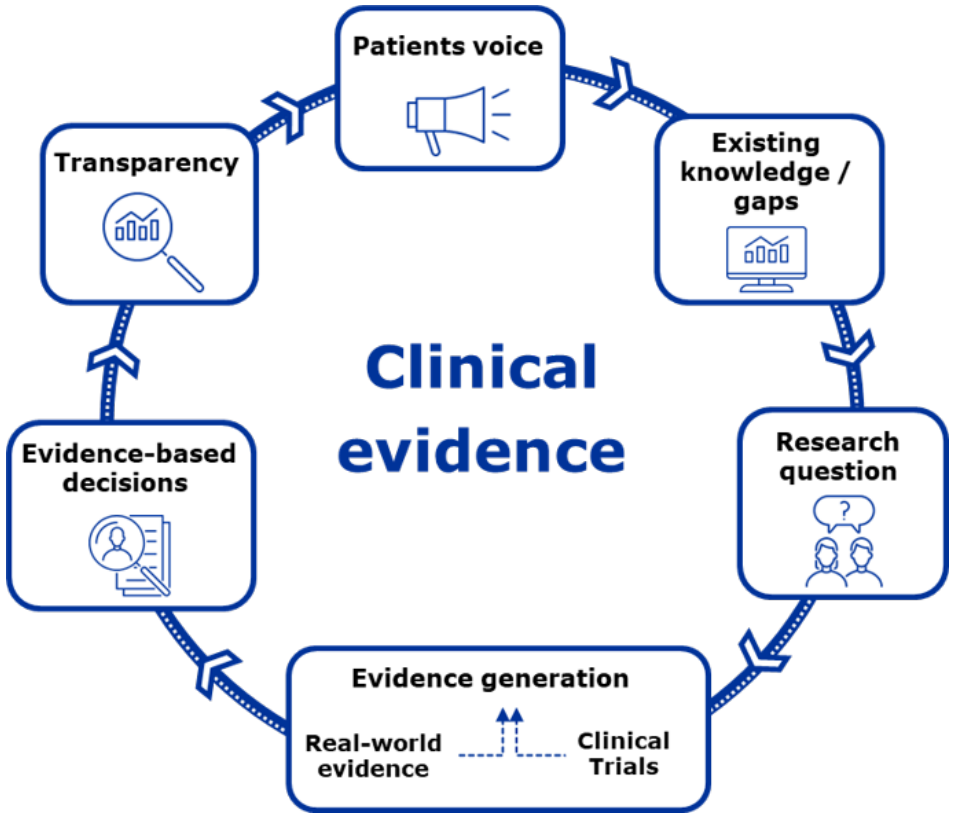
Generating clinical evidence
Shared vision towards 2030



- **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
- Clinical trials 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.

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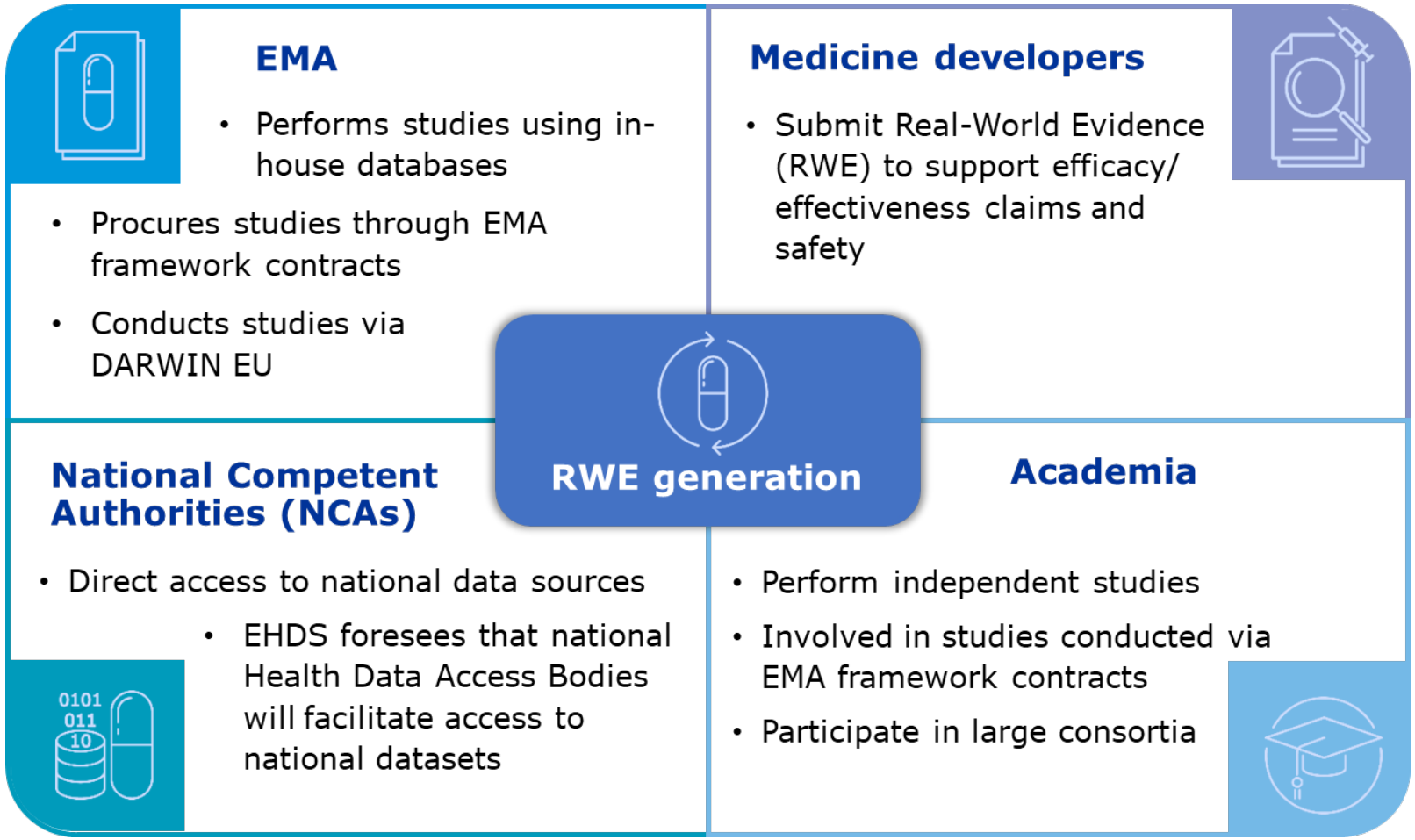
Who delivers RWE for regulatory purpose in the EU?



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3 main pathways to use RWD for generating RWE

Studies using in-house databases

- **Primary and secondary care** health records from **France**, **Germany and UK**

Studies procured through EMA FWCs

- Framework contract (FWC) since September 2021: services of **8 research organisations** and academic institutions
- Access to **wide network of data**: 59 data sources in 21 EU countries
- Ability to leverage external **scientific expertise**

DARWIN EU®*

- See next slides…

*Data Analysis and Real-World Interrogation Network

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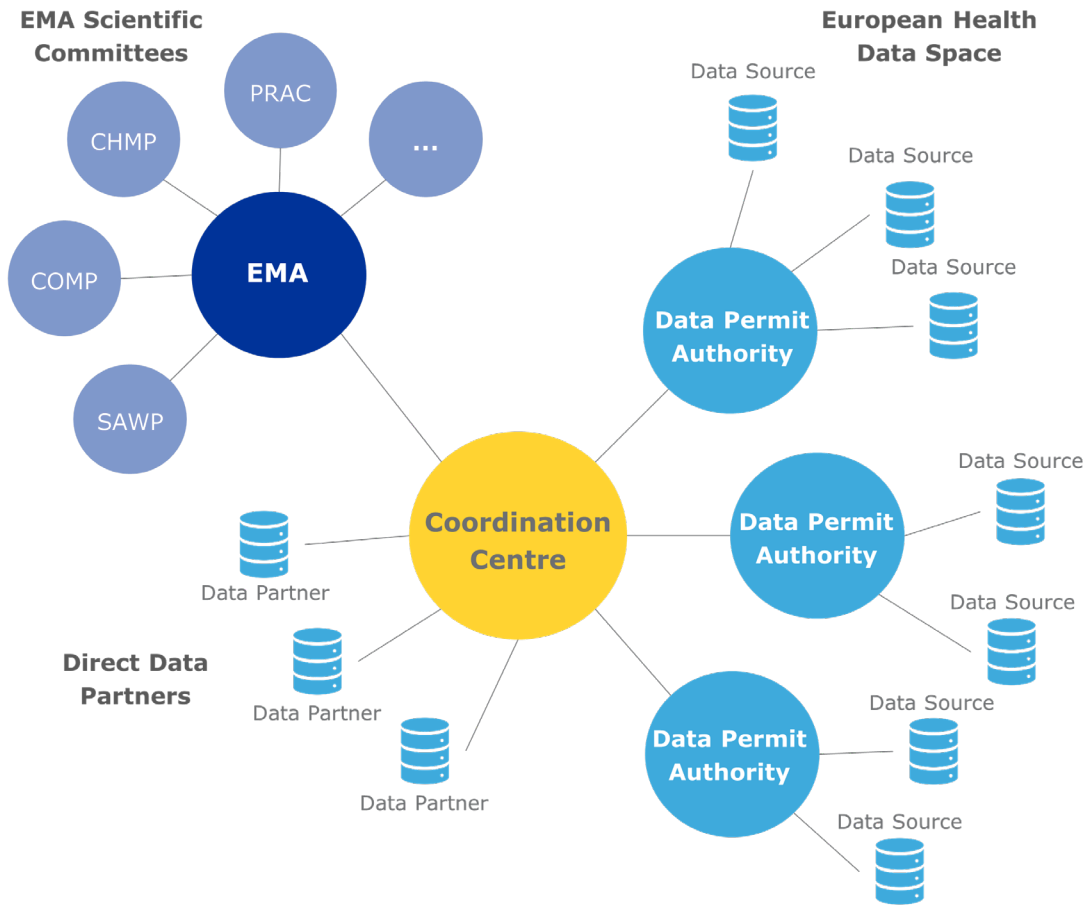
DARWIN EU®
Data Analysis and Real-World Interrogation Network

Federated **network** of **data**, **expertise** and **services** set up in *February 2022* to better support decision-making throughout the product lifecycle by generating **valid and reliable evidence from real-world healthcare data**

FEDERATED NETWORK PRINCIPLES

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



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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

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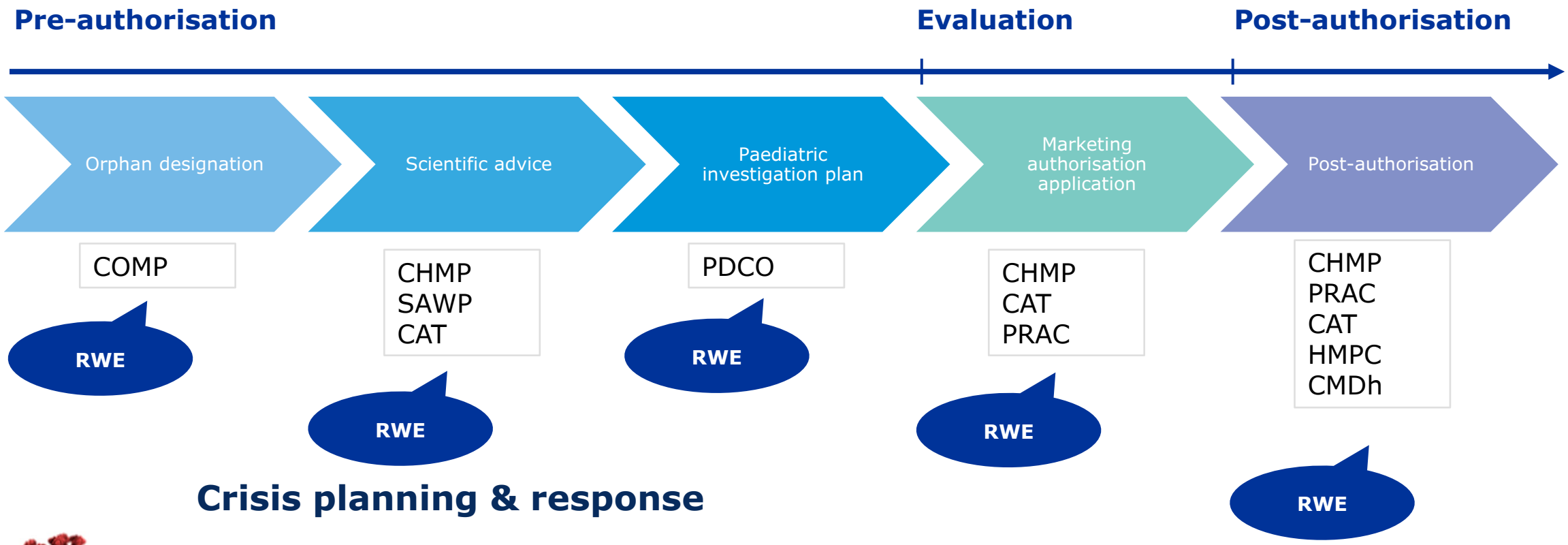
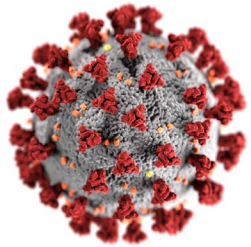
RWE use across the medicinal product lifecycle



Crisis planning & response



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

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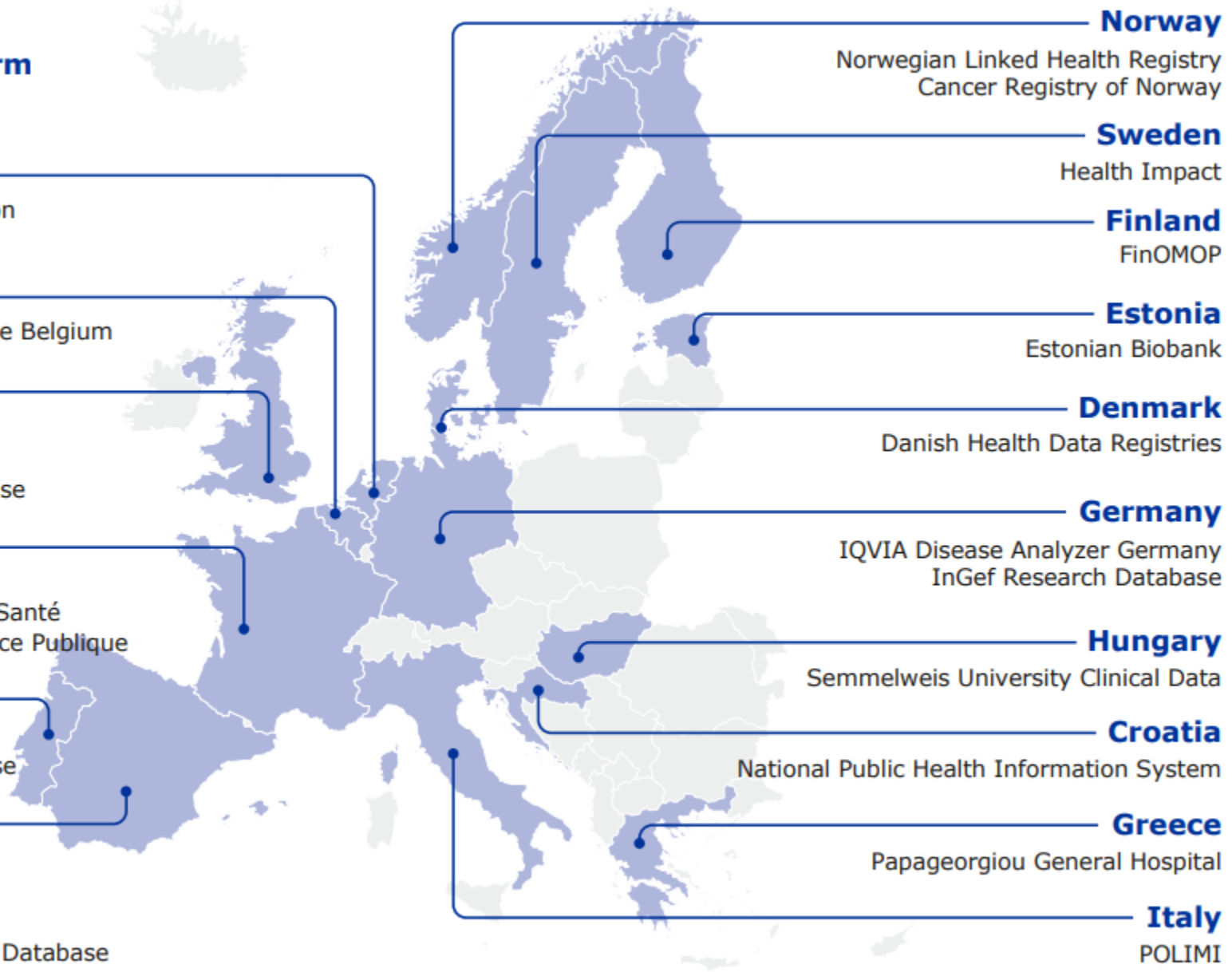
DARWIN EU Network of Data Partners



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

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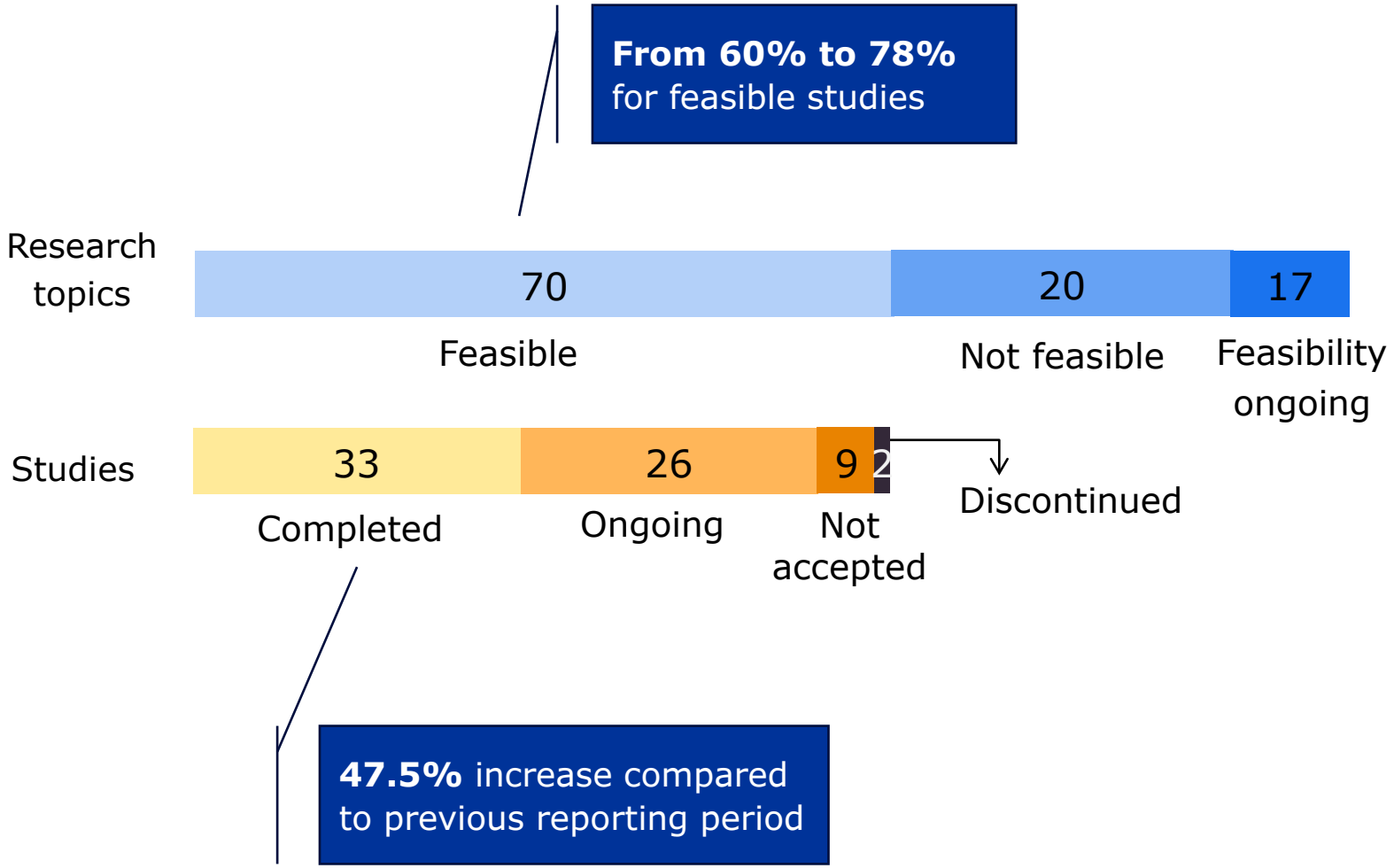
Where are we now?

107

Research topics

(Feb '24 – Feb '25)

89	9	9
DARWIN EU	I-H	FWC



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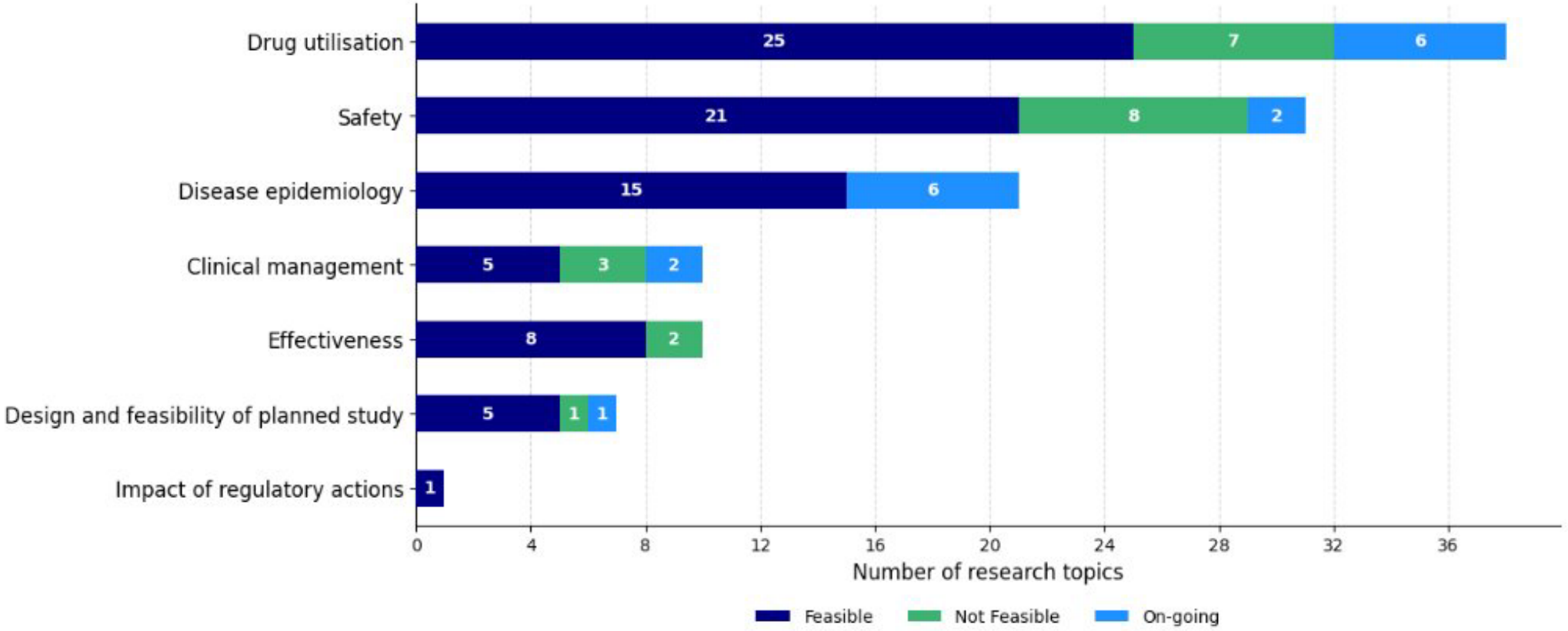
Feasibility of research topics by use case



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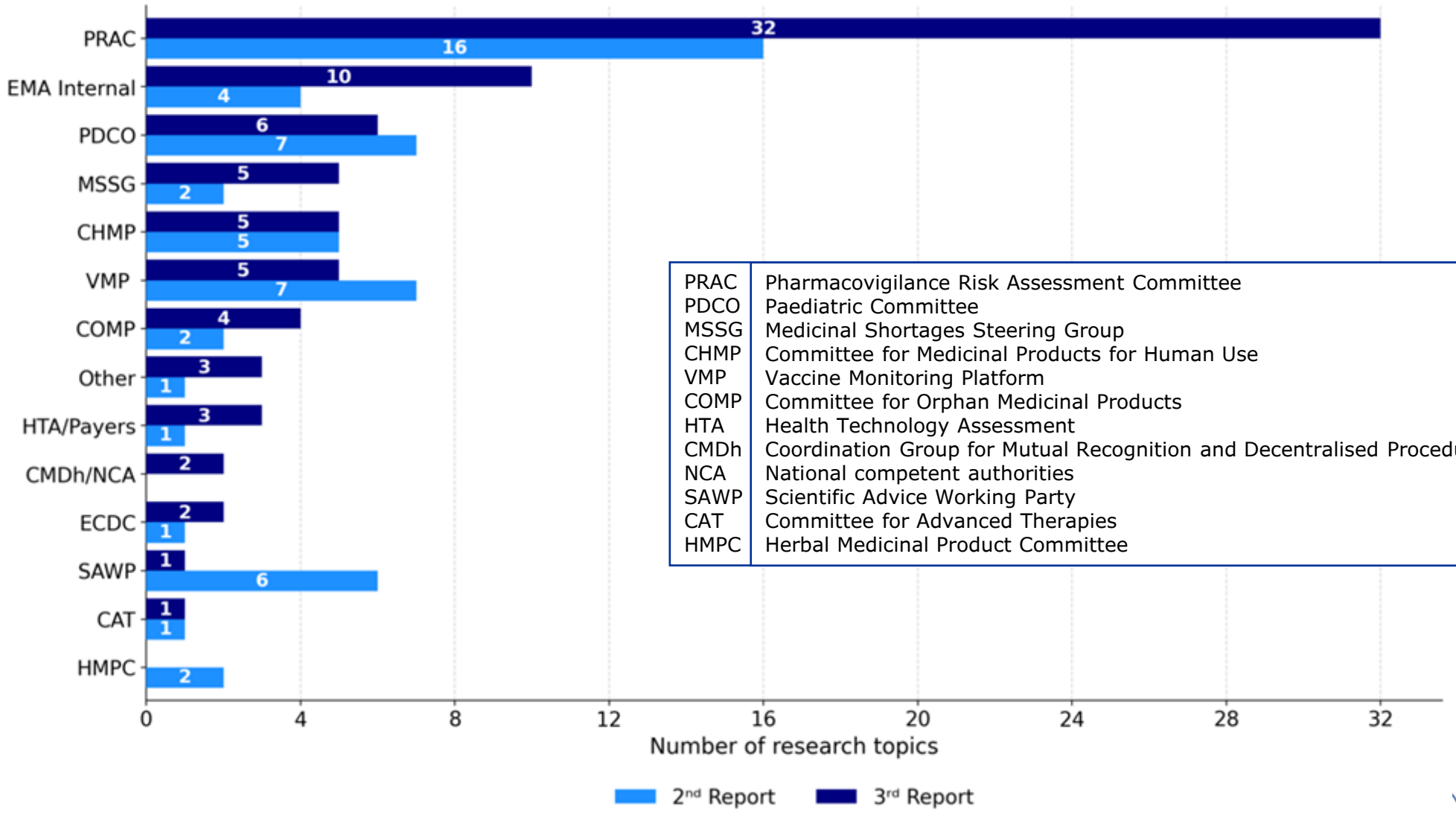
Requesters dynamic over time



PRAC	Pharmacovigilance Risk Assessment Committee
PDCO	Paediatric Committee
MSSG	Medicinal Shortages Steering Group
CHMP	Committee for Medicinal Products for Human Use
VMP	Vaccine Monitoring Platform
COMP	Committee for Orphan Medicinal Products
HTA	Health Technology Assessment
CMDh	Coordination Group for Mutual Recognition and Decentralised Procedures
NCA	National competent authorities
SAWP	Scientific Advice Working Party
CAT	Committee for Advanced Therapies
HMPC	Herbal Medicinal Product Committee

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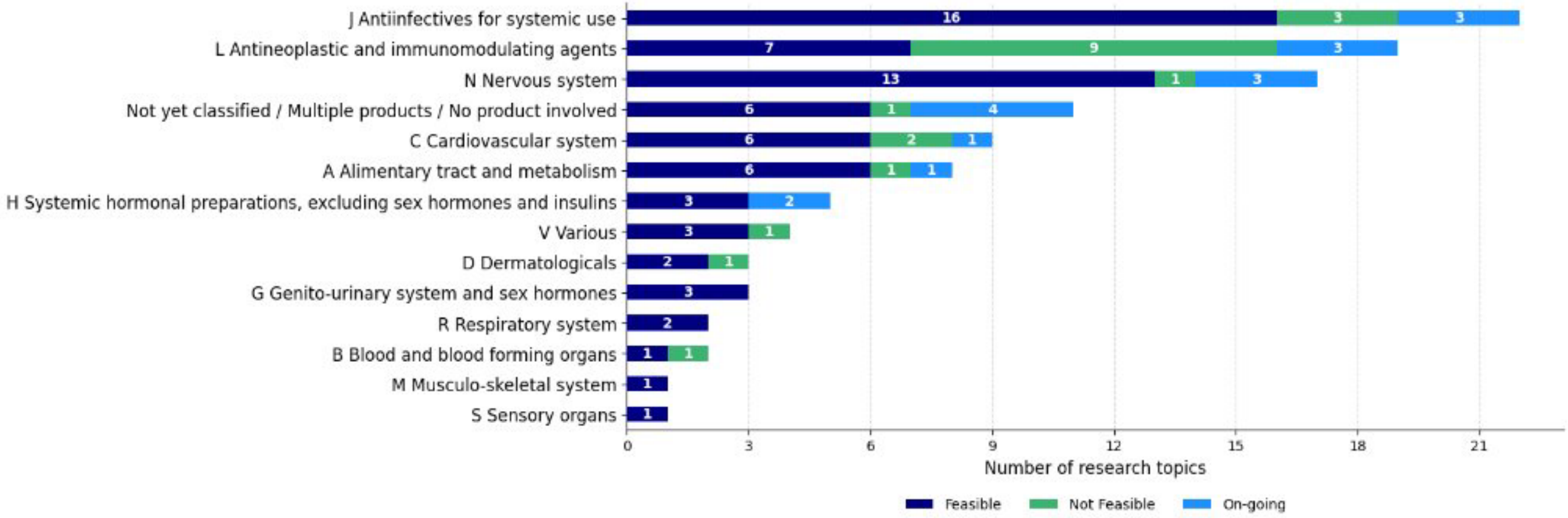
Feasibility of research topics by Anatomical Therapeutic Chemical (ATC) classification



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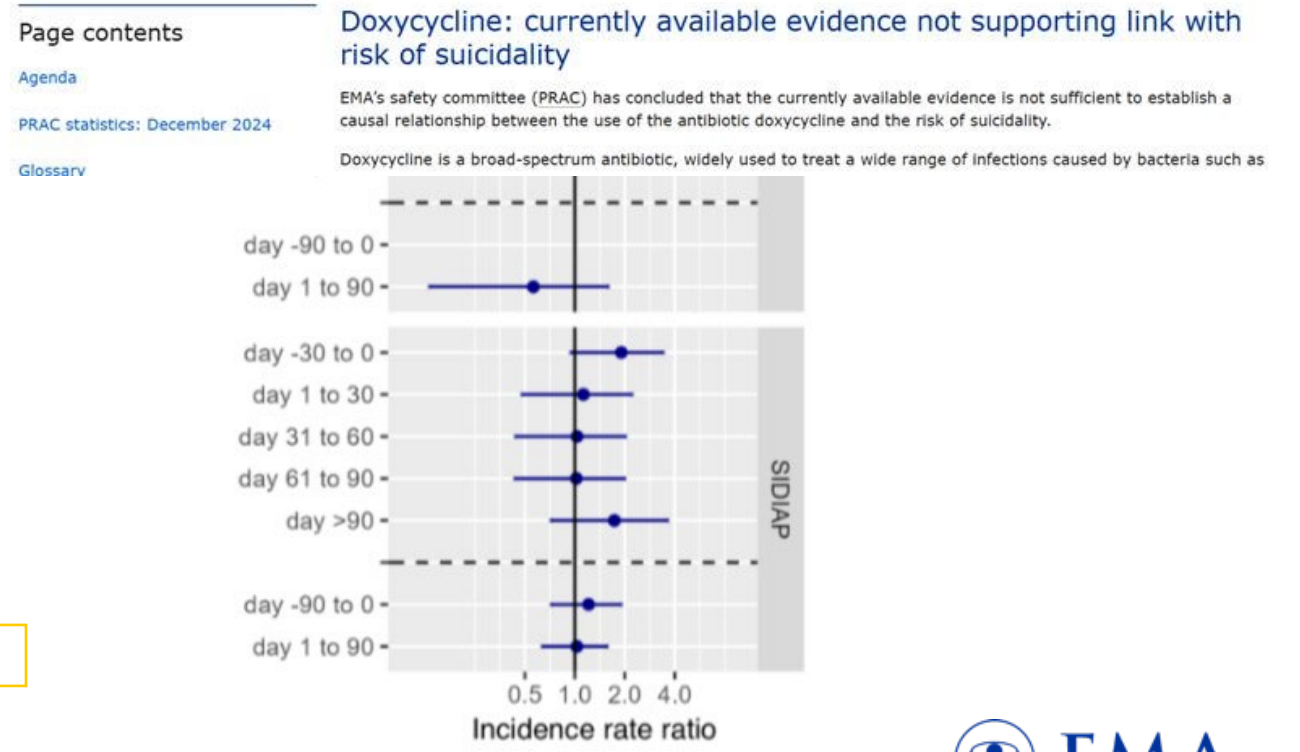
Examples of studies

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 drug and risk of suicidality
—> **No update to doxycycline product information warranted**

Pharmacovigilance Risk Assessment Committee



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Examples of studies

Respiratory Syncytial Virus (RSV) disease epidemiology (CHMP requested data to support discussion on unmet medical need)

Study results, including incidence data concerning RSV infection and hospitalization, especially for the 50–59-year-old age group (the MAH required an extension of indication for this age group) were used as evidence **to support the argument of unmet clinical need for this age group.** Results supported preparation of vaccine effectiveness studies

Committee for Medicinal Products for Human Use

Juvenile polymyositis (JPM) and dermato-myositis (JDM) and disease natural history in paediatric population (PDCO request to better understand the disease context)

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 a controlled clinical trial —> **Obligation placed on the applicant**

Paediatric Committee

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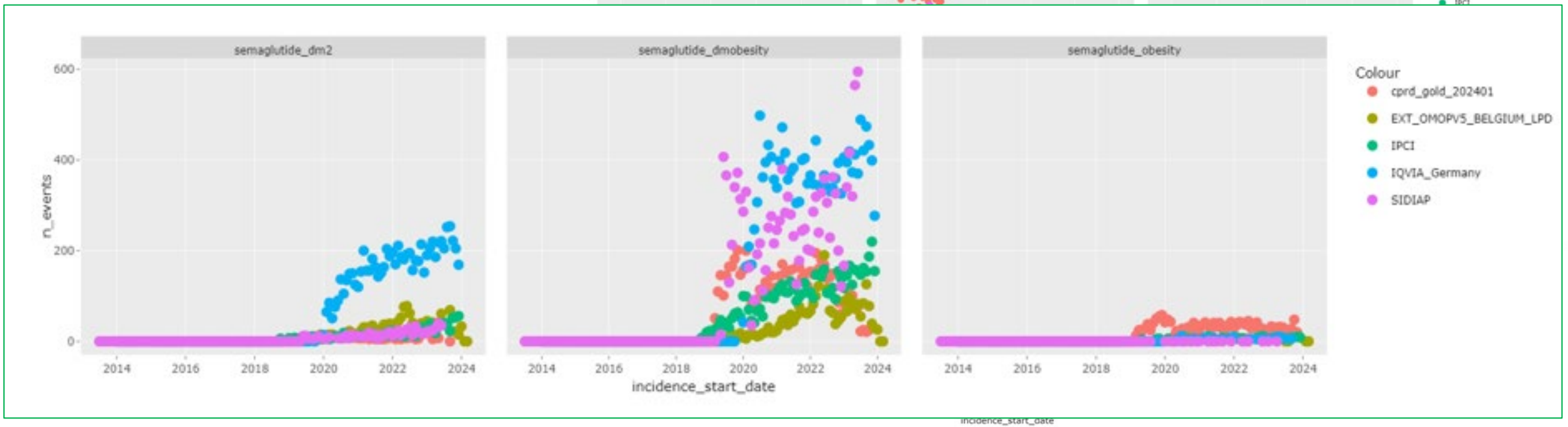
Examples of studies

GLP1-RAs utilisation (and shortages)



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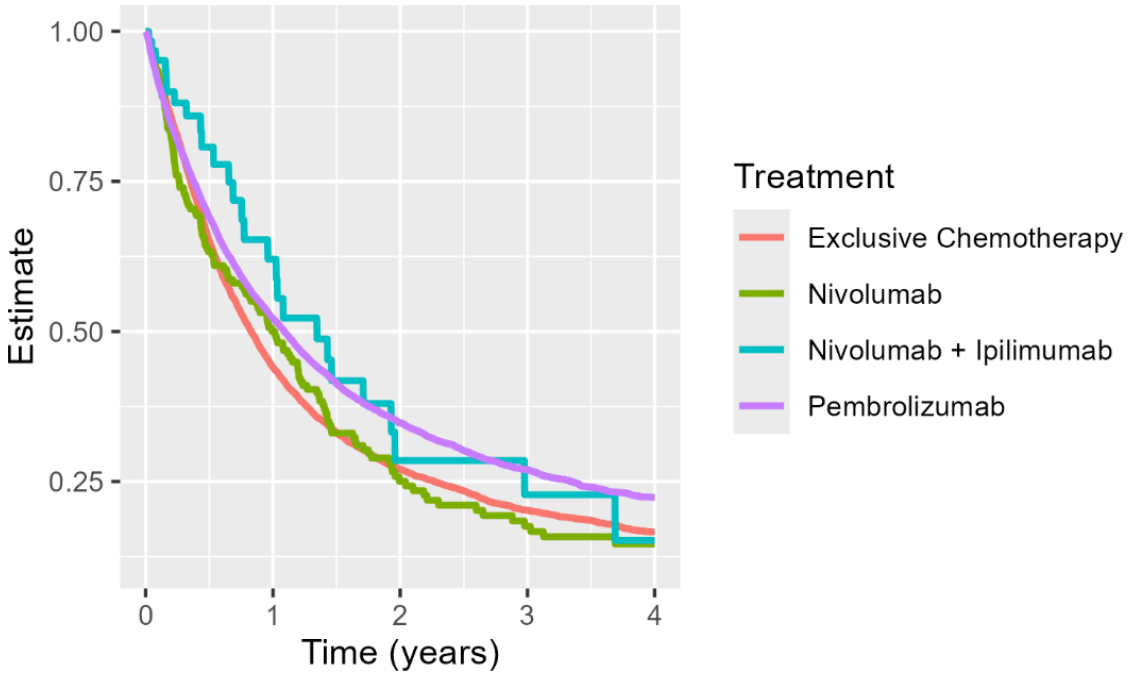
Examples of studies



Non-Small Cell Lung Cancer (NSCLC) and immunotherapy treatments (HTA bodies request to assess comparative overall survival)

Study describing the patient population treated with selected immunotherapies and chemotherapy, showing findings on the benefit of immunotherapy in comparison to chemotherapy in terms of overall survival

Results from the real-world setting **in line with results from clinical trials**

Health Technology Assessment Bodies

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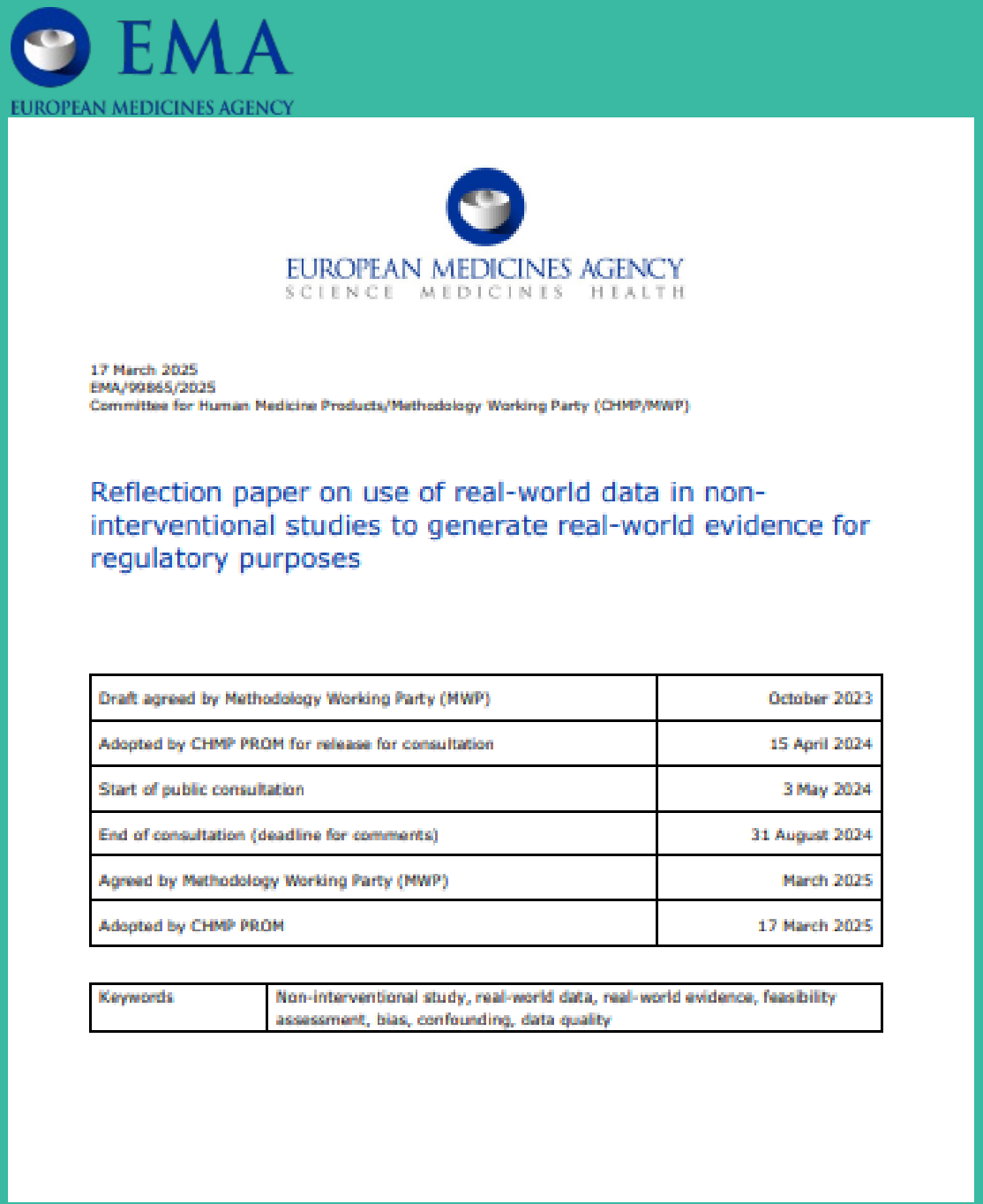

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17 March 2025
EMA/99865/2025
Committee for Human Medicine Products/Methodology Working Party (CHMP/MWP)

Reflection paper on use of real-world data in non-interventional studies to generate real-world evidence for regulatory purposes

Draft agreed by Methodology Working Party (MWP)	October 2023
Adopted by CHMP PROM for release for consultation	15 April 2024
Start of public consultation	3 May 2024
End of consultation (deadline for comments)	31 August 2024
Agreed by Methodology Working Party (MWP)	March 2025
Adopted by CHMP PROM	17 March 2025

Keywords	Non-interventional study, real-world data, real-world evidence, feasibility assessment, bias, confounding, data quality

Coming soon:

Q&A document aiming to clarify what is (is not) RWD in the context of current guidance (including the RP, the Registry-based studies guideline, and the HMA-EMA Data Quality Framework and its application to RWD)

A "***Journey towards a roadmap for regulatory guidance on real-world evidence***" published on 3 April 2025

Provides contextual information on the work done so far

Identifies RWD-related topics for potential guidance development in the future

Externally controlled trials, where external controls may come from RWD sources or clinical trials

Augmented-control trials, where a small control arm is recruited in the current trial but supplemented with external controls from RWD sources or clinical trials

Pragmatic trials

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Thank you

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