EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Regulator-led RWD studies to support decision-making

2nd Report on experience gained from February 2023 – February 2024 (2nd year of DARWIN EU)

ENCePP Plenary – 22 November 2024

Presented by María Clara Restrepo-Méndez
Data Analytics and Methods Taskforce, Real World Evidence Workstream

1st Report on the experience gained with regulator-led studies* to support regulatory decision

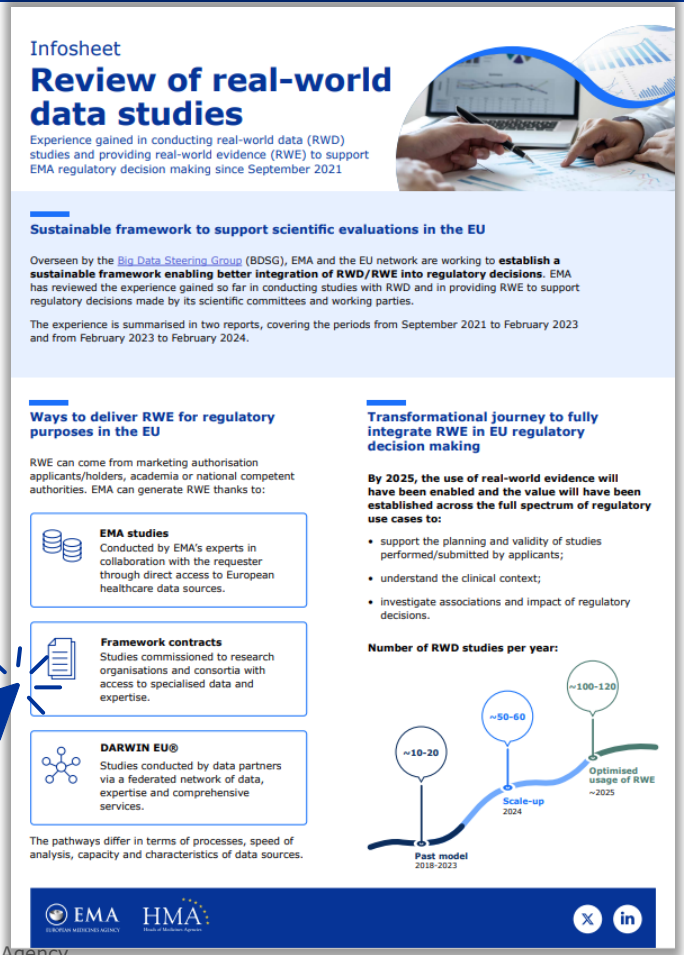
* Focus on ongoing RWE pilot activities (incl. CHMP, SAWP, CAT, PDCO, COMP, HTA/payers, ECDC) and routine RWE support to PRAC

Period covered: Sep 2021 to Feb 2023

Published in June 2023 on EMA Big Data webpage

Published in July 2024 in Clinical Pharmacology & Therapeutics: Prilla et al., 2024

Infosheet
(consolidated)



Infosheet

Review of real-world data studies

Experience gained in conducting real-world data (RWD) studies and providing real-world evidence (RWE) to support EMA regulatory decision making since September 2021

Sustainable framework to support scientific evaluations in the EU

Overseen by the Big Data Steering Group (BDSG), EMA and the EU network are working to **establish a sustainable framework enabling better integration of RWD/RWE into regulatory decisions**. EMA has reviewed the experience gained so far in conducting studies with RWD and in providing RWE to support regulatory decisions made by its scientific committees and working parties.

The experience is summarised in two reports, covering the periods from September 2021 to February 2023 and from February 2023 to February 2024.

Ways to deliver RWE for regulatory purposes in the EU

RWE can come from marketing authorisation applicants/holders, academia or national competent authorities. EMA can generate RWE thanks to:

EMA studies
Conducted by EMA's experts in collaboration with the requester through direct access to European healthcare data sources.

Framework contracts
Studies commissioned to research organisations and consortia with access to specialised data and expertise.

DARWIN EU®
Studies conducted by data partners via a federated network of data, expertise and comprehensive services.

The pathways differ in terms of processes, speed of analysis, capacity and characteristics of data sources.

Transformational journey to fully integrate RWE in EU regulatory decision making

By 2025, the use of real-world evidence will have been enabled and the value will have been established across the full spectrum of regulatory use cases to:

- support the planning and validity of studies performed/submitted by applicants;
- understand the clinical context;
- investigate associations and impact of regulatory decisions.

Number of RWD studies per year:

~10-20
Past model
2018-2023
~50-60
Scale-up
2024
~100-120
Optimised usage of RWE
~2025

EMA HMA

2nd report – Number of study requests

60

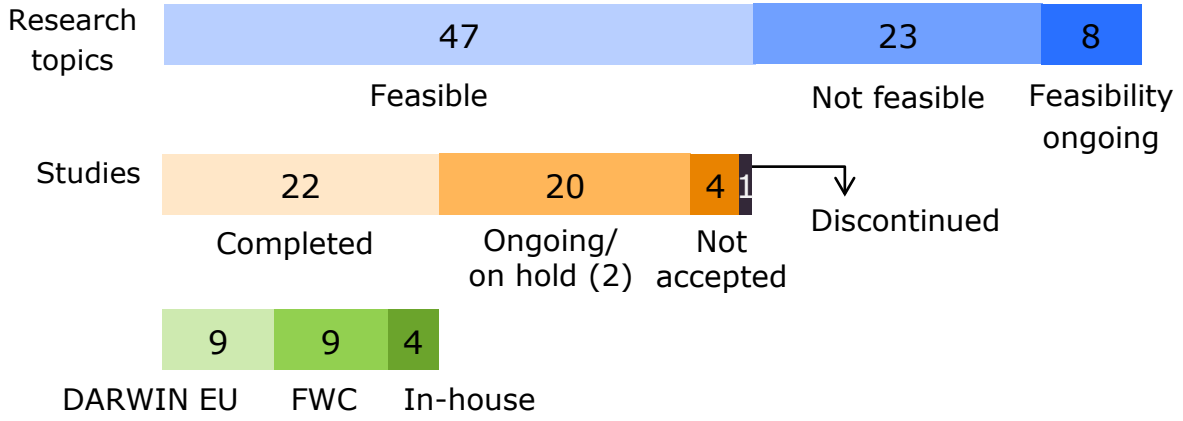
NEW research topics

(Feb '23 – Feb '24)

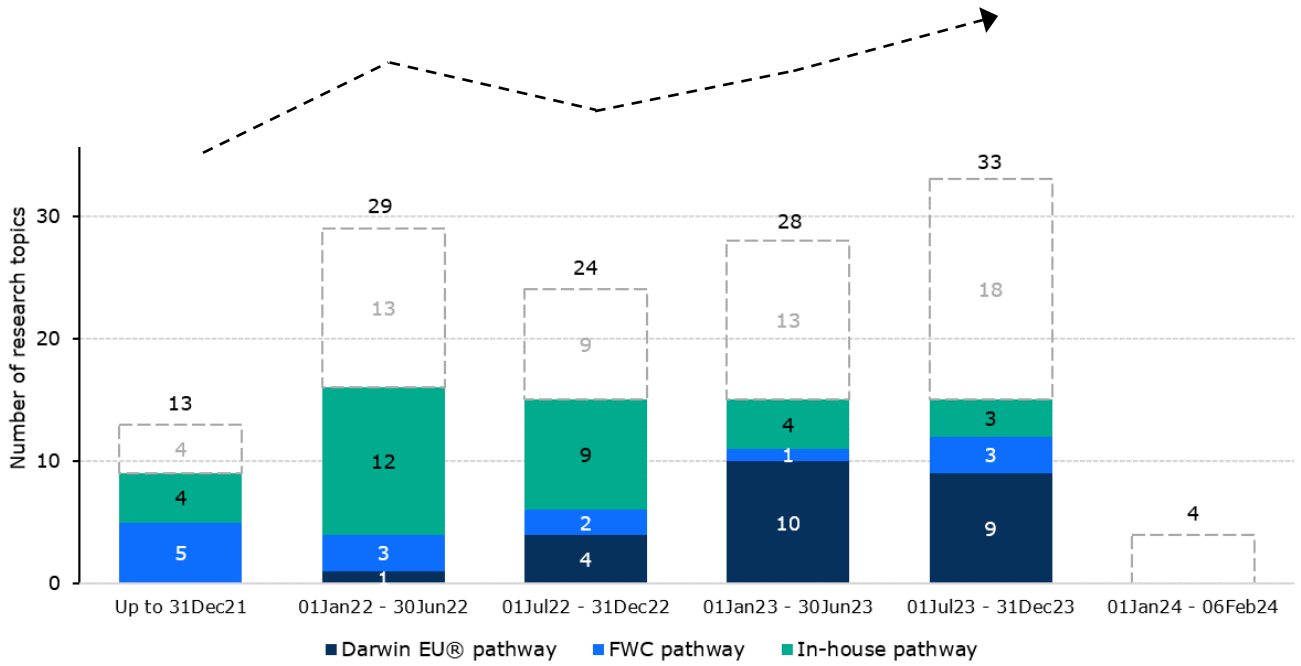
+18

Prior research topics

38	16	6
DARWIN EU	In-house	FWC

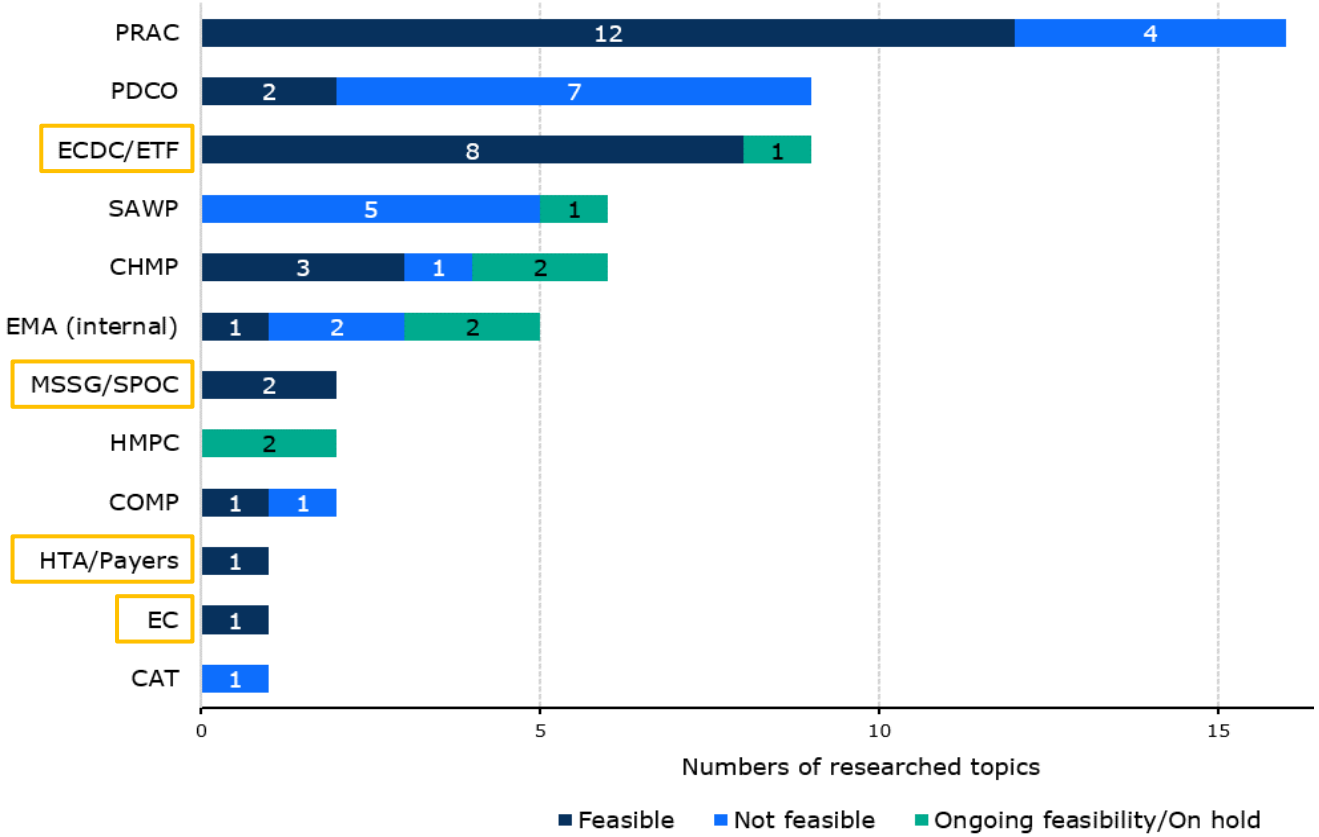


Number of studies requested over time

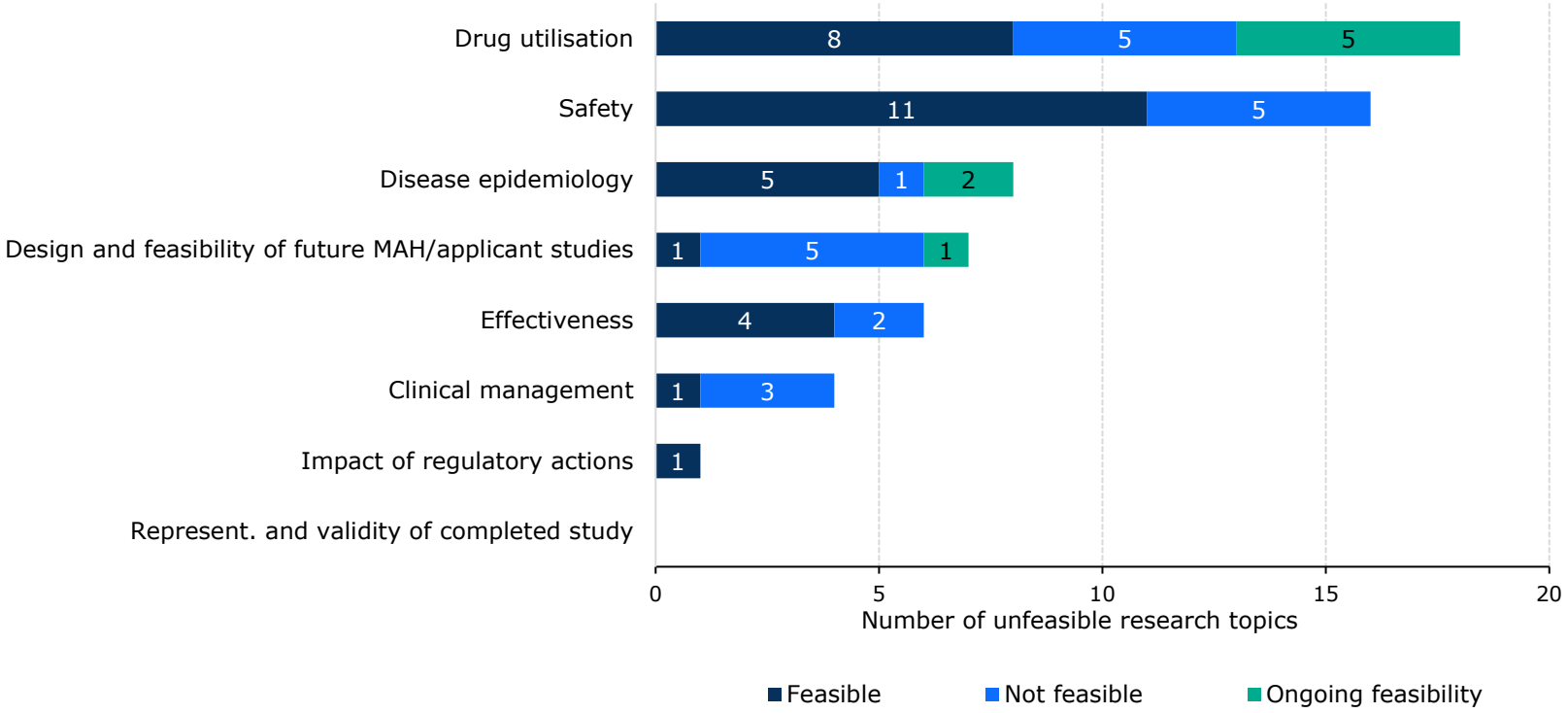


- The height of the bar reflects the total number of research topics that were identified over time
- **The coloured bars represent feasible studies (including studies ongoing, completed, or on hold)**
- The dashed bars illustrate research topics that were considered unfeasible, or where feasibility assessment was still ongoing

NEW study topics (n=60) by 'decision-maker'

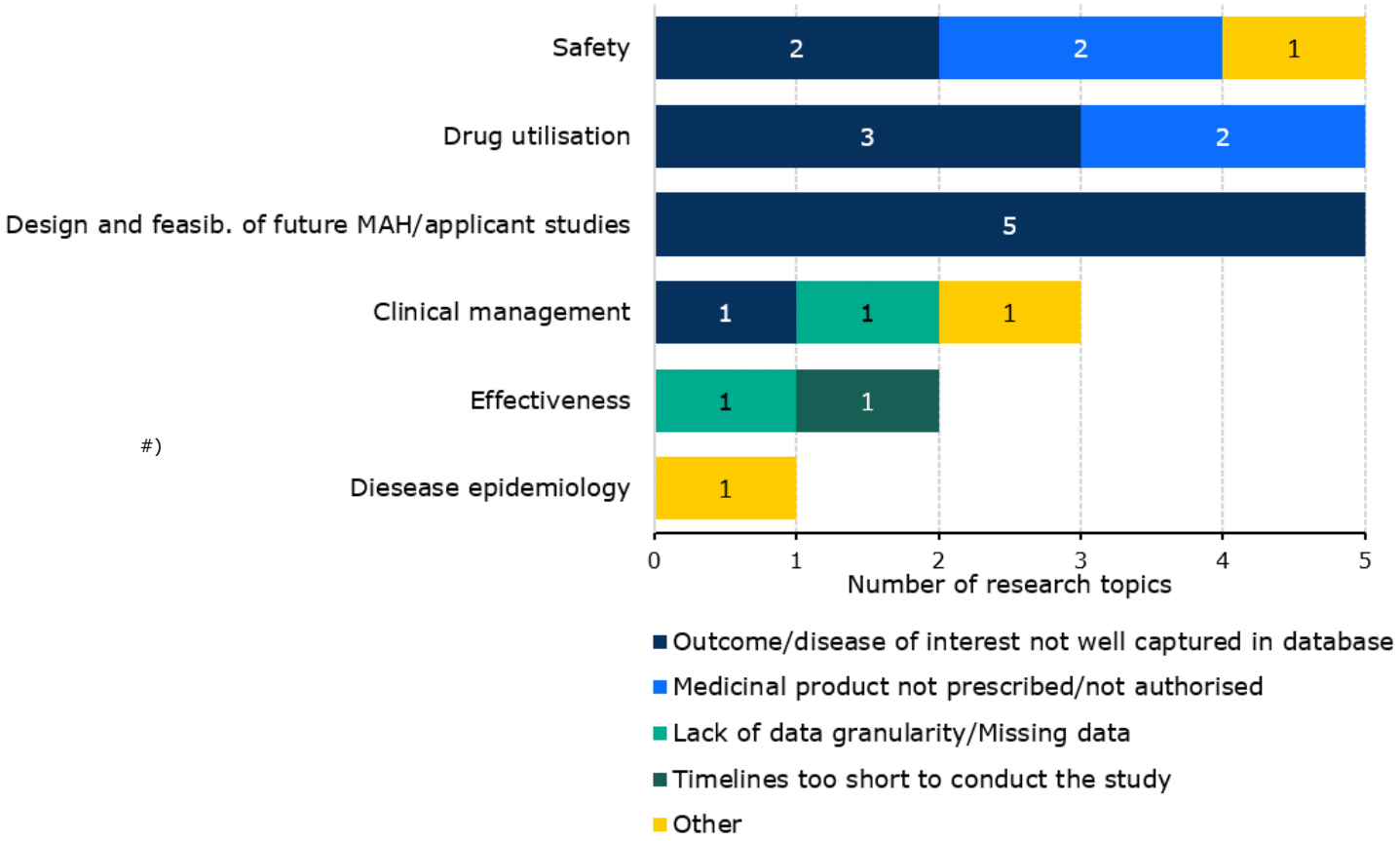


NEW study topics (n=60) by study use case



NEW study topics – reasons for lack of feasibility (n=21)

EUROPEAN MEDICINES AGENCY



#)

Highlights

- **DARWIN EU 2nd year of establishment** completed
  - 20 data partners → 130 million patients from 13 European countries
  - Main RWE generation pathway for studies to support regulatory decisions
- **40 studies** completed (22) or ongoing (18), including 13 studies to inform vaccine safety and effectiveness, and public health emergencies
- For the first time, studies conducted
  - to support monitoring of the **demand and stock levels of critical human medicines**
  - on **herbal** substances
  - for **HTA and payer** organisations
  - to support EMA's **geriatric medicines** strategy

Thank you!