EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA approach to the use of RWE in decision-making

Translating Real-World Data into Real-World Evidence – 20 July 2021

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Disclaimer

The presenter does not have any conflict of interests.

Outline

- HMA / EMA Big Data Steering Group
- Real-World Evidence (RWE) in marketing authorisation application (MAA) & extension of indications (EoI)
- RWE provided by EMA to support regulatory decision making

HMA / EMA Big Data Steering Group

The European Medicines Agency (EMA) and Heads of Medicines Agencies (HMA) set up a joint task force to describe the big data landscape from a regulatory perspective and identify practical steps to support of innovation and public health in the European Union (EU)

This led to the creation of the Joint HMA/EMA Big Data Steering Group and Big Data Steering Group Work Plan

Jan. 2020

'Ten recommendations to unlock the potential of big data for public health in the EU'

Sep. 2020

Making best use of big data for public health: publication of the Big Data Steering Group workplan for 2020-21

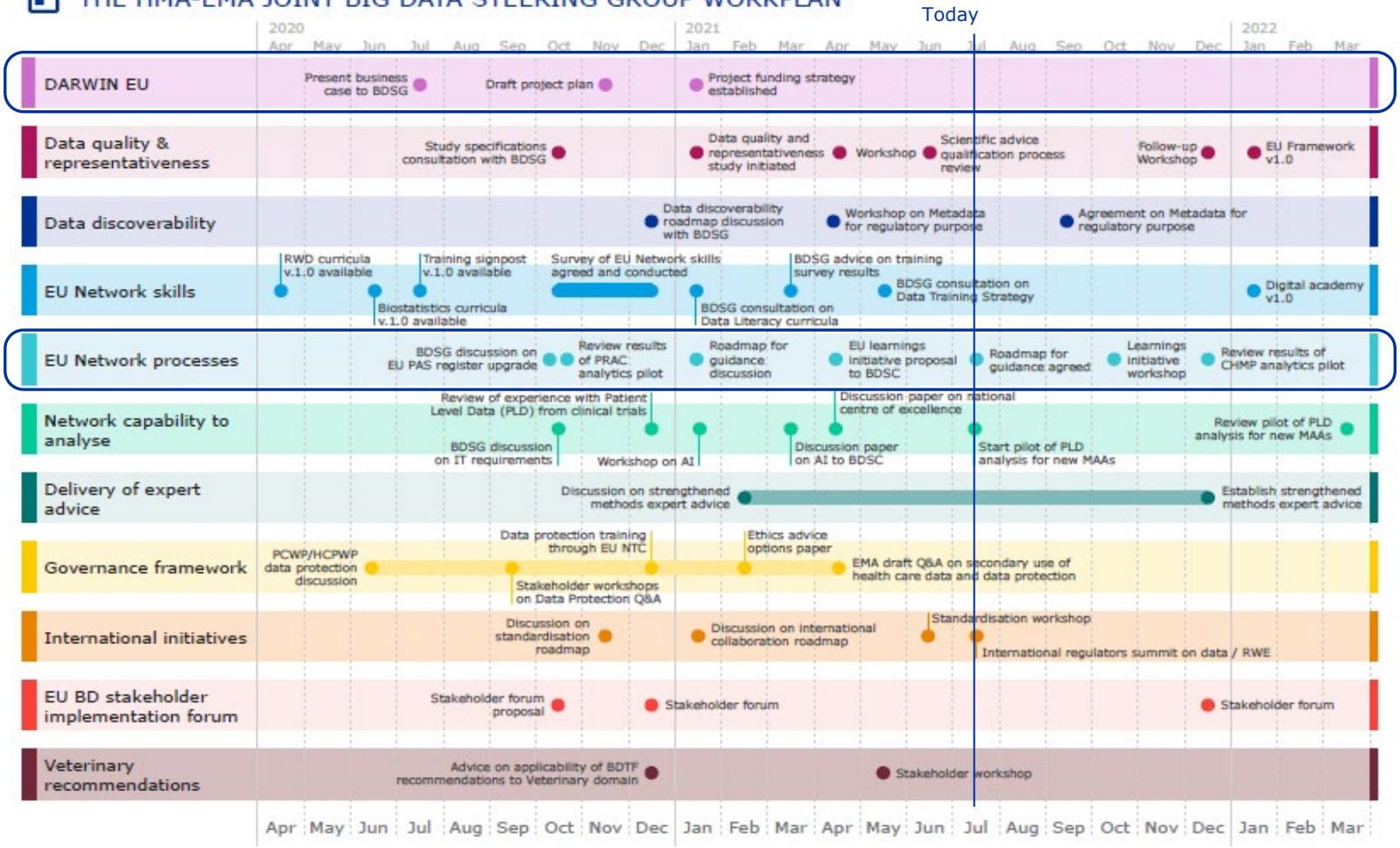
1st Big data steering group meeting in May 2020

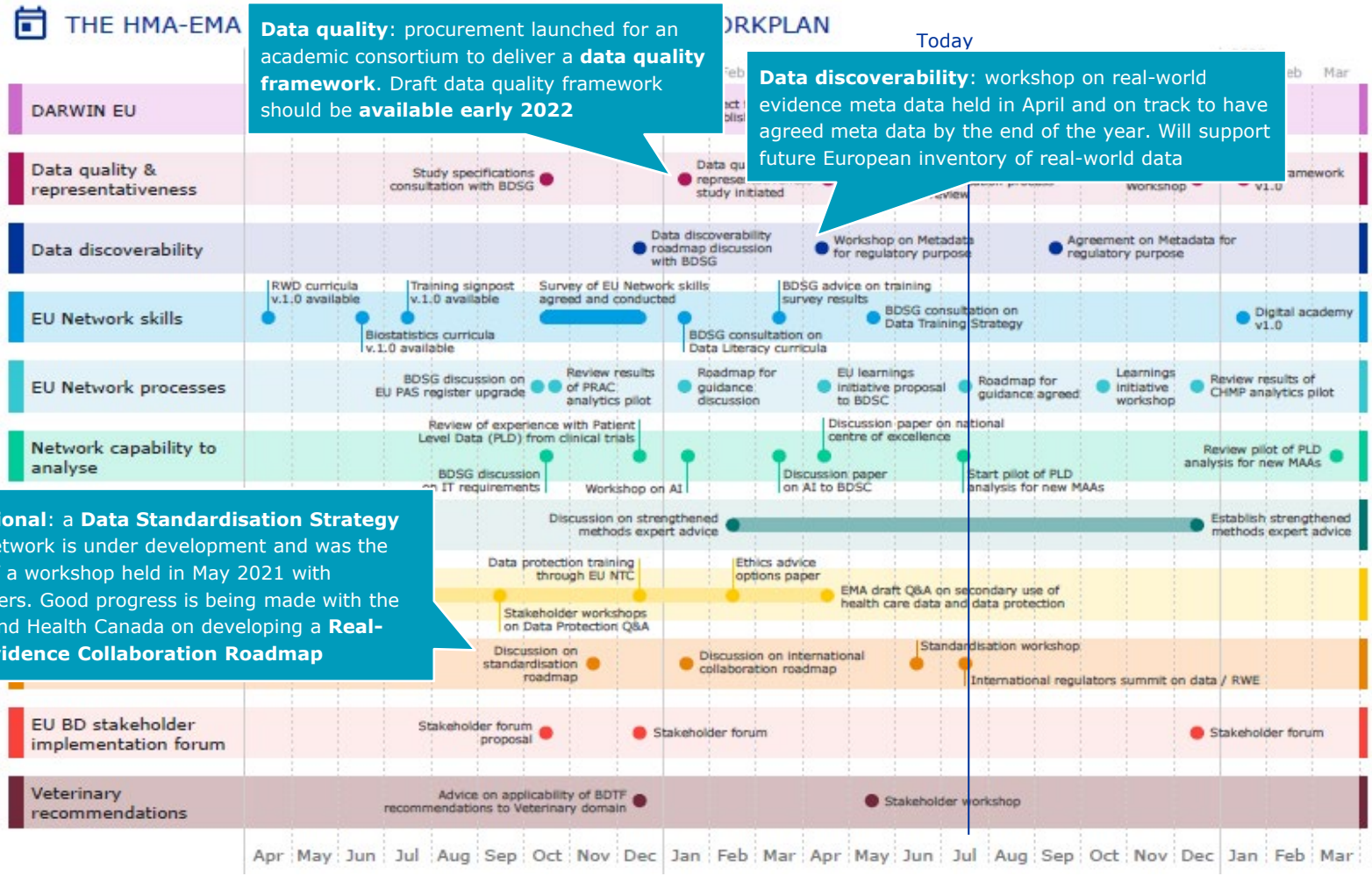
May 2020

1st big data stakeholder forum

Dec. 2020

THE HMA-EMA JOINT BIG DATA STEERING GROUP WORKPLAN





RWD/RWE in MAAs and EoIs in 2018-2019

Problem statement

- Lack of detailed information on the use of RWE in marketing authorisation applications in terms of objectives, data sources, methods, strength and weakness and outcomes of its assessment

Objective of the study

- Characterise RWD/RWE included in centralised marketing authorisation applications (MAA) and extensions of indications (EoI) submitted in 2018-2019 and explore its contribution to benefit-risk decision-making

Methods

- All submission of MAAs and EoIs including withdrawn applications
    - Exclusion of MAAs for generics, informed consent, well established use & duplicate applications
- Manual review of final/latest version of CHMP AR Report and Risk Management Plan with quality control
    - Extraction of data using standard form by seven investigators
    - Verification by two independent reviewers of samples of MAAs/EoIs reviewed by the investigators

RWE in MAAs and EoIs – Preliminary results

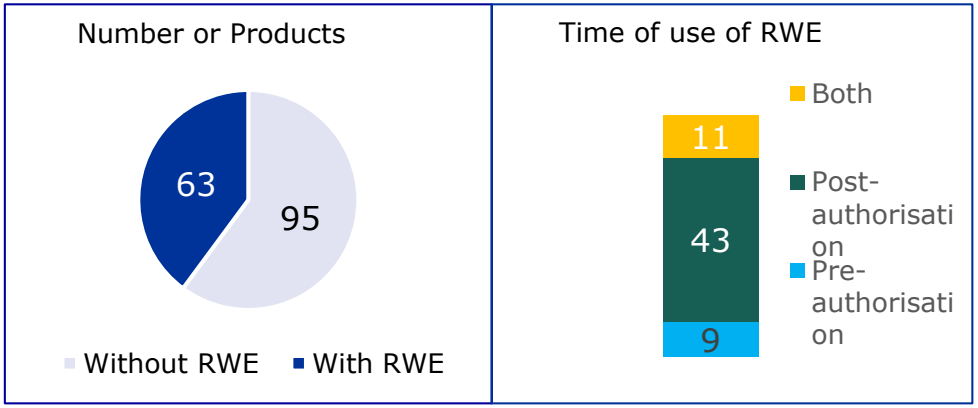
RWD/RWE used in 40% of MAAs (mainly post-authorisation) and in 18% of EoIs (both pre- or post-authorisation)

- Pre-authorisation: mainly supporting study looking at efficacy/effectiveness
- Post-authorisation: mainly RMP Category 3 (for studies included in RMP) looking at safety

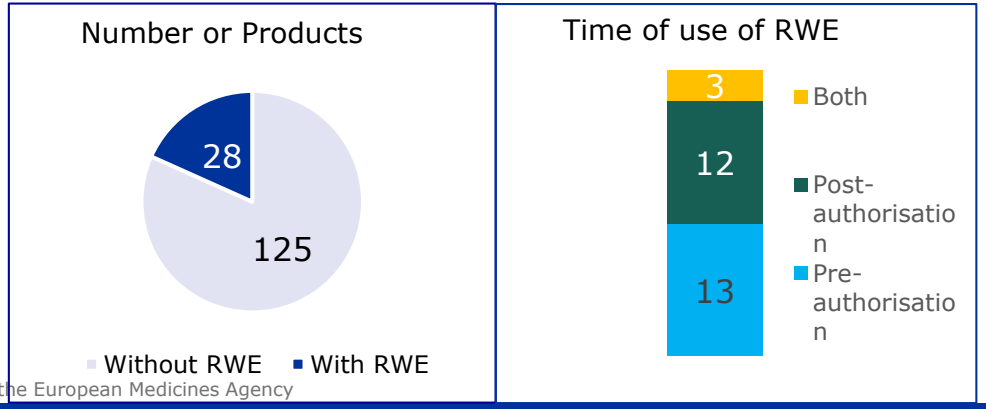
For both, the most common data sources were registries, followed by hospital data and electronic health care records

Majority of products: Antineoplastic and Immunosuppressants (35% MAA and 42% EoI)

Initial MAA (n=158)



Extension of indication (n=153)



RWE in MAAs and EoIs – Conclusions and further research

- There is a widespread use of RWE to support MAAs and EoIs

- Further work needed to evaluate the impact and usefulness of RWE in the regulatory evaluation
    - How is it used, which characteristics are most important, is a consistent approach followed in decision-making?

- Currently no framework for using RWE in submissions: need for guidance targeted to various stakeholders (industry, academia, regulators, registry owners etc.)
    - Draft guideline on registry based studies currently under amendment following public consultation

- No structure in the way RWE is submitted
    - Consideration for standard definitions (internationally agreed?), quality assessment…

The use of RWE by EMA committees

- There has been an increase in the use of RWE in the development, authorisation and post-marketing surveillance of medicines to facilitate decision-making
- Several mechanism for obtaining RWE are available to Scientific Committees

Current way to obtain RWE	Requests or obligations to pharmaceutical companies	Analysis of public information including public scientific literature	Data analyses and studies conducted or initiated by NCAs
RWE provided through the Agency with additional studies	1 **EMA studies** on the electronic health databases accessible in-house	2 **Studies procured** through the EMA framework contracts	3 **DARWIN EU** (starting from 2022)

Currently there are two mechanisms to provide additional RWE

1 EMA studies on in-house accessible databases

- Currently three primary care databases (UK, FR, DE)
- On-going procurement to increase geographical representation and access to hospital prescribing
- 98 EMA in-house analyses or full studies performed from 2013
  - The studies supported evidence needs of EMA Committees, mainly PRAC

2 Studies procured through the EMA framework contracts

- Allows access to different data sources and scientific expertise
- 30 studies funded from 2010
- A new framework contract, with a broader set of organisations will be in place in September

3 DARWIN EU®

EU Medicines Regulatory Network

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

The Coordination Centre

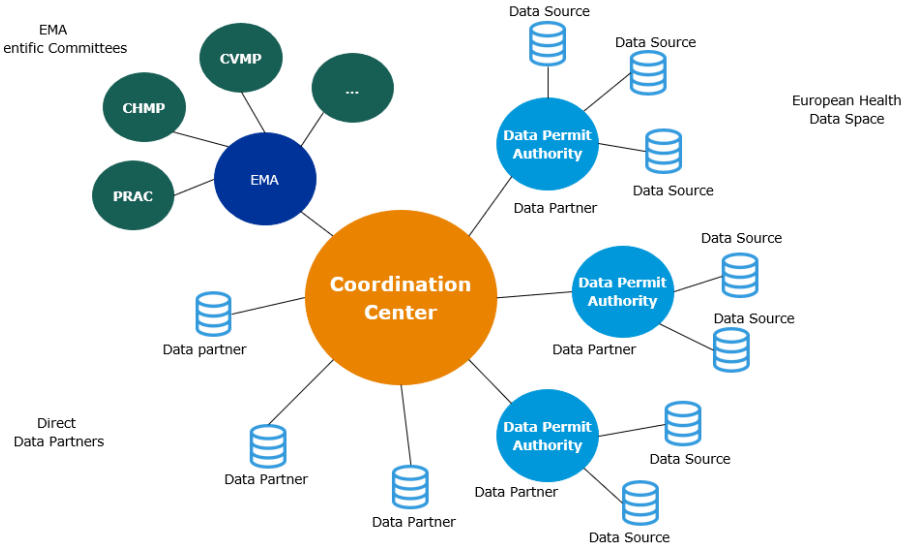
- Acts as **entry point** into this federated network
- **Establishes** and **maintains the network** (including onboard/maintain data sources), manage the **execution of scientific studies**

Data Partners, incl. Data Permit Authorities

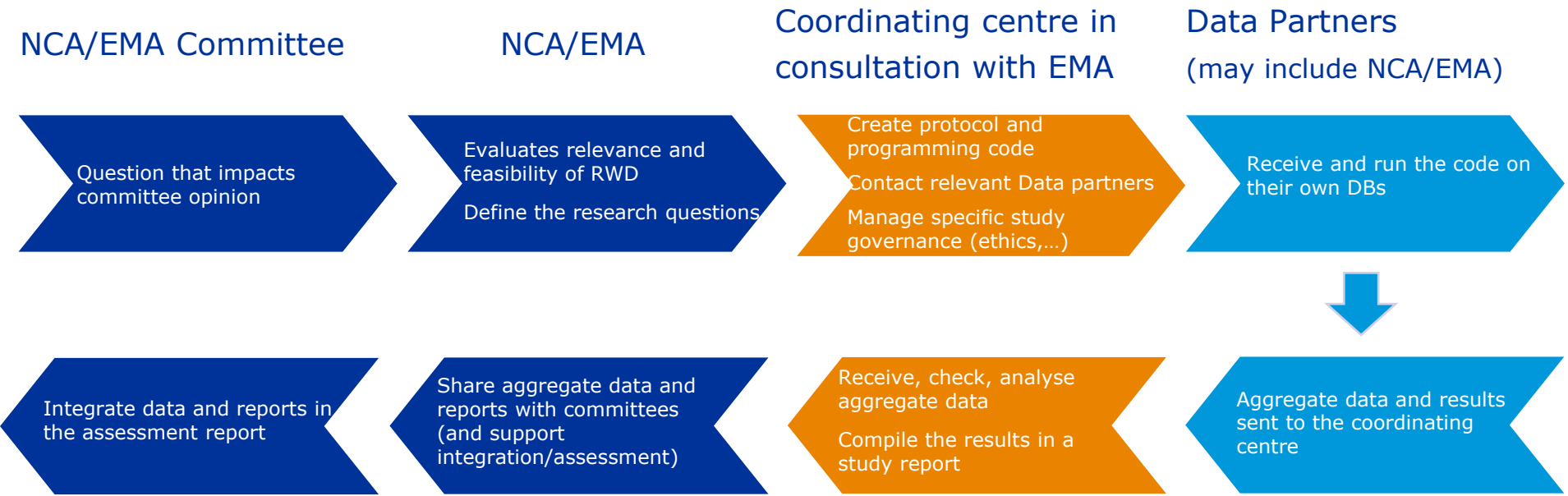
- **Partners** who have access to data, or who may request analyses in a data source and provide results to the Coordination Centre
- This includes **Data Permit Authorities** (DPAs), already existing or to be created as part for the EHDS

Other's organisations

- **European Network of Centres for Pharmacoepidemiology and Pharmacovigilance -** integrate and update methodological practice in pharmacoepidemiology, identification of data sources and registration of studies
- **European Health Data Space -** DARWIN EU will fully integrate with the EHDS
- **Ongoing EU and national projects** relevant to the quality, transformation, and/or analysis of real-world data, inc. European networking initiatives, such as European platforms for registries or the European Reference Networks (ERNs)



3 DARWIN EU® - Conducting analyses and studies



Any questions?

Further information

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