EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

HMA
Heads of Medicines Agencies

5.1 Big Data Steering Group: workplan and deep-dive on clinical trials raw data project

Agenda item 5.1

PCWP/HCPWP joint meeting, 21 & 22 September 2021

Presented by Francois Domergue and Eftychia Eirini Psarelli
EMA, Data Analytics and Methods Task Force

An agency of the European Union

Big Data Steering Group updated workplan

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BDSG update - workplan

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

July 2021

EMA MB and HMA informed of BDGS workplan 2021/2023

1st Big data steering group meeting in May 2020

May 2020

BDSG workplan 2022/2023

May and June 2021

Publication of BDSG workplan 2021/2023

Aug. 2021

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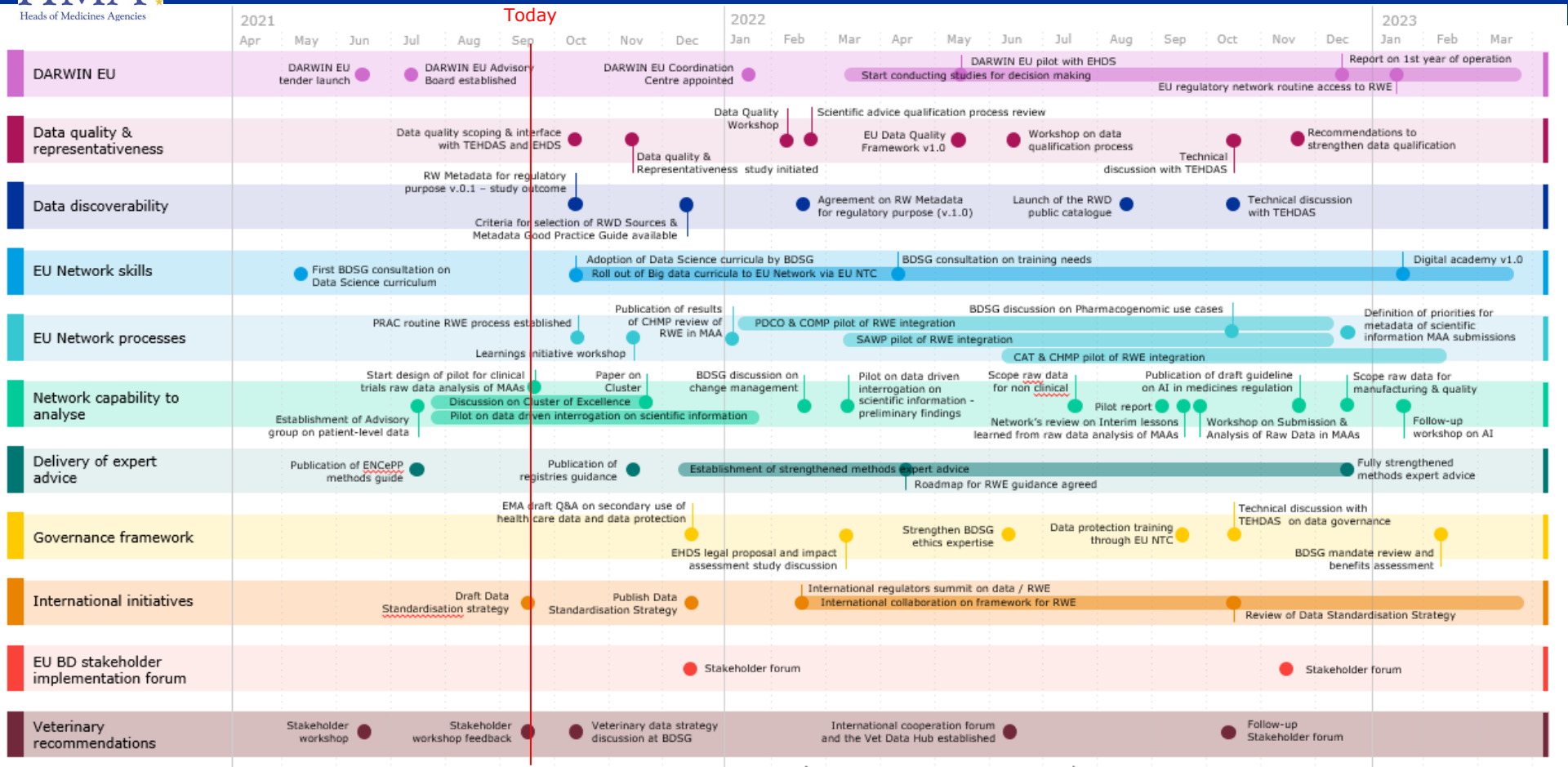
BDSG workplan 2021-23



Key progress and future highlights

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	2021	2022
DARWIN EU	**Procurement launched -** 1st meeting of the **Advisory board**	**Coordination Centre appointed** – Support EHDS2 pilot – **Start conduc pilot/studies** for decision making
Data quality & representativeness	**Procurement launched** for academic consortium to deliver a data quality framework	**EU Data quality Framework v1.0 available - 2 workshops** on data qua and data qualification
Data discoverability	Progress on **selection of metadata for RW**	**Agreement on RW Metadata for regulatory purpose** (v.1.0) - **Launch of RWD public catalogue**
EU Network skills	**Data science curriculum** finalis **skills** completed - **Training delivery** alised	**Roll out** of Big data curricula (content outsourced and delivered through EU-NTC)
EU Network processes	**RWE use cases developed** with P , PDCO, COMP – **Learnings initiative workshop**	**RWE integration pilots** (PDCO, COMP, SAWP, CAT, CHMP)
Network capability to analyse	1st discussion on **Cluster of Excellence** **Advisory group on CT raw data** established	**Workshop on Raw Data in MAAs** (including CHMP pilots) **Draft guideline on AI**
Delivery of expert advice	**ENcEPP RWE methods guide publish**	Publication of **registries guidance** - **Roadmap for RWE guidance ag**
Governance framework	**BDSG recommendations on ethics ac**	**EHDS** legal proposal and impact assessment study supported – publish **protection**
International initiatives	**Data standardisation strategy** adopted – Progr on **RWE Collaboration Roadmap** with FDA and H	onal **regulators summit on data and RWE**
EU BD stakeholder implementation forum	**Multi stakeholder forum on Big Data**	Stakeholder forum
Veterinary recommendations	**Workshop on the** Veterinary **Data Strategy**	**International** cooperation **forum** and the **Vet Data Hub** established

PCWP / HCPWP representatives

PCWP / HCPWP representatives invited

PCWP / HCPWP representatives invited

PCWP / HCPWP representatives informed

PCWP / HCPWP representatives informed

PCWP / HCPWP representatives informed

PCWP / HCPWP representatives invited

BDSG members: EU Patient associations representative + EU healthcare professional associations representative

Accessing and analysing RWD: Update on DARWIN EU

- RWE integration into EMA committees decision making has started with:
  - review of PRAC RWE integration lesson learned in 2021 and
  - review of RWD/RWE in Marketing authorisation applications (MAA) and extensions of indications (EoI) submitted in 2018-2019
  - pilots with EMA committees planned for 2022,
- DARWIN EU webpage has been launched
- The Tender for the service provider to act as the DARWIN EU® Coordinating Centre has been launched in June 2021 and is in progress:
  - Appointment of the Coordination Centre on track for early 2022 to start delivering pilot/studies in 2022 to support EMA committees
- 2nd DARWIN EU Advisory Board is organised for September 2021

More information in the back-up slides

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Deep-dive on clinical trials raw data project

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Definition & legal background

- Raw data / Individual Patient Data (IPD) / Patient Level Data (PLD) / is **defined** as:
  - 'data, including imaging data, at an individual patient level which is directly assessable in terms of reanalysis or additional analyses'
  - 'individual patient data in electronic structured data formats, e.g. Extensible Markup Language - XML (technical definition)'
- Clinical trial data **already provided by marketing authorisation applicants and sponsors** in modules 4 and 5 of all MAA dossiers
  - EMA currently receives this data in the form of PDF listings; in a format that does not support or even hinders data analysis
  - In contrast to PDF listings raw data is directly assessable in terms of reanalysis, additional analyses or visualisation

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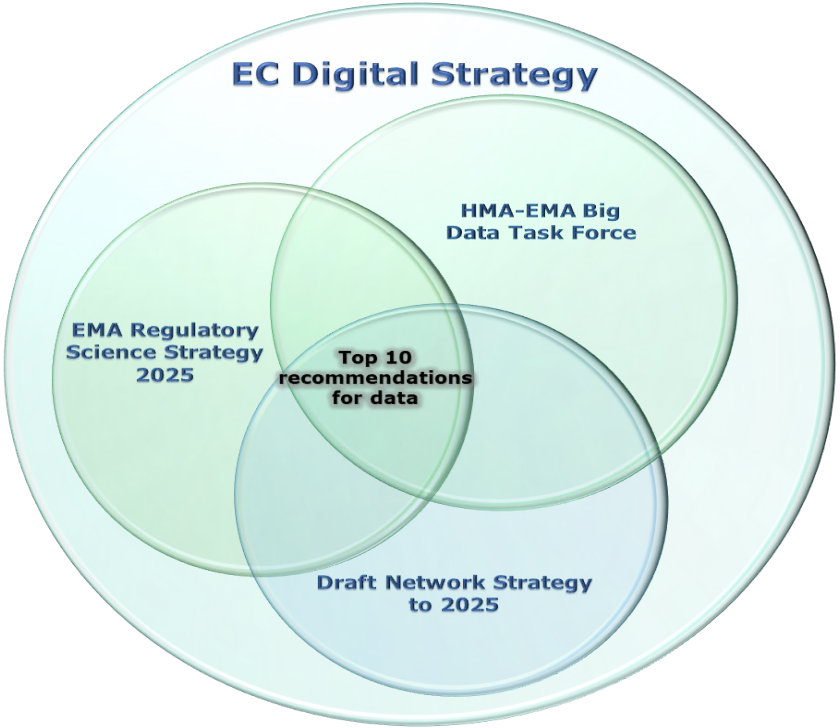
The timing is now… for raw data

- Commission digital strategy: **"EU health data space"** (EHDS)
- Joint HMA EMA Big Data Task Force **Top-ten data recommendations**
- **EMA Regulatory Science Strategy to 2025**
- **EU Network Strategy to 2025** includes data and digital pillar
- EC **Pharma Strategy** and **Health Union**



Vision: innovate to turn data into decisions on medicines that create a healthier world

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Big Data Task Force Priority recommendations

1	Deliver a sustainable platform to access and analyse healthcare data from across the EU Data Analysis and Real World Interrogation Network: **DARWIN EU**
2	Establish an EU framework for data quality and representativeness
3	Enable **data discoverability**
4	Develop EU Network skills in Big Data
5	Strengthen EU Network **processes** for Big Data submissions
6	Build EU Network capability to analyse Big Data (technology / analytics)
7	Modernise the delivery of expert advice
8	Ensure data are managed and analysed within a secure and ethical governance framework
9	Engage with international initiatives on Big Data
10	Establish an EU Big Data 'stakeholder implementation forum'
11	Veterinary recommendations

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Raw data Project Objectives

- The **purpose** of the project is to **determine the regulatory benefit of access to raw data** by building capacity and capability to receive, store, manage and analyse raw data.
- The project will **put in place procedures and safeguards to process raw data**, including *clinical* and *non-clinical* data, in accordance with data protection legislation.

- **Establishing the Advisory group on raw data** identified in HMA-EMA Joint Big Data Taskforce Phase II report on lifecycle regulatory submissions raw data in order to examine in detail network capacity and capability issues as;
- **Performing the proof-of-concept pilot** in order establish the value of IPD and to build, step by step, capacity to analyse raw data;
- **Fostering stakeholders' engagement** through a communication plan;
- **Establish the capability** (process, skills, technology) and **capacity** to process IPD, **leveraging**, where possible, **existing EU and international technology initiatives**.

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Expected Benefits (this list is not exhaustive)

1. **The speed and efficiency** of assessment will be increased by early identification of issues during the evaluation process; shorter and more targeted list of questions (for manufacturers), reduced overall burden of re-analysis through automated results verification, reduced number of rounds of questions. Increased ability to provide timely responses during critical regulatory issues.

2. **Faster patient access to innovative medicines and optimisation of safe and effective use**; reassurance that medicines continue to be authorised based on robust evidence

3. **Improved collaboration in response to emerging data-related and analysis-related issues**

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Approach to address Project Objectives

Learning

- Learn from past regulatory experience with raw data;
- Be opportunistic when assessment requires submission of raw data;
- Plan a series of proof-of-concept pilots.

Understanding

- Identify clinical and non-clinical use cases across the lifecycle of submissions;
- Interview Rapporteurs and assessors about practical use of raw data;
- Prioritise use cases based on business needs of the Agency.

Communication

- Big Data Steering Group and CHMP;
- Working Parties and cross-NCA advisory group;
- Stakeholders communication plan.

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Call for interest to join Advisory group on Raw Data

- A patient representative is required

This group would help design the pilot and examine the practical aspects of raw data analysis such as:

- the particular circumstances in which raw data assessment would add value
- the requirements for technical infrastructure, data standards and tools
- explore opportunities for training and support to assessors
- list not exhaustive

Please register your interest by **30th September 2021** to Eftychia Eirini Psarelli (eftychiaeirini.psarelli@ema.europa.eu)

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Thank you

Acknowledgements:

The members of the Big Data Steering Group

BDSG secretariat

HMA secretariat

Commission colleagues

EMA colleagues

Further information

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