5.2. Big Data Steering Group Workplan

PCWP/HCPWP and all eligible organisations Annual Meeting

14-15 November 2023

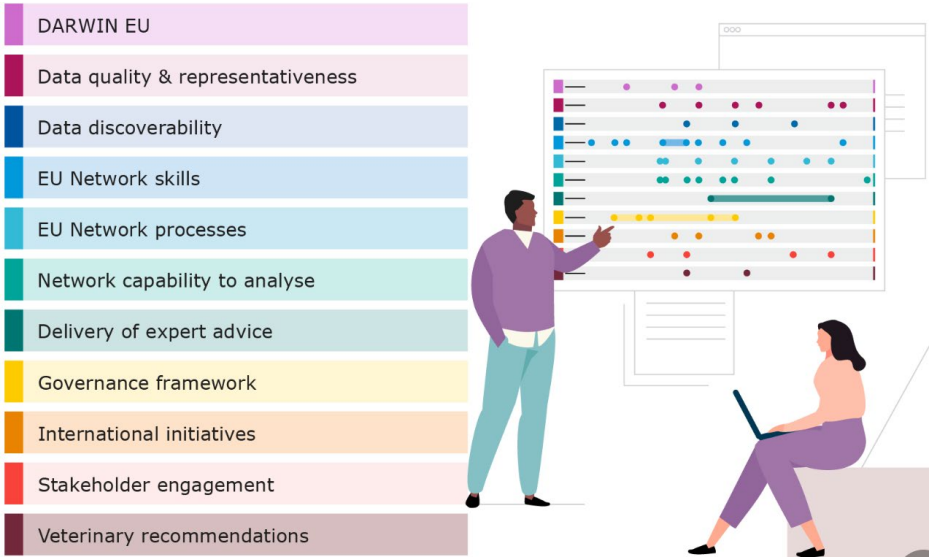
Presented by Denise Umuhire
Data Analytics and Methods Taskforce

Big Data Steering Group (BDSG)

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 for the European medicines regulatory network to make best use of big data** in support of innovation and public health in the European Union (EU)

Goal: "to increase the utility of big data in regulation, from data quality through study methods to assessment and decision-making. It is **patient-focused** and guided by advances in science and technology"

- BDSG workplan 2022-2025
- BDSG workplan 2023-2025

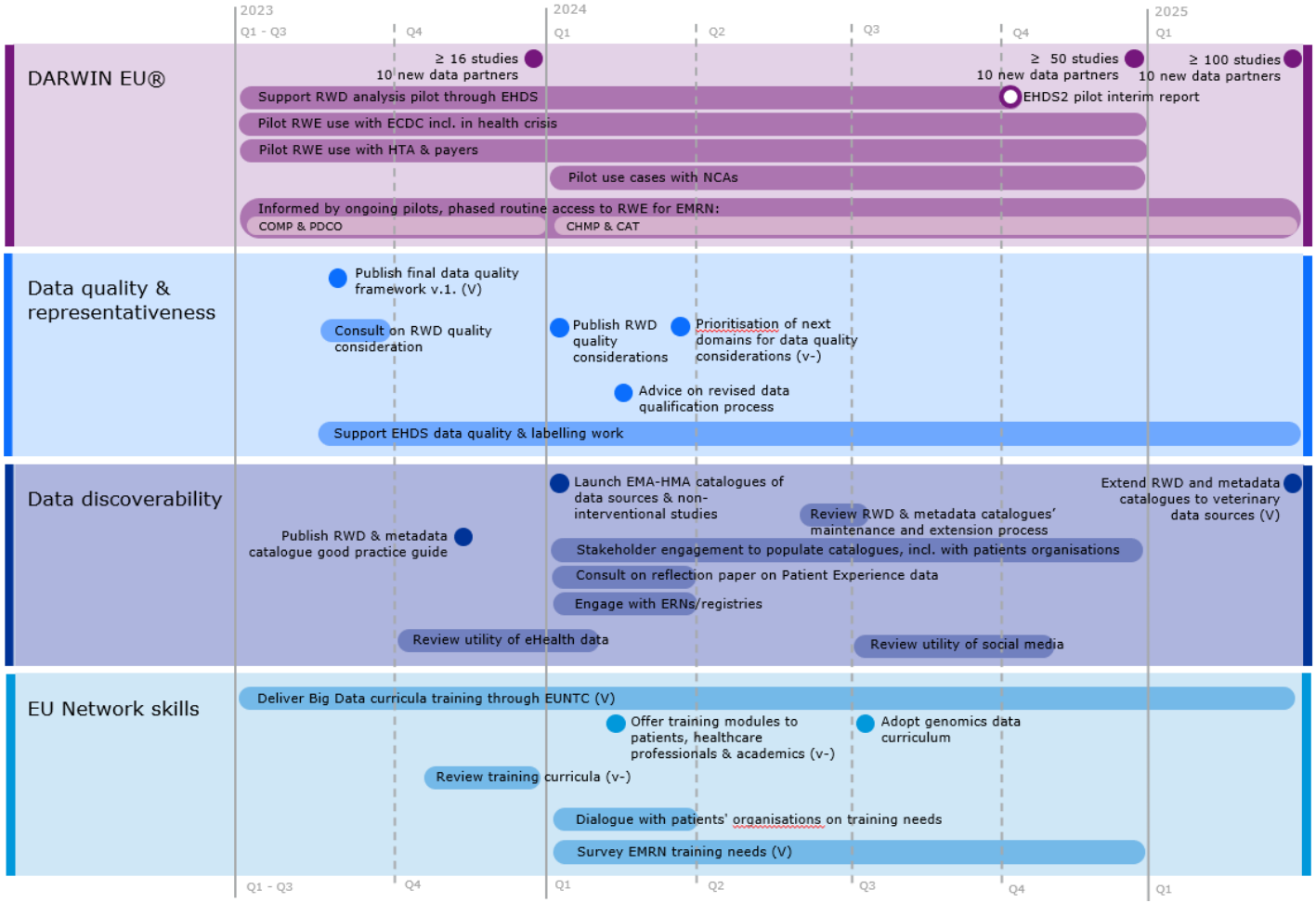


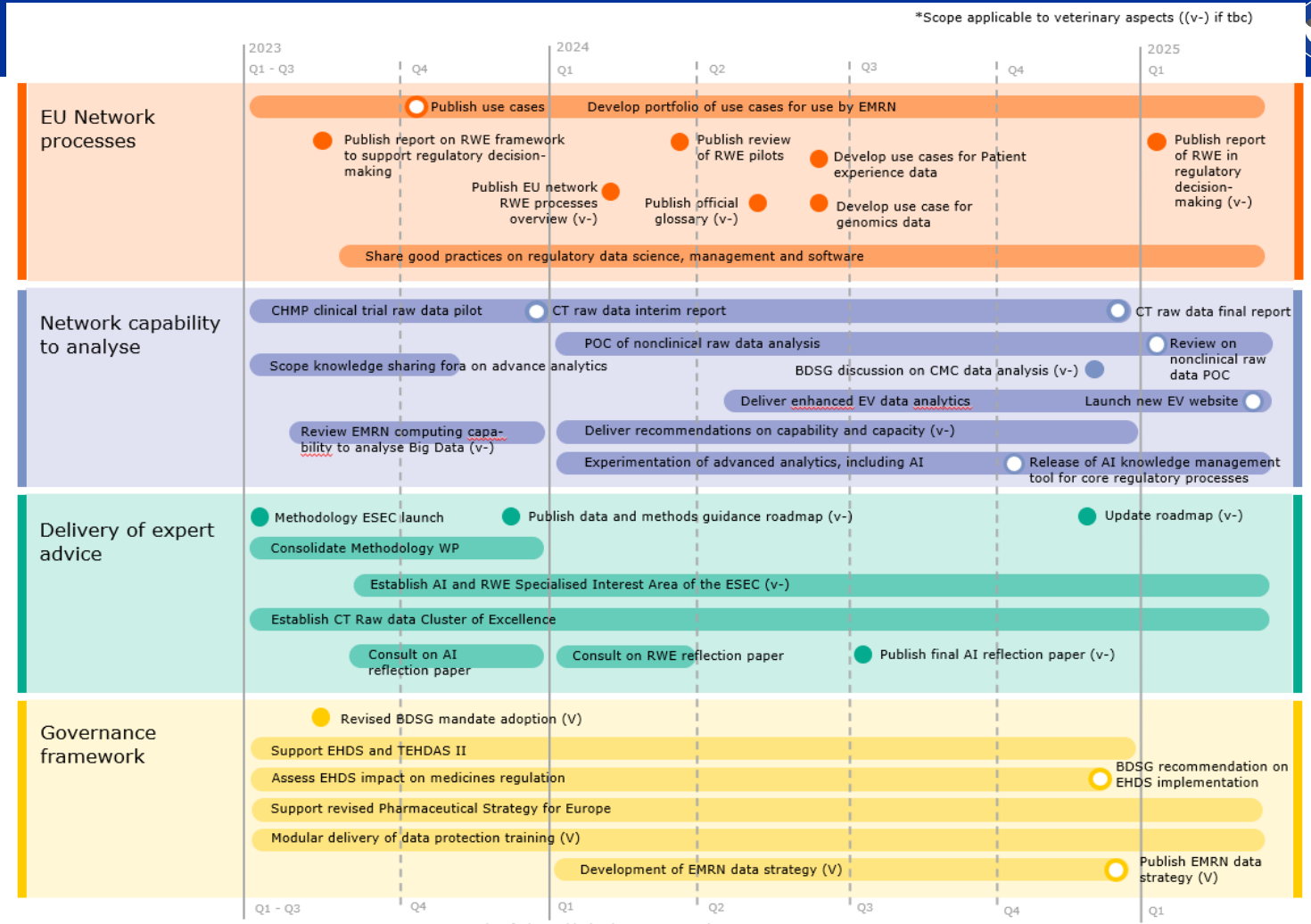
4th BDSG workplan 2023-2025 published – key changes

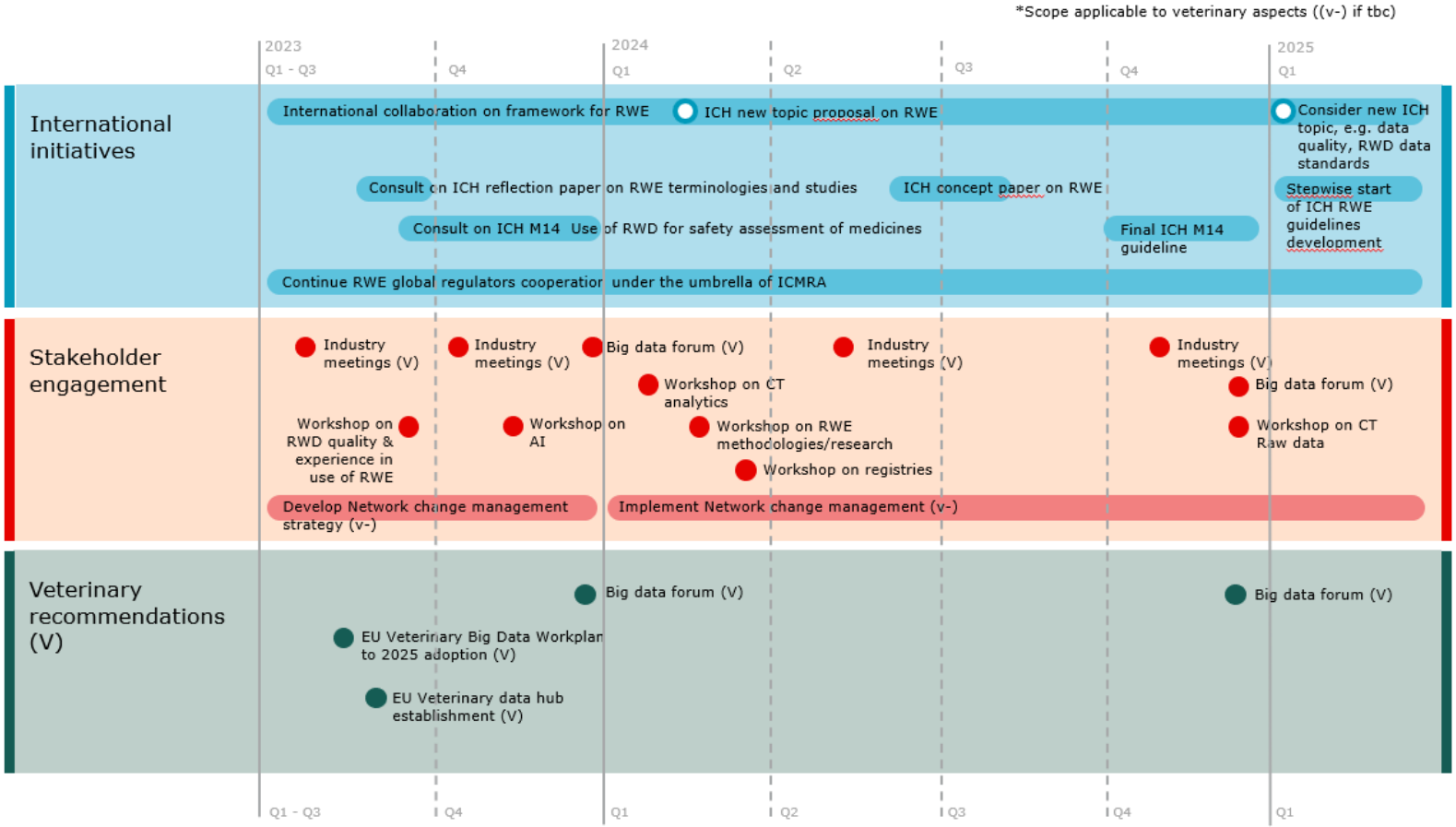
- **Intensification of engagement with patients' organisations:**
  - public consultation on a reflection paper on Patient Experience Data (PED)
  - collaborate to generate a research program exploring use cases to analyse and use PED in regulatory context
  - dialogue on training needs
  - workshop on registries
  - call to populate the real-world data (RWD) catalogues with patient data sources
- Consultation on **Real World Data (RWD) quality considerations**
- Clarifications on **plans for RWE guidance, at EU and International level**
  - public consultations (on the EU and ICH RWE reflection papers and the ICH M14 guideline) and collaboration with international regulators (via ICMRA)
- **Exploration of analysis of new data types**:
  - development of use cases for **genomics** data
  - launch of a Proof Of Concept on **nonclinical raw data analysis**
  - discussion on **Chemistry, Manufacturing and Controls (CMC) data analysis**
- **Experimentation with advanced analytics**, including AI, release of a 1st AI knowledge mining tool
- Development of overarching **EMRN data strategy**
- Addition of the **EU Veterinary Big Data Workplan to 2025**

BDSG workplan 2023-2025

*Scope applicable to veterinary aspects ((v-) if tbc)







Big Data SG workplan on PED

(included in the broader EMA's PED community workplan)

- **Understand and increase transparency on the current use of PED** in regulatory context to support evaluations and decision making
- **Identify challenges and opportunities** for enhancing optimal and impactful use of PED
- **Establish the value of PED** in regulatory decision making and beyond, in collaboration with patients and other stakeholders

BDSG workplan 2023-2025 will include key actions on PED (1/2)

	Key actions	Status / next steps
Governance framework	**Patient voice** to be represented in the new EMRN data governance structure and maintained in the BDSG membership	Patient organisation reps. included into BDSG membership and mandate
Data discoverability	Involve PCOs in **mapping of existing data sources** (launch engagement campaign to populate catalogues)	Engage with PCOs for populating the catalogue once openly published. Added in the BDSG workplan for 2024. Identify from the new catalogue data sources which capture PED, to define opportunities, challenges, and quality considerations (including harmonization and use of CDMs).
	Publish EMA **reflection paper on PED** and organise public consultation	Drafting about to start
Data quality and representativeness	Contribution of PCOs in data **quality framework**	Public consultation on RWD data quality considerations planned for 2024 Annexes to DQF on PED will be explored in the future
	Contribution of PCOs in **qualification of novel methodologies**	Several patient reps. in the workshop on qualification. Report in drafting and to be discussed with PCOs
	A **workshop** on the use of registries in regulatory decision-making	PED to be considered as a key theme of the workshop. Workshop planned in Q1 2024.

EMA-HMA Catalogues of data sources & non-interventional studies

The **EMA-HMA Catalogues of data sources and non-interventional studies** will describe **real-world data sources and studies** through a set of collected **metadata** to help pharmaceutical companies and researchers **identify and use** such data when investigating the use, safety and effectiveness of medicines.

The Catalogues will replace the current ENCePP Resources Database and the EU PAS Register at the beginning of next year, offering an improved, more efficient service for researchers, regulators, and pharmaceutical companies alike.

The catalogues aim to **promote transparency** and **build trust in observational research** and encourage the use of good practices.

- Using the **'FAIR'** (**F**indable, **A**ccessible, **I**nteroperable and **R**eusable) data principles, these catalogues will help users **identify** suitable studies and data sources to address research questions related to the use, safety and effectiveness of medicines
- Through a set of collected **metadata** (data elements characterising both data sources and studies) catalogues will provide helpful information to **facilitate** the conduct and interpretation of studies
- Users will benefit from a **modern technology** with enhanced view, search, export and data submission functionalities.

Launch of publicly available catalogues in early 2024!

BDSG workplan 2023-2025 will include key actions on PED (2/2)

	Key actions	Status / next steps
EU network skills	Dialogue with patients on their training needs	Added in the BDSG workplan for 2024.
	Offer big data training modules to patients	Added in the BDSG workplan for Q1 2024
EU network processes	**Develop a research program on Patient Experience Data (PED)** to support in establishing the value of PED in regulatory assessment and decision-making	- Complete the landscape analysis of ongoing initiatives & disseminating key insights. - Conduct a (literature) review of the use of PED in non-interventional studies to support regulatory decision making – opportunities and challenges. - Collaborate with patients to design a research program aimed at addressing some of the key hurdles in the use of PED in regulatory context.

Stepwise approach to establishing the value of PED

1) Short term

1. Patients' feedback on the proposed plan
2. Identify scientific research topic/question
3. Assess feasibility through EMA's pathways for RWE generation

2) Medium term

1. Landscape analysis of ongoing PED related initiatives & disseminate insights
2. (Literature) review of the use of PED in non-interventional studies to support regulatory decision making
3. Investigate how FAIR and ready to use PED are
   - review & address quality considerations, including data harmonization and use of CDMs
   - leverage new catalogue of data sources

3) Long term

1. Define and establish a set of programs and studies to further support establishing the value of PED in regulatory decision making
2. Explore European support (EC) for a multi-stakeholder approach to ensuring a systematic collection and use of PED over the entire cycle of healthcare decision

Next steps

- Research program (with some proof-of-concept studies)
  - Input received from selected patient organisations' representatives (end of September 2023)
  - Proposal drafted and undergoing review within EMA and then by patients representatives (Q4 2023)
  - Design, contract and launch studies in 2024

HMA/EMA Big Data Stakeholder Forum 2023

Session 3: Patient Experience Data (PED): realising the potential of PED in EU medicine regulation.

Chairs: Julian Isla (Dravet Syndrome European Federation) and Juan Garcia Burgos (EMA)

Reinforcing patient relevance in evidence generation is a key priority in the EMA's Network Strategy and the realisation of a data-driven regulatory network. Despite much progress in the EU in recent years, more efforts are needed to include Patient Experience Data (PED) in development and regulation. This session will discuss the current state of play, challenges and opportunities for enhancing impactful use of PED, and ultimately establish the value of PED in regulatory decision making and beyond.

State of play and ongoing regulatory initiatives Rosa Gonzalez-Quevedo (EMA)	10'
BDSG workplan opportunities to enhance use of PED for regulatory decision-making George Paliouras (NCSR "Demokritos"; DDF)	15'
What is needed by EU regulators Bruno Sepodes (CHMP, INFARMED)	15'
Front row comments with speakers and stakeholders' representatives: • Industry (1) • Patient representatives: Maria Cavaller (EURORDIS/COMP) and Begoña Nafría Escalera, Saint Joan de Deus Children's Hospital and European Young Persons Advisory Group Network • Health care professional: Despoina Makridaki (EAHP) • Academia (1)	30'
Questions and answers	10'

Contact EUBDStakeholderForum@ema.europa.eu to register for the event or follow the broadcast here

Thank you for your attention

Further information

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