3.1 Big Data Steering Group Updates – BDSG workplan on PED

PCWP/HCPWP Joint meeting, 19 July 2023

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State of play

- Although there has been much progress in the EU in recent years, **PED are still not systematically included** in all aspects of medicines development and regulation
- Reinforcing patient relevance in evidence generation is **a key priority** in the EMA's **Network Strategy** and the **Regulatory Science Strategy**
- Patient experience data is also relevant in the context of **the implementation of the new Health Technology Assessment (HTA) regulation**, thus in **value assessments** that inform subsequent decisions by payers
- **Guidance work ongoing at ICH for global harmonisation** of Patient Experience Data (PED)

Continuous dialogue to progress on PED

Workshop (September 2022)

- **Common understanding on PED definition** in the EU, including patient engagement, patient preferences and patient reported outcomes.
- **Current methods for collecting and incorporating patient data** into medicines development and regulatory assessments
- How **direct patient data collection from real-world healthcare** can be leveraged and used

Meeting (January 2023)

- Exchange with patient representatives on the topic of PED and patient-collected data is fundamental, meaning **patients' perspective** should systematically be **included in EMA activities concerning the use of Big Data and RWE** in regulatory decision-making
- Several opportunities to **work together**, as well as considerations for future needs

BDSG workplan on PED

(included in a 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 re. PCOs/HCPs (1/2)

	Key actions	Status / next steps
Governance framework	**Patient/HCP 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/HCPs in **mapping of existing data sources** (launch engagement campaign to populate catalogues)	Engage with PCOs/HCPs 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	Starting
Data quality and representativeness	Contribution of PCOs/HCPs in data **quality framework**	Public consultation on RWD data quality considerations planned for Q3 2023 Annexes to DQF on PED will be explored in the future
	Contribution of PCOs/HCPs in **qualification of novel methodologies**	Several patient reps. in the workshop on qualification. Report in drafting and will be discussed with PCOs/HCPs
	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.

BDSG workplan 2023-2025 will include key actions re. PCOs/HCPs (2/2)

	Key actions	Status / next steps
EU network skills	Survey patients training needs	Dialogue with patients organisations added in the BDSG workplan for 2024.
	Offer big data training modules to patients	Added in BDSG workplan for Q1 2024
EU network processes	**Develop a use case 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. - Set up a proof-of-concept study designed to address some of the identified key limitations and assess impact.

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 (FWC)

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

Timelines

Ongoing
Meeting with patients representatives in July 2023

Q4 2023 – Q2 2024

Start TBC, for 6-12 months

Next steps

- Proof of concept
  - Awaiting input from selected patient organisations' representatives (end of September 2023)
  - Proposal for a PoC project (Q4 2023)
  - Design, contract and launch study in 2024

- Scientific publications
  - Collaborative expert to start the work in October 2023

- Key upcoming BDSG events of interest:
  - AI workshop – 20/21 November 2023
  - Multistakeholder Big data forum - 4 December 2023

Thank you for your attention

Further information

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