EU Big Data Stakeholder Forum 2023

04 December 2022

Session 3: Realising the potential of PED in EU medicine regulation - BDSG workplan opportunities to enhance use of PED for regulatory decision making

Presented by George Paliouras (NCSR "Demokritos"; DDF; BDSG member)

HMA EMA Big Data Task Force vision

*".. delivering the vision of a regulatory system able to **integrate** Big **Data** into its assessment and decision making, we can support the development of **innovated medicines**, deliver **life-saving treatments to patients more quickly** and optimise the **safe and effective use of medicines** through measurement of a products performance on the market."* … December 2019

Why Patient Experience Data?

'Calls from patients to systematically include PED in all aspects of medicines development and regulation and leverage data collection from real-world healthcare'

- Workshop (September 2022) & Meeting with Patients Organisations (Jan 2023)

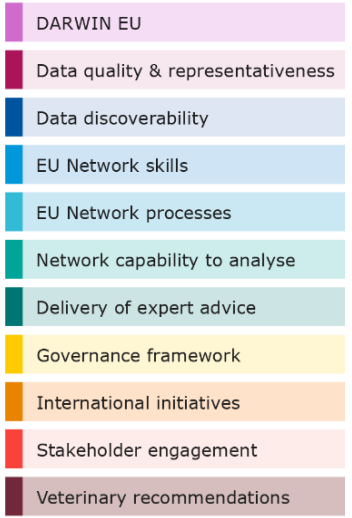
- PED is obviously very important.
- Patients and carers want their data to be used…
- especially when they put time and effort to provide them!

BDSG workplan on Patient Experience Data (PED)

- Understand and increase transparency on the use of PED
  - … support regulatory evaluations and decision making
- Identify challenges and opportunities
  - … enhance optimal and impactful use of PED
- Establish the value of PED
  - … in regulatory decision making and beyond
  - … in collaboration with patients and other stakeholders

How is this to be achieved?

- Implement the BDSG workplan
- Intensify involvement of patients
- Influence strategic plans and European policy
  - EMA Network strategy and Regulatory Science Strategy
  - European Health Data Space and Pharmaceutical Strategy for Europe
- Harmonise PED internationally (collab. with ICH)
- Adopt advanced data analytics



Key challenges and opportunities to realise the potential of PED

- What PED should be collected?
  … and by whom?
- What PED exist?
- How can data be made more available?
  … more accessible?

Findability

- How to assess the quality of PED?
- Which aspects of PED are more important?
- How to improve quality of PED?

Quality

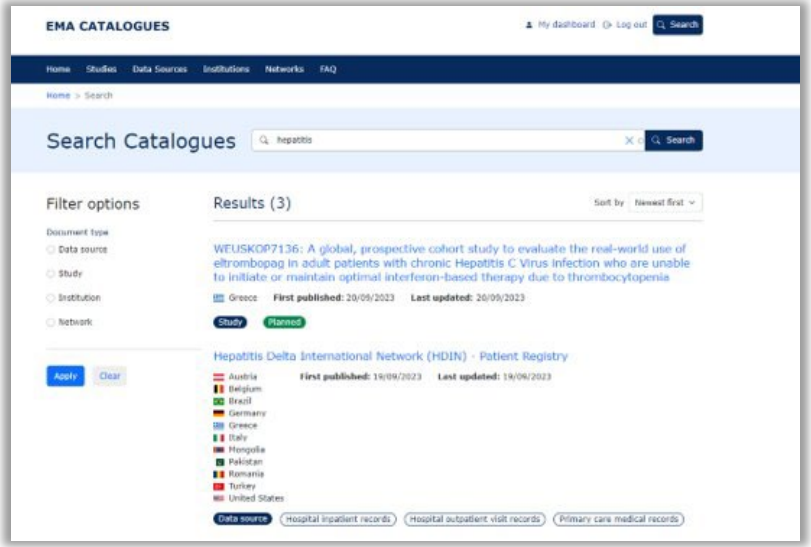
- How valuable are PED?
- How to increase use and value of PED?
  …particularly in regulatory decision-making?

Value

Finding PED: EMA-HMA catalogues

Catalogues of data sources & non-interventional studies
… launching early 2024

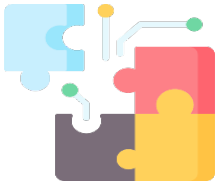
Opportunity to make PED more visible and findable



Finding PED

Make it easier to find data

- Describe real-world data sources and non-interventional studies
- Centralised platform to find and evaluate available data sources
- Allow to understand current landscape of PED



Make it easier to combine data

- Simplify integration and analysis of real-world data.
- Promote transparency and build trust in observational research
- Encourage the use of good practices

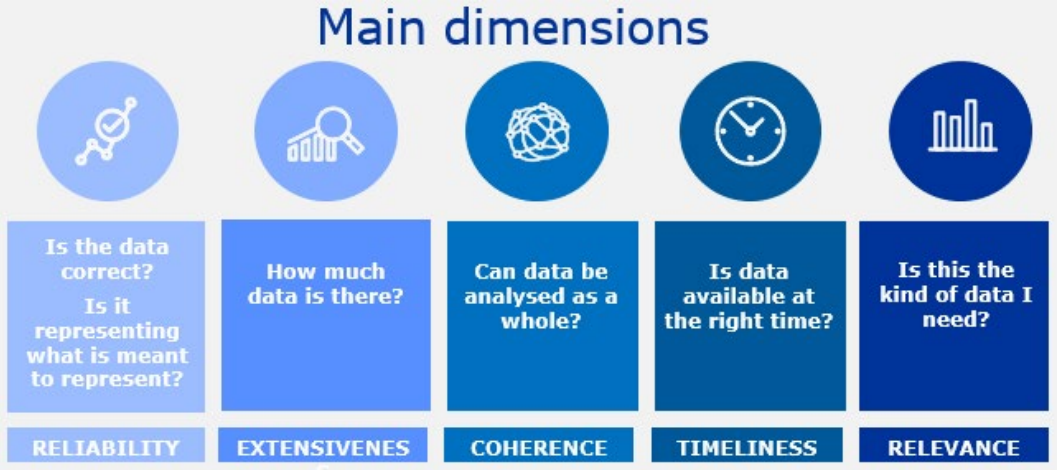
Quality of PED: EU Data Quality Framework

Data Quality Framework for medicines regulation
… first version to appear early 2024
… then to be applied to PED

Opportunity to contribute to foreseen public consultation

Collaboration:

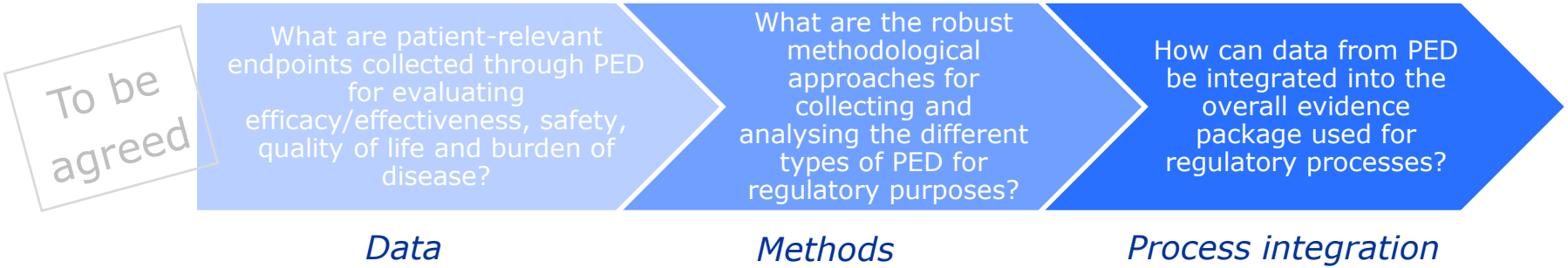
- Big Data Steering Group
- Methodological Woking Party
- TEHDAS Joint Action:
  Towards the European Health Data Space



Establishing the value of PED: research programme

- Establish the value of PED in regulatory assessment and decision-making
- Collaboration with patients on proof-of-concept studies
- Preliminary ideas under discussion:

Duration: 2-3 years

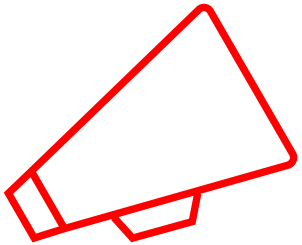


- Involvement of multiple stakeholders

Vision and outlook

- Show the value of PED across the spectrum of regulatory use cases
- Make systematic PED collection easier (infrastructures and automation)
- Make PED readily available through the EU Health data
- High-quality searchable catalogues of PED assist the work of regulators
- Enhance knowledge and experience of EU Network experts in data science to advise companies and assess applications based on PED.
- Guidelines and standards to help PED collection and use
- Continued full compliance with data protection and ethics of data sharing
- Continued engagement and collaboration with patients

How can we contribute?



- Patients and carers in the driving seat for PED
  - Train patients and representatives
  - Organise PED collection
  - Emphasize on quality data
- Leverage HMA-EMA tools
  - Use and contribute to the tools (e.g. catalogues)
  - Assess the tools and provide feedback
- Participate actively in shaping HMA-EMA actions
  - Participate in public consultation of shaping actions
  - Help shaping the use of PED by EMA
  - Propose new actions on the uptake of PED
  - Help communicate the impact of these actions to patients

Thank you!

Further information

See websites for contact details

Heads of Medicines Agencies www.hma.eu
European Medicines Agency www.ema.europa.eu

The European Medicines Agency is an agency of the European Union

More information

Big Data

Clinical Trials and ACT EU

Big Data Highlights

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Clinical Trials Highlights

EMA events