EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Report from the breakout session 1: Fitness of real-world data for regulatory purpose

Learnings Initiative webinar for Optimal Use of Big Data for Regulatory Purpose

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1. What information about real-world data should be included in regulatory applications to support decision-making?

- 1st step: clarify the research question(s) then look at the landscape of available RWD sources (RWDS), not the other way around!
- Feasibility analysis can help identify which RWDS is/are fit for purpose:
  - Wide overview of which RWDS exist, what do they collect, coverage/care settings, FQ of data updates, capture of medicinal products, (…), be aware of limitations (accept that there is no perfect RWD source, pros and cons) → Discussion as early as possible
  - Transparency is key! Importance of publication by data custodians of what data are collected, how and how they are validated → Catalogues of DS (EHDEN, Minerva, Data quality framework projects)
- RWD can provide real representativeness (e.g. patients characteristics) in terms of clinical context of disease (natural history and standards of care)
- Specific time points during development to consider the need for RWD in the frame of the whole data package

2. The CHMP Guideline on registry-based studies recommends to perform a feasibility analysis as an early stage of considering a registry as data source for regulatory purpose. Is such feasibility analysis applicable to other RWD (e.g. electronic health care records)? Which should be additional aspects to be included?

- Broad understanding of RWE
- RWD not limited to registries and EHR
- Digital health technologies
- OTC products
- Patients reported outcomes: need to take into account patients perspective: how is this integrated into the RWDS? Guidance needed
- Informed consent : should be as large as possible to allow for research and data sharing (GDPR) → feasibility analysis

3. Should minimum quality requirements be established in submissions of RWD for regulatory purposes? A distinction can be made between:
- technical data elements
- information needed for regulatory decision-making

- Context specific! Depends what data elements are needed and which impact each have on study analysis/results; quality issues may sometimes be minimised/adjusted for
- Definition of quality: data collection? Mapping? Analysis?
- Importance of validation to increase confidence in data quality:
  - Studies (e.g. linkage cancer diagnosis in primary care with cancer registry)
  - RWDS → data quality management processes of the DS (feasibility analysis)
- Metadata : look at how well these are collected across RWDS

4. Are there any other important aspects of RWD to be addressed in submissions for regulatory purposes?

- Regulators and HTA/Healthcare payers interested in same data but for different purposes
- Take HTA needs into account as much/early as possible during drug development (e.g. comparative data against standards of care)
- Joined scientific advice:
  - Regulators/HTA
  - Regulators across the globe (→ clusters EMA/FDA/HC/MHRA + other regulatory agencies)
- Foster understanding of RWD and its application in regulatory decision making to "harmonise acceptance" → while consistency is there, continuous collaboration needed