Report from the breakout session 3: Process optimisation

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

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At what stage should use of RWD be discussed with regulators, and with whom?

- **Early** discussion and allow for **planning** of pre- and post-licensing RWE generation

- At busines pipeline meetings, pre-submission meetings

- Before and during advice/assessment procedures – with **clarity of proposals** and taking into account **existing guidance** (eg ENCePP methodological guide + checklist)

- **Share learnings**, training, education - work with stakeholders

- Discuss with regulators - via **scientific advice, PRAC (PASS)** – or dedicated other group?

- RWE discussed standalone, or as part of overall SA package – if so, give prominence (to be sufficiently in-depth)

- Aim to **answer a research question** (safety, effectiveness, effectiveness of RMMs)

- Is study **feasible**?

Should there be different discussions for technical and regulatory questions?

- Separation of technical and regulatory questions could be **helpful** if this **streamlines** and free up resources (NB existing triage at pre-submission meeting phase)

- Specialised input required for answers eg regarding statistical methods

- Considering **capacity** for answers and bottlenecks

- While **considering data output uses** also more broadly (linking to HTA/PLEG)

- →Q&As, best practices for regulatory/administrative questions

The CHMP Guideline on registry-based studies recommends early discussions of proposals for use of registries in regulatory submissions. Should such recommendation be applied to other RWD sources? Should differences be made between data sources?

- Use of claims data common in the US vs registries

- No real need to distinguish between data sources

- **Recommendation** from registry-based guideline **applicable** more broadly to RWE

- Early engagement with some detail on proposed methods always beneficial, with more **details** to follow during a procedure (and **pre-specified** in a SAP)

How could other stakeholders than pharmaceutical companies contribute to process optimisation and what could be the vehicles through which such input could be provided?

- Need for **regulatory science research** – need for evidence on processes (and improvements)

- Development of **guidance and Q&As**

- **Multi-stakeholder involvement** and agreement on processes, methods

- Multi decision-maker integration, engagement with HTAs, patients, HCPs

- Plan for AI developments and future use – **research and methods**

- Other less developed **use cases** for RWE use and **methodology**

- **Qualification process** for registries (holders)

What else do you expect from process optimisation, especially in the field of use of RWE?

- **Clarity and guidance** – Q&As, FAQs, best practices – what the **expectations** are

- **Timeliness** for feedback

- Can **simplifications** of existing processes (procedures) be delivered for **quicker, 'less formal', iterative advice,** e.g. via SAWP?

- Clarity how RWE can be reflected in EPARs, in what structure

- **Continued engagement** and follow-up discussion