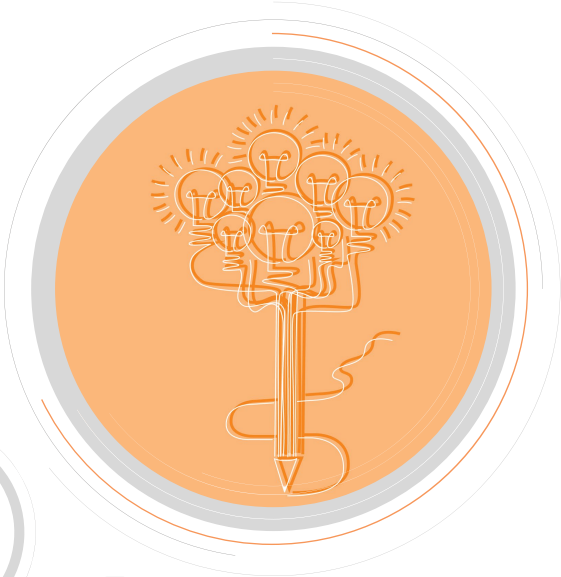
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Optimising evidence incl. RWD for decision making and communication



Feedback from breakout session 3B

EMA's Regulatory Science Strategy to 2025 – Human Stakeholder Workshop

Presented by Sabine Straus, PRAC and Violeta Stoyanova-Beninska, COMP on 19 November 2019

Disclaimer

Comments to the underlying actions represent the views of stakeholders and not the European Medicines Agency.

The fact that these comments from stakeholders are displayed in the presentation does not mean we endorse them or commit to fulfil them in any way.

About this breakout session

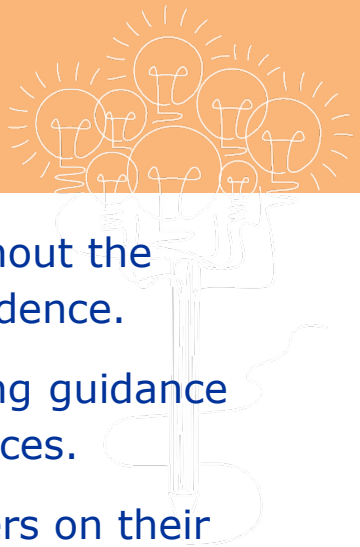
Structured around two themes:

1. Recommendations related to downstream decision making, HTA's preparedness, and collaboration with payers (recommendations 11 (part), 15 and 16)
2. Promote use of high-quality real-world data (RWD) in decision making (recommendation 18)

High-level observations from the discussions:

- Very engaged and open discussion with contributions from all stakeholder groups, enriched by contributions from remote participants
- Complementary views – mutual learning – no show-stoppers – concrete actions

Part 1 "Downstream decision making, HTA's preparedness, and collaboration with payers": **summary of response from the written consultation**



- Collaborate across decision-makers on evidence requirements throughout the medicine's lifecycle, with particular attention to post-authorisation evidence.
- Multi-stakeholder discussion on endpoints and methodologies, including guidance developed by regulators and HTAs, such as capturing patient preferences.
- Facilitate exchange of information between regulators, HTAs and payers on their respective assessments.
- Ensure coordination between the various horizon scanning activities and priority setting, including identification of unmet medical need.
- Permanent working structure and information exchange between EMA and HTA bodies/payers.

Breakout session: High level actions & feedback 1

Shared view that evidence needs should be identified early with involvement of all decision makers (regulators, HTAs, payers):

- Remits are different however the various decisions are based on "universal clinical evidence"
- Strengthen the parallel consultation framework by ensuring sustainability (landscape of decision makers) and enhancing further the involvement of other stakeholders (e.g. HCPs)
- Distilling the learnings from parallel advice into joint guidance from regulators and HTAs
- Collaboration on novel endpoints, across all stakeholder groups (including international)

Foster mutual understanding across decision making: move from sharing information between regulators and HTAs/payers to sharing understanding and knowledge

- Different remits to be respected (benefit/risk is different from affordability)
- Respecting boundaries between advising prospectively and evaluating generated evidence

Breakout session: High level actions & feedback 2

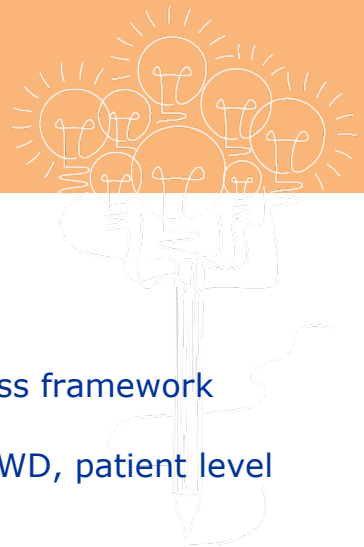
Need for shared perspectives on priorities including unmet medical need, requiring technical engagement platforms for different therapeutic areas

Consideration of developing a joint strategy across regulators and HTAs

First steps

- Call for developers to request development advice early involving several decision makers
- Perform reviews to demonstrate the value of early engagement: 1/ Experience with achieving convergence across decision makers through parallel scientific advice; 2/ Examples of products from scientific advice all the way through decision making
- Fine-tune the Parallel consultation platform based on experience
- Pilot how regulators and HTAs/payers can exchange views on their respective product reviews
- Progress between regulators and HTAs how therapeutic area specific knowledge from parallel advices can be made wider available

Part 2 "Promote use of high-quality real world data (RWD) in decision-making": **summary of response from the written consultation**



Pre-consultation

- Create framework for rapid access to and analysis of RWD throughout lifecycle (*network, data sharing platform + governance and funding*)
- Develop a capacity for Agency to access and analyse large amounts of healthcare data (*computing capacity and skills*)
- Implement a learning regulatory system based on RWD (*processes to feed data into assessment*)

Consultation feedback

- RWD learnings initiative
- Data quality/ representativeness framework
- Build computing capacity for RWD, patient level data, AI: analytics centres
- Regulatory capacity for studies
- Skills: training curriculum and strategy
- International collaboration and stakeholder dialogue
- Governance – clarity on data protection

Breakout session: High level actions & feedback 1

General alignment with written consultation response. Explicit support for:

1. Data quality framework
2. RWD learnings initiative
3. Building regulatory capacity and skills
4. International alignment and input across stakeholder groups
5. Work to clarify (and advise on) data protection
6. A multi-stakeholder effort to access and analyse existing RWD (incl. EHRs, claims, registry)

Remain focussed on benefits for patients and embed patient and HCP consultation in system design and data sharing approaches

Identify the use-cases for RWD i.e. use-cases that clinical trials cannot fully address (note compelling business case for using RWD for product safety)

Breakout session: High level actions & feedback 2

Map existing data sources, methods and initiatives to avoid duplication of effort

Existing disease registries offer an important RWD resource. Aim to optimise data collection and use

Data capture should be simplified (to not interfere with doctor patient relationship)

First steps

- List use cases not fully addressed by clinical trials
- Deliver sustainable access to existing RWD
- Deliver training on RWD and establish regulatory science centres
- Create public inventory of existing data sources (including quality + representativeness metrics)
- Establish a RWD framework: principles for use, standards, acceptance
- Initiate a patient-led RWD pilot using a rare disease

Any questions?

Further information

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