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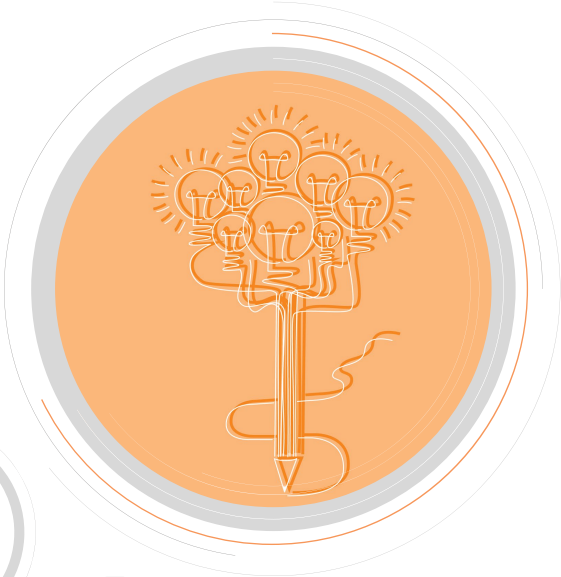


Reinforcing patient relevance in evidence generation

Feedback from breakout session 5C

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

Presented by Bruno Sepodes, CHMP and Koenraad Norga, PDCO 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.

Main and new insights

- **Objective** – to be able to generate patient (experience) data which satisfies the different stakeholder needs (including regulators, patients, HTAs, payers, etc)
- Comprehensive EU conceptualisation to cover:
  - What is patient (experience) data - beyond PROs (e.g. patient preferences, Observer-reported outcomes (ObsRO))
  - How to state its relevance and the need to incorporate it systematically into drug development and,
  - how to do that in practice - guidance
- Several underlying actions requiring collaboration with all stakeholders (including patients & patients' organisations)

Main and new insights

- Patient data to be properly reflected, discussed and analysed in the EPAR, and whenever relevant, incorporated into the SmPC.
- What is acceptable data form a regulatory point of view
- Quality of data should be guaranteed – including critical assessment of quality of data
- Need to involve academics in developing methodology
- For patients' organisations to actively -and independently- participate in the process, there is a need for them to obtain public support (funding)

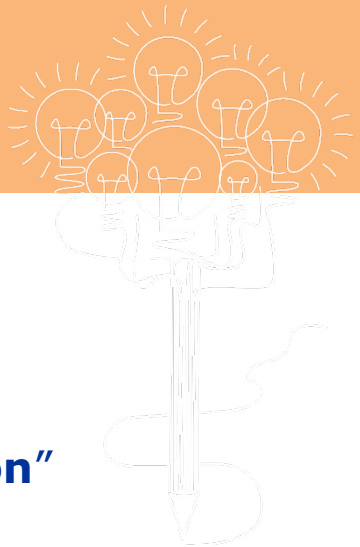
Main and new insights

- Patients to be involved systematically – guidance on the need to involve/consult patients (rationale use of resources)
- Facilitate validation of PROs in early development – involve patients for that
- Need for ‘general guidance’ on the one hand vs ‘specific considerations’ to generate patient (experience) evidence in the different therapeutic areas
- Pay attention to both frequent and rare diseases
- Need to address specific patient needs (paediatrics, geriatrics, special populations, etc.)
- Always incorporate the principles of **independence** and **transparency** into the process

Main and new insights

- Foster global alignment on the scientific methodology to gather patient contribution to drug development

Proposed expansion of core recommendation



From

"Reinforce patient relevance in evidence generation"

To

"Ensuring the patient voice is systematically incorporated throughout drug development, associated evidence generation and decision-making"

Any questions?

Further information

RegulatoryScience2025@ema.europa.eu

Temporary visiting address Spark building • Orlyplein 24 • 1043 DP Amsterdam • The Netherlands
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Send us a question via www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

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