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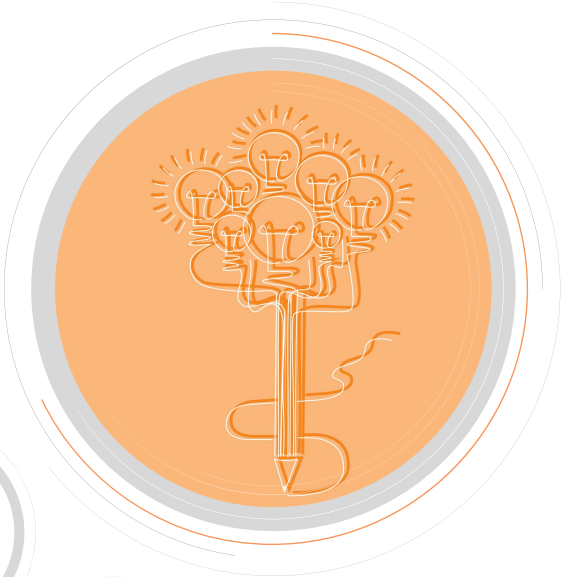


Reinforce patient relevance in evidence generation

Underlying actions

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

Chaired by Bruno Sepodes, CHMP, Koenraad Norga, PDCO on 19 November 2019
Presented by Juan Garcia Burgos, Head of Public Engagement Department and Ralf Herold, Oncology, Haematology and Diagnostics, EMA

Disclaimer

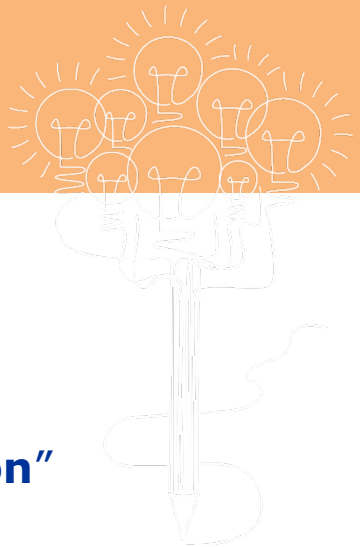
Comments to the underlying actions represent the views of stakeholders and not the European Medicines Agency.

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.

Reinforce patient relevance in evidence generation

- Coordinate Agency’s approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs
- While validating PROs, address patients’ needs and leverage patients’ expertise
- Co-develop with EUnetHTA a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment

Proposed expansion of core recommendation



From

"Reinforce patient relevance in evidence generation"

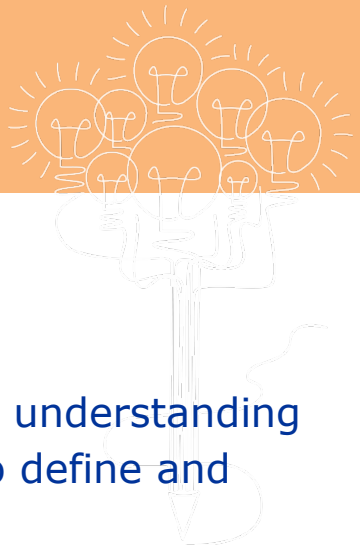
To

"Ensuring the patient voice is systematically incorporated throughout drug development & associated evidence generation"

Overview on underlying actions

1. Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation.
2. Coordinate Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis.
3. While validating PROs, address patients' needs and leverage patients' expertise.
4. Enhance patient involvement in EMA scientific committees.
5. Co-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment.
6. Expand the B/R assessment by incorporating patient preferences.
7. Drive global alignment on the scientific methodology to gather patient contribution to drug development.

Suggestions for participants



- **Purpose of session**: To have a multilateral, rich dialogue; to deepen understanding of ideas and comments received; to inform EMA's subsequent steps to define and prioritise steps and actions

Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation

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- EMA should be encouraged to take a more vigorous approach to the whole issue of patient-focused drug development. Patient perception of value should rightfully be reflected in the SmPC.
- Seek agreement on how and where to include patient experience/preference data in regulatory submissions and labelling - support multi-stakeholder agreement on a framework for evaluation of patient preference data.
- Embedding patient priorities into clinical trials design, via clear EMA guidance co-developed with patients, is needed to ensure that meaningful data is generated for regulatory assessment.

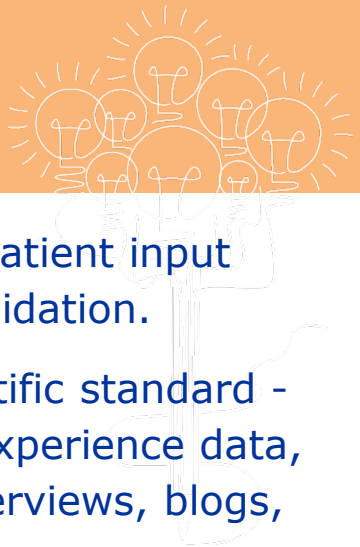
Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation

- Data from treatment optimisation studies, registries, observational clinical trials and electronic health records of patients should be interlinked and embedded into decision making process.

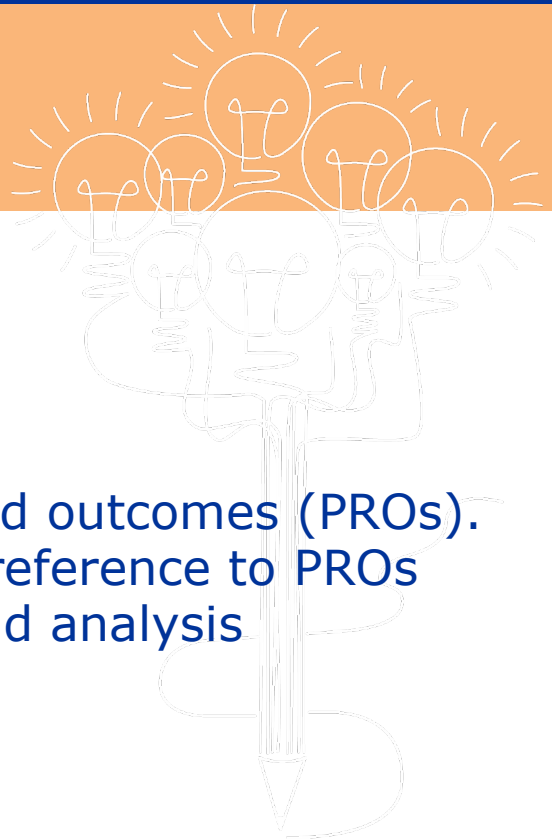
Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation

- EMA should strongly encourage and set clear expectations towards applicants by insisting to involve patients in the studies that are being submitted; E.g.
  - Update guidelines to require patient systematic involvement
  - That every study evaluates the need for PROs that are being tested for relevance by patients and ease of use
  - A justification that every outcome studied is relevant to patients
  - Expect documentation of the fact that patients have been involved i design, implementation and interpretation of pivotal studies

Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation



- Develop with EMA relevant patient-centred QoL measurement tools; Patient input into long term side effect and QoL. Involvement of patients in PRO validation.
- Define expectations for scientific rigour i.e. what constitutes the scientific standard - drive understanding across stakeholders of what constitutes patient experience data, where the data can take many forms: feedback from focus group, interviews, blogs, etc.



Coordinate Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis

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- Best practice guides on high-quality PRO trial tools would help to ensure that patients' voices are central to informing shared decision-making, labelling claims, clinical guidelines, and health policy and making patient-centred care a reality.
- A coordinated approach to PROs across therapeutic areas and a proactive update by the EMA of specific clinical guidelines on these.

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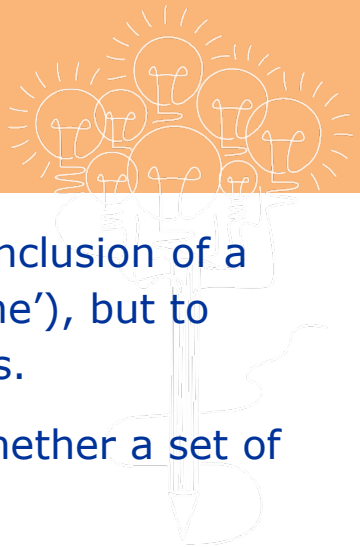

- Clinical guidelines should be updated to include not just reference to inclusion of a PRO (where PRO would be better phrased as 'Patient Relevant Outcome'), but to include reference to the inclusion of a COS when a relevant COS exists.
- Welcomed a core health-related quality-of-life PRO, but questioned whether a set of PROs would be more appropriate across disease areas.

Coordinate Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis

- Fostering effective use of PROs in decision making:
  - Address the lack of standardization and the perceived lack of rigor associated with "subjective data";
  - Provide transparency on how related data is assessed and rated;
  - Consider aspects linked to digital health (tools, endpoints etc.);
  - Consider early the perspectives of HTA.

Coordinate Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis

- Enhance international collaboration with regulators in ongoing initiatives, notably with regulators that are pioneering several initiatives on patient-focused drug development such as the US FDA.

While validating PROs, address patients' needs and leverage patients' expertise

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- Leverage patient-reported outcomes in diseases where the disease itself may impair the ability of the patient to self-report, such as neurodegenerative diseases or schizophrenia
- Paediatric outcomes reported by their caregivers and a focus on special patient populations are also essential to advance development
- A well-developed core outcome set will have included all relevant stakeholders, including patients or their representatives, in the determination of the most important outcomes to be measured.
- Patient perception of value to be reflected in SmPC.

Enhance patient involvement in EMA scientific committees

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- We recommend introducing the concept "co-creation" when patients are involved in the decision-making process as they become transformative agents of the process.
- It is important to ensure contribution from smaller patient organisations and allow them to contribute independently of umbrella organisations.

Co-develop with EUnetHTA a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment

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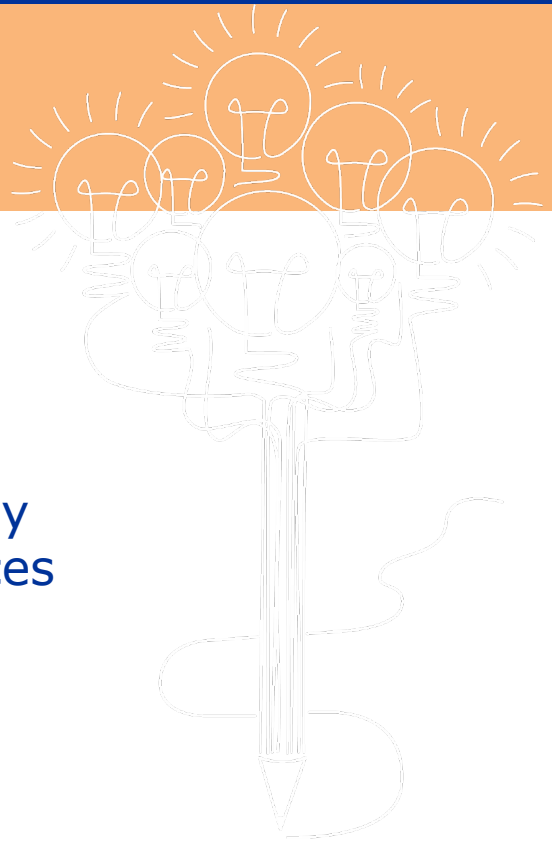
- It isn't clear what the need for the development of a new health related quality of life tool is. It is good that the proposal is to co-develop this with HTA agencies but any such work should start with a review of the need.
- It is not clear if the proposal is ONE core PRO along with HTAs, or core SET of measures. We suggest considering how robust/meaningful the comparison is e.g. across different diseases.
- Real challenge is the sensitivity of the measure. Before developing a new tool, there should be efforts towards consensus building on the appropriate tools across all stakeholders.
- Discrete Choice Experiments can be used on top of PROs.



Expand the B/R assessment by incorporating patient preferences

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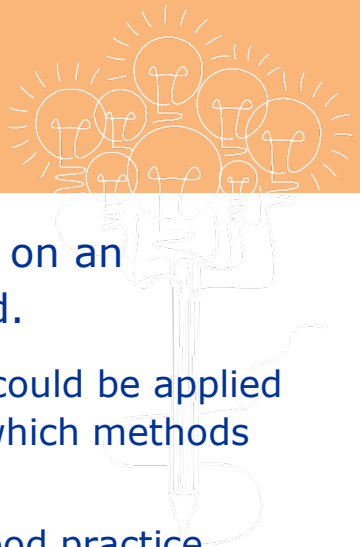
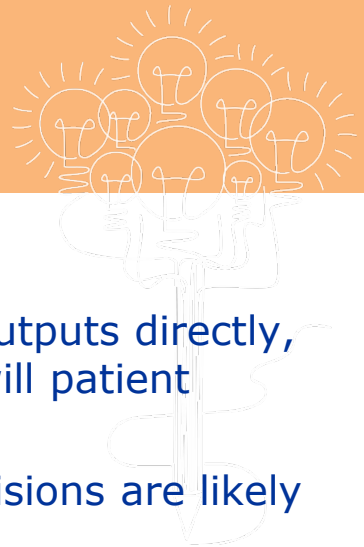
- When patient preferences are increasingly incorporated it has to be ensured that this is done in a transparent and impartial way with clear rules for conflict of interest.

Expand the B/R assessment by incorporating patient preferences



- It will be important for the EMA to provide guidance on how to deliver on an expanded BRA, and to consider how the quality of this work is assured.
  - There are many preference and structured decision-making methods that could be applied to support an expanded BRA. It will be important to provide guidance on which methods are considered appropriate.
  - In this endeavour it is important the EMA consider and build on existing good practice guidance (such as that issued by ISPOR), guidance provided by the FDA, and the results of IMI PREFER.

Expand the B/R assessment by incorporating patient preferences



- How will patient preferences be used in regulatory decisions making?
  - Are preferences intended to help regulators interpret clinical trial outputs directly, or provide a broader patient-centred benefit risk assessment? Or will patient preferences inform risk management strategies?
  - For which decisions are patient preference data helpful? Which decisions are likely to be preference-sensitive?

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

- EMA works closely with the FDA on this initiative to ensure a global approach. The output from the existing cluster group should be more transparent to industry.
- Support key EU initiatives like IMI PREFER, IMI PARADIGM & EUPATI whilst at minimum keeping pace with initiatives in other regions

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Any questions?

Further information

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