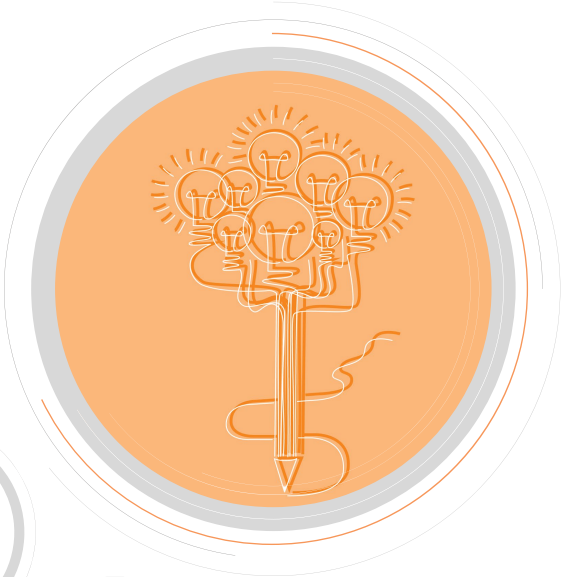
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Recommendations related to downstream decision making, HTA's preparedness, and collaboration with payers



Underlying actions

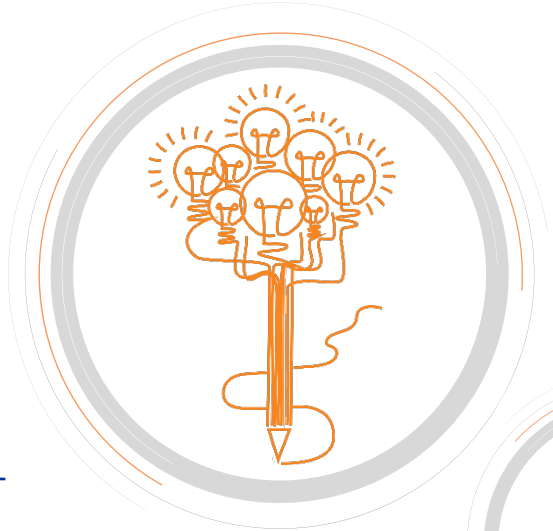
EMA's Regulatory Science Strategy to 2025 – Human Stakeholder Workshop

Chaired by Violeta Stoyanova-Beninska, COMP and Sabine Straus, PRAC on 18 November 2019
Presented by Michael Berntgen, Head of Product Development Scientific Support, 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.



Contribute to HTA's preparedness and downstream decision making for innovative medicines

Underlying actions

Contribute to HTAs' preparedness and downstream decision-making for innovative medicines

- Ensure the evidence needed by HTAs and payers are incorporated early in drug development plans
- Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment
- Discuss with HTAs guidance and methodologies for evidence generation and review
- Contribute to the identification of priorities for HTA
- Monitor the impact of decision-maker engagement through reviews of product-specific experience

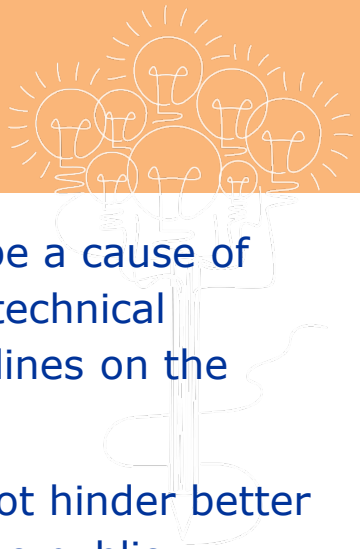
Ensure the evidence needed by HTAs and payers are incorporated early in drug development plans

- Strengthen parallel EMA/HTA scientific advice to reduce risk of inadequate information provided to EMA/HTA at time of evaluation; EUnetHTA can be used as a platform to exchange information between CHMP and HTA; allow HTA assessors to have this information in parallel to CHMP evaluation.
- Collaborate with HTA bodies on post-authorisation evidence requirements and introduce EU clinical registries post-authorisation in addition to existing managed entry agreements.
- Clinical registries would provide highly structured clinical data to healthcare professionals on safety and effectiveness, and can be used to compare the effectiveness of different treatments for the same disease or condition.

Ensure the evidence needed by HTAs and payers are incorporated early in drug development plans

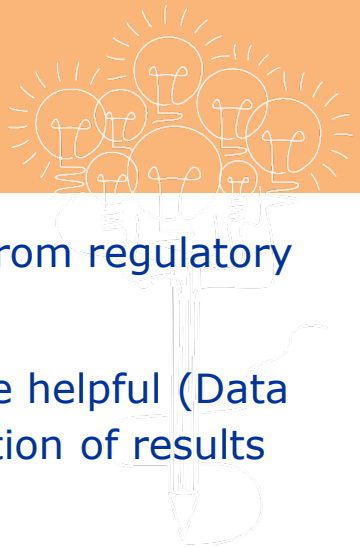
- HTA requirements include long-term efficacy, quality of life (QoL), activities of daily living (ADL), data in specific age groups, subgroups and biomarkers. Data quality e.g. if gathered using wearables is an additional important consideration.
- This would be particularly useful where evolving knowledge during development suggests a different endpoint or way of monitoring would be more appropriate in the post-marketing setting than utilised in clinical trials.

Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment



- Invite HTA experts to CHMP discussions for issues that are known to be a cause of difficulties for the downstream decision-making. The same applies to technical guidelines, where EMA and HTA bodies develop different sets of guidelines on the same topics, which can result in counter-productive divergences.
- Differences between HTA and EMA assessments are justified and do not hinder better cooperation; however, the differences should be better explained in the public domain.

Discuss with HTAs guidance and methodologies for evidence generation and review



- The inclusion of core outcome sets (COS) throughout the ecosystem from regulatory to HTA assessments.
- Guidelines on how to involve the patient in the process again would be helpful (Data collection, defining the research question, value to patient, dissemination of results etc.)

Discuss with HTAs guidance and methodologies for evidence generation and review

- The importance of discussing with HTA bodies, guidance and methodologies for evidence generation and review.
- Specific programs for HTA assessment in the field of ATMPs should be developed and implemented. Impact assessment should also be developed in routine evaluations of benefit-risk.
- It is critical that a framework for evaluating long term value, specifically in CNS medicines, is developed and endorsed across stakeholders.

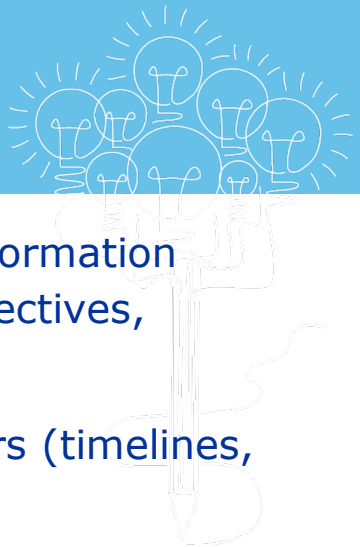
Contribute to the identification of priorities for HTA

- Ensure coordination between the various horizon scanning activities such as ICMRA strategic initiative on innovation and IHSI initiated by Beneluxa to identify what and when disruptive technologies could be made available.
- Ensure HTA involvement for PRIME designation to including a cross check for the unmet medical need.

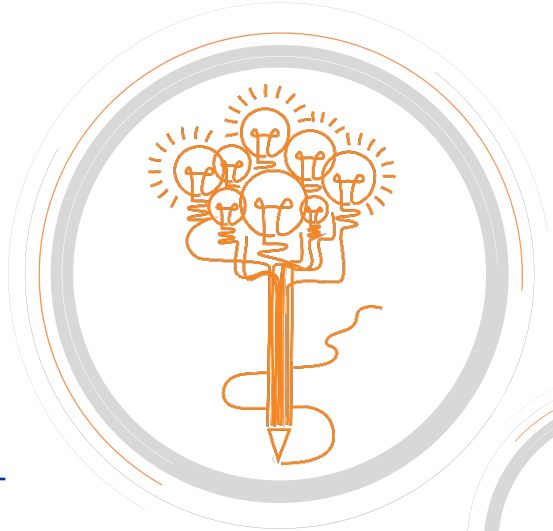
Monitor the impact of decision-maker engagement through reviews of product-specific experience

- Target parameters should be defined. While discussion often focusses on access alone, in reality, the triangle of access, affordability and added benefit is relevant.

Further develop the structured interaction between EMA and HTA bodies, respecting the respective remits



- Proposal to reflect on establish a permanent working structure and information exchange process with EMA and HTA bodies/payers, with relevant objectives, planning and responsibilities.
- Describe more clearly the proposed involvement plan with stakeholders (timelines, operational approach).



Expand benefit-risk assessment and communication

Underlying actions

Expand benefit-risk (B/R) assessment and communication

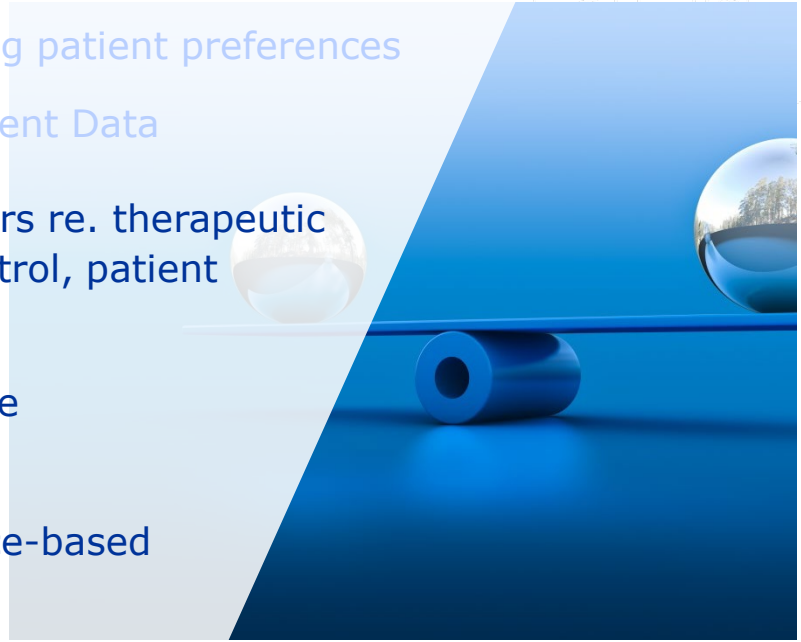
Expand the B/R assessment by incorporating patient preferences

Develop the capability to use Individual Patient Data

Improve communication with HTA and payers re. therapeutic context, comparison vs. placebo/active-control, patient perspective

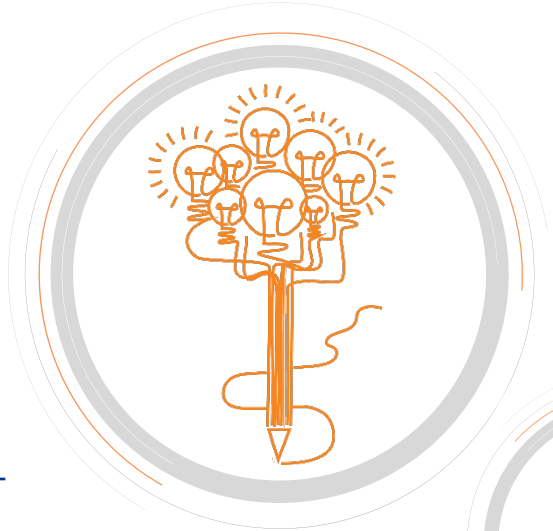
Apply structured B/R assessment to improve communication to the public

Incorporate academic research into evidence-based benefit-risk communication



Promote systematic application of structured benefit-risk methodology and quality assurance systems across the network

- Effects tables are often insufficient to render a B-R decision. A structured approach for the assessment, (not tabulation of key B-R data), is needed. This should be suitable for sponsor use and not be a regulators' communication tool, as currently.
- A deepened discussion about unmet medical need, severity of disease, existing treatment options and the size/amplitude in effectiveness in absolute terms would be very positive.
- How to ensure consistency; Importance of favourable and unfavourable effects… However, we also realize that this section is not always formulated in the same way.



Bridge from evaluation to access through collaboration with payers

Underlying actions

Bridge from evaluation to access through collaboration with Payers

- Contribute to the preparedness of healthcare systems by creating opportunities for collaboration on horizon scanning
- Enable involvement of payers' requirements in the prospective discussion of evidence generation plans
- Clarify the treatment-eligible patient population included in the labelling, and its scientific rationale
- Participate in discussions clarifying the concept of unmet medical need

Enable involvement of payers' requirements in the prospective discussion of evidence generation plans including post-licensing evidence generation

- Create a mechanism for early and frequent stakeholder involvement—between regulators, payers, and the manufacturer—in a safe harbour environment to determine unmet medical need and the information needed in a clinical trial and/or RWE study.
- Industry stakeholders would likely have important insights on the challenges of the current processes, and could assist the Agency by providing input or advise on potential strategies to address or mitigate them.
- The current FDA initiative to establish core, co-created sets of clinical outcome assessment and related end points is a good example of helping to define a common ground that reflects the patient perspective and which informs the whole lifecycle of medicine.

Contribute to the preparedness of healthcare systems by creating opportunities for collaboration on horizon scanning

- A robust horizon scanning system at national (and European) level could help decision-makers to plan and prepare for innovation. Cooperation and exchange of information between EMA and HTA/payers in the field of horizon scanning, including timely sharing of information regarding upcoming regulatory submissions should be envisaged, in order to impact on Health Care Systems' preparedness.

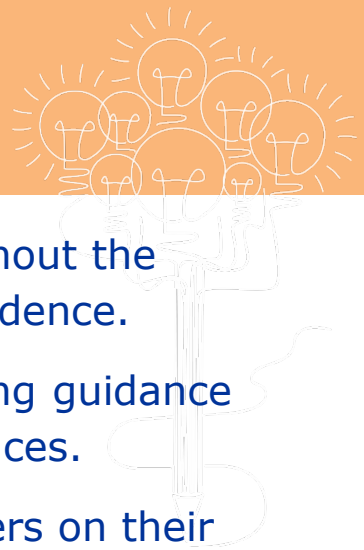
Clarify the treatment-eligible patient population included in the labelling, and its scientific rationale

- Rename underlying action as “Consider more structured interaction between EMA and payers, respecting the respective remits”.
- Establish a permanent working structure between EMA and payers with relevant objectives, planning and responsibilities.
- Identify opportunities to avoid duplicative efforts between EMA and its HTA/payer partners.

Participate in discussions clarifying the concept of unmet medical need

- There are benefits to engage with payers earlier to gain insight into their perspectives on unmet needs and priorities. Early engagement also helps to prepare payers for potential major impacts from breakthrough innovation.

Summary observations and recurrent themes



- 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.

Any questions?

Further information

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