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Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* Name

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Introduction

The purpose of this public consultation is to seek views from EMA's stakeholders, partners and the general public on EMA's proposed strategy on Regulatory Science to 2025 and whether it meets stakeholders' needs. By highlighting where stakeholders see the need as greatest, you have the opportunity to jointly shape a vision for regulatory science that will in turn feed into the wider EU network strategy in the period 2020-25.

The views being sought on the proposed strategy refer both to the extent and nature of the broader strategic goals and core recommendations. We also seek your views on whether the specific underlying actions proposed are the most appropriate to achieve these goals.

The questionnaire will remain open until June 30, 2019. In case of any queries, please contact: RegulatoryScience2025@ema.europa.eu.

Completing the questionnaire

This questionnaire should be completed once you have read the draft strategy document. The survey is divided into two areas: proposals for human regulatory science and proposals for veterinary regulatory science. You are invited to complete the section which is most relevant to your area of interest or both areas as you prefer.

We thank you for taking the time to provide your input; your responses will help to shape and prioritise our future actions in the field of regulatory science.

Data Protection

By participating in this survey, your submission will be assessed by EMA. EMA collects and stores your personal data for the purpose of this survey and, in the interest of transparency, your submission will be made publicly available.
For more information about the processing of personal data by EMA, please read the privacy statement.

Questionnaire

Question 1: What stakeholder, partner or group do you represent:

- ○ Individual member of the public
- ○ Patient or Consumer Organisation
- ○ Healthcare professional organisation
- ○ Learned society
- ○ Farming and animal owner organisation
- ○ Academic researcher
- ○ Healthcare professional
- ○ Veterinarian
- ○ European research infrastructure
- ○ Research funder
- ◉ Other scientific organisation
- ○ EU Regulatory partner / EU Institution
- ○ Health technology assessment body
- ○ Payer
- ○ Pharmaceutical industry
- ○ Non-EU regulator / Non-EU regulatory body
- ○ Other

Name of organisation (if applicable):

PPD Evidera

Question 2: Which part of the proposed strategy document are you commenting upon:

- (•) Human
- ( ) Veterinary
- ( ) Both

Question 3 (human): What are your overall views about the strategy proposed in EMA's Regulatory Science to 2025?

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

As the healthcare landscape continues to evolve, patients, caregivers, and patient advocacy groups are requesting larger and more substantive roles in impacting the drug/medical device development process. Ensuring these stakeholders are considered at every step in the continuum will empower them to OWN their own health care experience. In addition to enhancing patient involvement in regulatory process, collaboration with payers, early dialogues to align on requirements are critical steps for improving access of new and innovative medicines to patients.

The EMA has taken a noteworthy step by allowing all stakeholders to comment on its 2025 Strategy. Overall the EMA 2025 Strategy is a comprehensive plan outlining the need and areas of scientific advancement, from a regulatory perspective. However, the goals and sub goals should be considered differently to allow for adequate resource allocation and to actually drive the needle in a few key areas of need.

Question 4 (human): Do you consider the strategic goals appropriate?

Strategic goal 1: Catalysing the integration of science and technology in medicines development (h)

- (•) Yes
- ( ) No

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations (h)

- (•) Yes
- ( ) No

Strategic goal 3: Advancing patient-centred access to medicines in partnership with healthcare systems (h)

- (•) Yes
- ( ) No

Strategic goal 4: Addressing emerging health threats and availability/therapeutic challenges (h)

- (•) Yes
- ( ) No

Strategic goal 5: Enabling and leveraging research and innovation in regulatory science (h)

- (●) Yes
- ( ) No

Question 5 (human): Please identify the top three core recommendations (in order of importance) that you believe will deliver the most significant change in the regulatory system over the next five years and why.

First choice(h)

17. Reinforce patient relevance in evidence generation

1st choice (h): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

Added to feedback on this goal below

Second choice (h)

11. Expand benefit-risk assessment and communication

2nd choice (h): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

Added to feedback on this goal below

Third choice (h)

31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders

3rd choice (h): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

Added to feedback on this goal below

Question 6 (human): Are there any significant elements missing in this strategy. Please elaborate which ones (h)

Timelines and operational approach to achieve the goals
How will the different stakeholders including patients and PAGs be involved and consulted to achieve these goals

Question 7 (human): The following is to allow more detailed feedback on prioritisation, which will also help shape the future application of resources. Your further input is therefore highly appreciated. Please choose for each row the option which most closely reflects your opinion. For areas outside your interest or experience, please leave blank.

Should you wish to comment on any of the core recommendations (and their underlying actions) there is an option to do so.

Strategic goal 1: Catalysing the integration of science and technology in medicines development (h)

	Very important	Important	Moderately important	Less important	Not important
1. Support developments in precision medicine, biomarkers and 'omics'	○	○	○	○	○
2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments	○	○	○	○	○
3. Promote and invest in the Priority Medicines scheme (PRIME)	○	○	○	○	○
4. Facilitate the implementation of novel manufacturing technologies	○	○	○	○	○

5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products					
6. Develop understanding of and regulatory response to nanotechnology and new materials' utilisation in pharmaceuticals					
7. Diversify and integrate the provision of regulatory advice along the development continuum					

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation** you are commenting on:

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations (h)

	Very important	Important	Moderately important	Less important	Not important
8. Leverage novel non-clinical models and 3Rs	○	○	◉	○	○
9. Foster innovation in clinical trials	◉	○	○	○	○
10. Develop the regulatory framework for emerging digital clinical data generation	○	◉	○	○	○
11. Expand benefit-risk assessment and communication	◉	○	○	○	○
12. Invest in special populations initiatives	○	◉	○	○	○
13. Optimise capabilities in modelling and simulation and extrapolation	○	○	◉	○	○
14. Exploit digital technology and artificial intelligence in decision-making	○	○	○	○	○

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

Recommendation 3.2.4: Expand benefit-risk assessment and communication
We are delighted to see that EMA is continuing their interest in structured benefit-risk assessment (BRA) and patient preferences. These approaches hold the potential to improve the quality, consistency and transparency of regulatory decisions. This agenda will also mirror developments at the FDA and provides the possibility to clarify where the EMA and FDA are aligned in this area.
We would encourage the EMA to be ambitious and lead the regulatory sphere in implementation of such methods. This is an opportunity for the EMA to continue to build on recent good work and be viewed as leading the discussion of structured benefit-risk assessment in the regulatory domain.
As this recommendation is taken forward, it is important that the EMA address a number of important questions
1. How will patient preferences be used in regulatory decisions making? For instance:
a. Are preferences intended to help regulators interpret clinical trial outputs directly, or provide a broader patient-centered benefit risk assessment? Or will patient preferences inform risk management strategies?

b. How will preferences influence decisions? For instance, if patients are willing to tolerate treatment risks for its benefits, is that sufficient for product approval?
c. Given the answer to these questions, for which decisions are patient preference data helpful? Which decisions are likely to be preference-sensitive?
2. How would patient preferences interact with structured decision making? This partly depends on what is meant by structured decision making, and it will be important to be clear about this. Assuming this means structuring committee discussions and decisions, and perhaps even performing a quantitative benefit-risk assessment with committee preference data, how would committee and patient preferences both be incorporated into the benefit risk assessment? If they conflict, how should this be resolved?
3. How can quality be assured? 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.
a. 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 are considered appropriate. This should consider the particular use to which the EMA intends to put such methods (see above). It will also be important to provide guidance on how these methods should be implemented.
b. In this endeavor 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.
Given the subjective nature of preferences, and the potential biases that need to be considered when conducting preference research, there may some skepticism about the rigor of the preference data used in the expanded BRA. It will be important for the EMA to consider how best to assure quality. For instance: sponsors could be asked to publish protocols, a process could be established to provide scientific

Strategic goal 3: Advancing patient-centred access to medicines in partnership with healthcare systems (h)

	Very important	Important	Moderately important	Less important	Not important
15. Contribute to HTAs' preparedness and downstream decision-making for innovative medicines	◉	○	○	○	○
16. Bridge from evaluation to access through collaboration with Payers	○	◉	○	○	○
17. Reinforce patient relevance in evidence generation	◉	○	○	○	○
18. Promote use of high-quality real world data (RWD) in decision-making	○	◉	○	○	○
19. Develop network competence and specialist collaborations to engage with big data	○	◉	○	○	○

20. Deliver real-time electronic Product Information (ePI)	○	○	◉	○	○
21. Promote the availability and uptake of biosimilars in healthcare systems	○	○	◉	○	○
22. Further develop external communications to promote trust and confidence in the EU regulatory system	○	◉	○	○	○

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

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Strategic goal 4: Addressing emerging health threats and availability/therapeutic challenges (h)

	Very important	Important	Moderately important	Less important	Not important
23. Implement EMA's health threats plan, ring-fence resources and refine preparedness approaches	○	○	○	○	○
24. Continue to support development of new antimicrobials and their alternatives	○	○	○	○	○
25. Promote global cooperation to anticipate and address supply challenges	○	○	○	○	○
26. Support innovative approaches to the development and post-authorisation monitoring of vaccines	○	○	○	○	○
27. Support the development and implementation of a repurposing framework	○	○	○	○	○

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

The following represent integral aspects needed for achieving any of the other broader 5 goals. Rec: 3.3.3 Reinforce patient relevance in evidence generation and Rec: 3.3.4 Promote use of high-quality RWD in decision-making; Rec: 3.3.2 Bridge from evaluation to access through collaboration with payers. (Rec: 3.3.3) Reinforce patient relevance in evidence generation and (Rec: 3.3.4) Promote use of high-quality RWD in decision-making; Ensuring the patient is at the center of evidence generation, providing unique insights, information, and experiences that can impact actual treatment pathways as well as future research. The following suggestions are offered to enhance this.:•Patients/advocacy groups should be empowered to better understand the decision-making process and materials to be reviewed for decision making to contribute more actively in decision making. This could involve training and educating patients about regulatory process for meaningful involvement. Patient empowerment involves more that the provision of knowledge about materials to be reviewed and processes. It requires coaching/mentoring for proactive participation in decision making. The model should also include sustained monitoring and support to enhance and retain interest from patient, provider and decision makers: o This could also be linked to the Rec: 3.5.1. Since patient engagement is an evolving area, involvement of social scientists (to understand the trends and patient needs to inform the development of training and support would be essential

•Reviewers and decision makers must be able to understand and interpret outcomes of evaluations that are relevant to patients such as patient reported outcomes and qualitative research that illustrates patients

experience of the condition and treatment. This may require, resources or expertise to evaluate, interpret, weight and value patient data to inform decision making. (This suggestion is also linked to the Rec: 3.3.6 Deliver improved ePI. •EMA staff who engage with patients should have soft-skills to facilitate patient engagement beyond token representation. Formalized training for all stakeholders to ensure that patient research partners are “conversant in and familiar with the language and process of research” will allow meaningful dialogues amongst all team members.(This could also be linked to the Rec: 3.5.1, since the active involvement of patients in drug development and decision making is an evolving trend. If done without sensitivity it could cause more harm than good)•EMA evaluations of new products and information on the summary of product characteristics should include a clear discussion of outcomes- risks and benefits- that are relevant to patients for clinical decision making; i.e. to inform both patients, formal and informal providers of care, adequate information about the risks and benefits of medicines from a patient’s perspective must be considered to help them make informed choices at an individual level. The intention of to-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment may be challenging. It is also important to enhance: The development of tailored and meaningful PROs that measure concepts of importance and relevance to target population; Encouraging and facilitating consortia approaches to enable the use of similar metrics across treatments for the same condition;•The conduct of high-quality studies and interpretation of change score that are meaningful to patients and can be converted to outcomes that can be interpreted by HTA agencies and positioned to enable health care decision makers about the value of the new technology. The collection of patient experience data within clinical trials (exit / embedded clinical trial interviews/ surveys) to understand patient experience with the investigational medicine. These can also help illustrate the meaningfulness of change score on PRO instruments with illustrative examples. See examples in : Willgoss, T., et al 2017, October. Qualitative exit interviews . In Quality of Life Research (Vol. 26, No. 1, pp. 4-4). (Rec 3.3.2) Bridge from evaluation to access through collaboration with payers: There is a need to improve collaboration between payers and regulators – increase EMA/Eunehta parallel consultations to align on requirements/ expectations. There is also an opportunity to enhance patient involvement in those consultations. For example, Involvement of patients in early scientific advice is essential to develop more tailored treatments, improve the design of clinical trial, enhance retention in clinical trials and quality of the data etc. There is a need for more productive and collaborative relationships between HTA and regulators. suggestions related to (Rec 3.3.3) re: development of a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment

Strategic goal 5: Enabling and leveraging research and innovation in regulatory science (h)

	Very important	Important	Moderately important	Less important	Not important
28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science	○	○	○	○	○
29. Leverage collaborations between academia and network scientists to address	○	○	○	○	○

rapidly emerging regulatory science research questions					
30. Identify and enable access to the best expertise across Europe and internationally	◎	◎	◎	◎	◎
31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders	◎	◎	◎	◎	◎

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

Thank you very much for completing the survey. We value your opinion and encourage you to inform others who you know would be interested.

Useful links

EMA website: Public consultation page (https://www.ema.europa.eu/en/regulatory-science-strategy-2025)

Background Documents

EMA Regulatory Science to 2025.pdf

Contact

RegulatoryScience2025@ema.europa.eu