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Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* Name

* Email

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):

European AIDS Treatment Group

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.

- The strategy is overall a good document. It is clear, pragmatic and detailed enough.

- The strategy looks like something that has been designed to RESPOND to the issues the agency will likely face in the next years, but there is no effort to CREATE a vision for the future; it is reactive, not active; it responds, it proposes no issue. This can be very right, but we as patient organisations would like to see the agency take a more proactive role in this field.

- Patient involvement in this strategy remains as it is. The agency has put a lot of efforts to increase and improve patient involvement in its activities in the past years, but this should not be considered as enough. Patient involvement should be improved in each and every step of the whole process of medicine development, assessment and approval and the agency should take an active role in catalysing patient involvement. As it is now, the strategy has many paternalistic approaches in its language as well as in its recommendations, as described in some cases in the following sections.

- Strategy has good concept regarding human medicines. As always, there are so many abbreviations so sometimes it is very difficult to track. As always agency is working in way that she reacts on something when it happens. Prevention is not the strong side of EMA. EMA acknowledges importance of patient organisations but at the same time, we are not included in collaboration between healthcare professionals, industry and academics. My opinion is also that this document is poor on information about data protection. It is mentioned several times but not elaborated on.

- Pharmacovigilance is practically never mentioned in the strategic document, while it is a key role of the EMA. The Agency should first and foremost guarantee that the medicines on the market are safe, and the activities of pharmacovigilance should be strengthened with drugs arriving on the market at an early development stage.

- Require that one of the 2 RCT for approval should be done by an independent party. The EMA can demand raw-data for re-analysis. The EMA should make use of its existing power to mandate two RCT.

- Require superiority trials whenever possible rather than non-inferiority trials.

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

Comments on strategic goal 5 (h):

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

It is sad to read that “The Agency’s fifth goal is to develop the existing interaction between the EU regulatory network and academia further” while no goal of this kind has been written related to patients and their organisations.

Where is partnership with the community?

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.

This is the only recommendation explicitly related to patient involvement

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.

Benefits and risks vary rapidly and the regulatory approach should follow up to these changes

Third choice (h)

9. Foster innovation in clinical trials

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.

New clinical trial designs are needed to respond to the new patient needs.

To this end, it is important to strengthen the scientific rigour and relevance of RCT's used in the marketing authorisation process. Large simple RCTs in the later phase of development should be supported to collect meaningful data on the patient groups that will be treated in clinical practice. Gender differences and other relevant subgroups (such as the elderly) must be reflected in RCT.

In order to improve trust in the EU regulatory system, it could be envisaged to a) demand comparative RCTs where possible, b) require that one of the 2 RCTs for approval be done by an independent party, c) pool resources across Member States to do meaningful-pragmatic RCTs responding to the right questions of clinical practice, d) require superiority trial whenever possible rather than non-inferiority trial,e) studies should be done to validate surrogate endpoints. Moreover, the use of surrogate endpoints should be discouraged nor accepted where final outcomes are achievable within a reasonable timeframe and without harm for trial participants.

In terms of the post-marketing authorization generation of evidence (about the efficacy and safety of new medicinal products) emphasis should be paid to the reporting of adverse effects.

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

Yes, patient role, how to make patient voice heard in the right way through all the medicine development and assessment process, for example by making patient involvement an key element for new product evaluation or encouraging the use of patient reported outcomes measures.

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:

General comments for strategic goal 1:
Mentioning the move towards a “more patient-centred healthcare” is very good, but then, to do this, a “collaboration with academics, research centres and infrastructures” is considered as required, forgetting any further involvement of patients and their representatives.

Beyond this, precision or individualized medicine requires an approach from medicine developers which is not only focused on profit but allows for flexibility. In this sense, it is simply unacceptable that some medicines are only marketed as part of a fixed dose combination with other medicines, while combining them with alternative products could bring a much bigger benefit to the single patient. The agency should not grant marketing authorization to products which are only available in fixed dose combination, unless there is a clear, established futility in their availability as stand-alone compounds.

Would change in one sentence patients to humans. First sentence of the goal: “EMA seeks…. safe and effective treatment for patients.” Humans would be better option because the same document is talking about animals. For example at page 7 text is saying: “…ensuring that patients and animals and caregivers have the medicines…” and in my opinion it should be “ensuring that humans and animals…” Goal is promoting data collection but it isn’t elaborated sufficient. Goal 3.1.2. is mentioning “creative payment models” but not explaining what this is.

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**:

Language note: the term “protect”, “protection” etc. used for patients sounds extremely paternalistic (reference to the sentence: “while continuing to be protected from medicines whose benefits do not outweigh their risks”).

Huge amount of abbreviations which are not known for those not working in EMA. In section 3.2.4. one of the actions is: “develop the capability to analyse Individual Patient Data to support decision making.” I didn’t see and data protection measures regarding access to patient data: who, where, why, which data…

This is the only section where there is an important sentence on patient involvement: “This will require EMA to build on its existing frameworks that bring together stakeholders at all levels of the decision making chain, including, importantly, patients and healthcare professionals themselves”.

Unfortunately, there is no mention on how this will be achieved.

Again, mentioning large amount of data with additional needs to ensure privacy and security of the data but there everything about data security stops. No other sentence how will be this addressed.

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**:

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**:

Again, as a language note, avoid the term “protect” which sounds very paternalistic (“The Agency’s regulatory mission is to protect human and animal health”)

Again talking about data with no special interpretation how they will be gathered.

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