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 Federation of Allergy and Airways Diseases Patients' Associations

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.

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.

> Currently, patients' priorities are too little taken into account in clinical trials design, resulting in clinical trials data that may not include patient-relevant endpoints. Quality of life is often not adequately reported. 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. It is also important for bridging the evidence gap between marketing authorisation, HTA and payers. Linked actions are needed to enhance benefit-risk assessment with more integration of patient perspectives and patients' input; develop a regulatory framework for digital clinical data generation; and promote the use of high-quality real world data which should include patient-generated data. There is also a link and synergy with improved patient input into developing information and communication on medicines.

Second choice (h)

> 15. Contribute to HTAs' preparedness and downstream decision-making for innovative medicines

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.

> There is currently an evidence/data gap between regulatory requirements and those of HTA bodies, which impedes swift evaluation of new medicines and patients' access. We see a need to integrate discussions about the evidence and data requirements of all of these decision-points much earlier into the clinical development of a medicine. This would also require actions to enhance collaboration with payers, and improve the provision of regulatory advice along the development continuum, with the involvement of HTA, payers, patients and healthcare professionals.

Third choice (h)

> 20. Deliver real-time electronic Product Information (ePI)

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.

Patients need and want more up-to-date, accessible, tailored and interactive information on medicines. Delivering real-time electronic product information is important but should not only be limited to putting package leaflets online; rather it should also include a more in-depth review of the PIL to ensure it is really "fit for purpose" for informed decision-making, in line with the recommendations of the European Commission's 2017 report and the PILS and PILS-BOX studies commissioned by the European Commission. Patients' involvement and input in developing statutory product information and labelling should be improved. Currently, patient review of package leaflets comes too late and is limited to one or two reviewers; patients' comments can often not be taken into account because of the constrained format of the PIL; and there is no systematic approach to user-testing the PIL.

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

EFA believes pharmacovigilance is a significant missing element. One of the EMA's key tasks is to oversee the European system for medicines safety monitoring and reporting of side effects. In particular, we see the need to ensure effective communication of side effects both in clinical trials results (including the "lay" versions intended for patients and the general public), understandable and actionable information on side effects on medicines' package leaflets, and supporting greater patient involvement in pharmacovigilance and in reporting of side effects.

The strategy also misses another significant element: the meaningful, structured and systematic involvement of patients needed to realise each of the strategic goals. The Agency's Chief Executive Guido Rasi recently stated the EMA will play its role to make patient engagement the norm by 2025. EFA welcomes this important statement of intent. The EMA has been at the forefront of patient involvement in medicines regulation in the EU, but this is a fast-advancing area and EFA believes the current strategy needs to be more explicit and more ambitious in this regard. The strategy to 2025 should set standards for all actors in medicines research, development and evaluation. It should include concrete elements that will contribute to the goal of making patient engagement the norm, including specific activities, expected outcomes and an evaluation approach.

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:

EFA sees precision medicine, biomarkers, cell and tissue therapies as important emerging treatment classes, so this as well as the related evaluation pathway are high priorities. However "personalised" medicine is not only about precision medicine, and it needs a comprehensive approach that avoids fragmentation. "Omics" is a promising field where the convergence of science and technology can offer innovative solutions, provided it is organised in a structured and exploitable way across Europe. We also draw attention to the way technology can transform the relationship between patients and healthcare professionals in various ways, for example in terms of information, communication, shared decision-making and ethical issues; any regulatory strategy should take into account these impacts. Collaboration with the research community is necessary, but not sufficient to realise these objectives. As the end users of medicines, patients should occupy a central and active role in the development and evaluation of medicinal products and technologies, as well as the underlying evidence-generation, bringing their knowledge and experience to the evaluation process. The EMA as the EU's regulatory body should make patient involvement explicitly a sine qua non, open up opportunities for patient involvement and provide clear guidance to both industry and academic researchers. Finally the EMA should ensure that there are no conflicts of interest in the integration of science and technology in medicines development, as in other areas.

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**:

We consider it important to foster innovation in clinical trials, and see the role of EMA as ensuring the high quality of evidence on safety, efficacy and quality. It may be an important area to look at adaptive or pragmatic clinical trials designs in this context and see how the best possible evidence can be generated. Digital clinical data should include patient-generated data and its integration into high-quality regulatory assessment. Patients and patient organisations should be involved systematically to ensure full integration of real world data as an integral part of the "life cycle" of medicines.

Patients are a key stakeholder group that needs to be engaged in all activities and this is currently missing from the strategy. Innovation is also about finding better ways of doing things, and innovating meaningful, structured and systematic patient involvement should be a key responsibility of the EMA. Patient involvement brings added value to innovations in clinical trials, data generation and benefit-risk assessment, as well as communication.

We support actions on paediatric and geriatric medicines as well as women who are pregnant or breastfeeding, including generation of improved safety data. For better understanding of diseases across the board, the EMA should encourage investment in the development of treatment solutions addressing the needs of potentially vulnerable or under-served groups of patients, including the above. Finally, EFA believes it is important to advance on non-clinical models and on the 3R principles (to replace, reduce and refine animal testing).

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**:

EFA agrees that patients' access to new and better medicines should be a high priority. However, a more inclusive decision-making process must involve patients as key partners. Reinforcing the patients' role in evidence generation and in all related processes should be a high priority. Patients' involvement in clinical trials should be ensured from setting the research agenda to the design and conduct of trials. Quality of life and burden of the disease for the patient must be better addressed in clinical trials, with appropriate measures and endpoints that are co-developed with patients and validated to ensure they are relevant and meaningful. The EMA should pose a clear requirement for meaningful patient involvement in all clinical trials used for regulatory purposes, and provide clear guidance to industry, written together with patients. The EMA should furthermore play an appropriate role to encourage meaningful patient involvement in HTA and related processes at national level.

On real-world data and evidence, we would like to see the development of a framework to ensure the uptake of high-quality real-world evidence by regulators; harmonisation of patient registry requirements can be a driver in this respect. Communication for trust and confidence is a key priority. Transparency of the EMA's work and processes, and visible patient participation in all activities and governance should help improve trust in regulation. Transparency of clinical trials is also vital. Patients can play a role in ensuring effectiveness in communication. Electronic product information should be efficient and user-friendly, as well as being interoperable with other eHealth applications in the EU, including ePrescriptions and Electronic Health Records. However, regulatory information for patients on medicines needs to be further developed, going above and beyond "e-PI", to make it more fit for purpose. This should be done in close partnership with patients and patient representatives.

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**:

For EFA the most important objectives under this goal relate to collaboration on addressing medicines shortages, including supply challenges and the availability of all medicines (including old as well as new medicines) to patients. Supporting research and development of new antibiotics is also a high priority in this area. Patient involvement in this area should be further developed and described.

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**:

EFA believes these are important actions, but they are also highly inter-linked. Dissemination and sharing of knowledge should flow from enhancing partnerships and collaborations, and identifying the best expertise. Communicating knowledge, expertise and latest developments in medicinal products is key in helping patients make informed and safe decisions. Such initiatives can improve the coordination among regulators and stakeholders and enhance the understanding and enforcement of legislation. The strategy mentions engagement between regulators, funders and academia, but omits mention of patients. The patient perspective and patients' involvement is required to ensure that the most relevant knowledge and expertise is leveraged in regulation. Academic stakeholders are often in need of training on how to work with patients; the EMA could provide a useful platform for interaction and understanding, promoting partnerships between patients and academia and to encourage researchers to practice meaningful patient involvement. The EMA should do so with the involvement and support of patient representatives at the Agency – including the PCWP, the scientific committees and management board.

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