

Public consultation on EMA Regulatory Science to 2025

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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):

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.

Compliments with this highly ambitious agenda. From the PC-perspective many points are relevant and very much supported by the EFPC.

Very good is the wider scope of the EMA addressing problems with access and availability of drugs. We know it is difficult for political reasons to address the consequences of strategies of countries/ pharmaceutical companies. Yet, it is an obligation to the EU-citizen to make absolute clear where the patient interest is not served by protective or marketing mechanisms. We miss that attention a little.

We think the agenda could even more clearly focus on doing research where protective / self interest strategies of countries/ Pharma or other hindering mechanisms frustrate proper delivery/ research/ innovation.

To make visible where countries and industries could improve on performing in these aspects, may be of great service to the patient. It could be another argument for to citizens why Europe is beneficial for them.

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)

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

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.

Independent research is highly needed; supporting researchers in network led partnerships will stimulate the independence of researchers in their activities
Academia should be also linked to professionals (the best expertise) who should be involved to provide the real-world data, in particularly within the post-market surveillance of products

Second choice (h)

18. Promote use of high-quality real world data (RWD) in decision-making

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.

Third choice (h)

30. Identify and enable access to the best expertise across Europe and internationally

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.

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

See below

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'	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
3. Promote and invest in the Priority Medicines scheme (PRIME)	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Facilitate the implementation of novel manufacturing technologies	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>

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

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	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Foster innovation in clinical trials	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
10. Develop the regulatory framework for emerging digital clinical data generation	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
11. Expand benefit-risk assessment and communication	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
12. Invest in special populations initiatives	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
13. Optimise capabilities in modelling and simulation and extrapolation	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
14. Exploit digital technology and artificial intelligence in decision-making	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>

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:**

3.2.2 Foster innovation in clinical trials

Innovation may come, for example, through the use of novel trial designs.

Comment: it is important to start clinical trials that include the acquisition of data from the real world, not only in post marketing, but also in the preliminary stages to better identify the clinical features of the patients in the studio.

3.2.3 Develop the regulatory framework for emerging clinical data generation

Comment: it is appropriate to develop methods and tools to acquire clinical data from the collaboration of the HCP.

3.2.5. Invest in specialpopulations initiatives:

A population we miss here is the worrisome care avoiders, who need special strategies for compliance. Easy access and monitoring actively and being on a patient list helps (TB, HIV, addiction, etc.) Would that fit in this paragraph?

Proposed change (if any):Add a program on this issue.

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	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
16. Bridge from evaluation to access through collaboration with Payers	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
17. Reinforce patient relevance in evidence generation	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
18. Promote use of high-quality real world data (RWD) in decision-making	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
19. Develop network competence and specialist collaborations to engage with big data	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>

20. Deliver real-time electronic Product Information (ePI)	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
21. Promote the availability and uptake of biosimilars in healthcare systems	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
22. Further develop external communications to promote trust and confidence in the EU regulatory system	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>

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:**

3.3.

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.

Comment: This is maybe too soft. If you really want to get a finger behind what obstructs access and availability, you may have to do some more detective work. Find out about the bottlenecks and about the ownership of the problem. I think EMA has the authority and obligation to do so, even if it implies blaming and shaming.

Proposed change (if any): Add a goal about more probing research in bottlenecks and problems.

3.3.4

Promote use of high-quality real-world data (RWD) in decision-making

It would be recommended from a Primary Care perspective to invest in relevant qualitative research for the (long-term) follow up of medicine intake at individual and population level which goes beyond N1 but should be initiated by case-descriptions submitted by experienced Primary Care professionals with research expertise. The development of an online platform linked to the larger system of data collection would be an additional recommendation in this respect.

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	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
24. Continue to support development of new antimicrobials and their alternatives	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
25. Promote global cooperation to anticipate and address supply challenges	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
26. Support innovative approaches to the development and post-authorisation monitoring of vaccines	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
27. Support the development and implementation of a repurposing framework	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>

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:**

3.4.4. Support innovative approaches to the development, approval and post-authorisation monitoring of vaccines

Engage with public health authorities and NITAGs to better inform vaccine decisions. Of course, this is part of the broader strategy to overcome mistrust of authorities.

Comment: Yet we would recommend a more direct approach to probe if EMA could play a role in building trust in the Vaccine public misinformation. In this paragraph this is not addressed straight. In the Netherlands 1/3 of the people distrust RIVM, our public Health Authority. In Europe it is likely to be just as big of a problem.

People do trust their GP a lot more than Public health institutions, so vaccination should maybe made fully part of the more comfortable and trusted environment of The Family Practice.

We would highly recommend research to see if this would address part of the problem and if it would be recommendable.

Rereading the paragraph we do not agree with the problem analysis supporting the vaccination problem. It is a bit beside the point.

Proposed change (if any): Address straight the mistrust and add an extra subgoal addressing this point of how to improve trust in Vaccination.

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	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
30. Identify and enable access to the best expertise across Europe and internationally	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

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 \(https://www.ema.europa.eu/en/regulatory-science-strategy-2025\)](https://www.ema.europa.eu/en/regulatory-science-strategy-2025)

Background Documents

[EMA Regulatory Science to 2025.pdf](#)

Contact

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