

Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* Name

* Email



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.

Overall this is an impressive and well considered document. My views, although shaped by my own experience within my patient group, are intended to represent the views of all patients, or at least all of those affected by rare diseases. The particular needs of rare diseases are different as a result of the smaller populations and do need to be given separate consideration in my view.

EMA is in a unique and in some ways unenviable position. Whether desired or not, the institution is the most public face of European health policy and the most visible incarnation of the principles which we might all wish to see embraced throughout the community. This means that the power and influence of EMA extends far beyond its formal role as medicines regulator and represents a daunting burden of responsibility.

This presents numerous difficulties. EMA is restricted by a mandate that does not fully recognise this wider community role of the agency, although there is evidence that this wider role is acknowledged within EMA and there are initiatives that try to do justice to the task. Unfortunately, without the right political support and infrastructure these are very difficult to implement. Patients look to EMA to provide leadership in ways that are beyond the capabilities and, in some respects, competence of the agency.

With that in mind, I would ask that EMA remains aware of this heavy responsibility and endeavours to provide that leadership wherever it can. One overall impression I have from this impressive document is that some activities are considered in silos. This is inevitable in such a complex organisation but more effort to draw together functions which are (or will become) interdependent is needed.

Real World Evidence is likely to become of much greater significance in decision making, especially in rare diseases, and as registry data and AI technologies mature. AI is not just about big data - in fact some of the greatest potential is in more intelligent and effective use of small data. I am sure there is some recognition of this but not sure that it is fully represented in this document and the danger is that if it is not documented it can be lost.

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)

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

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.

I would conflate this goal with that of improving clinical trials, which may seem like heresy to some, but I believe that without effective RWD clinical trials are inefficient (again, with particular reference to rare diseases) but that wherever possible CTs should be used alongside RWD. The old-fashioned notion of CTs as the "Gold standard" of evidence generation is outdated and dangerous. Effective gathering of RWD requires new approaches to registries and natural history studies as well as the application of new and powerful statistical and other tools to analyse this data. As previously stated, this can be even more important for "Small data" as for "Big data."

I would also draw in the emergence of digital clinical data generation and these are all part of the same parcel IMHO.

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.

This topic keeps me awake at night. HTA authorities are woefully ill equipped for the challenges that face them and it does require much greater cross-agency collaboration than currently seems possible. EMA is in a unique and privileged position as a pan-EU agency and must show leadership and even courage in this area - difficult in the current political climate I know. In particular, the lack of a strong voice acting for the HTA community (EUnetHTA's efforts notwithstanding) is a particular problem and one that I am sure EMA recognises.

Third choice (h)

1. Support developments in precision medicine, biomarkers and 'omics'

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.

We can't always find the answers to the big questions but there is more that can and should be done to ask smaller questions on the path to getting the big answers. Biomarkers and "omics" are an important way to help us find those big answers, so long as they are applied intelligently.

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

I think most of it is there, but I worry that the connections are not always made between the different components that make up the strategy, and that as a result some opportunities are lost.

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)

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	Very important	Important	Moderately important	Less important	Not important
1. Support developments in precision medicine, biomarkers and 'omics'	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Diversify and integrate the provision of regulatory advice along the development continuum	<input checked="" type="radio"/>	<input 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:

6. Regulatory and HTA advice at early stage is vital but too frequently overlooked and the efforts to combine the two are so far inadequate (in large part because of structural obstacles). The value of this advice to medicines developers is enormous, but too many are too proud or stupid (or both) to recognise that fact and far too many applications are made for regulatory approval with basic errors that should have been fixed much earlier - and certainly before the CT is completed.

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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Foster innovation in clinical trials	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
10. Develop the regulatory framework for emerging digital clinical data generation	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
11. Expand benefit-risk assessment and communication	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
12. Invest in special populations initiatives	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

13. Optimise capabilities in modelling and simulation and extrapolation	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
14. Exploit digital technology and artificial intelligence in decision-making	<input checked="" type="radio"/>	<input 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 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
16. Bridge from evaluation to access through collaboration with Payers	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
17. Reinforce patient relevance in evidence generation	<input checked="" type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input 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 checked="" type="radio"/>	<input 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 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 checked="" type="radio"/>	<input 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 type="radio"/>	<input checked="" 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
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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 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	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

fundamental research in strategic areas of regulatory science					
29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions	<input checked="" type="radio"/>	<input 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 checked="" type="radio"/>	<input 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:**

For rare diseases (30) collaboration with ERNs has enormous opportunity but will need effective resourcing, both within EMA and the ERNs, neither of which is anywhere near ready for this task right now.

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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