

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

*** Please specify:**

between 1 and 1 choices

- ☐ Individual company
- ☐ Trade association
- ☒ SME

Name of organisation (if applicable):

Aparito

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 topics identified are relevant to the current and future trends in pharmaceutical development.

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.

Moving towards personalised medicines the current existing clinical trials model is no longer appropriate to deliver medicines to patients in a timely manner without compromising regulatory standards. Having regulatory authorities promoting the use of high-quality RWD in decision-making will encourage companies to move towards innovation that they reluctantly accept to embrace. Ideally to develop this new model, areas with the highest pressure in the systems such as rare disease and special populations are ideal pilots to initiate this culture change. Such a change could be more easily done under the umbrella of public/private partnerships. With EMA calls through the IMI.

Second choice (h)

14. Exploit digital technology and artificial intelligence 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.

There is a wealth of unexploited data available at country and EU level with a wealth of AI companies that could tremendously synthesise and leverage existing knowledge, combined with genetics AI and real word patient monitoring, that could address many of the on-going challenges faced particularly in rare disease when combined with CT.

Third choice (h)

13. Optimise capabilities in modelling and simulation and extrapolation

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.

Modelling/Simulation and Extrapolation require a culture change and a new interdisciplinary operational model. These tools should however not be left at the hands of mathematicians at risk of having patients and clinicians losing trust in regulatory decision-making that they might not understand or control, but rather integrated these tools in the clinical decision making by educating and adapting them to the clinicians need. Extrapolation will remain a clinical decision, particularly in areas when you don't have enough data to develop models and statistical approach, with still benefit-risk balance decision to be made. Having quantitative experts in all the EMA committees and their respective EMA teams to bridge with the quantitative working parties would be already a great asset. Otherwise quantitative tools will never be integrated in regulatory decision to their full potential.

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

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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2. Support translation of Advanced Therapy Medicinal Products cell,	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

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:

It would be good to correlate the priorities according to the time needed to move forward some of these topics. For instance to "Support developments in precision medicine, biomarkers and 'omics'" would not require much to progress as compared to "Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments" where many more stakeholders would need to be harmonised before being able to initiate such a project.

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 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 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:**

Same comment as above

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					
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:**

More publications are needed to promote trust and confidence, and there is a VERY URGENT NEED to educate ethical committees by engaging with them and involving them. They will kill any innovation that they don't understand or don't feel part of.

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"/>

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:**

All of this is very important but many other players than EMA have their role to play to get things moving in these areas.

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"/>
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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:**

Ethical committees roles should not be underestimated in the development of regulatory science. They won't approve something they don't agree with.

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

Contact

RegulatoryScience2025@ema.europa.eu