

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.

I feel it to be of vital importance that interaction between researchers in academia and industry is fostered, and therefore welcome this opportunity for public consultation. I further believe that concepts and technologies developed within academia, and experience gained needs to be shared with regulatory authorities to further the development and evaluation of safe and effective medicines.

In this framework I have participated in fundamental research on the development and evaluation of "nanomedicines" for almost three decades. I am therefore delighted that EMA recognizes the need to characterize and assess these complex drugs.

Such drugs, though showing high variability in terms of size, shape, materials used, etc. share complex structures, which cannot be characterized in their entirety. Their size and attributes at the molecular scale confer these systems certain properties that impact their interaction with their biological environment, and thus influence PK/PD and safety profiles.

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)

6. Develop understanding of and regulatory response to nanotechnology and new materials' utilisation in pharmaceuticals

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.

Due to their inherent complex structure, nanomedicines are impossible to be fully characterized by physicochemical methods alone. In vitro as well as in vivo studies, leading up to verification in clinical trials, are required. Therefore, an effort is needed to discover these correlations between specific critical quality attributes (CQA) and their impact on biological activity, ideally to be able to predict in vivo behavior. This can only be realized through a multi-pronged analytical approach and correlation to clinical data. In addition, my laboratory analysed several nanomedicines approved as generics (so-called "nanosimilars"), which showed significant differences in important properties when compared to the originator drug. This has also been corroborated in the scientific literature in prospective as well as retrospective clinical studies. Based on this, a review and revision of the regulatory strategy for the approval of nanomedicines and their follow-on products is advisable.

Second choice (h)

- ☐ 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
- ☐ 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
- ☐ 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
- ☐ 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
- ☐ 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

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)

- ☐ 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
- ☐ 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
- ☐ 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
- ☐ 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
- ☐ 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

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)

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 checked="" 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 checked="" type="radio"/>	<input 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 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:

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 checked="" type="radio"/>	<input 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>
12. Invest in special populations initiatives	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
13. Optimise capabilities in modelling and simulation and extrapolation	<input 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:**

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 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
17. Reinforce patient relevance in evidence generation	<input 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 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>
20. Deliver real-time electronic Product Information (ePI)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
21. Promote the availability and uptake of biosimilars in healthcare systems	<input type="radio"/>	<input 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 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
25. Promote global cooperation to anticipate and address supply challenges	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
26. Support innovative approaches to the development and post-	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

authorisation monitoring of vaccines					
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:**

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 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:**

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