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 Alliance for Access to Safe Medicines CIC (www.EAASM.eu)

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.

EAASM welcomes the opportunity to respond to the EMA's Regulatory Science to 2025 through this public consultation. Science and technology have never moved as fast as now. The Strategy rightly points that there are many areas where there is a need for more collaboration across the European regulatory landscape to enhance innovation and patient access to new medicines.

However, it needs to be pointed out as well that access to new medicines should not come at the expense of safety. The EMA should therefore ensure that the European regulatory framework is fit for purpose in order to guarantee that this fast-paced innovation and the tremendous breakthroughs we are experiencing are reaching patients safely.

It is for this reason we have decided to respond to this consultation.

In March 2019 our organisation hosted an event in the European Parliament entitled "Handling Innovation in nanomedicines: regulatory changes needed to realise new treatment opportunities and ensure patient safety." During the meeting, it was made clear that the complexity and the quantity of this new class of medicines are increasing at a rapid pace and so are the questions around assessing their quality, properties and therapeutic profiles.

We are concerned that the current regulatory framework and the way nanomedicines and their follow-on products are authorised and treated within the current system, are raising questions over the therapeutic equivalence of the copies over the reference products. This, de facto, challenges their safety.

We do recognise that the Strategy includes the need to better understand the potential of nanotechnologies in human medicine. However, it is our view that there are more concrete actions that need to be taken. We are expanding on those further below.

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.

Current regulatory approvals for nanomedicines and follow on products (also referred to as nanosimilars) are decided on an ad-hoc basis by product category. With the accelerating progress in the development of clinically significant follow-on products and in in the absence of a specific regulatory pathway for them, the EAASM believes that it is the right time to set the scene for building a consensus so that this regulatory weakness can be addressed. And to make medicinal products of high-quality and safety.

Another important element is the fact that nanomedicines and nanosimilars can be approved through a decentralized procedure in the EU. Europe has successfully pioneered and implemented a solid regulatory framework for biosimilars that ideally should serve as model for the approval of nanosimilars. Such framework avoids potential confusion and ambiguity of having a different policy or interpretation per country.

Nanotechnology in health can offer opportunities to address unmet medical needs and hence regulatory changes are needed to realise new treatment opportunities that will help ensure patient safety and wellbeing.

Clearly nanomedicines and follow-on products are complex, and patients must not be put in harm's way from products that are copies of the original without absolute scientific assurance that their therapeutic profile has been tested as rigorously as the originator's. Patient safety must always be the most important criteria when assessing the granting of a new product licence. That is why we need to create a robust and totally fit for purpose regulatory framework which is clearly needed in this exciting and developing field of medicine.

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)

In the absence of a specific regulatory pathway, more scientific, policy and practice knowledge on the quality, safety and efficacy of nanomedicines and follow-on products must be gained and there is a need to build a consensus dialogue as well as alignment between all players in Europe and beyond to further explore the field of nanomedicines at EU and national level. Awareness as well as a well-controlled robust manufacturing process are indeed fundamental to ensure quality, safety and efficacy.
We are asking the EMA to further develop these elements in its strategy and provide more clarity in order to ensure patient safety.

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:

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**:

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**:

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**:

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**:
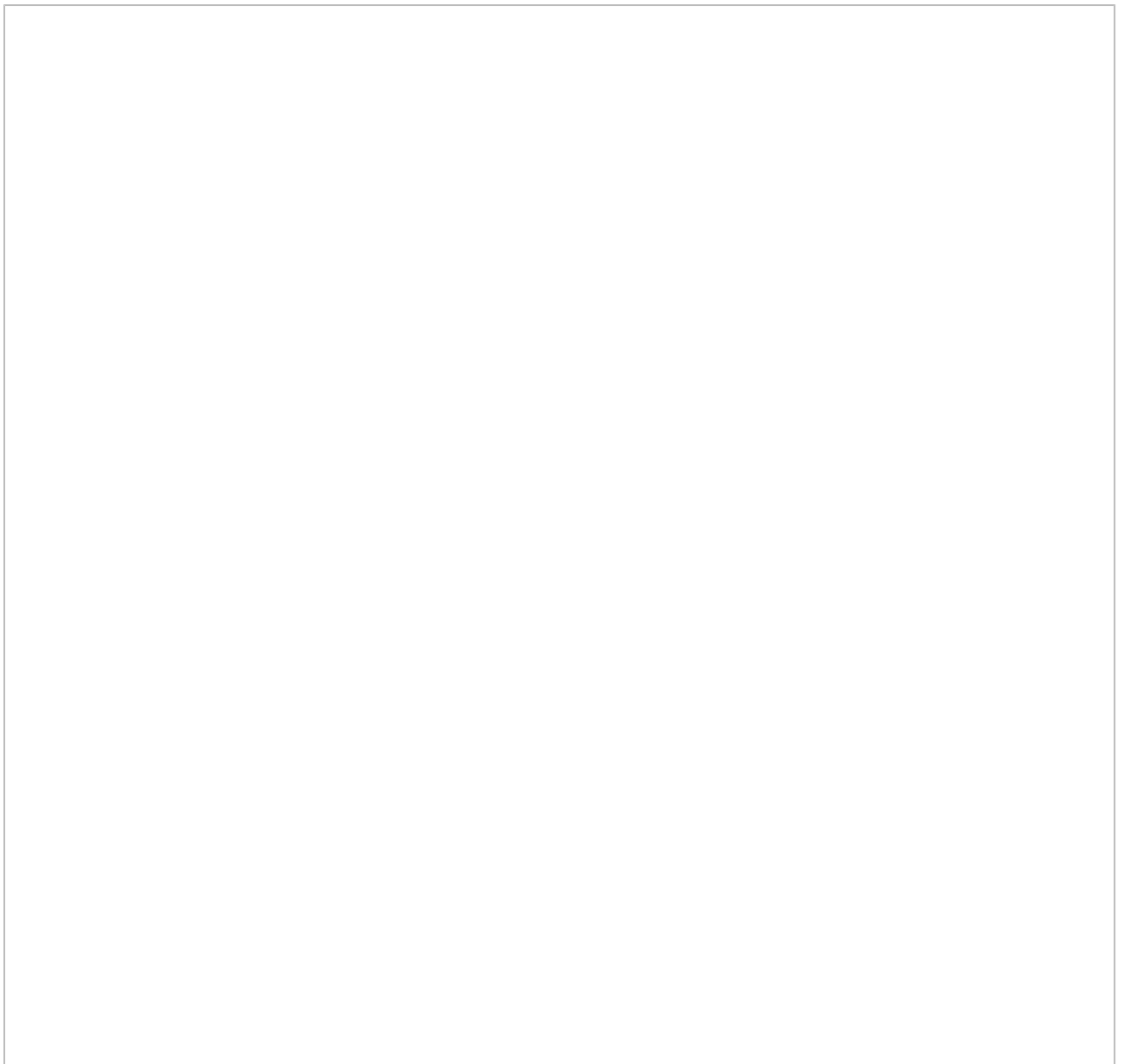
Thank you very much for completing the survey. We value your opinion and encourage you to inform others who you know would be interested.

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

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