

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.

HAS (Haute Autorité de Santé) is the French health authority in charge, among other duties, of performing health technology assessments (HTA) of all types of health technologies i.e. pharmaceuticals, medical devices, procedures and other health interventions upstream of Pricing and Reimbursement decisions. Although it is a legally-independent public agency and is particularly pursuing this basic principle in the HTA field, HAS recognizes the value of exchanging information with regulators both nationally and at the EU level.

HAS welcomes the project of EMA Regulatory Science to 2025 (Strategic reflection) and is pleased to have the opportunity to comment on it.

The goal of HTA and regulatory decisions is primarily focused on providing patients access to the best treatments, meaning treatments with a positive benefit/risk (B/R) ratio based on the demonstration coming from clinical trials (CTs) and with a demonstrated added value comparing to standards of care. The 2 steps (marketing authorisation (MA) and reimbursement) are essential. We believe patients and consumers deserve that demonstration.

HAS acknowledges some common priorities with EMA such as reinforcing patients' relevance in evidence generation and supporting innovative health technologies (i.e. precision medicine, biomarkers, ATMP and PRIME products as well as innovative devices).

1. Accelerating access to real innovations in particular situations is a common goal

HAS would like to raise some concerns on the mention of shortening time in the paper. It might be interpreted as a willingness to increase accelerated approvals based on limited evidence. It is increasingly frequent for those products, that EMA grants approval although the demonstration of positive B/R is not robust, only presumed; our understanding is that EMA is willing to increase the use of conditional MA (cMA) in order to accelerate access. In relation to this, HAS would like to make few comments and suggestions:

- Granting cMA is already an issue for HTA bodies (HTAb); due to the low level of evidence available, we are facing great uncertainties - particularly on the demonstration of efficacy and safety and added value; increasing cMA as it is planned by EMA in the strategic plan may lead to difficulties for HTAb and payers. We believe that gold standard to get the best level of evidence is still the comparative study and notably randomized clinical trials (CTs). We would be pleased if the principle of Evidence Based Medicine is not wiped out.

- We note a deep change of paradigm that the paper is introducing regarding the development of medicinal products: it is about authorizing them as quickly as possible and letting the demonstration of B/R be carried out during the post marketing phase, mainly on real world data collection. HAS believes that other regulatory tools may be used in the situations of B/R presumption, rare or serious disease and unmet needs like compassionate use (CU). We esteem that there is no need to decrease the level of demonstration and lower the level of MA but we should instead make better use of the CU programmes.

- Finally HAS underlines that it is not an infrequent occurrence that further CTs performed after the cMA are negative. The way those situations are to be solved should be taken on board by the paper. HAS believes that patients, consumers and prescribers deserve the best information. HAS strongly suggests that all parties be better informed (i.e. patient leaflet) when decisions on the product are conditional and on how to manage the situation of a possible negative demonstration of efficacy in the future.

2. Limit of post-launch evidence generation

While HAS sees an interest in post-launch evidence generation especially to monitor technology use and its impact on health care organizations, it should not prevent the conduct of comparative studies in an experimental setting pre-launch.

Post-launch evidence can be derived from clinical studies or real world data sources. In any case they should be of high quality. HAS suggests the development of international good practice guidelines for the development of real world data.

3. Parallel EMA/HTA Scientific advice

HAS appreciates the interest of interactions between HTA bodies and regulators in order to inform the developers on how to fulfil the evidence requirements of both regulatory and HTA bodies. However it should not take the form of continuous assistance to developers but rather punctual recommendations based on a proposal of advanced study protocol at a critical stage of the 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)

- 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

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.

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
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- 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)
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- 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"/>				
2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments	<input type="radio"/>				
3. Promote and invest in the Priority Medicines scheme (PRIME)	<input type="radio"/>				
4. Facilitate the implementation of novel manufacturing technologies	<input type="radio"/>				
5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products	<input type="radio"/>				
6. Develop understanding of and regulatory response to nanotechnology and new materials' utilisation in pharmaceuticals	<input type="radio"/>				

7. Diversify and integrate the provision of regulatory advice along the development continuum	<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 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"/>				

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

17. Reinforce patient relevance in evidence generation	<input type="radio"/>				
18. Promote use of high-quality real world data (RWD) in decision-making	<input type="radio"/>				
19. Develop network competence and specialist collaborations to engage with big data	<input type="radio"/>				
20. Deliver real-time electronic Product Information (ePI)	<input type="radio"/>				
21. Promote the availability and uptake of biosimilars in healthcare systems	<input type="radio"/>				
22. Further develop external communications to promote trust and confidence in the EU regulatory system	<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:**

HAS would be pleased to have clarifications on the following two points mentioned in the paper:

- P23 “Contribute to the identification of priorities for HTAbs”. While exchange of information with EMA could facilitate identification of emergent therapies, priorities for HTA should be defined only by HTAbs. Would the EMA please elaborate on this statement?
- P24 “Co-develop with HTAbs a core health-related quality of life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment”. Are we talking about a unique tool to be used in all trials and in real life independently of the patient’s profile?

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"/>				
24. Continue to support development of new antimicrobials and their alternatives	<input type="radio"/>				

25. Promote global cooperation to anticipate and address supply challenges	<input type="radio"/>				
26. Support innovative approaches to the development and post-authorisation monitoring of vaccines	<input type="radio"/>				
27. Support the development and implementation of a repurposing framework	<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 type="radio"/>				
29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions	<input type="radio"/>				
30. Identify and enable access to the best expertise across Europe and internationally	<input type="radio"/>				
31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders	<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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