

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.

CPME welcomes the public consultation undertaken by EMA on its strategic approach to 'Regulatory Science to 2025'. Technological advances, in drug development but also in the digital area, require a critical reflection on how to integrate them in existing regulatory frameworks, while simultaneously maintaining the highest standards of quality, efficacy and safety of new drugs for the benefit of patients.

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)

15. Contribute to HTAs' preparedness and downstream decision-making for innovative medicines

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.

See general comment to 'strategic goal 3'.

Second choice (h)

24. Continue to support development of new antimicrobials and their alternatives

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.

See general comment to 'strategic goal 4'.

Faced with the increasing threat posed by AMR, CPME has been looking at doctors' role in containing AMR, also in the context of healthcare associated infections, by promoting good practices on the prudent use of antibiotics and providing input to policies. CPME also considers that the development of new antimicrobials, alternative treatments and appropriate diagnostic tools would be a step forward in the fight against AMR.

Third choice (h)

26. Support innovative approaches to the development and post-authorisation monitoring of vaccines

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.

See general comment to 'strategic goal 4'.

Given the decreasing vaccination coverage in many European countries, CPME has been advocating that the prevention of communicable diseases through vaccination is safe and effective. We believe that doctors, other healthcare professionals and their organisations - alongside with public authorities - are essential to deliver facts based on scientific evidence and increase public awareness about the benefits of immunisation.

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, genes and tissue-based products into patient treatments	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Diversify and integrate the provision of regulatory advice along the development continuum	<input type="radio"/>	<input type="radio"/>	<input checked="" 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:

- General comment: Supporting the development of new drugs is an area where EMA can have an added value by ensuring that these drugs will meet the existing regulatory requirements for marketing authorisation. In this regard, the importance of pre-submission activities should be emphasised. It should be ensured that appropriate evidence will be generated and collected by pharmaceutical manufacturers before requesting a marketing authorisation at EU level and subsequent reimbursement decisions at national level.

- Recommendation 7: When early scientific advice is asked for, it must be ensured that it addresses the needs of regulators but also HTA bodies and payers. It is also necessary to avoid discrepancies between data requirements and level of evidence accepted at the different levels.

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 type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
9. Foster innovation in clinical trials	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
10. Develop the regulatory framework for emerging digital clinical data generation	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input type="radio"/>	<input checked="" 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 checked="" type="radio"/>	<input type="radio"/>
14. Exploit digital technology and artificial intelligence in decision-making	<input type="radio"/>	<input type="radio"/>	<input checked="" 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:**

- General comment: CPME endorses expressly that EMA attaches importance to the issue of evidence generation. In this regard, it is critical that the highest standards of quality, safety and efficacy of drugs are maintained. From CPME's perspective, the challenge is about completing the scientific evidence collected through clinical trials rather by collecting additional data rather than replacing clinical trial data (see CPME policy on access to medicines and pharmaceutical pricing). Consequently, the implementation of post-market authorisation studies (e.g. after accelerated marketing authorisation procedures or for orphan drugs) is particularly relevant to reduce the uncertainty about the efficacy and safety of new drugs.
- Recommendation 9: When discussing novel trial designs, endpoints and techniques, clinicians should be involved in the process.
- Recommendation 10: New digital technologies offer the opportunity to collect large amount of data. Nevertheless, a critical reflection on which data is relevant in the context of drug development processes needs to be undertaken.

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 type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
17. Reinforce patient relevance in evidence generation	<input type="radio"/>	<input checked="" 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 checked="" 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
20. Deliver real-time electronic Product Information (ePI)	<input type="radio"/>	<input checked="" 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 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 type="radio"/>	<input type="radio"/>	<input checked="" 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:**

- General comment: Ensuring that data requirements and evidence accepted by EU regulators and other decision makers at national level are more comprehensible would be highly beneficial to ensure better access to medicines across the EU. EMA would indeed benefit from a closer collaboration with other decision-makers in order to better understand their expectations when it comes to data requirements.

- Recommendation 15: See comment to question 5 (first choice).

- Recommendation 18: Real World Data (RWD) can provide useful supplementary data in the context of marketing authorisation processes and in particular post-market surveillance activities. In this context, it is important to have in place appropriate framework ensuring the quality, robustness, reliability and usefulness of collected data.

As mentioned in the strategy reflection document, CPME considers of utmost importance to also guarantee the confidentiality and privacy of patient data. It requires the existence of an appropriate data governance model.

- Recommendation 19: See comment to recommendation n°18.

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

- General comment: CPME agrees that EMA's support to address major health treats, such as antimicrobial resistance (AMR) and vaccine hesitancy, could be of added value. Worrying trends in AMR rise and vaccination coverage across the EU should lead all stakeholders to take actions in the remit of their competencies.

When it comes to areas of high unmet needs, the development of push incentives (to encourage innovation in specific fields) as well as the critical review of the EU legislation on orphan drugs (to target more specifically areas of unmet needs) can be mentioned as particularly relevant.

- Recommendation 24: See comment to question 5 (second choice)

- Recommendation 26. See comment to question 5 (third choice)

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"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
30. Identify and enable access to the best expertise across Europe and internationally	<input type="radio"/>	<input type="radio"/>	<input checked="" 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 type="radio"/>	<input checked="" 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:**

- General comment: Activities related to the identification of fundamental research topics in strategic areas (such as PROs, biomarkers and omics-based diagnostics, big data) as well as the dissemination and exchange of knowledge and expertise are also relevant.

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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