

# 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](mailto: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

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### 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.*

The strategy builds upon the excellent work already being done by the EMA, by emphasising the need for more collaboration across relevant stakeholders to improve research and patient access to medicines. The need for more efficient processes throughout the ecosystem, from regulatory to HTA assessments, is a clear priority throughout the document. This is essential for reducing waste. It is also great to see a movement towards patient-centred healthcare, echoed in the importance of patient-centred research and the inclusion of PROs in clinical trials and the evaluation process. This could be strengthened by the inclusion of patient important outcomes through the uptake of core outcome sets (an agreed minimum set of outcomes that should be measured and reported) throughout the ecosystem. A well-developed core outcome set will have included all relevant stakeholders, including patients or their representatives, in the determination of the most important outcomes to be measured.

**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)

17. Reinforce patient relevance in evidence generation

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.

It is important that relevant stakeholders are involved in the decision about which outcomes to measure in clinical research. Minimum standards for the development of core outcome sets (COS) ensure that COS appropriately reflect outcomes that are important to those groups, particularly patients, health care professionals and those that will use the COS in their research (<https://journals.plos.org/plosmedicine/article?id=10.1371/journal.pmed.1002447>). The use of COS in clinical trials will ensure that outcomes important to patients, trialists and health care professionals are considered. This is a more robust approach to generating patient-relevant evidence than involving just a few selected patients at the design stage of a trial.

Inclusion of PROs in trials is useful, but these need to be those most relevant and important to patients. We suggest that clinical guidelines should be updated to include not just reference to inclusion of a PRO (where PRO would be better phrased as 'Patient Relevant Outcome'), but to include reference to the inclusion of a COS when a relevant COS exists.

Second choice (h)

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

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.

The inclusion of core outcome sets (COS) throughout the ecosystem from regulatory to HTA assessments will facilitate the action 'Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans'. Their use allows research to be compared and combined as appropriate, and ensures that all studies provide usable information.

There is a current interest in identifying how COS might fit into the different stages of the healthcare research system, and with their proximity to reimbursement/coverage decisions, the EMA can increase awareness of COS, encourage COS development and support uptake of COS (see podcast from Hans-Georg Eichler (Senior Medical Officer, European Medicines Agency) [http://www.comet-initiative.org/assets/downloads/Hans-Georg%20Eichler\\_COMET.mp4](http://www.comet-initiative.org/assets/downloads/Hans-Georg%20Eichler_COMET.mp4)). It is important that the EMA works seamlessly with HTA bodies to ensure that relevant outcomes are included earlier in the research lifecycle (See podcast from SBU <https://www.youtube.com/watch?v=PGMhUkdoZag&feature=youtu.be>)

Third choice (h)

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.

Disseminate and share knowledge, expertise and innovation across the regulatory network and to its Stakeholders

Assessing the uptake of a core outcome set allows the impact of research on the development of core outcome sets to be assessed. The Rheumatoid arthritis COS was published in 1994. A recent assessment of the uptake of that COS showed an increase over time, to 81% of trials in Rheumatoid arthritis measuring and reporting the COS (<https://www.bmj.com/content/357/bmj.j2262>). The COS is endorsed by both the EMA and FDA, and this is likely to have contributed to trials measuring and reporting the outcomes in the COS. It is clear that regulatory bodies are highly influential in the outcomes included in clinical trials. Achieving this co-endorsement for other well-developed COS would increase the ability to compare and contract evidence from around the world more quickly, increasing the speed with which patient-relevant evidence is generated.

Further discussion between regulators about co-endorsement of specific well-developed COS would be welcomed.

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

A core outcome set (COS) is an agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical trials in a specific condition. They are also suitable for use in clinical audit or research other than randomised trials that report on health-related outcomes. This allows research to be compared and combined as appropriate, and ensures that all studies provide usable information. An increasing number of COS developers also intend their COS for use in routine health care practice. The COMET Initiative provides and maintains a database of COS (<http://www.comet-initiative.org/studies/search>), as well as carrying out methodological research and producing guidelines for COS development.

There is a current interest in identifying how COS might fit into the different stages of the healthcare research system. Relevant bodies have identified the potential challenge of different organisations' requirements for condition-specific versus generic quality of life outcomes in COS. COS may serve as a thread that pulls all the way through the healthcare research ecosystem. Many organisations now actively endorse the use of COS and the COMET database, including the EMA (see <http://www.comet-initiative.org/cosuptake>).

Strategic goal 2: This would be strengthened by including 'outcomes' that are relevant to all stakeholders, including patients. For example: Driving collaborative evidence generation – improving the scientific quality and relevance of evaluations to patients

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



### 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 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	<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 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:**

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**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 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"/>

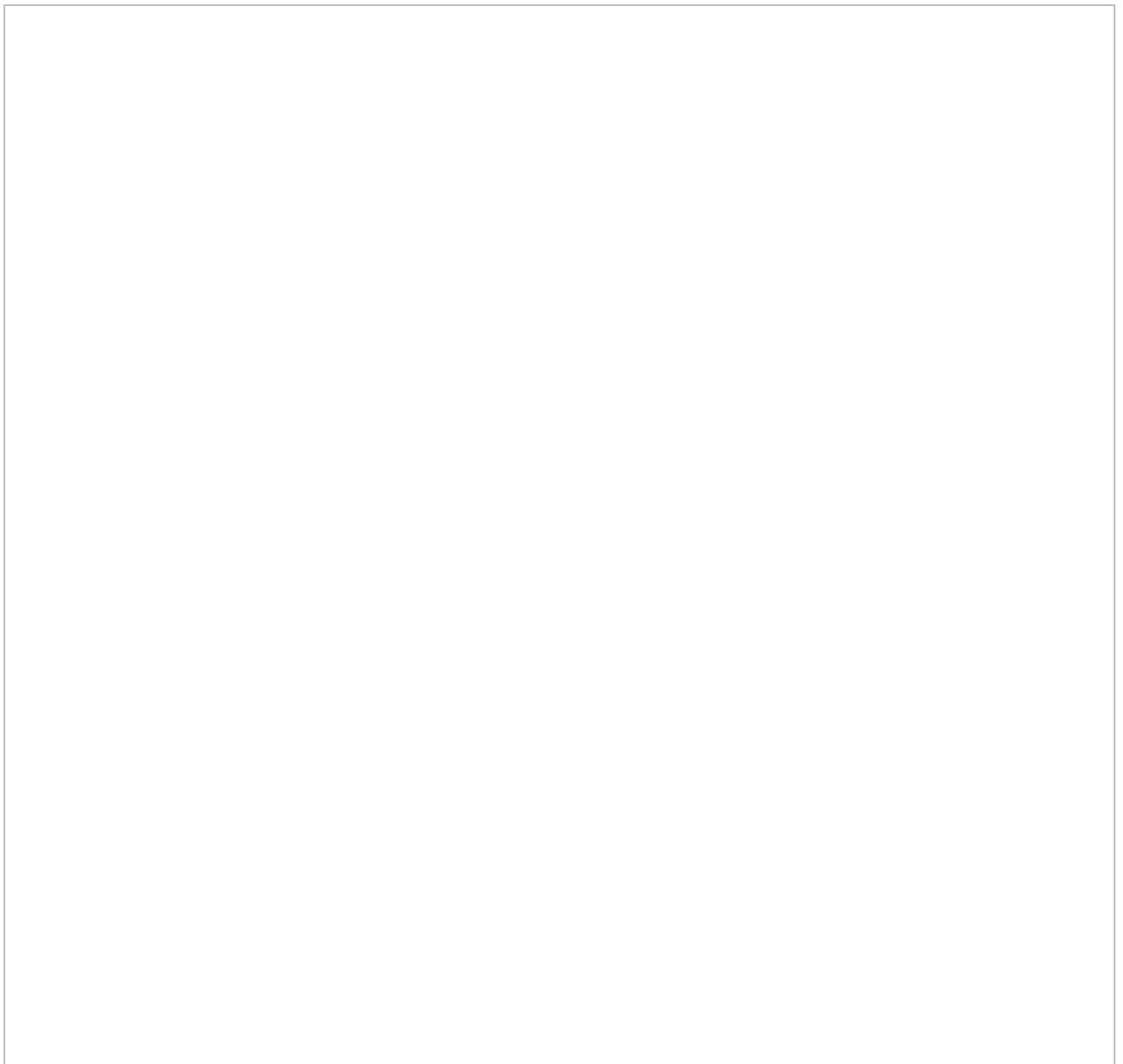
25. Promote global cooperation to anticipate and address supply challenges					
26. Support innovative approaches to the development and post-authorisation monitoring of vaccines					
27. Support the development and implementation of a repurposing framework					

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

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