

# 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

---

### 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 proposed covers a range of very relevant aspects from regulatory perspective. For many of the considerations and plans presented in the paper, it is considered highly important that National Competent Authorities (member states) are identified and integrated as important stakeholders. This pertains to planning as well as implementation of strategic steps towards adequate reactions to upcoming challenges.

**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)

11. Expand benefit-risk assessment and communication

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.

The mentioned subtask: Develop the capability to analyse Individual Patient Data to support decision-making addresses an issue that was already debated during the last years in relation to “transparency policy”. Any plans to (re-)analyse clinic trials individual patient data is associated with high investment within the regulatory network. Aside the required personal and technical resources, thorough consideration should be given to a potential shift in assessment paradigm within the licencing of new drugs, if access to CT data and the capabilities for data analyses would be implemented. Possible/potential roles of NCA’s assessments capacities in this context would need to be explored in a timely manner.

Second choice (h)

4. Facilitate the implementation of novel manufacturing technologies

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.

This topic is considered of high relevance in particular in relation to the Horizon 2020 Project „Continuous Downstreamprocessing of Biotechproducts“, where AGES is acting as partner organisation. This project exemplifies the future potential role of NCAs in this area of innovation.

Third choice (h)

19. Develop network competence and specialist collaborations to engage with big data

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.

Connection big data bases at the international level is expected to be a huge undertaking, requiring a thorough exploration of the possible contributions of member states (NCAs).

**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 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 type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
4. Facilitate the implementation of novel manufacturing technologies	<input checked="" 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					
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	<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 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

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:**

ad 8.: “Stimulate developers to use novel pre-clinical models, including those adhering to the 3Rs” will be in particular important for ATMP- developments; “Re-focus the role of the 3Rs working group to support method qualification”: relevant from AGES perspective as Rapporteurship for the revision of ICH S5(R2, Reprotox) is held by AGES employee; “Encourage implementation of IT tools to exploit the added value of SEND1 for the re-analyses of non-clinical studies to support both clinical trials authorisation FIM (first-in-man) and risk minimisation across EU” would need to be expected to have high impact on NCA’s resources in CT approval system;

Ad 9.: Novel designs and data sources require adapted statistical methodologies for their planning and analysis. In addition, new endpoints may need to be developed (for example when disease-modifying treatments replace symptomatic ones) and new biomarkers to support bridging of surrogate endpoints in early development to clinical endpoints in confirmatory studies. Regulators will need to work with other bodies involved to ensure that innovative designs meet the needs of all stakeholders.



### 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 checked="" 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 checked="" 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 type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
19. Develop network competence and specialist collaborations to engage with big data	<input checked="" 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 checked="" type="radio"/>	<input type="radio"/>
21. Promote the availability and uptake of biosimilars in healthcare systems	<input checked="" 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 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:**

ad 21.: From AGES perspective there is a strong connection to the topic, because Rapporteurship for EMA Reflection Paper on Statistical Methodology for the Comparative Assessment of Quality Attributes in Drug Development (under revision) is held by AGES employee;

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

-

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