

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.

The consultation is very long and uses terms that are not easy to understand for an average audience, even less if the audience does not have English as a first language.

It contains little examples of how it will be put into practice and how it will be translated in the day-to-day work of the Agency and other stakeholders the Agency collaborates with.

We feel that the strategy has not made of a priority to have a timely access for patients to the novel and innovative therapies, which, in the end, would be the whole purpose of developing therapies. Perhaps this may be due to the fact that the Patients and Consumers Working Party has not been consulted in advance of the publication of this strategy, having missed a chance of including the patient's voice upstream.

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

Comments on strategic goal 1 (h):

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

The concept per se is correct, it would be a matter of understanding in practice how this will be translated.

With regards to raising global awareness of ATMPs to maximise knowledge sharing and promote data collection: these are not the only two aspects of the importance of raising global awareness. The fact that by raising awareness patients will be more informed and will have more information on ATMPs will also facilitate their informed assessment on the specific ATMP.

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)

25. Promote global cooperation to anticipate and address supply challenges

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.

Today, the first threat to the health of patients with primary immunodeficiencies is the lack of access to immunoglobulin replacement therapies due to shortages. This is of key concern for IPOPI as we have receive reports of patients, due to the lack of sufficient therapy, having to receive lower dosages below the specifications of the treating physician, patients receiving their therapies not in the frequency recommended by their doctor or, in the most severe cases, patients not been able to access their therapy and suffering in their health to even die due to the lack of access.

Second choice (h)

17. Reinforce patient relevance in evidence generation

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.

Until now, the involvement of the patient in evidence generation, data gathering, has been quite limited, especially at national level in some Member States. We believe that in general, but especially for rare diseases, the patient is key in collecting information that is not so easily available to treating physicians, scientists or industry. The patient is the one taking the therapy and, as such, knows first-hand the effects of such therapy on his/her body and what would be a meaningful improvement, for instance, in his/her quality of life (that would have an impact on the definition of the clinical trial end points, for instance).

Third choice (h)

16. Bridge from evaluation to access through collaboration with Payers

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.

There is not real benefit to public health if the new and innovative therapies developed are not accessible to patients for economic reasons. This would, in fact, increase disparities in access and would be against the World Health Organisation objective of making health accessible to all, leaving no one behind.

Understanding early in advance the requirements for the development of a novel therapy by the payers and for researchers to have a clear view on what are the parameters that guide reimbursement decisions would be of paramount importance. It has also been highlighted by the creation at the UN level of the NGO Committee for Rare Diseases and the work this Committee has started doing and the exchanges that it has already promoted: "No country can claim to have achieved universal healthcare if it has not adequately and equitably met the needs of those with rare diseases" (Helen Clark, UN administrator, source here: https://www.ngocommitteerarediseases.org/wp-content/uploads/2018/05/NGO-CfRDs-Submission-The-Right-to-Health-in-Rare-Diseases_Feb-15-2018.pdf).

In this sense, complementary and creative solutions should be considered when approaching price setting for instance, in the case of therapies developed or funded totally or partially with public funds (regional, national or European) perhaps considering a return on the money spent or similar alternatives could be considered.

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

The strategy describes very ambitious objectives that, indeed, would benefit therapy development, but it remains unclear where all the expertise will be pulled from and what would be sought in order to avoid any potential conflict of interests, especially in those very specialised disease areas in which expertise is shared by very few individuals.

It would have been interesting in having an annex to this strategy that would contemplate the budgetary impact of the measures suggested and possibilities for its funding.

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 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 checked="" 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 checked="" 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 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 type="radio"/>	<input type="radio"/>	<input type="radio"/>
7. Diversify and integrate the provision of regulatory advice along the development continuum	<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:

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 checked="" type="radio"/>	<input 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	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
12. Invest in special populations initiatives	<input checked="" 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 checked="" 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<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 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 checked="" type="radio"/>	<input 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 checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input 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 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 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 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:**

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

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

RegulatoryScience2025@ema.europa.eu