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Public consultation on EMA Regulatory Science to 2025

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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):

Trials Methodology Research Partnership (Funded by Medical Research Council and National Institute for Health Research, UK)

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.

Overall the goals are reflective and pertinent to the research and healthcare landscape, today and for the future. Sharing best practice and improving global communication is essential in the delivery and achievement of the strategic goals, and this must be effective across academia, science, industry, regulators, funding bodies and healthcare systems.
How this strategy and its delivery fits with the changing political environment of Europe, particularly the future position of the UK in Europe, is also a perspective that might need some consideration in future.
There is an ever demanding need for new, improved approaches and methods to be applied within specific populations and disease states and several of the reflective goals relate to meeting this demand.

As might be expected several goals, are particularly perceived by the MRC-NIHR Trials Methodology Research Partnership as more relevant than others, particularly in the context of delivering trials, optimising trials methodology and producing the best evidence available to improve human health.

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations,
Strategic goal 3: Advancing patient-centred access to medicines in partnership with healthcare systems
Strategic goal 5: Enabling and leveraging research and innovation in regulatory science

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)

9. Foster innovation in clinical trials

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.

Clinical trials are the foundation of drug development and offer a pathway to achieving better patient health via identification of the best medicines, treatments and practices, supported by robust evidence.
Innovative and novel approaches in the methodology, design delivery of trials is essential to generate high quality, scientific evidence.

New opportunities are emerging in the field of informatics and precision medicine which will have a huge impact of the innovation of design and delivery of trials and the evidence they produce.

'Innovation' should also include continuing to identify ways to reduce research waste, particularly in trials.

There is increasing demand for development of innovative medical devices. Support for the improvement of quality scientific evaluation of medical devices should also be included under this strategic goal.

Fostering Innovation in Clinical Trials, is closely related to other recommendations including:

Support developments in precision medicine, biomarkers and 'omics'

Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

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.

The role, relationship and experience of the patient is crucial in evidence generation and is likely, therefore to determine the scientific quality of an evaluation involving patients.

When identifying outcomes to measure in clinical research and evaluations it is significant all potential stakeholders are consulted including Healthcare professions and patients, and where feasible future users of the research or evaluation undertaken.

One approach to this is by developing and utilising Core Outcome Sets* in clinical research. This enables that the outcomes reported in clinical trials are those of relevance to trialists, healthcare professionals and patients.

*an agreed standardised set of outcomes that should be measured and reported in clinical trials.

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.

Effective dissemination and sharing of these elements is essential to achieve success, enabling identifying, endorsing and apply best practice in clinical research and evaluation. Efficient dissemination and sharing will accelerate change and maximise impact in the future.

Many of the regulatory standards are based on developing and sharing best practice and working also with academia provides access to new and emerging expertise as well as training resources.

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

Identification of 3 core recommendations in question 5 is challenging as many of those listed of greatest importance, particularly in relation to the field of trials methodology, could be intrinsically grouped with other recommendations to deliver the most significant change going forward.

The improvement of quality scientific evaluation of medical devices- together with or independent of other medicines or healthcare.

Medical devices are increasingly used within research and to answer a research question, both on their own or as a combination product. The regulatory field governing this is ever tightening but still not yet as well governed as an investigational medicinal product. This area or field of research requires attention and investment.

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'	○	○	◉	○	○
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					

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:

This strategic goal is beyond our scope to comment on

however
2 As a newer (and advanced) model of research, achieving this goal would revolutionise rare and life threatening diseases where there has been no treatment or hope before.

The actions suggest looking at unmet medical needs where this disease area hasn't been the focus of research before.

This is ground-breaking medicine and could shape the future for many treatments going forward. Raising awareness of this need and knowledge sharing would be key to the success of this goal.

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					

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	○	○	◉	○	○

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**:

Delivering this goal (2) is dependent on strong collaborations, partnerships and effective communications across academia, science, industry, regulators, healthcare and patients.
Broad expertise may need to be recruited in collaborations to ensure scientific quality.

There is increasing demand for development of innovative medical devices. Support for the improvement of quality scientific evaluation of medical devices should also be included under this strategic goal.

Medical devices are increasingly used within research and to answer a research question, both on their own or as a combination product. The regulatory field governing this is ever tightening but still not yet as well governed as an investigational medicinal product. This area or field of research requires attention and investment.

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	◉	○	○	○	○
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	○	○	○	○	○

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**:

Patient-centred access to medicines should already be in place and progress in this area is welcomed. A patient-centred approach is supported by the significance of patient centred research and the inclusion of patient reported outcomes in clinical trials.

21 Biosimilar medicines should be widely available but less is known about them and they are regulated inconsistently. A uniform approach to this would improve access to such products and increase awareness of availability.

17. is one of our core recommendations as we believe it is highly significant in clinical research and evaluations particularly clinical trials

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	○	○	○	○	○
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					

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**:

23 Our competent authority (UK) already respond well to emerging threats with specific protocols in place but resource is however limited.

Obviously preparedness for such occasions is paramount to effectively facilitating actions should the need arise. If there is a unified approach to this, globally this would make a huge difference.

24 As new and mutated threats arise, novel antibacterial agents will be needed to address the threats so this goal is important in supporting not only the development of these but also the supply and demand of these products.

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	○	◉	○	○	○
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	◉	○	○	○	○

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**:

Identifying relevant and fundamental topics to base research upon and engaging with stakeholders to develop partnerships. This creates a fluid communication network on how to share research questions and best practice.

Many of the regulatory standards are based on developing and sharing best practice and working also with academia provides access to new and emerging expertise as well as training resources.

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