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):

Regulatory Science Ireland (RSI)

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.

Regulatory Science Ireland (RSI) has been set up as a not for profit Company Limited by Guarantee, registered in Ireland (www.regulatoryscienceireland.com). It is constituted to be a network of interested parties and currently involves academics engaged in research and teaching related to medicinal products and/or medical devices; individuals with experience and expertise from industrial employment in medicinal products and/or medical devices; representatives of various national organisations with an interest or, engagement in medicinal products and/or medical devices. Regulatory Science Ireland is committed to the development of an integrated Irish response to the global Regulatory Science effort by establishing an environment through which relevant research, training and communication creates a cohort of Irish based Regulatory Science experts to strengthen research in regulatory science in Ireland and to contribute to the development of the discipline internationally. RSI welcomes the opportunity to comment on this consultation document. The strategies expressed in the document are consistent with the mission of RSI, in particular the emphasis on collaboration between the regulators and other groups including academics 'to enable and leverage research and innovation in regulatory science'. The inclusion of ATPMs is also extremely relevant, as the development of these innovative therapies is likely to continue to increase it is vital that new regulatory tools are developed to more effectively assess these products.

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)

> 28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

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.

> Comment:
> RSI believes that the current rate of innovation in the technologies enabling novel new forms of medicines of the Advanced Therapy category far exceeds that of regulatory innovation. If regulatory innovation continues to lag there will be delays in providing novel therapies where needed and more dangerously a real risk that continued application of a regulatory paradigm that is not fit for purpose will see the occurrence of avoidable tragedies that creates a lack of public confidence in these new therapies or, the supporting regulatory system. This reality poses enormous challenges for all stakeholders to ensure so far as is possible that the level of regulation applied is in the best interest of the patient and balances possible risks versus potential benefits across many domains. RSI sees the blossoming of regulatory science as the basis of a solution to this enormous challenge. Furthermore, we consider that this will require an open multidisciplinary approach in which the voice of all stakeholders is heard and collaborations across academia, industry and the regulators are essential if true progress is to be achieved.
>
> Actions:
> •   Less emphasis on perceived 'conflict of interest'
> •   Provide funding opportunities for collaborative regulatory science initiatives at regional levels within the EU
> •   Promote cross-sectoral dialogue in the regulatory science area

Second choice (h)

> 2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments

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.

Comment: RSI agrees that ATMPs have the potential to change the therapeutic landscape to the benefit of patients. We note that the tremendous advances in medical research in cell and molecular biology of the last few decades, often in academic groups or small biotech companies, is now creating massive interest in the development of such medicines. RSI considers that effective translation of such complex products into commercial medicines will pose many challenges including the need to ensure that the available regulatory tools are capable of dealing with this new generation of products. We are convinced that a regulatory science approach resulting in the creation/adaptation of the regulatory paradigm and the available regulatory tools for these products is an essential part of this translational process.

Actions:
- Support for cross-sectoral research
- Facilitate 'wet lab' research
- Provide education and training for early stage researchers to increase awareness of regulatory challenges to development and translation

Third choice (h)

4. Facilitate the implementation of novel manufacturing technologies

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.

Comment: RSI agrees that ATMPs have the potential to change the therapeutic landscape to the benefit of patients. We note that the tremendous advances in medical research in cell and molecular biology of the last few decades, often in academic groups or small biotech companies, is now creating massive interest in the development of such medicines. RSI considers that effective translation of such complex products into commercial medicines will pose many challenges including the need to ensure that the available regulatory tools are capable of dealing with this new generation of products. We are convinced that a regulatory science approach resulting in the creation/adaptation of the regulatory paradigm and the available regulatory tools for these products is an essential part of this translational process.

Actions:
- Support for cross-sectoral research
- Facilitate 'wet lab' research
- Provide education and training for early stage researchers to increase awareness of regulatory challenges to development and translation

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

The need for 'wet lab' research to produce new tools and techniques to facilitate more efficient assessment and regulation of new innovative medicines (personalised medicines, ATMPs, vaccines).

Classification of emerging products such as bacteriophages and faecal transplants is often difficult under the current regulatory framework, this can lead to confusion and misunderstandings of the requirements needed for approval of such novel products.

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	◉	○	○	○	○

	Very important	Important	Moderately important	Less important	Not important
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	◎	◉	◎	◎	◎

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**:

Re recommendations 12, 13 and 14: To develop a successful medicine in a timely fashion a multidisciplinary approach is essential. The introduction and adaptation of in silico modelling techniques e.g PBPK and artificial intelligence can all be harnessed to accelerate the development pipeline and should facilitate the translation of novel concepts into precision medicines this will be particularly beneficial for special populations (paediatric and geriatric) and rare diseases.

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**:

> (blank comment box)

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**:

Re recommendation 23: With the increase in the aging population continued research into neurological disease (Alzheimers and Parkinsons) is essential.

Re Recommendation 24: Research into new antibacterial agents is definitely an urgent priority, novel approaches in this field including phage therapies which require innovative and flexible regulatory frameworks should be investigated. Likewise the role of the microbiome is another area from which innovative and challenging medicinal products may arise.

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**:

Re recommendations 23 and 24: Innovative medicinal products, such as cell-based therapies, gene therapies and tissue engineered products, have complex physicochemical and biological properties relative to traditional medicines and present challenges during manufacturing and at the pre-clinical and clinical stages of development. These challenges make assessment and regulatory approval of such medicines difficult as often the appropriate tools and techniques for thorough evaluation have yet to be established. Specific challenges include the need for a well-controlled manufacturing process that consistently results in high-quality product. It is necessary to investigate how the concepts/tools of QRM, knowledge management, QbD etc can be optimally deployed in this domain and how academia and industry working alongside regulatory authorities can explore ideas like "regulatory relief" in the best interests of patients needing to avail of such innovative medicines.

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