Contribution ID: 4fb5b579-158d-4ea8-b100-0f8212eabef5
Date: 11/06/2019 12:02:16

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):

EATRIS (EUROPEAN INFRASTRUCTURE FOR TRANSLATIONAL MEDICINE)

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 represents a sound exercise analysing the barriers, needs and potential solutions currently present in the European landscape. The document is clearly structured with ambitious goals that tackle the main challenges in the development of the regulatory process. The document has captured the current opinions of the main stakeholders from a holistic perspective and the analysis represents a faithful picture of the state-of-the-art in the development of regulatory science.
Some considerations that could to be taken into account for the next version are:
(i) Strategic reflections with an ambitious perspective run the risk of diluting the operational capacity of the strategy in becoming the pillar of a powerful action plan where the proposed underlying actions will have space to be developed. In this context, the strategy should contain a specific description of the operational consequences in organisational terms (i.e. standardization processes). This is an essential element in generating trust, as the European space has produced a number of reflection documents with a limited capacity of impact.
(ii) Although the patient community is mentioned as a stakeholder with voice and opinion. the document reflects, in our view, a passive role played by the patient community that does not reflects the potential capacities of the Responsible Research and Innovation (RRI) policies. This is an essential element as the patient community needs to have a deep understanding of the challenges and opportunities within the regulatory process in order to have efficient access to new therapies. Therefore, the strategy should tackle the challenge of proposing actions specifically addressed at creating a balanced knowledge within the patient community in order to manage expectations and guarantee the transparent involvement of citizens in a process that becomes a key pillar for the development of health care systems.
(iii) Although goal 5 tackles the challenge of creating an interface between the science and innovation space and the development of regulatory science, we feel that some underlying actions to specifically raise awareness of funding bodies (both at national and European level) need to be designed to guarantee that regulatory science elements (such as regulatory assessment of promising translational research) are fully integrated into the drug development process at very early stages.

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)

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

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.

With the increasing capacities of the industry to deliver more late-stage clinical and commercial ATMPs for serious diseases with high unmet medical need (e.g., T cell immunotherapies for cancer), bringing medicines to patients through optimized regulatory strategies and expedited pathways is becoming more important. However, the main bottleneck still remains at the level of generating scientific evidence. Promoting evidence generation requires strong support for translational medicine strategies in an area like ATMPs, that probably represents the main arsenal of therapy tools for cancer, neurodegenerative disorders, rare diseases and cardiovascular pathologies. To do this, close collaboration between regulatory and funding bodies in planning evidence generation becomes a priorit for key technologies (i.e. immunoprofiling, cell tracking, etc)

Second choice (h)

1. Support developments in precision medicine, biomarkers and ‘omics’

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.

Advances in the field of big data, omic sciences and non-invasive biomarkers will represent a major opportunity for changing the one-size-fits-all paradigm. Personalized or Precision medicine will need in the next few years strong public and private support to leverage the current state-of-the art and make possible the generalisation of sustainable and efficient patient-target solutions. This goal will require a proactive role from the regulatory bodies supporting public policies addressed at: (i) developing research projects to ensure the quality, completeness, validity and analysis of datasets, (ii) developing informatics, ICT and mathematics tools to integrate, analyse and extract value from databases (e.g. omics, health records, clinical data, imaging data, data from mobile devices and wearable sensors, behavioural, environmental) with specific attention on interoperability of the respective databases. This should include research to ensure the quality, completeness and validity of data.

Third choice (h)

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

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.

The “valley of death” - the large gap between basic scientific research and translation to novel therapeutics, underscores the need to restructure education and academic research to cultivate the fertile interface between academia and industry. But this gap becomes even deeper as knowledge of the regulatory processes at early stages of the discovery pathway is still scarce. Therefore, a structural change leading to the creation of network-led partnership with academia, industry and regulatory bodies becomes an urgent priority in order to bridge these frequently isolated actors. A bridging action plan to feed iterative and interactive engagements between these stakeholders should be a core strategy of the EMA and National regulatory authorities. In this context, one essential action would be training early-career scientists in regulatory science and training research institutions, which, although they have acquired accelerated expertise in the field of innovation, are still lacking training schemes on regulatory science in their own educational portfolios.

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'	◉	○	○	○	○
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:

Besides recommendations 1 and 2, we would like to draw attention to the emerging importance of creating evaluation pathways for the assessment of medical devices (recommendation #5). The generation of scientific evidence in this area represents a major challenge as the combination of medicines with medical devices introduces further levels of complexity in the evaluation process. A similar situation exist in the field of nanomedicines where the generation of evidence is still quite poor. Regulatory strategies must support actions generating evidence and recruit experts to create an adaptable regulatory framework that facilitates a sound, fair, efficient and sustainable implementation of these products.

Overall comment for strategic goal #1. This overall process of catalysing science and technologies in the drug development process requires the active participation of "bridging" mechanisms that will create connections st all levels of the drug development process. Along this line Research Infrastructures play a fundamental role in the advancement of knowledge and technology in translational research and drug development at different levels of the value chain. These organisations follow a model of "distributed infrastructure", which creates a stable network of capacities grouping a wide diversity of stakeholders who seek solutions to the challenges that we face in the development of new therapies. This is particularly relevant for the "medical research infrastructures", EATRIS (the research infrastructure for translational medicine), BBMRI (the research infrastructure for biobanks) and ECRIN (the research infrastructure for clinical research). All together, they represent a strong network of scientific knowledge and expertise in the efficient development of the drug development value chain. These type of organisations represent a powerful ally in the process of creating stable bridging tools integrating science and technology in the drug development process.

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**:

One specific comment for recommendation #9. The strategy suggests as an underlying action under this recommendation "to work with stakeholders to encourage collaborative clinical trials". In this context, the emerging importance of "platform trials" should be specifically mentioned. They represent an innovative development of "classical clinical trials" where a single master protocol in which multiple treatments are evaluated simultaneously defines the structure of the trial, allows collaboration between different industries, enhances the access of patients to different therapy options and maximises the capacities of the control arms, resulting in a more efficient strategy for innovative drugs. Adaptive platform designs offer flexible features such as dropping treatments for futility, declaring one or more treatments superior, or adding new treatments to be tested during the course of a trial. This is particularly important as platform trials can find beneficial treatments with fewer patients, fewer patient failures, less time, and with greater probability of success than a traditional two-arm strategy. Regulatory bodies must work with academia and industry in defining the regulatory boundaries of these instruments. Along this line, the EU-PEARL project, an international collaborative action of more than 50 European organisations that under the umbrella of IMI want to create a common framework for the development of adaptative patient-centered platform trials will become a reference for the regulatory authorities to assess the efficacy of this instrument in the era of personalized medicine as a tool providing the innovation needed to efficiently evaluate modern treatments.

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**:

Recommendation #15. HTA expertise for performing relative effectiveness assessment of novel therapy solutions represents a key element for the implementation of novel therapies. The strategy acknowledges the importance of discussing (we would prefer the term "co-creating") with HTA bodies, guidance and methodologies for evidence generation and review. Along this line, specific programs for HTA assessment in

the field of ATMPs should be developed and implemented. Impact assessment should also be developed in routine evaluations of benefit-risk.

Recommendation #17. RRI policies should be mentioned in the strategy as they provide the framework for enhancing patient involvement in EMA or national regulatory authorities. We recommend introducing the concept "co-creation" when patients are involved in the decision-making process as they become transformative agents of the process.

Recommendation #21. The use and extension of biosimilars faces challenges related to resilience of the health care providers and lack of public trust. The strategy recommends develop further strategic campaigns. This should be done not "for" the patient organisations but "with" the patient organisations. Again, an extension of RRI policies to develop strategies of co-creation with the "target" recipient of the communication action.

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**:

Nothing to comment here

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**:

Overall, we think this goal represents one the major pillars of the EMA strategy as it tackles the initial bottleneck that reduces the potential impact of regulatory science in an efficient process of drug development, which is the generation of scientific evidence while building a translational value chain. Although the ERA and the National Roadmaps have fully incorporated, with a variable level of success, strategic pillars focused on innovation strategies, integration of regulatory science into the research value chain remains far from being widely adopted.
The strategy identifies needs and challenges in consultation with academia and needs to engage with funding bodies to propose issue calls and establish research collaborations. We believe that a specific underlying action should be added to this recommendation, to create with these stakeholders regulatory science priorities to be incorporated into national and European translational calls, particularly in the field of novel therapies.

Also specific educational schemes for regulatory science should be promoted by EMA with educational postgraduate institutions and research institutions to provide educational tools to research communities. A strong focus here should be on early career scientists, complementing other priority needs for the optimum development of the research value chain such as the capacities of big data in the translational medicine pipeline. Along this line, we are deeply concerned about the lack of experts in regulatory science within research institutions responsible for the development of the European research agenda. In this context, a specific recommendation to tackle this barrier should be introduced in the strategy

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