Contribution ID: 771e0770-9cce-48f7-ada7-43a9adfdcf0b
Date: 28/06/2019 10:43:05

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):

Biomedical Alliance in Europe (BioMed Alliance)

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 Biomedical Alliance in Europe (BioMed Alliance) welcomes EMA regulatory strategic direction and commends the experts' efforts to build such a comprehensive document. A long-term strategy is urgently needed to tackle health issues and re-engineer healthcare systems. A strategic direction endorsed by various stakeholders will be a very useful instrument for decision making process. Strategic priorities with concrete steps of implementation will steer positive outcomes for patients and help healthcare system to assimilate disruptive innovation.

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)

1. Support developments in precision medicine, biomarkers and 'omics'

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.

Identify patients and societal needs first, and then develop research and treatment development accordingly, would be a more coherent approach and would ensure that innovation has a direct impact on patients. Patients have the right to benefit from the latest scientific discoveries and to be treated according to the highest level of clinical evidence. Technologies allowing the stratification of patients according to biological /genetic features should lead us to change our models: from "drug protocols looking for patients" to (fully biologically/genetically characterised) "patients looking for matching protocols". Thus, truly placing patients at the centre of the research and development (R&D) process, where they should be partners from its inception. Enabling patients looking for matching clinical trials will change drastically the way clinical trials are performed now and will enable to unlock the potential of personalised medicine.
This has been extensively analyzed in the White Paper on academic clinical research published in the European Respiratory Journal in February 2019.

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.

Data from treatment optimisation studies, registries, observationla clinical trials and electronic health records of patients should be interlinked and embedded into decison making process.

A way to identify patients' needs and assess therapies is through treatment optimization studies
Treatment optimisation studies should complement drug development regulatory clinical trials. Treatment optimisation studies are pragmatic prospective clinical trials randomized as much as possible in unselected patient population. Such studies can generate the evidence on the optimal way to use the drug i.e. treatment duration, dosage and combination with other treatment modalities.This evidence can be used not only to design a personalized treatment for a patient, but it can be linked and imbedded in the regulatory processes as well.

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.

The BioMed Alliance is well placed to provide expertise and collect feedback from a wide range of experts. Also, it can support EMA in disseminating information and in providing a platform for discussions and contact with the most relevant medical societies in Europe. We encourage and support our members to actively participate and contribute to regulatory discussions. Educational programmes and support are required to ensure that young researchers become more and more involved in this field.

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

A roadmap with major steps or milestones is needed to reach these goals. Seek implementation of these goals should be high on EMA's agenda and include it in the final document.
Some of the principles such as identification and prioritization of unmet needs are also addressed by different DGs within the European Commission. It is essential that EMA coordinates and work together with different EU Institutions and stakeholders to avoid overlapping and wasting of resources and time.

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:

1. To unlock the potential of personalized medicine, there is a need for an integrated pan-European infrastructure to support the use of patient data for health research.
Such a system circumvents the expense of active long-term follow up (and thus, allows adequate assessment of safety and cost-effectiveness of interventions) and provides information that is accessible for independent assessment by health authorities. European-wide clinical population-based registries, encompassing information on clinical data, biological and imaging data, biomarker test results, data on all therapies received and outcomes (e.g. via electronic patient records), are critical for the affordable implementation and validation of state-of-the-art precision medicine. Controlling the quality and accuracy of the data is essential and infers that minimal quality control requirements for building these databases and registries will need to be developed and implemented. Patients should also be empowered into (individual) data ownership and protection, as much as on the relevance of data.
The creation of such an infrastructure will enable researchers and clinicians to perform treatment optimization studies and state-of-the- art clinical trials. The evidence based, and data driven healthcare will improve patients' treatments and allow breakthroughs to be brought to patients.

White Paper on academic clinical research published in the European Respiratory Journal in February 2019

5. Medical devices are vital for the prevention, diagnosis and treatment of disease. The successful implementation of the Regulation is a fundamental priority for patients and healthcare professionals requiring access to safe, innovative and effective technologies. Freeing up resources and boosting the capacity of the European Commission and national competent authorities are needed to speed up the designation of notified bodies, complete implementing legislative acts, ensure a fully functional Eudamed database and introduce the system of expert panels.

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**:

8. It is an ethical requirement for all stakeholders in biomedical research to support the development and validation of such approaches. To do so would entail that alternative methods were tested using the same rigorous standards that apply to animal models, thus ensuring they can be safely used to replace animal-based approaches. Further, it will be necessary to develop standardisation and easy access of tissue cultures and biobanks for alternative studies. This will require a proactive attitude by the entire scientific community,including individual researchers, research organisations, and international associations including those represented in the Biomed Alliance and funding agencies,as well as on the political level.
A speedy increase in the availability, use and acceptance of alternative and innovative approaches in biomedical and veterinary research is very welcome.This could be achieved through bold measures to be taken by the European Commissionand national or,international funding agencies. Such an initiative could,for instance,take the form of specific calls for funding or quotas as well as the further development ofinternational repositories and databases of research protocols and methods related to alternatives.

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**:

15. It is more than obvious that there is a need for a robust and effective framework for collaborative EU-level HTA. Cooperation in HTA has the potential to streamline regulatory procedures, avoid duplication, shorten time for decision-making, and make the use of public and private human and financial resources most efficient, thereby boosting both the value and affordability of patient care across Europe.

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**:

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**:

29, 31
Learned societies have an essential role in enhancing science and educating researchers, clinicians and healthcare professionals. The BioMed Alliance represents 30 learned medical societies covering a wide range of specialities and diseases. Scientists should play a more consistent role in the regulatory frameworks and support regulators with guidance and advice. Throughout our activities, we try to involve more and more experts in the regulatory issues and make them aware of the need to participate and contribute to stringent regulatory aspects. Thus, we make sure the biomedical research community's recommendations are heard and taken into account. The BioMed Alliance can provide expertise and collect feedback from a wide range of experts. Also, it can support EMA in disseminating information and in providing a platform for discussions and contact with the most relevant medical societies in Europe.

.

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