Contribution ID: ea2b1c87-b905-4c5c-a476-d1e622743424
Date: 29/06/2019 14:02:34

Public consultation on EMA Regulatory Science to 2025

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* 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

* ***Please specify:***

between 1 and 1 choices

- [x] Individual company
- [ ] Trade association
- [ ] SME

Name of organisation (if applicable):

PWG Consulting (Biopharma) Ltd

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 proposed is broad in scope and contains significant reflections concerning new research and novel approaches to treatment which are reasonably foreseeable based on current knowledge. However the extension of concerns to include arguments for HTA approval in individual member states is not relevant to the consideration of benefit risk of individual medicines by an independent regulatory body. HTA assessment is a competence of individual member states, and decisions made in part reflect individual member states willingness to pay for medicines to be available to their citizens within their health care system and the resulting discrepancies are frequently a result of political decisions taken by member state governments. It is suggested that the EMA should refrain from extending its remit into this, highly politicized, arena while retaining its focus on scientific rigor in the assessment of MAAs submitted to EMA review and ensuring a level playing field for both centralized and decentralized review and approval systems.

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

Comments on strategic goal 3 (h):

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

While the intent of trying to enable access to approved medicines is laudable, this proposal extends the role of the EMA well beyond the scientific assessment of benefit risk into areas which involve political decision making across non uniform health care delivery systems in the member states. The EMA should focus on scientific rigor in product assessment and refrain from extending its remit into an area which is the competence of member state governments, who are, ultimately, responsible for their citizens access to healthcare.

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)

3. Promote and invest in the Priority Medicines scheme (PRIME)

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.

This scheme represents a pathway towards proactive collaboration between developers and regulators for medicines which may bring signficant changes for areas of high, largely, unmet need and could be usefully extended and used as a model system in guiding collaboration between industry and regulatory authorities for other medicines/diagnostics.

Second choice (h)

18. Promote use of high-quality real world data (RWD) in decision-making

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 PRIME scheme has offered several examples of use of real world data to supply information relevant to decision making which can act as a guide for further development of use of real world evidence for decision making in other areas. The development of standardized data collection systems and identification of critical data for decision making purposes would add value to this effort.

Third choice (h)

25. Promote global cooperation to anticipate and address supply challenges

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.

Common standards for manufacturing requirements and increased confidence in international standards of ensuring GMP would be of benefit internationally and reduce risk of medicines shortages which seem to have become commonplace.

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

Goal 4 needs to be extended to include emerging threapts due to viral disorders and promote and facilitate the devlopment of antiviral approaches, particularly those which can have broad spectrum effects. Furthermore, one major contributing factor to AMR is the inappropriate use of antibacterials for the treatment of viral upper respiratory tract infections. Stimulation of a competative market for antiviral therapies for respiratory viruses,such as exists currently in Japan and is emerging in the USA, by encouraging the registration of additional antiviral products other than the limited number currently approved in the EU would also contribute to this goal.

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:

Strategic Goal 1: Recommendation 2. ATMPs are rapidly emerging which have common characteristics, evolution of understanding can lead to creation of guidance for developers working in this space.

Strategic Goal 1: Recommendation 4. Focus not only on improvement of manufacturing efficiency but also the developmen of internationally accepted standards and monitoring practices to enable confidence in common quality standards across the globe.

Strategic Goal 1. Recommendation 3,7: Consider how the innovative approach to regulatory dialogue encouraged by the PRIME scheme can be extended.

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**:

Strategic Goal 2: Recommendation 9. This might include a revision to the current standard of randomized trials to include observational outcomes derived from 'real world' cohort use.

Strategic Goal 2: Recommendation 12: The agency should consider focusing on encouragement of inclusion of a better representative patient group into clinical trials as this would be more representative of 'real world' outcomes than a highly homogeneous group of patients with risk factors for adverse effects which drive the current exclusion characteristics of many trial protocols. Inherent in this would be the need for better acceptance of sub group analysis for regulatory decision making. Early inclusion of children without additional requirements for toxicology studies and better definition of extrapolation might be considered also.

Strategic Goal 2: Recommendation 14. Creating common data standards would be helpful and may enable pooling of data more efficiently.

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**:

Strategic Goal 3: Recommendations 15 and 16. These extend the remit of EMA beyond scientific evaluation into political decision making which is a member state government competence. The EMA might consider whether a free market, competitive system would better enhance medicines availability and lower prices.

Strategic Goal 3: Recommendation 17. Patients views on the relevance of data used for decision making should be heard and incorporation of these into endpoint definitions should be facilitated.

Strategic Goal 3: Recommendation 18: Encouraging greater use of real world data for primary registration would be desirable. This may require the development of common data collection systems for international use.

Strategic Goal 3: Recommendation 19. This is mandatory to achieve recommendation 18.

Strategic Goal 3: Recommendation 20. These systems can exist already but many patients do not have access. There will remain a need for paper based systems which can also be updated in real time.

Strategic Goal 21: Recommendation 21: This is already being done and thus is superfluous.

Strategic Goal 22: Recommendation 22. There is already high trust in the regulatory system, but further explanation of reasons for eg divergent opinions between FDA and EMA - both areas in which there is a high

degree of trust but where agencies may diverge in assessment of benefit risk - would be relevant, particularly as FDA is frequently faster to approve eg new treatments for cancer.

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 4: Recommendation 24 Should be extended to include aniviral therapies for treatment and prevention of emergent viral disease threats eg pandemic influenza.

Strategic Goal 4: Recommendation 26 The EU CDC already monitor the effectiveness and safety of vaccines in clinical use. The current approach to approving vaccines based on measurement of immune response, as opposed to clinical effectiveness, is efficient.

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**:

Strategic Goal 5: The issue here is the rapid advance of scientific knowledge and its application towards the generation of new approaches to diagnosis and disease management and the implications of this for a regulatory body charged with determing the parameters for benefit, risk and quality assessment. The EU is not alone in facing these challenges. Ergo collaboration with experts in the relevant scientific fields should not be restricted to EU experts alone. In addition, the extent to which industry employees may be expert in these fields should be considered,

Strategic Goal 5: Recommendations 28 and 29. These would be met by focusing on recommendations 30 and 31 and appear to be superfluous. In addition the IMI initiave already exists, in part, to address these needs.

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