Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* Name

* Email

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: Press/media/NGO/Not-for profit organisation/other scientific organisations/policy maker, etc.

Healthcare Technology Company

Name of organisation (if applicable):

Flatiron Health

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.

Flatiron Health supports the broad range of goals, recommendations and actions outlined in the EMA's Regulatory Science to 2025 reflection. We believe the holistic approach espoused in this document, including prioritization of guidance, creation of capacity within the EU network, and increased opportunities for stakeholders to engage with the EMA, drives toward more efficient research, development, and approval of medicines. In particular, we support EMA's emphasis on learning from recent advances in "big data," including both the use of RWD and genomic information. In particular for oncology, we believe that real world clinical data sources and genomics data from tumor profiling together can provide improved insights into the natural history of different cancers, and lead to more informed and potentially personalized treatment of cancer patients. We urge the EMA to prioritize work that seeks to understand how and when RWE may support regulatory decisions.

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)

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

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.

The ability to learn from the experience of patients through the use of real world data (RWD) generated from their clinical care has the potential to dramatically transform not only how care is delivered, but the sources of evidence that regulators can use in their evaluation of the safety and efficacy of medicines. With appropriate study design and analytical methods, Flatiron believes that fit-for-purpose, high quality RWD /RWE can support a variety of regulatory decisions and promote the shared goals of improving drug development and clinical care to ultimately benefit patients' lives. We have prioritized three recommendations (18, 10, 19) that we believe best drive toward these goals and maximize the untapped potential of RWD.

Second choice (h)

10. Develop the regulatory framework for emerging digital clinical data 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.

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Third choice (h)

19. Develop network competence and specialist collaborations to engage with big data

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.

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

> Where the EMA has prioritized the development of guidance for industry, Flatiron believes it may be beneficial to denote approximate timelines for their release.

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:

We applaud the EMA's goal of accelerating medicine development through the recommendations and actions outlined in this goal, in particular recommendation 7. We are pleased to see an emphasis on providing more opportunities for consultation with EMA throughout the drug development process, specifically through EMA's stated action of broadening efforts to expand multi-stakeholder consultation. As a data provider working in concert with life sciences companies to generate real world evidence (RWE) that could support regulatory submissions, we encourage EMA to identify and strengthen opportunities for future stakeholder engagement to help explore how and when such evidence may be appropriate for regulatory decision making. We believe that sponsors, academics, data providers, patients, professional and clinical societies, and other stakeholders can provide valuable input to further establish the scientific foundation of RWE, and ultimately advance patient care.

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**:

(Regarding recommendations 9,10, 11, and 12) We support the overall emphasis on fostering innovation in clinical trial design, including better integration of clinical care information, patient reported outcomes, and patient generated data. We further support the prioritization of novel endpoints and biomarkers, and would encourage the EMA to also explore the use of real-world endpoints in regulatory decision making, including outlining the acceptability of real-world endpoints for specific contexts of use and description of a framework for validating these endpoints.

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**:

Flatiron strongly supports EMA recommendation 18 to promote the use of high-quality RWD in regulatory decision making. We concur that further work is needed on the analytical and epidemiological methodologies needed to generate robust RWE and encourage EMA to engage with industry, academics, data vendors and patients in the development of these methodologies. Concomitant with this work, we support efforts toward creating clear regulatory guidance on the acceptability of RWE and encourage work with other international regulators to harmonize requirements and standards for using RWE, to the extent possible, perhaps under the context of the EMA/FDA's bilateral collaboration on medicines. Finally, we also believe that EMA's recommendation 19 to develop network competence in big data, including RWD/RWE, across the EU is a critical step toward facilitating access to and familiarity with, as well as the ability to analyze, these data in regulatory submissions.

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**:

(Recommendations 28-30) Flatiron agrees that pilot studies and research collaborations can lead to better understanding of evidence generated on efficacy and safety of medicines, particularly in areas of emerging regulatory science such as the use of RWD to generate RWE and the determination about when it is suitable for regulatory purposes. In addition to engagement with academics across the EU, we would encourage EMA to also prioritize work with sponsors, data vendors, patients, professional and clinical societies, and other stakeholders and to share learnings broadly. A diversity of opinions can provide valuable input to advance the regulatory science foundation, particularly as it relates to RWE, and ultimately advance patient care.

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