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Public consultation on EMA Regulatory Science to 2025

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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):

France Assos Santé

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.

In our opinion, this strategy paper tends to widen the mission of the EMA beyond its primary role as a regulator and a gate-keeper. Accelerating the development of new drugs (upstream) and speeding up patient access once medicines have received a marketing authorisation (downstream) are presented as top priorities, while the strategy is much weaker on monitoring drug safety and efficacy during the whole life-cycle of medicines. It is disconcerting that pharmacovigilance is hardly mentioned in the document, while it is a core duty of the EMA and a key priority for patients and public health.

Most strategic goals are science/technology/industry driven: as stated by Prof. Guido Rasi in the Foreword, the EMA aims to engage with emerging science and technological innovations and to create a favourable regulatory environment to support the development of increasingly complex medicines. Let's remind the primary mission of the EMA is to promote and protect human and animal health, not to support the development of medicines.

The proposed strategy reveals a shift in the evidence-collection from the pre-authorisation to the post-authorisation phase. However, the push for accelerated and conditional approvals should be better evaluated against the original purpose of those flexibilities and their downsides: a) increased potential risks for patients; b) burden of evidence-collection transferred from drug developers to other actors; c) lesser level of proof for the evaluation of the drugs' therapeutic added value.

The EMA recommends strengthening its current scientific advice activities to address a "need for earlier and more frequent dialogue". In doing so, the EMA runs the risk of appearing as a "co-developer of medicines", all the more so as the document does not cover the issues of conflict of interest between the advisory and evaluation activities of the Agency.

Advancing patient-centred access to medicines is a very relevant objective in so far as it aims at improving access of all patients to effective and safe drugs. A reflection on the context of raising inequalities due to high prices of medicines in Europe would have helped providing a more comprehensive overview of the issues at stake.

Several public health priorities are listed in the document, including shortages, which are a major concern for patients. They unfortunately appear of secondary importance compared to the promotion of innovation, while they would have deserved a stronger focus.

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

Comments on strategic goal 5 (h):

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

The concept of “regulatory science” is rather blurry and potentially very broad. We do not consider that regulatory research falls within the realm of the EMA’s mission. The Agency should rather focus on developing its scientific capacity in connection with its core duties.

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)

17. Reinforce patient relevance in evidence generation

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 lack of PROMs and relevant quality of life studies in most medicines' development plans is of great concern for patients, as it may block access to medicines that could help them live better and can lead to false assumptions. Patient organisations are available to collaborate with the EMA for the development and adoption of relevant patient-centred quality of life measurement tools.

Second choice (h)

11. Expand benefit-risk assessment and communication

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.

Improving the way benefit-risk decisions are made and communicated should be at the core of the EMA strategy. The inclusion of PROs – but also PROMs – and patient preferences shall help better reflecting patient's actual needs and expectations. It should however go hand in hand with a request for comparative RCTs whenever possible. Patients, HTA and payers need to feel confident that a new treatment works better in comparison to alternative options (if any) and this should be part of the risk-benefit assessment.

Third choice (h)

12. Invest in special populations initiatives

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.

Investing in special population initiatives to assess the effects of drugs on elderly patients, children and pregnant women is much needed, while the "areas of high unmet medical need" should be better defined and strictly circumscribed to justify adaptive development pathways.

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

YES
- Improve the pharmacovigilance system and the monitoring of approved drugs, including the swift withdrawal of inefficient and dangerous products from the market.
- Request to run comparative trials against standard of care therapies whenever possible.
- Improve the transparency of scientific advice, clinical trials protocols and results, and foster open access to data.
- Address the conflict of interest resulting from the coexistence of advisory and evaluation activities. A strong COI policy and the setting up of an ethics committee with external and independent personalities should be contemplated.

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:

ATMPs (2): We regret the very high price of ATMPs that may hinder access is not highlighted as an issue by the EMA, which only recommends the development of creative payment models.

PRIME (3): This scheme should apply in fully justified circumstances - drugs developed by SMEs to meet true unmet needs - as the monitoring of post-licensing evidence generation is often problematic, resource intensive and may expose patients to harms and risks.

Borderline products (5): Complex and borderline products will include more and more connected devices. The protection of patients' health data should thus be taken into consideration in the benefit/risk evaluation. The only concern of the Agency in evaluating such products should be patient benefit and interest.

Scientific advice (7): Early scientific advice needs to be associated with strict conflict of interest rules and increased transparency. The balance between advisory and regulatory activities of the Agency should not be altered in favor of the former.

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**:

Foster innovation in clinical trials (9): The Agency strategy should aim at improving current CT designs and defining meaningful endpoints, and not only propose to explore novel trial designs, new techniques for gathering data, the use of ‘omics’ to stratify populations, etc. Indeed, these innovations have not always proved to strengthen evidence generation and may, in some instances, increase uncertainties.

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**:

HTA & payers involvement in evidence generation (15, 16): Upstream collaboration with HTA & payers is welcome if it helps generating comparative evidence relevant to the downstream assessment of drugs' added value. Regulatory requirements could be adapted, so they meet the demands of HTA, payers and society. The pre-market phase of the development of a medicine provides a unique opportunity to generate evidence for healthcare decision making. Experience shows that this sort of evidence is unlikely to be generated after marketing authorisation.

Promote trust in the EU regulatory system (22): It is of the utmost importance to maintain European citizens' faith in the work of the EMA. The Agency should welcome and endorse constructive criticism and foster a dialogue with critical voices. It needs to fully apply the principle of transparency and make all data publicly available The EMA is a regulator defending the public interest and promoting public health. Therefore, it is the Agency's responsibility to proactively dispel any fears of regulatory capture.

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**:

Development of new antibiotics (24): We agree that new antibacterial agents are badly needed to fight AMR. Still, it is not in the EMA's mission to propose new business models and incentives.

Shortages (25): All Member States are faced with increasing shortages of medicines. We consider the EMA should play a more active role in addressing supply problems in collaboration with Member States, patient and consumer organisations, healthcare professionals and the industry. It should request enhanced transparency and better information on shortages and their causes, as well as promote the development of European management plans to limit the impact of shortages on patients.

Vaccines (26): Improved communication and complete transparency on the safety and efficiency of vaccines would certainly contribute to improve public trust.

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**:

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