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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):

European Association of Hospital Pharmacists (EAHP)

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.

EAHP welcomes the proposal by the EMA which puts forward a wide array of recommendations outlining how the agency envisions to engage with emerging science and technology. Close collaboration with stakeholders and timely access to medicines, which is currently jeopardized by the growing problem of medicines shortages across Europe, are two of the key elements mentioned in the strategy that are of relevance for EAHP.

The mission of the hospital pharmacy profession will always be connected to ensuring patients can receive the treatments they require. The strategy on the future of regulatory science is timely and essential for the preparation of the Agency's approach towards emerging science and technological innovations as well as their translation into patient access to medicines. The involvement of healthcare professional expertise, in particular in relation to pharmacovigilance and clinical trials, should be of uttermost importance for EMA's endeavour towards shaping the Strategy of the European Regulatory Network in the coming years. Hospital pharmacists are playing a crucial role in supporting present use of medicines, including taking responsibility for the governance of their safe use in the hospital sectors. As such, EAHP trusts that the hospital pharmacy profession will be kept in mind as key stakeholder as EMA further develops projects and activities linked to the implementation of the Strategy.

Regarding the strategic goals for the human medicines sector, EAHP supports the modernisation of the marketing authorisation process, in light of technological advances. This modernisation should however find a balance which on the one hand ensures that meaningful innovation reaches patients and on the other hand guarantees the safeguarding of EMA's rigorous processes. In particular the transparency of the European regulatory system for medicines should play a prominent role together with the Agency's pharmacovigilance activities.

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)

25. Promote global cooperation to anticipate and address supply challenges

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 problems caused by medicines shortages are serious, threaten the well-being of patients and have far reaching consequences for European health systems. Consequently, EAHP welcomes EMA's intention to work on the promotion of global cooperation to anticipate and address supply challenges. To minimise patient impact, all supply chain actors have the obligation and responsibility to collaborate more closely in terms of resolving the shortages problem. When it comes to medicines availability, hospital pharmacists are the key information holder inside the hospital. However, in order to fulfil this role, all supply chain actors must communicate more effectively to hospital pharmacies about likely and current shortages. Some of the deliverables of the HMA/EMA Task Force on availability of authorised medicines are trying to address shortcomings in relation to the timely access to information in case of a medicines shortage. However, EAHP believes that further efforts should be invested in the development of a comprehensive communication strategy, including the introduction of a unified European medicines identification system. Only a comprehensive communication strategy on shortages targeting all European states will ensure that all supply chain actors receive adequate information on the shortage of medicines in their countries.

Second choice (h)

24. Continue to support development of new antimicrobials and their alternatives

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.

There is, and has been over a number of years a lack of investment in the development of new antibiotics. Currently, there are only few products that are under development. Hospital pharmacists are concerned that in absence of new effective antimicrobials, there is a risk that resistance will continue to increase and that some infections may no longer be able to be treated effectively. Measures such as for instance the implementation of antibiotic stewardship teams and the use of infection prevention and control measures are contributing to lowering resistance. However, these alone are not sufficient. Therefore, EAHP welcomes the support provided under the Strategy by EMA for the development of new antimicrobials and their alternatives. In addition to fostering new antibiotics, the EMA should also consider the reinforcement of arrangements which ensure that essential medicines are maintained on the market, in particular for old antibiotics that are being utilised in new ways.

Third choice (h)

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

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.

Advanced-therapy medicinal products offer ground-breaking new opportunities for the treatment of disease and injury. These therapies have enhanced the roles of healthcare professionals in health-system decisions concerning clinical trials, selection, use, and management in hospitals. In addition to the assistance with early planning, awareness raising and identification of therapies that address unmet needs, EMA should also consider an investment into the further familiarisation of healthcare professionals with ATMPs. Training is needed to ensure that all healthcare professionals across Europe have the same education on these complex technical and scientific concepts to assist with the optimisation of patient outcomes.

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

A critical review of the implementation of the orphan drugs legislation is important to ensure that the incentives foreseen by the legislator are not abused, misused or overused to the detriment of patients.

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:

In relation to the implementation of the strategy EAHP would welcome a focus on the facilitation of the implementation of novel manufacturing technologies (point 4) and the promotion of developments in precision medicine, biomarkers and 'omics' (point 1) over the next five years.

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**:

In relation to point 9, EAHP believes that the EMA should endorse a patient-centric approach as opposed to a drug-centric approach in order to strengthen the diversity of clinical trials. Owed to the lack of clinical trial data for the older population, treatment decisions are in daily practice routinely based on medical data derived from studies of younger adults. In these situations, practitioners are left to treat patients over the age of 65 without adequate knowledge of older adults' response to medication, dosing ranges in acute and long-term use, side effect profiles, potential for accumulation in the body, and drug-drug interactions. EAHP considers that older patients, including the ones with multi-morbidity or those needing personalised treatment, are often being excluded from clinical trials unnecessarily. While obvious challenges exist in respect to their participation (including higher risk of adverse events and complications from multi-morbidity), these should not be considered insurmountable, especially in view of the need to optimise therapeutic interventions for this patient group. Linked to this EAHP also welcomes point 12 of the strategy covering the investment in special population initiatives.

Regarding, point 11 EAHP would like to note that the push for accelerated approval should not compromise patient safety. Accelerated access to medicines is sometimes needed and also supported by hospital pharmacists, but such access should remain the exception as it increases uncertainty and puts patient safety at risk. Consequently, patients need to be made fully aware of the harm-benefit ratio of these products. Clear and sufficient communication to both patients as well as to health care professionals and prescribers is in these cases of uttermost importance.

Digital technology is gaining more and more importance in for health systems. EMA's goal to foster its exploitation (point 14) is an initiative supported by EAHP. Together with the optimisation of modelling capabilities (point 13) and the development of a regulatory framework for emerging digital clinical data generation (point 10) these opportunities yield a lot of engagement possibilities for both regulators and healthcare professionals which should be fully utilised.

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**:

For EAHP it is very important that EMA maintains a high level of patient and healthcare professional involvement. In particular in relation to point 21, such an engagement could be useful, especially when promoting unbiased information to the relevant stakeholders to enable sound discussions resulting in appropriate decisions on the use of biosimilar medicines. As stewards of appropriate selection, procurement, logistics and use of medicines and key players in pharmacovigilance, hospital pharmacists are capable of and uniquely positioned to promote the appropriate utilisation of biosimilar medicines. They should therefore be considered as stakeholders when developing communication campaigns and training opportunities for non-EU regulators.

The creation of a sustainable, quality assured and flexible framework for real-world data should be fostered (point 18) in areas were the usage of such data adds value. Consequently, EMA should invest in identifying these areas. The importance of the protection of patient data needs to be one of the key considerations, wherefore stringent data protection rules should be adhered to.

Real-time availability of electronic product information is supported by EAHP. In addition to the delivery, EMA should also consider the improvement of the content, in particular of the summary of product characteristics (SmPC). The SmPC submitted by a marketing authorisation holder at the time of marketing authorisation application forms a crucial basis of information for healthcare professionals, such as hospital pharmacists, on how to use the medicinal product safely and effectively. This information is frequently being used, in particular in the hospital pharmacy for the compounding of intravenous medications. The accuracy of the gravimetric method which is applied by hospital pharmacists in such cases depends on an accurate determination of the density of the drug solution being used. Unfortunately the SmPC rarely includes this data, forcing hospital pharmacies to contact the manufacturers directly. This approach is often time consuming and the quality of the data received can vary considerably. The inclusion of data specifying the density of a drug solution in the SmPC would increase patient safety and consequently should be considered as an action linked to the delivery of real-time electronic product information.

Given the discussions on HTA at European level, EAHP welcomes EMA's engagement by contributing to HTA preparedness and downstream decision making (point 15). Efforts should be made to align this priority with the adoption and implementation of the Regulation on HTA.

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**:

As mentioned in relation to question 5, EAHP and its members are very concerned by the negative impact that medicines shortages are having on patient outcomes across Europe. The continued engagement healthcare professionals, patients and other supply chain actors to address the causes and consequences of lack of medicines' availability needs to be prioritised. This should be considered when determining the implementation priorities for the strategy. Similarly to shortages also other health threats need to be adequately addressed. EAHP consequently welcomes the EMA's intention to implement a plan, ring-fence resources and refine preparedness approaches together with the EU Network (point 23). In relation to the timely and effective communication to healthcare professionals, a coordinated approach should be taken that ensures that all Member States communicate the same information within their respective countries.

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**:

In light of the challenges posed by to the health systems which have to deal with more complex conditions, increasing overall costs, expanded use of digital technology and informatics, as well as more frequent medicines shortages, coordination and communication at EU level is crucial. EAHP consequently supports the proposal for network-lead partnerships (point 28) and increased collaboration with academia (point 29). Education and research, as outlined in Section 6 of the European Statements for Hospital Pharmacy, also plays an important role for hospital pharmacists. Due to their unique position as medicines experts, the inclusion of hospital pharmacists in research activities should be fostered.

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