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Public consultation on EMA Regulatory Science to 2025

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* Name

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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):

Federal Institute for Drugs and Medical Devices (BfArM)

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.

EMA´s strategic reflection on "Regulatory Science to 2025" is a well-structured and balanced approach addressing the most relevant up-to-date issues and challenges.
The strategic reflection proposes clear and relevant strategic goals not only relevant for EMA´s strategic direction for the next years, but also for NCAs in the EU/the entire EU regulatory network. The five strategic goals and underlying core recommendations for human medicines (also veterinary medicines; however, please note that further comments are only addressing the "human medicines" part since the BfArM is responsible for human medicines and medical device regulation only) are well taken as aim for the future.
The BfArM is committed to supporting the implementation of the recommendations so that we, as a European network, are optimally prepared for the increasingly faster development cycles, new (manufacturing) technologies (nanotechnology, etc.) and challenges (e.g. through digitization; big data; increasing Real World Evidence across the entire product lifecycle etc.) and can carry out our regulatory work based on the latest scientific findings.

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)

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

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.

Considering the fact that almost all data get digitalized now, clinical data will comprise all patient-related data including imaging data, Omics data, social media and behavioral data etc. This point seems to be centrally important; digitalization will lead to a new understanding what (clinical) data is about. These data, this information will modify the conclusions (benefit-risk) we draw at the initial authorization. If we do not want our assessments, resp. our regulatory decision making to become obsolete, we must use these data - and after all, Pharmacovigilance has been doing exactly this ever since – so we need to expand this from safety to efficacy.
Even RCTs will stay as gold-standard, complex clinical study designs in special populations (e.g. oncology; orphans) will gain further development and regulatory science based approaches for this must be available.
The HMA-EMA Big Data Task Force is a good starting point to answer the question on how to handle/use all these data in an appropriate way for regulatory decision making; concrete next steps of this Task Force based on their initial summary/roadmap are of great importance for the future.

Points 9, 10, 18 and 19 are therefore considered as a single coherent priority recommendation No.1 (and not only Point 19).

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.

Very important, in the end this is what it is all about.
If we do not develop/advance the way we make B/R decisions (rationale, consistent, transparent, predictive) and how we communicate these, they will become less meaningful for subsequent decision makers and in the end for patients.

Third choice (h)

5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

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.

A better integration of regulatory oversight of medicines and medical devices / technologies / analytics is important.

As an authority responsible for both pharmaceuticals and medical devices, we see the growing importance of medical devices/in vitro diagnostics in everyday care on the one hand, but at the same time also the questions and challenges that arise when such products are combined. It is therefore important to have a clear assessment framework that takes into account the specificities of both medicinal products and medical devices.

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

The strategy is already very comprehensive and well-balanced.
The only element that seemed to be a bit underrepresented/underprioritised in the overall structure could be the digital transformation of regulation itself.

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:

General comment to goal 1:
The main edge-cutting fields of modern medical science are addressed - like precision medicine, In Vitro Diagnostics, ATMPs, novel manufacturing technologies, nanotechnology. In order to identify – and support - new trends and developments at a very early stage, the recommended enhanced, earlier interaction with developers (via horizon scanning; PRIME promotion; Scientific Advice, external communication, etc.) is seen as an important factor.
Digital devices, which are increasingly integrated into the therapeutic landscape, and combinations of medicinal products and medical devices could be added as an important field to focus on, and also digitization and its consequences in all areas of our core work.

1 and 5: Stratfied Medicine is more and more important and entering current practice.

4 and 6: Highest quality and modern manufacturing technology are extremely important to ensure safe and effective therapy for patients.

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**:

General comment to goal 2:
Evidence generation and scientific quality of evaluation are centrally important.

The exchange with all stakeholders on the importance of and especially how to deal with new study designs and new data sources (increasingly complex study designs; new techniques for gathering data; new endpoints (e.g. patient reported outcomes based on digital devices)) is of particular importance in order to obtain meaningful evidence for benefit-risk and also HTA/pricing decisions- and thus to guarantee patients (earlier) access to high-quality and safe drugs.

The HMA-EMA Big Data Task Force is a good starting point to answer the question on how to handle Big Data in an appropriate way for regukatory decision making; concrete next steps of this Task Force based on their initial summary/roadmap are of great importance for the future.

The focus on evidence generation in specific populations/therapy situations (areas of high unmet medical need; due to demographic change in older populations/geriatrics) is also highly welcomed.

11. Benefit risk assessment is the core competence in regulation. This goal should be prioritized in importance, however, it should be considered that this B/R assessment is more and more an ongoing process in a sense of lifecycle management. Moreover, we will see more and more combination products, e. g. combination of medicinal products, borderline products of medicinal products and medical devices, etc. So benefit risk might be based on “complexer ecosystems” then on specific products.

12: The study of special populations and vulnerability in context of benefit risk evaluation is a growing need in a face of a more and more globalized world, demographic challenges, etc.
14. more and more important topic due to increasing digitization and additional use of real world data (data quality, use of algorhythms, etc.)

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**:

General comment to goal 3:
Important topics; interaction with patients as well as with HTA bodies has significantly increased and further interaction with these stakeholders (HTA/payers) is important (e.g. concerning the handling of Real World Evidence).

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**:

General comment to Goal 4:
Close cooperation with the EU network/NCAs would be highly welcomed.

Point 23: The global outreach of European regulatory work is important!
Point 24: The development of new antimicrobials is extremely important – but a lot of initiatives are on the way; so not one of the current most important activity needed by regulators.

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**:

General comment to goal 5:
Very important point to ensure that the evidence needed for regulatory framework development will be gained (from the early beginning to avoid unnecessary additional effort).
An important goal would be to communicate this need also to funding organizations in the EU to provide resources for research and development of academia-regulatory partnerships. Experiences made on national level in the EU member states can support these activities in a meaningful way.

Cooperative research on regulatory important science questions are crucial in order to develop the scientific evidence base for the development of the regulatory framework.
Develop network- led partnerships with academia to undertake fundamental research in strategic areas of regulatory science - but we would recommend to do this in a NETWORK partnership between EMA and NCAs.

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