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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

* ***Please specify:***

between 1 and 1 choices

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

Name of organisation (if applicable):

Bayer AG

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.

Bayer welcomes the possibility to comment on the EMA´s Regulatory Science to 2025.

All five strategic goals mapped out by the draft Regulatory Science Strategy address important priorities for the advance of medicines and therapeutic care in Europe. Overall we support the EMA´s 5 strategic goals.

We have followed EMA's request to highlight the goals that address the greatest needs. Our overall top three priorities are nested in each of the first three strategic goals. These top priorities are:

- Promote use of high quality RWD data in decision making
- Develop regulatory framework for emerging clinical data generation
- Contribute to HTA's preparedness and downstream decision making for innovative medicines

We continue to value and rely upon EMA's delivery of 5-year strategic plans. The process for RSS 2025 has been the most comprehensive to date including public workshop and extended consultation period. This has allowed the industry to undertake a robust approach to these comments. As such, we would appreciate if EMA continues its stakeholder engagement, including frequent status updates and outreach technology platform meetings, throughout the 5-year implementation phase of the plan.

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.

RWD is key for innovative clinical development, to speed up patient access and to support orphan designation.

Utilisation of different sources of evidence for the development and monitoring of medicines [and associated diagnostics] will support streamlined development times and foster early market access.

RWD/RWE is an integral part of the personalised medicines/personalised healthcare approach.

Second choice (h)

9. Foster innovation in clinical trials

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.

Methodologies to incorporate clinical care data sources in regulatory decision making will be supporting the overall objective of advancing use of RWD.

Engagement of all stakeholders and appropriates guidance are key to mitigate the current uncertainties on use in the Regulatory context.

Third choice (h)

15. Contribute to HTAs' preparedness and downstream decision-making for innovative medicines

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.

The EMA-EUnetHTA three-year work plan 2017 – 2020 covers scientific advice (pre and post evidence generation) and multi-stakeholder cooperation. The focus is on data generation that supports decision making by both regulators and health-technology assessment bodies. The time for this work plan is in line with the task given to EUnetHTA by the EU commission for a sustainable EU HTA cooperation beyond 2020.

It will be important for EMA to seek and continue facilitation of the ongoing scientific dialogue with HTA bodies.

New evidence type, new endpoints and specifically new product with long term effect (such as ATMP) highlight the need for multistakeholder collaboration on timely access for patients to innovative medicines in Europe.

This is the prerequisite for enabling drug developers to prospectively plan data generation in the pre- and postapproval setting

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

The strategy is clear and comprehensive. If anything, the number of elements may require significant resource investment beyond the current resource level at EMA

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:

Key priorities and why:

Overall, the recommendations in this Strategic Goal are important for Bayer to support our core business focus: advances in science and technology in medicines development.

We have focused priorities on:
o Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments
o Support developments in precision medicine, biomarkers and 'omics;
o Promote and invest in the PRIME scheme; create a more dynamic assessment environment in Europe that fosters continuos dialogue between applicant and health authority
o Facilitate the implementation of novel manufacturing technologies;
o Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products.
o A more flexible system of development advice could accelerate start of complex clinical trials in EU and could align the perspectives of various assessment bodies.

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**:

Key priorities and why

Evidence generation and analysis remains a core critical issue for medicines. Some of the recommendations proposed for this strategic goal will define the future for regulation, and we have prioritized these. Other recommendations are either seen as ongoing priorities for the EMA, which are already the focus for action; we expect these to be maintained as required.

We have focused priorities on:

- Foster innovation in clinical trials
- Develop the regulatory framework for emerging clinical data generation
- Expand benefit-risk assessment and communication
- Exploit digital technology and artificial intelligence in decision making
- Optimise capabilities in modelling and simulation and extrapolation

To foster patient focused drug development and inform benefit risk decision making, the patient perspective needs to be elicited. EMA should support the development of patient preferences research and the validation of appropriate methodologies.

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**:

Key priorities and why

Overall welcome the prioritization on initiatives to improve patient-centred access. We have prioritized recommendations which we believe hold the greatest potential to deliver this aim from the perspective of regulatory science.

We have focused our priorities on:

- Promote use of high-quality real-world data (RWD) in decision making;
- Contribute to HTA's preparedness and downstream decision making for innovative medicines;
- Deliver improved product information in electronic format (ePI) in real time.

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**:

Key priorities and why

Importance of EMA's public health mission is without question. To some extent, the relatively lower priority given to recommendations in this section reflects our view that these are not strategic goals to establish but ongoing priorities that the Agency must maintain.

Greater priority given to:

o Continue to support development of new antibacterial agents and their alternatives; Industry has advocated for collective action to address AMR. Welcome proposals on development, and critical importance of new business models although recognize not within EMA's remit to provide. Much needed proposal to work with HTA and payers to define and explain the relevance of evidence requirements for new antibacterial medicines.

o Promote global cooperation to anticipate and address supply problems; Increasingly politicised issue. Important to address the reasons for unavailability at the global level as they pertain to supply chain complexities. Some reasons, however, more related to procurement terms, and therefore important to continue to engage with health authorities on the causes of supply shortages.

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**:

Key priorities and why

Support the development of network-led partnerships with academia and pharmaceutical industry researchers to undertake fundamental research in strategic areas of regulatory science.

o Develop further platforms for scientific discourse and engagement through IMI and beyond, including to global research collaborations, which could also support global regulatory technical standards (ie in support of ICH development)

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