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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):

F. Hoffmann-La Roche Ltd

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.

We welcome the opportunity to provide feedback on the EMA's Regulatory Science to 2025. Whilst overall, we largely agree with the both EBE's and EFPIA's position, we would like to add some complementary comments, to best reflect our view as an individual company.

Overall:
- Roche appreciates and supports this initiative which covers arising topics broadly and proactively as a response to increased global competition and the need for the EU to enable innovations in the future to enable the evolution from personalised medicines to personalised healthcare.
- The number of strategic areas seems to be very broad given the current resource model at the EMA, hence prioritisation is key;
- The resource model of EMA/NCAs/HMA has to be reconsidered to be able to support the ambitious EMA's Regulatory Science to 2025 agenda, even after prioritisation;
- In a number of areas, EMA has already been highly active (e.g., under "Strategic goal 4: Addressing emerging health threats and availability/therapeutic challenges") for many years. While this should certainly remain an important objective or the future, and for the purpose of identifying additional resource needs, it may be worthwhile to separate those from "strategic areas" linked to new science and technology as well as those related to implementation of new legislation such as IVDR, MDR, CTR and GDPR;
- We would appreciate giving consideration to trying pilots on different areas such as what FDA is offering;
- Supporting innovation may also need more financial support and also an official mandate to work on policy development aspects;
- Roche supports all initiatives proposed under Strategic goal 5: "Enabling and leveraging research and innovation in regulatory science". However, since the initiatives proposed are enablers for the other strategic goals we would refrain from considering No. 5 a strategic goal in itself. The EMA's Regulatory Science to 2025 should rather focus on strategic deliverables than enablers.

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.

> We believe the EMA's Regulatory Science to 2025 should primarily focus on strategic deliverables. Enablers should not be considered a strategic goal in itself but support the other strategic goals.
>
> Top 3 priorities were agreed upon keeping in mind that the following recommendations from the Strategic 5: Enabling and leveraging research and innovation in regulatory science are key enabling factors for those priorities to become a reality.

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)

> 1. Support developments in precision medicine, biomarkers and ‘omics’

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.

Roche's vision on Personalised Healthcare (PHC): https://www.roche.com/about/priorities/personalised_healthcare.htm#A-revolution

Across Roche, we are creating meaningful value for patients and healthcare systems by leveraging meaningful data at scale (MDAS) and applying advanced analytics. Together we are generating unprecedented insights about patients, disease heterogeneity, and clinical outcomes. Our mission is utilising these insights to discover, develop and deliver highly personalised treatment plans that have the potential to significantly improve patient outcomes.

The importance of the development has been supported through numerous initiatives in the EU (e.g. Council conclusions 2015, European Parliament Resolution) and technical level (ICPerMed Initiative).

EMA, together with the European Network, has a key role to play in the establishment of a PHC ecosystem that integrates large datasets from diverse sources to generate meaningful insights, inform decision-making and drive innovation represents the most promising approach to establishing sustainable healthcare systems that deliver the best patient outcomes:
- While a growing emphasis on the building blocks of personalized healthcare - empowerment of individuals to leverage their health data, investment in systems to collect and integrate diverse datasets, and the use of RWD in healthcare decision-making - is beginning to realize tangible impact, there are many policy, regulatory and infrastructure considerations that need to be addressed to fulfill the potential of PHC;
- Stakeholders across the health sector - including government, providers, patients and industry - must establish innovative collaborative models dedicated to co-creating the policies, regulations and infrastructure to make PHC a reality.

To realise the potential of precision/ personalised medicines to evolve into personalised healthcare, we do see a strategic role of EMA in the following areas:
- Promote the use of high quality real world data in decision making (see link to Recommendation 18 which we selected as priority No.3, see below);
- Creation of an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products (Recommendation No.5) - specific reference is made to the EBE's related comments;
- Innovation in clinical trials (Recommendation No.9) - specific reference is made to the EFPIA's related comments;
- Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments - specific reference is made to the EBE's related comments.

In focusing on the above, we emphasize the need to put a strong focus on the following enablers:
- PRIME Scheme (Recommendation No. 3) - further evolution and expansion to new/extension of indications;
- Regulatory advice along the continuum (Recommendation No. 7) - specific reference is made to the EFPIA's related comments;
- HTA interface/ preparedness and downstream decision-making for innovative medicines (No. 15);
- aspect of digitalisation and Software as Medical Device (SaMD).

Roche's prioritisation of this recommendation is consistent with EBE and Roche fully supports EBE's related comments.

Second choice (h)

17. Reinforce patient relevance in evidence 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.

Roche is moving towards stronger integration of patient relevance in evidence generation for the following reasons:
- patients provide unique perspective regarding disease and treatment;
- mindsets are evolving from patients as a source of data to patients as a strategic partner in drug development
- getting patients input in early development (e.g. SMA, Huntington, Head and Neck, Breast) has been key to inform endpoint selection, study design, selection criteria, recruitment strategy;
- patients reported outcomes are used as key endpoints (e.g. I2O and CNS franchises) or supportive evidence (oncology) as part of the totality of evidence generated for the benefit/risk assessment.

With a view of fostering effective use of PROs in decision making, it will be particularly important for EMA:
- to address the lack of standardization and the perceived lack of rigor associated with “subjective data”, and varying levels of understanding of this type of evidence among reviewers;
- to foster interactions with industry and other stakeholders and provide transparency on how related data is assessed and rated;
- to consider aspects linked to digital health (tools, endpoints etc.);
- to consider early the perspectives of HTA.

Third choice (h)

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

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 concept of RWD and the process of analysing RWD to generate Real World Evidence (RWE) offers increasing potential, in particular due to:
- the greater availability and quality of electronic healthcare information (e.g. eHR cancer data following automated and manual quality control by Flatiron);
- the emergence of tools for advanced analysis of large data volumes (greater computing power, data handling and analysis techniques);
- the ability to link data from multiple sources, and broader awareness of the increasing limitations of traditional clinical trials.

Given the substantial opportunities to foster and improve medicines' development and for moving towards personalised healthcare (see comments to priority 1 on precision medicines) and the current challenges for implementing the concept more broadly, EMA needs to evolve its thinking and collaboration with other stakeholders (e.g. industry, patient organisations, HTA bodies, academia) in this area.

In particular, we expect prioritised action in related fields:
- develop network competence and specialist collaborations to engage with big data/ Implement the core recommendations emerging from the HMA-EMA Joint Big Data Taskforce (Recommendation No. 19);
- consider aspect of digitalisation and Software as Medical Device (SaMD);
- consider genomic profiling as part of RWE (registries etc.) and the related interface with the IVD framework;
- consider early the perspectives of HTA.

Roche's prioritisation of this recommendation is consistent with EFPIA and Roche fully supports EFPIA's related comments.

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

As mentioned above, we think the following aspects merit deeper consideration in the strategy under the above priorities:
- software as medical device (SaMD) in combination with medicinal products;
- genomic profiling (NGS) as part of RWD/ RWE.

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:

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**:

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**:

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**:

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