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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):

Regulatory Science Network Netherlands (RSNN).

Currently, the RSNN consists of the following members: the Association Innovative Medicines, the Medicines Evaluation Board, Utrecht University and the University of Groningen. Its secretariat and organisation is facilitated by Lygature, also acting as an honest broker.

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.

The EMA has prepared a comprehensive and ambitious strategic plan to anticipate on and prepare for the regulatory challenges ahead of us. We consider that the strategic plan foresees in significant contributions to innovating and improving regulatory aspects affecting the entire product life-cycle and its sub domains (quality, non-clinical, clinical and pharmacovigilance), societal and stakeholder interaction, and will also provide opportunities for operational optimisation of the European regulatory system by making use of emerging technologies (e.g. big data and AI, imaging, biomarkers, B/R quantification, patient-preference elicitation, etc.). However, exchange and use of scientific information with all stakeholders is currently underdeveloped in the current draft. We consider it essential for the success of the overall strategy to foster interactions and stimulate participation to contribute to the strategic programme by all stakeholders engaging in regulatory science, including in the development of the plans on how to reach the goals set out in this document. The challenges set out in the strategic document are ones we need to tackle with society as a whole to realize the potential that is contained within this strategic document.

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)

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

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.

Public private partnerships offer a unique opportunity to research regulatory challenges from all possible viewpoints in order to arrive at regulatory recommendations. Further synergy can be achieved when all stakeholders have equal seats at the table, as is the case in RSNN. The prerequisite for these interactions is that all regulatory discussions take place in the precompetitive space. RSNN is a network of experts from industry, academia, regulatory authorities, and the broader regulatory science field. RSNN offers a unique platform for stakeholders from different backgrounds to meet and discuss regulatory science as equal partners. Our mission is to advance an efficient and effective regulatory system that supports medicines development, marketing authorization, access, and appropriate use of medicines. RSNN shares and disseminates knowledge among all stakeholders and sets the agenda for further research (see e.g. our newsletter on innovating the SmPC; https://webtools.lygature.org/wp-content/uploads/2018/09/201805-RSNN_Nieuwsbrief5_A4_v0.5-MR_e-mail.pdf). The initiative was started by the Medicines Evaluation Board (MEB) and the Association Innovative Medicines. Its secretariat and organization is supported by Lygature, which previously managed the TI Pharma Escher programme. (Gispen-de Wied and Leufkens 2013). We recommend a similar approach in the EMA strategic document in order to include all (scientific) partners to contribute to the challenges that have been set. Furthermore, it is important to clearly define in this document who is identified as a partner for scientific engagement from the outset: as an editorial comment, we would propose to reword goal 28 as follows: 'Develop network-led partnerships with academia and other (scientific) partners to undertake fundamental research in strategic areas of regulatory science'

References

CC Gispen de Wied and HG Leufkens. From molecule to market access: drug regulatory science as an upcoming discipline. Eur J Pharmacol. 2013 5;719(1-3):9-15. doi: 10.1016/j.ejphar.2013.07.021.

Second choice (h)

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

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.

Within Europe, there are already networks in place engaging in regulatory science. Notably, and excluding our own network, these include the Centre for Innovation in Regulatory Science (CIRS), a UK based subsidiary of Clarivate Analytics, formerly the IP & Science business of Thomson Reuters), the Copenhagen Centre for Regulatory Science at Copenhagen University (CORS) and the European Federation for Pharmaceutical Sciences (EUFEPS) regulatory science network. We highly recommend making use of the existing networks that already have an established track record in engaging in regulatory science in order to fully leverage the potential for research and regulatory innovation. The strategic document should also outline what will be achieved as a result of the collaborative efforts. Finally, it is not clear what constitutes a network scientist and we recommend this to be as inclusive as possible.

Third choice (h)

31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders

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.

Acceptance and adoption of novel regulatory science and its consequences by all stakeholders requires intensive sharing and dissemination of knowledge. This requires a form of integrated knowledge translation to close the gap between science and practice (Graham et al. 2009). The same holds true for regulatory science. To fully realise the vision of the EMA set out in the strategic plan, a comprehensive and dedicated approach to share data across the network is recommended, and should include deliverables. Multi-stakeholder regulatory science networks, like the RSNN or CORS, could be a powerful platform for these activities.

References

ID Graham, J. Tetroe, M. Gagnon. Lost in Translation: Just Lost or Beginning to Find Our Way?. Annals of emergency Medicine Vol 54; Issue 2, 2009. pp. 313-314.

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

1. The EMA definition of regulatory science is focussed on informing regulatory decision making throughout the lifecycle of a medicine and contributes to the development of regulatory standards and tools. This is closely aligned to the definition used by the FDA (Hamburg 2011). However, this definition is limited in scope since it excludes research of the regulatory system itself (Schellekens, Moors and Leufkens 2011). We therefore propose that regulatory science is the science of developing and validating new standards and tools to evaluate and assess the benefit/risk of medicinal products, facilitating sound and transparent regulatory decision making, and also advancing knowledge of regulatory systems in general, via analysis of the frameworks used and of their effectiveness; does the system deliver what society expects and asks for? By including this crucial aspect of regulatory science, deliverables and rethinking of strategic goals can be further shaped. We underwrite the need for a high level document describing the strategy for the way forward. But this can only be reached by a subsequent detailed description on how this should be reached. It should be explicitly encouraged that all stakeholders should participate (Leufkens 2019). This should start at the onset of further developing the EMA strategic document and include the formulation of research questions, the research, analysis and dissemination of the knowledge.

2. While the strategic goals are virtually all output driven, what is missing is a reflection on the development and methodological strengthening of regulatory science itself. Indeed, our first remark drives this point home even further: there is, to date, no consensus on the definition of our chosen field of research. In order to fully capitalize on the outcome of the research programme that is being proposed, it is necessary to also focus on developing the methodology of regulatory science (e.g. Jonker et al. 2018). Every science needs an array of agreed upon principles, such as defining the research question, which theory to apply, which study design to choose or develop, how data sources are accessed and how data is extracted from them, how the analysis was performed, etc.

References

MA. Hamburg. Advancing Regulatory Science. Science 2011:Vol. 331, 6020, pp. 987. DOI: 10.1126/science.1204432

H. Schellekens, EHM Moors, HG Leufkens. Drug Regulatory Systems Must Foster Innovation. Science 2011: Vol. 332,6026, pp. 174-175. DOI: 10.1126/science.332.6026.174Jonker CJ, Kwa MSG, van den Berg HM, Hoes AW, Mol PGM. Drug Registries and Approval of Drugs: Promises, Placebo, or a Real Success? Clinical Therapeutics. 2018 May;40(5):768-773. DOI: 10.1016/j.clinthera.2018.04.005

Leufkens HG. Regulatory science: Regulation is too important to leave it to the regulators. Br J Clin Pharmacol. 2019 Apr 10. DOI: 10.1111/bcp.13917

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