Contribution ID: eab1ce3f-57cb-44e8-b9a3-7ae7db9ead01
Date: 30/06/2019 10:06:17

Public consultation on EMA Regulatory Science to 2025

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* Name

* Email

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):

Regeneron Pharmaceuticals

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.

Overall Comments on the Agency's Goals:
We commend the Agency's admirable goals in focusing on patients and on ensuring their access to innovative therapies. We believe that this document has the opportunity to increase the granularity of its recommendations and actions.
Within the EU, the perceived complexity of regulatory procedures and the time to approval of medicines, as well as the variability in the approach of the payer scheme, present challenges to the drug development and innovation paradigm. We welcome and encourage the efforts undertaken by the Agency and solicit ideas for new approaches to better enable streamlining of the regulatory processes through improved harmonisation with other regulatory authorities, as well as with the health technology assessment (HTA) procedures. Given the complexity of the aforementioned issues, it is our position that designing initiatives soliciting feedback and participation of multiple stakeholders, including the Industry, will ultimately strengthen the Agency's action plans, and benefit patients.

General Comment on Harmonizing the HTA Process:
While we would welcome new approaches to align HTA and regulatory needs, the strategic proposals suggest the EMA aims to integrate some HTA evidence requirements in the regulatory process. This may be challenging given current inconsistencies on evidence requirements across the various national HTA processes. The divergence in the HTA requirements/processes across different EU member states complicates the likelihood or extent to which this initiative could be meaningfully accomplished. We welcome the development of additional details on proposals in this regard and encourage further stakeholder engagement to 1) determine whether this initiative could lead to duplication of efforts, or 2) advise on feasible path(s) forward. More specifically, as the recipient of feedback from both payers and regulators, Industry stakeholders would likely have important insights on the challenges of the current processes, and could assist the Agency by providing input or advise on potential strategies to address or mitigate them.

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)

- 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
- 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
- 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
- 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
- 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

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.

While Regeneron understands the Agency's need to prioritise its different recommendations, we are unable to identify three core recommendations from all presented and to rank them in order of importance. Goals and core recommendations are likely to vary throughout the different drug development stages, across different stakeholders, and within different departments/functions of a given stakeholder. In addition, some of the core recommendations presented overlap in their scope, which further complicates this exercise. Regeneron would still like to highlight some of the core recommendations that we believe are particularly important in the advancement of drug development ecosystem: 'Foster innovation in clinical trials', 'Diversify and integrate the provision of regulatory advice along the development continuum' and 'Promote use of high quality real-world data (RWD) in decision-making'.

Additionally, as well as collecting feedback via a survey, Regeneron suggests further workshops with multiple representatives from different stakeholders (e.g. Academia, Industry, Patient Advocacy groups, Payers). These could serve as an opportunity for open dialogue and to collect valuable feedback on the Agency's strategic priorities and associated action plans, and would ultimately support the Agency's aim of improving patients' access to medicines.

Second choice (h)

- 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

- 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
- 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
- 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
- 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

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.

Regeneron is unable to identify three core recommendations from all presented and to rank them in order of importance, for the same reasons as described above.

Third choice (h)

- 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
- 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
- 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
- 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
- 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

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.

Regeneron is unable to identify three core recommendations from all presented and to rank them in order of importance, for the same reasons as described above.

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

One element missing in the strategy is consideration of “Brexit” and more specifically the expression of continued commitment to avoid negative repercussions of this political event on the evaluation of and timely access to medicines for all EU subjects. The disruptions associated with this event were briefly acknowledged in the report; however, considering the scale of the UK exit from the Union and potential for repercussions, continued expression of the Agency’s focus with respect to short and long-term plans to address the implications of Brexit would seem appropriate. These elements could include the EMA initiatives /projects expected to be most impacted by Brexit, the potential impact (e.g. delay of timelines, de-prioritisation of projects), and how the Agency is planning to strategically tackle these future challenges.

One additional area that could also be addressed is a re-expression of the Agency’s commitment in respect of Regulation 536/2014. There remains a significant delay in the implementation of the new Regulation due to technology challenges. The facilitation of the implementation of this regulation should remain a focus for the Agency including the support of Industry throughout the transition period. The Regulation offers the promise of enhancing Europe as a location for clinical research which also offers a means for patients to access innovative therapeutics. As such, the delivery of the Regulation and resolution of issues encountered to date would seem appropriate as strategic priorities for the Agency.

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:

Recommendation 3.1.1 ('Support developments in precision medicine, biomarkers and 'omics'). We believe the Agency's report presents an opportunity to address a more innovative approach to drug development through 2025. For example, the goals and actions currently presented would benefit from consideration of the creation/validation of new clinical endpoints, in addition to the development of new biomarkers. The development of new validated endpoints is likely to play a key role in the development and timely approval of novel therapies for serious and life-threatening diseases. By focusing resources on facilitating research of new validated clinical endpoints, the Agency would ultimately help improve patient access to and speed of development of innovative therapies.

Recommendation 3.1.4 ('Facilitate the implementation of novel manufacturing technologies'): While Regeneron recognizes the importance of some of the new manufacturing technologies discussed in this recommendation, it should be recognised that some of these innovative approaches are unlikely to be implemented within the timeframe of the strategy document (i.e. by 2025). Additionally, the Agency could elaborate on planned actions (e.g. publication of new guidelines, creation of opportunities for dialogue between experts in the field, stakeholder workshops or meetings) it intends to employ to facilitate the implementation of these new technologies. These actions would facilitate the creation and implementation of transition plans for these novel manufacturing technologies by manufacturers.

Recommendation 3.1.5 ('Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products'): This report is somewhat ambiguous when discussing horizon scanning initiatives related to drug-device combination products. While the intent is welcomed, in particular with reference to the upcoming medical device regulations (MDR), it could be helpful for stakeholders to understand some more detailed thoughts from the Agency on how this horizon scanning could be delivered (e.g. via the Business Pipeline system or some alternative proposal).

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**:

Recommendation 3.2.3 ('Develop the regulatory framework for emerging clinical data generation'): At present, this core recommendation is lacking proposals to include the Pharmaceutical/Biotechnology Industry when developing new regulatory frameworks in relation to emerging technology. By working with appropriate subject matter experts when embarking on this effort, and noting the relevant expertise that can often be found within the Industry, the common goal of ensuring timely patient access to therapies can be better delivered.

Recommendation 3.2.3 ('Develop the regulatory framework for emerging clinical data generation'): Regeneron welcomes any efforts by the Agency to engage patients and encourage them to participate in clinical trials, and not focus solely on educating and training. Given these engagement efforts, the EMA could help to improve healthcare professionals' and patients' confidence in clinical trials that use new technology, such as wearables, which would likely benefit/accelerate trial enrollment and subsequently spur drug development .

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**:

Regeneron considers goal 3 ('Advancing patient-centred access to medicines in partnership with healthcare systems') a very significant goal. Additional detail regarding implementation of this specific goal would assist Industry in fully appreciating and assessing the feasibility of the Agency's approach. As currently written, it is unclear how the Agency is proposing to include patients in the process, and best approaches to engage patients early in the development process are insufficiently detailed. At a minimum, early engagement should be one of the high-level objectives of this goal. For example, this reflection paper could include an action to engage patients on value of treatment and their perception of cost/value before HTA involvement, followed by their inclusion during HTA discussions or in the HTA process. As a first step in this regard, the Agency may refer to approaches presented by the US FDA within its Patient-Focused Drug Development (PFDD) initiative as a means to inspire EU-focused guidance on this matter. These recommendations may help the Agency develop a considered framework to advance patient-centred access to medicines. Additionally:

Recommendation 3.3.4 (Promote use of high quality real-world data (RWD) in decision-making): We commend the Agency's interest in increasing the use of RWD in drug development and regulatory processes, and we would welcome further Agency guidance on the use of RWD and its value in the drug development paradigm. We recommend that the Agency considers this topic and issue future guidance /position statements on their use in clinical trial design and drug development as a whole. New guidance from the EMA could help guide Sponsors when designing their clinical programs. Because RWD can provide an approach to minimize the number of patients required for clinical trial participation in increasingly complex clinical trials and limited patient populations, it could have the potential to help shorten trial duration and minimize the use of large trial populations, ultimately benefitting patients – one of the Agency's goals. Finally, although the recommendation is focussed on regulatory decision making, formal consideration of the utility of RWD in meeting the Agency's expectations for post-approval data generation (e.g. to fulfil specific obligations) would also be welcomed.

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**:

Recommendation 3.4.5 ('Support the development and implementation of a repurposing framework') – As a highly patient-centric company, Regeneron would like to congratulate the Agency on this recommendation. This recommendation and associated actions could benefit patients with rare diseases, and vulnerable patient groups as a means of incentivizing drug development in this space.

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**:

Regeneron commends the Agency's ambitions set forth in goal 5 ('Enabling and leveraging research and innovation in regulatory science'). Regeneron believes that this goal might be more appropriately addressed through the joint efforts of regulators, academia and Industry, in particular when we consider efforts such as horizon scanning for evolving scientific innovations. This collective expertise would enrich discussions around the current and future regulatory challenges and needs, and facilitate the development of adequate solutions to address them. Further multi-stakeholder workshops would be welcomed as a forum for such discussions.
Regeneron has no additional comments on core recommendations related to this goal.

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