Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* 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

Name of organisation (if applicable):

TLV

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.

Since the collaboration between the EMA, the HTA agencies and the payers is of importance in order to receive fast access to medicines, we find it positive that you mention increased collaboration between the agencies in several places in the document. We propose that the proposals are clarified further with examples of activities.

As an HTA agency we have experienced that the quality of the clinical data has been reduced over time since many approvals of new medicines have been based on single arm trials with few patients. We are aware of that the complexity of new products has increased over time (with for example orphan products, precision medicine and histology independent indications) with few patients in each treatment group, but anyway, we lack a strategic goal in the document that says that you are aiming for keeping the quality of the data as high as possible, encouraging the companies to make comparative clinical studies, even if we face all the challenges.

Such a goal could be as follows:
"Aim for increased or at least kept quality of evidence at approval in order to serve dowmstream decisions."

Another proposed goal:
Encourage PROM and/or PREM measurements during clinical trials in order to support evidence generation to downstream decision-makers.

The bullet about PRIME seems a bit different from the others. Most of the priorities are a way to describe the development that the pharmaceutical innovation takes but PRIME is already itself a measure to handle this development.

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)

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

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.

It is crucial for all stakeholders such as regulatory agencies, HTA bodies and payers, to work together in order to make the medicines available for the patients as early as possible.

Second choice (h)

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

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.

Since many approvals are based on limited data, due to small patient groups, there is a need to develop the use of high quality RWD.

Third choice (h)

1. Support developments in precision medicine, biomarkers and 'omics'

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.

In order to be prepared for the scarce data generated before approvals of small patient groups in precision medicine, we need to develop new methodologies for the use of data from evidence generation.

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

A proposed goal:
"Aim for increased quality of evidence at approval in order to serve dowmstream decisions"

Another proposed goal:
Encourage PROM and/or PREM measurements during clinical trials in order to support evidence generation to downstream decision-makers.

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:

2.

There is an action proposed within 3.1.2: "Support evidence generation, pertinent to downstream decision-makers" but it is not detailed how this should be done.

Proposal:
"Support evidence generation, pertinent to downstream decision-makers by increasing the communication with the HTA agencies and the payers regarding what evidence is important for them, in order to make a decision and make the medicines available for the patients at an early stage. Data extraction from registries/ hospital records/apps etc and sharing data between countries need to be simplified in collaboration with legal advisors at a European level".

3:
"Involvement of HTAs is crucial to ensure that scientific advice takes into account their evidence requirements, to facilitate decision making and patient access." but it is not detailed how this should be done.

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**:

9.

The sentence below within recommendation 9 below is of high relevance, since the HTA agencies are dependent on the data developed in the clinical trials in order to make decisions on use.

"Drive adoption of novel practices that facilitate clinical trial authorisation, GCP and HTA acceptance"

9.

The sentence below within recommendation 9 below is of extremely high relevance, since the clinical relevance of all new endpoints developed for the new therapeutic settings need to be assessed, in order to make the follow-up of the product relevant.

"Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines"

11.

"Improve communication with HTAs and payers regarding therapeutic context, comparison vs. placebo /active-control, patient perspective"

This is important in order to fulfil requirements for reimbursement. Not specified how.

12.

"Enhance multi-stakeholder advice in collaboration with patients, HCPs, payers and HTAs"

This is an important goal.

13.

"Enhance modelling and simulation and extrapolation use across the product lifecycle and leverage the outcome of EU Projects"

Of value if also the EMA would like to discuss extrapolation of efficacy over time, since many clinicians are involved at the EMA assessment.

"Promote development and international harmonisation of methods and standards via a multi-stakeholder platform"

Could be of value to have methodology workshops on this theme.

14.

within section 3.2.7:

"There is a need to develop cognitive computing tools to accelerate our ability to turn big data into meaningful scientific insight and activity. To ensure such tools are effective and appropriate for use they will need to be developed through close collaboration between multi-disciplinary scientists and computer scientists."

We propose that both scientists from regulatory and HTA agencies are involved and from payers in order to find useful data.

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**:

15:
"Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans"
We find that it is important to continue the parallell consultations in order to increase understanding between the EMA and the regulatory agencies.

Very important actions below, but not any practical solutions presented.
"Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment
Discuss with HTAs guidance and methodologies for evidence generation and review
Contribute to the identification of priorities for HTAs
Monitor the impact of decision-maker engagement through reviews of product-specific experience."

17:
"Co-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment.
Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation."

Important and useful goal.

18:
Proposed added action:
"Develop methods for creation, validation and usage of RWD"

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**:
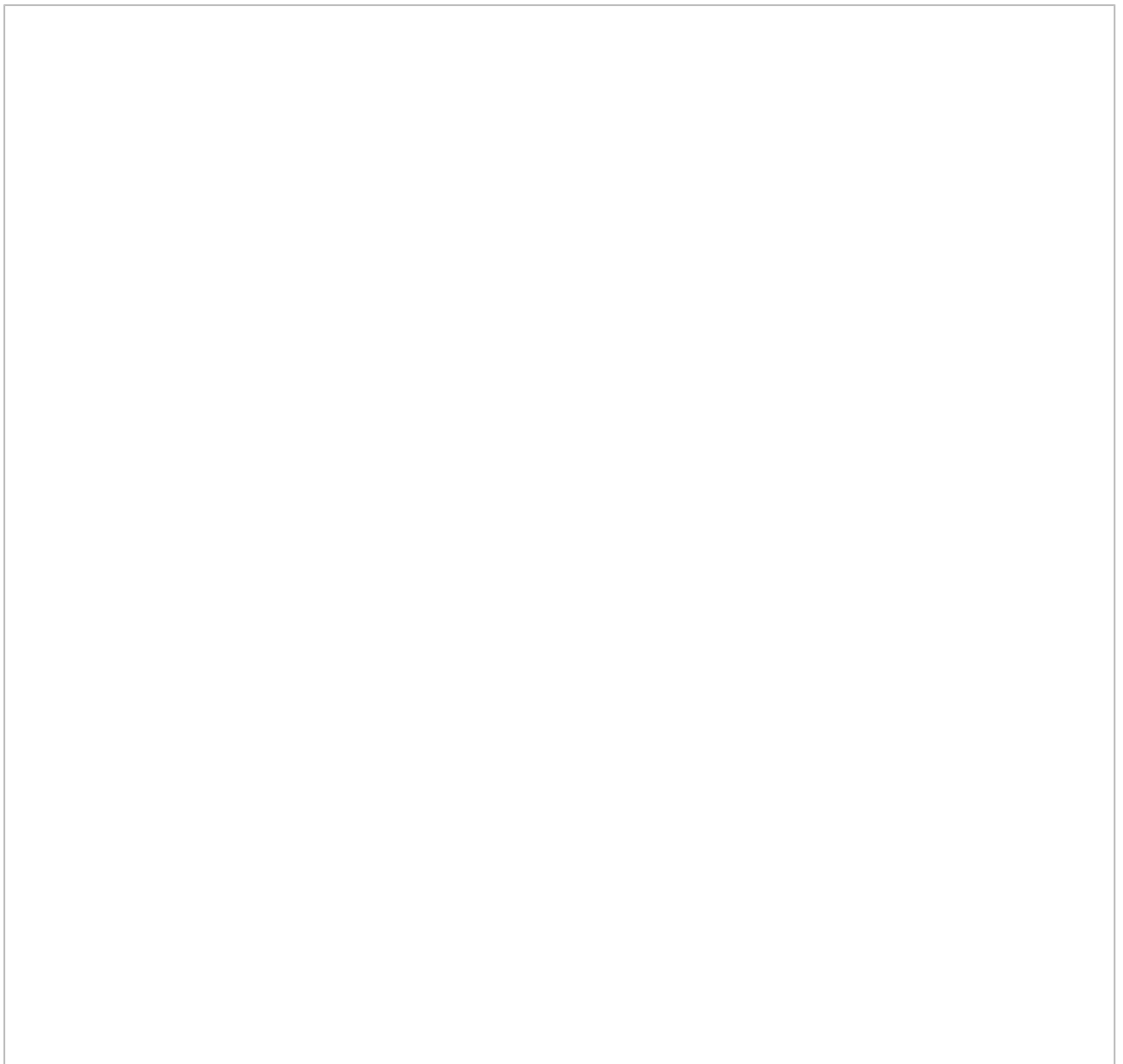
Thank you very much for completing the survey. We value your opinion and encourage you to inform others who you know would be interested.

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

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