Contribution ID: 9dc5af34-1619-4e37-a083-3facb829b8b0
Date: 27/06/2019 12:48:16

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):

National Health Care Institute (Zorginstituut Nederland)

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 Strategic Reflection includes many important objectives for the EMA. Nevertheless, there is room for improvement.
EMA states that its mission is the protection of human health. The paper seems to be focused (almost exclusively) on ways to bring new products to patients as fast as possible, implicitly assuming that all new products have an added benefit for patients and/or health systems. Many new products have no or a very limited added benefit but nevertheless come with extremely high price tags that put health systems under strain. Such a policy does not contribute to a higher level of health protection but rather endangers current levels of health protection and access.
The Strategic Reflection lacks a critical reflection on the risks of new products (referring little to pharmacovigilance as an essential part of EMA's work) and on the need for better quality clinical trials (at least randomisation).
The Strategic Reflection conveys the idea that Big Data and precision medicine will be widely operational in the period leading up to 2025. In reality, it would seem more reasonable to analyse current developments and leave flexibility for adaptation of current methodologies, in the case that these phenomena have a significant impact before 2025. In addition, the potential impact of new data generated throughout the life-cycle should be critically discussed (e.g. re-assessments, withdrawal of marketing authorisation, safety alerts, changes in SmPCs).
The current strategy would appear to extend the role of EMA far beyond that of marketing authorisation decisions and its regulatory competencies to for example, biosimilar uptake, drug shortages and data generation for down-stream decisions. However, EMA's role in these processes must be limited to information sharing and facilitating better cooperation between the different stakeholders, including the payers and the HTA bodies.

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.

Trial designs should reflect the requirements of HTA assessments.
HTA requirements must be essential for achieving MA.

Second choice (h)

16. Bridge from evaluation to access through collaboration with Payers

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.

Difficulties in obtaining reimbursement is mentioned as a factor for delayed or no market access necessitating better interaction of EMA and payers. While we welcome this concept generally, it has to be made clear that patients often do not have access due to the industries global pricing strategies where products are either not or only with a huge delay placed on the markets of poorer and small countries or excessive price expectations hinder reimbursement. « Difficulties in obtaining reimbursement » translates to health systems not being able to justify spending of large parts of finite health system resources in order to finance therapies that have not conclusively proven its effects. Therefore, increased collaboration between the regulator EMA and the payer community, as well as other public health actors, is of vital importance. Many of the proposed actions concern areas with unmet medical need. This concept has to be clearly defined in collaboration with all stakeholders. A better description of the eligible patient population and the underlying rationale are of utmost importance for payers and will improve EMA's labelling.

Third choice (h)

11. Expand benefit-risk assessment and communication

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.

A more systematic application of the benefit-risk assessment methodology is highly welcome, especially an improved communication with payers and HTAs on suitable comparators, therapeutic context and outcomes. When patient preferences are increasingly incorporated it has to be ensured that this is done in a transparent and impartial way with clear rules for conflict of interest.
Regarding communication, EMA should publicly explain its decisions and also provide insights into the benefit-risk balance, especially warning against possible harm so that patients are informed about side effects.

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

A critical evaluation:
- of EMA's current methods and potential adaptations (e.g. risk assessment/pharmacovigilance, conditional MA, withdrawing MA, orphan drug designation etc.).
- of EMA's actual role, e.g. "co-developing" and the assessment of added value should not be part of its portfolio
- concerning questions on transparency such as availability and access to data submitted by the marketing authorisation holder to the agency
- of EMA's possible role in guaranteeing market launch in all European markets, for example by implementing a "medicines tracker" to follow up if and when centrally authorised products are actually launched throughout Europe and avoid "strategic launch sequencing".

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:

1.
o validation of biomarkers has to be done by the market authorisation holder and prior to marketing authorisation.
o a proper description of methods for biomarker validation should be developed

2 and 3.
o emphasis on high unmet medical need
o develop methods for an impartial and transparent participation of all stakeholders involved throughout the life-cycle
o develop appropriate mechanisms for re-evaluation as well as withdrawing marketing authorisation when products don't live up to their expectations in the long run

3. PRIME should only be used for selected cases, which are clearly defined by stakeholders in advance, and SMEs, which lack capacities for regulatory issues. The promotion of PRIME is therefore not necessary

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**:

10.
- before developing methodologies to incorporate big data, it should be made clear under which circumstances, for which products (pharmaceuticals in vitro diagnostics vs. Borderline products) and for which purposes this kind of data can and will be used in regulatory decisions
- define which quality standards have to be fulfilled to incorporate such data into regulatory decision-making since HMA-EMA Joint Big Data Taskforce rightly identified the need for standardization and data quality as key prerequisite for data analyses
- questions related to data protection and data ownership need to be addressed as well

12.
- speedy access in populations of urgent need should not be a standalone aim without taking effectiveness/efficacy and safety into account.
- modelling and simulation enhancement should not apply to ALL products. It should be specified when these approaches will be used, foremost when they should replace clinical trials.

14.
Human intelligence is better suited to sufficiently consider ethical, societal and individual impact and to show empathy.

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. Please elicit what « contributing to HTA priority setting » is supposed to mean.

18.

- RWD should not be promoted, rather it should be emphasized that RCTs are still the gold-standard for demonstrating efficacy. In general, use of RWD can provide additional information but it is more suited in the post-authorization phase. It should thus be made clear under which circumstances and for which questions RWD can be used throughout a product's life-cycle. Issues such as standardization, data quality, registration in publicly accessible databases, reproducibility, validated statistical analyses and transparency on conflicts of interests of interested parties has to be ensured.
- Elicit issues surrounding data protection and ownership
- Highlight (financial) responsibilities for data collection and putting in place infrastructure for data exchange; incorporate specific reasons why and for what purposes evidence development is shifted into the post-marketing space; also explain strategies how these data will impact on any changes in MA such as withdrawals, re-assessment

20. The paper form of the package leaflet has to remain to ensure that also digitally- naïve patients can access the information provided.

21. Availability and uptake of biosimilar is very important for cost-containment. Guidelines on the exchangeability of biosimilars and clarification on the differences even within different batches of the same brand medicine would be appreciated.

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**:

27. Repurposing is a promising field for further support. The development of a suitable framework to support the repurposing of medicinal products is a topic of ongoing discussions within STAMP. Importantly, we need to avoid that the repurposed drugs lead to new intellectual property rights and therefore to higher prices – also for those patients using the drug for the current disease. Otherwise it will endanger accessibility. In general, a better definition of EMA's role in this regard should be provided.

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**:

See also comment on Strategic goal 5

28. It is unclear what is meant with « funders », national research centers, payers, commercial parties? Health care professionals, payers and patients should be involved. Under all circumstances, transparency has to be ensured in all research collaborations.

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