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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

- [ ] Individual company
- [x] Trade association
- [ ] SME

Name of organisation (if applicable):

Plasma Protein Therapeutics Association (PPTA)

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.

1. The overall strategy is appropriate and recommendations appear to be in line with the focus of integrating science and technology in the development of human medicinal products.
2. However, more concrete details and steps should be given, where appropriate, ensure implementation and deliverables.
3. PPTA would recommend to specifically include plasma protein therapies (PPTs) in the appropriate sections of the strategy.

See specific comments below:
PPTs represent niche medicinal products which are used to treat a variety of rare and serious medical conditions and are increasingly utilized for new and often rare disease indications.
With exception of recombinant clotting factors, PPTs are unique medicinal products as they are manufactured from plasma donated by healthy donors. In addition, and similar to vaccines, which are mentioned specifically in the document (see goal 4 addressing emerging health threats and availability/ therapeutic challenges), manufacturing of PPTs is characterised by long product life cycles, heavily regulated and highly-controlled manufacturing processes and complex supply chains based on the nature of the starting material – human donated plasma. Unlike for traditional bio- and chemical/biochemical pharmaceuticals, human donated plasma cannot be manufactured. In addition, 60-80% of the world's plasma supply for manufacturing of medicines is collected from donors in the US, hence PPTs should be separately considered when discussing availability and supply of human medicines in Europe.

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)

4. Facilitate the implementation of novel manufacturing technologies

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.

Plasma protein therapies (PPTs) could conceivably move towards point-of care manufacturing. PPTA would like to know how EMA will solicit input on the regulatory challenges regarding point-of-care manufacturing.

Second choice (h)

12. Invest in special populations initiatives

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.

Focus should be directed on specific populations (for example rare disease and paediatric populations) for unmet needs – see orphan paediatric drugs.

Support from additional stakeholders, where appropriate, should be included to ensure implementation and deliverables, such as from the EU Commission through better utilizing and integrating appropriate existing initiatives. Examples are Cross Border Initiative for rare-disease patients, European Platform for Rare Diseases Registries etc.

Third choice (h)

25. Promote global cooperation to anticipate and address supply challenges

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.

Plasma is collected and manufactured globally, most notably in the US - at the present, the USA supplies between 60-80% of plasma and plasma protein therapies (PPTs) utilized in Europe. Hence, any global cooperation should include concrete steps to streamline and harmonise regulatory standards as well as requirements for plasma and plasma-derived medicines. In the case of PPTs and other bio-pharmaceutical products, such as vaccines and immunological products, the mutual recognition of manufacturing facilities is a good example. PPTs and other biologics remain excluded from the mutual recognition agreement (MRA) between the US and the EU.

In addition, the clinical need for PPTs and for human donated plasma is likely to rise over the next decades due to a better diagnosis, treatment and new indications. Hence, PPTs should also be considered separately when addressing uncertainty regarding supply continuity for existing products/products in development and any regulatory steps needed to address availability of medicinal products in Europe (building on deliverables from the HMA/ EMA task force on availability of authorized medicines).

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

PPTA would recommend to specifically include plasma protein therapies (PPTs) in the appropriate section of the strategy (please see specific comments in the relevant sections).

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:

Comment on 1: Precision medicine:
Will EMA be prioritizing any specific 'omic ' area? As a consideration, could the EMA publish the plan by which it will assess these?

Comment on 2.Support translation of ATMPs:
Plasma protein therapies' manufacturing, along with other biologics, could also potentially be decentralized. Does the EMA have plans to address the concept of decentralized manufacturing more broadly beyond ATMPs?

Comment on 3. Promote and invest in PRIME:
Focus should be put on working on improved communication between all stakeholders (in particular regulators and industry) and shortening of time between scientific advice, clinical trials and marketing authorization application/submission.
Bridging of evaluation of medicines to access for patients will heavily rely on adequate and efficient collaboration with payers. Appropriate mechanisms would need to be strengthened, such as an implementation/ deployment plans, as well as putting in place system to assess participants (patients) and outputs.

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**:

Comment on 9. Fostering of innovation in clinical trials:
Critically assessing the clinical value of new & emerging endpoints in clinical trials will be important for plasma-derived therapies that are increasingly being utilized for new, and often rare, disease indications.

Comment on 11. Expansion of B-R assessment:
Given emerging infectious disease, like pandemic flu and pathogen X, within the expanded structured risk benefit assessment there may be value to balance overall public health metrics/strategies with patient preferences and individual data analyses, especially for non-straightforward assessments (i.e. define role /situations for these types of metrics).

Comment on 12. Investing in special population initiatives:
See comment in previous section: Focus should be directed on specific populations (for example rare disease and paediatric populations) for unmet needs – see orphan paediatric drugs. Support from additional stakeholders, where appropriate, should be included to ensure implementation and deliverables, such as from the EU Commission through better utilizing appropriate existing initiatives. Examples are Cross Border Initiative for rare-disease patients, European Platform for Rare Diseases Registries etc

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**:

Comment on 16. Bridging from evaluation to access:
Please see comments in previous section. This will heavily rely on adequate and efficient collaboration with payers. Appropriate mechanisms would need to be strengthened - mainly on the side of implementation/ deployment plans, as well as (monitoring) system to assess participants (patients) and outputs.

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**:

Comment on 23. Health threat plans:
In its evaluation of preparedness for health threats (for instance, new pathogens), PPTA recommends considering plasma protein therapies (PPTs) as part of this evaluation. As the starting material for plasma-derived PPTs is human donated material, emerging health threats can pose a potential issue for sourcing of plasma and availability of plasma-derived PPTs.

Comment on 26. Innovative approaches to development and post-authorisation monitoring of vaccines:
This is also applicable to PPTs and passive immunotherapy.

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