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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 indicate the capacity in which you are responding:***

between 1 and 3 choices

- [x] Citizen
- [ ] Patient
- [ ] Carer
- [ ] Animal owner
- [ ] Farmer

Name of organisation (if applicable):

Question 2: Which part of the proposed strategy document are you commenting upon:

- ( ) Human
- ( ) Veterinary
- (•) Both

Question 3 (human and veterinary): 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.

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

In principle, all aspects to ensure modern state of the art regulatory approaches are touched. Two major problems being major hurdles for quick licensing should be adressed in more detail: the cumbersomes procedures for scientific advice and the long pre- and post-meeting administrative procedures. Fast track licensing should preferably reduce these waste of time instead only shortening the time for scientific assessment.
The need to be pro-active in unexpected emergncy situations (eg. Ebola epidemics) should be addressed in a more condensed way. Existing procedures might be usefull but are scatterd like a steeple-chase run. These should be summarized in a comprehensive way to give guidance and quick answers for developers.

But on top of these, cross-fertilization between vet and human approaches would help developing more quickly medicinal products (vaccines in particular) in emergency situations. In practice, many emerging diseases have an animal reservoir, likely to be immunized in urgency and human infected subjects, likely to benefit from vaccine protection. In practice, both humans and animals are facing the same micro-organism and commonalities between approaches should be more closely inter-related for the benefit of both.

Question 4 (human and veterinary): Do you consider the strategic goals appropriate?

Strategic goal 1: Catalysing the integration of science and technology in medicines development (h & v)

- ( ) Yes
- (•) No

Comments on strategic goal 1 (h & v):

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

The approach to revisit the GMP requirements and to revise them in the light of modern manufacture technologies and in particular small product production and SME sis highly welcomed. In addition a better harmonized inspection made by multinational inspection teams is regarded as necessary. In addition GMP inspectors should be accompanied by assessors and OMCL members.
The outcome of such inspections could be (partly) common to human and vet products in order to avoid duplicating visits when a common platform is used for production (for instance vaccines)

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations (h & v)

- ○ Yes
- ◉ No

Comments on strategic goal 2 (h & v):

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.
Please remember to specify if a particular comment relates specifically to the human or veterinary part.

The increased attention to 3R methods is welcomed. These are considered by CEPI as ethically mandatory to gain support. This way of thinking is thought to become the rule, if not yet in force. Further the animal welfare is also an ethical requirement considered to be a pre-requisite for any animal investigation, as for instance Non-Human_Primates challenge studies.

The plan to foster the Benefit-risk assessment is welcomed but should not lead to increased requirements on quality and safety of products

In the case of a major threat for human and/or animal health, regulatory procedure should be in place to deploy quickly production of vaccines, antibodies and medicines needed for treatment and prophylaxis. (see comments above)
Platform technologies will play a major role in the surge production schemes, where platform means:

Definitions
‘‘Platform master file’ means a stand-alone part of the marketing authorisation application dossier for an immunological/biological veterinary medicinal product, which contains relevant information on quality, safety and efficacy concerning the platform technology, which are part of this veterinary medicinal product. The stand-alone part may be common to one or more immunological/biological veterinary medicinal products platforms.

‘Platform technology’ means the technology, the backbone or the vector for the production or presentation of epitopes, antigens (or biological substance) for treating or preventing infectious diseases.

The principles of Platform Master File (PfMF)
A PfMF is that part of a vaccine Marketing Authorisation Application (MAA) which describes the platform technology.
The same approved PfMF can be used for formulating monovalent and/or combined vaccines of a given manufacturer. Then the PfMF certificate issued by the EMA to the Applicant, will be valid for all the combinations it was approved for or will be extended.

Approved platforms / vectors used in licensing for exceptional circumstances:
- no additional requirements for quality
- definition of key requirements on safety and efficacy.

Non-approved platforms / vectors used in licensing for exceptional circumstances:
- definition of key requirements on quality, safety and efficacy

The way to reach the strategic goals as described is supported. Concerning the facilitation of licensing and reaction to emergency tthreats see comments to goal 1.
To be noted: there is an urgent need for training of assessors on Benefit-Risk assessment, safety of new technologies and existing GMO derived products as well as trust in non-animal tests intended to replace currently required in vivo tests.
In this respect, efforts should be made to harmonize the EU definition of GMO in the medical (not agricultural) world. Currently approaches differ from one to the other member state, hence the expert opinions.
The same comment applies to a number of new technologies, in particular in the field of nano-technologies

Strategic goal 3: Advancing patient-centred access to medicines in partnership with healthcare systems (h-only)

- ○ Yes
- ◉ No

Comments on strategic goal 3 (h):

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

The way to reach the strategic goals as described is supported.
The way to reach the strategic goals as described is supported. Concerning the facilitation of licensing and reaction to emergency tthreats see comments to goal 1.and 2. The approach for a differentiated approach on Benefit –Risk assessment depending on the target use of vaccines (regular versus special conditions for emergency) is highly welcomed. The facilitate this approach, a revision on the current scientific requirements is necessary, either to delete too high requirements for regular licensing as well as to define reduced requirements for minor market-minor use products and emergency licensing.
A specific regulatory status for stockpiled vaccines (licensed or close-to-end but awaiting the conduct of a confirmatory Phase 3 trial only possible during an outbreak) should be considered.

Strategic goal 4 (human) / 3 (veterinary): Addressing emerging health threats and availability /therapeutic challenges (h & v)

- ○ Yes
- ◉ No

Comments on strategic goal 4 (h) / 3 (v):

Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

The way to reach the strategic goals as described is supported. Concerning the facilitation of licensing and reaction to emergency tthreats see comments to goal 1.

In principle, it is welcomed that all scientific knowledge should be accessed to enhance the preparedness to new developments and new diseases. The exclusive concentration on academic partners is regarded as not appropriate. Based on the experience of CEPI, the academic world is not aware of the approaches manufacturer are obliged due to provisions on manufacture and licensing. Therefore such cooperation should be extended to manufacturers as PPPs.
(see also similar extended common above)

Strategic goal 5 (human) / 4 (veterinary): Enabling and leveraging research and innovation in regulatory science (h & v)

- ○ Yes
- ◉ No

Comments on strategic goal 5 (h) / 4 (v):

Please note you will be asked to comment on the core recommendations and underlying actions in the

subsequent questions.

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

In principle, it is welcomed that all scientific knowledge should be accessed to enhance the preparedness to new developments and new diseases. The exclusive concentration on academic partners is regarded as not appropriate. Based on the experience of CEPI, the academic world is not sufficiently aware of the approaches manufacturer are obliged to due to provisions on manufacture and licensing. Therefore this cooperation should be extended to manufacturers as PPPs.
Further some other players (like CEPI, MSF and even WHO with Avaref) should also be considered as communication partners, using both their expertise and channels to convey scientific knowledge and regulatory awareness.
In the case of a major threat for human and/or animal health, regulatory procedure should be in place to deploy quickly production of vaccines, antibodies and medicines needed for treatment and prophylaxis.
Platform technologies will play a major role in the surge production schemes, where platform means:
Definitions
''Platform master file' means a stand-alone part of the marketing authorisation application dossier for an immunological/biological veterinary medicinal product, which contains relevant information on quality, safety and efficacy concerning the platform technology, which are part of this veterinary medicinal product. The stand-alone part may be common to one or more immunological/biological veterinary medicinal products platforms.

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Approved platforms / vectors used in licensing for exceptional circumstances:
- no additional requirements for quality
- definition of key requirements on safety and efficacy.

Non-approved platforms / vectors used in licensing for exceptional circumstances:
- definition of key requirements on quality, safety and efficacy

There is an integrated approach for vaccines and diagnostics missing. In particular for DIVA strategies, the licensing of corresponding diagnostics is needed.

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'

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.

The regulatory framework focuses mainly on classical vaccines. The door opener included in Art xxx of the new regulation allows the setting of requirements for innovative vaccines and sera as well as for other novel therapies, minor market-minor use and vaccines and sera for use in emergency situations. There is currently an unique opportunity to adapt the scientific requirements to state of the art development and manufacture of IMPs.

Second choice (h)

4. Facilitate the implementation of novel manufacturing technologies

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.

Training of all stakeholder on new technologies, benefit-risk assessment and non-animal methods will increase to transfer efforts being necessary when new developments are transferred from research to industrial manufacture and testing. Regulators need to have in dephts view to manufacture facilities and QC controls.

There is an urgent need to revise the requirements on animal tests in preclinical and final product testing. Most of the tests are empirically developed and do not reflect the current knowledge on in silico evaluation and modern quality of production and the purity of substances used medicines production. The acceptance for 3R approaches needs to be increased.

Third choice (h)

13. Optimise capabilities in modelling and simulation and extrapolation

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.

See choice 1, since point 3 to be considered in conjunction

Question 5 (veterinary): 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 (v)

Please note that veterinary goals start at no.32

32. Transform the regulatory framework for innovative veterinary medicines

1st choice (v): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

The regulatory framework focuses mainly on classical vaccines. The door opener included in Art xxx of the new regulation allows the setting of requirements for innovative vaccines and sera as well as for other novel therapies, minor market-minor use and vaccines and sera for use in emergency situations. There is currently an unique opportunity to adapt the scientific requirements to state of the art development and manufacture of IMPs.

Second choice (v)

Please note that veterinary goals start at no.32

37. Collaborate with stakeholders to modernise veterinary pharmacoepidemiology and pharmacovigilance

2nd choice (v): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

The need for additional veterinary vaccines and human vaccines for rare diseases (travellers, for instance) in particular those where the return of investment is low is underlined. All efforts from EMA to strengthen development and marketing of these vaccines is highly supported.

Third choice (v)

Please note that veterinary goals start at no.32

39. Develop new approaches to improve the benefit-risk assessment of veterinary medicinal products

3rd choice (v): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

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

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Not really missing, but with too low prioritization: 3Rs and the deletion of lab animal testing in pre-clinical testing
Further, it is not clear under item 23 what is included in 'EMA to reinforce Health Threat Plans". The premises or even pre-requisite to elaborate have plans is probably to dispose of licensed medicines, in particular vaccines and specific antibodies.
A real strategy to facilitate the development (hence giving some predictability more than simple guidance to a project) is even more important in this field (lacking often alternatives, thus facing an unmet medical need) than any other.

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**:

Question 7 (veterinary): 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 (v)

	Very important	Important	Moderately important	Less important	Not important

32. Transform the regulatory framework for innovative veterinary medicines	○	◉	○	○	○
33. Reinforce and further embed application of the 3Rs principles	○	○	○	○	◉
34. Facilitate implementation of novel manufacturing models	○	○	○	○	◉

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 (v)

	Very important	Important	Moderately important	Less important	Not important
35. Update Environmental Risk Assessments in line with the latest scientific knowledge	○	○	◉	○	○
36. Apply the latest scientific principles to the assessment of the safety of residues of veterinary medicines	○	○	○	◉	○
37. Collaborate with stakeholders to modernise veterinary pharmacoepidemiology and pharmacovigilance	○	○	○	◉	○
38. Develop new and improved communication and engagement channels and methods to reach out to stakeholders	○	○	○	○	◉
39. Develop new approaches to improve the benefit-risk assessment of veterinary medicinal products	○	◉	○	○	○

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: Addressing emerging health threats and availability/therapeutic challenges (v)

	Very important	Important	Moderately important	Less important	Not important
40. Continue to promote the responsible use of antimicrobials and their alternatives	○	○	○	◉	○

41. Coordinate Network activities to improve data collection on antimicrobial use in animals	○	○	○	◉	○
42. Engage with stakeholders to minimise the risks of antiparasitic resistance	○	○	○	◉	○
43. Promote and support development of veterinary vaccines	◉	○	○	○	○

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: Enabling and leveraging research and innovation in regulatory science (v)

	Very important	Important	Moderately important	Less important	Not important
44. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science	○	◉	○	○	○
45. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions	○	○	○	○	○
46. Identify and enable access to the best expertise across Europe and internationally	○	◉	○	○	○
47. 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