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Public consultation on EMA Regulatory Science to 2025

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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: Press/media/NGO/Not-for profit organisation/other scientific organisations/policy maker, etc.

other scientific organisation supporting PPPs/NGO/Non-Profit
IABS is the only nonprofit organization dedicated solely to addressing key issues in regulatory science that underpin approvals for vaccines and biopharmaceuticals (human and animal) worldwide.

Name of organisation (if applicable):

IABS-EU

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 cumbersome procedures for scientific advice and the long pre- and post-meeting administrative procedures. Fast track licensing should preferably reduce the administrative time instead of only shorten the time for scientific assessment.
The administrative and scientific preparedness for emergency threats and new products including those based on GMO derived substances and nanomaterial needs to be fostered.

A common human and vet strategy on the approach for non-animal methods and the implementation in licensing procedures is needed. Lessons learnt from IMI VAC2VAC project: there are different approaches and requirements for validation and introduction of new methods in the testing of medicines. The hurdles to replace the use of animals in both quality control and safety testing should be lowered and harmonised on a lower level.
Concerning cooperation with other bodies, a strong cooperation with European Pharmacopoeia, WHO (h) and OIE (v) is regarded as necessary to avoid diverging requirements.

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.

human:
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 harmonised inspection made by multinational inspection teams is regarded as necessary. In addition GMP inspectors should be accompanied by assessors and OMCL members. It should be stressed that GMP should be considered as assistance to quality and not as an additional hurdle which slow down the production and increase the cost of good. Recent experience with national inspection after implementation of the GMP for ATMP guidelines has shown how reluctant is the inspection sector to help and assist SME in the installation of their manufacturing facilities and how inspectors are prone to impose approaches which are not proportionate to the risk identified. Another recent example deals with the finalization of the revision of annex 1 (sterile products) which will impose progressively the so-called “restricted access barrier system” with obvious impact on the cost and slowing down of the production yield whereas in the same time more flexibility and preparedness is expected from the industry for quick production of huge quantity of vaccines in case of pandemic or in case of supply shortage. There is here a dilemma to solve in terms of objectives for public health and implementation of the zero-risk culture and precautionary principle.

veterinary in addition to the comments made.:
There is an integrated approach for vaccines and diagnostics missing. In particular for DIVA strategies, the licensing of corresponding diagnostics is needed. To illustrate this, the Conclusions and recommendations of the latest IABS meeting on veterinary diagnostics is added.

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. See also the comments above.

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 realise quick 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 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

veterinary in addition to the comments made:
The way to reach the strategic goals as described is supported. Concerning the facilitation of licensing and reaction to emergency threats 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. There is a real need to overcome the reluctance of assessors to accept BRA and not rely on zero risk strategies. The application of GMO derived products should strictly rely on the current legal definitions (living organisms in which genetic information is inserted)

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.

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.

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

Veterinary:
The way to reach the strategic goals as described is supported. Concerning the facilitation of licensing and reaction to emergency threats 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 MUMS and special conditions for emergency) is highly welcomed. To 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:

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 the IMI projects VAC2VAC and ZAPI, the academic world is not aware of the approaches manufacturer are obliged to address, in addition to the purely scientific and academic aspects, due to provisions on manufacture(see comments on GMP above) and licensing. Therefore these cooperations should be extended to manufacturers as Public Private Partnerships (PPPs)

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)

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.

Most of these recommendations are attractive and valid. However recommendation #31 summarizes and gathers very valuable objectives. Indeed, 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 depths consideration the issues of manufacturing facilities and QC controls (and not only a blocking GMP approach).

Second choice (h)

8. Leverage novel non-clinical models and 3Rs

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.

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 and variation procedures for implementing new alternative tests duly validated should be facilitated.

Third choice (h)

26. Support innovative approaches to the development and post-authorisation monitoring of vaccines

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.

Increase of availability of vaccines against infectious diseases will reduce the need for antimicrobials and will be a good contribution to one health

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 4, 25 ff and 43 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 IVMPs. Again, the need for cooperation with OIE and Ph.Eur on harmonized requirements is stressed.

Second choice (v)

Please note that veterinary goals start at no.32

33. Reinforce and further embed application of the 3Rs principles

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 deletion of in vivo tests for final product testing is highly supported. To ensure the success of EMA's efforts it is of utmost importance that these efforts are synchronised with Ph.Eur. and OIE to avoid divergent requirements.
The training of assessors to overcome the reluctance to accept new methods and to end the trust in in vivo potency tests

Third choice (v)

Please note that veterinary goals start at no.32

43. Promote and support development of veterinary vaccines

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.

The need for additional veterinary vaccines 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. The use a large variety of vaccines will highly contribute to reduce the use of antiinfectives in food producing animals.

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.

Human and veterinary:
To reach the ambitious goals the interaction with manufacturers is needed. The use of the technical and scientific know how located at the manufacturers is not sufficiently considered. More PPP should be encouraged. If one considers the benefit of the (V) ICH structure, where both regulators and industry were discussing technical issues, each bringing into the discussion their experience and expertise, this is certainly this model, in the regulatory science approach, which should be encouraged for the elaboration of future guidelines.

Human:
Not really missing, but with too low priorisation or low visibility: 3Rs and the deletion of lab animal testing in pre-clinical testing and platform technology particularly for emerging disease or health threat.

Veterinary:
Requirements for licensing in vitro Diagnostics. To strengthen this remark, the conclusions and recommendations of the latest IABS meeting on Diagnostics in the Veterinary Field: The Role in Health Surveillance and Disease Identification, May 15-17, 2019, Wiesbaden, Germany. The Conclusions and recommendations can be foung on the IABS website https://www.iabs.org/index.php/documents/conferences/2019/diagnostics-in-the-veterinary-field/conclusions-recommendations

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 7:
The hurdles to access scientific advices need to be lowered particular to quick dates for discussion and deletion of fees. There is an urgent need to increase human resources for this approach. The PRIME approach may be supportive and should be expanded.

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**:

Recommendation 15 + 16:
The approach is welcomed but it is very likely (although unfortunate) that the EMA efforts will have no effect on political decisions in this field

Recommendation 18 :
At time of licensing the amount of statistical relevant RWDs may be low. For decision making process RWDs should be combined with BRAs.

Recommendation 21 :
Whereas we are dealing here with a strategic plan to promote innovation, it is not pertinent to mention biosimilars amonst the “innovation tools” !! In addition, the promotion and uptake issue”is not within the remit of EMA. EMA has to ensure the quality, safety and efficacy of biosimiliars at the time of registration, not their availability nor to promote their “uptake” when one considers that regarding the “uptake” there is an unresolved issue on the possible negative impact of substitution policy, as the scientific evidence of “interchangeability” is not a scientific parameter requested in the MA dossier for a biosimilar.. Promotion and uptake are in the remit of national policy…

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**:

Recommendations 24 :
EMA has to overcome the no-risk policies for new products and strengthen the BRAs as most pragmatic approach.

Recommendation 25 :
Global supply is dependent on money : the manufacturers deliver to the markets where the highest prices are paid. It is questioned how EMA can influence this process. Regions need to be independent on their decisions on the level of their health systems. EMA can contribute to the discussions,whereas the decisions will be made on another (political) level. One major contribution from EMA regarding supply challenges deals with the inspection policy and the best way inspection sector could “help” manufacturers to address specific production problems to avoid shortage and not contributing to “block” the system by more and more stringent (and dispropionate) GMP obligations which, by the end, lead the industry to leave Europe…

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**:

Recommendation 28 and 31:
As already mentioned the way from academic research to industrial produced final products is long. Academics are often not aware of the requirements on e.g. GMP, validation and other obligations, manufacturers are obliged to. Dialogue should be encouraged between academia, regulators but also with developers and manufacturers so as to bring together the necessary scientific, regulatory but also practical and industrial considerations.

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**:

Recommendation 32-34
As already mentioned training of assessors and politicians is of high importance to increase acceptance of new products, techniques, non-animal tests of final batches etc.

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**:

Recommendation 44:
As already mentioned the way from academic research to industrial produced final products is long. Academics are often not aware of the requirements on e.g. GMP, validation and other obligations, manufacturers are obliged to.

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