Contribution ID: a41e855b-eb3c-4ef5-8edb-82db19ffb9f1
Date: 28/06/2019 16:42:24

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

Name of organisation (if applicable):

Fondazione per la Ricerca Farmacologica Gianni Benzi onlus

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.

Fondazione per la Ricerca Farmacologica Gianni Benzi onlus fully acknowledges the proposed EMA strategy.

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

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

- ◉ Yes
- ○ No

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

- ◉ Yes
- ○ No

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

- ◉ Yes
- ○ No

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

- (●) 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)

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

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.

To strenghten regulatory knowledge of academia and scientists results of paramount importance, given the increasing role of not-for-profit institutions in the R&D process of drugs.
In particular, the Benzi Foundation fully acknowledges the aim to network scientists and academia to collaborate in exploring specific, evolving regulatory questions in order to develop the skills and tools that the network needs to respond and to deliver tangible impact through translation of this applied research into new drug products and regulatory tools.

Second choice (h)

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

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

Nowadays high-quality real world data are more and more needed for regulatory purposes.
In particular, the Benzi Foundation fully acknowledges the need for analytical and epidemiological methodologies to deliver robust evidence, given the often heterogeneous nature of the data sources, as well as for ensuring privacy and data security.

Third choice (h)

16. Bridge from evaluation to access through collaboration with Payers

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.

Too often - in many European countries - patients have to wait a long period of time before the medicines, approved by the European Commission on the basis of the CHMP positive opinion, are really available. This is particularly relevant for high therapeutic needs. Therefore, the EMA should intervene notifying any possible need for urgent procedure to speed up the negotiation-price-reimbursement procedure right after the European Marketing Authorization on the basis of the severity of disease and lack of alternative valid therapies.
This should be also indicated in the EPAR.

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 field of veterinary medicines has very different needs compared to human medicines.
The market is considerably smaller and fragmented as it covers numerous different species. Differences exist among countries and regions.
Significant investments are required to extend marketing authorisations to cover other species or indications. It is necessary to take into account these differences.
It will be necessary to ensure that the new regulatory environment is applied in a timely manner. To this end, priority to investments in new research for innovative drugs at national and European level should be provided through specific programs.

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.

Pharmacovigilance is critical to enabling innovation to reach the market.
The institution of a pharmacovigilance network and robust system will provide better tools to constantly monitor the benefits and risks of veterinary medicines, a clear allocation of roles and responsibilities and transparency.
This will strengthen the benefit risk management of medicines on the European market.

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.

Uses of veterinary medicinal products have multiple risks, e.g. risks for treated animal, risks for humans and risks for environment.
A standardized methodology for the benefit-risk assessment should be developed, considering the scientific and policy development of each Member State.
The benefit-risk evaluation must include the activities to improve the responsible use of antimicrobials / antiparasitic drugs and their alternatives and to promote the development of veterinary vaccines, that play a major role in protecting animal health by preventing and controlling serious epizootic diseases. They also have an impact on human health by ensuring safe food supplies and preventing animal-to-human transmission of infectious diseases.

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.

With regard to human strategy, the role of academic and other non-industrial people engaged in big data and real-world data should be more specifically addressed as well as the need for properly training and networking them in the regulatory environment.
This is because regulatory actions should be undertaken to allow the use of real-world data for regulatory purposes, including the MA, post-marketing assessment and other regulatory procedures, including orphan designations and PIPs.
Nowadays, it is well acknowledged that big data are a real value for scientific research and initiatives aiming to promote the secondary use of existing data are ongoing. The concept of sharing and merging data coming from different sources to generate evidence for decision-making process has already been pointed out during a 2014 EMA workshop on Big data and is still under discussion. Dyke et al describe the era of the “learning healthcare” where every data sharing process may contribute to scientific research.
A large amount of data is generated from clinical trials, from clinical practices and from patients and may be used to derive evidence within regulatory procedures.
Furthermore, the role and involvement of EMA and other EU and national institutions should be strenghtened and clarified in the public and private funded projects, given the increasing non profit nature of regulatory procedures and applicants.
Importantly, the need for proper and sufficient data to support decision-making is even more true for small populations. This should be focused more specifically.

Finally, too often - in many European countries - patients have to wait a long period of time before the medicines, approved by the European Commission on the basis of the CHMP positive opinion, are really available. This is particularly relevant for high therapeutic needs. Therefore, the EMA should intervene notifying any possible need for urgent procedure to speed up the negotiation-price-reimbursement procedure right after the European Marketing Authorization on the basis of the severity of disease and lack of alternative valid therapies.

With regards to veterinary medicines, generally, the strategy should better consider the need for considering the differences existing among countries and regions, including those dealing with EU and non-EU territories.

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 n 1: Enhance early engagement with novel biomarker developers to facilitate regulatory qualification is fully supported.
Recommendation n 5: specific guidance to perform preclinical and clinical studies should be provided.
Recommendation n 6: guidance and collaboration with device regulators and notified bodies results 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**:

The need for improved evidence generation is even more true for small populations, for which the need for proper and sufficient data to support decision-making is high.
Recommendation n 9: this field, harmonisation of procedures, such as clinical trial authorisation and HTA acceptance, still results a need to really improve clinical trials.
Recommendation n 14: Digital Technology and artificial intelligence is becoming increasingly fundamental to generate knowledge for medical decision support.
In particular, the Benzi Foundation fully supports initiatives to exploit digital technology and artificial intelligence and the development of tools to accelerate our ability to turn big data into meaningful scientific insight and activity. We believe that this will allow to understand how such data and analysis can be used to support regulatory decision-making.

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 18: regulatory actions should be undertaken to allow the use of real-world data for regulatory purposes, including the MA, post-marketing assessment and other regulatory procedures, including orphan designations and PIPs.

Recommendation 19: We fully endorse that regulators need to collaborate with relevant specialists to develop a deep understanding of the data, understand how it may be presented and how it should be analysed, and create guidelines on standards and validation to ensure it is robust enough for regulatory decision-making.
Importantly, stakeholders engaged in big data and real-world data include academic and other non-industrial people. they should be trained and more strictly connected in the regulatory environment.

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**:

Recommendation 23: the role and involvement of EMA and other EU and national institutions in the coordination of scientific and regulatory activities within the EU network, should be strenghtened and clarified in the public and private funded project.

Recommendation 24: Regulatory guidance and support of alternative/innovative approaches to new antibacterials development and prevention and treatment of infections should be provided both within and outside the EU, given the impact of migrations.

Recommendation 25: The unavailability of medicinal products in the EU is still affected by national issues and significant differences among EU countries still exists.

Recommendation 27: The support to development and implementation of a framework for repurposing medicines is strictly connected with the use of real-world data for regulatory purposes. these issues should be also considered together.

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**:

The Benzi Foundation fully endorse the development of the existing interaction between the EU regulatory network and academia.

Recommendation 29: Ring-fence EMA funding to address rapidly-emerging regulatory science research questions is to strenghten regulatory knowledge of academia and scientists.

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.

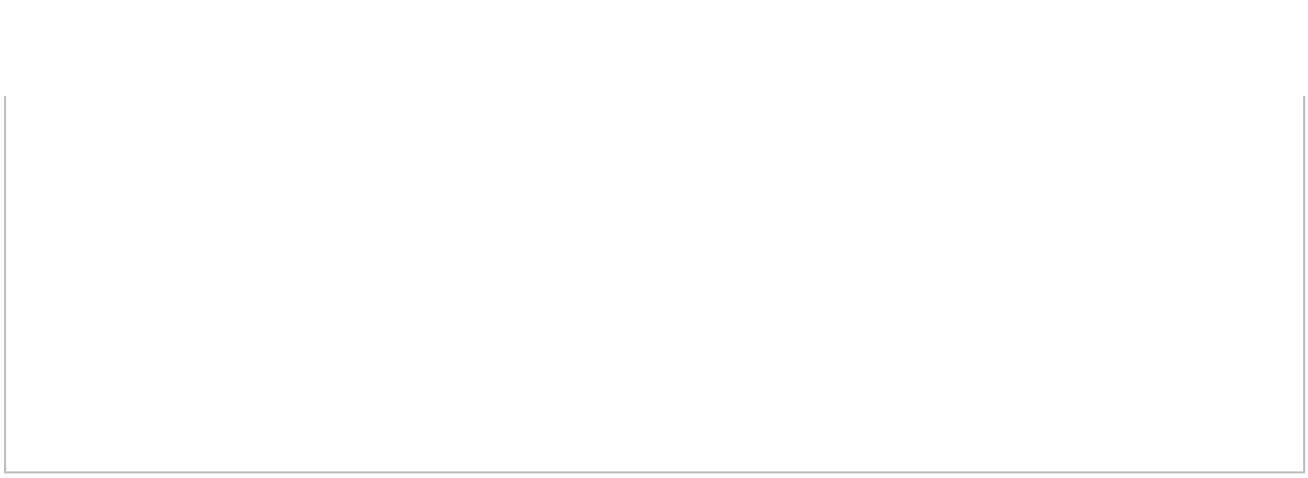
Should you wish to comment on any of the core recommendations (and their underlying actions) there is an option to do so.

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 33. to implement the application of the 3Rs as necessary to support scientific advancements in the development of alternative approaches.



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**:

Recommendation 35: The cascade prescription could be a disincentive for companies because, if the medicine for human use is considered a possible veterinary medicine authorized for other species or another Member State, the company may not be interested in developing innovative medicines for animals. Furthermore, the use of a non well-designed, formulated and tested medicinal product for a animal specie may result in both pharmacokinetic and pharmacodynamic changes, based on differences related to the formulation and anatomical, physiological and biochemical characteristics of the animal).

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**:

Recommendation 40: It is necessary increasing control activities on production, distribution and retail sales taking into account the still fragmented realities.

Recommendation 43: veterinary vaccines play a major role in protecting animal health by preventing and controlling serious epizootic diseases. They also have an impact on human health by ensuring safe food supplies and preventing animal-to-human transmission of infectious diseases. Veterinary vaccines can be an efficient tool in reducing the need for using antibiotics in animals, thereby contributing to the fight against antimicrobial resistance.

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 47: An interdisciplinary collaboration should be considered as well.

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