# Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

**\* Name**

**\* Email**



## Introduction

The purpose of this public consultation is to seek views from EMA's stakeholders, partners and the general public on EMA's proposed strategy on Regulatory Science to 2025 and whether it meets stakeholders' needs. By highlighting where stakeholders see the need as greatest, you have the opportunity to jointly shape a vision for regulatory science that will in turn feed into the wider EU network strategy in the period 2020-25.

The views being sought on the proposed strategy refer both to the extent and nature of the broader strategic goals and core recommendations. We also seek your views on whether the specific underlying actions proposed are the most appropriate to achieve these goals.

The questionnaire will remain open until June 30, 2019. In case of any queries, please contact: RegulatoryScience2025@ema.europa.eu.

# Completing the questionnaire

This questionnaire should be completed once you have read the draft strategy document. The survey is divided into two areas: proposals for human regulatory science and proposals for veterinary regulatory science. You are invited to complete the section which is most relevant to your area of interest or both areas as you prefer.

We thank you for taking the time to provide your input; your responses will help to shape and prioritise our future actions in the field of regulatory science.

# Data Protection

By participating in this survey, your submission will be assessed by EMA. EMA collects and stores your personal data for the purpose of this survey and, in the interest of transparency, your submission will be made publicly available.
For more information about the processing of personal data by EMA, please read the privacy statement.

# Questionnaire

**Question 1: What stakeholder, partner or group do you represent:**
- ○ Individual member of the public
- ○ Patient or Consumer Organisation
- ○ Healthcare professional organisation
- ○ Learned society
- ○ Farming and animal owner organisation
- ○ Academic researcher
- ○ Healthcare professional
- ○ Veterinarian
- ○ European research infrastructure
- ○ Research funder
- ○ Other scientific organisation
- ○ EU Regulatory partner / EU Institution
- ○ Health technology assessment body
- ○ Payer
- ○ Pharmaceutical industry
- ○ Non-EU regulator / Non-EU regulatory body
- ● Other

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

Not for Profit Organisation for Professionals in Biomedical Research

## Name of organisation (if applicable):

European Forum for GCP (EFGCP)

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

- ◉ Human
- ○ Veterinary
- ○ Both

## Question 3 (human): What are your overall views about the strategy proposed in EMA's Regulatory Science to 2025?

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

The strategy is engaging and forward-looking.

Meeting the goals which we considered equally high priority throughout, will be challenging when there is also an identified need for up-skilling the workforce to adopt and understand technology changes.

## 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)

> 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.

> This goal linked with our other top three core recommendations relating to the fostering of innovation and development of the regulatory framework for emerging digital clinical data generation and was only slightly above the development of competence with collaboration to engage in big data AND the recommendation relating to real world data.
>
> Our discussions related to the interconnectivity of all these goals to achieve the best outcomes for patients. Timely publication of guidance, reflections, Q&A as the Agency adapts or deems a necessary flexibility in interpretation or need for evidence in a specific area (for example in submissions) would be important to our community.
>
> The potential barrier to innovative research of existing GCP guidelines (for example the remit of the Sponsor Versus the Investigator, rather than the Sponsor in collaboration with the Patient, Investigator and/or Technologies as foreseen) was raised as an area for action (though development of ICH E6 guidelines was acknowledged)
>
> It will be important for Computerised System/IT Experts andLaboratory analysts to fully understand their role in delivering technologies, in a legal GXP compliant manner (when often they are third party vendors used for their expertise). This was seen as a challenge, and action may need to be taken to engage further with these experts raising their awareness of legislation, guidance and expectations in the area to safeguard patients rights, safety and wellbeing as defined in GCP Principles and the Declaration of Helsinki.

Second choice (h)

> 9. Foster innovation in clinical trials

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.

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Engagement and collaboration with academic institutions (a recurrent theme throughout the strategy) was highlighted as both an asset and a potential challenge. Our discussions did not centre on a lack of scientific knowledge or capability but the challenge in completing a successful collaboration which results in the necessary records to satisfy Inspectors for licensing decisions, and the extent and balance of information available to Healthcare Professionals.

The extent of current record-keeping for the support of submissions increases, and as technology enables the linking and collecting of data, file and database management becomes in itself an operational task which is commonly a hidden cost (rarely funded).

EMA may be in a position to play a positive role with funders to support administrative aspects such as database validation, file and record management) as well as play a role in moderation/proportionality through CTTI, ICH and Inspection Standards/expectations.

Global collaboration (and if possible harmonisation) of expectations, guidance and standards will support delivery and trust in the data for this goal.

## Third choice (h)

10. Develop the regulatory framework for emerging digital clinical data generation

## 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.

This goal linked closely with the big data goal, and we found it difficult to dissociate the two. It followed from our previous choices and raised the questions: Does more data mean more quality? Have we the competence (statistics or otherwise) to fully evaluate data to todays standards and expectations if the datasets could be significantly larger?

We agreed with all the goals and actions - linking again to comments above regarding assessment capability, GCP oversight etc. Agreeing also that better science is supported by better analytical skills (manual, laboratory and/or computational!)

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

We wondered only about the ability to engage with Healthcare Professionals to ensure the information is provided in an unbiased format; this links to existing transparency initiatives, but we did wonder if Scientific Publications expect the same rigour as a regulatory decision-maker, when potentially they have a wider influence. Has EMA a role to play in working with the Scientific press?

**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 | ○ | ● | ○ | ○ | ○ |

| | Very important | Important | Moderately important | Less important | Not important |
|---|---|---|---|---|---|
| 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**:

<br><br><br><br><br><br><br><br><br><br><br>

## 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**:

How will you prioritise if all goals are Moderately to Very Important?  We were unable to demote many of your proposals to Less Important and no-one thought any were 'not important' in our team of reviewers.

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**

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