Public consultation on EMA Regulatory Science to 2025

Fields marked with * are mandatory.

* Name

* Email

EUROPEAN MEDICINES AGENCY

SCIENCE MEDICINES HEALTH

Introduction

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

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

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

Completing the questionnaire

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

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

Data Protection

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

Questionnaire

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

* *Please specify:*
  between 1 and 1 choices

☐ Individual company
☑ Trade association
☐ SME

Name of organisation (if applicable):

AESGP - Association of European Self-Medication Industry

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.

While being a comprehensive reflection, the focus is understandably on supporting new therapeutic approaches and medicinal products for unmet medical needs (PRIME), we believe that the following orientations should be added:

- Support innovation in the selfcare sector by supporting change of the legal status notably for medicinal products which meet unmet selfcare needs. This also takes into account the important contribution selfcare can make to healthcare (economical but also freeing national resources that become tight at national level) and the growing role and empowerment of patients and citizens who want more and more to take care of their health.

- Reduce bureaucratic burden and optimise regulatory processes, notably making best use of telematics systems and their interconnection (eg SPOR becoming operational and feeding products' information in Eudravigilance, etc.) may be considered such as how the EMA can support the change of legal status from prescription to non-prescription from a scientific point of view.

- Further the risk-based approach across areas of competences (pharmacovigilance, quality)

- Reflect on the fact that there is no one size fits all in terms of products categories (eg well-known products, herbal medicines), sectors' specificities and companies' size. Specificities of each has to be considered, in guidance application of new science developments, methodologies, guidance, etc.

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

Comments on strategic goal 1 (h):
Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

Considering the quick pace of development of medical device / digital technologies, creating an accessible, timely coordinated evaluation pathway for the assessment of medical devices, IVDs and borderline products between notified bodies and medicines regulators at an early stage of implementation of the MDR/IVDR is crucial.

The EMA (and medicines authorities) have an increased new role; they should:
•   Support the objective to increase/enrich expertise at the interface between medicines, medical devices and borderline products. There is a need for better and harmonized understanding of what is not acting by pharmacological, immunological or metabolic means to allow the different legal frameworks to be effective and coexist. MDR for sure does not come before science but recognizes the development of science and knowledge of products' mode of action

•   Facilitate the regulatory pathway between NBs and medicines' regulators and this requires to clarify the respective and collaborative role between notified bodies and medicines' regulators to avoid disproportionate regulatory burden in the device assessment

•   Define regulatory science in the way that it informs the regulatory decision-making process but does not take over the decision-making process notably related to the regulatory status of products

•   Borderline products/Consultation on regulatory status: Foster an inclusive regulatory system where concerned stakeholders are properly involved at an early stage to allow a case-by-case assessment of each product individually

•   Avoid having too many products stuck at the 'border' and preventing the patient access to useful healthcare solutions

We concur with need for a scientific and regulatory advice following the life cycle of a medicinal products. AESGP would support a multi-stakeholder scientific advice for non-prescription medicines which could constitute a 'one stop shop' to get advice on a potential change of legal status with authorities and additional stakeholders.

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations (h)
○ Yes
◉ No

Comments on strategic goal 2 (h):
Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

With regards to the goals on benefit-risk, we acknowledge the goal to promote the systematic application of structured benefit – risk methodology and quality assurance systems across the network. Currently the structured B-R is illustrated by the effect – table which is more adapted to an innovative products but does not meet the needs and specificities of a change of legal status or switch. The so called Brass model (Brass et al Clin Pharmacol Ther 90:791, 2011) on benefit risk was developed as a tool to improve the B-R analysis for non-prescription medicines and we believe it is more appropriate model for our sector.

We believe it could also be a useful tool not only to communicate on benefit-risk but also to aid SA, regulatory advice and pre-meetings and CHMP discussions.

It would be useful to also explore possibilities of behavioural change approaches in support of the scientific evaluation of non-prescription medicines. Behavioural change approaches may help regulators to overcome risk-averse attitudes regarding non-prescription medicines.

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

○ 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.

Aligned with the need to use high quality real-world data in regulatory decision making, not only to address the safety of product but also to assess efficacy in particular the potential of Big Data in regulatory decision making with regard to changing the legal status of medicines from prescription to non-prescription should be considered. We also see a role in RWE to substantiate efficacy/ efficiency of older products which B – R would be potentially challenged by the lack of clinical data according to modern standards.

We would also welcome the discussion regarding preparedness of national healthcare systems could also cover facilitating access to non-prescription medicines. Telemedicine services, associated invitro diagnostics could amongst others help reinforce patient monitoring and surveillance which could be beneficial in discussing suitability of non-prescription status more consistently across EU member states. The need for national electronic medical records to also record non-prescription use is also key to enable EMR as a source for RWE data for our sector.

As part of the Inter-association TF on ePI, AESGP supports and acknowledges the importance of ePI as a priority from a public health perspective to ensure patients have timely access to up-to-date regulatory approved product information. Industry has been working on the ePI concept in the last years and we believe we should be contributing together with healthcare providers and patients to the strategic plan to deliver the ePI program.

It is clear that ePI will make it easier for patients/consumers to have access to up to date PI, search and retrieve information in a more suitable way however it will not solve poor compliance or low literacy per se. therefore we very much support the goal to improve the content of the leaflet.
We recommend alignment with the Telematics Strategy/Health Strategy and use the same common electronic standard and to reassure interoperability. Lessons learned from the implementation of eCTD, xEVMPD, CESP and PIM should help to guide the discussion, and collectively demonstrate the importance of stakeholder engagement. It is considered positive to consider co-operation with the European Common Data Model and European Interoperability Framework (EIF). It is recommended to design the ePI to take into consideration the core recommendations of EIF to achieve efficient sharing and re-use of structured and semi-structured data. Principle 4 (recommendation of re-usability of data for optimization of processes) should be also included.

Since ePI is part of the future Telematic Strategy, common electronic standard and the Process Governance should encompass significant criteria such as quality of data, and its re-use, inter-dependency and

connection to all EU telematics projects, including SPOR, TOM and CTIS.
Future funding models of EU telematics projects are under discussion within the Telematic Management Board; so an integrated approach using common building blocks would help to assure cost-efficiency between projects.

Therefore, incentives for regulators and industry would be helpful:
- efficient process for having the current PI changed to an ePI
- Process optimisation for changes to the PI; easier process for variations where PI is impacted. The ePI should not lead to increase of workload on maintenance of PI, it should in fact give opportunity to decrease the workload
- Easier implementation of safety variations/referrals (reducing the urgency to provide updated information to EU citizens, which are currently managed through the appropriate transition/grace periods);
- Free tool for smaller companies/CROs and application programming interface (API) for companies with a wish to have machine-2-machine communication.

Industry also recommends having a transparent and open discussion regarding the "data stewardship" of the content of the Product Information. A clear responsibility assignment needs to clarify the accountability and liability for each step, in particular for the final content that is publicly available. We believe this openness will facilitate a collaborative and efficient regulatory evaluation between Industry and Authority and improve the governance aspect.

Strategic goal 4: Addressing emerging health threats and availability/therapeutic challenges (h)

◌ Yes
◉ No

Comments on strategic goal 4 (h):
Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

With regards to availability of medicines, it will be important to ensure the management of a shortage should be proportionate to the impact on the patient: it is necessary that notification to authorities on shortages and the further evaluation follow a risk-based approach taking into account several factors in particular the duration of shortage, the criticality of the medicine and whether an alternative exists. We also favour the use of a harmonised EU template for the communication to authorities.

In addition, we would like EMA to support the coordinated development and application of not only Point-of-Care diagnostics but also self-tests.

Strategic goal 5: Enabling and leveraging research and innovation in regulatory science (h)

◌ Yes
◉ No

Comments on strategic goal 5 (h):
Please note you will be asked to comment on the core recommendations and underlying actions in the subsequent questions.

We support that EMA reinforces its ties with academia to support the delivery of its strategic goals. We also support its contribution to IMI, CIOMS, other research projects provided its resources enable the Agency to do so without compromising its normal activities.

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)

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

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.

We see the use and regulatory acceptance of RWD as an important opportunity in our sector to substantiate the request for a change of legal status and also address possible particular concerns from authorities at early stage concerning the availability of the product without prescription. Authorities more reticent regarding a switch may hence through RWD receive reassurance that the availability of the product as OTC is well-monitored from a post-marketing surveillance perspective.

We believe a workshop with industry on this topic would be beneficial to kick off reflection about a reflection paper or guidance. Exchange of best practices as well within the network but also with other regulatory authorities like the FDA would be good first steps.

Second choice (h)

5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

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.

Considering the quick pace of development of medical device / digital technologies, creating a coordinated (rather than integrated) evaluation pathway for the assessment of medical devices, IVDs and borderline products between notified bodies and medicines regulators at an early stage of implementation of the MDR /IVDR is crucial. There is a need for better and harmonized understanding within medicines and devices regulators of the remit of each framework and their interactions. In particular, the EMA regulatory science strategy should allow the different legal frameworks to be effective and coexist notably by recognizing the development of science and knowledge of products' mode of action including those that are not pharmacological, immunological or metabolic. In light of our membership's knowledge at the interface of the medical devices, medicines and borderline products, AESGP would be interested in future cooperation on this topic.

Third choice (h)

14. Exploit digital technology and artificial intelligence in decision-making

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.

We welcome the work done by the HMA/EMA Joint Big Data Taskforce and the recommendations put forward in the summary report. we would however appreciate additional opportunities for the involvement of Industry and to consider Industry's potential participation in the further work on this topic. A clear orientation / roadmap would also be desirable. We support the harnessing of ICH or other harmonisation programmes to ensure the data readability, usability and re-usability even if different standards are applied. We agree with the important consideration of the legal requirements to protect patient privacy when sharing data. Technologies such as BitLocker for the sharing of encrypted data need to be explored.

We see the potential of novel analytical approaches for modelling of Big Data sets for regulatory purposes (artificial intelligence, machine learning), and recommend stakeholder pro-activity in regard to sharing of learnings and in keeping a watching brief on new developments and successful use cases.

Change of legal status is one of the areas where Big Data could be of significant relevance and therefore AESGP would be interested in future cooperation on this topic.

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

We refer to our comments under point 3 and under each strategic goal in response to question 4 regarding change of legal status from prescription to non-prescription, behavioural change approaches or self-tests.

A further area for focus of the network in the coming years remains to ensure the most appropriate legal classification is applied to products and the mechanisms for allowing those that can be safely reclassified as non-prescription medicines are in place, effective and being used, thereby improving patient access. The use of the Brass model, RWD-RWE, big data, IVDs (self-tests) are new tools which can contribute to unleash the potential of the centralised procedure to authorize innovative first in line switches.

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:

1 – 3: Not relevant for non-prescription medicines
5: Very important topic to ensure coordination rather than integration, but critical that this is done in the near term (not as part of 5-year strategy), to address implementation of new EU Medical Devices and In Vitro Diagnostics Regulations.
7: Essential that advice, decisions and actions across the EU Medicines Regulatory Network is aligned and integrated and involve multiple stakeholders.

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	◉	○	○	○	○

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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**:

9: Interesting for consumer healthcare industry with regards to the following aspect: new data sets from digital technologies, e.g. patient reported outcomes captured by new technologies such as wearables and for EMA plans to work with stakeholders to encourage collaborative clinical trials.

10: The following actions from the paper: "develop methodology to incorporate clinical care data sources in regulatory-decision making" and "develop capability to assess technology such as wearables" are very important for consumer healthcare company. Actions are closely linked to other recommendations on RWD (#18) and "big data" (#19).

11: Not all underlying actions are of equal importance. Incorporating patient preferences is important. Ability for EMA to analyse individual patient data is less important: the EU regulatory system functions well without this capability. Other actions address communication, rather than directly advancing regulatory science. Behavioural change approaches would be welcome to be explored as well as the use the Brass model as they may be valuable to the B:R evaluation of non-prescription medicines.

13: Modelling, simulation and extrapolation use across product lifecycle could facilitate registration for our products, for instance extrapolation of adults' data to paediatrics.

14: The following action: "develop capacity and expertise to engage with digital technology, AI and their applications in the regulatory system" is crucial for our portfolio. Use of AI could become increasingly important in supporting robust and consistent regulatory decision-making and EMA needs to be ready. Necessary to support use of "big data".

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	○	○	●	○	○

Recommendation					
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**:

15: not relevant for the selfcare sector.

16: We would welcome the discussion regarding preparedness of national healthcare systems could also cover facilitating access to non-prescription medicines (e EMR including use of non-prescription medicines). Telemedicine services and increased postmarket surveillance could help reinforce patient monitoring and surveillance which could be beneficial in discussing suitability of non-prescription status more consistently across EU member states.

17: Evidence generation and EMA decisions should be based on real patient needs and perspectives. We would highlight the relevance to "explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation" and hence would rate this goal as "moderately important".

18: Aligned with AESGP workplan to look deeper into the use of RWD/RWE. Use of complementary or alternate data sources should support streamlined drug development.

19: need to implement outcomes of HMA-EMA Joint Big Data Task Force and work in collaboration with the industry. Supports other recommendations, particularly #18 on RWD.

20: Important; the content should not be forgotten and the system chosen for ePI should be sustainable and interoperable.

21: Not relevant for us

22: We supports this goal as the lack of trust in the regulatory system undermines trust in medicines approved in EU and citizens seeking non-proven alternatives through internet supply channels. Particularly self-medication is very sensitive to patient's trust. It would therefore be useful that when the EMA develops

'more targeted and evidence-based communications facilitated by updated web content and format', they consider developing specific communication on self-medication as it is subject to same regulatory framework as other pharmaceuticals. It would also be useful there is a collaboration with EC, EMRN, EFSA, ECHA. However, these actions should not support a specific recommendation but be undertaken following or as part of other recommendations, rather than as a separate specific activity

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**:

24: The EMA encourages the development of rapid diagnostic tools while only mentioning Point-of-Care (PoC) diagnostics, without any reference to self-tests. We would like EMA to support the development and application of both PoC diagnostics and self-tests. For example, self-test (at home or in a pharmacy) allowing a patient to detect whether his/her sore throat is due to a virus or a bacteria can allow self-medication (virus) or reference to a doctor (bacteria). This would best serve, patient and the healthcare

system as a whole by preventing secondary effect of untreated strep throat in one case or unneeded doctor visit and antibiotic prescribing and thus tackle AMR in the other.

25: Supply shortages should indeed be addressed globally, and strict reporting should not be necessary for all products, a risk-based approach should be used. Essential that work is done in close partnership with industry.

27: This goal is viewed as 'less important' in view of the other goals previously described that would require substantial resources.

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**:

28, 29, 30 and 31: These proposed recommendations and actions should be supportive of other recommendations

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