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

Name of organisation (if applicable):

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.

Introduction

The document fulfills EMA's remit to promote and protect human and animal health by putting science at the foundations of its vision and the patients at the center of its strategy.

The 5 goals and core recommendations incorporate all the most compelling challenges that regulatory science is facing, from the integration of digital health, to the understanding of new materials and manufacturing technology, from the need for an accessible communication strategy to the openness towards an increasing number of stakeholders, including payers. The document recognizes that the growing pace of innovation requires new expertise and new regulatory pathways, as well as the need of updating the regulatory framework in a timely manner, to avoid delays that might increase the distance between current approaches and scientific and technologic evolution.

On the other hand, it is noted that the whole document is focused on the role of EMA as centralized regulatory body, barely considering that the European Agency fully relies on expertise and competence from National Competent Authorities (NCAs) in order to fulfill its objectives, and that some of the tasks fall within the remit of NCAs and not EMA (such as clinical trials, shortages, HTA etc.).

With this in mind, the Italian Medicines Agency has developed an integrated document, as a result of extensive internal expert consultation, aimed at improving and critically supporting EMA strategy.

General comments

• The document appears quite long, since some of the topics are repeated in different sections, with minor and/or negligible differences. Moreover, many concepts are implicitly contained in others or strictly interconnected so that one could not exist without the other. This makes reading and understanding sometimes confused.

• In the EMA document it is acknowledged that innovation refers to new advanced products but most importantly to new regulatory approaches. It's our opinion that new approaches require the highest effort. New manufacturing technologies, different data sources, digital health, virtual trials, extrapolation and artificial intelligence are transformative and to this extent, require a comprehensive revision of the current regulatory processes. The European Agency, as an infrastructure supporting National Competent Authorities should, in principle, incorporate innovation in the way its own regulations are delivered and consider a profound restructuring of the Committees and Working Parties; novel, more rapid processes to deliver and update guidance documents and an extensive collaboration with international agencies to learn from best practices.

• Innovation is certainly an opportunity and it is EMA's mission, in consideration of its centrality with reference to the 28 MS of the European Union, to translate it into patients' access, by enabling a favorable European environment.

• Communication became a central pillar of each strategy in modern society. The EMA document

expands on the use of big data or data generated by digital devices in the evidence building contributing to the evaluation of a medicinal product. It is acknowledged that these types of data sources are subject to challenges related to data protection and security. For this reason, a communication campaign on digital health could be implemented in order to build public confidence.

• Training sessions on digital technologies (i.e. artificial intelligence, Big Data, virtual clinical trial, sensor generated data) represent an important opportunity for the experts working in the European regulatory agencies. Nonetheless, due to the complexity of those technologies, training may not be sufficient and the involvement of different professionals (i.e. information engineers and data scientists) is required. It could be an opportunity for the EMA to establish a multidisciplinary working party dedicated to the application of digital technologies to drug development, authorization and post- marketing surveillance.

• Transform the patient experience in science of patient engagement (standardization of outcomes, validation of data and sources- digital- etc..).

• Encourage the open data and data sharing along with interoperability and accessibility of data sources.

• A close monitoring of the safety of medicines put on the market following the implementation of new technologies, novel pre-clinical models, innovative clinical trial designs or authorized with different schemes, should also be standardized and implemented, in order to quickly assess the impact these innovations have on patient health.

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.

The session is adequate but we have the following comments.

Building the latest and innovative scientific and technological knowledge into the development of more patient-oriented medicines is a strategic goal of high relevance.

Support and promotion of high level treatments tailored to specific clinical needs should be a priority for the next years to come; however, development of new means of monitoring or predicting safety issues should be promoted and supported as well, in order to ensure that during new approval procedures safety in special population or in long term use could be predicted with a higher grade of certainties.

In order to facilitate this process, one should leverage on existing infrastructures and integrate experiences and data monitoring. In this frame it would be desirable a foster collaboration and an experience exchange with both the European Commission's Classification and Borderline Expert Group and the HMA-EMA Joint Big Data Taskforce.

The newly created AI-test Laboratory could be supervised in its potentials to affect regulatory decisions by the EU-IN.

It is also underlined that:

i) support in training for national experts in these areas, both belonging to regulatory as well as non-regulatory bodies, should be included among the actions.

ii) Clinical trials are not within the remit of EMA: alignment of assessment between CT and scientific advice and GCP is welcomed, but this should be planned through the active involvement of NCAs.

iii) It would be helpful to identify new advice mechanisms to allow the development of new innovative products/process and, in parallel, address new regulatory guidance/position papers.

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.

> The session is adequate but we have the following comments.
>
> The objectives outlined in this item are particularly relevant and challenging.
>
> • A closer interaction with NCA is needed, since the core activities are strictly connected with national mandates. As previously mentioned, some actions are outside the EMA remits (such as clinical trial authorization and HTA acceptance).
>
> • It is acknowledged that the system could take advantage from data exchange, analysis and interpretation of large amounts of data; however, a big amount of different and often non validated data obtained from these sources could in principle impair the quality of evaluations. The standardization and validation of data is thus considered necessary even if this might imply high costs, long times and possible bias.
>
> Consideration should be given to setting up of common standards, objectives, standard formats and terminology in order to make real world evidence reliable and usable.
>
> • The proposal to develop novel preclinical models and the use of modeling and extrapolation (core recommendations 3.2.1 and 3.2.2) could allow a better use of resources and a shortened approval process. The use of risk assessment models, in a complex regulatory systems, if promoted/developed, will become an essential tool for regulators in the decision making process.
>
> • An additional role for EMA could be identified in facilitating equal access among MSs.
>
> • With reference to core recommendation 3.2.4, it is noted that academic research is currently incorporated in evidence-based benefit-risk "communication", while it would be more beneficial to incorporate it into benefit-risk "assessment".

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.

The session is adequate but we have the following comments.

Innovation is a multi-stakeholder process that should include patient's vision and experience. In this perspective, it is mandatory to define a science of patient engagement by establishing standards such as outcomes, data and data sources validation, with the ultimate goal to ensure quality and integrity of data. Patient-centred regulatory science assumes that patients should be trained to raise awareness of their own rights (i.e. property of data), pivotal role in the regulatory system and responsibility.

• It is endorsed a systematic implementation of educational and training programs to educate patients as experts in regulatory science.

• Horizon Scanning (HS) is considered an efficient tool to support decision-making and rational use of available resources and an additional regulatory instrument for promoting early access to medicines. Indirectly, by providing timely information for early and efficient decision-making and identification of medicines and therapeutic areas of special interest, it also reduces possible barriers to the adoption of new technologies. The Health Technology Assessment International (HTAi) Global Policy Forum, has pointed out HS as efficient complementary tool for programming of health care resources. The Italian Medicines Agency (AIFA), unlike the majority of other Medicines Agencies in Europe, is responsible for both national Marketing Authorization (MA) and price, reimbursement and innovativeness definition. Italian Agency relies on three main tools to reduce budget impact of novel therapies: price negotiations, cap on specific drug expenditures and performance-based schemes. Different regulatory tools (e.g. different authorization paths, scientific advice procedures as well as the European Medicines Agency - EMA Priority Medicine Scheme - PRIME) have been developed with the aim to promote a timely access to new medicines in EU and are based on early information availability and anticipated assessment. However, there is still room for the implementation of additional/complementary tools to fully address increasing health care requests. A robust horizon scanning systems at national (and European) level is needed to help decision-makers to plan and prepare for innovation. Our experience remarks the importance of development and implementation of methodological approaches for HS activities and shows how HS methods could be sensible enough to allow for discrimination of different categories of medicines with diverse characteristics and effects on HCS. The implementation in routine regulatory practice of HS could support early identification of medicinal products of special interest and allow for anticipated programming of resources by National health Systems.

• A more defined approach to make access more equal among MSs after CHMP approval and EC Decision should be provided, as EMA can only play a supportive role in access to medicines. In order to better address the priorities for HTA, the European Agency could play an important role in collecting and elaborating inputs from HTA bodies and payers. Indeed, the role of EMA in identifying unmet medical need is considered out of proportion, while academia and HCP could play a crucial role.

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.

The session is adequate but we have the following comments.

- This strategic goal is of particular relevance considering the need of new antibacterial agents and their alternatives to prevent emerging infectious disease due to the antimicrobial resistance issue. Regulators should identify those diseases that pose a risk to public health due to their epidemic potential and for which countermeasures do not exist yet or are not sufficient. Development of high level and harmonized guidance to assist the research of new antibacterial and diagnostic tools.
- Furthermore, it is necessary to identify effective interventions in different health contexts to optimize the prescription of medicines and their use by patients (including aspects of pharmacovigilance).

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)

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.

As a general comment, it is underlined that most of the listed recommendations are particularly relevant and strictly interconnected, thus the choices here reported are considered only partly reflective of real priorities.

Regulators and academia have different points of view and different approaches. They should be brought together to interact more, so that they could share the same strategies and same objectives.

Second choice (h)

24. Continue to support development of new antimicrobials and their alternatives

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.

This point is considered one of the most important as it impacts the whole population.
The lack of effective antibiotics poses a serious health threat due to the rapid spread of infectious diseases and AM resistance. The strategy so far adopted to support development of new antimicrobials is not leading to the expected results. More than continuing with the current approach, different tools could be explored. FURTHERMORE THE REVISION OF THE CURRENT ORPHAN LEGISLATION IS CONSIDERED OF THE UTMOST IMPORTANCE.

Third choice (h)

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

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.

It can be envisaged that this comprehensive approach will be needed more and more in the next years. Common criteria and standards of evaluation need to be defined.

Dedicated guidance and possibly a common Committee could be a way to address this evolution in treatment.

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

In Goal 1

Understanding of best use and opportunities of digital technologies across the entire chain of pharma lifecycle with the aim of facilitate its implementation could be considered as additional recommendation. In this frame, it could be an opportunity for EMA to establish a multidisciplinary working party dedicated to the application of digital technologies to drug development, authorization and post- marketing surveillance.

An example of the best use and opportunities of digital technologies is represented by blockchain technology. As it guarantees identity, integrity, security, quality and privacy of shared data, blockchain technology could be useful to control data transfer and management across the entire chain of pharma lifecycle (e.g. GMP, supply chain, Big Data sharing, data from digital sensors and wearables etc).

In this context it is noted that the strategy does not specifically highlight technical training for the Regulators evaluating new digital technologies, mainly for assessors and inspectors who will have to deal with these new systems, developed at Industry level.

In addition, besides technical training, also developing new regulatory standards for the assessment of data provided by digital technologies should be in the priority list of activities for the next years.

In Goal 3

It is stated that

"the public health aim is to ensure that patients receive timely access to affordable, high-quality medicines that meet their medical needs."

FIRST OF ALL, TO ENSURE THE PATIENTS RECEIVE THE MOST APPROPRIATE MEDICINES, THE NEW THERAPIES SHOULD BE EVALUATED IN COMPARISON WITH THE BEST AVAILABLE ALTERNATIVES FOR THAT INDICATION, WITH SUPERIORITY TRIAL TESTS.

WITH REGARD TO MEETING THE MEDICAL NEEDS OF THE PATIENTS, THE APPLICATION FOR A REGISTRATION OF THE OFF-LABEL INDICATION WHICH ARE OF CONSOLIDATED USE, SHOULD BE NOT LIMITED TO THE COMPANY OWNER OF THE PRODUCT.

Innovation leads to the development of highly promising products for patients' health that imply challenges in terms of structural, organizational and technological access. This already happened for CAR-T therapies and it would be easily replicated for new digital therapies or new digital tools that specifically require technological infrastructures development. Although these aspects are not in EMA remit, it is desirable that EMA will strengthen collaboration with National Competent Authorities, National healthcare systems and any other possibly involved European bodies in order to guarantee fair and timely access to all European patients.

Furthermore, from AIFA's point of view, the strategy should include the goal of monitoring with even more attention the safety of medicines put on the market following the implementation of new technologies, novel pre-clinical models, innovative clinical trial designs or authorised with different schemes, in order to quickly assess the impact these innovations have on patients health.

Further stress should be put on EMA role in reinforcing the connection between different stakeholders, including at global level, with the EU network and NCA experts supporting EMA scientific activities and assessment.

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 2:
Over one-third of the medicines in the PRIME scheme are ATMPs and most, if not all, of these products are gene therapies. This implies that the latter ones hold the promise to fulfill unmet medical needs, even for pathologies that are less rare as compared to the past. All the actions proposed are supported. Additional /revised actions that may foster ATMPs development are proposed:

    REVISED ACTION 4
Address the challenges of ATMP manufacturing (decentralised manufacturing and delivery locations, expedited quality aspect development, conduction and evaluation of comparability excercise, e.g., manufacturing process changes, new manufacturing site(s) implementation, etc)

    PROPOSED TO ADD ACTION 6
The European Commission is actively addressing the issue of extension of GMO authorization harmonization across the EU community to additional ATMPs (besides human cells genetically modified by means of retro/lentiviral vectors). This is currently discussed within the GMO Interplay WG, where both representatives from NCAs and relevant Ministries are present, and should be included among the actions.

    COMMENTS ON ACTIONS 2 and 5
These should imply a capillary and proactive communication to developers about the usefulness to engage a very early dialogue with the Agency, starting from the classification of their product. This would help in particular small entities to exploit all available tools to rightly orientate their development plan (e.g. CAT ATMP classification, PRIME scheme, SME office, ITF, EU-IN through national Innovation Offices) and would decrease the de-regulated/underground use of these products.

    COMMENTS ON ACTIONS 2 and 3
These actions should imply early and constant involvement of stakeholders, in particular HTA bodies.

Recommendation 3
•   First, it is questioned whether the recommendation "Promote and invest in the PRIME scheme" should be in the context of Goal 1, rather than Goal 2/3.
•   In the proposed text it is rightly pointed out that "involvement of HTA is crucial to ensure that scientific advice takes into account their evidence requirements to facilitate decision making and patients access". Acknowledging that actions to enable involvement of HTA and payers' in prospective discussion of evidence generation plans are proposed in the context of Goal 3 it is notable that HTA/payers involvement is not explicitly mentioned in any action in this recommendation.
•   One proposed action is "Shorten the time between scientific advice, clinical trials and MAA submission". Shortening is not deemed beneficial per se. Rather, it is much more relevant that the process is improved in the ability to shorten the time while ensuring that the pre-licensing evidence generation plan is of sufficient quality to meet the requirements of HTAs/payers, so that approval can results in true access.

Recommendation 4

3D bioprinting represents an emerging and promising technology with strong implication in drug manufacturing, quality, efficacy and safety. An action planned on this area is mandatory.
It is worthy to underline that deficiencies in the assessment of "traditional" manufacturing processes have been recently highlighted with the sartans case at worldwide level. Lesson learnt from this negative experience (e.g. how to avoid similar events in the future, reinforce the EU network-EDQM, OMCLs), should be considered while approaching both consolidated and novel manufacturing processes.

Recommendation 5
This recommendation is particularly important in light of the new Regulations on Medical Devices (Reg. 745 /2017/EC) and In-vitro diagnostics (Reg. 746/2017/EC). In particular, companion diagnostics specifically appear for the first time in the IVDR where they are included in the highest risk classes (Class C-rule 3, Class D-Art.48). More stringent requirements will then apply to these diagnostics and the conformity evaluation procedure will involve NCAs/EMA. In light of that and considering the proposed actions, a structured cooperation plan between Medicines and Medical Devices competent authorities is deemed needed.

Recommendation 7
Before promoting more integrated medicines development, it could be relevant to look into experience gained so far e.g. verifying the adherence rate to scientific advice given and analyzing the reasons why scientific advice were not met.

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**:

Recommendation 8
In order to consider the non-clinical toxicological models based on 3Rs principles as alternative systems to standard models, it is pivotal their validation and the building of regulatory awareness. Interaction with 3Rs toxicological model developers and validation body (i.e. ECVAM) is particularly relevant.

Recommendation 9
The proposed EMA strategy mentions innovation in clinical trials coming, for example, through the use of novel trial designs, endpoints, techniques for gathering data, use of "-omics". Drivers for such innovations include small eligible patients populations, limited endpoints to demonstrate efficacy and b/r, and the availability of new data sets from digital technology. If from one side this could reduce the time to make a medicine available, from the other side could limit the level of knowledge on b/r profile collected at the time of authorization and increase the risk of occurrence of unforeseen safety issues that could undermine patients' health and lead to costly and time consuming urgent measures (urgent safety restrictions, safety referrals, etc.).

Digital Health technologies (artificial intelligence, big data, predictive analytics, wearable and sensors, smartphones) will be most likely to improve clinical trial facilitating patient engagement and participation. A communication campaign on digital health could be implemented in order to build public confidence. Virtual clinical trials (VCT), also known as siteless or de-centralized clinical trials, are in their experimental phase actively pursued by sponsors with the aim of enabling remote access, reducing cost and infrastructures, expediting recruitment and applying digital technology. Indeed, there are many uncertainties into how data generated by VCT could be compared to current standard of evidence, and the relevance of such approach is questioned, since it would be limited to a small subset of clinical trials (i.e. post marketing, or targeted only to few therapeutic areas, or only focused on observational studies, etc.). There are also issues in adapting this new framework to the upcoming clinical trial regulation as well as to EU data protection standards and to GCP requirements.
On the other hand, since clinical trials are multicentered, Europe wants to keep the pace of innovation and be a hub for companies and developers allowing this technology in its territory. A pragmatic reflection starting from CTTI recommendations and their compliance with EU regulations is warranted. NCA, via CTFG, must be involved in this discussion, being the clinical trial a national mandate.

Recommendation 11
Expand benefit-risk assessment and communication is the main scope, EMA should reinforce it by updating it to the current scenario in the fastest way possible.

Recommendation 12
Preconception healthcare should be included into maternal and fetal healthcare as scientific evidences demonstrated that use of certain medicines before conception is associated to increased risk of adverse pregnancy outcomes and birth defects. Develop a strategic initiative which targets also childbearing women is pivotal to increase awareness of the correct use of medicines during a such important moment of women's life and protect maternal and child's health. It is suggested the following rewording of the text 3.2.5 pag 18: understanding the consequence of medicines exposure in preconception period and during pregnancy needs to be intensified and broadened.

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

14

	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
It is important that a strengthening in (early) cooperation with HTA/payers is pursued based on the acknowledgment that these are key players in determining medicines use and availability in health care systems. It is endorsed that actions aimed to "ensure the evidence needed by HTAs and payers is incorporated early in drug development plans". In this regard, the action "Discuss with HTAs guidance and methodologies for evidence generation and review" appears critical and is endorsed. Together with this

action, a strong action to increase the early involvement of HTAs/payers in scientific advice on clinical development should be pursued. Notwithstanding the respective remits of regulators and HTAs/payers, ensuring that relevant questions regarding the place in therapy of new medicines are properly addressed in clinical development should be a common goal.

With regard to the action "Contribute to the identification of priorities for HTAs" it would be appear more appropriate "Discuss with HTAs/payers to identify priority areas of cooperation aimed to positivelky impact on access to medicines".

Recommendation 16

In our opinion, a robust horizon scanning system at national (and European) level could help decision-makers to plan and prepare for innovation. Cooperation and exchange of information between EMA and HTA /payers in the field of horizon scanning, including timely sharing of information regarding upcoming regulatory submissions should be envisaged, in order to impact on Health Care Systems' preparedness-

In the text it is written "since payment models vary so much across the EU, a single platform for such dialogue would be desirable". This appears as going too far taking into account the remits of EMA and the scope of the document.

Recommendation 18

A robust HS system at national and EU level could be helpful in terms of early clarification and quantification of the treatment-eligible patient population together with use of high-quality real world data (RWD).

Implementation of a strategy for the use of Real World Evidence is endorsed with the following proposed goals: to generate post marketing data aimed at monitoring long-term benefit / risk in real clinical practice; to incorporate the patient's perspective into data generation and drug value attribution processes; to provide citizens and patients with communication and scientific information based on their real information needs.

Recommendations n. 17 and 19

The perception of the value and the risk of a specific medicine could be differently appreciated by regulators, companies, health professionals and patients. Therefore, it is important that regulators collaborate and engage with other stakeholders for an effective identification of high value medicines. In particular, the patients' relevance in evidence generation should be reinforced and promoted. Network competence and specialist collaborations to engage with big data should be obtained at NCAs level as well. Furthermore, patients' experience should be translated in objective and rigorous outcomes collected through trustable sources that can be applied in different contest of diseases. Application of digital technologies can facilitate engagement of patient, data collection, sharing and analysis.

Recommendation 20

ePI should also include risk minimisation measures and possibility to report suspect ADRs.

Recommendation n. 21

Based on our experience, HS activities could promote the uptake of biosimilars in healthcare systems, by increasing the availability of early information.

Recommendation n. 22

HS reports for external use could support this recommendation in terms of additional tools for further

development of external communications to promote trust and confidence in the EU regulatory system.

While continuing to provide evidence on the quality, efficacy and safety of medicines approved, communication should be targeted on information needed by population and healthcare professionals, through real-time web surveillance and analysis of their needs.
Vaccine communication strategies so far implemented, mainly described as product communication and defensive communication, do not show significant results in increasing vaccine confidence and may not be enough. New models of communication should be explored and evaluated. In consideration of traditional theories of advertising, it would be auspicable reframing contents of communication focusing on the positive, emotional values of immunizations. This change of message should be implemented along with a multidisciplinary and cross fertilized collaboration among scientists and communication experts.

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
As already implemented in the real time surveillance of emerging public health disease outbreaks, digital tools and integration of information sources providing RWE should be exploited for conducting a surveillance of adverse reactions in the post-marketing phase.

Recommendation 25
PROPOSED TO ADD ACTION 7
Foster the widening of Mutual Recognition Agreements (MRAs) in terms of products covered to reduce inspections costs for manufacturers and streamline authorities work, thus facilitating market access and medicines availability in EU.

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 30
> Conflict of interest possibly arising from a collaboration with experts and scientists from academia may represent an issue for implementing it.

<p style="text-align:center;color:blue;">Thank you very much for completing the survey. We value your opinion and encourage you to inform others who you know would be interested.</p>

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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