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Public consultation on EMA Regulatory Science to 2025

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Introduction

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

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

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

Completing the questionnaire

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

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

Data Protection

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

Questionnaire

Question 1: What stakeholder, partner or group do you represent:

- ○ Individual member of the public
- ○ Patient or Consumer Organisation
- ○ Healthcare professional organisation
- ○ Learned society
- ○ Farming and animal owner organisation
- ○ Academic researcher
- ○ Healthcare professional
- ○ Veterinarian
- ○ European research infrastructure
- ○ Research funder
- ○ Other scientific organisation
- ○ EU Regulatory partner / EU Institution
- ○ Health technology assessment body
- ○ Payer
- ◉ Pharmaceutical industry
- ○ Non-EU regulator / Non-EU regulatory body
- ○ Other

* ***Please specify:***

between 1 and 1 choices

- [ ] Individual company
- [x] Trade association
- [ ] SME

Name of organisation (if applicable):

Medicines for Europe

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.

EMA has set an ambitious path for regulatory science to 2025, following the fast pace of innovation in developing novel complex medicines, setting objectives towards creating a new regulatory framework that will support innovation throughout medicines development.
While it is important to foster innovation and shape the regulatory science to be able to support novel therapies entering the market, we see further opportunities of translating regulatory science and innovation into patient access by ensuring that regulatory pathways which support the life-cycle of innovation are in place in time to enable a multi-source environment when the market exclusivity is over.
Off-patent medicines have a track record in opening and broadening access to medicines. Ensuring a fit-for-purpose regulatory environment for multi-source products is a key enabler to realising EMA's mission "to promote and protect the health of those it serves through medicines regulation. This means ensuring that both people and animals in Europe have timely access to medicines that are safe, effective and of suitable quality, as well as the information needed to use those medicines and make informed choices about their treatment.''
Innovation brings value to healthcare systems by providing new therapy opportunities to the patients. At the same time, cost pressures on healthcare systems in the EU from innovation are increasing, especially with further developments in the oncology, orphan and advance therapy medicinal products (ATMPs) sectors.
The evolution of the market is putting additional pressure on the off-patent sector calling for lean/cost-efficient development and manufacturing of off-patent medicines.
To ensure that advances in the innovation and regulatory science are truly translated into greater patient access, there is a need to build an understanding of how this translates into market access to the medicines and the potential influence on competition.
To help the off-patent sector remain a sustainable and valuable part of healthcare systems, ensuring equity of access to all medicines, it is important to:
-Keep in mind that a multi-source environment requires different approaches and speed to innovative medicines.
-Learn actively from the system by optimising EU processes and infrastructures, create fit-for-purpose requirements and risk-based approaches taking into account the available body of evidence (prior-knowledge, real world data), and ensuring regulatory consistency.
-Ensure coordinated global regulatory science and regulatory policy advances.
-Actively prepare the regulatory framework for the upcoming "life-cycle of innovation": e.g. targeted therapies (non-blockbuster) oncology, orphan medicines, ATMPs.

Given the importance of our sector in Europe (currently 70% of prescribed medicines are off patent) we would like to see an ambitious and dedicated strategy, supporting the EU network's readiness for future development and access to off-patent medicines. There is a need to nominate one dedicated body/platform to put in place a more coordinated holistic off-patent medicines policy, taking into consideration early on the specificity of follow-on products and covering all aspects from development to marketing authorisation, post-licencing maintenance and market access interplay (similar to i.e. orphan, paediatric, biosimilar, herbal medicines).
The number of off-patented medicines undertaking centralised regulatory procedures is growing, and already represent a broad majority of the de decentralised procedures. There is therefore a need for the entire EU Regulatory Network (EMA, HMA, CMDh) to elaborate its strategy with a clear focus on the off-patent sector.

In general, for the next 5 years, it is strategically important for the EMA to allocate proportionate efforts on optimisation of existing procedures and processes, as well as the regulatory science advances where experience and prior knowledge is important.
We would like a dedicated off-patent medicines platform/dedicated body and contact point within the EMA.

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)

25. Promote global cooperation to anticipate and address supply challenges

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 strongly support EMA in the objective to increase availability of medicinal products in the EU.

We would encourage EMA to work on international harmonisation and develop global standards together with other agencies as an enabler to the optimisation of the development of off-patent medicines for multiple jurisdictions.Reduction in the number of Clinical Trials should be made possible with the harmonisation of requirements for bioequivalence studies. Once harmonisation is achieved, companies will be able to develop one formulation to market globally avoiding different studies in different regions, putting human subjects through unnecessary and thus unethical experimental trials, and increasing time and cost for bringing products to market and creating barriers to entry for smaller markets. Regulatory science shall support the evidence and the acceptability of a single bioequivalence study carried out using the Comparator Product authorised by a stringent regulatory authority. Results from a pilot study on the indirect comparison of

reference products from different markets (based on data from bioequivalence studies) are expected to be available in 2020 Q1/2. These results could be used to discuss the methodologies for acceptance of foreign reference products under the scope of single global development of generic medicines.

Some of the older generation of antibiotics that are currently not being used as common first-line or second-line treatments risk disappearing from the market.
To preserve future sources of off-patent antimicrobial agents, we encourage EMA to open a discussion on possible incentives to maintain the marketing authorisations for this very important group of medicines.

We would recommend further optimisations in the current regulatory framework for the lifecycle management of medicinal products that would improve availabilty of medicines.
The current framework needs to evolve to better reflect scientific and technical progress and ensure operational efficiency in line with the objective of Better Regulation. Experience gained since the last amendment of the variations framework in 2008 presents an opportunity to move to a more adaptable, proportionate and optimised approach that better supports innovation and life cycle of medicines. Such changes have the potential to facilitate continual improvement, reduce manufacturing delays and mitigate supply issues. Furthermore, developments in new information technology (IT) systems provide the opportunity to incorporate efficiency and innovation in the variation management system freeing-up regulatory capacity to enable a greater focus on those changes that may impact on quality, efficacy or patient safety, with consequent benefits to public health.
The industry is currently working on case studies showing the factors which have influenced the maintenance of the medicinal products over last 10 years, taking account of the technological and scientific evolution. Industry will also provide examples where the current EU regulatory system to report the changes to the MA constitute a barrier rather than a support in bringing updated information and innovation to products on the market in a timely manner. Those case studies are expected to be ready in III/IV Q 2019.
The evolution of the maintenance of medicinal products shall better reflect advances in science and technology as well as the practical implementation of the risk-based approach:
-To accommodate better knowledge and experience gained with a risk-based approach for well-established biological products.
-To recognise continuous improvement of and a new approach to manufacturing optimisation (ICH Q12, ICH continuous manufacturing, Q14).
-To benefit from significant progress in digitalisation and from an availability of tools and IT solutions revolutionising data collection and processing.
-To implement a new approach to life-cycle management of the supply chain by changing the way of informing about the changes to supply chains/ making a more risk-based distinction between elements to be included in the dossier (and reported via variations) and those covered by GMP and audit principles (or eventually reported via SPOR only).

We should avoid potential regulatory barriers that could contribute to shortages. Regulatory science should contribute to a more scientific/risk-based approach to Environmental Risk Assessment (ERA), instead of applicability to all products in the same way and independently of their impact on the environment (high/low risk).

Second choice (h)

21. Promote the availability and uptake of biosimilars in healthcare systems

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.

The strategic recommendation to promote the availability and uptake of biosimilars in healthcare systems is very welcomed and fits the aim of ensuring patients timely access to affordable high-quality medicines very well.
We propose to further develop the biosimilar framework for tailoring the clinical part of the development, where we see the biggest potential for efficiency gains, driven by regulatory sciences, with advantages for developability, patient access and healthy biosimilar competition. Such efficiency gains would also enable biosimilar competition for biologics where efficacy powered confirmatory studies are not feasible or too costly, due to complexities in the clinical application, limitations in patient recruitment, challenges for targeted biologic medicines with smaller population sizes (i.e. non-blockbuster). Increasing capabilities in physicochemical analysis and, more importantly, recent improvements of the in-vitro functional characterisation toolbox may create additional opportunities to waive confirmative efficacy/safety trials, in some cases even with the absence of a qualified pharmacodynamic marker. We support the adoption of ambitious risk-based approaches in the planning and development of biosimilar clinical comparability trials, which reflect product and patient related factors. Towards the end of 2019, we will be providing a thorough analysis of the evidence and experience available in the EU and the international context on this topic as a basis for a dialogue with the regulatory authorities.

Third choice (h)

27. Support the development and implementation of a repurposing framework

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.

Repurposing framework represents untapped research potential for the healthcare community and presents a very important tool to address an unmet medical need.
We encourage EMA to build further on tailored scientific advice to support step-by-step development and MAA submission, mproving trial designs and avoiding unnecessary trials in patients/healthy subjects while maintaining appropriate safeguards.
For generic, biosimilar, and value-added medicines containing well known active substances and being follow-ons from the reference products after expiry of IP rights, it is very important to consider all existing sources of information and sources of data in the regulatory processes related to known molecules.
Big data is an extremely important tool to transform data into information. The set of recommendations provided in a Big data Task-force report/summary report represents a good starting point to improve regulatory efficiency and the regulatory decision-making process. We also welcome a data-sharing culture that could inspire all the regulatory network and stakeholders involved, with the condition that patients' privacy is protected.
However, to use RWD in the decision-making process, the regulatory environment needs to be prepared to validate and ensure reliability, quality and regulatory compliance of the data.

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

Actively prepare the regulatory framework for the upcoming "life-cycle of innovation": e.g. targeted therapies (non-blockbuster) oncology, orphan medicines, ATMPs.
The EMA should include the development of clear multi-source registration requirements and incentives (where needed) for all innovations as a strategic pillar to achieve EMA's objective in terms of better access to all medicines.

Learn actively from the system by optimising EU processes and infrastructures, create fit-for-purpose

requirements and risk-based approaches taking into account the available body of evidence (prior-knowledge, real world data), and ensuring regulatory consistency.Optimisation of existing regulatory pathways could further benefit European healthcare systems, by releasing resources where scientific and technological advances allow for optimisation, leveraging years of experience and tailoring to the specific product needs. Not only industry resources, also medical authority resources can be re-invested in "unknown" territories by streamlining the processes in place.

Holistic approach to Telematics programmes and optimisation of IT infrastructure
The effective use of IT systems can be a powerful enabling tool for regulatory efficiency across Europe. Several benefits could be achieved by maximising the opportunity of the SPOR database and the concept of the Target Operating Model (TOM), by moving towards electronic product information (e- leaflet) and by building on the success of CESP (Common European Submission Platform) to harmonise and make redundant national portals. There is a major opportunity by linking systems and making multiple use of databases to accelerate procedural efficiency, accuracy and at the same time remove redundant infrastructure. We recommend avoiding multiple standards and different Telematics tools and systems across Europe that can generate unnecessary complexity and impede trusted access to information, restricting the flow of data between authorities, industry and patients.
-Holistic approach to Telematics programmes: interdependency and connection of different IT Systems as the main drivers; there is a general tendency to work in silos on different Telematics projects. This implies the duplication of data submissions and a huge increase of administrative burden for regulators and industry in keeping data consistent and reliable in centralised and national databases. The regulatory system should anticipate the change in data generation and knowledge management. This requires harmonisation and optimisation of future business processes and current and future Telematics systems. We recommend adopting common and harmonised standards as well as a holistic approach to Telematics systems across Europe. The implementation of different systems for the same regulatory purposes can generate unnecessary complexity and impede trusted access to information, restricting the flow of data between authorities, industry and patients. Therefore, we recommend using a holistic and harmonised approach to all on-going and future Telematics projects with a focus on data quality, interoperability and inter-dependency of Telematics projects, when needed.
-The future telematics strategy should also provide a strategic view of moving from a document-based review towards a structured data-based review. Our long-term vision is that regulatory submissions should be paperless (enabled by the removal of any national requirements), with a direct exchange of structured-data between Industry and Agencies. Ideally one single communication channel should be foreseen. Improving interconnection between EMA, NAs and MAHs via digitalisation and submission and re-use of structured data is essential to support better outcomes and efficiency in the Regulatory network.
The implementation of the Target Operating Model (TOM) for ISO IDMP is critical for the use and acceptance of the data elements and processes. For TOM implementation, the entire process, from development, registration, to placing the product on the market, including the prescription phase, should be analysed, discussed and agreed with Industry Stakeholders. This programme should be a priority for Regulators and Industry. Achieving a more agile regulatory Telematics system will improve the efficiency of the Regulatory network with the final goal being to improve public health for the benefit of patients.

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:

2.
The life-cycle of innovation evolves towards multi-source medicines being supplied once intellectual property, regulatory and market exclusivity periods have expired. The EU regulatory framework continuously evolves, adjusting to emerging challenges and opportunities.
The creation of regulatory pathways that allow a life-cycle of innovation and the increase of patient access to modern therapies, by supporting a multi-source environment once the market exclusivity is over, is key to sustaining healthcare systems.
There is a further need to design regulatory pathways to enable future developments in the off-patent sector in line with future opportunities. High costs of novel therapies are placing stress on healthcare budgets, which we can observe in different areas (e.g. targeted therapies, oncology products, orphan medicines, Advanced Therapy Medicinal Products -ATMPs) and a decreasing number of patients per product are being catered for. Therefore, to further improve access, frameworks need to be established or re-shaped to allow a natural evolution of the innovation life-cycle.
Areas for consideration include, but not are limited to: repurposing of existing active substances (e.g. extending parallel consultation to repurposed medicines), orphan, ATMPs, paediatric, geriatric medicines.
Advanced therapy medicinal products undeniably present a paradigm shift in healthcare. However, the high cost of ATMPs is putting the sustainability of healthcare systems and accessibility of those products under question. In addition to supporting the translation of ATMPs into patient treatments, EMA should also consider developing a regulatory framework that will support a multisource environment of ATMPs that will lead to a competitive market and affordable therapies in the future.
The off-patent medicines development and registration paradigm is different from that of New Chemical or Biological Entities. (NCEs, NBEs) and requires fit-for-purpose designs.

5.
We support EMA in developing competence and expertise in the field of complex products that combine medicine and a medical device. This type of product is not solely reserved for the innovative medicines sector but is also common in generic, biosimilar and value-added medicines development. Furthermore, single integral products (Medical Device Regulation, MDR, Article 117) should be expressly included and the scope should not be limited to complex products since non-complex products will also benefit from these measures, in the light of changes brought about by the implementation of the MDR.

7.
We welcome the agency recognising the need for earlier and more frequent dialogue to foster development, improving trial designs and avoiding unnecessary trials in patients/healthy subjects while maintaining appropriate safeguards. We encourage the agency to clarify or define data ‘thresholds’ – e.g. what is the data that needs to be generated by the sponsor in order to make meaningful scientific advice possible. We also encourage EMA to build further on tailored scientific advice to support step-by-step development of new biosimilar medicine candidates as well as value added medicines with known active substances.

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**:

10.
Technology that supports clinical trials comes not only in the form of methods of data collection (such as wearables) and new endpoints, but also as alternative approaches complementing clinical trials or as novel technologies that support the running infrastructure of traditional trials, such as electronic consent forms, communication with electronic health records, etc. These technologies can have a positive impact on the efficiency of classic clinical trials and should also be part of the EMA priorities.

13.
We welcome the initiative to validate new tools to demonstrate bioequivalence of complex generic products, which could potentially decrease the cost of development and advise the agency to investigate further into the optimisation of the regulatory framework. An example of an innovative approach of successful implementation in the US is the approval of generic glatiramer acetate without any supporting clinical study. This links into the need for harmonisation of regulatory requirements to allow for global development of a product to allow faster access for patients to generic medicines. Off-patent medicines could be a learning opportunity on gathering evidence through real-life use data and modelling, simulation and extrapolation - as there is already a lot of data gathered on quality, manufacturing, safety and efficacy.

14.
We call for a proportionate consideration of the strategic use of big data in both the known and unknown territories to ensure that the progress and optimisation made in the known field can efficiently help redirect needed resources to the great challenges of the unknown.
Off-patent medicines producers typically generate a vast amount of information / data on the great number of medicinal product batches they manufacture and release for patient use; all contributing to the collective knowledge of a given molecule. Big data and real-world evidence should be further used to avoid unnecessary repetition of studies and generation of data which are already known but may not be sufficiently well collected and analysed.

It is crucial to keep in mind that a multi-source environment requires different approaches and a different data source to innovative medicines. This should be a base concept in designing and preparing the decision-making process of the regulatory environment, using the benefits of Big data.
Regulatory consistency will be achieved by learning actively from the system by optimising processes, creating fit-for-purpose requirements and risk-based approaches and taking into account the available body of evidence.
Factoring in the Integration of Artificial Intelligence (AI) to regulatory processes and decision making will be important for the EMA to be future proof.
Our proposal is for EMA to consider piloting the AI involvement in regulatory decision making with an off-patent registration initiative, particularly where tailoring or regulatory science advances would be integrated. That way progress is made on innovative tools yet with lower risk, well known and understood candidate medicines.

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**:

18.
The set of recommendations provided in a Big data Task-force report/summary report represents a good starting point to improve regulatory efficiency and the regulatory decision-making process. We also welcome a data-sharing culture that could inspire all the regulatory network and stakeholders involved, with the condition that patients' privacy is protected. However, to use RWD in the decision-making process, the regulatory environment needs to be prepared to validate and ensure reliability, quality and regulatory compliance of the data. It is crucial to keep in mind that a multi-source environment requires different approaches and a different data source to innovative medicines. This should be a base concept in designing and preparing the decision-making process of the regulatory environment, using the benefits of Big data.

20.
Delivering real-time information in the form of electronic Patient Information (ePI) will be of great benefit to the patient. We fully support greater focus on patient engagement by providing real-time/up to date /regulatory approved patient information which is user friendly and understandable and has great potential to improve patient adherence. ePI should also represent a tool which guarantees a stronger connection between all stakeholders. ePI is the best example to show how regulatory efficiency and empowering patients in the Health system have a common pathway. As recognised in the EMA key principles document, this programme should "offer possibilities to streamline, simplify and speed up the regulatory process in the creation and updating process (variation) of Pi, just using existing data of SPOR…both for regulators and the pharmaceutical industry". The opportunities that ePI could generate in the health system for patients and the whole regulatory network are great. Therefore, the process of delivering ePi is critical. This programme should be designed with other ongoing or future telematics projects in mind. It is important to avoid the risk of starting a new initiative and working in a silo. There is also the high risk of new technology advances by the time of its implementation. TOM and its potential optimisation of the variation process represents a very important stepping stone also with regard to the eSmPC/ePIL/eLabel project. TOM would improve the speed of updating patient information dramatically and reduce the effort for preparation and review by Industry and Agencies, with benefits for all actors involved. The future Telematics Roadmap should include this project and its interconnection with TOM and other linked programmes.

22.
While the EMA 2025 strategy looks towards improving regulatory frameworks and communication towards stakeholders, it lacks assertiveness. A lot was done in the past to put Europe at the forefront of regulatory science and in terms of creation of communication tools and videos on regulations, pharmacovigilance systems etc. We recommend further strengthening confidence in the existing regulatory framework, reassurance of the system's quality should be reinforced and acknowledged throughout the strategic paper. Further strategic communication campaigns to reinforce trust and confidence of stakeholders is one of the key roles EMA can play. The European pharmacovigilance system is one of the opportunities for EMA to showcase what has already been built in gathering real world data.

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.
One of the biggest healthcare threats to modern societies is AMR and, while we are looking for novel antimicrobial therapies, we should also preserve what we already have.
Some of the older generation of antibiotics that are currently not being used as common first-line or second-line treatments risk disappearing from the market.
To preserve future sources of off-patent antimicrobial agents, we encourage EMA to open a discussion on possible incentives to maintain the marketing authorisations for this very important group of medicines. New systems can help address the global threat of antibiotic resistance by leveraging existing antibiotic products.
The recognised global public health threat of AMR already causes 25 000 deaths in the EU and 700 000 deaths globally per year and may cause up to 10 million deaths annually by 2050.
Using existing antibiotics properly is a critical component towards building a lasting strategy to combat AMR.
Off-patent and generic antibiotics may be at risk of market exit due to low margins, prescribing behaviours and other factors. Indeed, antibiotic shortages have been observed across the EU and around the world.
Some of the oldest antibiotics, often called "forgotten antibiotics," are particularly effective for resistant bacterial infections, but are the most vulnerable to market exit. When prescribers are forced to use suboptimal treatments due to unavailability, it is costlier and may accelerate the development of AMR.
In order to ensure that effective antibiotics are available to address the threat of AMR now and in the future,

EMA should:

- develop an evidence-based list of critical off-patent antibiotics with a multi-sector stakeholder group;
- evaluate the potential for a scientific approach to antibiotic cycling or rotation schemes;
- prevent future market exit by providing targeted regulatory relief for MAHs of critical antibiotics. EMA could do this through decreased cost of maintaining authorisations via a reduction in post-approval regulatory fees or an introduction of a special reduced annual fee structure applicable to antibiotics for these vital public health products;
- optimise the regulatory pathway for older antibiotics that have previously been unavailable in some or all European markets and provide incentives by means of a reduction in regulatory fees for Marketing Authorisation Applications; and
- work with the European Commission and member states to create a framework of procurement incentives for off-patent and generic antibiotics such as multi-winner tenders and non-price selection criteria, to ensure a stable supply of highly-effective antibiotics.

25.
We strongly support EMA in the objective to increase availability of medicinal products in the EU. EMA should have a stronger focus on leading and championing the exchange of information on medicines shortages. The agency is the privileged entity that has access to information that competitors do not or rather cannot have. For that, it should take the lead in connecting information on the market and manufacturing, improving the management of shortages.
EMA should clearly state the need for harmonisation of definitions and procedures regarding availability at European level. Regulatory incentives for established, but essential, generic medicines should be considered and promoted as a strategic decision to the National Competent Authorities (NCAs).
Unavailability is not related solely to shortages, there are many contributing factors. Where urgent actions are required to be taken in the supply chain (e.g. non-compliance of manufacturer), Medicines for Europe is looking towards a more transparent discussion between inspectors and Marketing Authorisation Holders (MAHs) and having transparent mitigation plans for both regulators and MAHs to take firm and risk-based decisions.
We should avoid potential regulatory barriers that could contribute to shortages. Regulatory science should contribute to a more scientific/risk-based approach to Environmental Risk Assessment (ERA), instead of applicability to all products in the same way and independently of their impact on the environment (high/low risk). The recommendation for a risk-based approach to ERA and the need for more detail on ERA Waiver of Requirement for ERA Studies for Generic products should be taken into careful consideration to avoid high costs of repeat ERA studies with no value-added benefit to either the patient or the environment, as well as the creation of guidelines on sharing the data among different MAHs.

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**:

We support the importance of regulatory science remaining at the cutting edge so that EMA can deliver its fundamental mission of protecting human and animal health and facilitating the availability of medicines to patients.
Most recent scientific advances can support innovation in off-patent, biosimilar and value added medicines sector and present possible solutions to regulatory needs and challenges in creation/design of new models as well as offer opportunities for optimisation of the existing systems, by releasing resources where scientific and technological advances allow for optimisation, by leveraging novel technologies and scientific research.

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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