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Public consultation on EMA Regulatory Science to 2025

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* 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

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

Name of organisation (if applicable):

Janssen Pharmaceutical Companies of Johnson & Johnson

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.

Janssen welcomes the strategy proposed in EMA's Regulatory Science to 2025. The proposals should help to ensure regulators are ready to support the development of increasingly complex medicines to promote and protect human health, to which our industry is committed.

We highlight the following points:

- The strategy is ambitious and commitment to clear top priorities will be needed to allow stakeholders to also align so that real progress is made on these.
- For several of the core recommendations the EMA is proposing the development of guidance, for example digital technologies, novel trial models, digital biomarkers and digital therapeutics. This may be the appropriate action in some areas, but we also suggest some reconsideration of this approach in case of non-mature and fast evolving areas which will require agility and flexibility. In such areas there may be other approaches such as workshops and position papers to promote discussion and consensus-building. Formal guidance can then be a longer-term objective.
- A global approach is important, recognising local variation, but reducing complexity for the Industry in regional differences.

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.

(Please note that we consider Strategic goal 1 to be appropriate but we have checked the 'No' option above to allow entry of some comments).

The areas identified align with pharmaceutical industry trends in innovation and anticipated areas of growth in near future. Health authorities should be addressing these to ensure that current or future regulatory pathways are nimble and flexible enough to accommodate these evolving tools and technologies, secure sufficient resources and expertise to be able to aid in the development of future products and proactively engage with industry on shaping these emerging fields.

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.

(Please note that we consider Strategic goal 2 to be appropriate but we have checked the 'No' option above to allow entry of some comments).

Strategic goal 2 is appropriate. While the pharmaceutical industry is traditionally conservative in nature, there is a vast opportunity to adopt new approaches in generating evidence. In particular, this means harnessing the exponential increase in new technology that is becoming available and encouraging uptake of complex trial designs (such as master protocols, adaptive design, etc) as well as the use of modelling and simulation to enable decision making, novel endpoints and use of biomarkers. Where possible, this should be accomplished on a collaborative basis to develop harmonized and streamlined approaches for the benefit of all e.g. consortia applications for relevant Qualification Opinions. This is necessary to address the urgent need to facilitate earlier patient access to medicines for unmet clinical need. It is noted that the goal refers to the provision of better evidence to other stakeholders such as HTA bodies, however, the supporting core recommendations have limited reference to addressing the needs of these stakeholders. It is also noted that in light of recent developments with novel treatment modalities, information sharing is essential for rapid learning about novel treatment paradigms. Consideration of public repositories with population level baseline data to serve as comparator for novel treatment options will be important.

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.

(Please note that we consider Strategic goal 3 to be appropriate but we have checked the 'No' option above to allow entry of some comments).

This goal is an appropriate and important goal for the EMA that is strongly supported as it focuses on the patient as key stakeholder. It takes into consideration that patient access requires endorsement from, and collaboration with, multiple stakeholders in healthcare systems beyond the regulators/ a regulatory approval. It also builds upon ongoing collaborations with the EMA and HTA bodies. In several places, the EMA strategy document refers to collaboration with HTA bodies and payers. While EMA-HTA body collaboration on matters of scientific advice is supported, it is not considered to be appropriate to extend this to payers, the decisions of which are guided by the input of HTA bodies and, indeed, made on a national basis.

As stated in the strategic reflection document, patients and other stakeholders in the healthcare system need to be at the centre of regulatory decisions and actions to be able to advance patient access to medicines. All those stakeholders have their perspective that they bring to the table and cooperation between them would enhance the decision-making processes; it would also educate each individual stakeholder as to what other stakeholders deem to be important to them. More attention to patient focused drug development, patient engagement and patient-centered access could be introduced across the recommendations.

The EMA strategy document does mention healthcare professionals (HCPs) as one of the (important) stakeholders in the introductory paragraph to Goal 3. However, we consider that this point is not carried through and there are not any recommendations focused on HCP involvement and on the role of HCPs in evidence generation and access. We propose this should also be considered.

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.

(Please note that we consider Strategic goal 4 to be appropriate but we have checked the 'No' option above to allow entry of some comments).

Inclusion of this goal is welcomed. The resurgence of diseases and impact of climate change and migration patterns/quantities will have to be understood, both for population level healthcare consumption and portfolio of treatment options. The core recommendations are focused on actions within EMA but emerging health threats are a global concern and should be approached accordingly. EMA could consider augmenting their leadership role through coordinating preparedness for potential health threats with international regulators, WHO and other interested parties involved with healthcare in low- and middle-income countries e.g. non-governmental organisations, Gates Foundation etc.

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.

(Please note that we consider Strategic goal 5 to be appropriate but we have checked the 'No' option above to allow entry of some comments).

This is an important goal. Additionally, the role of public private partnerships in medicines research is important to foster synergies amongst several stakeholders with a collective aim of facilitating patient access to medicines where there is unmet need.

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)

9. Foster innovation in clinical trials

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.

9. Foster innovation in clinical trials

The recommendation to foster clinical trial innovation is strongly supported. This core recommendation is intrinsically linked with the core recommendations of “13. Optimise capabilities in modelling, simulation and extrapolation” and “14. Exploit digital technology and artificial intelligence in decision making” and therefore the underlying actions associated with these are also a priority. The increase in availability of digital technology is such that its role in collecting endpoint clinical trial data as well as real world data is becoming of increasing importance, especially in relation to demonstrating reimbursement value. There is also a need to facilitate implementation and acceptance of new complex trial designs as well as facilitate increased use of modelling and simulation in decision making.

A number of missing elements have been identified in relation to fostering clinical trial innovation (see also Question 7). In particular the following are most critical:

- Operationally, it should be noted that the conduct of complex clinical trials potentially will be hampered by interpretation of the Clinical Trial Regulation such that parallel substantial amendments are precluded. Action to address this pragmatically would be beneficial.
- EMA/FDA parallel scientific advice on novel approaches is currently challenging to accomplish due to the usual timing of EMA scientific advice. It is therefore recommended that EMA allows for earlier access to scientific advice consistent with that in the USA.
- Consideration could be given to developing guidance on approaches such as the use of Bayesian methods for design and analysis, hierarchical modelling for borrowing historical control, synthetic control arms, etc.
- While discussion of innovative designs is an option via the Innovation Task Force, EMA could initiate a pilot programme that would allow for broader discussion and shared learning relating to novel designs.
- Consideration should be given to the practical application of the orphan drug regulation when addressing tissue agnostic indications.

Regular interaction with other stakeholders such as HTA bodies and to facilitate acceptance of novel approaches to clinical trials, share key learnings from pilot programmes and foster mutual understanding of data requirements.

Second choice (h)

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

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

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

The proposal to promote the use of high quality RWD in decision making is strongly supported. This core recommendation is directly linked to that of “Develop network competence and specialist collaborations to engage with big data”. EMA is encouraged to provide guidance on scenarios where RWD will be acceptable in the support of regulatory decision making and to build on its work on patient registries ensuring that its scope encompasses the broader range of RWD sources. The proposal for capacity building across all aspects of RWD/big data collection, access, curation, interoperability, analysis etc is supported. While the HMA-EMA Joint Big Data report is welcome we also recommended more specific deliverables be adopted.

EMA could also consider the following:

- Introduce a pilot programme for RWE case studies to explore how RWD can be better incorporated into decision making stakeholders.
- Develop a framework for using RWD in decision making including appropriate guidance on how to achieve labelling (changes) with the support of RWD such as by identifying the type of outcome measures that can appear on the label.
- Consider further exploration of the development of post licensing evidence generation (PLEG) or related evidentiary measures not only within Europe, but across different regions.
- Collaborate with regulatory authorities in other regions as well as other stakeholders to facilitate the development of harmonised approaches (where appropriate) in the future.
- Collaborate with regulatory authorities, especially Data Protection Agencies, and where appropriate European Data Protection Board (EDPB) to facilitate a harmonised approach regarding the use of RWD/big data, especially the re-use of data for secondary purposes, and diminish barriers that might hamper big data use.
- Promote adoption of a common data platform.

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.

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

The recommendation to exploit digital technology and artificial intelligence in decision making is considered to be a key priority and note that this recommendation is intrinsically linked with other recommendations, in particular: 10. Develop the regulatory framework for emerging clinical data generation, and also recommendations 1, 5 and 9. All these recommendations involve leveraging digital technologies, both for drug development by innovators and assessment by regulators. These new technologies raise a number of legal and regulatory challenges and uncertainties (qualification and integration with other regimes, regulatory requirements, assessment etc.) which may in turn hinder innovation. These are important as pharmaceutical companies are increasingly developing personalised medicines, gene therapy etc. 'Digital Transformation' is a key strategic theme to be successful in delivering innovative medicines to patients in the future.

Much work is already being done without a clear regulatory framework being in place. The digital and data world is evolving very rapidly which hinders adoption and has a broad impact on research quality, costs as well as speed. In turn, this adversely impacts the entire downstream life sciences value chain. A regulatory framework is required for not only emerging clinical data generation but also re-use and sharing is key. New digital technologies and AI touch upon regulatory, privacy, transparency and ethical concerns that are currently not being addressed in existing laws & regulations (the Clinical Trial Regulation and GDPR do not anticipate this). Harmonisation and further guidance is welcomed e.g. use of blockchain technology for patient data donation and consent tracking.

Increasingly large volumes of clinical data are being generated, enhanced by that available from wearables. Purely statistical techniques could produce an explosion of hypotheses with the testing hierarchy or multiplicity tests being ill-suited to the resulting potential insights. As such, a new approach is required that is acceptable to regulators and allows for more than one definition of study success. Supporting these analyses with a data capture and integration framework that the EMA trusts would be highly valuable on its own but will also yield dividends that support many of the other components of goals 2 and 3. The need to address the regulatory framework is especially important for PLEG.

The following is encouraged:

- More position papers, guidance that outlines the EMA's recommendations for using digital technologies and novel trial models. More proactivity on the part of EMA is encouraged as currently much of the initiative on this topic comes from industry.
- More accessible mechanisms to engage with the EMA regarding specific plans for trials, programmes and novel endpoints.
- More flexibility to consider other evidence-based measures as endpoints and capture mechanisms for key endpoints.
- EMA should increase transparency regarding the acceptability of novel approaches.
- Consideration of ethical and transparency issues associated with the use of data obtained via the artificial intelligence approaches.
- Consideration of Privacy/Regulatory issues and further guidance would be welcomed associated with use of data & sharing of data obtained via new technologies. New data sharing technologies might facilitate or promote the use of high quality RWE/RWD. We encourage exploiting the use of digital technologies & AI in decision making, but not only in decision making, also the use of AI and new digital technologies for (digital) clinical data generation and re-use of that data via new digital technologies, like Blockchain, that are being used to support decision making. The bar should be set as high for everyone and this can be a competitive (dis)advantage.

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

It is considered that the EMA has captured within its overall strategy, the key issues facing the pharmaceutical industry currently.

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:

Rec. 1.

Disease classification is evolving rapidly. By integrating knowledge and tools based on evidence in precision medicine, biomarkers etc. and applying it to human therapeutics, significant public health benefits could be gained. A huge amount of data is now available, and further development requires academics, regulators and industry to collaborate on addressing the challenges and develop the science and novel human therapeutics with improved benefit-risk ratios. Fundamental academic biomarker research should be stimulated. Use of clinical meaningful digital data such as endpoints, personalised treatment and remote monitoring should be stimulated.

The EMA biomarker qualification procedure could be shortened with greater flexibility or a different pathway to develop biomarkers offered, outside of the qualification procedure, to speed progress.
With advancements in precision medicine, we should consider how specific mutations within a condition can be appropriately addressed under the orphan medicine framework.

Rec. 2.

Goals identified within this initiative align with Janssen's priorities.
Support evidence generation, pertinent to downstream decision-makers: This is understood to refer to generating a data package to secure regulatory approval and address HTA queries sooner. Approval alone is not sufficient to make the product available to patients, alignment with HTA bodies is essential. This includes the role of real world evidence as part of the evidence package, prior to and after marketing approval.
Identify therapies that address unmet medical need: It is recommended this action is undertaken in collaboration with other stakeholders, including industry, to provide a wide range of perspectives.

The hospital exemption pathway should be clarified with more protection for products with marketing

authorisations. There are questions on quality and potential impact on patient safety and efficacy of products manufactured via this pathway.

Rec. 3

Evaluation of current capacity and further enhancement of PRIME is welcomed. Early application to PRIME would enable products in fast track development to benefit from the interaction e.g. oncology medicines. Administrative duplication within the scheme could be reduced e.g. automatic eligibility to scientific advice would reduce complexity and shorten timelines. Allowing topics to be addressed as they arise instead of pooling these for formal scientific advice would enhance the scheme.

HTA involvement in PRIME is crucial to consider their needs and to agree, early, a development plan. Leveraging collaboration with international partners to further development PRIME is also important as patients in all regions should have timely access.

Rec. 4.

Higher quality medicines and more reliable/efficient manufacturing processes requires implementing novel technologies. Current guidance/regulation may be inadequate or even hinder such implementation. EMA should facilitate implementation by easy and early access to discuss new manufacturing/control strategies and technologies.

Rec. 5.

The EU IVDR has significant implications on the regulation of companion diagnostics and an efficient and predictable regulatory review process and co-ordination by medicines regulators, Notified Bodies (NBs) and developers is essential to ensure a workable and flexible pathway and allow rapid access of innovative targeted medicines. Guidance addressing roles and responsibilities, process, bridging studies and follow on test panels will be key. Coordinated action across Member States to ensure rapid access of both medicine and companion diagnostic test to patients is needed.

The EU MDR also brings significant changes for drug device combinations and clear co-ordination between NBs/ medicines regulators is critical.

Guidelines for evaluation of digital biomarkers and digital therapeutics are required, to clarify regulatory pathways and interactions between NBs and medicines regulators for these innovative products.

Rec. 7.

The EU regulatory system contains different provisions to enable early patient access and there are many touch points where industry can seek advice in the development and post authorisation phases through EMA, parallel with FDA, jointly with HTAs or nationally. Interactions can, however, be isolated from each other resulting in disconnected advice. It is important to link different engagement points for a more integrated and continuous dialogue, especially as the complexity of development increases with scientific and technological advances. It should also look to best leverage the expertise across the network.

Advice mechanisms that expand multi-stakeholder platforms are important for consistent advice. A more flexible system is also needed to support agile and quick development decisions. The ITF should be integrated into other advice platforms and advice on more general topics or concepts should be afforded.

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**:

Rec. 9.

This recommendation is supported. Digital technology is increasingly used to collect trial endpoint data and RWD. Wearables facilitate collecting extensive data but their use necessitates novel endpoints.

New complex trial designs that streamline development also need acceptance. Using “omics” facilitates developing precision medicines for patient subgroups with different treatment responses and predicting response downstream, compared to current approaches.

Other considerations:

- Proposed actions could be more specific e.g. possibly prioritising innovations scenarios where collaborative trials are encouraged; etc.
- Developing guidance e.g. use of Bayesian methods for design and analysis, hierarchical modelling for borrowing historical control, synthetic control arms, etc.
- Allowing earlier scientific advice thus facilitating early EMA/FDA parallel advice.

- Engage with patient organisations e.g. when developing novel endpoints.
- Initiate a pilot programme to facilitate broader discussion and about novel complex designs.
- Consider the practical application of the orphan drug regulation when addressing tissue agnostic indications.
- Clarifying the objective about adopting novel practices that facilitate clinical trial authorisation.
- Adopting a pragmatic approach to allowing parallel substantial modifications under the Clinical Trial Regulation, to facilitate operation of complex trials.
- Revising clinical guidelines to allow for new endpoints associated with digital technology.
- Taking into consideration and complementing other ongoing EU activities e.g. IMI.
- Future ICH guidance on complex designs (when more experience is available).

Rec. 10 and Rec. 14.

These recommendations are supported. Much work is being done in the absence of a clear regulatory framework including one that addresses privacy issues. The digital world is evolving rapidly, hindering adoption and impacting research quality, costs as well as speed.

Increasing amounts of clinical data are being generated, including that from wearables. Purely statistical techniques could produce many hypotheses with the testing hierarchy or multiplicity tests not suiting resulting potential insights. Thus, a new approach is required that allows for more than one definition of study success. Supporting these analyses with a data capture and integration framework that EMA trusts would be valuable on its own but will also benefit some other components of goals 2 and 3. The regulatory framework for PLEG is also important. In future, digital transformation will be key to delivering innovative medicines.

Other considerations
- More position papers, guidance on using digital technologies and novel trial models e.g. virtual trials, how/how much digital data are collected.
- Introduction of/piloting alternative mechanism to manage large submissions e.g. cloud submissions.
- Alternative ways to discuss trial plans, programmes and novel endpoints with EMA.
- More flexibility on use of other evidence-based measures as endpoints and capture mechanisms for key endpoints.
- More EMA transparency on acceptability of novel approaches.
- Collaborate with regulatory authorities, especially Data Protection Agencies and, where appropriate, EDPB to facilitate a harmonised approach to use of RWD, especially data re-use for secondary purposes, and diminish potential barriers to RWD use.

Rec. 11.

This recommendation is supported. EMA has addressed certain aspects of benefit-risk (B-R) assessment. However, effects tables are often insufficient to render a B-R decision. A structured approach for the assessment, (not tabulation of key B-R data), is needed. This should be suitable for sponsor use and not be a regulators' communication tool, as currently.
Currently, patient preference studies are conducted and submitted to EMA with no EMA response. Multiple stakeholder consider patient preference studies but, without regulatory guidelines for their assessment and application, the output is often not used. This is a loss for patients. Also regulators cannot defend subjective assessment and sponsors cannot demonstrate objectivity in weighting benefits and risks.

Rec. 13

Increased use of modelling and simulation (M&S), which is linked to complex trial designs, real world data,

etc, is supported. Extrapolation facilitates development in new indications and populations and broadens knowledge across multiple products used for the same indication.

Other considerations:

- Accepting modelling (and surrogate) endpoints for clinically relevant outcomes measures.
- M&S use in longitudinal dose response analyses in Phase 2 trials; use within adaptive designs, etc.
- Increased EMA network expertise in applying M&S and evaluating/interpreting M&S results.
- Collaborating more with other regulatory authorities regarding acceptance of innovative approaches.
- Collaborating more with other partners e.g. IMI.

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**:

Rec. 15.

Continued interaction of EMA and HTA bodies is supported, as streamlining decision making will facilitate earlier access to medicines and minimise duplication of development effort. However, while EMA can contribute to the process of planning for generation of relevant evidence for HTAs (and regulators), ultimately the developer is responsible for development plans. For clarity, it is recommended that a distinction be made between the role of HTA bodies and payers.

Missing elements: Suggest focus on opportunities for early dialogue/parallel consultation with all stakeholders.

Rec. 16.

Continued cooperation of EMA and HTA bodies is supported and proposals for a single platform with one evidence generation plan which could enable pricing models, is welcomed. It is helpful to share the rationale for authorising a particular patient population and it is suggested that EMA should focus more on this. However, it is not appropriate to extend this to payers, decisions of which are guided by input of HTA bodies and are made nationally.

Rec. 17.

This recommendation is supported, as if this is achieved more meaningful data can be collected, which HTAs and regulators are more likely to accept.

The actions proposed are supported, with the following comments:

- The patient perspective should be already incorporated into development and qualification of PRO measures, so there should be clarity on additional measures proposed.
- Co-development of a core health-related quality-of-life PRO with HTAs is supported, but it is not clear if the proposal is ONE core PRO along with HTAs, or core SET of measures. We suggest considering how robust/meaningful the comparison is e.g. across different diseases.

Missing elements:
Consider the ability to bring all stakeholders together and harmonise for regulatory, HTA, clinical, and patient decisions. Consider what other regulators e.g. FDA are doing, such as harmonised measures.

Rec. 18.

This recommendation is supported, in particular for efficacy/effectiveness decisions and complementing randomised controlled clinical trials, as this is typically accepted in limited circumstances. EMA is encouraged to provide guidance on scenarios where RWD will be acceptable to support regulatory decision

making. EMA could build on its patient registries work to encompass the broader range of RWD sources becoming available through new technologies in a GDPR compliant manner. Implementing a learning regulatory system is also supported, for which availability of suitable data is key, and thus recommends EMA supports activities relating to generating a common her format via the Transformation of Health and Care - Digital Single Market.

Missing elements

- Consider a pilot programme for RWE case studies to explore how RWD can be better incorporated into stakeholder decision making. This should include considering the possibility of more frequent EMA interaction on study design, etc, in order to facilitate shared learning.
- Consider developing a framework for RWD use in decision making, including guidance on labelling (changes) with support of RWD and types of outcome measures permitted on the label.
- Consider developing PLEG or related evidentiary measures across different regions to establish a global evidence base.

Rec. 19.

The proposal for capacity building for RWD/big data is supported and it is advocated that EMA develops expertise across all aspects of collection, access, curation, interoperability, analysis, etc. The HMA-EMA Joint Big Data report is welcome however we recommend adopting more specific deliverables. Janssen agrees that trust of patients (and health care providers) is fundamental to ensuring that RWD are collected and shared.

Missing elements

Propose EMA should:

- Collaborate with regulatory authorities in other regions to facilitate harmonisation.
- Promote adopting a common data platform and identify criteria for RWD for decision making.
- Engage with the EC to encourage adopting recommendations on healthcare practice to promote data sharing.

Rec. 20.

The ePI initiative is supported, as it offers several benefits. A framework for access is critical. The ePI platform and its governance should be determined.

Comments on actions:

- Clarify real-time interactivity and consider that personalised viewing could be a later step.
- Add “Manufacturers” to the action to develop a strategic plan, as industry needs input.
- Clarify if there would be a standard interface for all manufacturers, which is needed.
- Clarify ‘reuse’ to ensure accessing of the current version directly from the central repository. A standard interface between the central repository and third-party apps is critical, to ensure real time availability of current approved PI.

Missing elements

It is unclear if the intention is to replace completely the paper Package Leaflet within the pack; consideration should be given for patients with no electronic access.

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**:

Rec. 23.

It is important for EMA, industry and global public health stakeholders to work together to overcome factors such as:

- Types of epidemics (e.g. Ebola) that have become difficult to contain.
- The lack of R&D in diseases that impact the developing world.
- The population within the EU will over time changing due to migration and therefore healthcare needs of the population will also increase e.g. potentially diseases not traditionally prevalent in the EU may increase over time e.g. multi-drug resistant tuberculosis. "Climate change, emerging diseases, exploitation of the rainforest, large and highly mobile populations, weak governments and conflict were making outbreaks more likely to occur and more likely to swell in size once they did" (Dr Michael Ryan, WHO; BBC.co.uk).

Suggested areas of focus:

- Broaden the scope from "vaccines" to "vaccines and drugs" to "prevent" and "treat" infectious diseases.
- EMA to invest more resource into training regulators / scientists in partnership with WHO to enable healthcare systems in low- and middle-income countries (LMIC) cope with health emergencies.
- Consider modifying current procedures e.g. combining prequalification & registration procedures that involves EMA, WHO, country regulators for products that are important for the LMIC.
- Focus on prevention e.g. accelerate development and approval of vaccines - consider updating the

guidance on vaccine development for disease specific and general guidance. To address concerns surrounding manufacturer liability for vaccines and to find better means of ensuring safety and protecting for vaccine recipients, EMA could help drive the debate with national EU and supranational bodies to help design and put in place appropriate indemnification mechanisms in the world for when non-licensed vaccines would be used to tackle outbreaks.

It is important for EMA to partner with the public on changing healthcare needs within the EU and what that means for the population. Communication is key on the need for vaccines to be administered especially for children as an important prevention tool and the benefit vaccines have provided especially with respect to childhood diseases.

Rec. 25.

Supply problems cannot always be anticipated, and when they occur they need to be addressed urgently. Therefore, regulatory mechanisms need to be developed to allow alternative supplies of the medicinal product in an expedited manner.

Rec. 26.

Ensure patients have access to the most innovative prevention therapies and consider options to accelerate vaccine development and approval. While supply challenges and shortages need to be adequately addressed in the public health interest with all relevant stakeholders, obligations should not be imposed on the marketing authorisations holders that go beyond legal requirements and their current scope of responsibilities.

Suggested areas of focus:

- Develop guidelines to allow for harmonized GMO assessment across the EU instead of individual national procedures- these latter can be time-consuming.
- In light of developing technologies e.g. platforms used in vector (including bacteriophage) derived medicines develop guidelines that enable the concept of a “drug master file” type approach to be used across different clinical trial applications for different vaccines using the same vector (e.g. stability data, tox data, safety).
- Consider early PRIME applications of non-SME companies for vaccines based on proof of principle data. The substantial time needed for vaccine clinical studies & development costs makes it challenging for companies to develop vaccines; this would allow for regulator input on vaccine innovative clinical trial design early in the development. This could shorten the timeline to bring vaccines to the people.

EMA is well-positioned to help build public trust and overcome vaccine hesitancy and a broad communication strategy would be helpful i.e. reaching out to all stakeholders (healthcare workers, patients/ general population/ Government agencies responsible for healthcare).

The clinical landscape is changing more quickly than the regulatory framework within the EU; EMA could consider closer engagement with other regulatory bodies such as WHO, national African regulators to adapt its thinking and to influence as needed.

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**:

Rec. 28.

The role of public private partnerships in medicines research is supported. These foster synergies amongst stakeholders who have a collective aim of facilitating patient access to medicines where there is unmet need. Specifically, it is noted that the output of many Innovative Medicines Initiative (IMI) projects comprised tools and methods with a potential regulatory impact. IMI has developed guidance for projects to raise awareness of the various opportunities to interact with regulators in the framework of research on regulatory sciences with a potential impact on public health. We encourage EMA to post this guidance on its website.

The recent joint statement between EMA and new HCP groups (GP/primary care physicians), committing to strengthening interactions, is welcomed. In particular, sharing best practices on the collection of data generated in clinical practice (eHealth records, registries, etc.) is viewed as an opportunity to address fundamental aspects related to quality of RWD.

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