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Public consultation on EMA Regulatory Science to 2025

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* Name

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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

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

Name of organisation (if applicable):

Novartis Pharma

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.

Novartis welcomes the opportunity to comment on EMA Regulatory Science to 2025. As a global healthcare company, we generally support EMA's vision for the upcoming years; which will serve to contribute to a competitive forward-looking regulatory system that is more agile and rapid to embrace science and technology. However, we also note that this strategy is very ambitious and will require major prioritization in order to realize the aspirational agenda.
Some core recommendations are clearly interrelated and interdependent. In practice, pursuing one recommendation may require the need to progress others. Thus, Novartis have taken the liberty of connecting some recommendations together in our comments.

Furthermore we highlight the importance of also incorporating NCAs perspectives as EMA will be unable to deliver on this strategy without NCAs involvement and collaboration as integral contributors of the EU network

Given the global context in which innovation is taking place, we believe this strategy should reflect greater focus to enhanced international cooperation and the alignment with regulatory science and regulatory frameworks in other jurisdictions.

Novartis is a member of EFPIA (European Federation of Pharmaceutical Industries and Associations) and is fully aligned with the comments submitted by EFPIA on this strategy. We wish to highlight in this response comments based on Novartis's priorities. Accordingly we provide input in questions 5 and 7 as below:

- What is very important (our company's top priorities – see question 5)

1. 3.4. Promote use of high-quality real-world data (RWD) in decision making
2. 2.2. Foster innovation in clinical trials and 2.3 Develop the regulatory framework for emerging clinical data generation (these two core recommendations are so closely linked that we considered them together as our second priority)
3. 2.7 Exploit digital technology and artificial intelligence in decision making

- What is also of importance to Novartis (albeit not given the highest, very important designation) – see question 7

We do not provide comment on other recommendations.

Novartis understands that it will be impossible for EMA to embrace all of the initiatives in this ambitious strategy simultaneously, and we trust that sharing our priorities in this response will help the EMA to identify areas for greater focus. While we understand the need for prioritization, we do expect EMA to continue its work on its key activities. We would like to stress the importance of recommendation 5.3 Identify and enable access to the best expertise across Europe and internationally. We consider this is a key responsibility for EMA, and one that is already part of the Agency's activities. This will be particularly important with regard to the fast-moving environment of innovation and EMA will require appropriate specialized expertise, to be able to fulfil its mandate.

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)

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

1st choice (h): please comment on your choice, the underlying actions proposed and identify any additional actions you think might be needed to effect these changes.

Rationale for the choice:

- Novartis believes that leveraging real-world data, has the potential to bring medicines more rapidly to patients. Using novels methodologies to interrogate and assess RWD has the potential to facilitate development particularly in areas with sparse populations (orphan and ultra rare diseases). Not leveraging such data would be a missed opportunity to speed up development and access to much-needed therapies.
- Priority 3.4 is closely linked to 3.5 Develop network competence and specialist collaborations to engage with big data and 2.7 Exploit digital technology and artificial intelligence in decision making (our 3rd choice, see below). To be able to expand the applicability of RWD, there is a need for:
  - a broader range of data sources (which include new digital means to gather RWD),
  - methodologies to ensure that data is of high quality and it can be of use (such as novel analytical techniques (e.g., AI, modelling),
  - and ultimately the implementation of global standards.
- Promoting the use of high-quality RWD in Regulatory decision-making should apply to both Regulatory and HTA decisions, and should apply especially in pre-approval settings.
- There is a strong need for international alignment on the standards and methodologies for collecting, analyzing and validating RWD use to enable future decision making.

Suggested EMA activities that Novartis particularly supports:

- We note the suggestion to “Develop a capacity that will enable the Agency to rapidly and securely access and analyse large amounts of healthcare data”. Novartis believe that this would not be the best use of EMA resources. We strongly encourage EMA to develop the capacity to critically evaluate RWD rather than investing resources to support performing in-house analyses on such data.
- The actions listed by EMA under 3.4 are considered too high-level.
- Novartis suggest that the actions proposed under 3.5 Develop network competence and specialist collaborations to engage with big data are the most relevant to support implementation of these priorities. We especially support:
  - “Implement the core recommendations emerging from the HMA-EMA Joint Big Data Taskforce addressing areas such as harmonisation of data standards, characterisation of data quality, and provision of regulatory guidance as to acceptability of evidence” (3.5)
  - “Invest in capacity building across the network to acquire new skills to engage with these emerging areas” (3.5). Reviewers will need continuous education to have a full understanding of this fast-moving environment (RWD source, RWD quality considerations, evolving analytical methodologies) to be able to review complex dossiers.

Underlying actions proposed:

- EMA is encouraged to work globally to align with other regulators and especially the US FDA. Importantly, data standards should be agreed internationally.
- EMA/EC are encouraged to organize fora to align on design, collection and use of RWD with regulators and HTAs
- The Big Data taskforce should clarify its roadmap: the current roadmap lays out the strategic goals for EMA/HMA but is importantly requiring associated timelines.
- The Big Data taskforce should have a more open and inclusive approach: of the five key recommendations of the report regarding data (standardisation, quality, sharing and access, linkage and integration and analytics), some are listed as being owned by regulators while others have a common ownership. In practice, input from a wider range of stakeholders (HTAs, patients and consortia) with expertise would be beneficial in all aspects.

(1) https://www.ema.europa.eu/en/documents/minutes/hma/ema-joint-task-force-big-data-summary-report_en.pdf

Second choice (h)

9. Foster innovation in clinical trials

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

Rationale for the choice:

- Clinical trials are the foundation of drug development. New trial concepts (e.g. novel designs) and the use of different data sources offer important potential including: to decrease the number of patients enrolled and exposed to clinical trials, to identify the best targets and most relevant patient populations for new chemical or biological entities and to speed up access to innovative medicines. Future innovations in clinical development may enable the generation of high quality scientific evidence in smaller patient populations, minimizing unnecessary patient exposure, and are particularly important in supporting the development of medicines in rare diseases where high unmet medical needs still exist.
- 2.2. Foster innovation in clinical trials is clearly linked to 2.3 Develop the regulatory framework for emerging clinical data generation as the use of novel trial designs, endpoints, or techniques for gathering data is tightly connected to the way data are now produced and collected in clinical trials and digital technologies play an increasing role.
- Innovation in clinical trials is intimately linked to the ability to conduct complex, novel clinical trials within the regulatory framework:
  - 2.2. Foster innovation in clinical trials
  - 2.3 Develop the regulatory framework for emerging clinical data generation
  - 2.6 Optimise capabilities in modelling, simulation and extrapolation
  - 2.7 Exploit digital technology and artificial intelligence in decision making
  - 3.4. Promote use of high-quality real-world data (RWD) in decision making
  - 3.5 Develop network competence and specialist collaborations to engage with big data
- It is critical if future clinical development is to be supported in the EU that there is broad acceptance of new clinical trial designs (e.g., master protocol: umbrella, basket, platform trials) by regulators and HTAs
- Similarly, broader acceptance of new clinical data sources (e.g., historical controls, registries, real-world data (RWD), Big Data, historical controls) and measures (e.g., biomarkers, new endpoints including. digital endpoints, such as those obtained using wearable digital devices), is required in order to significantly advance the conduct of efficient clinical trials which are reflective of patient needs and experience. EMA should support the development of a regulatory framework for the use and acceptance of these new digital tools and methods.
- Fulfilment of this objective requires strong engagement and alignment with the NCAs as responsible parties for reviewing and approving CTAs. Close collaboration with the CTFG is key to support clinical trial innovation and maintain competitiveness of the clinical trial environment in the EU.

Suggested EMA activities that Novartis particularly supports:

- Drive adoption of novel practices that facilitate clinical trial authorisation, GCP and HTA acceptance
- Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines
- Develop methodology to incorporate clinical care data sources in regulatory decision-making
- Modernise the GCP regulatory oversight to enable decentralised models of clinical trials coupled with direct digital data accrual

Underlying actions proposed:

- Novartis strongly recommends that the activities to advance innovation in clinical trials must be accompanied by a modernization of GCP.

- EMA should foster open-dialogue and ensure regulatory acceptance of new clinical trial designs by publishing guidance on how these can be authorized and managed within the current regulatory framework.
- EMA/EC should ensure that the upcoming Clinical Trial Regulation is not preventing the advance of innovative designs (currently simultaneous amendment are impossible precluding use of innovative designs). The corresponding CTIS should also able to support innovative CT designs.
- All stakeholders should work on defining patient centricity. Novartis believes that patient centricity should ensure that the CT model adapts to the patient needs from a scientific and operational perspective for example allowing decentralised CTs using patient-centric endpoints.

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.

Rationale for the choice:

- Novartis is embracing digital technologies, advanced analytics and artificial intelligence to help drive innovation and improve efficiency. These technologies are increasingly used in the healthcare sector though uptake lags behind other industries (e.g. automobiles, aviation industries).
- Priority 2.7 is closely linked to 3.4. Promote use of high-quality real-world data (RWD) in decision making and 3.5 Develop network competence and specialist collaborations to engage with big data. Digital technologies are giving stakeholders access to advanced novel sources of data and facilitating the collection of RWD and novel analytical techniques (e.g., AI, modelling) are needed to analyse these new data.
- Greater use of RWD is consistent with the drive for greater digital innovation; use of digital tools is emerging in the healthcare sector and in clinical development. Lack of ability to adapt to the increasing availability of RWD is likely to unduly delay approval of new innovative medicines and may impair the attractiveness of EU for pharmaceutical innovation.
- Acceptance by the regulators will have a downstream effect on HTAs. There is a need for strong collaboration with HTAs through fora such as parallel scientific advice to ensure equal preparedness to assess these innovative technologies
- The B/R of medicines may be based on data from digital endpoint(s) or using digital processes for data management requiring specialised expertise for assessment It is important for EMA to work to develop capabilities and expertise across the EU network to assess the use of RWD and/or AI in sponsor applications.

Suggested EMA activities that Novartis particularly supports:

- Establish a dedicated AI test “laboratory” to explore the application of innovative digital technology to support data-driven decisions across key business processes
- Develop capacity and expertise across the network to engage with digital technology, artificial intelligence, cognitive computing, and their applications in the regulatory system. EMA is encouraged to consider HTA representative(s) as member/ invitee to this network„

Underlying actions proposed:

- EMA is encouraged to engage in efforts for greater global alignment with other regulators and especially the US FDA
- EMA/HMA should develop capacity and expertise across the network to engage with digital technology, artificial intelligence, cognitive computing and their applications in the regulatory system.
- The EC is encouraged to develop a tripartite dialogue between the DGs Grow (Notified Bodies), DG Santé (EMA) and DG Connect to integrate its vision of e-and m-health. A similar platform is also needed between EMA and the Notified Bodies and extended to HTAs.
- The EMA should develop guidance on the use of digital endpoints to ensure that they can be qualified in a timely and robust manner.
- The EMA should also develop guidance on data management in a holistic way with dialogue that includes experts in data management such as data privacy, data IT integrity, etc.
- Novartis understands that the MDR (Medical device Regulation) is not within the remit of EMA. However, we believe that with the advent of developments in the field of ‘software as a medicine’, EMA should actively reflect on whether digital technologies should be jointly regulated with NBs (Notified Bodies) or even be assessed in-house. EMA should also examine the further challenges brought by new MDR regulations, which increase hurdles for software (e.g. classifying virtually all SaMD applications as Class 2a or higher) a significant step-up from current MDD requirements and not aligned with the broader regulatory environment in other jurisdictions (e.g. US/FDA.)

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

Industry is also undertaking research
The researchers based within the pharmaceutical industry (such as those working in the Novartis Institute for Biomedical Research) have not been taken into account in this strategy, in particular when considering goal 5:" Enabling and leveraging research and innovation in regulatory science". This goal is fundamental to support numerous other recommendations and should not focus solely on collaboration with academia. To truly enable and leverage contemporary research and innovation, industry-based researchers should be acknowledged in this strategy alongside their academic counterparts.

In the same way, information technology companies are essential contributors to include as they are actively advancing the fields of eHealth, big data, artificial intelligence, wearable devices etc. They undertake research and should also be considered as partners in some activities.

Proposed action:
- EMA should include industry-based researchers in the activities in this regulatory strategy to which they can usefully contribute.

International cooperation
It is acknowledged that EMA is aware of the global nature of medicines development and research, and refer to international cooperation through the Regulatory Strategy to 2025. However, we would welcome a more thorough strategy to achieve greater alignment in regulatory science and regulatory frameworks, including priorities and timelines in areas of key priorities such as RWD utilization for decision making.

Proposed actions:
- EMA should use the existing framework of work with the FDA to identify areas where guidance needs to be fully aligned between the two regions, in terms of content and/or timelines (e.g. data standards for RWD, endpoints, etc).

IT infrastructure
Novartis strongly stresses the need for an efficient IT structure and for IT systems to support this strategy. We are concerned about the current lack of common IT systems that are available for all stakeholders to use (e.g. the Clinical Trials Information System (CTIS), which is still yet to be launched, or the Article 57 database, which is currently a limited Excel spreadsheet on the EMA website). Efforts are being made to build the IDMP/SPOR standards but this not sufficiently advanced to provide confidence that these will be available in the near future.
EMA should ensure the presence of an optimal IT system to handle large amounts of clinical data as well as manufacturing data. Furthermore, EMA is encouraged to elaborate a more aspirational goal and associated needs to advance an IT infrastructure for concepts such cloud based application in the future.

Proposed actions:
- EC should initiate an external review of all existing IT systems within the EMA, for both internal (e.g. SIAMED, PaedDatabase, etc) and external systems (e.g. Article 57 database, CTIS, etc) and establish areas where harmonisation is needed in terms of data standards.
- EC should ensure that IDMP is implemented throughout EMA, HMA and NCAs databases.

Question 7 (human): The following is to allow more detailed feedback on prioritisation, which will also help shape the future application of resources. Your further input is therefore highly appreciated. Please choose for each row the option which most

closely reflects your opinion. For areas outside your interest or experience, please leave blank.

Should you wish to comment on any of the core recommendations (and their underlying actions) there is an option to do so.

Strategic goal 1: Catalysing the integration of science and technology in medicines development (h)

	Very important	Important	Moderately important	Less important	Not important
1. Support developments in precision medicine, biomarkers and 'omics'	○	◉	○	○	○
2. Support translation of Advanced Therapy Medicinal Products cell, genes and tissue-based products into patient treatments	○	◉	○	○	○
3. Promote and invest in the Priority Medicines scheme (PRIME)	○	○	○	○	○
4. Facilitate the implementation of novel manufacturing technologies	○	◉	○	○	○
5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products	○	○	○	○	○
6. Develop understanding of and regulatory response to nanotechnology and new materials' utilisation in pharmaceuticals	○	○	○	○	○
7. Diversify and integrate the provision of regulatory advice along the development continuum	○	◉	○	○	○

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation** you are commenting on:

1. Support developments in precision medicine

- It is critical to target therapies to the patients that are most likely to benefit from them through greater acceptability of targeted biomarkers and genetic tests. Developing these medicines is challenging as the current regulatory and HTA environment is not suited to such curative therapies highlighting the need to further enhance collaboration with HTAs.

Suggested EMA activities supported:

- Enhance early engagement with novel biomarker developers [...]
- Evaluate [...] the impact of treatment on clinical outcomes measured by biomarkers

Additional suggestion:

- EMA/EC should develop a coherent prioritisation of personalised medicine that goes hand in hand with existing health strategic plans
- EMA/EC should develop a consistent diagnostic testing infrastructure throughout Europe.
- In order to improve evidence development we recommend that EMA engage with HTAs to align data requirements and to increase future acceptability of biomarker-based endpoints as primary source of evidence for decision making.

2. Support translation of ATMPs

- The future of healthcare is undergoing a revolution with a greater number of curative or preventive approaches to treatment.
- A large number of HAs are actively regulating or considering regulating ATMPs. Novartis believe there is a need for global convergence in the development of ATMP standards

Suggested EMA activities supported: The actions proposed by EMA are not sufficiently detailed and require further elaboration.

Additional suggestions:

- Global convergence: EMA is strongly encouraged to engage with other international regulatory agencies to foster global convergence of requirements for ATMP
- Multi-level regulations: there are still significant inconsistencies between national and European requirements
- Framework supporting manufacturing innovation: The regulatory framework should be flexible enough to allow adoption of more advanced technology once available and thus continual improvement in the manufacturing processes

4. Facilitate the implementation of novel manufacturing technologies

- Novartis supports implementation of novel technologies for manufacturing of pharmaceutical medicines. We envision consistent improvement in our manufacturing processes.
- Adoption of innovation in pharmaceutical manufacturing requires scientific evaluation from regulators. Making changes manufacturing processes in the commercial lifecycle and approval of these changes will require regulator expertise in both review and GMP inspection.

Suggested EMA activities supported: Recruit expertise in novel manufacturing technologies [...]: EMA is encouraged to recruit expertise representing both assessment and GMP perspectives. Novartis consider that the other proposed actions are not really appropriate. We believe that Regulatory challenges with production at point of care deserves a separate discussion. We recommend delinking it from Continuous Manufacturing (CM) since this is a completely different topic.

Additional suggestions:

- We recommend that EMA should expand the current goal 2.7 Exploit digital technology in decision making to include the context of CM. The rate of data generation in CM is comparable to other pharmaceutical areas.

• EMA is strongly encouraged to ensure that the EU framework stays aligned with evolving developments with the global environment
• EMA should continue working with ICH Expert WGs to facilitate regulatory convergence for continued acceptance of evolving innovative manufacturing technologies.
• EMA should facilitate initial and post approval lifecycle management of products manufactured using advanced continuous technologies.

7. Diversify and integrate the provision of regulatory advice
• There is a need for more flexibility and faster SA to support innovation; the current process is too lengthy. Value and potential adoption of SA will increase if received in a timely manner.
• There is need to strengthen engagement and inclusion of HTAs as key stakeholders early in development. If HTAs do not accept a given data package it will lead to downstream delay in patient access or denied access.
• Innovative and complex development programs need special support and competencies from the agency.
• There is a needed to further strengthen alignment between EMA committees and the SAWP but also between NCAs especially for CTs.
Suggested EMA activities supported: the actions proposed by EMA are endorsed and we support all of them but, as an additional suggestion we stress the need to ensure that evidence needed by HTA (and payors) is incorporated early in drug development discussions in joint/parallel advices. Novartis believe that the process would be more flexible if SAWP could have the possibility to ask questions to the applicant during the first 40 days of their review.

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**:

Very Important:

- 9. Foster innovation in clinical trials and 10. Develop the regulatory framework for emerging digital clinical data generation are grouped in our second priority - please refer to Question 5
- 14. Exploit digital technology and artificial intelligence in decision-making is our third priority - please refer to Question 5

Moderately important:

11. Expand benefit- risk assessment and communication
- Novartis welcome the recommendation to incorporate patient preferences, develop new approaches to address B/R and improve communication with HTAs bodies
- This is supporting informed decision making and value assessment
- Improve communication with HTAs and payers regarding therapeutic context, comparison vs. placebo /active-control, patient perspective to enable HTA acceptance, especially in rare diseases.
- This is considered only moderately important as this recommendation is already supported by IMI Prefer, this PPP will validate methods for patients preferences

Suggested EMA activities supported: Expand the benefit-risk assessment by incorporating patient preferences however we are not supportive of the action to Develop the capability to analyse Individual Patient Data to support decision-making, we consider that this is not the best use of the regulators resources. EMA should focus on understanding and reviewing the Individual Patient Data but not re-analysing data in-house.

12. Invest in special populations initiatives
- There is a need to better define and focus on high unmet need populations
- Need for better engagement with HTA to ensure joint support for an evidence generation plan.
- Special populations will also benefit from other recommendations in this strategy (e.g., M&S, adapted CT design, RWD, use of wearables, registries) that could help generate data from such patients where the feasibility of standard randomised CTs is known to be challenging.
- Novartis has rated this moderately important as work is already ongoing in that area (in particular within the EC joint evaluation of the paediatric and the orphan Regulations, IMI Conception, etc.)
- EMA is encouraged to continue its current efforts to support drug development for special populations and improve patients' early access

Suggested EMA activities supported:
- Focus on speedy access for patient (sub-)populations in urgent need
- Identify areas of highest unmet needs where clinical care data can supplement clinical trial data
- Enhance multi-stakeholder advice in collaboration with patients, HCPs, payers and HTAs
- Progress implementation of the joint EMA/EC paediatric medicines action plan

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**:

Very Important:
18. Promote use of high-quality real world data (RWD) in decision- making
This is our first priority, please refer to Question 5

Important:
15. Contribute to HTAs' preparedness and downstream decision-making for innovative medicines
- Currently there is not always alignment; evidence generated during development of a medicine is not always appropriate to meet to the needs of all subsequent, post-approval decision makers such as HTA bodies and payors.
- Important for broader stakeholder agreement and alignment early in drug development on the data and evidence to be generated in order not to delay regulatory approval and patient access.
- Alignment EMA/HTA on evidence requirements will facilitate patient access, particularly when HTA early feedback is that reimbursement will be granted only in a sub-population of the label
- Development of new innovative clinical trials/endpoints will further increase this need for alignment

Suggested EMA activities supported:
- Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans
- Discuss with HTAs guidance and methodologies for evidence generation and review

17. Reinforce patient relevance in evidence generation
Suggested EMA activities supported:
- Coordinate the Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis
- While validating PROs, address patients' needs and leverage patients' expertise
- Co-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment

Additional suggestions:
- Qualification of PROs: the current qualification procedure is too lengthy to fit the pace of development. Best practice guides on high-quality PRO trial tools would help to ensure that patients' voices are central to informing shared decision-making, labeling claims, clinical guidelines, and health policy and making patient-centered care a reality
- Reinforcing the need for patient preference elicitation: IMI PREFER believes that CHMP and EUnetHTA's opinion on the acceptability of such a framework and the DCE (Discrete Choice Experiments) method can promote the broader use of patient preference studies in research and development and evaluation of novel treatments and ultimately result in the development of dedicated guidance.

19. Develop network competence and specialist collaborations to engage with big data
This is closely linked to our first priority 18. Promote use of high-quality real world data (RWD) in decision-making, please refer to Question 5

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

	Very important	Important	Moderately important	Less important	Not important

23. Implement EMA's health threats plan, ring-fence resources and refine preparedness approaches	○	○	○	○	○
24. Continue to support development of new antimicrobials and their alternatives	○	○	○	○	○
25. Promote global cooperation to anticipate and address supply challenges	○	○	○	○	○
26. Support innovative approaches to the development and post-authorisation monitoring of vaccines	○	○	○	○	○
27. Support the development and implementation of a repurposing framework	○	○	○	○	○

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

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

	Very important	Important	Moderately important	Less important	Not important
28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science	○	○	○	○	○

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions					
30. Identify and enable access to the best expertise across Europe and internationally					
31. Disseminate and share knowledge, expertise and innovation across the regulatory network and to its stakeholders					

Please feel free to comment on any of the above core recommendations or their underlying actions. **Kindly indicate the number of the recommendation you are commenting on**:

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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