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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

***Name of organisation (if applicable):***

BEUC- The European Consumer Organisation

## 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*.

BEUC welcomes the opportunity to comment on the EMA Regulatory Science to 2025 strategic reflection.

The EMA has the role to safeguard public health by ensuring that only medicines with demonstrated safety and efficacy are authorised. Accomplishing this task to the highest standards is essential to protect citizens from harm and maintain public trust in the regulatory system. While the EU has one of the world's most advanced regulatory frameworks, it is important to revisit it and strengthen it where necessary.

As demonstrated by the informal meeting of Health Ministers held under the Austrian Presidency in September 2018 evidenced, and the 2016 Council conclusions on pharmaceuticals evidence agreed under the Dutch Presidency, governments in the EU are interested in addressing the question of medicines approval and patient benefit. At the same time, the European Parliament's Report on EU options for improving access to medicines recalls that robust clinical trials and thorough pharmacovigilance monitoring are necessary to assess the quality, efficacy and safety of new medicines. We believe that the EMA's strategic reflection process offers an opportunity to look forward and further strengthen the framework.

In this regard, BEUC fully supports the EMA's proposals for expanded benefit-risk assessment, for example, by incorporating data on patient reported outcomes and developing capacity to analyse individual patient data. We also welcome the commitment to improve public communication on medicines. We call on the EMA to concentrate more in-depth on these two questions.

Nonetheless, we acknowledge that there are important public health threats (e.g. drug shortages) and regulatory challenges (e.g. integrated pathway for the assessment of health technologies) that require the EMA's involvement. Considering the number of proposed workstreams in this strategy, we recommend that the EMA sets clear priorities and develop workable action plans in line with available resources. Precisely to help the Agency in this prioritisation exercise, we have indicated below which recommendations we find more or less relevant in relative terms.

BEUC will follow closely the implementation of the future strategy and looks forward to contributing input to work plans and initiatives that are most relevant to consumers.

## 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)

| |
|---|
| 11. Expand benefit-risk assessment and communication |

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.

BEUC finds certain proposals in the EMA's strategic reflection document on how to improve benefit-risk assessment very pertinent, such as including patient reported outcomes and develop capacity to analyse individual patient data. We call upon the Agency to prioritise this matter and take on board our additional recommendations to make the market authorisation system work better for patients and consumers (see question 7).

While some new medicines improve health outcomes significantly and represent a remarkable advance, many offer no or little added therapeutic benefit.(1) As a matter of fact, cancer medicines are increasingly in the spotlight. In a recent report, the World Health Organization (WHO) raised concerns about the fact that a considerable proportion of cancer medicines approved in the past 15 to 20 years have only data for improvement in surrogate endpoints, without evidence on the impact in survival or quality of life.(2) The use of surrogate endpoints is often justified on the grounds that patients need earlier access to new treatments. But the balancing exercise between early access and the requirement for data on benefit/risk is something to be approached with caution. In oncology, most studied surrogates have been found to have low or modest correlation with overall survival.(3) This means that vulnerable patients are exposed to treatments that carry safety risks and for which there is no re-assurance of efficacy.

When medicines are authorised based on less comprehensive data, it is important that post-marketing studies are requested to confirm benefit. The much-welcomed report from the EMA on the 10 years' experience with conditional marketing authorisation (CMA) shows that, on average, it takes four years to switch to full marketing authorisation (on occasions, seven years). While conditional approval is justified in some situations, the fact that patients need to wait years for re-assurance on clinical value is an issue of genuine concern.

As rightly mentioned by the EMA, further improvements in the application of conditional marketing authorisation are possible. More discussion on the quality of post-marketing studies and the extent to which initial uncertainties are adequately resolved later on is necessary.(4) In the interest of patients and healthcare systems, medicines that do not offer benefit should be promptly withdrawn from the market. In this regard, we note that recently the EMA recommended revoking a conditional marketing authorisation on these grounds for the first time.

References

(1) Prescrire's ratings of new products and indications over the past 10 years. https://english.prescrire.org/en/81/168/57229/0/NewsDetails.aspx
(2) World Health Organization (2018). Technical report. Pricing of cancer medicines and its impacts.
(3) Haslam A, Spencer PH, Gill J, Prasad V (2018). A systematic review of trial-level meta-analyses measuring the strength of association between surrogate endpoints and overall survival in oncology. European Journal of Cancer. Vol 106, p. 196-211.
(4) Banzi R, Gerardi Chiara, Bertele V, Garattini S (2017). Conditional approval of medicines by the EMA The process fails to improve the evidence after early licensing. BMJ 2017;357:j2062.

## Second choice (h)

17. Reinforce patient relevance in evidence generation

## 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.

Taking patients' and consumers' needs and expectations duly into account during the development of medicines is crucial to ensure that studies are designed to answer important questions. While evidence on improvements in quality of life (QoL) is relevant, this type of data is not consistently collected. BEUC thus supports the EMA's proposal to reinforce patient's voices in evidence generation by means of updating relevant guidelines to include references to patient reported outcomes (PRO).

In our recommendations further below we call upon the use of robust methodologies for data collection, and optimal methods for patient and consumer engagement in the regulatory processes.

## Third choice (h)

22. Further develop external communications to promote trust and confidence in the EU regulatory system

## 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.

BEUC fully supports the EMA's commitment to expand external communication on medicines. Better information and more transparency are essential to enhance public trust in the regulatory system.

The EMA invests a lot in the provision of information and over the years has made many efforts to make this information more understandable (e.g. involvement of patients and consumers in the readability of package leaflets, Q&As and European Public Assessment Report summaries) and more accessible. The 2017 perception survey on EMA's communication indicates that in general there is a high level of satisfaction, but also areas for improvement. For example, on ease of understanding of information materials among non-experts.

A remaining shortcoming is that package information leaflets (PILs) are not user-friendly enough. Problems have been identified with the content and lay-out of PILs, which still include language that is too complex. While the elderly and those with low literacy skills are the most disadvantaged, other groups report similar problems.(1) As mentioned below, while the development of electronic product information is welcome, it must not come at the expense of efforts to improve PILs. Providing better information on approved medicines to end users should be an EMA priority in the years to come.

The last years saw a growing demand for transparency on information on medicines which the EMA responded by adopting an ambitious new policy on the proactive publication of clinical data, and reviewing the policies on access to documents and EudraVigilance data. In addition, sponsors started publishing clinical trial results on the EU Clinical Trials Register. Further efforts are now needed to ensure that summary results of all trials are timely reported in the registry, and the proactive publication of clinical reports is resumed after being put temporary on hold.

References

(1) Van Dijk L, Patrício Monteiro S, Vervloet M, de Bie J, Theo Raynor DK (2014). Study on the Package Leaflets and the Summaries of Product Characteristics of Medicinal Products for Human use.

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

Concrete actions in the area of pharmacovigilance and reporting of adverse drug reactions (ADRs) are largely missing in the strategic document, despite being an issue of high relevance to patients and consumers. Evidence suggests that the burden of ADRs in Europe is significant.(1) The EU has an advanced pharmacovigilance framework but it is important to continuously strive to make it more proactive and quicker in responding to safety concerns. A thorough evaluation of the EMA's implementation of the pharmacovigilance legislation and its impact to public health could help identify gaps and inform measures for improvement.

The question of clinical trial data transparency is also missing. While the EMA has made remarkable improvements on this front over the years, there is more to be done. For example, on the EU Clinical Trials Register where still today more than 40% of registered clinical trials are missing due summary results.(2) As the manager of the register, the EMA should implement the necessary measures to ensure full compliance by trial sponsors with reporting requirements and an optimal functioning of the system. This should be done in close collaboration with the European Commission and national competent authorities.

As an umbrella consumer organisation, BEUC brings the voice of consumers, be it as patients, carers, actual and future medicine users to the EMA. The consumer and patient voices complement each other, and both contribute to improve the quality of EMA's work. The EMA's reflection document, however, does not refer much to consumers.

References
(1) Bouvy JC, De Bruin ML, Koopmanschap MA (2015). Epidemiology of Adverse Drug Reactions in Europe: A review of recent observational studies. Drug Saf. 2015; 38 (5):437-453.
(2) EU Trials Tracker

## 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:

As the EMA rightly identifies, the development of medicines needs to reflect the latest scientific knowledge. Drug development should also be driven by high evidentiary requirements by regulators. After all, innovative medicines are those that represent a remarkable advance and improve health outcomes in a meaningful way.
BEUC supports the EMA's general goal to catalyse the integration of science in medicine development with some observations.

• Precision medicine (recommendation 1): Developments on precision medicine must be driven by genuine public health needs and supported by scientific evidence. Medical research should not, however, be shaped with the sole purpose of fitting into a scheme of incentives.
The incentives provided in the EU Orphan Regulation are 'pull' and 'push' mechanisms adopted with the intention to reward medical innovation in the area of rare diseases. Bearing in mind the link between precision medicine and more narrow patient populations, as well as increasing concerns with a potential misuse of the Orphan Regulation, we call on the EMA and especially the Committee for Orphan Medicinal Products and the Committee for Medicinal Products for Human Use (CHMP) to ensure that the letter and spirit of the Regulation are fully respected.

• Pre-submission activities (linked to recommendations 3 and 7): Publishing scientific guidelines is an efficient way by which regulators can advise on drug development and inform on evidentiary requirements. This allows all medicine developers to benefit equally from such information. The EMA should update existing guidelines and adopt new ones as often as needed to keep pace with scientific advances. Other means to resolve questions and encourage discussion on drug development is through public workshops. It is important that such forums ensure balanced representation of stakeholders, including patient and consumer organisations, and are carefully managed to avoid inappropriate influence.
Personalised scientific advice provided by the EMA to medicine developers has the potential to improve clinical trial design and support the development of innovative medicines. However, such activities may pose a risk that the EMA's marketing authorisation assessment is influenced by the pre-submission exchanges between the Agency and medicine developers. Actual or even perceived biases reduce trust in the regulatory system. BEUC acknowledges the EMA's efforts to safeguard the independence of the regulatory processes, for example, through its policy for managing conflict of interests and its collective process for the evaluation of medicines (e.g. CHMP). Nonetheless, we encourage the EMA to adopt additional safeguards:
1. Limit pre-submission activities to strictly necessary and duly justified situations (e.g. genuine scientific questions not addressed in guidelines, non-profit medicine developers).
2. Reconsider the strong involvement of CHMP members in pre-submission activities, for example in PRIME, where a Committee member is appointed as a rapporteur to help building medicine developers' knowledge on marketing authorisation procedures.
3. Publish the minutes and other material documenting pre-submission exchanges at least once the medicinal product has been approved.
4. Continuously assess and report about the extent to which pre-submission activities contribute to the generation of robust evidence, and the availability of innovative medicines.

• Integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products (recommendation 5): BEUC strongly supports EMA's recommendation 5 to create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products. However, we also note that many of the novel borderline products are increasingly complex, often including a software component. Depending on the complexity of such products, there might be a need for additional regulatory safeguards to ensure a clear framework on their development, assessment and monitoring. There is also a need for clear guidance on how to ensure safety, security and trustworthiness of such products. In addition, a comprehensive market surveillance system must be introduced for such products, possibly, integrating with and/or complementing the existing pharmacovigilance and Eudamed databases.

## 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**:

BEUC strongly supports this overaching goal. Some observations:

• Clinical trials (recommendation 9): Many clinical trials fall short from being rigorously designed, conducted and reported.(1) BEUC considers that it is extremely important that clinical trials answer questions that are relevant to patients and consumers, are representative of the population to treat and follow robust methods. The EMA can promote improvements by stimulating public debate on clinical trials to inform scientific guidelines and ensuring that patients and consumers are adequately consulted in these discussions. A critical look at used surrogate endpoints and their validity is necessary. In addition, the EMA should report on actions taken to ensure that medicines were tested ethically in particular in low and middle-income countries, in accordance with EU legislation and as requested by the European Parliament.(2) There should be closer collaboration with competent authorities and the European Commission to ensure that all trials report results in due time, and the proactive publication of clinical reports should be resumed.

• Benefit-risk assessment and communication (11): Consumers need the highest level of assurance that medicines on the market have established safety and efficacy. For this:

1) Medicines' marketing authorisation must be supported by high evidentiary standards. RCTs are a particularly valuable tool to assess safety and efficacy.
2) Scientific guidelines should require the submission of comparative clinical trial data against standard treatment, and exceptions be duly justified in guidelines, marketing authorisation applications and in the EPARs.
3) Medicines already available with a full marketing authorisation, but for which there is still some uncertainty on clinical benefit, must be further studied.
4) 'Early access' schemes should remain the exception and be used only when there is no available treatment (genuine unmet medical needs). Patients using these schemes face risks comparable to clinical trial participants and deserve the same level of protection by means of close monitoring and damage compensation.
5) When medicines are early authorised, we should not wait unnecessarily for conclusive evidence on safety and efficacy. Post-marketing obligations must be closely monitored and delays publicly reported. Consequences for unjustified delays should be considered.
6) Better communication is needed for public understanding of the meaning 'full vs conditional marketing authorisation', approval under 'exceptional circumstances' and the 'black triangle'.
7) More efforts are needed to increase awareness on ADR reporting.
8) Package leaflets must be user-friendly.
BEUC supports the proposal to develop capability to analyse individual patient data. This can help flag information that has not been adequately captured in clinical study reports.

- Regulatory framework for clinical data generation/Exploiting digital technology and artificial intelligence in decision making (recommendations 10 and 14): BEUC urges the EMA to proceed with caution with the implementation of these recommendations. Big data analytics in healthcare have a potential to achieve greater outcomes in clinical data generation and as a result, improve patient and consumer health while reducing costs. However, personal data and privacy could be easily exchanged in return to a promise of improved health condition. Therefore, it is very important to establish a clear framework to ensure that AI is applied considering the principles of fairness, transparency, purpose limitation, data minimisation, accountability and privacy and security by design and by default. Automated processes based on algorithms must be transparent to consumers and discrimination avoided. To achieve this, it is important to ensure that existing legislation (e.g. the General Data Protection Regulation) is not circumvented, and that new laws are created to enhance patient and consumer protection, especially when it comes to the sensitive category of health data. BEUC also recommends to the EMA to collaborate closely with the European Data Protection Board to align the use of analytics for biomedical data with the data protection framework and with the EU's Cybersecurity Agency (ENISA) on the issues of security of these data.

- Special populations (12): Priorities should be set according to identified public health needs and in participatory processes. Patient and consumer groups should be duly consulted on health topic prioritisation processes and in the design of specifi actions.

References:
(1)Heneghan C , Goldacre B, Mahtani KR (2018). Why clinical trial outcomes fail to translate into benefits for patients. Trials, 18:122
(2) European Parliament. Discharge 2015: European Medicines Agency. Decision of 27 April 2017

## 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**:

• Collaboration with HTA bodies and payers (15, 16): When companies fail to generate the evidence needed by HTA bodies and payers, timely access to medicines is jeopardised. To avoid this, EMA's scientific guidelines should require the submission of comparative trial data against standard treatment. Increased dialogue among regulators, HTA bodies and payers is needed on core concepts (e.g. unmet medical need, significant benefit) while respecting their distinctive needs and roles.

• Patient relevance in evidence generation (17): Medicines' users provide relevant information on symptoms, changes in QoL or functioning experienced through new therapies. BEUC agrees that it is important to capture much better PROs, in ways that ensure quality. At the same time, patients' and consumers' input on the expected benefits of new medicines and their views help inform regulatory decisions. Due attention should be paid to methods and representativeness, and stakeholder consultation be well-documented in the EPARs.

• Use of real-world data (18): BEUC is cautious with expectations on ''real-world data'' in the broader sense of the word, but acknowledges the added value of some of these data and supports initiatives to further explore RWD as a complementary information source. Further debate is needed to establish which questions observational studies can or not help answer with sufficient reliability, and better guidelines to ensure quality in the context of different data sources (e.g. patient registries, electronic health records). Observational studies should not replace robust demonstration of evidence in the pre-market phase, nor post-marketing requirements in the form of clinical trials (ideally RCTs). The latter is particularly relevant in early access schemes. Transparency of observational study results must be ensured.

• Develop competence on big data (19): BEUC welcomes this recommendation. The deployment of biomedical big data from varied sources and use of advanced analytics make privacy protection a more complex task than just establishing the 'standard' protection mechanisms foreseen by the existing European data protection framework. For example, while user consent is one of the main means to control personal data, it will not provide alone sufficient protection regarding all possible future data uses, especially in the context of health research. Therefore, we urge EMA to ensure: 1) Quality and safety standards for all information systems where health data is generated, used or stored 2) More transparency of how data is used and by whom through the entire data use process. In the context of AI, 'black-box algorithms' use in health must be avoided 3) More clarity about data access and data control, especially when it comes to the use of algorithm-based solutions and multiple source data (e.g. data from electronic health data, connected medical device, social media) 4) More oversight mechanisms to monitor compliance of all involved in handling of personal biomedical data with privacy protection rules and other ethical norms, and to ensure their accountability in case of data misuse 5) Clear accountability and liability rules for algorithm-based health-related decisions.
Complex AI-tools make it difficult to determine who is responsible if something goes wrong and current liability rules are not up to date to deal with autonomous products.

• eProduct Information (20): Electronic leaflets as a complement to the paper version hold the potential to improve the readability and layout of leaflets, as well as patients' access to the most updated version. We welcome this development but it is important to make sure that the paper leaflet and e-leaflet are well-integrated, and provide safe, comprehensive and unbiased information on medicines. We also recommend that a single portal for eProduct information is managed by medicines agencies, as well as any apps developed to facilitate eProduct information (e.g. ADR reporting apps and WEB-RADR).

• External communication and public trust (22): The EMA has made remarkable improvements in this area but more could be done. These actions should be prioritised: 1) Learn from best practices on user-friendly PILs and improve user testing by including the views of laypeople 2) Consult patient and consumer groups on relevant communication campaigns e.g. biosimilars, AMR 3) Ask patients and consumers for feedback on their engagement in regulatory processes and improve them as needed 4) Test the interface of the new clinical trials database with end users 5) Make the EMA's website more user-friendly.
Patient and consumer engagement strengthens legitimacy and accountability. We commend the EMA for its inclusive approach and call for continued involvement in ways that ensure meaningful participation, balanced representation and transparency. Financial support from the EMA is important for optimal engagement and language barriers should be lifted.

### 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**:

BEUC welcomes the EMA's commitment to strengthen the capacity of the regulatory system to address health threats. In particular, we recommend that the Agency prioritises:

• Support the development of high-quality new antibiotics (recommendation 24): It is estimated that 33.000 people die every year due to infections with antibiotic-resistance bacteria in Europe.(1) The development of novel antibiotics is urgently needed but it is largely neglected, with major pharmaceutical companies abandoning their research pipelines in this area.
Government funding for biomedical research and development is essential to address public health gaps. As a major global research funder, the EU should step up efforts to support the development of new and affordable antibiotics. Funding schemes should apply a combination of 'push' and 'pull' mechanisms with attached conditionalities to ensure public return on public investment and medicines' affordability. The EMA can provide support through guidance on the development of antibiotics and improved information on evidentiary requirements. A key ask from BEUC is that international collaboration to harmonise regulatory requirements for the approval of new antibacterial medicines must converge towards the highest standards on quality, safety and efficacy.
Regarding veterinary medicines, BEUC supports the update/adoption of new guidelines to promote responsible use of antimicrobials and improved collection and analysis of data on their use. To date, the ESVAC (European Surveillance of Veterinary Antimicrobial Consumption) project is only collecting data on sales of antimicrobial veterinary medicinal products. Under the new legislation on veterinary medicines however, Member States will have to collect data on sales and use of antimicrobial veterinary medicines. Therefore, EMA's work to validate a robust methodology for the harmonised and standardized collection of usage data per species is very important.

• Promote global cooperation to anticipate and address supply challenges (25): Given that shortages of medicines are becoming increasingly frequent both in Europe and globally, BEUC highly welcomes recommendation 25 to address existing supply challenges. BEUC urges EMA-HMA to continue promoting a harmonised approach among Member States on how medicine shortages should be communicated within and between the countries.
However, harmonised communication on shortages at the EU level will not be effective unless a clear and widely used formal definition(s) is developed. At present, formal and legally binding definitions of drug shortages do not exist in most EU countries (with the exception of Belgium, France, Italy and Spain). Absence of definitions, as well as inconsistencies and incomparability of existing definitions across different systems and countries, are likely to hinder comprehensive reporting, communication, and comparative analyses of the problem of drug shortages, as well as the scale and effects of shortages. To address this problem, BEUC recommends developing a harmonised definition (or set of definitions) of medicine shortages.
In addition to a commonly agreed terminology, there is a need to develop an EU-wide centralised monitoring system to collate the reports from the national systems and identify common causes of shortages in Member States. Such a database could be coordinated by EMA-HMA and should be open for healthcare professionals, patients and consumers in order to notify and learn about ongoing shortages. The common system could also serve as a tool to ensure better compliance of pharmaceutical manufacturers and pharmaceutical full-service healthcare distributors with Directive 2001/83/EC5 requiring to provide a notice at least 2 months in advance in case of a temporary or permanent medicine shortages.

References
(1) European Centre for Disease Prevention and Control

## 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**:

Some important recommendations linked to the underlying actions proposed by the EMA:

• Safeguard the independence of the regulatory process:The work performed by the EMA is highly dependent on the engagement of external experts. Any exercise seeking best available expertise needs to carefully consider the question of conflicts of interest. Robust policies on the handling conflicts of interests safeguard the independence of the regulatory process and contribute to public trust.

• Public health needs-driven research partnerships: Research projects funded by the EU Framework Programme, and to which the EMA gets involved in one way or another, should be oriented towards public health needs and ensure public return on public investment. Public funding should be subject to clauses on data-sharing, open access to publications and product affordability. Clinical trials by non-commercial entities should be further supported as a means of improving understanding on the effects of medicines on the regulatory and clinical sides.

We support the EMA's commitment to strengthen collaboration with academia and gear it towards filling knowledge gaps in regulatory science.

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