Contribution ID: 455a02f8-95b8-4ac5-abcc-26f25094f164
Date: 28/06/2019 17:31:20

Public consultation on EMA Regulatory Science to 2025

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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):

RHAPSODY ("Risk Assessment and ProgreSsiOn of DIabetes"), a public private consortium funded by the Innovative Medicines Initiative (IMI) and EFPIA Companies with contributions from academic institutions. This is a project focused on assessing the risk of progression of pre-diabetes to overt diabetes and of rapid deterioration of type 2 diabetes (T2D).

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 115881 (RHAPSODY). This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA. This work is supported by the Swiss State Secretariat for Education, Research and Innovation (SERI) under contract number 16.0097. The opinions expressed and arguments employed herein do not necessarily reflect the official views of these funding bodies

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.

IMI Rhapsody is grateful to EMA to have the opportunity to comment. The strategic goals of Goal 1 Catalysing the integration of science and technology in medicines development and Goal 5 Enabling and leveraging research and innovation in regulatory science are extremely relevant to Rhapsody.

Under Goal 1 the Core recommendation 1 "Support developments in precision medicine, biomarkers and "omics" is completely aligned to the goals of the Rhapsody programme.

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)

1. Support developments in precision medicine, biomarkers and 'omics'

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.

The areas identified align with IMI2 Rhapsody's aims. Health authorities should be addressing the integration of science and technology to ensure that current or future regulatory pathways are nimble and flexible enough to accommodate these evolving tools and technologies. Their active involvement would further help secure sufficient resources and expertise to be able to aid in the development of future products. Proactive engagement with both Industry and academia within those Consortia made possible by IMI funding will help shape these emerging fields.

Background of Rhapsody
The stated goal of IMI2 RHAPSODY is to define a molecular taxonomy of type 2 diabetes mellitus (T2D) that will support patient segmentation, inform clinical trial design, and the establishment of regulatory paths for the adoption of novel strategies for diabetes prevention and treatment. To address these goals, RHAPSODY brings together prominent European experts, including several leaders of the diabetes-relevant IMI1 projects to identify, validate and characterize causal biomarkers for T2D subtypes and progression. Our plans are built upon: (a) access to large European cohorts with comprehensive genetic analyses and rich longitudinal clinical and biochemical data and samples; (b) detailed multi-omic maps of key T2D-relevant tissues and organs; (c) large expertise in the development and use of novel genetic, epigenetic, biochemical and physiological experimental approaches; (d) the ability to combine existing and novel data sets through effective data federation and use of these datasets in systems biology approaches towards precision medicine; and (e) expertise in regulatory approval, health economics and patient engagement. These activities will lead to the discovery of novel biomarkers for improved T2D taxonomy, to support development of pharmaceutical activities, and for use in precision medicine to improve health in Europe and worldwide.

IMI2 Rhapsody supports the core recommendation of supporting developments in precision medicine, biomarkers and omics, particularly early engagement with novel biomarker developers to facilitate regulatory qualification. The early involvement of stakeholders at all levels is key and is supported. Continuous evidence generation and ways to handle the large volumes of data likely from new diagnostics is supported, and advice from the regulators is key.

Advice on biomarker selection, and the data needed to validate the biomarkers in a variety of context of use (s) is critical. Advice on use of biomarker panels, in addition to advice on individual biomarkers would be beneficial as currently guidance is only on single biomarkers whereas.

At present Regulatory Advice is available through EMA qualification procedure, also Innovation Task Force Meetings. However, having more frequent interactions would be useful.

Second choice (h)

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

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.

- IMI2 Rhapsody strongly supports network led partnerships with academia to undertake fundamental research in strategic areas of regulatory science.

- RHAPSODY is focused on the discovery and validation of biomarkers to monitor progression of the prediabetic state, to T2D and of biomarkers for beta-cell dysfunction and insulin resistance in target tissues like muscle, fat and liver. These biomarkers will allow improved patient stratification for use in innovative clinical trials aiming at prevention and better management of T2D, i.e. steps towards precision medicine: "The right prevention and treatment to the right patients at the right time".

Third choice (h)

9. Foster innovation in clinical trials

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.

IMI2 Rhapsody strongly supports the recommendation to foster clinical trial innovation.

The use of omics will facilitate development of medicines to treat smaller patient populations within a precision medicines framework. The ability to partition populations is a very mechanistically rational approach for finding subgroups with different responses to treatment or to predict response to treatment downstream, in comparison to using arbitrary diagnostic categories or simple demographic categories.

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

IMI2 Rhapsody considers that the EMA has captured within its overall strategy the key issues facing the consortia currently. More interaction with EMA and guidance on the validation of biomarkers would be beneficial.

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, biomarkers and 'omics'

• The classification of diseases with modern knowledge and technology is evolving rapidly. Past clinical paradigms may not be appropriate. By integrating knowledge and tools based on data and evidence in precision medicine, biomarkers and omics and applying it to human therapeutics, significant public health benefits could be gained. There is a huge amount of information now available, no one body or institution has the resources to drive this area forwards on their own. It is extremely important that Academics, Regulators and Industry work together to solve some of the challenges, keep abreast and move forwards with the science and facilitate development of novel human therapeutics with improved benefit:risk ratios. Stimulation of fundamental biomarker research by academic centres is important and making available the biomarker qualification information would facilitate the development of new drugs, new indications for existing drugs or extension of intended use populations.

• For novel biomarkers, consideration should be given to enhance the current EMA qualification procedure to allow the procedure to be accelerated and provide for greater flexibility, or a different pathway to develop and discuss biomarker development outside of the qualification procedure to facilitate rapid progress.

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**:

9. Foster innovation in clinical trials:

IMI2 Rhapsody strongly supports the recommendation to foster clinical trial innovation.

The use of omics will facilitate development of medicines to treat smaller patient populations within a precision medicines framework. The ability to partition populations is a very mechanistically rational approach for finding subgroups with different responses to treatment or predict response to treatment downstream, in comparison to using arbitrary diagnostic categories or simple demographic categories.

8. Leverage novel non- clinical models and 3Rs
One of Rhapsody objectives is to establish dialogue between clinical studies and preclinical models to support efficient biomarker discovery and establish the link between these biomarkers, dysfunctions of islet cells and insulin target tissues, and the progression of diabetes.

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**:

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**:

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

IMI2 Rhapsody very much supports the role of public private partnerships in medicines research. 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 IMI projects comprised of tools and methods with 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.

https://www.imi.europa.eu/resources-projects/guidelines-engaging-regulators

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