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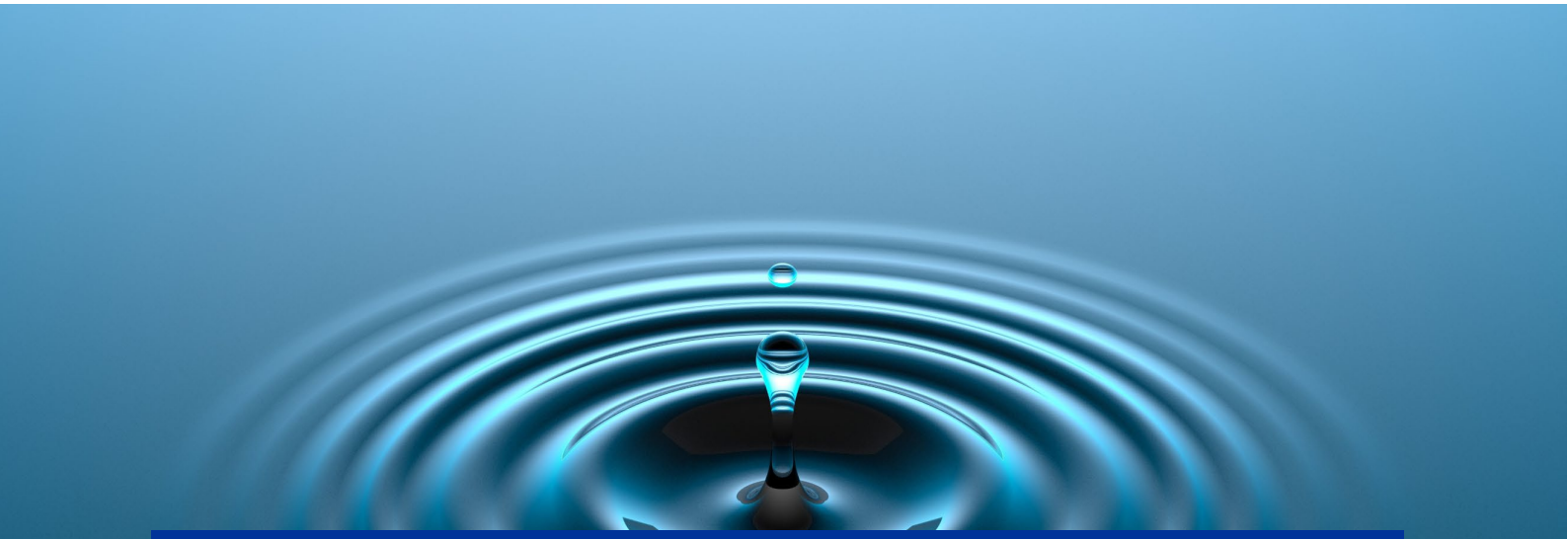

Advancing Regulatory Science Research

Event report
18 November 2024

On 18 November 2024, the European Medicines Agency (EMA) organised a public event on Advancing Regulatory Science Research. Two initiatives were launched during this event: the 2024 update of the EMA Regulatory science research needs (RSRN) and the EMA/HMA European Platform for Regulatory Science Research, a new initiative to enhance collaboration between academic researchers and regulators across Europe and beyond. The event stimulated interactions and discussions on how to advance regulatory science research addressing key regulatory science needs.

The presentations and recording of the event are available on the EMA event page.

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Introduction - Regulatory science research to support scientific and technological advances and inform their evaluation

Promoting and achieving excellence in regulatory science is at the core of EMA's mission and that of the broader network of medicines regulators. Regulatory authorities have evolved over time, expanding their role from traditional gatekeepers to enablers of innovation, ensuring the safety and efficacy of medicines while facilitating timely access to patients. Despite significant progress, aligning fast-paced scientific advancements and developments with the requirements of regulatory decisions remains a challenge. New methodologies and innovations introduced during regulatory procedures can clash with the need for evidence and consistency of regulatory decision-making processes. Systematic identification of issues, before they occur in product specific scientific submissions, allows early start of regulatory science research, such as investigating and generating evidence for tools, methods and data sources that are used for the development and assessment of medicines, as well as for investigating in how far the regulatory system is responding to scientific advancements. Past examples were given in the presentations to highlight the practical impact.

Embracing a systematic approach would result in a continuous cycle of research that drives change and that can ultimately improve the regulatory framework by engaging researchers and developers working on new medicines and methodologies. For this lifecycle of research to work, the collaboration and dialogue between stakeholders, including regulators, developers and researchers, needs deepening. This is particularly relevant today, given the public health challenges experienced in recent years. EMA continues to strengthen its role as a partner in scientific research, shaping the regulatory landscape alongside academic and industry researchers. Collaboration between multiple researchers, stakeholders and regulators is essential for regulatory science research to impact medicines development and regulatory frameworks.

The event explored these needs and the two corresponding initiatives, with the European Platform for Regulatory Science Research to support scientific progress through early and pro-active dialogue between researchers and regulators. This discussion takes place during a period of significant change in medicines regulation, with the ongoing revisions of the pharmaceutical legislation and updates to the regulatory network strategy.

The 2024 update of EMA's Regulatory Science Research Needs (RSRN)

EMA scientific staff is systematically identifying gaps in regulatory science and translating them into regulatory science research needs. As part of this effort, the 2024 update of the Regulatory Science Research Needs (RSRN) was published to highlights key areas for research across several domains, including clinical trials, regulatory system evolution, developments for specific medicines and animal health. The aim is to inform researchers and funding organizations to consider these needs in their research and funding programmes.

Identification of RSRN for the update was informed by interviews with EMA scientific leads and by reviewing horizon scanning reports, regulatory interactions with developers and assessment reports. The RSRN will be periodically updated to reflect emerging research needs. Stakeholders were encouraged to engage in the public consultation to provide feedback on the research needs.

The panel discussion highlighted that addressing gaps in regulatory science requires ongoing engagement with stakeholders, including academic institutions and public-private consortia. The panel stressed the importance of collaboration in regulatory science, particularly through initiatives like Horizon Europe projects. To make such opportunities more accessible, the discussion suggested leveraging existing research networks to disseminate regulatory research needs and promote informal and continuous brainstorming as well as facilitating exchange through the new European Platform for Regulatory Science Research. The panel also explored what defines high-quality regulatory science research, focussing on the importance of methodological rigor and potential impact of the research. Stimulating meaningful advancements with funded research was considered paramount.

A European Platform for Regulatory Science Research

Collaboration in regulatory science research allows to address cross-cutting issues and horizontal questions concerning medicines and to foster faster, efficient pathways to bring these medicines to patients. To achieve these goals, the EMA and the Heads of Medicines Agencies (HMA) launched the European Platform for Regulatory Science Research. The Platform serves as forum for academic and non-profit organisations, regulators and other stakeholders to explore regulatory science research issues related to both human and veterinary medicines and facilitate collaboration. This is expected to increase the relevance and usefulness of research outputs, so that they can be effectively applied in the work of regulators and developers.

Speakers explained how the concept for this Platform was informed by experience gathered in the EU Medicines Regulatory Network. Event participants were encouraged to respond to the public consultation on the draft concept paper for the Platform and were invited to express interest[1] to become involved.

The panel highlighted the importance of early and continual interaction between stakeholders. For example, regulators can engage early with research and researchers can hear about regulatory thinking, to help shape rigorous and impactful studies. For researchers, engaging with regulators may present challenges and a learning curve, but will be interesting to broaden the relevance of their scientific work. Consideration should be given to how regulators can maintain an active role in the research process yet minimise potential conflicts of interest when assessing new regulatory science tools or medicine applications using these tools.

A recurring suggestion was to raise academic awareness on regulatory science including in education and research, such as through regulators' participation in academic conferences. Also, providing more webinars on regulatory guidelines and involving academia in their development can help bridge the gap.

Besides scientific publications, impact is determined by how regulatory research translates into real-world changes in the health care and research system. The European Platform for Regulatory Science Research reflects the commitment to this goal and will raise awareness among researchers, promote structured

[1] How to join the European Platform for Regulatory Science Research: https://ec.europa.eu/eusurvey/runner/european-platform-regulatory-science-research, Regulatory science research | European Medicines Agency (EMA)

exchange, encourage collaboration across the research lifecycle, and build a larger and stronger research community for regulatory science.

Funding programmes and regulatory science research

Speakers highlighted that early engagement of regulators in research and development projects has been beneficial for both researchers and regulators. As part of Horizon Europe[2], several calls have highlighted the necessity for researchers to engage with regulatory bodies early in the process. In some cases, this engagement is required as part of the funding process to ensure that the research meets the scientific and regulatory standards needed for approval.

Public-private partnerships are important vehicles for advancing regulatory science, bringing together academia, industry, healthcare providers, and regulators. They foster knowledge transfer and ensure that projects are informed by the expertise of diverse stakeholders. Industry partners contribute regulatory expertise, helping to bridge the gap between academic research and practical application.

An example was the use of real-world data (RWD) in regulatory decision-making. Recent projects investigate artificial intelligence and machine learning to analyse RWD for regulatory and health technology assessment (HTA) purposes. These projects involve regulators, academia, health technology assessment bodies and patient organisations to align proposed methods with regulatory needs.

Multi-stakeholder perspectives on how to advance Regulatory Science Research

During the multi-stakeholder panel, researchers, regulators, patients, healthcare professionals, funders and industry shared their perspectives on how to shape the future of regulatory science research. Panellists acknowledged the growing importance of regulatory science and flagged that more collaboration is needed to better advance regulatory science given that often scientific, regulatory, and industry stakeholders engage late, when a solution has already been developed. By designing research in awareness of regulatory readiness and expectations of other stakeholders, developers can generate more impactful evidence. The panel encouraged regulators to share regulatory science agendas more widely across academia, consortia, and global networks. The importance of research for methods to handle future challenges in regulatory science was underscored.

Academic researchers and regulators as well as industry experts, patients and healthcare professionals have expertise and experience to bring to the table. By fostering collaborative environments, regulatory science research can evolve in a most informed and impactful way. The role of policy in advancing regulatory practices with broader healthcare goals was highlighted. The panel stressed the need for a global perspective for working on regulatory science. Scientific challenges are global in nature, and solutions have no borders.

Adequate resources are crucial to the success of regulatory science initiatives. Effective collaboration requires not only structures but also funding and expertise, which impacts national competent authorities and regulatory bodies seeking to meaningfully support efforts to improve regulatory science.

Stakeholders emphasized the importance of focusing on practical outcomes. Regulatory science can speed up patient access to new medicines. By identifying priorities and coordinating research efforts, the impact of regulatory science can be maximized.

The event closed with thanks to the more than 600 attendees, online and onsite, for their participation and feedback on key initiatives introduced, and to the speakers, panellists, and moderators who shaped this event to advance regulatory science research.

[2] Horizon Europe - European Commission, Innovative Health Initiative | IHI Innovative Health Initiative

List of speakers

Liese Barbier	European Medicines Agency (EMA)
Niklas Blomberg	Innovative Health Initiative Joint Undertaking (IHI)
Marco Cavaleri	European Medicines Agency (EMA)
Magda Chlebus	European Federation of Pharmaceutical Industries and Associations (EFPIA)
Ivo Claassen	European Medicines Agency (EMA)
Emer Cooke	European Medicines Agency (EMA)
Emmanuel Cormier	European Medicines Agency (EMA)
Tomasz Dylag	DG Research and Innovation, European Commission (DG RTD, EC)
Harald Enzmann	German Federal Institute for Drugs and Medical Devices (BfArM)
Alessandro Faia	European Medicines Agency (EMA)
Evangelos J. Giamarellos-Bourboulis	Medical School, National and Kapodistrian University of Athens (NKUA), Greece
Rosa Giuliani	Healthcare Professionals' Working Party (HCPWP) Chair, Guy's and St Thomas' NHS Foundation Trust
Ralf Herold	European Medicines Agency (EMA)
Franz Koenig	Medical University of Vienna (MUW)
Bert Leufkens	Utrecht University (UU)
Claudia Louati	European Patients' Forum (EPF)
Pierpaolo Moscariello	European Medicines Agency (EMA)
Marjon Pasmooij	Dutch Medicines Evaluation Board (MEB), Utrecht University (UU)
Francesco Pignatti	European Medicines Agency (EMA)
Kit Roes	Dutch Medicines Evaluation Board (MEB)
Sophia Samodelov	Zurich University Hospital (USZ)
Steffen Thirstrup	European Medicines Agency (EMA)
Andrew Thomson	European Medicines Agency (EMA)
Spiros Vamvakas	European Medicines Agency (EMA)
Günter Waxenecker	Austrian Federal Office For Safety In Health Care (AGES)