Welcome and introduction

Welcome and introduction to the strategic approach to regulatory science research

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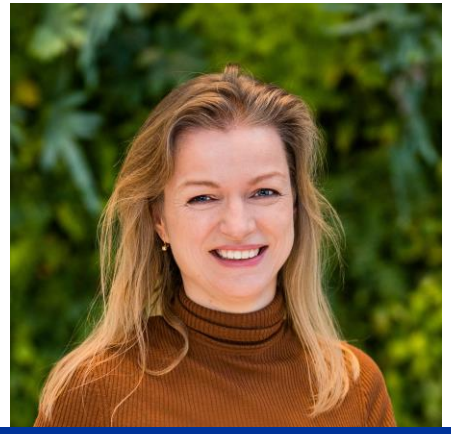
EMA



Marjon Pasmooij

Co-chair European Platform for Regulatory Science Research

MEB

Join at
slido.com
#RSR2025
Passcode: 20052025

HMA Heads of Medicines Agencies

EMA

EMA
EUROPEAN MEDICINES AGENCY

Regulatory science research lifecycle – a strategic approach

1st Platform meeting

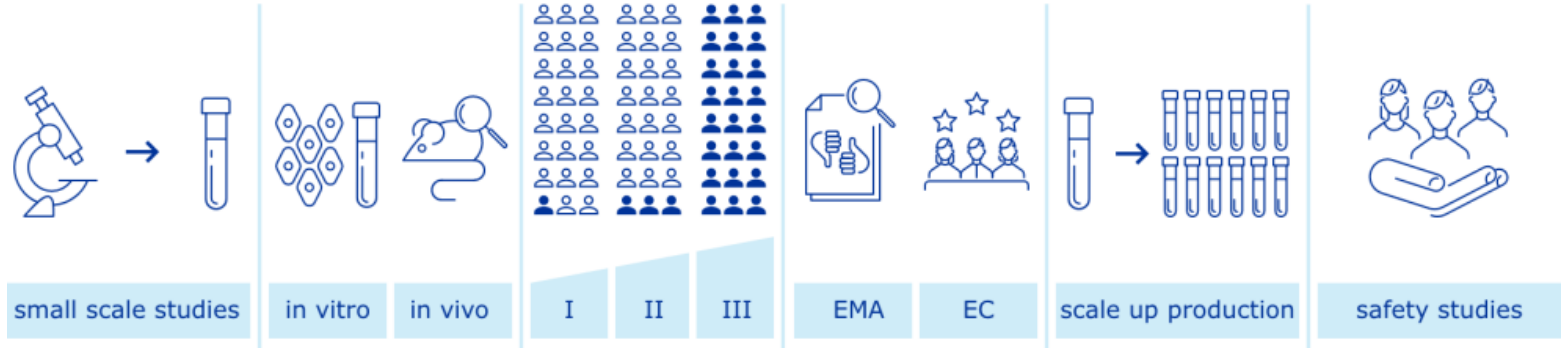
20 May 2025

Ralf Herold and Marjon Pasmooij

Evidence is needed…

…on the particular medicine under investigation

Product R&D development



…on methods and their application in developments

Regulatory science R&D

Investigating and establishing tools and methods for specific activities in drug development

Investigating and establishing approaches for optimising the evidence generation

Investigating and evolving the regulatory activities and system

What do we mean with…

Regulatory science

- applying various scientific disciplines to the quality, safety, and efficacy assessment of medicines
- informing decision-making throughout the lifecycle of the medicine
- evaluating existing, and development of new regulatory standards, tools, methods as well as principles used for developing medicines

Research into regulatory science

- addressing cross-cutting issues in development and gaps that hinder regulatory advancement
- for horizontal questions on medicines and evidence gaps that cannot be addressed through EMA data
- multidisciplinary field using a diverse range of methods, from wet lab to desktop

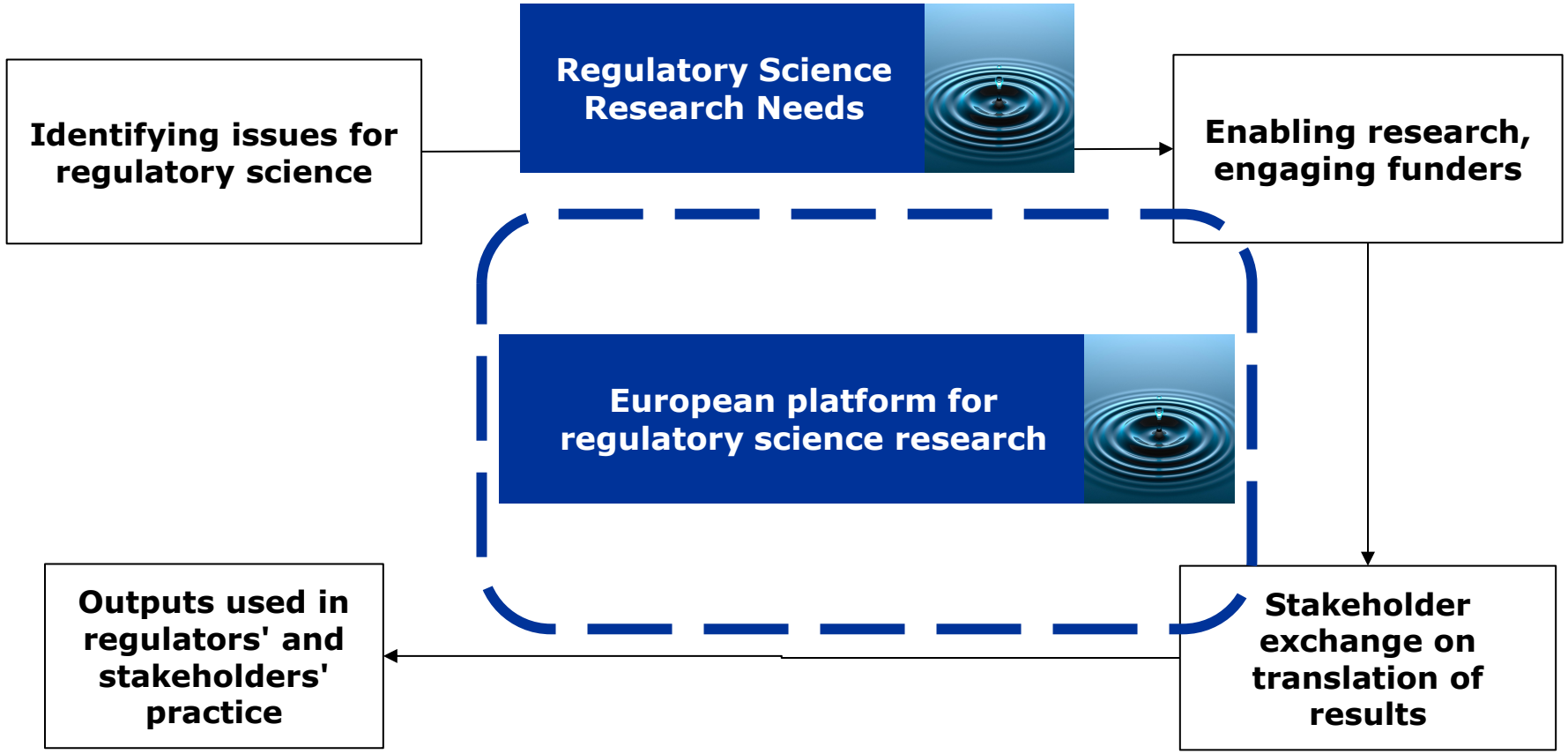
Examples of issues encountered

“it is unknown”	it is unknown if the difference observed between trials is relevant for the interpretation of results
“not established”	clinical relevance of the findings is not established
“not acceptable”	in … [neonates], it is not accepted that the SmPC is updated on the basis of predicted (simulated) doses only
“not validated”	up to date, scores on functional scales are not validated as surrogate markers
“could not be accepted”	data to support the model proposed were not available, and this approach could not be accepted

Research to address

- **Knowledge gaps**
- **Conceptual gaps**
- **Methodology gaps**
- **Empirical gaps**
- **Practical gaps**

A strategic approach to Advancing regulatory science research



Updated Regulatory Science Research Needs

1. Research needs to improve clinical research

- Statistical analysis in clinical trials
- Innovative approaches in clinical trials design
- Clinical trials optimization in specific therapeutic areas

2. Research needs to improve regulatory system evolution

- Going beyond current regulatory pathways
- Shortages
- Communication
- Data management

3. Research needs to foster technologies for development of specific types of medicines

- Platform technologies and vaccines
- Antimicrobial resistance
- Non-animal models / 3Rs
- Non-animal models / ATMPs
- Real-world data / real-world evidence
- Biomarkers

4. Research needs to advance specifically animal health and the regulation of veterinary medicines

- Non-animal models / 3Rs
- Environmental risk assessment
- Antiparasitic resistance
- Pharmacovigilance
- Novel therapies and innovation
- Vaccines
- Antimicrobial resistance
- Veterinary big data

Summary

- *Advancing* regulatory science enables new medicines that serve patient needs
- Experience is substantial, yet opportunities remain and communities are small
- A platform to:

  - foster collaboration among and between academic researchers and regulatory scientists from Europe and beyond on key topics in regulatory science research.

  - advance and accelerate research, focusing on questions of common interest, informing research on these key questions, increasing the usefulness of research outcomes and support the translation of solutions in the work of regulators and developers.