EMA
EUROPEAN MEDICINES AGENCY

Regulatory science research lifecycle – a strategic approach

Advancing Regulatory Science Research event

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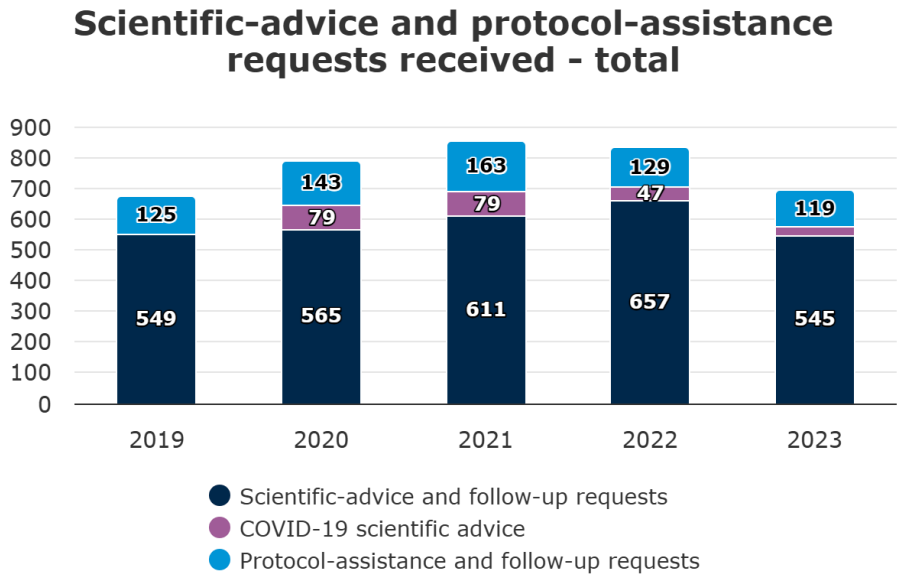
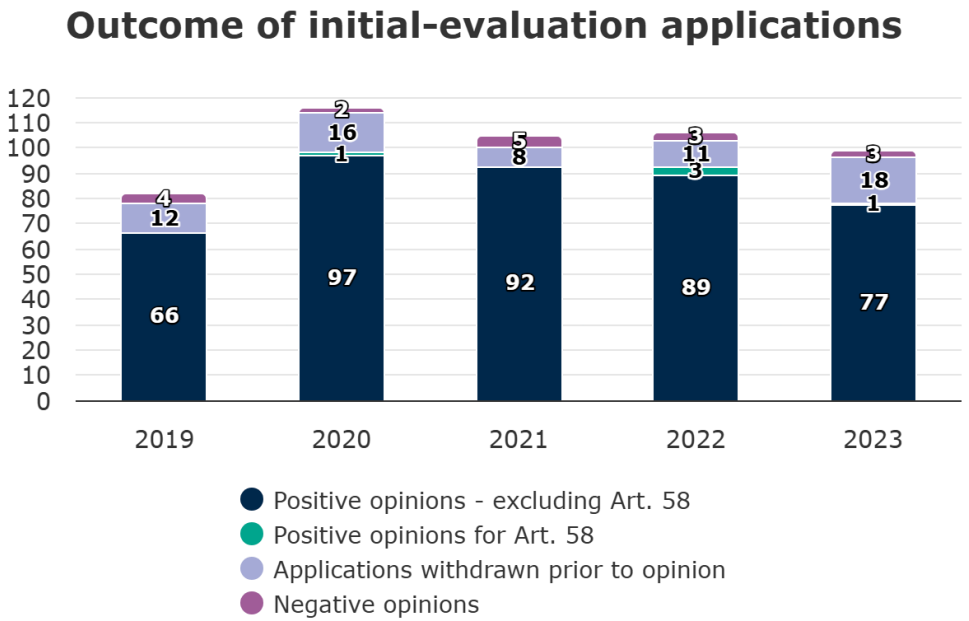
Regulatory science and Academia Workstream

Regulatory work on Product R&D: from many advices to ~100 new medicines for patients



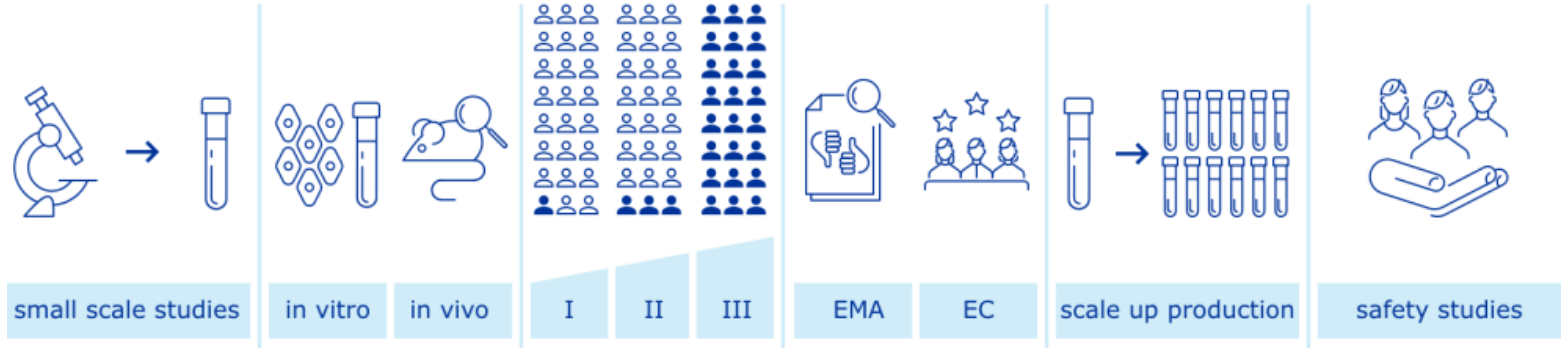


https://www.ema.europa.eu/en/annual-report/2023/human-medicines.html

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

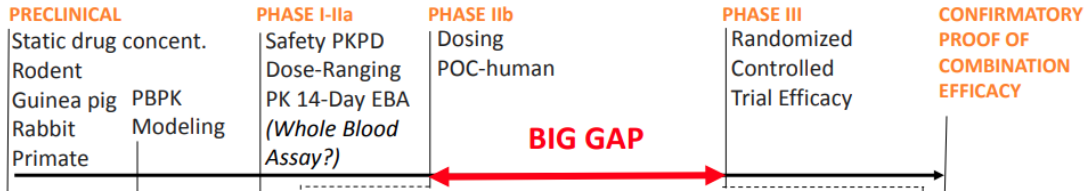
- applying various scientific disciplines to the quality, safety, and efficacy assessment of medicines
- informing decision-making throughout the lifecycle of the medicine
- evaluating existing, and inform development of new regulatory standards, tools, methods as well as principles used for developing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

Research into regulatory science

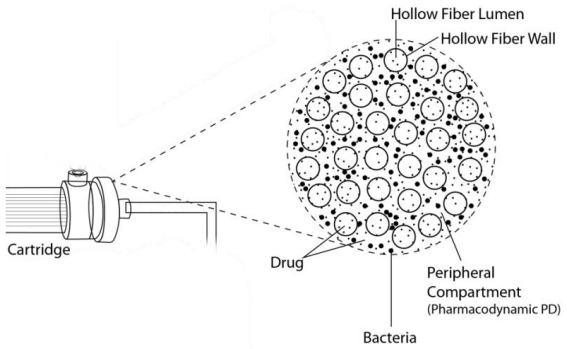
- addressing cross-cutting issues in development and gaps that hinder regulatory advancement, or
- for horizontal questions on medicines and evidence gaps that cannot be addressed with available data
- multidisciplinary field using a diverse range of methods, from wet lab to desktop

Example: hollow fibre models

Regulatory science issue



Novel drug development tool (DDT), EMA-qualified in 2015



- Drug-resistant Mtb CFU counts
- Drug concentration
- Macrophage count
- Bacteria per macrophage
- RNA expression, WGS

Used for Product R&D

At least 5 anti-infectives authorised since 2018

https://www.ema.europa.eu/en/documents/presentation/presentation-drug-regimens-consortium-cptr-scientific-advice-meeting-european-medicines-agency-hollow-fiber-system-tuberculosis-hfs-tb_en.pdf

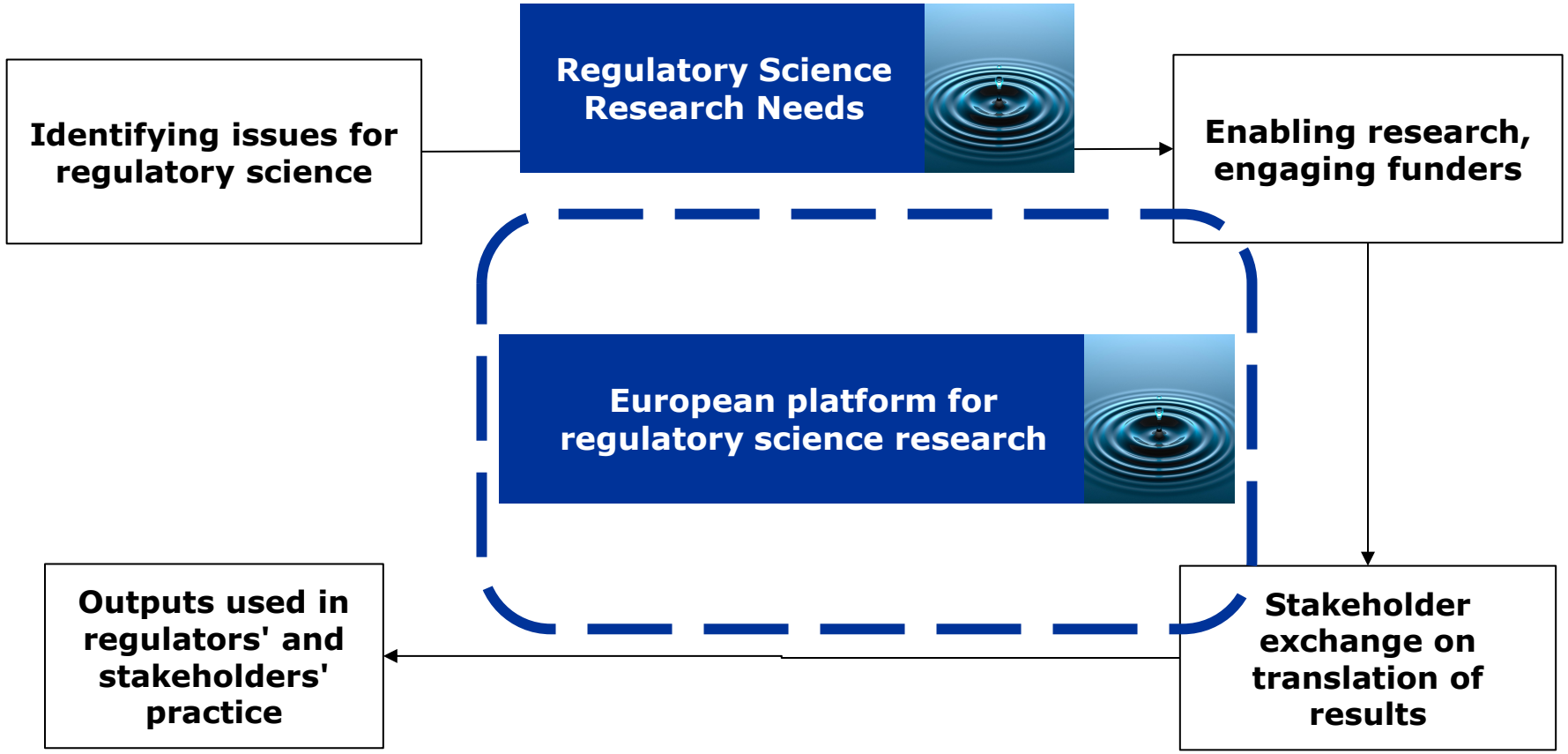
Examples of issues encountered

“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

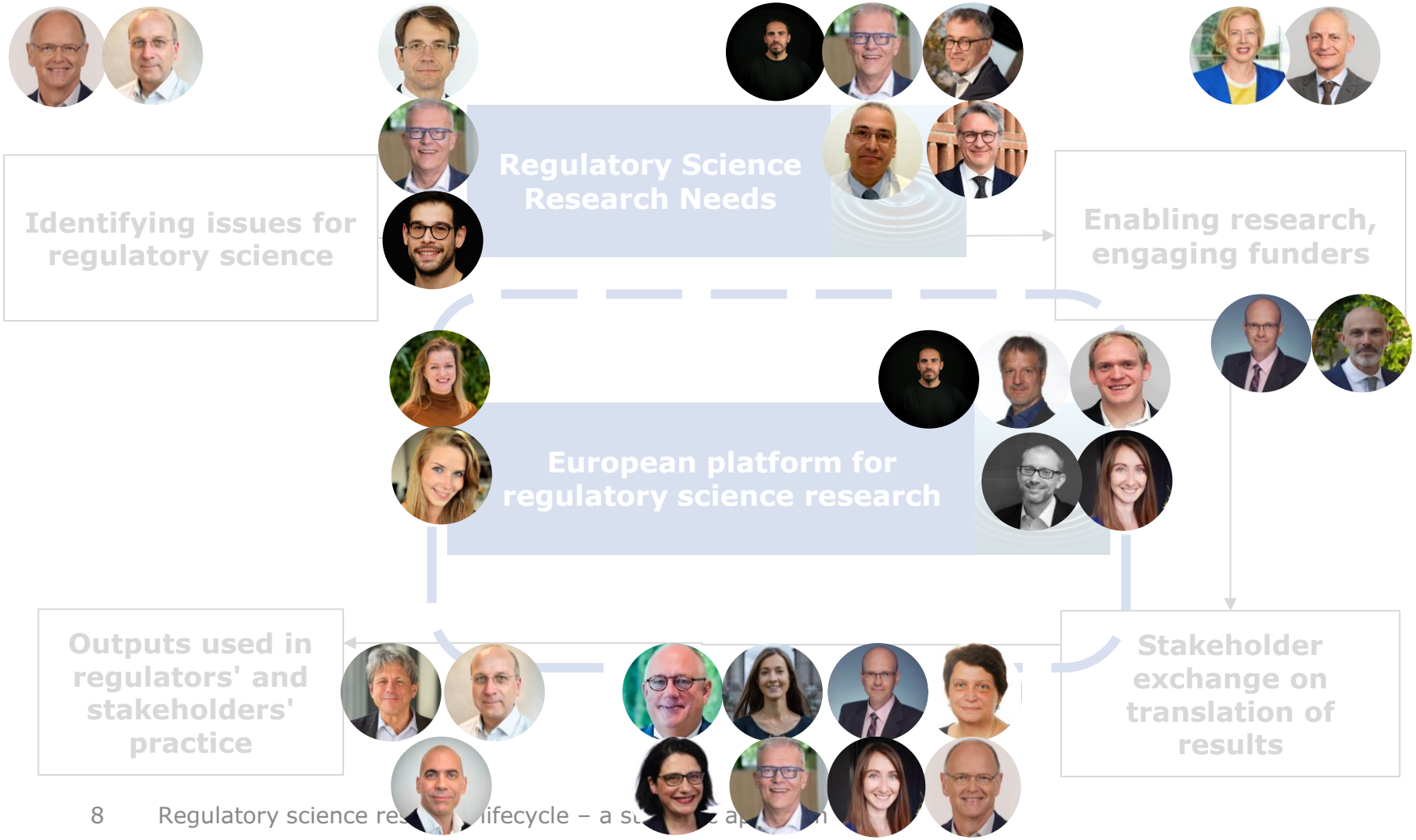
Research to address

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

A strategic approach to Advancing regulatory science research



Advancing regulatory science research



Summary

- *Advancing* regulatory science enables new medicines that serve patient needs
- Experience is substantial, yet opportunities remain and communities are small
- A system is proposed to *accelerate* picking up and addressing issues
- Regulators seek to work with stakeholders towards high-quality solutions
- This event seeks to inform on regulatory science opportunities,
  to help recognize much research can be relevant for regulators and
  to broaden interest in multi-stakeholder research activities

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Thank you

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