EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA's Regulatory Science Research Agenda

High level overview of compilation process with examples

Joint meeting of the Health Care Professionals' Working Party and Patients' and Consumers' Working Party

Presented by Lifang Liu, 2 June 2021
Regulatory Science & Innovation Task Force

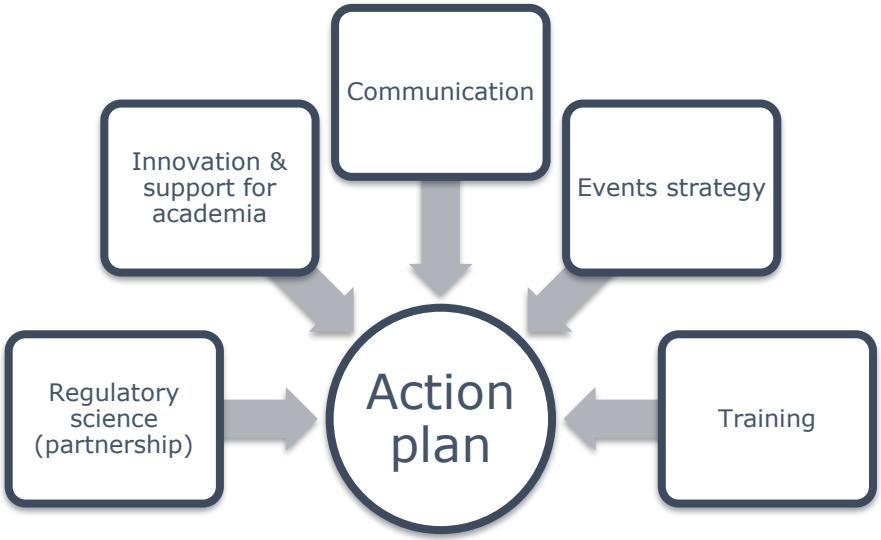
The why of a EMA Regulatory Science Research agenda

- Need to advance on regulatory science to be prepared for future challenges as well as opportunities offered by advances in science and technology
- Agenda is a tool to deliver on activities identified in EMA strategic plans
- Research needs to be identified and then delivery on these by EMA or external research groups

The how of a EMA Regulatory Science Research Agenda

- EMA programming document 2021-2023 states as an objective for the Regulatory Science and Innovation (TRS) Task Force to "Leverage collaborations between academia and network scientists to prepare for engagement with Horizon Europe and Innovative Health Initiative (IHI), define EMA's regulatory science research agenda and enable exchange of knowledge and expertise."
- Building on and reinforcing strong working relationship with European academics and researchers.
- Targeted engagement with academia, learned societies and research groups in a range of areas.
- Collaboration with HCPWP and PCWP as key partners.

Academia Action Plan 2021-2023



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28 April 2021
EMA/159144/2021
Research and Innovation (TRS-RNI)

Academia Collaboration Matrix action plan (2021 – 2023)

Introduction

The European Medicines Agency (EMA) is committed to maintaining a strong working relationship with European academics and researchers. Collaboration with academia is necessary for the Agency to be prepared for future challenges as well as opportunities offered by advances in science and technology. The Agency has targeted engagement with academia, learned societies and research groups in a range of areas.

This Academia Collaboration Matrix action plan was conceived as a tool to deliver on activities identified in EMA strategic plans while giving due consideration to the environment in which the Agency operates, including resources availability.

The EMA programming document 2021-2023 states as an objective for the Regulatory Science and Innovation (TRS) Task Force to "Leverage collaborations between academia and network scientists to prepare for engagement with Horizon Europe and Innovative Health Initiative (IHI), define EMA's regulatory science research agenda and enable exchange of knowledge and expertise." Specifically the programming document refers to "an Agency-wide plan for interaction with academia is being developed, which aims (1) to support governance and oversight of interactions with externally funded research and networks; (2) identify academic disciplines/research topics; (3) support the establishment of staff-exchange programmes and placements; (4) create academia-targeted materials to promote existing regulatory tools; (5) set up a communication strategy."

This action plan is in line with the EMA programming document 2021-2023 and the Regulatory Science Strategy to 2025 (RSS 2025), and captures the objectives the Agency must pursue in order to achieve an optimal collaboration with Academia:

- Develop network-led partnerships with Academia / research centres to undertake research in strategic areas of regulatory science;
- Leverage collaborations between Academia and network scientists to address rapidly emerging regulatory science research questions;
- Identify and enable access to the best expertise across Europe and internationally;
- Disseminate and exchange knowledge, expertise and innovation across the network and to its stakeholders.

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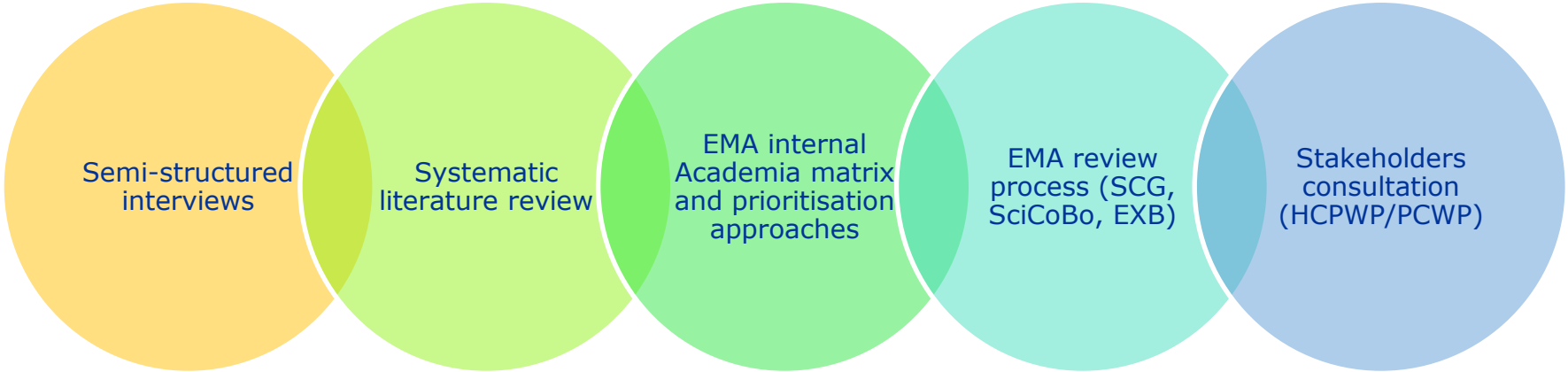
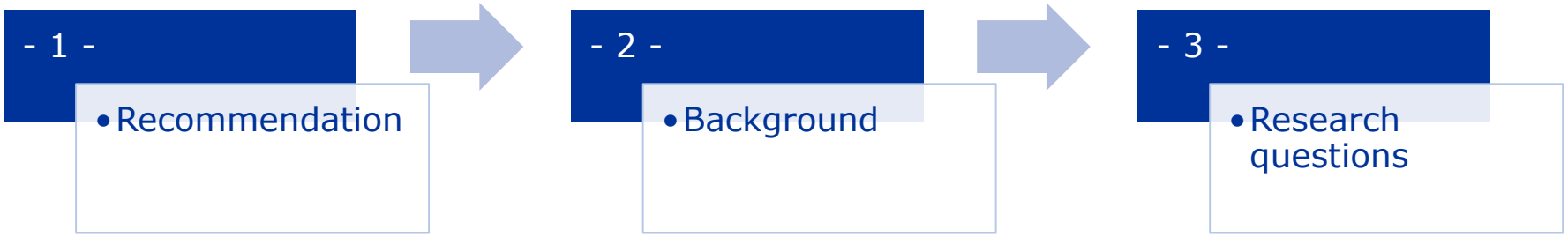
Outline of the research agenda

Materials and methods



EMA's Research Agenda Structure



The 14 Core recommendations from the RSS 2020-2025

1. **Support developments in precision medicine, biomarkers and 'omics'**
2. **Support translation of Advanced Therapy Medicinal Products (ATMPs) cell, genes and tissue-based products into patient treatments**
3. **Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products**
4. **Diversify and integrate the provision of regulatory advice along the development continuum**
5. **Foster innovation in clinical trials (efficient design, biomarkers, endpoints)**
6. **Develop the regulatory framework for emerging digital clinical data generation**
7. **Expand benefit-risk assessment and communication**

The 14 Core recommendations from the RSS 2020-2025

8 **Optimise capabilities in modelling and simulation and extrapolation**

9 **Contribute to HTAs' preparedness and downstream decision-making for innovative medicines**

10 **Bridge from evaluation to access through collaboration with Payers**

11 **Reinforce patient relevance in evidence generation**

12 **Promote use of high quality real-world data in decision-making**

13 **Continue to support development of new antimicrobials and their alternatives**

14 **Support innovative approaches to the development and post-authorisation monitoring of vaccines**

EMA's Research Agenda-Example 1

Core recommendation: Promote use of high quality real-world data in decision making

Q1

- Develop a set of evidentiary standards to be pre-specified and used in the analysis of real-word evidence applied to different types of regulatory advice and decisions on the safety and efficacy/effectiveness of medicines (e.g. in complement to clinical trial data in an authorisation application, or for extension of indications, amendment to product information or regulatory actions on the marketing authorisation due to safety concerns).

Q2

- Establish methods and processes to enable continuous learning from pre-authorisation procedures and authorisation applications to medicines regulators that contain RWD.

Q3

- Enhance the performance and efficiency of large randomised clinical trials by developing standardised processes and methods to access real-world data (RWD), including electronic health care record systems, specialised registries and hospital databases.

EMA's Research Agenda-Example 2

Core recommendation: Foster innovation in clinical trials

Q1

- Assess the utility of the wealth of available real healthcare data to improve the quality of randomised controlled trial (RCT) simulations

Q2

- Evaluate and improve the methods used for indirect comparisons of treatments in light of the Estimand Framework of ICH E9(R1).

Q3

- How to implement complex clinical trials (e.g. umbrella, basket, adaptive, registry-based trials) in the current Clinical Trial Information System (CTIS)?

EMA's Research Agenda-Example 3

Core recommendation: Invest in special population initiatives

Q1

- Develop a strategic initiative in maternal-fetal health.

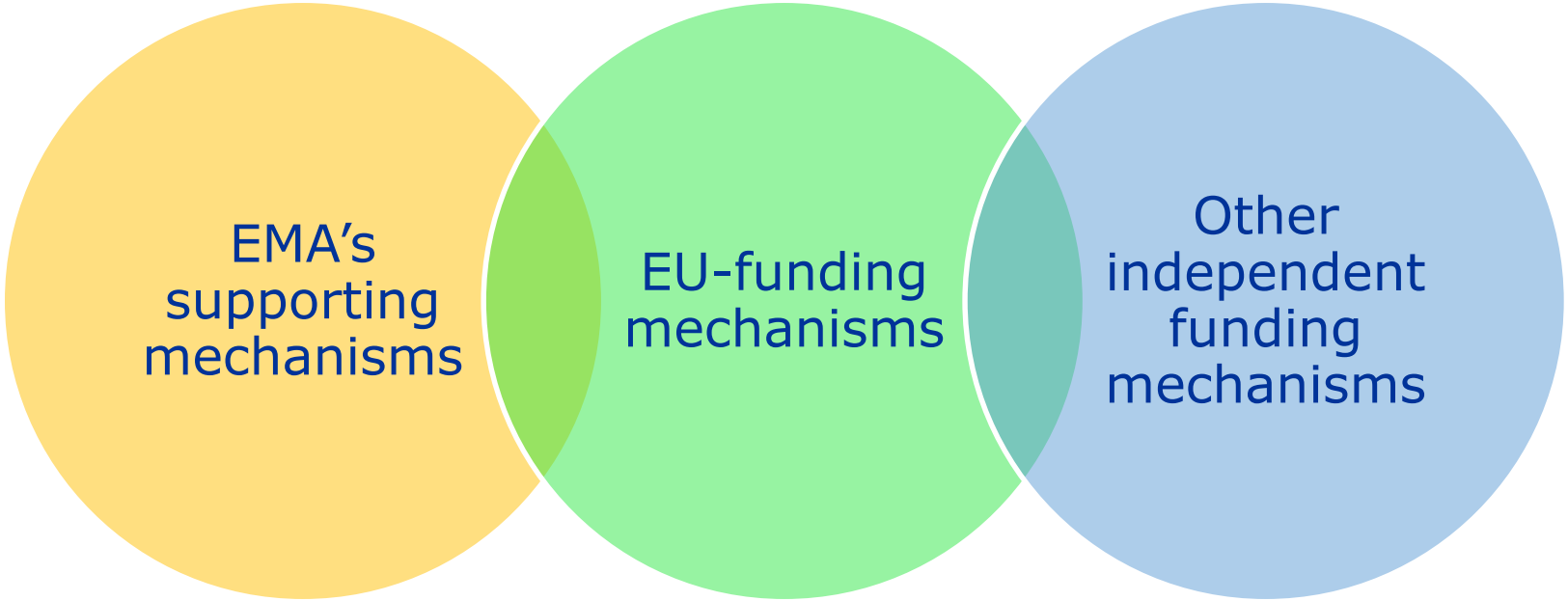
Q2

- Support targeted studies to advance paediatric and rare disease medical product development through characterisation of the natural history of the diseases/conditions, identification of genotypic and phenotypic subpopulations, and development and/or validation of clinical outcome measures, and biomarkers.

Q3

- Pre-clinical models: design new toxicology studies building on the review of data already available for new toxicology models (organoids and foetal stem cells systems) in foetal, young and old animals.

Implementation mechanisms



Questions to you

- Would you consider the proposed methodology appropriate for developing the Research Agenda?
- Would you think the 14 core recommendations cover your research interests and needs?
- What funding mechanisms you think would facilitate implementation of the Agenda?
- Other considerations, please?

Any questions?

Further information

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