Academic perspectives on the need for regulatory scientific research

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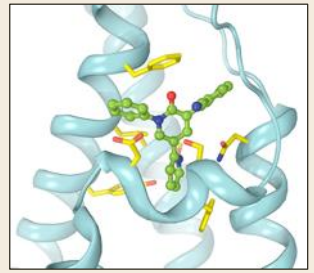
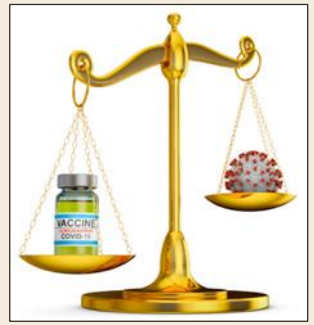
Rationale for EMA-academia collaboration in regulatory science

EMA's role is to protect and improve human health and, specifically, to catalyse and enable science to be translated into patient-centred healthcare. This requires:

- mastery of the relevant science fundamentals, and their application in the drug product review and approval process, and
- being critically informed of key areas of scientific innovation that have the potential to impact on EMA's core business…
- … including scientific advice, evaluation of risks and benefits, stakeholder engagement, and drug product lifecycle management.





Why is there a need for research in regulatory science?

1. The regulator's rationale

- Translation of discoveries in fundamental biomedical science into improved clinical practice demands rigorous procedures to evaluate the safety, efficacy and quality of potential new therapies and diagnostic tools.
- "Regulatory science", therefore, must produce the basic knowledge and understanding essential for robust regulatory decision-making.
- In this way, regulators are equipped to apply scientific advances and cutting-edge technological tools to enable objective assessment of the benefit/risk ratio of new medicinal products.
- **Academia represents a significant resource (facilities, people, grey matter) through which regulators can address evolving questions/needs.**

Why is there a need for research in regulatory science?

2. The regulatory-academic intersection

- Scientific research depends on hypothesis-testing through experimentation, and regulatory science is no exception.
- Challenging scientific questions for regulators are evolving from the most active and competitive areas of biomedical science.
- For example, transformational, cutting-edge research on cell-based therapies, genomics-based diagnostics, drug-device combinations, novel clinical trial design, modelling and simulation, predictive toxicology, real-world evidence, and the impact of 'big data'/A.I.
- **Academics, presented with regulatory science challenges (and the right incentive!) will respond.**
- **A synergistic and collaborative partnership offers a win-win for both regulator and academic.**

Mechanism for EMA-academia collaboration in regulatory science

- Synergistic collaborations between EMA and academic research centres to establish novel science and innovation platforms
  - that undertake fundamental research in strategically key areas of regulatory science, and
  - upon which emerging innovations demanding, in a relatively short time-span, new regulatory competency, methods and/or tools to be developed.
- Identify research priorities that promote regulatory science - including breakthrough research, development of new technology tools, education, and scientific exchange – and facilitate authorisation of safe, effective and high-quality medical products.

Fundamental research

Applied research

Education; manpower

Training; dissemination

Horizon-scan; exchange

Justification for regulatory science research

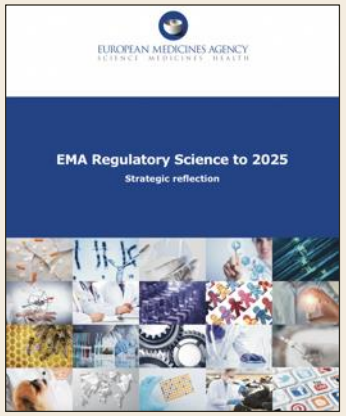
- Realising a paradigm shift in regulatory science and innovation is possible because of
  - the strength of the pharmaceutical and biotechnology industries in Europe,
  - the high-level regulatory expertise at EMA, and
  - the world-leading quality of biomedical research related to medical product innovation and development in European universities.
- A synergistic partnership between EMA, the European regulatory network, academic researchers and established pharmaceutical companies and SMEs, offers the opportunity for…
  - effective translation of clever research ideas into new and effective medical products,
  - development of novel tools to facilitate the regulatory review and approval process and, thereby,
  - accelerating patient access to innovative therapies.

Return-on-investment in regulatory science research

- The US Food & Drug Administration (FDA) currently funds four *Centres of Excellence in Regulatory Science & Innovation*, each with a particular focus associated with the Agency's priority areas.
- The UCSF-Stanford Center aims to develop new models and methods for moving drugs and other medical products (e.g., devices and cell-based therapies) from laboratory to clinical trials.
- The Centre provides training and educational programs (including internships and laboratory rotations) for PhD students, postdoctoral fellows, faculty and scientists in industry and at FDA.
- FDA also funds *The Center for Research on Complex Generics* at the Universities of Maryland and Michigan to enhance research collaborations with the generic industry and further FDA's mission to increase access to safe and effective generic products.
- 5-year funding cycles supported by user-fees.

EMA regulatory science to 2025

➡ RSRN launch event



- Develop network-led partnerships with academia to undertake fundamental 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 share knowledge, expertise and innovation across the regulatory network and to its stakeholders.
- **Investment is key: Horizon Europe, Innovative Health Initiative (IHI), national funding bodies…**

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- The views expressed in this presentation are mine alone and do not reflect the official policies of the FDA or the U.S. Department of Health & Human Services; nor does any mention of trade names, commercial practices, or organization imply endorsement by the United States Government.