

EMA Regulatory Science to 2025: Strategic Reflection

Summary of Strategic Goals, Core Recommendations, and Underlying Actions

Strategic goal 1: Catalysing the integration of science & technology in medicines development Support developments in precision medicine, biomarkers and 'omics

1. Support developments in precision medicine, biomarkers and 'omics

- Enhance early engagement with novel biomarker developers to facilitate regulatory qualification
- Address the impact of emerging 'omics' methods and their application across the development life cycle
- Evaluate, in collaboration with HTAs, payers and patients, the impact of treatment on clinical outcomes measured by biomarkers.

2. Support translation of advanced therapy medicinal products (ATMPs) into patient treatments

- Identify therapies that address unmet medical need
- Provide assistance with early planning, method development and clinical evaluation
- Support evidence generation, pertinent to downstream decision-makers
- Address the challenges of decentralised ATMP manufacturing and delivery locations
- Raise global awareness of ATMPs to maximise knowledge sharing, promote data collection

3. Promote and invest in the PRIME scheme

- Invest in external communication to better explain and promote PRIME
- Evaluate current capacity and identify areas for increased investment
- Shorten the time between scientific advice, clinical trials and MAA submission
- Collaborate with stakeholders to ensure efficient oversight post-approval
- Leverage collaboration with patients, healthcare professionals, academia and international partners

4. Facilitate the implementation of novel manufacturing technologies

- Recruit expertise in novel manufacturing technologies to enhance the assessment process
 - Identify bottlenecks and propose modernisation of relevant regulations to facilitate novel manufacturing
 - Address regulatory challenges in point-of-care manufacturing, e.g. concept of batch control, role of the Qualified Person
- Facilitate a flexible approach in application of Good Manufacturing Practice

5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

- Define how benefit-risk of borderline products is assessed and communicated
 - Enrich expertise at the interface between medicines, medical devices and borderline products
 - Facilitate the regulatory pathway between notified bodies and medicines' regulators
- Gain insight in innovation on drug-device combination products via horizon scanning

6. Develop understanding of, and regulatory response to, nanotechnology and new materials in pharmaceuticals

- Raise awareness of new nanomedicines and materials via the EU-Innovation Network
- Generate guidance addressing PK/PD requirements and long-term efficacy and safety
- Develop guidance on regulatory pathways with device regulators and notified bodies

7. Diversify and integrate the provision of regulatory advice along the development continuum

- Promote more integrated medicines development aligning scientific advice, clinical trials approval and Good Clinical Practice oversight
- Create complementary and flexible advice mechanisms to support innovative product development expanding multi-stakeholder consultation platforms
- Facilitate translation of innovation via a re-engineered Innovation Task Force and synergy with an evolving EU-Innovation Network platform

Strategic goal 2: Driving collaborative evidence generation – improving the scientific quality of evaluations

8. Leverage non-clinical models and 3Rs principles

- Stimulate developers to use novel pre-clinical models, including those adhering to the 3Rs
- Re-focus the role of the 3Rs working group to support method qualification
- Encourage implementation of IT tools to exploit the added value of SEND for the re-analyses of non-clinical studies to support both clinical trials authorisation FIM (first-in-man) and risk minimisation across EU

9. Foster innovation in clinical trials

- Drive adoption of novel practices that facilitate clinical trial authorisation, GCP and HTA acceptance
- Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines
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10. Develop the regulatory framework for emerging clinical data generation

- Develop methodology to incorporate clinical care data sources in regulatory decision-making
- Modernise the GCP regulatory oversight to enable decentralised models of clinical trials coupled with direct digital data accrual
- Develop the capability to assess complex datasets captured by technology such as wearables
- Facilitate training and understanding of healthcare professionals and patients to access and participate effectively in such trials

11. Expand benefit-risk assessment and communication

- Expand the benefit-risk assessment by incorporating patient preferences
- Develop the capability to analyse Individual Patient Data to support decision-making
- Promote systematic application of structured benefit-risk methodology and quality assurance systems across the network
- Improve communication with HTAs and payers regarding therapeutic context, comparison vs. placebo/active-control, patient perspective
- Enhance structured benefit/risk assessment to improve communication to the public
- Incorporate academic research into evidence-based benefit-risk communication

12. Invest in special populations initiatives

- Focus on speedy access for patient (sub-populations in urgent need Identify areas of highest unmet needs where clinical care data can supplement clinical trial data
- Enhance multi-stakeholder advice in collaboration with patients, HCPs, payers and HTAs
- Progress implementation of the joint EMA/EC paediatric medicines action plan
- Progress implementation of the geriatric strategic plan
- Develop a strategic initiative in maternal-fetal health

13. Optimise capabilities in modelling, simulation and extrapolation

- Enhance modelling and simulation and extrapolation use across the product lifecycle and leverage the outcome of EU projects
- Promote development and international harmonisation of methods and standards via a multi-stakeholder platform
- Increase capability and redesign the operations of relevant working parties to ensure wider knowledge exchange

14. Exploit digital technology and artificial intelligence in decision making

- Establish a dedicated AI test "laboratory" to explore the application of innovative digital technology to support data-driven decisions across key business processes
- Develop capacity and expertise across the network to engage with digital technology, artificial intelligence, cognitive computing, and their applications in the regulatory system

Strategic goal 3. Advancing patient-centred access to medicines in partnership with healthcare systems

15. Contribute to HTA's preparedness and downstream decision making for innovative medicines

- Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans
- Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment
- Discuss with HTAs guidance and methodologies for evidence generation and review
- Contribute to the identification of priorities for HTAs
- Monitor the impact of decision-maker engagement through reviews of product-specific experience

16. Bridge from evaluation to access through collaboration with payers

- Contribute to the preparedness of healthcare systems by creating opportunities for collaboration on horizon scanning
- Enable involvement of payers' requirements in the prospective discussion of evidence generation plans
- Clarify the treatment-eligible patient population included in the labelling, and its scientific rationale
- Participate in discussions clarifying the concept of unmet medical need

17. Reinforce patient relevance in evidence generation

- Enhance patient involvement in EMA scientific committees
- Coordinate Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis
- While validating PROs, address patients' needs and leverage patients' expertise
- Co-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment
- Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation

18. Promote use of high-quality real-world data (RWD) in decision making

- Create a sustainable, quality assured, flexible framework delivering rapid access to and analysis of representative, longitudinal RWD throughout a product's lifecycle
- Develop a capacity that will enable the Agency to rapidly and securely access and analyse large amounts of healthcare data
- Accelerate the implementation of a learning regulatory system based on electronic health records and other routinely collected clinical care data (including RWD)

19. Develop network competence and specialist collaborations to engage with big data

- Implement the core recommendations emerging from the HMA-EMA Joint Big Data Taskforce addressing areas such as harmonisation of data standards, characterisation of data quality, and provision of regulatory guidance as to acceptability of evidence
- Engage proactively with new stakeholders relevant to the big data landscape
- Invest in capacity building across the network to acquire new skills to engage with these emerging areas

20. Deliver improved product information in electronic format (ePI)

- Enable real-time interactivity within the Summary of Product Characteristics and Patient Leaflet
- In conjunction with healthcare providers and patients, develop a strategic plan to deliver the ePI programme
- Enable the reuse of structured medicinal product information by third parties through development of a standardised interface
- Address the need for PI content improvements identified in the EC report, such as package leaflet layout and readability

21. Promote the availability and support uptake of biosimilars in healthcare systems

- Further develop strategic communication campaigns to healthcare providers and patient organisations to reinforce trust and confidence
- Enhance training of non-EU regulators in the evaluation of biosimilars with extension to all therapeutic areas
- Address regulatory challenges in manufacturing e.g. statistical assessment of CQAs in the comparability exercise and the evolution of multisource biologicals/biosimilars

22. Further develop external engagement and communications to promote trust and confidence in the EU regulatory system

- Develop content strategy, particularly in key public health areas and hot topics in regulatory science
- Enhance professional outreach through scientific publications & conferences
- Proactive approach to key public-health areas (e.g. vaccines)
- Improved communications for patients, healthcare professionals, HTAs and payers
- Develop more targeted and evidence-based communication facilitated by updated web content and format

Strategic goal 4: Addressing emerging health threats and availability/therapeutic challenges

23. Implement EMA's health threats plan, ring-fence resources and refine preparedness approaches

- Coordinate scientific and regulatory activities within the EU network
- Evaluate preparedness for emerging pathogens and 'disease X'
- Coordinate discussions with the EU network, international partners and stakeholders on the identification, development, authorisation and post-authorisation follow-up of relevant medicinal products
- Effective and timely communication to healthcare professionals, the public and regulatory partners

24. Continue to support development of new antibacterial agents and their alternatives

- Evolve regulatory guidance and support alternative approaches to new antibacterial drug development and innovative approaches for prevention and treatment of infections
- Support initiatives, such as the clinical trials network, to facilitate and accelerate clinical development
- Encourage new business models that provide "pull" incentives beyond the current "funding research" strategy in the EU
- In collaboration with HTAs and payers, define the evidence requirements for new antibacterial medicines
- Support the development and application of rapid diagnostic tools

25. Promote global cooperation to anticipate and address supply problems

- Build on deliverables from the work plan of the HMA/EMA Task Force on availability of authorised medicines
- Explore mechanisms to increase manufacturing capacity in Europe and internationally
- Enhance collaboration with WHO in the area of supply disruptions due to manufacturing quality issues
- Promote greater knowledge exchange with international stakeholders on shortages due to quality/manufacturing issues
- Continue to engage with healthcare professionals, patients and consumers organisations and the industry to address the causes and consequences of lack of medicines' availability
- Support international harmonisation of regulatory science standards for complex generic medicines addressing bioequivalence, waivers and modelling

26. Support innovative approaches to the development, approval and post-authorisation monitoring of vaccines

- Advance methods/tools (e.g. biomarkers) to characterise immune response and to support definition of vaccine quality attributes
- Examine innovative clinical trial approaches to expedite vaccine development
- Engage with public health authorities and NITAGs to better inform vaccine decisions
- Establish a platform for EU benefit-risk monitoring of vaccines post-approval
- Communicate proactively with key stakeholders on benefit-risk using evidence-based tools to tackle vaccine hesitancy

27. Support the development and implementation of a repurposing framework

- Enhance regulatory advice on evidence generation and MAA submission
- Frame suitability of third party data-pooling, relevant RWD and historical non-clinical datasets
- Translate experience with EMA's registry pilot to guide RWD collection
- Explore utility of low-intervention clinical trials for evidence generation

Strategic goal 5: Enabling and leveraging research and innovation in regulatory science

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

- Identify, in consultation with academia and relevant stakeholders, fundamental research topics in strategic areas of regulatory science (such as PROs, omics-based diagnostics, drug-device combinations, modelling and simulation, big data, and artificial intelligence)
- Proactively engage with DG Research & Innovation, DG-SANTE, IMI and Member State funding agencies to propose and issue calls to establish research collaborations

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

- Ring-fence EMA funding to address rapidly-emerging regulatory science research questions (such as diagnostics, precision medicine, distributed manufacturing, wearable devices, drug re-purposing)
- Ensure close interaction between network scientists and academia to deliver tangible impact through translation of this applied research into new drug products and regulatory tools
- Actively engage, through these applied projects, in training early-career researchers in regulatory science (e.g. via placements within the network)

30. Identify and enable access to the best expertise across Europe and internationally

- Invest in a knowledge management system to track innovation, share information, enable linkages and create new insights across the product lifecycle
- Facilitate more flexible access to global expertise in regulatory science and increasingly specialised and new areas of innovation therapies

31. Disseminate and exchange knowledge, expertise and innovation across the network and to its stakeholders

- Engage with academia to develop regulatory training modules, including describing innovation of new medicines and their progression from laboratory to patient
- Conduct horizon scanning in key areas of innovation via collaborations with academia, the EU-Innovation Network and ICMRA
- Drive a data-sharing culture to foster open science which is mutually beneficial for all stakeholders