

Is Europe a leader in healthcare's digital transformation?

Six bold actions for modern,
future-proofed regulation

In early 2023 we set out six bold actions for regulatory reform in Europe. Yet in the context of the European Commission's review of the General Pharma Legislation, the dramatic emergence and promise of a modern use of data and Artificial Intelligence (AI) in medical research, and the growing challenges of health systems (workforce, financial, demographic), we reiterate our bold actions and call on them to be upheld, and bolder still!

Our vision for the EU regulatory framework is that it serves the needs not only of today but also tomorrow. It must integrate with speed and agility what we can already anticipate from the dynamic and accelerating digital transformation of healthcare:

1. Modernising to interactive, dynamic, and digitally enabled regulatory assessments

New tools, types, and sources of data (e.g. patient reported outcomes, digital biomarkers, DARWIN¹ EU studies, imaging, EHDS²) enrich the evidence generated across the development and approval of medicines, and into clinical practice.

A digitised, cloud based regulatory platform is a precondition for interoperability and iterative interaction with developers across and within the EU Health Authority Network, and with other regions, increasing efficiency and agility in the delivery of EMA's mission.

A modernised framework also enables the involvement of notified bodies in scientific advice for medicines combined with in vitro diagnostics (IVDs) or devices.

The framework has expedited pathways for meeting patient-centered, unmet medical needs.

2. Assigning EMA as an orchestrator authority on integrated solutions³

Healthcare innovations are increasingly at the intersection of medicines and technology such as medical devices, IVDs, and digital tools. Due to different frameworks, the overall regulatory environment is highly complex and difficult to predict and navigate. AI is further compounding this challenge.

It is imperative that EMA's role is expanded to streamline, orchestrate and signpost regulatory pathways for today and tomorrow's integrated solutions.

This will help foster innovation as it provides industry with the needed predictability of a well-defined process to determine the pathway.

3. Creating a sandbox mechanism for future novel healthcare and regulatory solutions

A regulatory sandbox mechanism anticipates and addresses future gaps in regulatory frameworks. Novel products and solutions will emerge over time that don't have a clear pathway, or only highly complex and unattractive pathways.

The proposed regulatory sandbox needs to expand beyond pharmaceuticals, to include medical devices, digital tools and IVDs but also taking into account Advanced Therapeutic Medicinal Products (ATMPs) and Substances of Human Origin (SoHO). It needs also to connect with other sandbox mechanisms already enacted, e.g. in the future AI Regulation.

Synthetic data⁴, for example, is an emerging field with potential in data sharing, AI system training, and to enrich evidence. The field is young and evolving fast. A regulatory sandbox enables exploration, evaluation, and insight into how best to regulate the use of synthetic data, fostering a quicker uptake.

4. Transforming EMA's Committee Structure to focus and leverage scientific expertise

Scientific expertise in special fields (e.g. paediatrics, orphans, ATMP) is scarce and the workforce in healthcare systems severely constrained. At the same time, additional expertise is needed on data/ AI and digitalisation.

EMA's structure needs to be more agile to absorb the evolving digital transformation of regulatory systems. Moreover, the combination of scientific and digital expertise needs to be applied in a focused way. This calls for EMA efficiency in the organisation of work with experts from Member States.

A full committee structure of all Member States must be reserved for the scientific decision making committees (CHMP, PRAC). We support the proposal to transfer other technical committees into a lighter and more efficient structure of working parties or advisory groups.

While preventing conflicts of interest is crucial, the regulatory process overall needs that experts and committees maintain the requisite flexibility to appoint appropriate experts to ensure best scientific expertise.

5. Securing sufficient EMA funding and optimal resource allocation

The digital transformation of the Agency is consistent with the European Commission's goals of the EU Digital Decade and a future Digital Society. We see encouraging engagement, training and expertise being developed in AI across the network.

Appropriate infrastructure and resource funding is essential to meeting the EU's healthcare and digitalisation ambitions and to the EMA and its network of national agencies in reaching their goals.

Digitalisation requires specific expert skills (e.g. data science, digital technologies, and algorithmic) and new ways of working in order to access the ecosystem efficiencies and patient outcomes available from interoperable cloud based platforms and phased/ dynamic assessment.

6. Formalising the informal collaboration and work sharing of global pandemic times

Platform and cloud technologies (e.g. DEEP⁵, Accumulus⁶) allow collaboration and work sharing approaches beyond borders.

This can promote regulatory productivity in the delivery of public health and on common issues such as global supply shortages.

Cloud solutions can enable scaling of phased review and ultimately the dynamic assessment that modern digital knowledge management systems can now support.

Given expert shortages, all major agencies are encouraged to consider scientific work sharing activities based on trust and reliance.

EMA's international work assessment collaboration is crucial to advancement of science in global clinical developments in the future. However, a mandated blending of the scientific remit of EMA with the economically focused remit of HTA bodies and payers could add to the complexity and cost of pharmaceutical development and challenge global regulatory collaboration and harmonisation efforts.

We urge the European Parliament and Council to consider this perspective as part of the review of the EU's General Pharmaceutical Legislation. The finalised legislation will be decisive: To European healthcare innovation, to health systems, and to patients and society.

Roche supports [EFPIA's Regulatory Road to Innovation](#) and further highlights and asserts the need for bold and ambitious reforming actions to support the European Commission's goals on innovation, access and availability for the long term.

¹DARWIN: Data Analysis and Real World Interrogation Network hosted by EMA

²EHDS: European Health Data Space

³Integrated solutions include a combination of therapeutics, diagnostics, technologies, tools and/or data intended to address gaps in the patient journey. See also "[When healthcare joins the dots](#)".

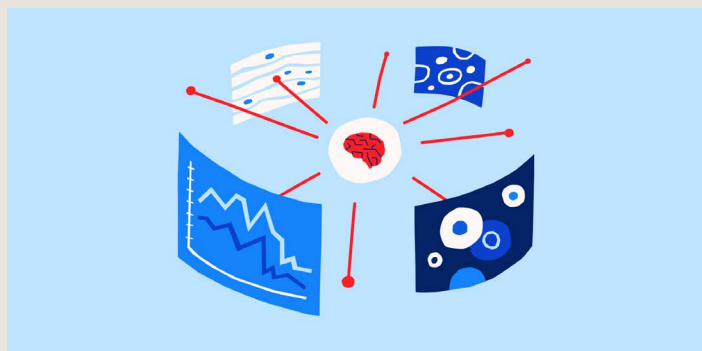
⁴Synthetic data are artificial data generated by simulation to mimic complex clinical relationships and are clinically indistinguishable from real data, but contain no real patient's individual data.

⁵DEEP: Digital Evidence Ecosystem & Protocols: A pre-competitive industry consortium prototype platform to accelerate utilization of digital measures in clinical research.

⁶Accumulus: <https://www.accumulus.org/>: global nonprofit organization developing a communication and collaboration platform for the life science-regulatory ecosystem

Six Bold Actions for regulatory reform

Why we now reiterate and underscore

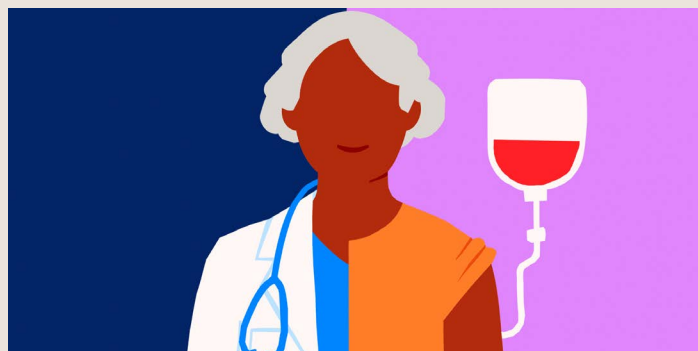


Artificial Intelligence (AI)

The digital transformation of healthcare has been predicted for more than 20 years: With the advent of large language models over the past several months society is alerted to both the opportunities and potential risks (known and unknown) of AI. In general, and also in healthcare.

The European Union (EU) is a pioneer of policy and legislation in the digital domain. The Artificial Intelligence (AI) Act and the regulation on the European Health Data Space (EHDS), among others, will ensure that we unlock the potential of digitalisation in line with the core values of the EU, protecting the rights, safety, and well being of citizens. However, fragmentation and outdated regulatory frameworks remain a challenge to the pace of delivering new medicines and solutions to health systems and patients.

The General Pharmaceutical Legislation must now ensure that the regulatory framework can both anticipate and address this new, fast-moving reality, and be future-proofed to accommodate with speed and agility, the further progress to come in the years ahead.



Health system constraints

Health systems globally are at breaking point, wrestling with surging demand and staff shortages. Similarly, health authorities are also called on to assess ever more complex products, often drawing on different fields, and calling for multidisciplinary expertise. Smart, fully digitally enabled regulation of medicines can help bridge the gap of healthcare system staffing pressures. It can also help foster dynamic innovation towards unmet needs in the clinical care of patients.

Digital data is becoming the norm of clinical evidence whether in using computational tools to surface the genes driving cancer, making real world evidence meaningful, combining drug and device solutions, optimising clinical workflow and decision support, or the use of digital endpoints in clinical trials. While the EMA was the first authority anywhere to approve the use of a primary digital endpoint in a clinical trial, the digital transformation of healthcare and the increasing prevalence of innovative, integrated solutions requires a fully modernised, fit-for-purpose regulatory framework.

We call on EU legislators to balance the regulatory framework to facilitate appropriate levels of oversight (that ensure newly approved products are safe, efficacious and of high quality) while providing the foresight and flexibility needed to accommodate the next generations of healthcare and therapeutic solutions.

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