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European Federation of Pharmaceutical Industries and Associations

EFPIA'S REGULATORY ROAD TO INNOVATION COULD HELP RESTORE EUROPE AS WORLD LEADER

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AN INNOVATION PERSPECTIVE

As Europe re-evaluates its pharmaceutical policy framework, the challenge for the next decade is not if medical innovation will happen but where it will happen. The European Union (EU) has been eclipsed by the US as a world leader in medical innovation and faces increasingly intense competition for life science investment from China, Asia and other regional actors.[1]

For instance, in the past decade the global share of clinical trials located in Asia grew from 16% in 2010 to 24% (2020), Northern America's share remained constant at 31% whereas Europe's share fell from 29% to 25%.[2] Europe is also the slowest region to approve new medicines in comparison to the US, Japan, Canada and Australia.[3]

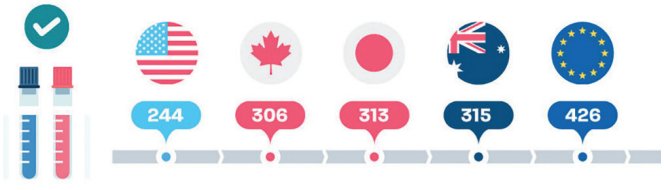

Figure 1: EMA takes **426 days** to approve a new active substance, compared to 244 days in the USA, 306 in Canada, 313 in Japan or 315 in Australia.

Where innovation happens matters; to patients, to health systems, to academia and SMEs. It matters for jobs, resilience and economic growth in Member States and across the EU. If pioneering research continues to leave towards other regions of the world, so will the opportunity to deliver the best care to patients across Europe.

The pharmaceutical industry's R&D pipeline is highly innovative, with almost half of the therapies in development being new products, over 40% of the pipeline targeting rare diseases, and ground-breaking cell and gene therapies growing in importance.

As many promising new therapies across different disease areas may become available over the next years, the revised European framework must ensure that the European Medicines Agency (EMA) remains a strong and sustainable regulatory authority globally; more efficient processes, strategic resourcing and enhanced capabilities are required.

REGULATORY ROAD TO INNOVATION PROPOSAL

In particular, EFPIA has identified **eight actions** that can be achieved now, within the existing legislative framework, and **four areas** for the future legislation, to ensure that Europe can be at the forefront of the development and authorisation of these next generation of diagnostics, treatments and vaccines.

The decisions we make over the coming weeks and months will define Europe's future ability to discover, develop and deliver the next generation of diagnostics, treatments and vaccines as well as ensure faster and more equitable access for patients across Europe.

By working together to evolve the regulatory framework that supports pharmaceutical innovation, we have the potential to regain our position as a world leader in medical innovation.

ABOUT US

The European Federation of Pharmaceutical Industries and Associations (EFPIA) is the trade association representing the research-based biopharmaceutical industry in Europe. EFPIA's mission is to create a collaborative environment that enables our members to innovative, discover, develop and deliver new therapies and vaccines for people across Europe.

Discover more on the Regulatory Road to Innovation

1. Revision of the EU pharmaceutical legislation: Directive 2001/83/EC on medicinal products for human use and Regulation (EC) No 726/2004 on the European Medicines Agency.
2. EFPIA, Pipeline Review 2021, slide 8, https://www.efpia.eu/media/602564/iqvia_efpia_pipeline-review_final.pdf
3. CIRS, RD Briefing 81, New approvals in six regulatory authorities 2011-2020, https://cirsci.org/wp-content/uploads/dlm_uploads/2021/06/CIRS-RD-Briefing-81-6-agencies-v5.pdf

THIS IS EFPIA'S REGULATORY ROAD TO INNOVATION

	Issue	Recommendations
1	Whilst **Complex clinical trials** (CCTs) have been available for many years and despite a recent uptake of Decentralised Clinical Trials (DCTs) during the pandemic, their use is still limited today. Despite the increasing importance of **biomarkers**, current procedures for their use are lengthy and complex.	Continue the multi-stakeholder dialogue to increase awareness and acceptance of CCTs and DCTs and further develop guidance. The new Clinical Trials Regulation should be an enabler rather than a hurdle for innovation CTs. EMA to streamline the biomarker qualification process and design guidance to support navigating the process.
2	The full potential of **RWE/RWD** is not being realised under the current regulatory framework.	EMA/Heads of Medicines Agencies to develop and adopt guidance for a RWD/RWE framework with clear principles for data quality and interoperability, access, analysis and regulatory acceptance.
3	The submission of one **regulatory** dossier only at the end of the **drug development process** limits innovator's ability to identify issues in their approach or gaps in their data package during the process.	EMA to work with stakeholders to design a regulatory pathway which includes a process for seeking early and iterative dialogue on data as they are generated and using international data standards and technology.
4	1 in 4 medicines approved at EU level includes a medical device component. While medicines are assessed by the EMA, devices are regulated by national authorities.	The possibility of a parallel advice with EMA and Notified Bodies and an integrated EU pathway for the assessment of **drug-device combinations** and in vitro diagnostics.
5	**Unmet medical need** is referenced from drug discovery, selection of eligible products to accelerated regulatory processes, to pricing and reimbursement without a shared understanding of its definition.	European Commission (EC) and EMA to ensure a coordinated, multi-stakeholder approach to determining what constitutes an unmet medical need, in particular working with the patient community.
6	Digitalisation can s**peed up and increase quality** in the R&D manufacturing processes as well as make compilation and assessment of regulatory information more efficient.	Medicine developers and EU regulatory network to ensure that the infrastructure, data security framework, and mindset required are in place to realise the potential of digitalisation.
7	**Agile and resilient supply chains** need to cope with different sources of disruption to ensure patient needs are met.	EMA and EC to set up a European reporting system, with a common definition of 'shortage', real time information and a streamlined alert system utilising the data in the European Medicines Verification System.
8	The current framework for updating the terms of a marketing authorisation **(variation regulation)** is inflexible and carries too high administrative burden for both industry and regulators.	EC and EMA to evolve the legislation to incorporate principles and tools described in ICH Q12 guidance. This includes extending risk-based approaches for well-characterised products and developing vaccine-specific annex to EU Variations guideline.
9	Europe is the slowest region to approve new medicines in comparison to the US, Japan, Canada and Australia.	EMA and EC to shorten times-frames from EMA approval to EC decision and reconsider EMA committee structure to enhance Member States ability to bring forward their best expertise.
10	The use of expedited regulatory pathways (ERP) such as PRIority Medicines Evaluation scheme (PRIME), needed for a future-proof regulatory framework, has been limited in Europe.	A suite of effective ERPs should be put in place and adequately resourced so they can be leveraged to support the introduction of innovative therapies to patients.
11	The EU regulatory oversight of medicines and medical devices remain profoundly different. This creates **great uncertainty for combination products (>25% of the current pipeline)** at the interface between the different legislations.	EC to create a new legal category and streamlined process for those combinations of medicines and medical devices so that they are regulated and assessed as medicinal products in the EU.
12	Under current EU legislation, the paper version of a medicine patient information leaflet is mandatory , whilst electronic Product Information (ePI) ensures that healthcare professionals and patients always have access to latest approved Information.	EC to ensure legislative readiness to transition from paper leaflets to ePI while taking into account the elderly population and those who may not have access to computers or mobile technology.

Table 1: Defined proposals that cover EFPIA's Regulatory Road to Innovation. Proposals 1-8 are actions that can be realized within the current legislative framework, whilst proposals 9-12 are to be reserved for future legislation.