European Federation of Pharmaceutical Industries and Associations

EFPIA resources to inform and support the development of the EU Biotech Act

The following questions under Section 3 - Access to capital did not load, hence EFPIA responses can be found below:

Q6a. *If you would like to indicate why, you can do so here.*
From an SME perspective, growth is closely linked to the US, with the availability of large investment and cross over funds, and NASDAQ liquidities and valuation more efficient than Euronext. In addition, the SME definition in Europe does not fully reflect the complexity of today's Life Science ecosystem. Introducing a "small mid-cap" category with relevant incentives as used in different EU funding instruments would reflect industry reality and ensure that innovative mid-cap companies remain and grow in Europe.

Q7. I*s the EU a priority region under your investment strategy?*
Neutral

Q7a. *If you would like to indicate why, you can do so here.*
In EFPIA report on "factors affecting the location of biopharmaceutical investments", key drivers are: presence of world-class appropriately funded innovation hubs; Enhancement of end-to-end capabilities and funding of disruptive pharma innovation; Appropriate policies along the supply chain to attract ATMP investment; Support of innovation by implementing early access mechanisms, including RWE; Boost EU digital transformation; Adoption of sustainable procurement and pricing policies for innovation; Development of collaborative method to attract biopharmaceutical investments. These elements are weak or missing in Europe.

List of abbreviations used in the EFPIA response to the questionnaire is pasted below (point 5)

Input specific to the EU BioTech Act

- Proposals for a Competitive, Best-In-Class EU Intellectual Property Environment – **full paper pasted below (1)**
- One vision on the regulatory ecosystem
- Key recommendations from the comparative analysis of biopharmaceutical strategies in 10 countries **– pasted below (2)**
- CIRS RD Briefing 101 – New drug approvals by six major authorities 2015-2024
- EFPIA THEMATIC ANALYSIS ON CUMULATIVE LEGISLATIVE IMPACTS https://www.efpia.eu/media/i0ihfkys/efpia-cumulative-legislative-impacts.pdf
- Paediatric Regulatory Simplification Proposals – **full paper pasted below (4)**

Input specific to Strengthening the Manufacturing Base

- EFPIA paper on location of manufacturing – **pasted below (3)**
- Delivering treatments to patients: The medicines manufacturing journey

Input specific to Clinical Trials in Europe

- Improving EU Clinical Trials: Proposals to Overcome Current Challenges and Strengthen the Ecosystem https://www.efpia.eu/media/pl0nag0s/efpias-list-of-proposals-clinical-trials-15-apr-2025.pdf
- Assessing the clinical trial ecosystem in Europe https://efpia.eu/media/3edpooqp/assessing-the-clinical-trial-ecosystem-in-europe.pdf
- EFPIA Vision for 2030+
- EFPIA Press Release - 60,000 fewer clinical trial places for Europeans, despite global surge in research projects
- Unlocking cross-border clinical trials for patients in Europe
- Tracking Availability in China of Medicines Approved in Six Key Global Markets

Input specific to the needs of start-up and scale-up that are different from other sectors

- An Agenda for Action: Expanding Funding Options for Small and Medium-Sized Pharma companies in Europe

Input specific to data and digital (AI):

- EFPIA position on the use of artificial intelligence in the medicinal product lifecycle
- EFPIA position on the Regulation on the European Health Data Space (EHDS)

Input specific to the industry's economic footprint, research pipeline

- Economic footprint of the pharmaceutical industry in Europe https://efpia.eu/media/3dqjpl3x/economic-footprint-of-the-pharmaceutical-industry-in-europe-report.pdf
- 2024 Pipeline Review – Innovation for Unmet Need

Input specific to market conditions for innovation:

- https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/guidance-needed-to-ensure-eu-joint-clinical-assessment-improves-patient-access-to-innovative-cancer-treatments/
- https://www.efpia.eu/media/qrjah2ij/efpia-evidera-research-on-eunethta21-methods.pdf

Input related to industry investment in greening and waste reduction

- EFPIA White Paper on Circular Economy https://www.efpia.eu/media/554663/circular-economy.pdf
- https://www.efpia.eu/more-than-medicine/responsible-innovation/
- EFPIA White Paper on Climate Change https://www.efpia.eu/media/554662/white-paper-climate-change.pdf
- EFPIA submission to the Environment omnibus Have your say: https://ec.europa.eu/info/law/better-regulation/info/law/better-regulation/have-your-say/initiatives/14794-Simplification-of-administrative-burdens-in-environmental-legislation-/F3693516_en
- EFPIA submission to the Circular Economy Act Have your say https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/14812-Circular-Economy-Act/F33113219_en

- https://pharmeco.eu/
- https://imi-premier.eu/
- https://enkoreecohealthcare.eu/
- IHI PFAS open call

Input related to FP10

- EFPIA preliminary response to the European Commission Multiannual Financial Framework package
- EFPIA recommendations for Framework Programme 10

1. **Proposals for a Competitive, Best-In-Class EU Intellectual Property Environment**

The Status Quo from a Bird's Eye View: Intellectual Property (IP) Issues Facing the Innovative Pharmaceutical Industry

The current IP framework, while functional, still has a number of gaps that prevent the EU from being considered as having a best-in-class system when compared to its peers. Furthermore, progress on recent policy proposals, both in the General Pharmaceutical Legislation and the Patent Package, do not meaningfully move the needle to a more competitive IP system, and in some cases, actually do the opposite. The Biotech Act, however, provides an excellent chance to ensure the European IP system is fit-for-purpose and can support a more competitive future for Europe, driving innovation in biotechnology to bring the benefits of rapid scientific advancement to European patients.

The innovative pharmaceutical industry is faced with a number of challenges in its current operating climate. First and foremost, developing innovative medicines fundamentally entails challenging science, very lengthy and risky clinical development, multiple failures of assets that do not reach the market and overall, extensive development and regulatory approval timelines. The sum of these realities is that remaining patent protection is often very short and insufficient to offset these inherent burdens; this was, in effect, the rationale for the introduction of the Supplementary Protection Certificate (SPC) to

create an avenue for at least partial compensation. The challenges, however, have only grown, as the industry is also facing increasing delays before medicines can effectively be made available to meet patients' needs, including lengthy regulatory procedures, as well as sector-unique and steadily increasing data transparency and sharing obligations, which require ever-earlier patenting and undermine incentives for the industry to invest into research and development (R&D). Existing incentives are also broadly undermined by the difficulties in practically, timely and efficiently enforcing IP rights in Europe, such as the SPC manufacturing waiver and the expansion of the exemption to the protection of IP rights in the proposed revision to the general pharmaceutical legislation (GPL).

The Draghi Report underscores the urgency of addressing these gaps, calling for bold reforms to unlock innovation, reduce regulatory fragmentation, and increase investment in digital infrastructure and data ecosystems. Importantly, it recognized IP as a cornerstone of economic growth and competitiveness.

Among its other proposals below, concretely, EFPIA proposes **strengthening the baseline of RDP** (and orphan market exclusivity for orphan medicinal products) compared to the existing legislation or ongoing legislative proposals. In addition, and in light of the challenges described below, while all therapeutics, regardless of whether they are small molecule or biologics, ought to benefit from an increased period of RDP, facilitating biopharmaceutical R&D in cutting-edge technologies could be achieved with an attractive RDP regime for biologics and certain complex therapies that require additional measures to encourage investments.

The rest of this submission outlines in more detail the specifics of these challenges and proposes principles for solutions that can drive a stronger IP system for the benefits of patients.

RDP

Companies submit a significant body of data related to pharmaceutical tests, preclinical tests, and clinical trials as part of the marketing authorisation process. Currently, this data is protected during a set period during which it cannot be relied upon by a follow-on applicant to obtain a marketing authorization, referred to as Regulatory Data Protection (RDP).

The current EU legal framework aimed to create a dynamic and competitive market for medicines as the period of exclusivity also enables off-patent producers to quickly enter the market following the loss of market protection.

However, in the ongoing revision of the General Pharmaceutical Legislation, current discussions on the duration of regulatory exclusivity and potential conditionalities have and will dramatically increase the legal uncertainty of regulatory exclusivity for medicinal products, despite the Regulatory Scrutiny Board's comment that the reduction of the regulatory protection periods could impact "the sector capacity to finance future innovation and international competitiveness" and there could be "unintended consequences for the long-term capacity on innovation, pricing, access, and competitiveness".

This is particularly problematic as RDP may sometimes be the only effective incentive for biopharmaceutical R&D. A curtailed or insufficient RDP framework risks leaving behind many promising therapies.

For instance, limited patent protection may be left for therapies that have taken longer to progress in the pipeline, such as those for rare diseases or other conditions that have inherently lengthy clinical development timelines, e.g. in neurological conditions. Also, groundbreaking new technologies may encounter regulatory and other challenges in progressing though clinical development.

The prospect of a narrow period of regulatory exclusivity leads to uncertainty regarding the return on investment and this can shift the priority to other pipeline assets. In the absence of sufficient incentives, R&D programs of potentially life-saving therapies or therapies that significantly improve the quality of life may be abandoned by companies who have to make difficult decisions to prioritize investment into the most promising and economically viable therapies.

An Extended Regulatory Exclusivity Term

Europe has for several decades been falling behind other regions both in terms of competitiveness of the system and R&D investments. If the EU genuinely seeks to be at the forefront of biopharmaceutical innovation, attracting investments into the development of novel medicines and clinical trial participation for its patients, it must take this opportunity to strengthen – rather than undermine – this critical underlying framework via meaningful, achievable and predictable incentives that actually encourage additional cutting-edge R&D investment relative to today, as opposed to the ongoing proposals in the General Pharmaceutical Legislation.

Concretely, EFPIA proposes strengthening the baseline of RDP (and orphan market exclusivity for orphan medicinal products) compared to the existing legislation or ongoing legislative proposals. In addition, and in light of the challenges described above, while all therapeutics, regardless of whether they are small molecule or biologics, ought to benefit from an increased period of RDP, facilitating biopharmaceutical R&D in cutting-edge technologies could be achieved with an attractive RDP regime for biologics and certain complex therapies that require additional measures to encourage investments.

Such measures would bolster EU competitiveness versus other regions,and thereby revitalise the innovative medicines pipeline and help bring the latest technical advances to European patients.

SPC

The SPC (Supplementary Protection Certificate) Regulation was introduced with the aim stated in its fourth recital (Regulation (EC) No 469/2009):

> 4. *At the moment, the period that elapses between the filing of an application for a patent for a new medicinal product and authorisation to place the medicinal product on the market makes the period of effective protection under the patent insufficient to cover the investment put into the research.*

While this acknowledges the lengthy development timelines which medicinal products must undergo, this nonetheless fails to represent accurately the time that elapses between when an invention is made and when it can actually be placed on the market.

First, in the biopharmaceutical sector, certain therapies – e.g. in the neuroscience space – have required and will increasingly require particularly lengthy development timelines until they actually reach the

patients. It is counterintuitive that those products should suffer from reduced exclusivity prospects, which endangers the very development of highly innovative solutions that can meet patients' needs, especially in these therapeutic areas where demonstrating efficacy is inherently lengthy. Thus, it remains critical to incentivize all innovators to continue developing new therapeutic solutions for patients, even if these may require a longer development time.

Additionally, the current SPC term fails to accurately compensate for the time that elapses between when an invention is made and when it can actually be made available to patients. In most European countries, the primary market for any innovative pharmaceutical product is the state (whether directly, or indirectly via insurers or hospitals). Also, in the majority of countries in the EU, prior to the formal approval of the price and reimbursement of a medicine, only *de minimis* quantities of a product will be sold on the private market.

Yet, currently, the SPC Regulation compensates for the period that elapses between the filing date of the patent and the issuance of the marketing authorisation, but only for a maximum of five years. In addition, the total protection from marketing authorisation to SPC expiry is capped at a maximum of fifteen years.

Thus, while the additional compensation could theoretically reach 15 years after approval, most products with SPCs receive a shorter protection as a result of the 5-year cap on the SPC term and an average time to market of 12-13 years,[1] effectively settling around a median of 12 years of exclusivity from approval. This is further exacerbated by the fact that current regulatory requirements, particularly for clinical trials, induce innovators to file patents earlier than before.

This renders uncertain the return on the high investment necessary to bring these therapies to market, while also negatively influencing companies who have to make difficult decisions to prioritize investment in the most promising, but also economically viable, therapies. A revision to the current calculation of the SPC term could ensure that all promising medicines have appropriate incentives for development.

SPC duration cap adjustment

EFPIA proposes an adjustment to the SPC cap, which would address some of these challenges and better promote R&D incentives and innovation. A more competitive SPC regime could be achieved by removing the 5-year cap on the SPC term, while retaining the 15-year cap from marketing authorisation. This would allow innovating companies to rely on the certainty of the full 15 years following its first approval in the EU to incentivize the significant investments necessary for an invention to reach patients, especially in these therapeutic areas which require particularly lengthy development timelines and may therefore currently be discouraged.

In effect, the eligibility of the SPC for a new medicine would remain subject to the condition that the medicine should be approved within the usual 20-year term of the underlying patent for the SPC and would not extend the duration of the SPC for medicines that manage to progress rapidly through development until approval. As such, the proposed revision does not unduly extend the term of patent protection of the underlying invention for an overly long period of time. Lastly, this solution would be readily applicable to any SPC based on a Unitary Patent, once the relevant legislation ultimately goes into effect.

[1] https://efpia.eu/media/2rxdkn43/the-pharmaceutical-industry-in-figures-2024.pdf

By removing this cap on the duration of SPC, while retaining the total maximum exclusivity allowable under the SPC Regulation, the EU would be sending a strong signal and commitment to supporting the competitiveness of the innovative pharmaceutical industry, ultimately for the benefit of patients, as well as reflect its understanding that institutional delays are inherent in bringing a new pharmaceutical product to market. Adopting this approach will also strengthen the EU's position when it engages in FTA negotiations with states that have an SPC-analogous system. Extending the duration of protection in such third-party states, by similar reasoning, will benefit the domestic EU innovative pharmaceutical industry.

Paediatric Incentives

The conduct of a Paediatric Investigation Plan (PIP) is compulsory for most new medicines, as well as for new indications and certain other developments of medicinal products while they still benefit from patent-based protection. Each PIP typically contains several studies, and the preparation and conduct of these requires substantial investment, resources and time on the part of the developer. Moreover, patient recruitment into PIP studies can be very challenging and result in extended timelines for these trials. Where a product is developed for multiple different medical conditions, multiple PIPs will be required, necessitating significantly more effort and resources.

Provided that a PIP is completed as agreed with the EMA and on time, in accordance with the Paediatric Medicines Regulation, there are two possible types of rewards that may be granted:

- **For non-orphan medicinal products**: A 6-month extension of the Supplementary Protection Certificate (SPC) may be granted, only once per product/SPC, even if more than one PIP for the product has been complied with and completed on time, despite the additional efforts and investment that will have been required on the part of the developer in that circumstance.
- **For orphan medicinal products**: An additional 2-year Orphan Market Exclusivity (OME) period can be granted where a PIP has been complied with and completed on time. Under current legislation it is possible to have separate OME periods (and the potential for separate 2-year paediatric extensions) where the product is approved for additional, entirely separate orphan designated conditions. The separate OME periods, and the paediatric OME extension, are both removed in the proposed revisions to the general pharmaceutical legislation (GPL).

Under current law, companies can be granted a PIP waiver on the grounds that the condition the product is intended for occurs only in adults. However, recognizing that many unmet needs remain for this special population, pursuant to the proposed GPL, companies will in the future be required to develop their medicines for children if, based on a product's mechanism of action (MoA), the compound could be effective in treating a different, paediatric condition. Conducting PIPs based on the MoA of the product, rather than in the intended adult condition, will bring benefits to paediatric patients whilst entailing a significant expansion of developers' current obligations. The new and challenging scientific and development efforts required to conduct MoA PIPs addressing unmet medical needs in children will potentially involve new and innovative technologies and methods, and may take longer, than other PIPs. Consequently, it may be more difficult to complete such PIPs, especially within the prescribed deadline for obtaining the reward, making the chances of obtaining one lower.

In consideration of all these factors, expanded and proportionate rewards for paediatric medicines development, where PIPs are successfully completed, would be appropriate.

Paediatric Rewards

EFPIA proposes to improve the SPC protection for medicines developed to treat paediatric conditions. In order to implement an expanded and more proportionate reward model for paediatric medicines, the incentives framework could provide:

- A maximum of two SPC extensions per product if two different PIPs in two different conditions/indications are completed; and
- A longer reward for PIPs based on Mechanism of Action (MoA): 12-month extension to the SPC for MoA PIPs;
- Therefore, the maximum total extension available if two MoA PIPs were completed would be 24 months.

This improved reward would be fairer and more proportionate to the efforts required to deliver innovative treatments in compliance with developers' expanding obligations, and provide a better incentive to pursue paediatric R&D even beyond what is mandatory, in turn serving the interests of EU competitiveness in bringing new innovative treatments to this patient population.

IP Enforceability

Without the ability to readily and efficiently enforce granted IP rights, the value of the rights themselves is significantly undermined. Patent litigation between patent-holding innovator companies and generic/biosimilar companies wishing to commercialise generic/hybrid generic/biosimilar versions of innovator products in the current European system is inherently imbalanced, inconsistent, and unreliable in terms of preventing the infringement of granted patent rights. This situation has been recognised globally, and has been specifically addressed by numerous developed countries around the world, including the United States, Canada, South Korea, Japan, China, Taiwan, Mexico, Saudi Arabia, among others.
Europe too must strive for predictable, strong and enforceable IP. This is essential to underpin a globally competitive IP regime and life science ecosystem, enhancing health and access to innovative medicines.

As a key part of this, the EU urgently needs a system where granted IP is respected and generics/biosimilars are able to launch on "day one," which is the first day after all of the patents to the innovative product are expired, have been finally invalidated, or the inventions contained in those patents are not infringed or included by the generic/biosimilar product.

Therefore, a modernised market entry system should be established, providing governing principles for a concrete, procompetitive, ordered solution to the problem. This ensures maximum transparency and certainty to innovators, generics/biosimilars, payers and patients by setting a concrete "day one" for follow-on launch whilst taking account of the dynamics of the current generic market in terms of the so-called "first mover advantage."

Clear, Confirm & Control

EFPIA proposes a clearer, modernised, more enforcement-capable market entry system, which would comprise the following steps:

First, the innovator company will publicly list patents that cover the innovative product.

Next, the system sets out two alternative timelines for generic product launch, depending on the chosen patent litigation strategy of the generic/biosimilar company. The generic/biosimilar company will be required to take a position on those patents by no later than the point of application for a marketing authorisation (MA), i.e. once the regulatory data exclusivity period has expired.

In *path A*, the generic/biosimilar company intends to wait until the expiry date of the last to expire of the listed patents. The generic/biosimilar company will declare this in a binding way and the relevant MA will become operationally active only as of that expiry date.

In *path B*, the generic/biosimilar company chooses not to wait until the expiry date of the last to expire of the listed patents. The generic/biosimilar company will then initiate patent litigation proceedings to confirm whether the listed patents, or a selection thereof, are valid and infringed by the generic/biosimilar product at issue. This is an exercise in "Clear and Confirm." Only once the "Clear and Confirm" exercise is concluded through patent litigation would the generic/biosimilar company have an operationally active MA.

Further, within *Path B*, the first generic/biosimilar company to successfully challenge the relevant listed patents and/or establish that the inventions validly claimed therein are omitted from the generic/biosimilar product at issue, will benefit from a "head start" over other generic/biosimilar companies seeking to commercialise generic/biosimilar products based on the same innovator reference product. The MA for the generic/biosimilar that qualifies under the "head start" will become operationally active for 3 months before any other generic/biosimilar products with MAs for the same innovator reference product.

The concept of a marketing authorisation having to become operationally active before it can be used, is new. The intention is that this system does not delay the work of the regulatory agency assessing safety and efficacy of the generic/biosimilar product. Instead, it introduces a 'control step' that could be in the form of a conditionally granted generic or biosimilar MA, that only becomes valid, or operational, on completion of the steps set out in either Path A or Path B above. Only once this condition has been satisfied would the MA then be a valid basis for commercialisation. This is essential to providing security for the innovative medicine patent holder and for implementing the generic/biosimilar "head start". These changes would be incorporated into the EU legislative framework for authorising generic and biosimilar products.

Why Stronger Protection of CCI and IP in EU Databases Is Essential

Clinical Trial Transparency Requirements, Digitisation, and Data Sharing

Unlike other regulated industries, the pharmaceutical industry is subject to a uniquely high level of mandated transparency. Under the EU Clinical Trials Regulation (EU) No. 536/2014, the Clinical Trial (CT) Protocol and associated documents (such as the informed consent form) must be published in the EU Clinical Trials Register following the first decision by a Member State where the trial is intended to take place.[2] The published documents often contain information relating to potentially patentable innovations such as new therapeutic uses, dosing regimens, pharmaceutical formulations or biomarkers.

[2] *With some limited exceptions in Category 1 studies.*

The EU regulatory framework creates a tension for innovators in the pharmaceutical industry that must comply with transparency obligations while simultaneously striving to protect their investments and their intellectual property (IP). The tension is particularly acute when seeking patent protection for CT-related innovations, as premature disclosure of CT related documents may negatively influence their patentability. A patent application may be filed based on a CT protocol approved by national health authorities and ethics committees before posting the trial summary on a public registry. The registry posting is then not a prior art during the examination of the application. However, CT results are typically unavailable at this early stage, whereas these are often needed as support for the filing of the underlying patent application. In rare cases, preliminary results may be added during the priority year, but most trials do not yield data within the first 12 months. Thus, while this early filing may avoid prior art issues, the absence of clinical data may considerably limit patent grant prospects in Europe when compared to peer jurisdictions.

Alternatively, patent application filing after the trial concludes or at predefined milestones (e.g. interim analysis) ensures inclusion of clinical data. However, this strategy means filing occurs after the mandatory public registry posting which becomes prior art for the examination of the application. While this alternative strengthens patentability through clinical results evidence, it introduces significant risks due to public registry posting.

This tension further undermines the EU's potential to play a role as a global leader in pharmaceutical research and only risks accelerating EU's clinical trials decline. Indeed, despite a 38% increase in global clinical trials over the past decade, Europe's share has dropped from 22% in 2013 to 12% in 2023: that's 60,000 fewer clinical trial places for European patients.

Yet, on the other hand, Europe's digital, data and life sciences strategies rightly aim to position the EU as a global leader in innovation, with initiatives like the Data Act and EHDS promoting data sharing and re-use for public benefit. However, these efforts expose a critical weakness: the EU's current intellectual property (IP) framework does not adequately protect the commercially confidential information (CCI) and proprietary datasets that underpin innovation, particularly in high-value sectors such as life sciences, AI, and digital health. The lack of robust safeguards for these assets creates legal uncertainty, discourages investment, and risks undermining the competitiveness of European innovators.

To succeed, the EU must modernize its IP framework, strengthening protections for CCI and proprietary data in order to ensure legal certainty, incentivize innovation, and secure Europe's leadership in the global digital economy.

The sui generis database right, created in 1996, is outdated. It fails to protect modern datasets that are high-value but not "original" in the copyright sense, such as cleaned, annotated, or machine-generated data. Copyright law doesn't cover raw data, and relying on contracts alone creates legal uncertainty. As a result, this outdated framework means innovators face unclear rights, fragmented enforcement, and diminished incentives to invest in Europe's data economy.

The situation is even more concerning for trade secrets. The Data Act and EHDS introduce mandatory data sharing obligations without robust safeguards for sensitive business information. There is no formal recognition that trade secret protection is often used to preserve competitive advantages provided by high-value proprietary datasets and annotations. Enforcement mechanisms are unclear, and transparency requirements under AI legislation further risk exposing proprietary algorithms and logic.

Enhanced Protection of Confidential Data

Our ask to improve data ecosystem in the EU is simple but urgent: modernize EU IP law to reflect today's data realities. EFPIA proposes the following actions to that purpose:

- Expanding database rights to cover structured, high-effort datasets
- Recognizing proprietary datasets and annotations as trade secrets
- Modernizing copyright laws to incentivize the digital and data economy
- Embedding clear opt-outs and safeguards in laws like the Clinical Trial Regulation, the EHDS and Data Act to preserve and strengthen the protection of trade secrets, CCI and intellectual property rights
- Supporting progress on substantive patent law harmonization efforts and the introduction of a grace period or non-prejudicial disclosure provision type in the European patent system to mitigate the concerns with mandated transparency

These reforms will restore legal certainty, protect innovation, and ensure Europe remains competitive in the global digital economy and allow for the balancing of regulatory requirements with the critical need for IP protection to incentivize research and development of solutions for patients. Without them, we risk undermining the very investment and ingenuity the EU seeks to promote and EU's ambition to become a global leader in pharmaceutical research.

EFPIA urges the EU to take this valuable opportunity towards global competitiveness, and secure investment into EU-based companies and future medicines.

2. Key recommendations from the comparative analysis of biopharmaceutical strategies in 10 countries

Increased global competition for biopharmaceutical leadership is coming at a challenging period for Europe in today's context of threats to Europe's security and geoeconomic and macroeconomic challenges.

Over the past two decades, Europe's share of global pharmaceutical R&D investment has decreased from 37% in 2001 to 31% in 2020, with projections indicating a further decline to 21% by 2040, if no countermeasures are taken. EU's member states have under-estimated the combined impact that different drivers of investment in other third countries is having on Europe's own attractiveness as a region (for example, the lack of sense of urgency in improving Europe's clinical trials ecosystem considering the rapid rise of China in this field over the past few years), combined with an under-investment in the structural issues that are eroding its biopharma ecosystem and at risk of eroding it further.

The Commission's Biotech and Biomanufacturing Communication of 2024 acknowledges these gaps: regulatory hurdles, technology transfer bottlenecks, financing gaps. The impact of incomplete and under resourced implementation of EU legislation is well documented (such as the Clinical Trials Regulation), Mario Draghi's report on the future of European competitiveness highlighted the impact of Europe's fragmented pricing and reimbursement ecosystem.

Recent global developments, notably the potential of a 15% U.S. tariff on pharmaceuticals, and the possible introduction of Most-Favored Nation pricing in the US, will impact the dynamics of accelerating

global competition, investment flows and global launch incentives. These developments further reinforce the need for building Europe's geopolitical resilience in biopharmaceuticals, de-risking of supply chains, safeguarding incentives and reinforcing Europe's ability to leverage its strengths in the biopharmaceutical value chain.

Competition from China and its growing strengths in several key segments of the biopharma value chain is a reflection of today's importance of speed and cost effectiveness to address R&D productivity. The Draghi report's assessment that innovation and R&D are non-negotiables for global tech leadership, will require that all member states pull in the same direction with a much greater sense of urgency (keeping in mind that a recently launched report highlights a lack of action on Draghi's recommendations). Europe should focus on what it already does well and scale its own unique model through joined up efforts by its member states, many of which run their own national biopharmaceutical policies. It should also agree on key objectives and core metrics for performance, just like many national strategies do. This also requires a much better understanding of the link between the value added drawn from more biopharma activity and supply-side measures (R&D, skills, biomanufacturing) in Europe and demand-side incentives (faster, more predictable market access) to further advance investment.

Treat single market completion as a top priority

Enrico Letta's report "Much more than a Market" (April 2024) was very clear on the imperative of completing the single market. Other countries analysed here play to their strengths. Europe's strength is the single market. Europe's lower competitiveness vs the US and increasingly China is partly due to the lack of all pulling in the same direction with a strong political will to invest the required resources. As in other sectors, there are still many areas of underperformance in biopharmaceutical competitiveness that are caused by an insufficient level of implementation of existing EU legislation.

Investors are very clear on this: faster, predictable routes of access of innovation to patients in Europe will pull in investment and impact location decisions for various stages in the biopharma value chain (for example, location of clinical research). This isn't only a question of how much financing is available. Speed to patients should be a measurable investment incentive (with measurements along clinical trials, regulatory processes, HTA processes etc). The EU and member states should double down on efforts to address those areas where a strong foundation had already been set through agreed legislation or EU policies:

- Uniform implementation of the Clinical Trials Regulation, with necessary targeted adjustments: remove bottlenecks and reliance on workarounds, ensuring faster and more predictable reviews through harmonised timelines. Optimise the Clinical Trials Information System (CTIS), strengthen infrastructure for multi-country trials, and establish coordinated ethics reviews and cross-border patient participation to reduce delays and enable more rapid access to innovation.
- Accelerate regulatory processes (to address the gap with other regulatory agencies)
- Move towards a more coherent European cutting-edge innovation access pathway: make the adoption of emerging technologies more efficient (e.g., gene therapies, mRNA platforms), as this requires more flexible and adaptive regulatory responses, with the EU HTA Regulation now applying, focus on delivering EU-added value outputs which policy coherence and encourage uptake of cutting-edge science through managed entry agreements and outcomes-based contracts to cut access delays.
- Invest in health data and AI: Operationalise the European Health Data Space with data access for trials and RWE under strong governance. Create EMA/HTA sandboxes for AI/ML in discovery, trial design, and labelling, connect Biotech/biopharma with the European Commission's AI Factories roll-out (for example, consider biopharma use cases), create health-specific infrastructure in AI capacity to succeed in the Biotech-AI nexus, leveraging existing national strengths, such as Denmark's Gefion supercomputer.

Facilitate the administrative, compliance processes, financing and scaling up processes for smaller biotechs, for example through the proposed 28th regime and the Start-up and Scale-up Strategy.

Excel at cutting-edge science

Leverage cross-border scale building on existing expertise: This should draw on practices and learnings from public-private consortia that operate within the EU and on the learnings from non-EU countries. The EU's biotech/biomanufacturing strategy rightly highlights the need for investing into regulatory sandboxes, standards, skills, and test-bed access, i.e., the translational infrastructure. Efforts should leverage what is already happening in countries, fix cross-border frictions, and couple finance with translational infrastructure (such as GMP testbeds/pilot facilities as exist in the UK with the Cell&Gene Therapy's Stevenage Manufacturing Innovation Centre, or in Singapore, South Korea and Denmark, for example).

It is encouraging that the EU's Life Sciences Strategy supports cross-border cluster collaboration and the scaling from national towards pan-European bioclusters. This analysis has shown the significant role played by clusters in every country analysed, and the existence of a comprehensive and complementary ecosystem composed of bigger pharma companies, biotech startups and a rich network of research centres for attracting talent and creating a positive financing dynamic (which will require public funding support). Therefore, scale what works, fund multi-country bioclusters with shared platforms (eg imaging, biobanks) and ensure efficient governance. Tie EU funding to industry utilisation and private co-investment, as well as to supporting the drivers on key shared objectives, such as for example clinical trials timelines. This action should also have an international component, through the EU's international partnership strategy to connect the EU's leading hubs with hubs in partner countries.

Inspired from South Korea, leverage Europe's Biomanufacturing strength, including in vaccines, fast-track variations for process intensification and greener bioprocessing. Make "Clean Biomanufacturing" a European brand (energy-efficient plants, circular single-use, greener solvents), where countries such as France and Denmark can take a leadership, and develop appropriate incentives, while ensuring that the regulatory framework remains fit for purpose.

Integrate the international dimension in Europe's biotech strategy. The challenges for the biopharmaceutical sector and risks to a country's health security extend beyond a narrow supply chain and manufacturing scope. Countries analysed show an increasing awareness of the geopolitical risks and the need to adapt to these through more investment into various parts of the biopharma value chain with tariffs and non-tariff barriers, data localisation obligations, investment screening regimes are becoming a more frequent feature across geographies. A European international partnership strategy should include considerations such as health data flows, AIxBiotech standards, securing supply chains for biomanufacturing etc.

Financing at scale – unlock late-stage capital and anchor it in Europe

Move quickly on enabling start-up and scale up finance: It is encouraging to see that the EU now explicitly acknowledges the startup-to-scale-up financing gap and is seeking to act on several levers: the role played by public catalytic finance through the instruments of an expanded InvestEU and strong EIB role in lifesciences, proposals to free insurer/pension capital , advance on a potential evolution in the role for the European Innovation Council towards a European DARPA-type agency. A clear overall financial architecture for the lifescience sector will be key.

Europe needs to offer an attractive early-stage environment for venture capital funds. The EU's Biotech communication of 2024 ties finance to scale-up infrastructure (such as biomanufacturing, standards, skills), mirroring what successful hubs (eg US, Denmark, Singapore) already do. The proposals for the next

MFF include ambitious objectives for R&I. The EU should use InvestEU, the Competitiveness Fund, EIB, EIF to crowd in private capital for scale-ups and biomanufacturing testbeds. Public co-investment can bridge Europe's weaker late-stage VC pool. Even small increases in VC allocations (via dedicated vehicles) could materially change late-stage availability and reduce leakage. IMF/EIB analyses highlight this lever.
EU policymakers need to continue pushing for pan-EU scale mechanisms so as to create new levers that Europe can use to compete on a global scale: co-investment, InvestEU-style funds, pension reforms, establish guarantee fund for biopharma SMEs to minimise their losses through lower hurdle rates and risk sharing harmonised incentives, and the structural reforms needed to mobilise long-term capital. Together, these measures target the same bottlenecks that push EU founders abroad and pair risk capital with scale-up infrastructure. Politically, the climate also calls for industry to need to reflect on its acceptance of approaches that tie these measures to manufacturing and/or trial commitments in the EU. Europe needs to scale its own model: world-class science and talent, cross-border clusters, industrial-grade translation, and credible, faster routes to market, backed by serious capital and geopolitical realism.

3. EFPIA Paper on manufacturing

Executive summary

Decisions on the location of pharmaceutical manufacturing for innovator companies is driven by a number of factors. These include access to global supply chains (including for raw materials and exports) and a stable and harmonised regulatory landscape. Equally important is the ready availability of global talent and the focus on innovation of the relevant national regulator. Incentives[1] such as low tax rates can also play an important role in investment decisions.
A more detailed analysis of the drivers for location of manufacturing investments can be found in the October 2022 CRA report on 'Factors affecting the location of biopharmaceutical investments and implications for European policy priorities' (see section 3.3, pages 38-49, available here).
In order to localise manufacturing, Governments must ensure there are limited barriers to talent and trade, whilst balancing incentives for manufacturing with other fiscal priorities. Regions such as Europe need to recognise that pressures on pricing and ongoing erosion of a country's global market share can also influence investment decisions for innovator companies. Regulators also have a clear role to play in simplifying regulatory pathways for change and new technologies to enable new manufacturing sites and processes.
EFPIA companies welcome effective incentive policies to support manufacturing, provided they are compliant with WTO rules, which are especially important for globally operating supply chains for innovative medicines.

Localisation of Manufacturing for Innovative Medicines

There has long been global competition for the location of pharmaceutical manufacturing. In recent years this has become more visible in public legislation, with major initiatives such as the EU Critical Medicines[2] and Biotech[3] Acts and the 2025 US Executive order[4] on critical medicines' production all focusing on promoting domestic manufacturing.
Incentives and disincentives for the location of manufacturing innovative patented medicines (the focus of EFPIA companies) differ in part from generic medicines, where cost drivers are more critical. For innovative medicines, the impact of incentives and enablers can be clearly seen in the success of manufacturing hubs such as Ireland and Singapore.
EFPIA companies operate global supply chains. Moving to a regional or national models will decrease the robustness of supply and could increase cost for patients and payers and significantly increase the risk of supply disruption.
EFPIAs's position regarding location of manufacturing can be summarised as follows:

Drivers for Manufacturing Investment

- National Tax Policies and Investment Incentives
- Access to global supply chains, with minimised costs and regulatory barriers to imports and exports of raw materials, chemicals intermediates, APIs and finished products.
- Government and regulatory and commercial environment focus on enabling innovation
- A healthy, established ecosystem for pharmaceutical manufacturing and research, with integrated access to technologies, industry, and research.
- Access to a diverse, highly skilled talent pool, locally and internationally.
- Integration into stable and harmonised global regulatory frameworks, embedded in ICH, and PIC/S principles
- Accessible and innovation focused National Competent Authorities, with focused on international harmonisation reliance, recognition and collaboration.

Headwinds, Impacting Manufacturing Investment

- Tax and trade policies can impact costs and export to global markets
- Restriction on supply chains for raw materials, chemicals and APIs, including tariffs & import testing.
- Lack of access to global talent and expertise
- Complex chemical and environmental legislation, disproportionately targeting pharmaceutical manufacturing
- Threats and restrictions on IP and exclusivity
- A commercial environment that does not reflect value of innovative medicines
- Lack of integration into globally harmonised regulatory and inspectional frameworks.

Summary: Enabling investment by innovative pharmaceutical companies

The global nature of pharmaceutical supply chains is essential to deliver access of new medicines to patients worldwide and enable the investment in manufacturing. Linked to this, access to global talent, free flow of materials and a stable and globally harmonised regulatory landscape are all underscore the reasons for localisation of manufacturing.

Fundamentally, governments can deliver a supportive environment for innovative manufacturing which will attract industry investment. Equally, governments must be mindful of geopolitical pressures impacting investment and consider the implications of long trends in global market share. Finally, regulators have a clear role to play in simplifying regulatory pathways for change and new technologies to facilitate the establishment of new manufacturing sites and processes.

4. Paediatric Regulatory Simplification Proposals

Labelling exemption for small population products (paediatrics and OMPs)

Current situation: There is a legal obligation for the labelling and the package leaflet to be in the official language(s) of the Member State where the medicinal product is placed on the market. Competent authorities can allow exemption to this obligation (labelling exemption) for medicines that are not delivered directly to patients or where there are availability issues. Similarly, certain orphan medicinal products may also benefit from labelling exemption.

Paediatric products, like orphan medicinal products, often target a very small patient population for which consequently there is very limited demand. Small patient populations constrain economies of scale, making production and distribution economically challenging.

Country-specific packaging places heavy administrative, logistical, and regulatory burdens on biotech companies, particularly but not only in small markets, constraining innovation and access to medicines.

Proposal: An automatic labelling exemption for paediatric products (and other products for small populations and/or subject to particular availability challenges) would allow the use of the English language pack and leaflet while the ePI code would allow patients and Health Care Provider to have access to the digital leaflet in their national language electronically.

Why does it matter: This is a simplification initiative. The number of patients diagnosed with pediatric diseases is often small – in some countries there may be no demand at all for certain products – leading to low usage and potentially many write offs. Introducing regulatory flexibilities would decrease the administrative and logistical burdens and barriers to making paediatric products available to the patients and would also enable more agile supply chains.

Align CSR submission timelines

Current situation: In EU there are two distinct timelines for submission of clinical study reports to the competent authorities depending on the population of the study

- Under the Clinical Trials Regulation, for studies completed in adults, the sponsor must submit the summary of study results in the EU Clinical Trials Register within 12 months after the end of the study. This is broadly aligned with global requirements.
- For studies completed in children, as per the current paediatric regulation, the sponsor must submit the study results to EMA & national authorities as applicable, within 6 months of study completion. This includes the full clinical study report (CSR) and the summary.
- This EU-specific requirement for earlier submission of paediatric study results is particularly burdensome and leads to complexity in planning the varying formats and timelines to meet the varying requirements globally. Moreover, paediatric studies are often complex and challenging, especially for vaccine products, necessitating a request for additional communications with EU authorities in relation to the submission of the CSR, creating unnecessary, additional, administrative steps and challenges.
- Since the product information can only be updated with the PIP results once the full regulatory submission (usually, a variation) has been made and assessed by the EMA/competent authorities, this interim step with reduced timelines for submission of paediatric data serves no practical purpose and does not provide more efficient or swifter access to treatment for paediatric patients.

Proposal: Align requirements of CSR submission of paediatric studies with that of adult studies (i.e., summary submission within 12 months of study completion).

Why does it matter: it will lead to streamlining and simplification in terms of planning and execution for both the industry and regulators when the summaries become available at the same time irrespective of the study population. Leads to coherence within the implementation of different regulations that today govern studies in adults and children differently, and better global alignment and efficiency.

5. List of Abbreviations

AI – Artificial Intelligence
ATMP – Advanced Therapy Medicinal Product
CCI – Confidential Commercial Information

CMC – Chemistry, Manufacturing and Controls
CTR – Clinical Trials Regulation
CTA – Clinical Trial Application
DG RTD – Directorate-General for Research and Innovation (European Commission)
E2E – End-to-End
ECHR – European Charter of Human Rights
EHDS – European Health Data Space
EMA – European Medicines Agency
EMRN – European Medicines Regulatory Network
EIB – European Investment Bank
EFPIA – European Federation of Pharmaceutical Industries and Associations
EHR – Electronic Health Record
EIT – European Institute of Innovation and Technology
EU – European Union
EU HTA – European Union Health Technology Assessment
GDPR – General Data Protection Regulation
GMO – Genetically Modified Organism
GMP – Good Manufacturing Practice
HPC – High-Performance Computing
HTA – Health Technology Assessment
ILAP – Innovative Licensing and Access Pathway (UK)
IPO – Initial Public Offering
IP – Intellectual Property
IVDR – In Vitro Diagnostic Regulation
MDR – Medical Device Regulation
MS – Member States
MSCA – Marie Skłodowska-Curie Actions
ODF – Online Discussion Forum
PML – Product Master List
R&D – Research and Development
RWD – Real-World Data
RWE – Real-World Evidence
SME – Small and Medium-sized Enterprise
SoHO – Substances of Human Origin
SPC – Supplementary Protection Certificate
STEM – Science, Technology, Engineering and Mathematics
UK – United Kingdom
US – United States
VC – Venture Capital
WTO – World Trade Organization
xEVMPD – Extended EudraVigilance Medicinal Product Dictionary