The Future of Health is Biotech: EuropaBio position on the revision of the EU Pharmaceutical Legislation

In the last 20 years, biotech transformed the pharmaceutical industry and enabled the development of breakthrough therapies that are live-saving or significantly improving quality of life of patients and their families. The development of biotech in Europe also continues to positively contribute to the region's economic well-being. In 2018, healthcare biotech's total GDP contribution was €63.3 billion, and the sector contributed to over 700,000 jobs in the EU[1].

A supportive and future-proof EU General Pharmaceutical Legislation (GPL) is critical to ensure Europe can fully benefit from healthcare biotech innovations for patients, society, and economic development. European patients need legislation that can harness our scientific knowledge to develop breakthrough therapies. It should be designed to address health challenges, unmet medical needs, and allow the EU to regain its global leadership in R&D and cutting-edge biotechnology.

The proposed revision of the GPL will significantly impact the environment in which the biotech industry operates and the predictability and stability of Europe's incentives regime that has successfully supported transformative innovation from our industry for decades. EuropaBio shares the European Commission's objectives for the revision but believes it leverages the wrong tools to achieve them. A blunt approach to achieving policy aims may deliver the exact opposite, by degrading Europe's ability to identify and develop therapies itself and becoming a tertiary market for therapies increasingly developed outside Europe.

EuropaBio is committed to work with policymakers and stakeholders to ensure the revised Pharmaceutical Legislation can support the biotech industry to deliver life-changing therapies to patients across Europe by:

- Regaining the lead in biotech innovation by creating a positive environment for investments, competitiveness to ensure patients can rapidly benefit from novel therapies
- Fostering a patient-centric approach to unmet medical needs that can deliver innovation to all patients
- Supporting continued innovation for rare disease patients building on the existing framework
- Making the regulatory framework faster and more flexible to accommodate future innovation.

[1] WiFOR Institute (2020), Measuring the Economic Footprint of Biotechnology in Europe, pp. 18 and 22. Available here.

Improving Access to Medicines Requires a Competitive Industry in Europe

Improving patient access to innovative medicines is an objective shared by our industry. However, the Commission's proposal to condition essential incentives on launching a medicine across the EU within 2 or 3 years runs the risk of weakening competition, increasing market access barriers, and decreasing the attractiveness of the region for early launches and investment. EuropaBio believes the proposal would only provide limited improvement to access at the cost of significant harm to the European innovation ecosystem with negative consequences for patients.

Access to medicines is determined by a series of complex factors at the Member State level that are often beyond the control of marketing authorisation holders (MAHs). The system proposed by the

Commission for all medicinal products fails to consider the following key factors that will impact the success of the revision:

- The inability to widely supply certain biological medicines due to product characteristics and the specific needs for orphan medicines and advanced therapies (ATMPs) linked to lack of patient population, adequate infrastructures, health systems readiness, and healthcare professionals across the EU.[2]
- The inability of small and mid-size companies to launch in all EU markets regardless of the time granted.
- The significant difficulties for MAHs to comply with the release and continuously supply criteria within 2 or 3 years and the lack of recognition of the role of other key stakeholders when it comes to access to medicines.
- The complexity of market access in Europe, including different requirements and needs as well as the limited capacity or willingness of national systems to process applications within 2 or 3 years.

Regulatory data protection (RDP) and orphan market exclusivity (OME) are intended to de-risk investments and prevent competitors from leveraging data shared with regulators to obtain a marketing authorisation. They are not designed as levers to improve access. Even as Europe struggles to keep pace with other regions, EuropaBio believes that reducing and conditioning incentives to access conditions will not achieve the intended objectives of the revision.[3] The EU should use the right tools within its competences, such as accelerating the centralised authorisation procedure and improving the cross-border healthcare framework while

[2] Most biological medicines have to be administered through injection or intravenous infusion to retain their quality, safety, and efficacy as oral administration would degrade the medicine before absorption by the system.

[3] IQVIA (2023), Global Trends in R&D 2023, pp. 6, 8, and 20 Available here.

Member States should work with MAHs today to address the significant barriers which delay access to medicines.

Supporting Europe's Biotech Ecosystem from Emerging to Established Companies

As biological medicines continue to represent an ever-growing share of the global R&D pipeline and medicines approved, the EU must ensure it remains a nurturing environment for all biotech companies. Stable and predictable regulatory incentives and intellectual property rights are critical to de-risk therapeutic development. Where less than 1 out of 10 candidate therapeutics at the human testing phase reach marketing authorisation, incentives are needed to ensure the pipeline of innovative medicines keeps flowing to Europe's patients.[4]

The Commission's proposals on RDP will be particularly harmful to developers of biologics for whom it is the most predictable protection as patent protection is often narrower and easier to circumvent than for non-biologics. For biologics, composed of large molecules produced by living cells and organisms, research and development is a high-risk endeavour, with higher capital costs, higher material costs, greater manufacturing costs and uncertainties, longer development times, and lower late-stage success rates compared to small molecule products. Failure to maintain adequate RDP for biologics will significantly undermine the ability of biotech companies to innovate from Europe, even as other regions continue to grow their biologics pipelines.[5]

Today, emerging and smaller companies are responsible for over two-thirds of the R&D pipeline that will become the medicines of tomorrow.[6] As Europe struggles to retain its emerging biotech companies and lags behind on key metrics linked to investment flows, company creations, and deals, strong incentives are critical to those companies as they leverage them to raise capital and find partners to bring innovation from bench to European patients.[7] The lack of a proper SME stress test accompanying the proposals means the co-legislators will be unable to make informed decisions to support Europe's small and mid-size companies, potentially denying patients from timely access to future therapies. Ultimately, streamlined regulatory approval or enhanced regulatory support will prove ineffective if the reduction in incentives means smaller companies are unable to conduct innovative research within the EU.

Europe cannot rely on other regions to address its own health needs as it will delay patient access to innovative medicines as developers focus on other countries' needs first. Stronger incentives are necessary to make up for Europe's fragmented market that leads to duplication

[4] Biotechnology Innovation Organisation (2021), Clinical Development Success Rates and Contributing Factors 2011-2020, p. 3. Available here.

[5] IQIVIA (2023), Global Trends in R&D 2023, p. 38. Available here.

[6] IQIVIA (2023), Global Trends in R&D 2023, p. 21. Available here.

[7] Ibid., p. 12.

of work and increased entry and administrative costs while reducing the effective protection periods. As stated in a 2018 study for the Commission, a decrease in the effective protection period will impact spending on R&D, increasing development time, delaying the time to market as fewer products reaching the market. The decrease would also have a knock-on effect on access to generics and biosimilars as they rely on originators to be developed.[8]

EuropaBio acknowledges the policy objectives of the Commission's proposals but is concerned that the overall reduction together with the conditionality of RDP and OME will undermine the stability and predictability of Europe's incentives based on which investment decisions are made, impacting actors across the innovation ecosystem and ultimately European patients.

Making Europe Patient-Centric and Fit to Tackle All Unmet Medical Needs

For decades, the healthcare biotech industry has pushed the boundaries of innovation by developing medicines that improve patients' quality of life and address unmet medical needs (UMN) independent of any conceptualisation or definitions of UMN. Addressing remaining and future UMN at societal and patient levels cannot be achieved through legislative means and will require significant investments in health ecosystems supported by the right policies and incentives.

EuropaBio is concerned that the legislative approach to UMN and high UMN proposed by the Commission is at odds with the pace of scientific progress, medicine development, and patient needs. The proposed definitions will stifle innovation by undermining the incremental nature of scientific progress and exclude certain patient populations from future therapeutic innovations. It would rather serve as a signal to innovative companies that the medical needs of certain patient populations are less important than others. Patient access to transformative therapies should be driven by scientific and technological progress rather than the EU's policy cycle.

EuropaBio considers that any conceptualisation of UMN should be agreed upon in a multi-stakeholder approach with patient voices duly represented. The EU approach should be scientifically sound, flexible, and future-proof to accommodate the rapid evolution of our scientific knowledge and understanding of disease.

Building on this reflection and the expertise of its members, EuropaBio recommends a non-legislative approach for UMN based on the following elements: disease severity; absence of satisfactory treatments; quality of life; burden of disease; and burden of available treatment.

Continued EU Innovation for Rare Disease Patients

For over two decades, the Orphan Medicinal Products (OMP) Regulation has been instrumental in supporting successful R&D for rare diseases with over 231 orphan medicines

[8] European Commission (2018), Study on the economic impact of supplementary protection certificates, pharmaceutical incentives and rewards in Europe, pp. 159-160. Available here.

now approved. EuropaBio shares the Commission's objective to find therapeutic solutions for the 95% of rare diseases currently without treatment. However, we believe that this should not be achieved at the expense of the 80% of rare disease patients affected by the most prevalent rare diseases. The revision of this important legislation is an opportunity for Europe to do more for rare disease patients and reflect on some of the significant advancement in the treatments of orphan diseases in Europe that has resulted from the current regulatory framework.

The revision is an opportunity to drive further research into developing innovative medicines for people living with rare diseases and attract further life science investments in Europe. Existing IPR and incentives have proven successful and remain essential to foster R&D in all rare diseases.[9] Reducing or narrowing them would significantly increase the challenges for the development of innovative treatments and would have a negative impact on the environment in which the biotech industry operates.

The EU legislation should continue to guarantee the baseline OME period that has been instrumental to the success of the current legislation and reflects the therapeutic value and required investments of OMPs. To support companies in their mission to unlock science for underserved and high-risk areas, an additional period of OME should be introduced to complement the baseline period. To encourage further clinical development of existing compounds, these should benefit from the OMP framework with a specific OME period. This approach would enable continued development for all rare diseases and offer additional incentives for those rare diseases where no treatment currently exists, achieving the revision's objectives.

The Orphan Designation (OD) is an important step in the development of OMPs as it provides confidence that a medicine will benefit from the EU's orphan regime and the revision should preserve its simplicity and predictability. It is therefore essential that the revision maintains the predictability of the designation. For small to mid-sized companies, the granting of an OD is often critical to their continued development as it enables them to attract investments and they often file for OD at an early stage in the development process. The Commission proposals to limit the validity of the OD at 7 years and to use secondary legislation to establish different criteria for the OD will severely limit the ability of MAHs to successfully complete development.

Making Europe's Regulatory Framework Fit for the Next Decades

The proposed revision brings several positive changes which should support the development of Europe's regulatory framework to be more agile, flexible, and adapted to novel therapies. The ambitious streamlining of the European Medicines Agency's constitutes a significant step towards improved quality, safety, and efficacy of medicinal products. The proposed faster

[9] Technopolis/Ecorys (2019). Study to support the evaluation of the EU Orphan Regulation, pp. 206 and 301.

assessment timelines will contribute to accelerated regulatory approval and thus patient access to medicinal products.

Although the proposals foresee a greater use of electronic product information, a more ambitious transition for the adoption of the digital leaflet can be a simple tool that can hugely improve supply chain agility and tackle the shortages issue.

The creation of regulatory sandboxes is an important element of the proposals to make Europe's regulatory framework fit for the 21st Century, but it is important to ensure their scope are not too narrow and avoid becoming an unused tool in Europe's regulatory toolbox. The possibility to establish adapted frameworks for certain categories of products is also a positive development in so far as their application are limited to sandbox products. However, in both cases, it is important to ensure that the EU's regulatory standards remain homogenous and avoid a system where different safety, efficacy, and quality standards are established for different medicines.

The proposed changes to the hospital exemption (HE) system are welcomed to increase transparency, data collection, and protect patient health. The misuse of the exemption has led to unintended consequences due to its fragmented implementation across the Member States and used, in some cases, in deviation from its original purpose to circumvent seeking a marketing authorisation. HE has a legitimate role to play in meeting unmet patient needs but it is essential that its use remains exceptional, where no authorised or investigational products are available. To preserve a single regulatory pathway for ATMPs and Europe's high standards, it is necessary to ensure the revision guarantees the harmonisation and transparency of the use of HE across the Member States.

About EuropaBio

EuropaBio, the European Association for Bioindustries, promotes an innovative and dynamic European biotechnology industry. EuropaBio and its members are committed to the socially responsible use of biotechnology to improve quality of life, to prevent, diagnose, treat and cure diseases, to improve the quality and quantity of food and feedstuffs and to move towards a biobased and zero-waste economy.