ECL and Anticancer Fund recommendations for the revision of the EU general pharmaceutical legislation

The Association of the European Cancer Leagues (ECL) and the Anticancer Fund welcome the revision of the general pharmaceutical legislation, namely Directive 2001/83/EC and Regulation (EC) No 726/2004, to align it with the new Pharmaceutical Strategy for Europe. It is an opportunity to make the pharmaceutical system patient-centred and fit for purpose. Therefore, the revision should prioritise:

- addressing unmet medical needs - diseases for which there is no treatment or only suboptimal treatments
- future proofing legislation for innovative treatments that bring proven added value at fair prices
- ensuring timely access to safe, effective, and affordable medicines for all patients in need
- enhancing the security of supply and addressing medicine shortages

To that end, we propose several policy recommendations.

- **Define unmet medical needs**

There is a need for a clear and transparent definition of unmet medical needs to ensure consistency across different regulatory frameworks and along the medicine life cycle. It should be established in the EU legislation and detailed in scientific guidelines, which could be updated regularly.

The following criteria are key for defining unmet medical needs: absence of satisfactory treatment authorised in the EU, seriousness of a disease, and lack of access to an authorised treatment for patients across the EU.

- **Redesign system of incentives**

The current system of incentives should be revised. To allow earlier market access for generic and biosimilar medicines, current data (8 years) and market (2 years) protection periods should be reduced by at least 2 years. This will stimulate affordability of medicines across the EU. However, an additional period of regulatory protection, while never exceeding the current one, should be granted for a combination of:

- medicinal products that address an unmet medical need
- placing products on the market in all Member States within 2 years of market authorisation
- disclosing any relevant public funding received for research and development

We do not support the introduction of a transferable exclusivity voucher for new antimicrobials, as it could be extended to rare diseases or oncological therapeutics and therefore further worsen access to these medicines.

To further incentivise innovation around unmet medical needs, opportunities for not-for-profit entities to develop medicinal products should be created. Moreover, the PRIME scheme should be codified with the legislation, ensuring that the European Medicines Agency (EMA) would continue to provide sustainable enhanced advice and early dialogue with the developers of medicines. Also, the scheme's scope should be expanded to repurposed medicines.

- **Create a future-proof regulatory framework for new treatments**

Certain definitions, such as real-world data and real-world evidence, should be established. While these two elements can add information, they should not be considered as a new way to assess

medicines – clinical trials should remain the gold standard in assessing the safety, efficacy, and value of new medicines.

In addition, medicine developers should take a quality by design approach when conducting clinical trials and include endpoints such as overall survival and quality of life to provide a more robust evidence base to assess the effectiveness of new treatments and help decision makers along the value chain. Considering this, scientific advice by the European Medicines Agency (EMA) should become mandatory for medicine developers. Moreover, there should be an EU-wide centrally coordinated process that promotes early dialogue and more coordination among different actors involved in clinical trials, marketing authorisation, health technology assessment and pricing and reimbursement for integrated medicines development and post-authorisation monitoring, pricing, and reimbursement. Just like the U.S. Food and Drug Administration (FDA), EMA should be empowered to approve the registration of trials for medicines under the centralised procedure to verify that the trials which will be executed conform to the guidance provided by EMA as much as possible. By assuring the maximal output of the registration trials, EMA will demonstrate its respect for patients participating in these trials and will protect them against futile trials.

Finally, we support broader use of regulatory sandboxes, in the context of the approval and overview of innovative medicinal products developed for non-commercial purposes.

- **Improve patient access to medicines across the EU**

Companies should be required to notify regulators of their intentions to launch their medicines on the market at the same time of they seek medicine authorisation. In addition, companies should be required to place a centrally authorised medicine on the market in most Member States, including the small markets, within a certain period after authorisation. If the market launch of medicines across the EU is delayed, an early introduction of generics should be allowed. Finally, companies that withdraw a medicine from the market should offer another company the possibility of taking over the medicine.

To reinforce competition, joint procurement of medicines at the regional or the EU level should be introduced.

- **Prevent shortages**

To ensure security of supply of medicines in the EU, earlier reporting of shortages (at least 6 months in advance) and market withdrawals (at least 12 months in advance) to national authorities using a common template is necessary. Also, companies should provide more information to regulators on their supply chain, including of active supply sites and volume supplied. Moreover, they should have shortage prevention plans and safety stocks. Penalties for non-compliance with proposed new obligations should be introduced.

We support an introduction of the EU definition of a shortage and a shortage monitoring system for all medicines at the EU level.

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About the Association of European Cancer Leagues (ECL)

ECL is a non-profit, pan-European umbrella organisation connecting 24 national and regional cancer societies in 22 European countries. Its Access to Medicines Task Force (A2M TF) aims to make safe and effective medicines available to all cancer patients in Europe by insisting on accessibility, availability, affordability, and increased transparency related to medicine prices, which will make healthcare systems more sustainable. Contact ECL:

- Toma Mikalauskaitė, Senior Policy Officer
- Ward Rommel, Chair of Access to Medicines Task Force

About the Anticancer Fund

The Anticancer Fund is a Belgian non-profit organisation with an international scope. In 2018, the Anticancer Fund became a Foundation of Public Utility under Belgian law, an official recognition that the fund's effort is used to achieve a well-defined altruistic goal: investing in promising cancer treatments, putting patients' needs first. Contact the Anticancer Fund:

- Lydie Meheus, Managing Director
- Patricia Vandamme, Policy Officer