# CALL TO ACTION TO IMPROVE CANCER PATIENTS' ACCESS TO THE BEST TREATMENTS AVAILABLE ACROSS THE EUROPEAN UNION (EU)

## ECL position on the proposal for a new Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency

The Association of European Cancer Leagues (ECL), representing national and regional cancer societies across Europe, welcomes a new proposal for the Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency (EMA) (COM (2023) 193) (henceforth Regulation), published by the European Commission on 26 April 2023. This legislative proposal represents a once-in-generation window of opportunity to make the pharmaceutical system more patient-centred and fit for purpose by addressing unmet medical needs and ensuring availability and timely access to safe, effective, and affordable medicines for all patients in need. We must make the most of this opportunity, and to that end, we are sharing here our views on key elements that we support and those that we are concerned about and suggest amending.

### 1. Development of medicines and new uses for existing medicines

In Europe, around 2.7 million people are diagnosed with cancer every year – and this number is expected to grow. Despite the increased provision of cancer medicines on the market due to medical and technological developments, many of these cancer patients have unmet medical needs, meaning that there are no targeted or only limited treatment options for them. For example, there is no standard post-remission therapy to prevent relapse of acute myeloid leukemia. There is also a lack of approved pharmaceuticals to treat glioblastoma multiforme, mainly because of the inability to bypass the blood-brain barrier. In addition, less than half of patients diagnosed with mesothelioma survive the first year after diagnosis. Other cancers with low survival rates include metastatic non-small cell lung and pancreatic cancers and diffuse intrinsic pontine glioma, which primarily affects children[1].

While the new Regulation alone cannot solve the issue of unmet medical needs, it is key in addressing some of the root causes of the problem.

[1] Coppens D, Rommel W. (2023). The potential for academic development of medicines in Europe: case study of advanced therapy medicinal products. The Association of European Cancer Leagues. Available at: https://www.cancer.eu/wp-content/uploads/2023-03-23-Policy-paper_The-potential-for-academic-development-of-medicines-in-Europe.pdf.

We support the provisions described in **Article 48**, that allow not-for-profit entities to provide data to the EMA on repurposing existing medicines, potentially resulting in new therapeutic indications of off-patent medicinal products for unmet medical needs.

We strongly support **Article 59** on parallel scientific advice which will empower the EMA to provide scientific advice to medicine developers in parallel with the scientific advice given by the health technology assessment bodies. This will help medicine developers generate clinical evidence that meets the needs of the different authorities along the medicinal products' life cycle, while respecting the different remits of the legal frameworks concerned.

We support **Article 71** on market exclusivity for orphan medicinal products and **Article 72** on its prolongation as they provide modulated incentives.

We support broader use of regulatory sandboxes (**Articles 113-115**) for testing new regulatory approaches for novel technologies before formal regulation as long as it does not lead to a lowering of the evidence bar.

We fully endorse a support scheme, consisting of regulatory, procedural and administrative support and reduction, and the deferral or waiver of fees, dedicated to SMEs and not-for-profit entities as outlined in **Article 164**.

We applaud provisions that enhance scientific and regulatory support for priority medicines in **Article 60**, however this support should not be restricted to orphan medicinal products.

| **Article 60 – paragraph 1** | | |
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| **Text proposed by the Commission** | **Amendment** | **Justification** |
| 1. The Agency may offer enhanced scientific and regulatory support, including as applicable consultation with other bodies as referred to in Articles 58 and 59 and accelerated assessment mechanisms, for certain medicinal | 1. The Agency may offer enhanced scientific and regulatory support, including as applicable consultation with other bodies as referred to in Articles 58 and 59 and accelerated assessment mechanisms, for certain medicinal products that, | Currently it is not clear whether the developer must meet all three conditions (a b c) in order to receive the support, or if it is sufficient if only one or two of them are met. |

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| products that, based on preliminary evidence submitted by the developer fulfil the following conditions:<br>(a) are likely to address an unmet medical need as referred to in Article 83(1) of [revised Directive 2001/83/EC];<br>(b) are orphan medicinal products and are likely to address a high unmet medical need as referred to in Article 70(1);<br>(c) are expected to be of major interest from the point of view of public health, in particular as regards therapeutic innovation, taking into account the early stage of development, or antimicrobials with any of the characteristics mentioned in Article 40(3). | based on preliminary evidence submitted by the developer fulfil ***any of*** the following conditions: (a) are likely to address an unmet medical need as referred to in Article 83(1) of [revised Directive 2001/83/EC];<br>(b) are orphan medicinal products and are likely to address a high unmet medical need as referred to in Article 70(1);<br>(c) are expected to be of major interest from the point of view of public health, in particular as regards therapeutic innovation, taking into account the early stage of development, or antimicrobials with any of the characteristics mentioned in Article 40(3). | |

In addition, we endorse the European Commission's proposal to provide protocol assistance and in particular research support for orphan medicinal products in **Article 68**, however such support should not prioritise SMEs only, but should be equally available for the not-for-profit entities.

| Article 68 – paragraph 2 | | |
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| **Text proposed by the Commission** | **Amendment** | **Justification** |
| 2. Medicinal products designated as orphan medicinal products under the provisions of this Regulation shall be eligible for incentives made available by the Union and by the Member States to support research into, and the development and availability of, orphan medicinal products and in particular aid for research for small- and medium-sized undertakings provided for in framework programmes for research and technological development. | 2. Medicinal products designated as orphan medicinal products under the provisions of this Regulation shall be eligible for incentives made available by the Union and by the Member States to support research into, and the development and availability of, orphan medicinal products and in particular aid for research for small- and medium-sized undertakings ***and not-for-profit entities*** provided for in framework programmes for research and technological development. | Not-for-profit entities can play an important role in development of innovative treatments (e.g., advanced therapy medicinal products, which are often orphan medicinal products) and improve their access for cancer patients at a fair price[2]. |

[2] Coppens D, Rommel W. (2023). The potential for academic development of medicines in Europe: case study of advanced therapy medicinal products. The Association of European Cancer Leagues. Available at: https://www.cancer.eu/wp-content/uploads/2023-03-23-Policy-paper_The-potential-for-academic-development-of-medicines-in-Europe.pdf.

## 2. Access and affordability

Patient access to oncology medicines varies considerably across the EU. While in Germany 45 out of 46 (98%) new cancer medicines authorised between 2018 and 2021 at EU level were accessible to patients, countries with comparatively low prices or with low GDP, like Romania, had only 14 cancer drugs available. Moreover, small Member States such as the Baltic ones had less than 10 available (e.g., Latvia and Estonia - 8, Lithuania - 4)[3]. The time to patient access is also significantly longer for most of these latter countries, e.g. approximately 991 days after marketing authorisation in Romania compared to 102 days in Germany[4].

In addition to unequal access across the EU, the prices of cancer medicines also differ significantly between Member States. One study showed that official or list prices differ substantially between countries (up to 92% lower than the highest), and actual prices also differ between countries (up to 58% lower) and some medicines are more expensive in countries with lower GDP[5].

We are happy to see facilitation of the dialogue among the EMA and national authorities or bodies responsible for health technology assessment and pricing and reimbursement in **Article 162**.

We do not support the introduction of a transferable data 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 that end, we recommend deleting **Articles 40 – 43**. Development of antimicrobials should be supported by other types of incentives, such as direct financial incentives, see here.

## 3. Availability and prevention of shortages

Medicine shortages are an increasing problem in the EU. Shortages of oncology medicines pose a special challenge because cancer affects many people and because many oncology medicines have a narrow therapeutic window, meaning that these products cannot be easily substituted. According to a 2014 study[6], cancer medicines are one of the classes of medicines most commonly affected by shortages.

[3] Newton et al. (2023). EFPIA Patients W.A.I.T. Indicator 2022 Survey. Available at: https://www.efpia.eu/media/s4qf1eqo/efpia_patient_wait_indicator_final_report.pdf
[4] Newton et al. (2023). EFPIA Patients W.A.I.T. Indicator 2022 Survey. Available at: https://www.efpia.eu/media/s4qf1eqo/efpia_patient_wait_indicator_final_report.pdf
[5] van Harten, W. H., Wind, A., de Paoli, P., Saghatchian, M., & Oberst, S. (2016). Actual costs of cancer drugs in 15 European countries. Lancet oncology, 17(1), 18-20. https://doi.org/10.1016/S1470-2045(15)00486-6.
[6] Boshnakova A, Karnad A. (2017). Cancer medicines shortages in Europe: policy recommendations to prevent and manage shortages. The Economist Intelligence Unit. Available at: https://www.eiu.com/graphics/marketing/pdf/ESMO-Cancer-medicines-shortages.pdf.

The proposed Regulation can provide harmonised tools to allow Member States to better handle medicine shortages and thus act as an enabler for addressing the problem.

We support an introduction of the EU definition of a shortage in **Article 2**.

The provisions in **Article 24** requiring the market authorisation holder to offer, on reasonable terms, to transfer its market authorisation in case of an intended permanent withdrawal are very important and welcome.

Mandating market authorisation holders in **Article 116** to give earlier notification to the respective authorities if they intend to permanently cease marketing medicinal product (must be reported at least 12 months in advance) or to temporarily suspend it (at least 6 months in advance) will help secure the supply of medicines

We applaud the provisions in **Article 117** that obliges market authorisation holders to have a shortage prevention plan in place for any medicinal products put on the market and to keep it updated.

Setting obligations on the marketing authorisation holder in **Article 125** in case of a critical shortage is key to ensuring the availability of critical medicines for patients. To increase the transparency and avoid any shortages due to economic causes, such as withdrawals, market authorisation holders should also disclose the causes of shortages to the Agency.

| **Article 125 - paragraph 1 - point f** | | |
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| **Text proposed by the Commission** | **Amendment** | **Justification** |
| (f) inform the Agency of the end date of the critical shortage | (f) inform the Agency ***of the causes and*** of the end date of the critical shortage | To increase the transparency and avoid any shortages due economic causes, such as withdrawals, market authorisation holders should also disclose the causes of shortages to the Agency. |

## About the Association of European Cancer Leagues (ECL)

The Association of European Cancer Leagues (ECL) is a non-profit, pan-European umbrella organisation connecting 32 national and regional cancer societies in 27 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:


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