ECL and EFPN recommendations for the draft European Parliament's ENVI Committee reports for the pharmaceutical package

In view of the upcoming voting on the draft report for the pharma directive by MEP Pernille Weiss and the draft report for the pharma regulation by MEP Tiemo Wölken in the European Parliament's Committee on the Environment, Public Health and Food Safety (ENVI), the Association of European Cancer Leagues (ECL) and the European Fair Pricing Network (EFPN) call on members of the Committee to support reports that address unmet medical need (UMN) and ensure availability and timely access to safe, effective, and affordable medicines. To that end, we highlight key amendments below.

Draft ENVI Committee report for pharma directive

Article 2 Advanced therapy medicinal products prepared under hospital exemption

The hospital exemption (HE) plays a crucial role in providing timely access to safe, effective, and affordable advanced therapy medicinal products (ATMPs) in cases when there is a UMN, and pharmaceutical industry is not willing to invest in developing treatments due to lack of return on investment[1].

The harmonisation of the HE is needed as the current divergent national applications hinder equitable patient access across Europe. To that end, the directive should clearly define that *non-routine basis* entails a treatment scale that does not exceed the treatment capacity of a hospital. Moreover, the application of the HE should target UMN in situations when similar treatment is not available and/or affordable. Finally, holders of the HE that exceed preparation on a non-routine basis should be granted a transition period to obtain a centralised marketing authorisation (MA). To ensure this, amendment **415** (= **417** together with **423**) should be adopted.

Allowing cross-border exchange based on UMN is needed to guarantee EU-wide patient access when travelling is not possible due to severe illness. Adoption of amendment **428** would guarantee this.

It is crucial to provide academics developing ATMPs with scientific and regulatory advice for them to fulfil the procedures and requirements - amendments **432**, **437**, **444** and **468** would enable this.

The authorised ATMPs under the HE should be made public to provide insight into the available treatments across the EU. Adopting amendment **35** or **453** or **454** or **455** would facilitate this.

In addition, to ensure that non-commercial pathway for access to medicines is not affected by MA, amendment **474** should be adopted.

Finally, amendments **30**, **31**, **422** and **426** severely limit the applicability of the HE and should be rejected.

[1] https://www.cancer.eu/wp-content/uploads/2023-03-23-Policy-paper_The-potential-for-academic-development-of-medicines-in-Europe.pdf

Article 57 Responsibility to report on public financial support

We applaud efforts by many members of the European Parliament to strengthen the provisions aimed at increasing transparency around public funding for medicinal products development. Extending reporting obligations to the financial support received from charities, non-commercial organisations, academia, etc., as well as providing clarity on financial support such as tax breaks gives a better insight into stakeholders who contributed to a development of a medicinal product. This information, when provided in an easily accessible, full and timely manner, can help national authorities in the price negotiations and stakeholders in a more informed discussion on fair prices and public accountability. To that end, amendments **87**, **812**, **813**, **822** – **825**, **827**, **830**, **832** (= **833**), **834** (= **835** / **836** / **837**) and **838** – **844** should be adopted, whereas amendments **85** – **86**, and **819** – **820** should be rejected.

Article 81 Regulatory data protection periods

The redesign of the regulatory protection system from 'one size fits all' to a modulated one promotes patient access to affordable medicines across the EU and addresses the issue of UMN. We strongly support the reduction of basic regulatory data protection period as this would allow cheaper generic and biosimilar medicinal products to enter the market faster and therefore substantially improve access to treatments. To that end, amendment **1015** should be adopted.

Article 83 Medicinal products addressing an unmet medical need

To refocus the innovation on UMN, a clear definition of medicinal products addressing such needs is key. We fully support the establishment of a criteria-based definition of UMN, believe that the European Commission has correctly identified key criteria for such definition, and welcome its further specification in implementing acts, considering scientific input by the European Medicines Agency (EMA). To ensure that the concept of UMN reflects scientific and technological developments and current knowledge in underserved diseases, we support the involvement of all pertinent stakeholders and patient organisations when adopting scientific guidelines by the EMA for the application of this article. Therefore, amendment **1202** should be adopted, and other amendments should be rejected.

Article 85 Exemption to the protection of intellectual property rights

To improve access to medicines and their affordability across the EU, generics and biosimilars should enter the market at day-1 of the expiry of the market exclusivity period. Therefore, broadening the scope and harmonising the application of the 'Bolar exemption' is very important. To that end, amendments **1251** (= **1252** / **1253** / **1254** / **1255**), **1261**, **1287**, **1298**, **1299**, **1307**, and **1324** should be adopted, whereas amendments **116** - **119** should be rejected.

Draft ENVI Committee report for pharma regulation

Article 24 Suspension of marketing, withdrawal from the market of a medicinal product, withdrawal of a marketing authorisation by the marketing authorisation holder

The requirement for the MA holder to offer, on fair and reasonable terms, to transfer its MA for a medicinal product in case of an intended permanent withdrawal is very important as it contributes to preventing medicine shortages. Therefore, amendment **77** should be adopted.

Article 40 Granting the right to a transferable data exclusivity voucher + Article 41 Transfer and use of the voucher + Article 42 Validity of the voucher + Article 43 Duration of application of Chapter III

The transferable data exclusivity voucher for new antimicrobials could be extended to rare diseases or oncological therapeutics, and therefore further worsen access to these medicines. There are better alternatives to this policy option, therefore amendments **89**, **91**, **93**, **94** should be adopted.

Article 48 Scientific opinion on data submitted from not-for-profit entities for repurposing of authorised medicinal products

It is very important that not-for-profit entities are allowed to provide data on repurposing existing medicines to competent authorities, potentially resulting in new therapeutic indications of off-patent medicinal products. However, repurposing should not be limited to the areas of UMN, it should be possible for all indications. To that end, amendments **97** and **98** should be adopted. In addition, we propose to reject amendment **965** which obliges the EMA to consider the position of the MA holders on the totality of evidence submitted in its scientific evaluation of the benefit-risk of the use of a medicinal product with a new therapeutic indication.

Article 60 Enhanced scientific and regulatory support for priority medicinal products ('PRIME')

The provision of scientific and regulatory support for priority medicines is crucial, however it should not be restricted to orphan medicinal products only. Adopting amendment **107** would ensure this.

Article 68 Protocol assistance and research support for orphan medicinal products

Protocol assistance, and in particular research support for orphan medicinal products, should not be restricted to SMEs; it must be available for the not-for-profit entities too, as amendment **114** provides.

Article 71 Market exclusivity

The European Commission's proposal on length and modulation of market exclusivity for orphan medicinal products strikes the right balance between accessibility, affordability, and innovation. Shortening market exclusivity periods could lead to a more rapid introduction of competing products, such as biosimilars, to the market. However, overly limiting incentives might hinder development of innovative treatments. Moreover, the current market exclusivity period enjoyed by MA holders did not necessarily lead to more innovations compared to other markets with shorter market exclusivity periods but did in fact hinder competition[2]. We suggest maintaining the Commission's proposal in general with a few exceptions - we invite MEPs to adopt amendment **1197**, which allows the revision of market exclusivity to ensure that it incentivises innovation and new research by excluding products approved without new clinical trials by the MA holder. In addition, adopting amendment **1207** would incentivise development of treatments for the paediatric population specifically.

Article 72 Prolongation of market exclusivity

In general, we support the Commission's legislative proposal on prolongation of market exclusivity, however several amendments would improve it. While a modulated system of incentives that encourages developers to cater to all patients across Europe, including those belonging to smaller patient populations, is the step in the right direction, it is also necessary to curb evergreening and "salami slicing", and to re-evaluate orphan status. Market exclusivity should be extended one time only for a period of one year. If no new indication is registered three years prior to market exclusivity

[2] Giannuzzi, V., Conte, R., Landi, A. et al. Orphan medicinal products in Europe and United States to cover needs of patients with rare diseases: an increased common effort is to be foreseen. Orphanet J Rare Dis 12, 64 (2017). https://doi.org/10.1186/s13023-017-0617-1

expiration, it is time to pave the way for competition, enabling more affordable options. Also, if a product is highly profitable or no longer covers an orphan indication, its market exclusivity should be reduced. This approach will help facilitate more affordable and equitable treatment options for patients, including those battling cancer. To achieve this, amendments **123**, **1221**, **1273** and **1276** should be adopted.

Article 113 Regulatory sandbox

Many new scientific advances will require adapted regulatory frameworks for medicinal product development in the future. The current regulatory framework for MA is not able to predict all new developments in the domain of personalised treatment or tailored treatment for a small patient subgroup. Hospitals are at the forefront of such innovation and aim to develop 'plug-and-play' CAR-T cell therapy, for example, one product which can be modified to express different CAR's based on patient characteristics, for different indications. Novel medical solutions may also include a combination of products that enable diagnosis using advanced tools such as AI to predict or develop treatment. The regulatory sandbox will provide the needed regulatory space for developers to innovate and bring new technologies further. Exploration within the regulatory sandbox can create new insights for regulators, who can in turn innovate regulatory frameworks more rapidly and make them future proof. It is important that the regulatory sandbox is used to incite development of innovative treatments, yet it should not lead to lowering evidence requirements. To facilitate this, amendments **1398**, **1404**, **1407**, **1414**, **1419** and **1429** should be adopted.

Article 125 Obligations on the marketing authorisation holder in case of a critical shortage

Setting obligations on the MA holder 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, MA holders should also disclose the causes of shortages to the EMA. Adopting amendment **1624** (= **1625** = **1626**) would ensure that.

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.

About the European Fair Pricing Network (EFPN)

In November 2020, ten European cancer societies launched the European Fair Pricing Network (EFPN) – the first-ever EU-wide collaborative network to improve transparency, access, and affordability of cancer medicines for the benefit of cancer patients. EFPN has invested €1 million to team up with the Netherlands Cancer Institute (NKI) and the Organisation of European Cancer Institutes (OECI) to shed light on medicine pricing and translate findings into evidence-based policy for national and European decision-makers.