EFA response to the public consultation on the European Commission adoption of a proposal for a Directive on the Union code relating to medicinal products for human use (2023/0132)

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is the voice of 200 million people living with allergy, asthma, and chronic obstructive pulmonary disease (COPD) in Europe. We bring together 45 national associations from 26 countries and channel their knowledge and demands to the European institutions. We connect European stakeholders to ignite change and bridge the policy gaps on allergy and airways diseases so that patients live uncompromised lives, have the right and access to the best quality care and a safe environment.

EFA is a full member of the European Patients' Forum (EPF) and has contributed to the EPF's position papers on this proposal,[1,2,3] specifically on issues related to the incentives driving medical innovation to the patients most in need and the reduction of inequalities in the actual access to medicinal products in the EU, which are very important for our community and which we fully support.

EFA welcomes the proposed revision of the EU pharmaceutical framework, one of the most impactful EU files in the area of health, and in particular the proposal for a Directive on the Union code relating to medicinal products for human use, and repealing Directive 2001/83/EC and Directive 2009/35/EC. EFA has highly appreciated taking part in all phases of the consultative process launched by DG SANTE to inform this revision,[4,5,6] started in 2020, and welcomes the opportunity of this last public

[1] EPF, 'The European Patients' Forum calls for a patient-centred revision of the EU pharmaceutical legislation', July 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/epf-recommendations-for-the-revision-of-the-eu-pharmaceutical-legislation/.
[2] EPF, 'The European Patients' Forum calls for ensuring access to paediatric medicines', September 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/the-european-patients-forum-calls-for-a-patient-centred-revision-of-the-eu-pharmaceutical-legislation/.
[3] EPF, 'A patient-centred vision for unmet medical needs', October 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/a-patient-centred-vision-for-unmet-medical-needs/.
[4] EFA, 'EFA response to the new EU Pharmaceutical Strategy: towards safe and more accessible medicines', September 2020, available at https://www.efanet.org/news/news/3907-efa-response-to-the-new-eu-pharmaceutical-strategy-towards-safe-and-more-accessible-medicines.
[5] EFA, 'EFA response to European Commission consultation on the EU General Pharmaceuticals Legislation', December 2021, available at https://www.efanet.org/news/news/4110-efa-response-to-ec-consultation-on-the-eu-general-pharmaceutical-legislation.
[6] EFA, 'EFA participates in European Commission targeted workshop on the EU General Pharmaceuticals Legislation', January 2022, available at https://www.efanet.org/news/news/4111-efa-participates-in-ec-targeted-workshop-on-the-eu-general-pharmaceuticals-legislation.

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consultation to bring the perspective of allergy and airways diseases patients to the proposed Directive:

- Have a **patient-centred definition to address unmet medical needs**, that takes into account the patients' experience and the burden associated with disease;
- **Retain a mandatory paper package leaflet**, which is to be complemented by an electronic version leaflet;
- Introduce **information about the environmental impact of the medicinal product**, including the environmental footprint and guidance for product use and disposal on the package and/or leaflet;
- Improve the **regulatory process of combination products**, that is currently highly fragmented across legislations.

A 'unmet medical needs' definition that is patient-centred

EFA welcomes the Commission efforts to address unmet medical needs (UMN) through the introduction of a definition in Article 83 as well as three criteria to assess them. To fine-tune the legislation, EFA encourages the Commission to drive medical innovation further than "just" addressing the mortality and morbidity associated with a disease, closely linked to medical criteria, by also linking to **unmet patients' needs**, such as the disease severity and the disease burden, which is considerably high in patients with chronic diseases on patients and their carers. Moreover, the patient experience data (PED), which refers to patients' health status, symptoms, disease course, treatment preferences, quality of life and impact of health care,[7] should be better defined, collected, and embedded into the regulatory process, as they can provide with breakthrough perspectives when assessing the UNM.

The participation of patients' organisations in the definition and assessment of 'unmet medical needs' will be therefore crucial in this process, due to their unique experience and perspectives around disease. Patients' organisations must be therefore consulted throughout the process of defining 'unmet medical needs' to drive innovation and incentives.

In this context, EFA recommends that the proposed Directive on the medicinal products for human use addresses the following considerations:

- Have a **patient-centred definition to address 'unmet medical needs'** that includes the burden associated with diseases, by referring severity and quality of life as primary criteria, aspects which while not considered life-threatening, they can be significantly impairing the lives of patients and their caregivers and leading to the preventable healthcare costs. The use of PED will be crucial in this regard.

[7] EMA, Patient experience data in EU medicines development and regulatory decision-making, September 2022, available at https://www.ema.europa.eu/en/documents/other/executive-summary-patient-experience-data-eu-medicines-development-regulatory-decision-making_en.pdf.

- **Consult patients' organisations in defining 'unmet medical needs'**, to assess the impact of the disease and whether a medicine fulfils a UMNs.

Electronic package leaflet for medicines should not replace paper leaflets

Package leaflets are a direct information tool for patients using a medicine. They explain the active principle of a treatment, administration instructions and storage. Importantly, they provide with safety information and possible side effects. For patients and carers, it is imperative that this information is always easily accessible in all circumstances, as it allows for the prevention and recall of adverse effects, for example in the event of allergic reactions to a medicine or a cross-reaction.

EFA strongly supports the digital health transformation and the use of digital tools in health and care. In the case of medicines, electronic product information will allow for personalised information (i.e. in the case of pregnant patients and people with co-morbidities), warnings (i.e. the use of allergens as excipients or in the device), easier reporting of adverse effects (i.e. one stop shop for medicines information) and to make the European market more flexible to conduct joint procurement and react against shortages (online translations). However, we cannot turn a blind eye to the reality in the European Union regarding access to digital tools, the internet and digital literacy inequalities. Access to internet varies greatly within the Union, and it is especially more problematic in rural areas,[8] as well as when it comes to people over the age of 40.[9] EFA confirmed these findings in its 'DIG_IT Report: The asthma and COPD patients' digital journey', where it was emphasised that, particularly in rural areas, reliable internet can be a problem. Therefore, relying only on an electronic version of the package leaflet and placing the burden of accessing crucial information on patients to look for it or to request it at the time of purchase, can have serious unattended consequences on the access to safety information.

Patients' safety should prevail over savings. Consequently, EFA **opposes to article 63 of the proposal as it does not oblige for a mandatory paper format for package leaflets.** En revanche, EFA recommends that the **availability of an electronic package leaflet complements, and not replaces, the mandatory paper format of a package leaflet,** to ensure speedy, easy, and efficient access to up-to-date information independently of the digital capacity of the patient. Moreover, the proposal does not define who should provide the package leaflet to the patients that request them, leaving a major question mark in terms of the responsibilities/liabilities and costs related to the printed version of the package leaflet. As representatives of allergy patients, EFA witnesses the problems linked to the provision of information to consumers across the fast-evolving liberalisation of the drug market that

[8] Eurostat, Digital economy and society statistics – household and individuals, available at https://ec.europa.eu/eurostat/statistics-explained/index.php?title=Digital_economy_and_society_statistics_-_households_and_individuals#Internet_access.

[9] Patient Engagement Synapse, Understanding patients' and HCPs challenges, needs and preferences for patient information leaflets, available at https://www.ema.europa.eu/en/documents/other/executive-summary-patient-experience-data-eu-medicines-development-regulatory-decision-making_en.pdf.

includes physical pharmacies, online pharmacies, and package delivery companies. leaving to Member States to define who and how the paper leaflet is provided (at purchase, order or delivery) would create inequalities in the EU and lead to the burden of educating patients about how to get safety information about medicines.

Provide patients with environmental information of medicines

The environmental footprint of medicines and its impact on climate change is of great concern for EFA and its members. In this context, we welcome the proposal for an Environmental Risk Assessment (ERA) process in all medicines, including those already authorised, to limit the potential adverse effects of medicines within a One Health approach, and to make ERA mandatory for all pharmaceutical companies authorised to commercialise their products in the EU market.

It is regrettable that the proposal only foresees the ERA being part of the application dossier. In EFA's views, the main findings of ERA should also be accessible by patients, especially when the product's sustainability is intimately linked to the use of the drug, its storage, deposit, and disposal, and when the product directly contributes to antimicrobial resistance (AMR), environmental pollution and global warming.

Without access to environmental and climate information - like an environmental footprint scale on the package leaflet or climate friendly seals - patients are not able to play a full role in their care and their very own environmental footprint, despite their vulnerability to pollution. Medicines should not lead to preventable sources of pollution; patients wish to use medicines consciously, increase medicines efficacy in the intake and their adherence, as the pharmaceutical and healthcare sectors are set to experiment a green transition too. For example, critical medicines such as metered-dose-inhalers (MDIs) used as rescue medicines for obstructive respiratory diseases such as asthma and COPD, are due to accomplish a major transition towards less polluting treatments[10]. However, despite their known global warming potential, there is not package information about the footprint of these medicines, and patients continue to be highly dependent on these treatments despite they are advised and prescribed to use them more exceptionally.

EFA therefore recommends **including relevant information about the environmental impact of the medicinal product to improve use and disposal of medicines**, as part of the contents of the package leaflet (Annex VI of the proposed Directive).

[10] EFA, Statement on the Proposal for a Regulation of the European Parliament and of the Council on fluorinated greenhouse gases, November 2022, available at https://efanet.org/news/news/4192-statement-on-fgas-regulation-proposal.

Missed opportunity to improve the regulatory process for drug-device combinations and improve patient education

In general, combination products are often developed and evaluated by different authorities under different legislations, but they are then authorised as one product. This situation implies a regulatory divide between medicines and medical devices that might lead to basic patient considerations being forgotten in the regulatory process. While medicinal products benefit from extensive research and clinical trials -ensuring their quality, safety, and efficacy- combinations with medical devices undergo a distinct process, that lacks an EU level assessment and authorisation (there is no EMA for medical devices) and dilutes patients sustained and coherent input on the process (authorisation is mostly conducted at Member State level).

Improving the current legislative fragmentation through this directive is therefore paramount, as respiratory and allergy patients rely daily on the use of combination products such as inhalers, pens, auto-injectors, drops, sprays and patches to manage their disease. For EFA, patients' needs should be evaluated and integrated into the whole development and authorisation process of the combination, and not fragmented as it currently happens.

Moreover, specific legislative provisions of combination products could be linked to the level of complexity of the combined medicine, and therefore to the support that a patient needs to use those medicines correctly. Unfortunately, respiratory patients report not being taught how to use an inhaler correctly, which is their basic medication, or not to be monitored on the efficacy of their technique.[11]

If combination products would be recognised and authorised under a specific category within the EU, it would then be easier to recognise the patients' needs linked to that category of medicines. For example, allergy and respiratory patients' needs would translate into the information, training and monitoring patients receive when being prescribed, frequently or not, combination products. Information around intake technique, preparation, and hygiene of the medicine or even carer support when the patient is a child, would recognise the level literacy and monitoring a patients' needs, leading to positive health outcomes.

Whether these considerations should be integrated into this directive, the Medical Devices Regulation (2017/745), or in a separate (new) piece of legislation, is to be analysed by the co-legislators, but if and when these points will be addressed would probably define if the EU is ready to accommodate with success the ongoing increase in combination products, that now are including digital medical devices and patient- fed artificial intelligence tools.

[11] EFA, 'Active Patients Access Care' Report, September 2019, available at https://www.efanet.org/news/3763-efa-launches-european-report-active-patients-access-care.