EHA's recommendations for the revision of the EU pharmaceutical legislation

A key opportunity to improve the availability of and access to medicinal products

The European Hematology Association (EHA) is the leading representative of hematology and hematology professionals in Europe, with over 8,300 members. EHA advocates for a revised EU pharmaceutical legislation that improves the availability and access to medicinal products across the European Union. Research and innovation are only worth the investment - public as well as private - if they reach patients.

Drug repurposing *Article 48, 2023/0131 COD*

EHA strongly supports drug repurposing - beyond unmet medical needs - as a means to improving access to and affordability of medicines across Europe. Repurposing can accelerate drug development by leveraging existing innovation and data, and entails lower costs than developing a novel medicinal product from scratch. The creation of a publicly accessible database would facilitate the detection of new indications. We also call for a streamlined regulatory procedure and for the term "substantive evidence" to be clarified.

Hospital exemption *Article 2, 2023/0132 COD*

EHA calls for a robust and harmonized hospital exemption (HE) framework that does not curtail this essential complement to the commercial pathway, serving patients with unmet medical needs and requiring personalized treatment. The HE is essential for patients whose conditions are rare and/or not commercially viable. HE should not be confined to cases where no medicine for that indication is approved (at EU level), as approval does not always translate to availability and access.

The cross-border exchange of ATMPs prepared under the hospital exemption framework and a publicly available repository on hospital exemption are amendments we strongly support.

Reporting financial support *Article 57, 2023/0132 COD*

There are major differences between EU Member States when it comes to their health budgets, reimbursement decisions and, consequently, access to medicines. More insight into the costs incurred during the research and development of medicinal products, as well as how these have been shouldered (e.g. NGO funding, state aid, tax breaks, etc.), is crucial for more fair and transparent pricing negotiations. Reporting obligations in article 57 should be expanded to include financial support from charities and non-commercial organisations, as well as tax breaks. Funding associated with acquired medicinal products should also be disclosed.

Defining unmet medical need

Article 83, 2023/0132 COD

EHA supports an inclusive and future-proof definition of unmet medical need (UMN), that responds to the needs of patients. A new medicinal product should therefore be measured on how it impacts: (1) quality of life, (2) disease severity, progression and duration, (3) burden of illness on the patient (as well as society and healthcare systems), and (4) whether there are alternative treatments available.

Guidelines formulated within the context of Article 83.3 should include mandatory input from healthcare professionals and patients, as their insights are crucial to ensure a framework that is viable and fit-for-purpose. A future-proof UMN framework will require periodic re-evaluations of the applicable parameters, in order to keep up with clinical and patient needs.

Regulatory incentives

Articles 80-82, 2023/0132 COD

Finding a balance between promoting the development of innovative medicines and managing national healthcare expenditures is vital. EHA therefore supports a lower RDP and making additional regulatory protection conditional on the fulfilment of requirements increasing the availability of innovative therapies for patients across the EU.

Furthermore, incentives should be leveraged to encourage R&D within Europe. This is essential to better understand the genetic and environmental factors that contribute to diseases in the EU population and respond to its unique needs. Capacity building is important to ensure the EU's strategic autonomy and guarantee patient participation in innovative clinical trials.

EHA supports the creation of an access to medicinal products reporting system, as well as the obligation (introduced by the EP) to submit an application for pricing and reimbursement in all Member States that have requested this, with a complementary access notification system.

Bolar exemption

Articles 85 2023/0132 COD

Generics and biosimilars should be able to enter the market on 'Day 1' after the expiration of the proprietary rights of the reference medicine. A broad bolar exemption, as proposed by the European Parliament, is fundamental to prevent undue delays and improve medicine access and affordability – crucial for both patients and national health budgets.

Medicine shortages

Articles 2, 24, 116-8, 2023/0131 COD

EHA strongly supports the measures introduced to prevent and mitigate shortages, namely: (1) the requirement to draft shortage prevention plans with mandatory input from clinicians and patients, (2) the requirement to transfer the marketing authorization in case of market withdrawal of a critical medicinal product, and (3) the requirement to notify competent authorities in case of permanent marketing cessation or temporary suspension or supply disruptions.

EHA furthermore supports the definition of 'demand' introduced by the European Parliament.