POSITION PAPER

PHARMACEUTICAL LEGISLATION REVISION

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epha european public health alliance

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Introduction

The European Public Health Alliance (EPHA) welcomes the opportunity to provide feedback on the European Commission's proposed pharmaceutical package (proposed Regulation and Directive).

The proposed pharmaceutical package is a very positive step towards addressing unequal access to medicines across the Union as well as sky-rocketing prices of medicines and increasing shortages.

Timely and equitable access to quality healthcare and medicines are crucial to ensure universal health coverage and the long-term sustainability of healthcare systems.

In this short paper, we provide targeted recommendations to the current proposal on issues related to shortages of medicines, transparency of pharmaceutical R&D costs, the paper-based and electronic package leaflets, treatment optimisation, and incentives to develop new antimicrobials and access to new and old antimicrobials.

Regulation

Shortages of medicines

The proposed regulation is a significant step forward compared to the current situation. In particular, the proposal moves from a reactive towards a preventive approach to addressing shortages of medicines.

Nevertheless, to achieve the goal of preventing, forecasting, mitigating, and managing shortages of medicines the current proposal must overcome certain limits in the scope of the foreseen obligations.

Recommendations

- **The possibility for EU rules to be supplemented by national legislation to better prevent and address shortages should be explicitly acknowledged.**

- **A rule should be introduced to facilitate tracking the actual duration of shortages.** According to the proposal text, shortages lasting for less than 2 weeks do not have to be notified. However, the absence of notification does not allow the actual duration of the shortage to be checked.

- **All shortages should be notified regardless of their extension.** The current obligation by the national and European medicine agencies is too limited as it only requires market authorisation holders (MAH) to notify shortages expected to last for more than two weeks. The notification of all shortages would allow the competent authorities to monitor the actual duration and impact of such shortages.

- **All medicine shortages should be made public as soon as they are notified.** This will ensure that the competent authorities as well as interested parties such as patients, healthcare professionals and the public in general have timely access to this information and can better assess the frequency, extent, and impact of shortages. Additionally, healthcare professionals should be provided with information on available alternative treatment options, including generic or therapeutic substitution. In case an available alternative does not exist, healthcare professionals should be properly informed on the scope of clinical impact that delay or interruption of treatment may cause in patients.

- **MAH should be required to quickly and timely submit their shortage prevention plans to the competent authorities.** The current proposal text only requires MAH to have the prevention plans ready for inspection. However, it is impossible to monitor the effectiveness and the appropriateness of shortage prevention plans without a proactive transmission to the competent authorities and the possibility that patient associations and healthcare professionals can also have access to the plans. This would contribute both to the transparency of information and control of the adequacy of the plans.

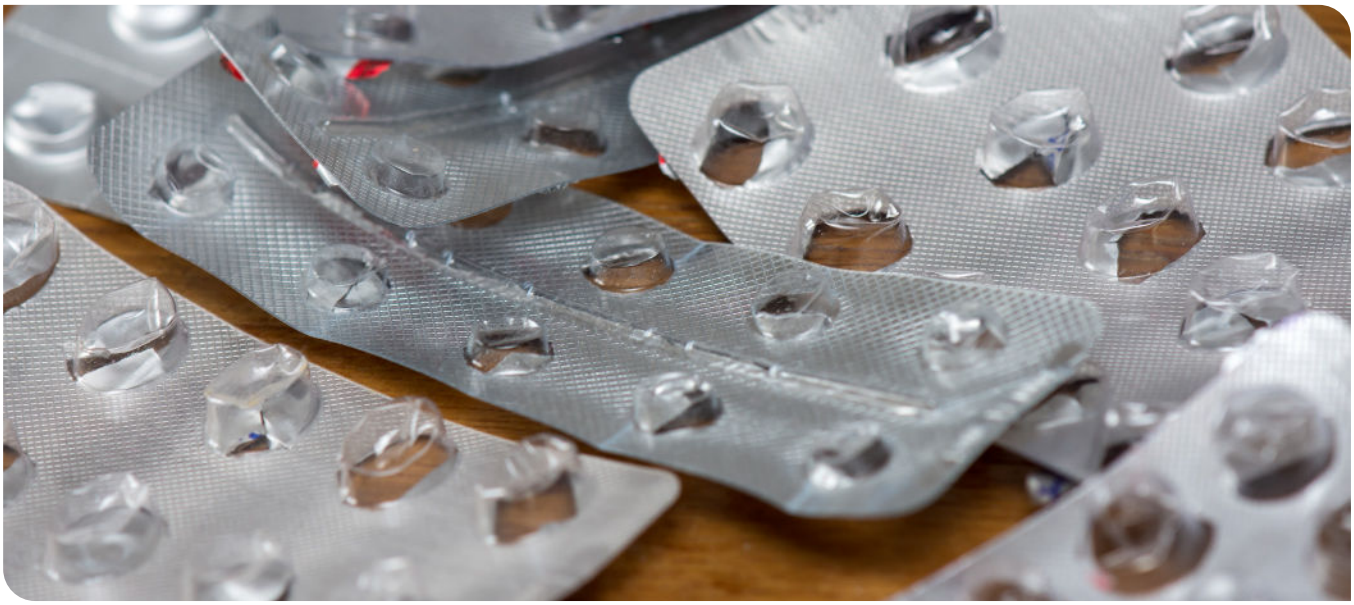
- **A real obligation to constitute safety stocks of medicine should be set up.** The possibility for the Commission to impose, via ad hoc decisions, contingency stock requirements of active pharmaceutical ingredients or finished products for a very limited list of medicines considered as critical at EU level is not sufficient.

- **The adoption and definition of methodology for the critical medicinal products list should involve healthcare professionals as well as patient and consumer organisations in a participatory process.** To make sure that the proposed provisions to strengthen the security of supply benefit all patients, it will be key to ensure consensus on the critical medicines that will be subject to these measures.

- **The role of the Commission in ensuring security of supply should be clarified in the Regulation.** The current proposal states that the Commission can impose contingency stock requirements of active pharmaceutical ingredients with an implementing act. However, this is not enough to ensure the security of supply and these requirements should be included in the Regulation.

- **A specific measure in case of infringement of the provisions on shortages should be added.** The proposal leaves it up to Member States to lay down the rule on penalties to be applicable to infringements. However, this provision is too general and does not include a deadline whereas dissuasive penalties in case of infringement are crucial to ensure the effectiveness of the provisions on shortages.

- **Extend the possibility for hospital pharmacists to produce medicines during shortages.** Hospital pharmacies can help to meet the needs of patients during shortages or withdrawals from the market of medicines with no equivalent or alternative. Because of this, it will be crucial to that the provisions of the Directive do not limit the possibility for hospital pharmacists to prepare medicinal products whether in terms of quantities produced or supply of the medicines to all patients in need.



Transferable Exclusivity Extension (vouchers) for new antimicrobials

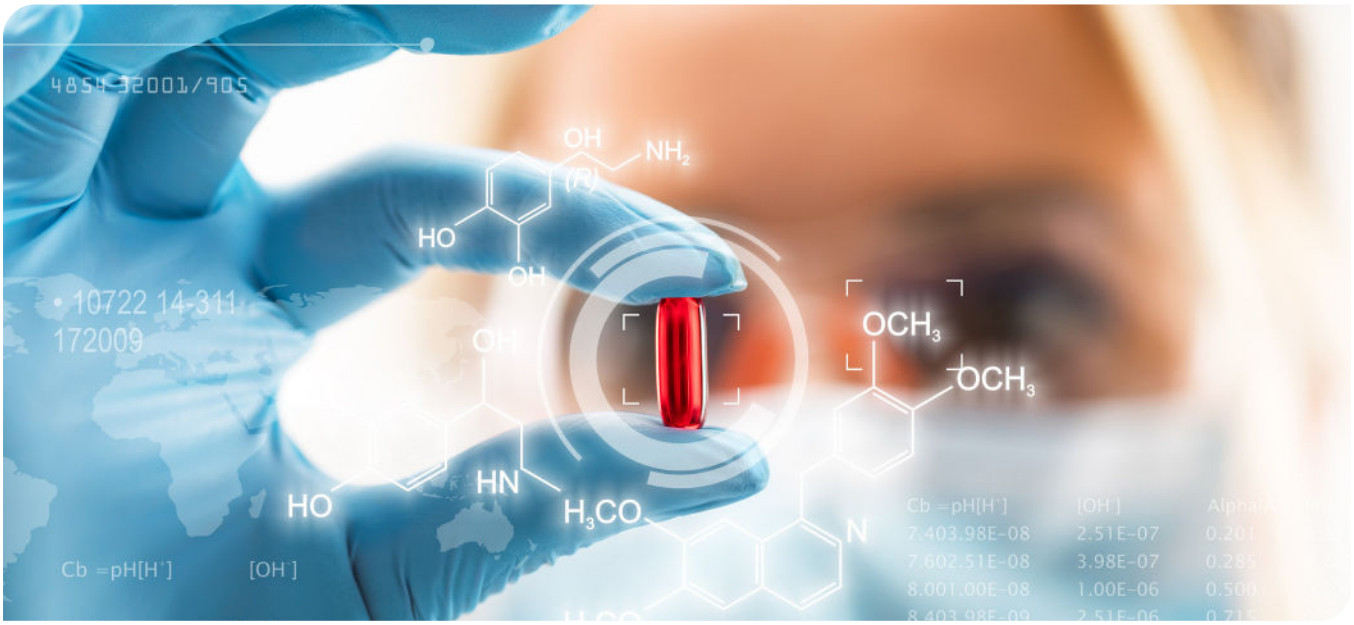
As argued by EPHA[1] and by 14 member states,[2] the transferable exclusivity extension (voucher) would create a very costly, non-transparent and indirect pull incentive to spur the development for new antimicrobials.

In lieu of this, direct incentives such as the ones identified in a report for the EU HERA and proposed by member states (e.g., market entry rewards, revenue guarantee, milestone R&D payments) should be included in the pharmaceutical legislation while their exact design should be left outside of the scope of the legislation. This would be in line with the Council Recommendations and will also help to address the threat of AMR and the lack of new antibiotics with urgency.

Recommendations

- **Substitute the transferable exclusivity extension (voucher) with a better set of push and pull incentives.** This could be done by including a reference to the options developed by the EU HERA and other push and pull incentives as the most suitable to tackle the problem of lack of new antibiotics.

- **Set up a fee-based system to raise additional funding from marketing authorisation applicants that are not investing in the development of priority antimicrobials.** This system (pay-or-play model) could be introduced with more detail by the delegated act.



1 https://epha.org/antibiotic-incentives-in-the-revision-of-the-eu-pharmaceutical-legislation/

2 https://www.permanentrepresentations.nl/documents/publications/2022/12/01/novel-stimuli-for-the-development-and-keeping-on-the-market-of-antimicrobials

Treatment optimisation

Many pharmaceutical products enter the market without clear evidence of their real clinical benefit for patients. To avoid this, independent clinical research studying the optimal way to use medicines should complement other clinical trials used for the authorisation of those medicines. Treatment optimisation (TO) studies are clinical trials questions related, for instance, to the dosage, treatment duration and treatment combinations, and that are not usually included in clinical research led by industry. Treatment optimisation can lead to similar therapeutic benefits with fewer toxicities for patients and potentially generate savings for healthcare systems.

A 2019 study[3] found that the obligation for the marketing authorisation holder to submit TO data to the EMA within a certain period of time after the authorisation would be effective to implement TO research.

Recommendation

- Add treatment optimisation to the post-authorisation studies that the Agency may require from the marketing authorisation holder.

Participation of patients and healthcare professionals

While the proposed text of the Regulation includes a consultation process of the Agency with the patients, medicine developers, healthcare professionals, industries and other stakeholders, the possibility to consult patients and healthcare professionals is kept open but not made mandatory.

It will be key to ensure that an obligation to consult patients and healthcare professionals is included in the final text of the proposal.

Recommendation

- Ensure that participation of patients and healthcare professionals is not optional but mandatory.

3 https://www.europarl.europa.eu/RegData/etudes/STUD/2020/641511/EPRS_STU(2020)641511_EN.pdf

Directive

Transparency of pharmaceutical R&D costs

The purpose of introducing transparency obligations is to promote fair prices and ultimately to advance access to affordable medicines. This was recognised in 2019 by the World Health Assembly Resolution WHA72.8[4], which states that "the availability of comparable price information may facilitate efforts towards affordable and equitable access to health products".

The proposal to review the EU pharmaceutical legislation aims to improve the current situation by creating an obligation for the marketing authorisation holder to declare direct financial support received from any public body in relation to the R&D activities of the authorised medicinal product. However, in its current form, the obligation is unlikely to enhance access to affordable medicines as it does not cover indirect financial support such as those obtained via tax advantages, which can provide significant advantages to companies. Moreover, while the stated aim of the obligation is to inform the public about the public contribution to pharmaceutical R&D, by leaving out indirect public contributions to pharmaceutical R&D, it risks giving a very incomplete (or even distorted) picture of these contributions, which are often very significant.

Recommendations

- **Include direct as well as indirect public funding under the scope of the obligation.** Including both direct as well as indirect public funding within the scope of article 57 of the Directive and including timeline for the submission requirement of 30 days after the marketing authorisation would improve the current scope of the obligation.
- **Include information about all R&D costs within the scope of the obligation.** Furthermore, for transparency obligations to contribute to more affordable medicines, such obligations would need to improve the understanding of all pharmaceutical R&D costs. Only in this way would policymakers be better able to shape incentives according to R&D costs and enhance the negotiation position of EU member states.
- **Further develop methodological guidelines to capture indirect public funding contributions and to capture all R&D costs via implementing acts.** Methodological guidelines should be developed in collaboration with academic researchers and international organisations working to improve transparency in pharmaceutical markets such as the WHO and the OECD.

4 https://apps.who.int/gb/ebwha/pdf_files/WHA72/A72_R8-en.pdf

Electronic Product Information

The digital gap across the EU is still very wide, partially linked to the age of users and mostly to socio-economic inequities. On top of the digital gap, a large share of European citizens does not have uninterrupted access to the digital tools and services necessary to guarantee access to electronic leaflets (ePILs). Gaps in the availability of Internet services and internet distruptions can prevent people living in areas not well covered from accessing the necessary information in ePILs. In addition, cyberattacks, which have already affected hospitals, can also affect the ePILs system.

Therefore, eliminating the obligation of paper-based leaflets while substituting them with electronic leaflets is very likely to increase inequities and health risks. While the proposed text of the Directive (Article 63(3)) establishes that patients should be guaranteed a paper leaflet upon request in the pharmacy, this policy, known as 'Print on Demand' (POD), would require pharmacists to print the full product information leaflet for the patient. This is not a suitable alternative to the current system of paper leaflets as printing the leaflet requires additional time for the pharmacist, whereas their role in administering medicine is vital in ensuring the patients' health and wellbeing. In addition, desktop printers used by pharmacies are subject to a risk of character substitution, possibly altering the content and meaning of the pharmaceutical leaflets.

Recommendations

- Maintain the mandatory obligation for paper-based package leaflets along with electronic package leaflets.
- There should no additional burden be placed on healthcare professionals to make available the paper version of the leaflet.

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