Joint Statement on the Revision of the Pharmaceutical Legislation by associations representing healthcare providers, patients, healthcare professionals and payers

Enhancing patients' access to high-quality and affordable medicinal products across the European Union while preserving the financial sustainability of healthcare systems should be the overarching aim of the current revision of the general pharmaceutical legislation.

AIDES, AIM, Consilium Scientific, CPME, EAHP, EATG, ECL, EPHA, GHA, HOPE, NoGracias, PGEU, Pharmaceutical Accountability Foundation, Prescrire, Salud por Derecho and Wemos have joined forces to outline how the revision of the general pharmaceutical legislation should:

1. Foster affordability to improve access to high-quality medicinal products,
2. Improve the assessment and evidence requirement of medicinal products' effectiveness and safety, and
3. Ensure a sufficient supply of medicinal products and combat shortages.

We call on the European Commission to include the considerations that have been put forward in this joint statement in the revision of the general pharmaceutical legislation.

<u>Foster affordability to improve access to high-quality medicinal products</u>

The future pharmaceutical legislation should foster the affordability of medicinal products by requiring the disclosure of research and development (R&D) costs and reducing the duration of exclusivities that impact competition and access. More transparency of R&D costs will help national authorities negotiate fairer prices. The duration of incentives should be reduced for those products that bring limited added therapeutic value and/or are not placed on the market across the EU. No further incentives should be created. To achieve consistency, this approach should be applied to the revision of the general pharmaceutical legislation and related instruments such as the orphan legislation and supplementary protection certificates (SPCs) legislation.

1. Request manufacturers to disclose precise information on the R&D costs of the medicinal product for which a European market authorisation is requested and on the public funding received. This information should be communicated to the national health authorities, so that they can take it into account when negotiating the price of the medicinal product. A Member

States Expert Group on medicines cost transparency should be tasked with developing the conditions for such a disclosure.[1]

2. The current unconditional duration of data and market protection incentives should be reduced to allow for earlier generic competition in the following situations:
    a. If the product is not placed on the market across the EU, and/or
    b. If the product only provides limited added therapeutic value in comparison with alternatives already on the market, and/or
    c. If the follow-up schemes on safety and efficacy of the product is not adhered to or if data provided is not sufficient and satisfactory.
3. The creation of transferable exclusivity extensions, i.e. vouchers, for the development of new antibiotics, which will lead to costs exceeding potential benefits and unpredictable public health expenses with no clear foreseen benefits, should be excluded.
4. We also call to address incentives beyond the basic pharmaceutical legislation:
    a. Revise the orphan medicinal products' legislation and implementing regulations to make the insufficient return on investment route mandatory in all cases.
    b. Revise the SPC Regulation to make sure that SPCs are granted in a transparent and uniform way across the EU to remove obstacles to generic and biosimilar market entry.
    c. Link the granting of an SPC to the proof that a company has not covered investment in research during the time of protection under the patent.

<u>Improve the assessment of medicinal products' effectiveness and safety</u>

Decision-makers need to have complete, relevant, and robust data to be able to evaluate medicinal products that are approved for the European market. The EU cannot compromise on safety and quality, even for accelerated procedures. Authorisations should be based on the assessment of the medicinal product's safety, quality, efficacy, survival, and quality of life at pre-authorisation stage via high-quality randomised clinical trials. If post-marketing data are needed, then their timely delivery must be guaranteed. Any relevant post-marketing data must be used for further enhancing safe use of medication.

1. Double-blind randomised clinical trials ensure the best access to quality comparative data on the benefit-risk and effectiveness profiles of medicinal products. They must therefore be clearly acknowledged in the legislation as the preferred form of evidence generation on medicinal products' safety, quality, and efficacy.
2. In order to facilitate decisions at HTA and pricing and reimbursement stages, the clinical evidence supporting regulatory decisions should also include as primary endpoints data on survival and quality of life compared to comparative therapies or best supportive care in the relevant patient population. Surrogate endpoints should be considered of secondary relevance.
3. The use of accelerated assessment procedures should be restricted and conditioned to a better definition of the circumstances in which the use of such procedures can be accepted. In addition, when benefiting from such procedures, companies must be compelled to collect and deliver under a strict timeline post-registration data in disease registries, ideally at EU level, to be shared with relevant decision makers and authorities at national level.
4. Valuable information on treatment optimisation is currently missing at the time of marketing authorisation. The review should aim to cover these evidence gaps by encouraging confirmatory trials to generate this missing data too.
5. Adverse events reporting by healthcare professionals, citizens, and patients, needs to be fostered.

[1] Cost transparency should cover: the costs to be taken into account; who should disclose these costs to whom; how to foster efficiency of R&D spending under such a system; how costs should be factored in prices, as well as which non-price elements should be taken into account (i.e. medicine's added therapeutic value).

6. Electronic product information (ePI) should complement but not replace printed leaflets, to avoid weakening access to essential information, in line with the ongoing work at EMA on risk minimisation measures.

Ensure a sufficient supply and prevent shortages of medicinal products.

Shortages of medicinal products have been increasing rapidly in all EU countries. They strongly impact patients' care and health, as well as healthcare professionals' work. The pharmaceutical legislation must include stringent rules to better prevent and mitigate shortages of medicinal products while also safeguarding the availability of other medicines and treatment options for patients, so as to ensure the continuity of best care for patients. It should not prevent Member States from adopting or maintaining stricter national rules.

1. The reporting of medicine shortages by manufacturers needs to be improved to enable the implementation of prevention measures as soon as the first supply tensions arise and to ensure that health authorities, healthcare professionals and patients receive all relevant information as early as possible.[2] The obligation for manufacturers to notify health authorities extends to shortages of any medicinal product and covers both unexpected shortages and supply tensions when the medicinal product cannot be supplied in sufficient quantity for all patients who need it.
2. In case of market withdrawal for commercial reasons, the manufacturer of the medicinal product must notify the competent health authorities at the latest one year in advance. It must allow and facilitate the transfer of the production to a third party to avoid any supply disruption.
3. The manufacturers of medicinal products must be required to prepare, submit, and regularly update transparent shortage prevention and management plans. When set up at European level, these plans also need to take into account the specificities of the countries in which the medicinal product is marketed (e.g., alternative therapies).
4. The problem of medicine shortages can only be addressed if all supply chain stakeholders are involved. Member States should be required to set up national task force teams, comprised of national agencies, industry, healthcare providers and professionals and patient organisations that discuss and adopt proactive measures for combating shortages. National shortages monitoring systems should be reinforced and coordinated with the European Medicines Agency taskforce, aiming at deploying a swift response in case of shortages.
5. Compounding possibilities to offer adequate patient care should be enhanced. To ensure the continuation of therapies, hospital pharmacists and community pharmacists should be clearly authorised to compound medicinal products in case of for example a shortage or product unavailability without any limitation as to the quantity and distribution of the compounded products.
6. The manufacturers should be required to keep a stock of semi-finished medicinal products to ensure the continuity of supply on the European market. This comes in addition to any national obligation to maintain safety stocks of finished products for the national market.
7. Any infringement of the rules and obligations intended to fight medicine shortages needs to be punished by dissuasive financial penalties. These sanctions should be made public.

Signatories

- AIDES
- Association of European Cancer Leagues (ECL)
- Consilium Scientific

[2] Reported information should include: harmonised information on shortage causes; start and planned end dates of the shortage; available stocks; available alternatives of medicinal products in shortage, etc.

- European Association of Hospital Pharmacists (EAHP)
- European Hospital and Healthcare Federation (HOPE)
- European Public Health Alliance (EPHA)
- Global Health Advocates (GHA)
- International Association of Mutual Benefit Societies (AIM)
- NoGracias
- Pharmaceutical Accountability Foundation
- Pharmaceutical Group of the European Union (PGEU)
- Prescrire
- Salud por Derecho
- Standing Committee of European Doctors (CPME)
- The European AIDS Treatment Group (EATG)
- Wemos