The Standing Committee of European Doctors (CPME) represents national medical associations across Europe. We are committed to contributing the medical profession's point of view to EU and European policy-making through pro-active cooperation on a wide range of health and healthcare related issues.

Position on the revision of the EU general pharmaceutical legislation

Main Messages:

1. The revision of the EU general pharmaceutical legislation provides a not to be missed opportunity to restore the balance in the pharmaceutical sector in the interest of patients and healthcare systems.
2. To ensure availability of centrally approved medicines, pharmaceutical companies should be obliged to launch their products in all Member States.
3. The revised legislation should require that the R&D costs of medicines are transparent and include a breakdown between private and public investments.
4. To prevent and manage medicine shortages, the EU legislation should ensure notification requirements, safety stocks of medicines and deterring penalties for non-compliance.
5. Any exclusivities granted to pharmaceutical companies to incentivise medicine development must be proportionate and prevent excessive prices and profits.
6. For the development of new antibiotics, market-based regulatory incentives such as transferable exclusivity extension should be avoided.
7. To ensure the safety and high quality of approved medicines, the revised legislation should limit the use of accelerated and conditional procedures and strengthen the obligations and requirements for market authorisation holders.
8. Medical professionals should have a clear process to feedback any adverse events experienced by their patients to promote patient safety.

Introduction

The revision of the legal framework governing medicinal products for human use in the EU should aim at improving standards to ensure a high level of public health protection and the quality, safety and efficacy of authorised medicines. The updated legislation should also ensure access to affordable medicines for patients and address unmet medical needs. Given that the public co-creates and is a major investor in health technologies, the EU should play a more active role in defining directions for health innovation to create a system that is aligned with medical and societal needs, rather than leaving it to be driven mainly by commercial interests.

The revision of Regulation 726/2004 and Directive 2001/83/EU provides a not to be missed opportunity to restore the balance in the pharmaceutical sector in the interest of patients and healthcare systems.

According to CPME, to achieve these goals, the European Commission should give priority to:

- availability and affordability of medicines;
- more resilient supply chains;
- fit for purpose incentives to address unmet medical needs;
- safety and quality of medicines.

Improving availability and ensuring affordability of medicines

The EU should adopt measures to promote the availability of centrally approved medicines by the European Medicine Agency in all Member States. Pharmaceutical companies should be obliged to file for pricing and reimbursement in a timely manner in all Member States, with any exceptions approved by national competent authorities on a case-by-case basis. It should not be possible for companies to withdraw these products later from certain markets for commercial reasons. Legislation should allow for an early introduction of generics in case of delayed market launch of medicines across the EU. Originator companies wishing to launch a product only in some Member States should be provided with an option to grant generic companies the right to market and sell the medicine under fair and reasonable licensing conditions.

Affordability can be ensured by putting in place concrete fair pricing conditions for any kind of public funding or incentives. Specific commitments in this regard can also be linked to marketing authorisation. The revised legislation should require that the R&D costs of medicines

that have benefited from public funding are transparent and include a breakdown between private and public investment. This would empower national authorities by reducing information asymmetry in pricing negotiations and enable informed discussion on what constitutes a fair price for these medicines.

As the use of generic and biosimilar versions of medicines leads to lowering costs of and increasing access to treatments, their immediate market entry after the expiry of patents on brand-name medicines is of crucial interest to the public. Generic companies should be able to conduct the necessary studies for obtaining marketing authorisations early on, without this being considered an infringement of IP rights. Clarification and unification of this exemption should be introduced in the revised legislation.

Ensuring resilient supply chains and managing medicine shortages

Medicine shortages are a growing public health threat with a serious impact on health care systems and public health. They can severely limit doctors' ability to provide appropriate treatment. Current legislation does not ensure the stable supply of medicines, as it contains only a few general provisions.

Communication is crucial in preventing shortages. Doctors and pharmacists must have access to up-to-date information to be able to adequately respond to arising and existing shortages. The revised legislation should require companies to notify national competent authorities and the European Medicines Agency as soon as possible. The current notification period should be examined and adjusted to the best national level practices. Moreover, it is critical to establish a standardised reporting system giving guidance as to what, when and how to report.

Medicine shortages can be addressed by measures related to the production of raw materials and to distribution of medicines that include stockpiling. The revised legislation should require companies to maintain safety stocks of medicines. When needed, stockpiling of medicines should take place at EU level. National stockpiling should only be introduced when not endangering neighbouring countries, regions or health care facilities with patients in need of the stockpiled medicine.

The revised legislation must be strengthened to hold companies accountable. It should require Member States, and the European Commission where applicable, to lay down deterring penalties.

Recognising the broader policy context of the Pharmaceutical Strategy, the European Commission should make recommendations on criteria other than the price to be taken into account in national procurement practices, such as diversification of supply chains, security of supplies and compliance with environmental and social standards, also in third countries. The Commission should establish a regular dialogue with member states and monitor the uptake of these recommendations and its effects.

Reviewing current system of incentives to address unmet medical needs

The current system of incentives based on intellectual property rights needs to be reshaped in the public interest to be truly patient-centred. The revised legislation needs to introduce a new system of incentives that would limit the granting of market exclusivity extensions and provide tailored and proportionate rewards for relevant innovations. It should ensure that exclusivities granted to companies that incentivise medicines development are proportionate and prevent excessive prices and profits. It should also reflect divergent cost of R&D, public contribution, and revenues from the end product. A high degree of transparency should therefore be a prerequisite for obtaining or benefiting from any form of incentives.

The revised legislation should provide reduced and differentiated data and market protection periods depending on the purpose of the medicine (i.e. longer period of protection in areas of unmet medical need). Lack of access to medicines due to the unwillingness of the market authorisation holders to market them in some Member States should never happen and therefore this should not be part of the definition of "unmet medical need". Unmet medical need exists when no disease-specific therapy is available and only supportive care is possible or when an established treatment does not significantly improve quality of life or provide significant or substantial additional benefit. Besides the above considerations, when defining an unmet medical need different disease-related aspects, e.g. mortality and severity of the disease, or its prevalence among others should also be considered.

The optimisation of the incentive system requires the definition of an "innovative medicine" as one that meets a previously unmet or inadequately met, substantive health need and offers enhanced effectiveness or other incremental benefit relative to existing therapeutic alternatives (see OECD report on Pharmaceutical Innovation and Access to Medicines, 2018). A product that is new or novel but does not offer additional benefit over existing therapies should not be considered innovative.

The lack of new antimicrobials shows that the current market-based R&D model is not appropriate for developing antibiotics. Consequently, market-based regulatory incentives such as transferable market exclusivity "voucher" (which anyway risk overcompensation and could disproportionately subsidise one area of healthcare at the expense of another) or data exclusivity extensions are not effective and should be avoided. First and foremost, the public sector needs to take more leadership. There is a considerable lack of pull incentives to facilitate the transition of antibiotic products from early clinical phases to commercialisation, which would reward the whole R&D value chain. Of the various pull incentives options, "de-linkage models" seem likely to stimulate antibiotic innovation most effectively.

Ensuring safety and quality of medicines

The increasing number of advanced medicinal products enter the market with limited information on safety issues and effectiveness. Accelerated approval processes have resulted in new medicines being more likely to be withdrawn for safety reasons. It is important that the use of accelerated and conditional procedures is limited to situations where no other medicinal alternative is available or when the quality of life is severely under pressure due to the condition. A strictly regulated framework is indispensable to safeguard patient safety and healthcare systems.

The legislation's revision should lead to an increased generation of comparable and robust data. Such data are a precondition for doctors to be able to assess adequately the health gains of new medicines versus current treatments. This can be done by harmonising clinical trial designs, by choosing reasonable endpoints and clinically most relevant performance targets, by early dialogues between relevant stakeholders, and by requiring randomised controlled trials to be conducted whenever possible. It is essential to ensure a high level of transparency in the operations of the European Medicines Agency with regard to the information on medicines it evaluates at different stages during the marketing and post-marketing authorisation process.

Post marketing evidence generation is key to assess medicines already present on the market. The legislation should strengthen the obligations and requirements for market authorisation holders to conduct necessary study introducing penalties in case of noncompliance, including a withdrawal of marketing authorisation. Healthcare professionals should have a clear process to feed back any adverse events experienced by their patients to promote patient safety. The information should be used to ensure that only safe and effective medication is prescribed.

The revision should help to achieve greater transparency on research data and evidence, decision-making process from clinical trials to marketing authorisation and HTA, but also on pricing negotiations and reimbursement.

Ensuring that product information is accessible to all, and in particular to patients/consumers with diverse abilities, is essential, and therefore the electronic product information (ePI) should never replace the paper version included in medicine packets but remain complementary. If used, ePI must meet standards of objectivity, be transparent, independent, and free of any advertising or commercial interests.