EPF's analysis of the European Parliament reports on the revision of the EU pharmaceutical legislation

June 2024

On 10 April 2024, the European Parliament adopted the reports on the Directive and the Regulation, thus formalising the **Parliament's official position on the revision of the EU pharmaceutical legislation**. The reports of the European Parliament amend the proposals for a Directive and a Regulation of the European Commission published in April 2023.

Below is a summary of the main changes proposed by the Parliament that are relevant to the patient community[1].

Key points:

- Inclusion of **patient representatives in the ad hoc working groups** set up by the Committee for Medicinal Products for Human Use.
- Inclusion of a **patient organisation representative in the Coordination Group for Decentralised and Mutual Recognition Procedures.**
- **Reimbursement of patients' expenses** incurred in performing their duties as members or alternates of EMA's scientific committees.
- Consultation of patients in drawing up the Union's **list of critical shortages and critical medicines**.
- Establishment of a system for **patients to report shortages at national level**.
- **Consultation of patients** if a Member State decides to implement electronic patient information leaflets only.
- Inclusion of a **key information section** in the package leaflet reflecting the results of consultations with patient organisations.
- Allowing patient organisations to **submit data for new indications for any medicine**, beyond those addressing unmet medical needs.

The functioning of the European Medicines Agency

The European Parliament's reports maintain the **Commission's proposal to switch from a committee-based** to an expert-based structure for the European Medicines Agency (EMA), but significantly **strengthen patient involvement**.

Under the new EMA structure, which retains only two scientific committees, the Commission envisaged that the Committee for Medicinal Products for Human Use (CHMP) would set up ad hoc working groups without specifying their topics. The Parliament establishes **ad hoc working groups on paediatric, orphan**, and **advanced therapy medicines**.

In addition to increasing patient representation in the Pharmacovigilance Risk Assessment Committee (PRAC) and introducing patient representatives in the CHMP, as planned by the Commission, the Parliament specifies that **patients should also be represented in the CHMP ad hoc working groups**.

[1] The incentives for developing medicines targeting rare diseases are not covered in this report.

Representatives of patient organisations serving as members or alternates of scientific committees will also be entitled to reimbursement of expenses incurred in the performance of their duties.

Finally, the Parliament agreed on the inclusion of a representative of patient organisations in the **Coordination Group for Decentralised and Mutual Recognition Procedures**, where patients are not represented currently.

Incentives for the development and access of medicines

The European Parliament **revises the Commission's modulated incentive model** and **proposes another mechanism to improve access to medicines across the EU**.

The Commission proposal reduces the regulatory data protection period of new medicines to 8 years (6 years of data protection[2] and 2 years of market exclusivity[3]), with a possible extension to 12 years under specific conditions such as launch in all EU member states, comparative clinical trial data or targeting unmet medical needs. In contrast, the Parliament's proposal sets a baseline of 7.5 years for regulatory data protection, extendable by 1 year for addressing unmet medical needs, 6 months for comparative clinical trials, and an additional 6 months for significant EU-based research and development with public involvement. This could potentially bring total regulatory data protection to 8.5 years, plus 2 years of market exclusivity, which can be extended by a further year for new therapeutic indications. This means that the total protection period could reach up to 11.5 years, compared to the current 11 years.

The Parliament **keeps the Commission's definition of "unmet medical need[4]"**, linked to mortality and morbidity, but clarifies in a recital that morbidity includes the patient's quality of life, disease and treatment burden, inability to perform daily activities, and relevant patient experience data in the assessment. Patients should also be consulted in developing scientific guidelines on unmet medical needs.

Of note, the Parliament **rejected the Commission's proposal** to make 2 years of regulatory data protection conditional on companies launching new products in all Member States. Instead, Member States will have 1 year to request the launch of the product in their national market, which will oblige **companies to apply for pricing and reimbursement** within 1 year (or 2 years for small and medium-sized companies). If Member States and companies comply with the Parliament's deadlines and the Transparency Directive, medicines could be on the market across all requesting EU countries in less than 2.5 years.

Finally, the Parliament calls on the Commission and Member States to **develop indicators to measure access to medicines in the EU**. It proposes the creation of a dedicated public website to provide transparent information on access indicators and availability of medicines across the EU.

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[2] Period during which generic or biosimilar applicants are not allowed to rely on originator data to obtain approval for their products through an "abridged" application.

[3] Period during which a generic or biosimilar cannot be placed on the market. However, a generic or biosimilar manufacturer may rely on the full data set to prepare its own marketing authorisation dossier.

[4] A medicine is considered to meet an unmet medical need if it treats a life-threatening or seriously debilitating condition and addresses the following conditions: (a) there is no approved medicine for the condition, or there is an approved medicine, but it is associated with high mortality and morbidity; (b) the medicine reduces morbidity or mortality in the relevant patient population.

Measures to tackle antimicrobial resistance

The Parliament introduces **milestone payment** and **market entry reward schemes** for 'priority' antimicrobials[5]. These schemes provide early-stage financial support for the achievement of specific research & development milestones before market authorisation. They are complemented by a voluntary **joint procurement scheme** based on a subscription model to encourage investment in antimicrobial development.

For companies not eligible for milestone payments, the Parliament maintains the **Transferable Exclusivity Vouchers** (TEVs) granted to companies developing a new antimicrobial, but adds more requirements. The voucher's regulatory data protection reward will now depend on the type of antimicrobial: 12 months for 'critical' antimicrobials, 9 months for 'high' priority antimicrobials and 6 months for 'medium' priority antimicrobials, as defined by the European Commission in subsequent implementing legislation. It remains transferrable once, which means that a company can sell the voucher to another company. That company will be able to use it on any product that has not already benefited from the maximum regulatory data protection period, within its first 4 years of regulatory data protection.

To promote the prudent use of antimicrobials, MEPs call for more stringent measures such as limiting prescriptions and dispensations to necessary treatment quantities and limiting prescription duration.

Medicine shortages

Beyond the measures proposed by the Commission, including earlier notification of shortages and withdrawals, a requirement for manufacturers' shortage prevention plans for all medicines, and stronger EU coordination mechanisms, the Parliament introduces additional provisions to tackle shortages. These include the institutionalisation of a **Voluntary Solidarity Mechanism**, which allows Member States to redistribute medicines to other Member States experiencing shortages. The Parliament also calls for Commission guidance to support **public procurement practices** that include criteria other than price.

Importantly, the Parliament significantly **enhances patient involvement in the management of shortages**. At the EU level, the EMA's Patients and Consumers Working Party (PCWP) would be consulted on future EMA guidelines on shortage prevention plans and on the Union's lists of critical shortages and critical medicines. At national level, patient organisations must be consulted on the identification of critical medicines within Member States. National authorities are also required to set up **a system for patients to report shortages**.

Changes to shortage information have also been introduced. All relevant information, including available alternatives, must be **actively communicated to healthcare professionals and patients** by the competent national authorities in a publicly accessible and user-friendly manner. In addition to national databases, the EMA should include information from other pertinent sources and databases wherever possible.

[5] An antimicrobial is considered a 'priority antimicrobial' if it helps fight antimicrobial resistance and has one of the following characteristics: (a) it is a new type of antimicrobial; (b) it works differently to other authorised antimicrobials; (c) it contains an active substance not previously authorised that addresses a multi-drug resistant organism and serious or life-threatening infection.

Information to patients

The Parliament's proposal **eliminates the option to provide package leaflets only in electronic form**. It is now up to Member States to decide whether package leaflets should be available only in paper form, only in electronic form, or in both forms for certain products, categories or all products. If a Member State chooses electronic-only availability, it must first consult patients, carers and relevant stakeholders. In the absence of specific national requirements, package leaflets should be available in both electronic and paper formats.

Patients retain the **right to request a paper copy** if the leaflet is only available electronically, and the Parliament specifies that patients should be informed of this right. In addition, companies may choose to provide a paper version on a voluntary basis. Whether electronic or paper, **a key information section** should be included, reflecting the results of consultations with patient organisations, to ensure that the leaflet is legible, clear, and easy to use.

Of note, medicines that are dispensed and administered by a qualified healthcare professional, rather than for self-administration, may have electronic-only leaflets.

Finally, the **antimicrobial resistance (AMR) awareness card** may be made available in paper format or both paper and electronic formats.

Updates to the marketing authorisation processes

The Parliament introduces a new provision for granting marketing authorisation on the basis of **a platform technology master file**. This means that once the relevant information has been reviewed and approved by a competent authority, it can be referenced in future submissions without having to be resubmitted. The details of the information to be included in this file will be determined by the EMA.

In addition, the Parliament **extends transparency requirements** beyond the Commission's proposal. It requires disclosure of all direct funding from public authorities or publicly funded bodies for the development of a new product, including philanthropic or non-profit organisations worldwide, as well as indirect financial support from EU public authorities or publicly funded bodies. Companies must also disclose any licensing agreements or acquisitions related to the medicine in earlier stages of development, specifying the stage of research and development.

In addition, **environmental risk assessments** for medicines will now assess the entire life cycle of the medicine, including manufacturing processes, and this information must be made publicly available.

Regulatory procedures and additional support for medicine development

The Parliament imposes stricter measures for non-compliance with conditional marketing authorisations' (CMA) requirements, including mandatory post-authorisation studies and the creation of a CMA database by the EMA. In addition, companies must now justify withdrawals or suspensions of marketing of medicines for commercial reasons.

The Parliament also includes several measures to promote patient safety. The Eudravigilance database, the system for managing and analysing information on suspected adverse reactions to medicines authorised or undergoing clinical trials, will now include data on medication errors. Member States should also develop and implement plans for the safe administration and handling of medicines, which may include the use of digital medication safety systems in hospitals and outpatient care settings.

In addition, the possibility for not-for-profit organisations, including patient organisations, to **submit data for a new indication** of an approved product is extended to all medicinal products, beyond those that

address an unmet medical need. Based on these data, including any additional evidence submitted by the marketing authorisation holder of the product in question, the EMA may assess the risk-benefit balance of the new therapeutic indication.

Compassionate use, which allows the use of unauthorised medicines outside a clinical study, now covers patients with treatment-resistant diseases, diseases causing psychological distress or in palliative care. **The Parliament also expands the proposed scope of PRIME**, an EMA scheme that supports the development of promising medicines targeting an unmet medical need. Medicines that address an unmet medical need, have orphan status ***or*** are of major public health importance will be eligible for PRIME.

Finally, the Parliament maintains but tightens the Commission's proposal for **regulatory sandboxes.** When faced with new innovative products that do not "fit" into the "traditional" regulatory framework, the Commission can set up an environment with the EMA, developers and other relevant stakeholders to test adapted, derogatory or deferred requirements for a product or category of products that offer major benefits to patients and cannot be developed in full compliance with the current rules (e.g. software-dependent implants). Sandboxes will only be possible on a case-by-case basis and must lead to an adapted regulatory framework when completed. The EMA will consult patients where appropriate.

Next steps

The dossier is now in the hands of the Council of the European Union, which started negotiations on provisions related to shortages and incentives under the Belgian Presidency (January-June 2024). Negotiations are expected to continue for several months to come. Once the Council has adopted its negotiating position, the trilogue will begin, involving inter-institutional negotiations between representatives of the European Parliament, the Council, and the European Commission.

Throughout this process, EPF will continue advocating for a revision that meets patients' needs and puts patients at the heart of the regulatory system for medicines in the EU.

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