EFA response to the public consultation on the European Commission adoption of a proposal for a Regulation on laying down procedures for the authorisation and supervison of medicinal products for human use and establishing rules for governing the European Medicines Agency (2023/0131)

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is the voice of 200 million people living with allergy, asthma, and chronic obstructive pulmonary disease (COPD) in Europe. We bring together 45 national associations from 26 countries and channel their knowledge and demands to the European institutions. We connect European stakeholders to ignite change and bridge the policy gaps on allergy and airways diseases so that patients live uncompromised lives, have the right and access to the best quality care and a safe environment.

EFA is a full member of the European Patients' Forum (EPF) and has contributed to the EPF's position papers on the topic,[1,2,3] and specifically on issues related to addressing shortages and threats to health like antimicrobial resistance (AMR), which are very important for our community and which we fully support.

EFA welcomes the proposed revision of the EU pharmaceutical framework, one of the most impactful EU files in the area of health, and in particular the proposal for a Regulation on the Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency (EMA). EFA has highly appreciated taking part in all phases of the consultative process launched by DG SANTE to inform this revision,[4,5,6] started in 2020, and

[1] EPF, 'The European Patients' Forum calls for a patient-centred revision of the EU pharmaceutical legislation', July 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/epf-recommendations-for-the-revision-of-the-eu-pharmaceutical-legislation/.

[2] EPF, 'The European Patients' Forum calls for ensuring access to paediatric medicines', September 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/the-european-patients-forum-calls-for-a-patient-centred-revision-of-the-eu-pharmaceutical-legislation/.

[3] EPF, 'A patient-centred vision for unmet medical needs', October 2023, available at https://www.eu-patient.eu/news/latest-epf-news/2023/a-patient-centred-vision-for-unmet-medical-needs/.

[4] EFA, 'EFA response to the new EU Pharmaceutical Strategy: towards safe and more accessible medicines', September 2020, available at https://www.efanet.org/news/news/3907-efa-response-to-the-new-eu-pharmaceutical-strategy-towards-safe-and-more-accessible-medicines.

[5] EFA, 'EFA response to European Commission consultation on the EU General Pharmaceuticals Legislation', December 2021, available at https://www.efanet.org/news/news/4110-efa-response-to-ec-consultation-on-the-eu-general-pharmaceutical-legislation.

[6] EFA, 'EFA participates in European Commission targeted workshop on the EU General Pharmaceuticals Legislation', January 2022, available at https://www.efanet.org/news/news/4111-efa-participates-in-ec-targeted-workshop-on-the-eu-general-pharmaceuticals-legislation.

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welcomes the opportunity of this last public consultation to bring the perspective of allergy and airways diseases patients to the proposed Regulation.

In this context, EFA would like to highlight the following points in light of the proposed Regulation:

- Welcomed **increased patients' involvement** in the overall regulatory process, with representations of patients in the CHMP and PRAC at the EMA;
- **Retain the expertise of the paediatric and orphan committees** at the EMA, by maintaining the PDCO and COMP, or similar permanent structures;
- Reinforce the patients' voice through **financially compensated patients' representatives for their contribution in the EMA Committees.**

Welcomed increased patients' involvement in the overall regulatory process

The meaningful involvement of patient organisations and individual patients in the regulatory process leading to the authorisation of medicines in the EU is of utmost importance: patients are the end users of medicines and the ones who can attest to the efficacy and efficiency of a drug, and their overall patient experience is contributing to the quality and control of the medicines placed on the market. To this end, patient organisations and individual patients, are crucially contributing their unique perspective, expertise and knowledge to the regulatory work of EMA, participating in EMA committees, procedures, workshops and reflection processes, and in the EMA Management Board.

Given the excellent collaboration between EMA and eligible organisations such as EFA, we **welcome the Commission proposal to enlarge the patients' involvement to the Committee for Human Medicinal Products (CHMP), with four patient representatives and four alternates. Equally important is the proposal to increase the patient representation in the Pharmacovigilance Risk Assessment Committee (PRAC), from one member and one alternate to two members and two alternates.** The permanent participation of patient representatives in these committees is beneficial for the regulatory process, as it provides with patient-centred analysis in the authorisation of medicines, it reinforces safety mechanisms, and increases transparency and trust in the process.

Illogical loss of paediatric and orphan expertise

The step forward proposed for CHMP and PRAC is nevertheless detrimental to the current EMA-patients collaboration. It is unexpected and discouraging to see the Commission proposal to eliminate two crucial EMA committees, namely the Paediatric Committee (PDCO), which currently has three members and three alternates representing patients' organisations,[7] and the Committee for Orphan Medicinal Products (COMP), with three members nominated to represent patients organisations,[8] at the same time the Union pretends to drive medicinal innovation towards underserved populations

[8] EMA, Rules of procedure of the Committee for Orphan Medicinal Products (COMP), October 2021, available at https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/comp-rules-procedure_en.pdf.

such as children and rare disease patients[9]. From a patient perspective, the proposed reduced structure will not be up to the challenge of serving children and rare disease patients.

Most rare diseases do not have yet an approved treatment,[10] and often children are prescribed medicines which have not been clinically tested to their needs, off label. Due to their expertise, PDCO and COMP have been imperative in prioritising research and development in paediatric and orphan needs, areas that lack incremental and breakthrough therapeutic innovations, as in general product developments tend to target the more prevalent diseases and profitable population segments.

While some of the expertise of the current committees might be retained with increased patient representation in CHMP, the absence of specific committees focusing exclusively on the needs of children and rare disease patients, will lead to less involvement of patients in the early stages of the development process. Moreover, getting this expertise in an ad-hoc basis through working parties will not produce the same long-lasting benefits as under the current framework where the work of the PDCO and COMP is continuous, with the committee being clearly structured, with solid rules of procedure in place, and patient representatives building their expertise through their regular interactions at the EMA and ongoing review of applications.

Therefore**, EFA recommends keeping the current PDCO and COMP, or similarly structured committees,** with regular meetings, continuous work, and robust composition.

Opportunity to reinforce the patient voice, expertise and scrutiny

The revision of this regulation should not be a missed opportunity to recognise the importance of patient participation. To date, putting patient representatives involved in the work of EMA in the same footage as Member State representatives lead to inequalities within the members of the committees. Patient representatives or carers who dedicate the time and resources to be involved in the work of the EMA are volunteers who spend a considerable amount of time (usually two or three days a month without discretion). They provide this work without any backup due to confidentiality issues and nomination on a personal basis, to represent the patient community in the regulatory process, finding themselves in a vulnerable position. The involvement of patients in such important work at the EMA scientific committees for the advancement of medicines must be reasonably compensated for the efforts in preparing, contributing, travelling for, and attending the meetings, which will also increase the patient involvement and ensure independence. As such, **EFA recommends that patient representatives are financially compensated for their work in the EMA Committees.**

[9] EFA, Feedback to the revision of the EU general pharmaceuticals legislation – proposal for a Directive, November 2023, available at https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12963-Revision-of-the-EU-general-pharmaceuticals-legislation/F3443053_en.

[10] '95% of the over 6000 recognised rare diseases still have no treatment option and for those that have, the majority of the treatments are symptomatic and not curative.' - European Commission, Commission Staff Working Document, Impact Assessment Report on Proposal for a Directive of the European Parliament and of the Council on the Union code relating to medicinal products for human use, and Proposal for a Regulation of the European Parliament and of the Council laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, available at https://health.ec.europa.eu/system/files/2023-04/swd_2023_192_1-2_ia_en.pdf.