European Lung Health Group recommendations on the revision of the EU pharmaceutical legislation

Contents

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1. Introduction

The European Lung Health Group is an informal group of European level patient organisations and healthcare professionals, representing 179 member associations across 34 European countries, active in the respiratory health spectrum which ranges from common infectious diseases (such as tuberculosis, pneumonia and SARS) and non-communicable diseases (such as allergies, asthma, chronic obstructive pulmonary disease (COPD), bronchiectasis and lung cancer) to those classified as rare and ultra rare (alpha-1, idiopathic pulmonary fibrosis, pulmonary hypertension).

The ELHG centres around bringing together know-how to support our organisations in empowering patients with lung diseases, improving quality of life, optimising multidisciplinary care, and early diagnosis, and research. At the European level, the ELHG strives for better care, increased patient participation and improved prevention.

The ELHG welcomes the proposed revision of the EU pharmaceutical framework, which is one of the most impactful works in the area of health at the European level. In particular, the ELHG are keen to inform legislation seeking to drive medical research, medicines innovation especially to underserved populations such as children and patients with rare diseases, broader and equal patient access and the involvement of patients and healthcare professionals' organisations across the regulatory process. The recommendations proposed below address the views of the ELHG on how to improve the framework for medicinal products for human use.

2. Proposed Directive 2023/0132

Amendment 1 (Directive)

Recital 50

Text proposed by the European Commission	*Amendment*
(50) The establishment of a criteria-based definition of 'unmet medical need' is required to incentivise the development of medicinal products in therapeutic areas that are currently underserved. To ensure that the concept of unmet medical need reflects scientific and technological developments and current knowledge in underserved diseases, the Commission should specify and update using implementing acts, the criteria of satisfactory method of diagnosis, prevention or treatment, 'remaining high morbidity or mortality', 'relevant patient population' following scientific assessment by the Agency. The Agency will seek input from a broad range of authorities or bodies active along the lifecycle of medicinal products in the framework of the consultation process established under the [revised Regulation (EC) No 726/2004] and also take into account scientific initiatives at EU level or between Member States related to analysing unmet medical needs, burden of disease and priority setting for research and development. The criteria for 'unmet medical need' can be subsequently used by Member States to identify specific therapeutic areas of interest.	(50) The establishment of a criteria-based definition of 'unmet medical need' is required to incentivise the development of medicinal products in therapeutic areas that are currently underserved. To ensure that the concept of unmet medical need reflects scientific and technological developments and current knowledge in underserved diseases, the Commission should specify and update using implementing acts, the criteria of satisfactory method of diagnosis, prevention or treatment, 'remaining high morbidity ~~or~~**,** mortality, **or *severity***', 'relevant patient population' following scientific assessment by the Agency. The Agency will seek input from a broad range of authorities or bodies, ***including relevant patient organisations and healthcare professionals*** active along the lifecycle of medicinal products in the framework of the consultation process established under the [revised Regulation (EC) No 726/2004] and also take into account scientific initiatives at EU level or between Member States related to analysing unmet medical needs, burden of disease and priority setting for research and development. The criteria for 'unmet medical need' can be subsequently used by Member States to identify specific therapeutic areas of interest.

Justification: The proposed definition of what constitutes an 'unmet medical need' should be significantly improved, as it is at the moment solely based on considerations of mortality and morbidity, excluding the burden of chronic diseases on patients and carers. In order to drive pharmaceutical innovation where it is most needed, 'unmet medical need' should take into account life-changing aspects for patients, including the severity of the disease. Moreover, as the definition of unmet medical needs will determine companies' eligibility for incentives and accelerated regulatory processes, thereby driving their R&D efforts, it is essential that it is comprehensive and inclusive. For that reason, it will be of utmost importance to define the concept of "unmet medical need" in

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cooperation with patients, in particular to avoid the "orphanisation" of new medicines, where more and more drugs are labelled as "unmet medical need" only to apply for accelerated approval and justify higher prices. Unmet medical need is not always a yes/no question.

Amendment 2 (Directive)

Recital New – indicative Recital 50

Text proposed by the European Commission	*Amendment* *(New)* ***To identify the unmet medical needs within the criteria-based definition of this Directive, a consensus on the definition shall be found with the consultation of relevant patient and healthcare professional organisations at the Agency level.***

Justification: See justification Amendment 17.

Amendment 3 (Directive)

Recital (new) – indicative Recital 69

Text proposed by the European Commission	*Amendment* *(New)* ***To address the impact of the lifecycle of medicinal products on the environment and public health, patients shall be informed of the environmental impact of the medicines on the package leaflet, including the environmental footprint, and sustainable ways for product disposal.***

Justification: The environmental footprint of medicines and its impact on climate change is of great concern for the ELHG. In this context, the main findings of ERA should also be accessible by patients, especially when the product's sustainability is intimately linked to the use of the drug, its storage, deposit, and disposal, and when the product directly contributes to antimicrobial resistance (AMR), environmental pollution and global warming. Without access to environmental and climate information - like an environmental footprint scale on the package leaflet or climate friendly seals - patients are not able to play a full role in their care and their very own environmental footprint, despite their vulnerability to pollution.

Amendment 4 (Directive)

Recital 127

Text proposed by the European Commission	*Proposed EFA amendment*
(127) The use of electronic and technological possibilities other than paper package leaflets can facilitate access to medicinal products, medicinal products distribution and should always guarantee equal or better quality of information to all patients compared to the paper form of product information.	*(127) The use of electronic and technological possibilities ~~other than~~* ***complementary to*** *paper package leaflets can facilitate access to medicinal products, medicinal products distribution and should always guarantee equal or better quality of information to all patients compared to the paper form of product information.*

Justification: The ELHG supports the digital health transformation and the use of digital tools in health and care. In the case of medicines, electronic product information will allow for personalised information, warnings, easier reporting of adverse effects, and to make the European market more flexible to react against shortages. However, the electronic package leaflets should complement, and not replace the paper package leaflets. The access to digital tools, internet, and digital literacy levels vary greatly within the European Union, and it is especially problematic in more rural areas, as well as when it comes to people over the age of 40. Therefore, relying on only on an electronic version of the package leaflet and placing the burden of accessing crucial information on patients to look for it or to request it at the time of purchase, can have serious unattended consequences on the access to safety information.

Amendment 5 (Directive)

Recital 129

Text proposed by the European Commission	*Proposed EFA amendment*
(129) Where Member States decide that the package leaflet should be made available in principle only electronically, they should also ensure that a paper version of the package leaflet is to be made available on demand and without additional cost to patients. They should also ensure that the information in digital format is easily accessible to all patients, for instance by including in the outer packaging of the product a digitally readable barcode, which would direct the patient to the electronic version of the package leaflet.	*(129) Where Member States decide that the package leaflet should be made available ~~in principle only~~ electronically, they ~~should~~* ***must*** *also ensure that a paper version of the package leaflet is ~~to be made~~ available. ~~on demand and without additional cost to patients.~~ They should also ensure that the information in digital format is easily accessible to all patients, for instance by including in the outer packaging of the product a digitally readable barcode, which would direct the patient to the electronic version of the package leaflet.*

Justification: See justification Amendment 4.

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Amendment 6 (Directive)

Article 4 – Definitions

Paragraph 1 – point 53a (new)

Text proposed by the Commission	*Amendment*
	(53)a. 'patients' organisations' means not-for-profit organisations which are patient-focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in every of governing bodies".

Justification: Patients' organisations, led and integrated by patients and carers, are irreplaceable stakeholders in the development and authorisation of medicines, and only their involvement can ensure that patients views are legitimately represented. Therefore, the centrality of patient involvement in decisions around treatment should be recognised, and integrated into the EU medicines legislation through a clear definition of 'patients' organisation'. The proposed definition is the current EMA definition of a patient organisation.

Amendment 7 (Directive)

Article 18 – paragraph 1: Integral combinations of medicinal products and medical devices

Text proposed by the Commission	*Amendment*
1. For integral combinations of a medicinal product and a medical device the marketing authorisation applicant shall submit data establishing the safe and effective use of the integral combination of the medicinal product and the medical device. As part of the assessment, in accordance with Article 29, of the integral combination of a medicinal product and a medical device the competent authorities shall assess the benefit-risk balance of the integral combination of a medicinal product and a medical device, taking into account the suitability of the use of the	1. For integral combinations of a medicinal product and a medical device the marketing authorisation applicant shall submit data establishing the safe and effective use of the integral combination of the medicinal product and the medical device. As part of the assessment, in accordance with Article 29, of the integral combination of a medicinal product and a medical device the competent authorities shall assess the benefit-risk balance of the integral combination of a medicinal product and a medical device, taking into account the suitability of the use of the

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medicinal product together with the medicinal device.	medicinal product together with the medicinal device, ***especially by paediatric patients, including storage, assemblage, hygiene and application/intake technique required.***

Justification: Combination products are often developed and evaluated by different authorities under different legislations, but they are then authorised as one product. This situation implies a regulatory divide between medicines and medical devices that might lead to basic patient considerations being forgotten in the regulatory process. While medicinal products benefit from extensive research and clinical trials -ensuring their quality, safety, and efficacy- combinations with medical devices undergo a distinct process, that lacks an EU level assessment and authorisation (there is no EMA for medical devices) and dilutes patients sustained and coherent input on the process (authorisation is mostly conducted at Member State level).

Amendment 8 (Directive)

Article 18 – paragraph 5

Text proposed by the Commission	*Amendment*
5. The marketing authorisation applicant shall, upon request from the competent authority, submit any additional information related to the medical device and that is relevant for the benefit-risk balance assessment of the integral combination of a medicinal product with a medical device referred to in paragraph 1.	5. The marketing authorisation applicant shall, upon request from the competent authority, submit any additional information related to the medical device and that is relevant for the benefit-risk balance assessment ***and efficacy*** of the integral combination of a medicinal product with a medical device referred to in paragraph

Justification: Improving the current legislative fragmentation through this directive is therefore paramount, as respiratory and allergy patients rely daily on the use of combination products such as inhalers, pens, auto-injectors, drops, sprays and patches to manage their disease. Patients' needs should be evaluated and integrated into the whole development and authorisation process of the combination, and not fragmented as it currently happens. Finally, a harmonization of the basic features technology platforms is always welcomed by patients, as it would simplify the intake of medicines for patients with several medicinal products in their treatment.

Amendement 9 (Directive)

Article 19 – paragraph 1: Medicinal products in exclusive use with medical devices

Text proposed by the Commission	*Amendment*

1. For medicinal products in exclusive use with a medical device the marketing authorisation applicant shall submit data establishing the safe and effective use of the medicinal product taking into account its use with the medical device. As part of the assessment, in accordance with Article 29, of the medicinal product referred to in the first subparagraph, the competent authorities shall assess the benefit-risk balance of the medicinal product taking into account the use of the medicinal product together with the medical device.	1. For medicinal products in exclusive use with a medical device the marketing authorisation applicant shall submit data establishing the safe and effective use of the medicinal product taking into account its use with the medical device. As part of the assessment, in accordance with Article 29, of the medicinal product referred to in the first subparagraph, the competent authorities shall assess the benefit-risk balance of the medicinal product taking into account the use of the medicinal product together with the medical device, ***especially by paediatric patients, including storage, assemblage, hygiene and application/intake technique required.***

Justification: Such a detailed assessment could link to the level of complexity of the combined medicine, and therefore to the support that a patient needs to use those medicines correctly. Unfortunately, respiratory patients report not being taught how to use their basic medication correctly (i.e. inhaler), or not to be monitored on the efficacy of their technique.

Amendment 10 (Directive)

Article 22a – (new)

Text proposed by the Commission	*Amendment*
	Pursuant to article 6(2), the marketing authorisation applicant shall include patient experience data in the marketing application dossier. Where this is not possible, the marketing authorisation applicant should provide a detailed justification to the Agency. ***The Agency, in collaboration with patient and healthcare professionals organisations, competent authorities of the Member States and other relevant parties, shall draw up guidance to design, conduct, analyse, and report relevant studies incorporating robust***

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	and meaningful patient experience data for regulatory submission.

Justification: Authorised medicines must respond to patients' needs. Patient-relevant outcomes should be included in the marketing authorisation dossier as patient experience data, which would oblige pharmaceutical companies to genuinely involve patients and their organisations/representatives from the start of development and in the design of research, and to fully integrate the parameters that matter most to patients into their clinical development plans. Unfortunately, meaningful patient involvement is far from being a reality. Many clinical trials still do not include outcomes that matter to patients, such as quality of life indicators. An independent assessment of medicines approved in Europe in 2021 has also shown that only 17 of the 108 new marketing authorisations represent a major or significant therapeutic advance for patients. Additional guidance is needed from the Agency regarding methods and approaches to be used in capturing and measuring patients' experiences and perspectives and clarifying how to integrate this data into regulatory decision-making.

Amendment 11 (Directive)

Article 37 – Coordination group for decentralised and mutual recognition procedures

Paragraph 2

Text proposed by the Commission	*Amendment*
2. The coordination group shall be composed of one representative per Member State appointed for a renewable period of three years. Member States may appoint an alternate for a renewable period of three years. Members of the coordination group may arrange to be accompanied by experts. [..]	2. The coordination group shall be composed of one representative per Member State ***and one representative from patient organisations*** appointed for a renewable period of three years. Member States and patients may appoint an alternate for a renewable period of three years. Members of the coordination group may arrange to be accompanied by experts. [..]

Justification: Patient organisations, especially those operating at the EU level, have practical knowledge of medicines requiring harmonised product information as the patients they represent may receive care in different Member States, or live in a different Member State that the one that provides for their care. Therefore, representatives from patients' organisations would bring the legitimate user and patient added value for the Coordination Group. They will further ensure that patients are involved in all parts of the regulatory cycle of medicines that have not been centrally approved, beyond being ad hoc experts.

Amendment 12 (Directive)

Article 57 – Responsibility to report on public financial support

Text Proposed by the Commission	*Amendment*
1.The marketing authorisation holder shall declare to the public any direct financial support received from any public authority or a publicly funded body, in relation to any activities for the research and development of medicinal product covered by a national or centralised marketing authorisation, irrespective of the legal entity that received that support.	1. The marketing authorisation holder shall declare to the public any direct **and indirect** financial support received from any public authority or a publicly funded body, in relation to any activities for the research and development of medicinal product covered by a national or centralised marketing authorisation, irrespective of the legal entity that received that support.

Justification: Transparency is needed to understand better costs of R&D and by asking both the direct and indirect public funds it will be possible to properly create a framework that boost innovation while ensuring that medicines are affordable across Europe.

Amendment 13 (Directive)

Article 63 – Paragraph 3: General principles on package leaflet

Text proposed by the European Commission	*Amendment*
Article 63 (3) Member States may decide that the package leaflet shall be made available in paper format or electronically, or both. In the absence of such specific rules in a Member State, a package leaflet in paper format shall be included in the packaging of a medicinal product. If the package leaflet is only made available electronically, the patient's right to a printed copy of the package leaflet should be guaranteed upon request and free of charge and it should be ensured that the information in digital format is easily accessible to all patients.	*Article 63* *(3)* Member States may decide that the package leaflet shall be made available ~~in paper format or~~ electronically***, complementary to the mandatory paper format.*** ~~*In the absence of such specific rules in a Member State, a package leaflet in paper format shall be included in the packaging of a medicinal product. If the package leaflet is only made available electronically, the patient's right to a printed copy of the package leaflet should be guaranteed upon request and free of charge and it should be ensured that the information in digital format is easily accessible to all patients.*~~

Justification: The ELHG supports the digital health transformation and the use of digital tools in health and care. In the case of medicines, electronic product information will allow for personalised information, warnings, easier reporting of adverse effects, and to make the European market more flexible to react against shortages. However, the electronic package leaflets should complement, and not replace the paper package leaflets. The access to digital tools, internet, and digital literacy levels vary greatly within the European Union, and it is especially problematic in more rural areas, as well as when it comes to people over the age of 40. Therefore, relying on only on an electronic version of the

package leaflet and placing the burden of accessing crucial information on patients to look for it or to request it at the time of purchase, can have serious unattended consequences on the access to safety information.

Amendment 14 (Directive)

Article 63 – Paragraph 5 : General principles on package leaflet

Text proposed by the European Commission	*Amendment*
Article 63 (5) The Commission is empowered to adopt delegated acts in accordance with Article 215 to amend paragraph 3 by making mandatory the electronic version of the package leaflet. That delegated act shall also establish the patient's right to a printed copy of the package leaflet upon request and free of charge. The delegation of powers shall apply as of [OP please insert the date = five years following 18 months after the date of entering into force of this Directive].	*Article 63* (5) The Commission is empowered to adopt delegated acts in accordance with Article 215 to amend paragraph 3 by making mandatory the electronic version of the package leaflet. ~~*That delegated act shall also establish the patient's right to a printed copy of the package leaflet upon request and free of charge*~~ ***The electronic version shall complement the mandatory paper package leaflet.*** The delegation of powers shall apply as of [OP please insert the date = five years following 18 months after the date of entering into force of this Directive].

Justification: See justification Amendment 13.

Amendment 15 (Directive)

Article 69 – Paragraph 2: Special information requirements for antimicrobials

Text proposed by the Commission	*Amendment*
2. The marketing authorisation holder shall include in the packaging of antimicrobials a document that contains specific information about the medicinal product concerned and that is made available to the patient in addition to the product leaflet ("awareness card") with information on antimicrobial resistance and the appropriate use and disposal of antimicrobials. Member States may decide that the awareness card shall be made available in paper format or electronically, or both. In the absence of such	2. *The marketing authorisation holder shall include in the packaging of antimicrobials a document that contains specific information about the medicinal product concerned and that is made available to the patient in addition to the product leaflet ("awareness card") with information on antimicrobial resistance and the appropriate use and disposal of antimicrobials. Member States may decide that the awareness card shall be made available in paper format* ***and*** ~~***or***~~ *electronically*~~*, or both*~~*. In the absence of*

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specific rules in a Member State, an awareness card in paper format shall be included in the packaging of an antimicrobial.	~~*such specific rules in a Member State, an awareness card in paper format shall be included in the packaging of an antimicrobial.*~~

Justification: In the same way as the package leaflet, the awareness card should be available on paper for people who do not have access to the internet or who have a low level of digital literacy, to ensure equity.

Amendment 16 (Directive)

Article 81 – Paragraph 2 – point c: Regulatory data protection periods

Text proposed by the Commission	*Amendment*
(c) six months, for medicinal products containing a new active substance, where the clinical trials supporting the initial marketing authorisation application use a relevant and evidence-based comparator in accordance with scientific advice provided by the Agency;	(c) *six months, for medicinal products containing a new active substance, where the clinical trials supporting the initial marketing authorisation application use a relevant and evidence-based comparator in accordance with scientific advice provided by the Agency* ***in consultation with health technology assessment authorities, set out in a delegated act in accordance with Article 215***

Justification: The incentive for using comparative clinical trial data should encourage alignment with data requirements in health technology assessment (HTA) processes, helping national authorities to better assess the cost-effectiveness of new medicines and reduce access time in the patient journey. The evidence and data requirements are different between the marketing authorisation, the HTA bodies and the payers. The scientific evidence provided to obtain marketing authorisation is often considered insufficient by HTA agency assessors, which creates delays and inefficiencies.

Amendment 17 (Directive)

Article 83 – Medicinal products addressing an unmet medical need

Text proposed by the European Commission	*Amendment*
1. A medicinal product shall be considered as addressing an unmet medical need if at least one of its therapeutic indications relates to a life threatening or severely debilitating disease and the following conditions are met: (a) there is no medicinal product authorised in the Union for such disease, or, where despite medicinal products being authorised for such disease in the Union, the disease is associated with a remaining high morbidity or mortality; (b) the use of the medicinal product results in a meaningful reduction in disease morbidity or mortality for the relevant patient population	1. A medicinal product shall be considered as addressing an unmet medical need if at least one of its therapeutic indications relates to a life threatening**,** or severely debilitating disease and the following conditions are met: (a) there is no medicinal product authorised in the Union for such disease, or, where despite medicinal products being authorised for such disease in the Union, the disease is associated with a remaining high morbidity or mortality. (b) the use of the medicinal product results in a meaningful reduction in disease morbidity ~~or~~ , mortality ***or disease severity*** for the relevant patient population;

Justification: To drive medical innovation further than "just" addressing the mortality and morbidity associated with a disease, closely linked to medical criteria, it is important to also link this definition to unmet patients' needs, such as the disease severity and the disease burden, which is considerably high in patients with chronic diseases on patients and their carers. Moreover, the patient experience data (PED), which refers to patients' health status, symptoms, disease course, treatment preferences, quality of life and impact of health care,[1] should be better defined, collected, and embedded into the regulatory process, as they can provide with breakthrough perspectives when assessing the UNM.

Amendment 18 (Directive)

Article 83 – paragraph 2a (new)

Text proposed by the Commission	*Amendment*
	2a. The Agency shall, in consultation with patient organisations, healthcare professionals for all relevant age groups, and other relevant stakeholders, draw up the

[1] EMA, Patient experience data in EU medicines development and regulatory decision-making, September 2022, available at https://www.ema.europa.eu/en/documents/other/executive-summary-patient-experience-dataeu-medicines-development-regulatory-decision-making_en.pdf.

	definition of unmet medical need referred to in paragraph 1.

Justification: It will be of utmost importance to define the concept of "unmet medical need" in cooperation with patients, in particular to avoid the "orphanisation" of new medicines, where more and more drugs are labelled as "unmet medical need" only to apply for accelerated approval and justify higher prices. Unmet medical need is not always a yes/no question. A relevant definition should include criteria that are crucial to patients, such as disease severity, disease burden and of quality of life. Healthcare professionals for patients of all age groups, including for paediatric, adult, and older population care should be included to ensure the inclusion of a wider expertise for all patients needed.

Amendment 19 (Directive)

Article 105 – paragraph 2: Recording and reporting of suspected adverse reactions by the marketing authorisation holder

Text proposed by the Commission	*Amendment*
2. Marketing authorisation holders shall not refuse to consider reports of suspected adverse reactions received electronically or by any other appropriate means from patients or healthcare professionals.	2. Marketing authorisation holders shall not refuse to consider reports of suspected adverse reactions received electronically or by any other appropriate means from patients, ***carers*** or healthcare professionals.

Justification: Carers play an important role in the healthcare ecosystem, and they can provide with valuable insights into the potential adverse effects of medicines, especially when patients are not able to represent and report on these findings by themselves. As such, carers should be involved in the consultation process by having their findings and reports on the suspected adverse reactions taken into account by marketing authorisation holders.

Amendment 20 (Directive)

Annex VI – paragraph 2 a (new): Contents of package leaflet

Text proposed by the Commission	*Amendment*
	(2a) a key information section reflecting the results of consultations with target patients' organisations to ensure that it is legible, clear and easy to use;

Justification: The inclusion of a key information section in the package leaflet, drafted in collaboration with patients, would allow patients and healthcare professionals to quickly identify key safety messages with information on the benefits of the medicines. According to a study[15] commissioned by the Commission, patients' understanding of the package leaflet and its legibility could be improved,

while the summary of product characteristics is less problematic. The elderly and people with low literacy skills are particularly disadvantaged, but in general, these problems affect all patients.

Amendment 21 (Directive)

Annex VI – paragraph 9 (new): Contents of package leaflet

Contents of package leaflet *Text proposed by the European Commission*	*Amendment* ***(9) information about the environmental impact of the medicinal product, including the environmental footprint of manufacturing, and ways in which the product should be deposited and disposed of.***

Justification: The environmental impact and climate change footprint of medicines is of great concern to patients. The outcomes of the ERA and the overall environmental impact of medicines should not only be limited to the application dossier. Patients should have easy access to information on how to dispose of the product, and about the environmental footprint of its manufacturing. This would increase awareness of products that directly contribute to AMR, pollution or global warming. Many patients are not able to play a full role in their care and the environmental impact of their treatments, while they represent a subpopulation uniquely vulnerable to environmental disruptions. Medicines should not be a source of pollution, and every effort should be made to minimise their environmental impact without compromising patients' access to the medicines they need.

3. Proposed Regulation 2023/0131 on procedures for the authorisation and supervision of medicinal products for human use and rules governing the European Medicines Agency

Amendment 22 (Regulation)

Recital 77

Text Proposed by the Commission:	*Amendment:*
The development of antimicrobial resistance is a growing concern, and the pipeline of effective antimicrobials is obstructed due to a market failure; it is therefore necessary to consider new measures to promote the development of priority antimicrobials that are effective against antimicrobial resistance and to support undertakings, often SMEs, which choose to invest in this area.	The development of antimicrobial resistance is a growing concern, and the pipeline of effective antimicrobials is obstructed due to a market failure; it is therefore necessary to consider new measures to promote the development of priority antimicrobials that are effective against antimicrobial resistance and to support undertakings, often SMEs, which choose to invest in this area***. Moreover, all strategies***

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	shall be considered in the battle against AMR, as there is a necessity of innovation in non-antimicrobial strategies as an alternative to manage infections.

Justification: Without negating the importance of research into new antimicrobials, there is also a necessity of innovation in non-antimicrobial strategies as an alternative to manage infections. Antimicrobials always face the risk of resistance developing and with AMR rising globally.

Amendment 23 (Regulation)

Article 95 - European Network

Text proposed by the Commission	*Amendment*
The Agency shall develop a European network of patient representatives, academics, medicines developers, investigators and centres with expertise in the performance of studies in the paediatric population.	The Agency shall develop a European network of patient representatives, academics, medicines developers, investigators, ***healthcare professionals for all relevant age groups,*** and centres with expertise in the performance of studies in the paediatric population.

Justification: Healthcare professionals should be included as they are key actors in the care and treatment of patients. Their expertise is an added value to the decision-making process. Healthcare professionals for patients of all age groups, including for paediatric, adult, and older population care should be included to ensure the inclusion of a wider expertise for all patients needed.

Amendment 24 (Regulation)

Article 121 - Paragraph (b): Role of the competent authority of the Member State

Text *proposed by the European Commission*	*Amendment*
(b) publish information on actual shortages of medicinal products, in cases in which that competent authority has assessed the shortage, on a publicly available website;	(b) publish information on ***expected and*** actual shortages of medicinal products, ***the causes and measures planned as soon as the*** competent authority has assessed the shortage, ***on a publicly available website. Moreover, clear recommendations and possible alternatives should be provided and (easily accessible) to healthcare professionals and patients.***

Justification: It is of great importance that all the stakeholders (i.e healthcare professionals, patients, member states) can access the correct information to be able to prevent as well as react quickly to shortages.

Amendment 25 (Regulation)

Article 121 – Paragraph 1 – point b (a) – new: Role of competent authority of the Member State

Text proposed by the Commission	*Amendment* ***(ba). Establish an accessible and easily understandable system for patients, patient organisations and healthcare professionals to report shortages of medicinal products;***

Justification: The involvement of patients and their representatives in the development of policy solutions is essential, as they are the main actors at the end of the supply chain. Together with the healthcare professionals, they are able to provide information on the shortages to the authorities and provide information on the alternatives. Enabling patients and healthcare professionals to report on drug shortages will improve the collection of data and the understanding of their societal impact, thus improving drug shortage management.

Amendment 26 (Regulation)

Article 124 - Paragraph 3: Management of the critical shortage

Text *proposed by the European Commission*	*Amendment*
The Agency shall establish within its web-portal referred to in Article 104 a publicly available webpage that provides information on actual critical shortages of medicinal products in cases in which the Agency has assessed the shortage and has provided recommendations to healthcare professionals and patients. This webpage shall also provide references to the lists of actual shortages published by the competent authorities of the Member State pursuant to Article 121(1), point (b).	The Agency shall establish within its web-portal referred to in Article 104 a publicly available ***and user friendly*** webpage that provides information on **all** critical shortages of medicinal products, ***including the reasons for the shortages. After assessing the shortages,*** the Agency ***shall provide*** recommendations to healthcare professionals and patients. This webpage shall also provide references to the lists of actual shortages published by the competent authorities of the Member State pursuant to Article 121(1), point (b).

Justification: Healthcare professionals and patients should have easy access to understandable information to react appropriately to medicine shortages, following the recommendations of the agency.

Amendment 27 (Regulation)

Article 125 - Paragraph (f): Obligations on the marketing authorisation holder in case of a critical shortage

Text *proposed by the European Commission*	*Amendment*
(f)inform the Agency of the end date of the critical shortage.	(f) inform the Agency of the end date of the critical shortage ***in a timely manner.***

Justification: Time is a key factor to react in case of emergency. The Agency need to have all the information related to actual shortages in order to inform and guide both patients and healthcare professionals.

Amendment 28 (Regulation)

Article 146 Paragraph 4a (new): – Scientific committees – general provisions

Text proposed by the Commission	*Amendment* ***4a. patients' organisations representatives appointed as members and/or alternate members on scientific committees shall be remunerated through the Agency's budget, in accordance with the financial rules applicable to the Agency.***

Justification: The involvement of patients in the regulatory life cycle requires training, availability, personal commitment and sustainability. This is why patients' time and efforts spent in preparing, travelling for, and attending Agency meetings deserves reasonable and sustained compensation from the EU budget. Patients and their representatives are scarce and mostly operate on a volunteer basis, devoting considerable amount of time to the work of the EMA scientific committees, often up to three days a month, without possibility of backup due to the confidential nature of the work and the personal nomination. It is therefore paramount that patients and their representatives in the EMA committees are compensated to ensure their structural participation and reinforce their independence and accountability.

Amendment 29 (Regulation)

Article 142 (New): Administrative and management structure

Text proposed by the Commission	*Amendment* ***(fa) the Paediatric Committee;***

Justification: The disappearance of the Paediatric Committee (PDCO)) and the subsequent lack of an appropriate forum to discuss and assess paediatric investigation plans (PIPs), may lead to an unintended deprioritisation of research and development on paediatric medicines and less paediatric marketing authorisation applications (MAAs). The PDCO acts as an independent third party with broad representation and considerable oversight. Without this expertise, it is unclear whether the EMA will have the influence and resources needed to push for specific studies, particularly in the most underserved and complex populations, such as neonates. In addition, PDCO experts have gained considerable expertise and experience in paediatric diseases, research and medicines, as well as in the assessment of paediatric development plans. Without specific committees to address the paediatric, this expertise will likely be lost, as Member State experts will be assigned to different tasks and topics.

Amendment 30 (Regulation)

Article 142 (New): Administrative and management structure

Text proposed by the Commission	*Amendment* ***(fb) the Committee for Orphan Medicinal Products;***

Justification: Similar to the disappearance of the PDCO, the disappearance of the Committee for Orphan Medicinal Products (COMP) may lead to the deprioritisation of research and development for rare diseases, an area that already lacks incremental and breakthrough therapeutic innovations, as 95% of over 6000 recognised rare diseases still don't have treatment options.[2] The expertise of the COMP has been imperative in prioritising developments for rare diseases, and the lack of such a forum will lead to less involvement of patients in the early stages of the development process.

Amendment 31 (Regulation)

Article 150—Paragraph 3: Scientific working parties and scientific advisory groups

[2] 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.

Text proposed by the Commission	*Amendment*
3. The composition of the working party and the selection of members shall be based on the following criteria: (a) a high level of scientific expertise; (b) meeting the needs for the specific multi-disciplinary expertise of the working party to which they will be appointed. The majority of the members of the working parties shall consist of experts from the competent authorities of the Member States. Where appropriate, the Committee for Human Medicinal Products may, following consultation with the Management Board, set a minimum number of experts from the competent authorities in a working party.	3. The composition of the working party and the selection of members shall be based on the following criteria: (a) a high level of scientific expertise ***or being representative of patients and healthcare professionals;*** (b) meeting the needs for the specific multi-disciplinary expertise of the working party to which they will be appointed. The majority of the members of the working parties shall consist of experts from the competent authorities of the Member States ***and patient representatives***. Where appropriate, the Committee for Human Medicinal Products may, following consultation with the Management Board, set a minimum number of experts from the competent authorities in a working party.

Justification: The specialised committees on Orphan Medicinal Products and the Paediatric Committees have invaluable expertise in advancing the work on orphan and paediatric needs. As such, the expertise of patients involved in these committees must be retained in the newly proposed EMA structure. It is of utmost importance that the participation in the scientific working parties set up by the CHMP of patients, as well as healthcare professionals, is ensured. The participation of these stakeholders can bring immense value to the discussions.

Amendment 323 (Regulation)

Article 152 – paragraph 1 – Rapporteurship

Text proposed by the Commission	*Amendment*
1. Where, in accordance with this Regulation, any of the Committees referred to in Article 142 is required to evaluate a medicinal product for human use, it shall appoint one of its members to act as rapporteur, taking into account existing expertise in the Member State. The	1.Where, in accordance with this Regulation, any of the Committees referred to in Article 142 is required to evaluate a medicinal product for human use, it shall appoint one of its members to act as rapporteur, taking into account existing expertise in the Member State. The

Committee concerned may appoint a second member to act as co-rapporteur.	Committee concerned may appoint a second member to act as co-rapporteur, ***who may be a member representing patient organisations or a member representing healthcare professionals.***

Justification: Within the current legislation, patient representatives and healthcare professionals' representatives are not allowed to become co-rapporteurs in the evaluation of marketing authorisation applicants. Given patient representatives provide valuable expertise based on lived experience and a practical understanding of the research and development of new medicines, some national competent authorities may want to work with patients and healthcare professionals as co-rapporteurs on specific cases.

Amendment 33(Regulation)

Article 162 - Paragraph 2: Consultation process

Text *proposed by the European Commission*	*Amendment*
The Agency may extend the consultation process to patients, medicines developers, healthcare professionals, industries or other stakeholders, as relevant	The Agency ~~*may*~~ ***shall*** extend the consultation process to patients, medicines developers, healthcare professionals ***for all relevant age groups,*** industries or other stakeholders as relevant.

Justification: The experience of all the stakeholders in the healthcare sector will be key to develop and implement the legislation in a swift and appropriate way, especially to address potential issues and bottlenecks through the whole process. Health care professionals for patients of all age groups, including for paediatric, adult, and older population care should be included to ensure the inclusion of a wider expertise for all patients needed.