Biomedical Alliance in Europe

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Revision of the EU pharmaceutical legislation must find the right balance between access to medicines & sustainable reform

European medical societies call on EU policy makers to find the right balance in their review of the EU pharmaceutical legislation. As trilogue negotiations on the package of proposals begin, healthcare professionals hope that decision makers will reach an agreement that improves equitable patient access to medicines, responds to unmet medical needs and the needs of patient groups-including paediatric patients, whilst simultaneously fostering European innovation and improving the sustainability of EU healthcare systems.

The community of 35 medical societies represented by the BioMed Alliance has followed discussions on the reform of the EU pharmaceutical legislation, which entails one of the most comprehensive reforms of the sector in over two decades.

The BioMed Alliance highlights the importance of creating legislation that is both fit for purpose *i.e.*, enhances availability, affordability and equitable access to medicinal products across the EU, and future-proof. We believe that both the Commission proposal, the Parliament position, and the Council position contain important elements that must be considered in the final text of the legislation.

Defining unmet medical needs

The definition of unmet medical needs in the proposed Article 83 of directive 2023/0132(COD) requires more clarification and should include factors impacting patient health, such as quality of life, patient reported outcomes, and availability of alternative treatments. Part of this is addressed in the Parliament's newly proposed recital 50a, by clarifying what is understood by 'morbidity' but we believe this definition should be expanded and moved to article itself 83. In the Council text, the wording is changed to include a reference to 'a clinically relevant improvement in efficacy', but this is also not sufficiently detailed, and it is essential that issues related to quality of life are specifically mentioned. Recital 50 refers to the development of scientific guidelines that will specify clinical improvement and efficacy, but we reiterate that healthcare professionals must be involved in the development of such guidelines.

The definition of unmet medical needs should go beyond reduction in disease morbidity and mortality and should be flexible to account for societal changes. The COVID-19 pandemic, for example, underscored the critical importance of addressing not only immediate health crises but also the broader societal impacts of such events. This includes the strain on healthcare systems, the rise of mental health challenges, and the exacerbation of socioeconomic inequalities. The definition should emphasise the added clinical value of the proposed medicine and ensure that it effectively addresses persisting health threats such as chronic, progressive diseases. It is also essential for the definition to be broad enough to remain relevant and adaptable to future medical advances and emerging health

challenges. To achieve this, it should be developed in close collaboration with patients and healthcare professionals in order to better reflect the needs of patients.

Key parameters that should be included in the definition:

- Impact on the quality of life of the patient;
- Impact on disease severity, progression and duration;
- Availability of alternative treatments, as the standard of care is often not available or inaccessible to patients;
- Burden of illness on the patient, in terms of physical, psychological and social health, but also the burden on society and the healthcare system;
- With respect to paediatric patients, it should also consider negative implications on long-term development and maturation;

These parameters should be specified in the scientific guidelines, to be developed by EMA, to ensure that they are future-proof and grounded in scientific reality. Patient reported outcomes (PRO) and patient experience data (PED) must be considered.

Involving healthcare professionals and patients in the application

The consultation process set out in Article 162 of the proposed Regulation (2023/0131 COD) states that patients and healthcare professionals can be consulted at the Agency's discretion. The BioMed Alliances urges decision-makers to make input from health professionals and patients mandatory in the formulation of the guidelines foreseen by Article 83.3. This is also common practice in other regulatory frameworks, such as in the medical devices system where stakeholders were involved in the development of guidance on the Clinical evaluation of orphan medical devices through participation in an Orphan Devices MDCG sub-group. The insights of clinicians and patients – from across the EU, to account for regional differences – are crucial to ensure a framework that is viable and fit-for-purpose. In addition, a future proof UMN framework will require periodic re-evaluations of the applicable parameters, in order to keep up with clinical and patient needs.

In this context, it is essential that the final text will preserve the Parliament's addition in article 83.3 requiring the European Medicines Agency (EMA) to engage with stakeholders when developing guidance to implement this article and to strengthen the wording by changing 'may extend the consultation process' to 'shall consult'. This consultation will play a crucial role in defining ambiguous but critical criteria such as "high morbidity/mortality" and "meaningful reduction" in patient burden. Strengthening the involvement of stakeholders such as healthcare professionals in this process ensures that the guidance adequately reflects real-world medical needs and provides a robust foundation for addressing unmet medical needs effectively, we therefore believe a stronger wording is essential.

In addition, we believe that the voting rights of healthcare professional and patient representatives in the Committee for Medicinal Products for Human Use must be maintained, and that provision in the Council position on regulation 2023/0131 (COD) article 148 (4a) should be scrapped. Healthcare professionals work directly with medicines and see the concrete impact on patient care, and their voices must be heard. Similarly, voting rights for healthcare professionals and patients should be maintained in the work of the Committee for Medicinal Products for Human Use as they represent

key actors in the health sector. We therefore do not support the Council's amendment to restrict the voting rights of these groups in article 148 4a of the regulation.

Medicine shortages

Medicine shortages are a key issue that healthcare professionals and their patients face across Europe and collective effort and investment are necessary to prevent and address them. Therefore, BioMed Alliance strongly supports measures preventing and limiting the impact of shortages on patients' health outcomes:

- **Transfer of Marketing Authorisation**: Requiring marketing authorisation holders to transfer their marketing authorisation in the event of an intended permanent withdrawal (Article 24, 2023/0131 COD). We have noted that the Council position intends to scrap this provision, and we hope that negotiators in the trilogue will agree to maintain the provision proposed by the Commission in the final text of the regulation.
- **Advance Notification for Cessation or Suspension**: Requiring that market authorisation holders (MAHs) notify authorities at least 12 months prior to a permanent marketing cessation or at least 6 months before a temporary suspension. This is an important measure to safeguard the continuous supply of medicines (Article 116, 2023/0131 COD).
- **Shortage Prevention Plans**: Requiring marketing authorisation holders to prepare and regularly update a shortage prevention plan for all medicinal products they market. Additionally, the inclusion of the Healthcare Professionals' Working Party (HPWP) and the Patients' and Consumers' Working Party (PCWP) in developing guidance for Marketing Authorisation Holders on creating these plans is a welcomed provision in the Parliament text (Article 117, 2023/0131 COD), which should be maintained in trilogue and included in the final text of the regulation. We do not support the Council's proposal to narrow the scope only to critical medicine products, in its amendment to article 117 of the Regulation. All medicines should benefit from the plans, and input from the EMA's HCPWP should be taken into account.
- **Definition of demand:** We strongly support the Parliament's amendment providing a definition of 'demand', which specifies when 'demand is satisfactorily met' (Article 2.14a, 2023/0131 COD). This is an important clarification to ensure the continuity of patient care.

Furthermore, as mentioned in the proposed regulation 2023/0131(COD), in order to enhance preparedness critical medicines and alternatives need to be identified, and shortage prevention plans must be in place. A collaborative environment is necessary where industry, competent authorities, Health Emergency Preparedness and Response Authority (HERA), European Medicine Agency (EMA), and healthcare professionals work together, ensuring open and prompt communication. It is essential that healthcare professionals are informed in a timely matter of upcoming shortages, enabling them to consult with their patients to determine the best course of action for continuing treatment.

The expansion of HERA's role

The revised proposal from the European Parliament for regulation 2023/0131(COD) suggests a slightly more extensive role for the Health Emergency Preparedness and Response Authority (HERA) under the European Centre for Disease Prevention and Control (ECDC) in the new article 11a. HERA will be granted more responsibility for creating, coordinating and implementing a long-term European biomedical research and development agenda regarding medical countermeasures against current and emerging public health threats. The Alliance is supportive of increasing HERA's role in research as

this may enhance coordination in research and strengthen health emergency preparedness. We therefore believe it will be beneficial to maintain the Parliament's amendment in the final text. Additionally, as highlighted during negotiations on Regulation (EU) 2022/2370, the ECDC's mandate could be further extended to comprehend major non-communicable diseases, enabling the collection of vital epidemiological data to inform research and policymaking.

Repurposing of medicines

The repurposing of medicines offers a sustainable, safe, innovative, and efficient way to improve access to treatments and address unmet medical needs. Drug repurposing can turn academic research into patient benefits at a fraction of the cost of developing new drugs. It plays a crucial role in addressing unmet medical needs, particularly for rare diseases, where 95% of the 7,000–8,000 identified conditions lack approved treatments in Europe[1]. The new regulatory framework should incentivise and facilitate this process by establishing pathways to approve new indication for authorised medicines already on the market. In the Commission proposal for the Regulation, there is a provision allowing not-for-profits to lead in research efforts in the use of existing drugs, which is a positive step in the right direction. We therefore welcome the European Parliament's amendment to extend the scope of article 48, 2023/0131 COD; as opposed to the Council amendments to this article might reduce the scope only to Unmet Medical Needs. Drug repurposing must not be confined to Unmet Medical Needs in order to broaden access to treatments and ultimately maximise benefits for patients.

Funding transparency

The BioMed Alliance acknowledges that the affordability and fair pricing of medicines is essential for patient access. There are significant disparities among EU countries regarding health budgets, reimbursement decisions, and, consequently, access to medicines. Understanding the costs associated with research and development, as well as how these costs have been shouldered, is crucial for ensuring transparency, fairness, and strengthening the pricing negotiating power of EU Member States. However, the wording of Article 57 in the proposed directive 2023/0132 COD is inadequate to achieve these goals. Marketing authorisation holders (MAHs) should disclose any direct or indirect funding (including tax incentives) received from public authorities, publicly funded bodies, NGOs and/or charities. Additionally, we support the European Parliament's amendments requiring Marketing Authorisation Holders to disclose the full funding history of medicinal products, regardless of geographic origin, as well as any funding associated with acquired medicinal products. A clearer funding history will empower Member States to negotiate fairer drug prices for their markets, and we therefore believe the Parliament 's amendments are preferable to the original Commission proposal and the Council text.

Conflict of Interests

The BioMed Alliance supports the requirement for regulators and experts involved in the authorisation and surveillance of medicinal products to declare their interests and update them. We emphasise that conflict of interest must be appropriately declared and managed, but we must take a balanced approach considering the realities of a sector where high-level experts are often engaged in

[1] Fetro C. Connecting academia and industry for innovative drug repurposing in rare diseases: it is worth a try. Rare Dis Orphan Drugs J. 2023;2:7. page 2. http://dx.doi.org/10.20517/rdodj.2023.06

multiple initiatives involving a wide range of stakeholders. The stricter wording in Article 208 (2023/0132 COD) of the European Parliament's position on the directive may hinder the availability of competent experts, particularly in the field of rare diseases, where the smaller pools of experts are often much sought-after. A balanced approach ensures the quality and relevance of regulatory processes, including scientific advice, safety and efficacy evaluations, and clinical effectiveness assessments.

Incentives

The pharmaceutical legislation should support robust research and innovation in Europe as more investment is needed to tackle current and future health needs. A balanced approach is necessary to facilitate the development of innovative medicines while limiting the impact on national healthcare expenditure. Specifically, the BioMed Alliance supports Article 81.2ca (2023/0132 COD) introduced by the European Parliament granting an additional 12 months of regulatory market protection if efficacy trials have been conducted in more than one Member State. Localised research and development can help build capacity in the EU to conduct clinical trials for new treatments. This would ensure that patients in the EU can participate in clinical trials and access the latest treatments as soon as possible[2].

Incentives are crucial when developing innovative new medicines including new antibiotics, as antimicrobial resistance (AMR) is one of the greatest global public health threats. AMR also threatens the safety of crucial medical procedures such as surgeries and cancer chemotherapy but also impacts national health systems by creating a need for prolonged hospitalisation and intensive care. While the proposed transferable exclusivity vouchers could incentivise the development of new antibiotics, they also risk further burdening national healthcare expenditure. Additionally, we should continue to optimise the treatment with existing antibiotics through additional research as well as training and guidance for clinicians. A balanced approach with a variety of push and pull initiatives and extra support for collaborative translational research are essential tools to drive the search for new antibiotics. Furthermore, incentives such as regulatory data protection periods for innovative medicinal products are useful but need to be combined with a broad set of measures to encourage pharmaceutical companies to conduct their development and manufacturing of pharmaceuticals in Europe.

Improving access

The BioMed Alliance welcomes the new provision introduced by the European Parliament in its position on the proposed Directive, on 'Reporting on access to medicinal products' (article 86a). Having evidence-based, measurable indicators for measuring access, as well as having a publicly available report assessing access and the barriers thereto, will be fundamental to ensure the value derived from innovation actually reaches the end users. Moreover, we welcome articles 58a and 58b of the EP's position on the Directive, creating an obligation to submit an application for pricing and reimbursement in all Member States (that have requested this) and introducing an EU access to medicines notification system.

Bolar Exemption

The delayed entry of generics and biosimilars on the market is a loss for patients and prejudicial to national health expenditures. Generics should be able to enter the market on 'Day 1' after the

[2] European Parliamentary Research Service, PE 697.197, 2021 pages 60-65.

expiration of the proprietary rights of the reference medicine. However, that can only happen if all the preparatory steps can be taken beforehand. The BioMed Alliance strongly supports the Parliament's proposal to extend the scope of article 85 of the directive, covering activities (including but not limited to studies and trials) conducted for the purpose of obtaining marketing authorisation, undergoing health technology assessment, and obtaining pricing and reimbursement approval. The absence or weakening of the bolar exemption creates a *de facto* extension of regulatory protection to the detriment of patients and their care.

Supporting research & innovation

Incentives alone will not be enough to create an environment that actively supports European research and innovation. A variety of measures is necessary, including enhanced coordination and support for biomedical research. The next Framework Programme for research must support innovative health research by increasing funding, simplifying access to funding, reducing administrative burdens, supporting bottom-up collaborative research, and strengthening coordination and agile strategic leadership. In addition, the EU must reduce regulatory complexity, including by limiting and clarifying regulatory overlap, and avoiding unnecessary bureaucracy.

In addition, in order to ensure groundbreaking and essential medical research will be able to take place, we must take a careful long-term approach to any phasing out of research using animals, only in areas where scientific advances allow this. The scientific sector is already reducing the number of animals used in research by refining animal models and developing alternative non-animal methods including *in vitro, ex vivo*, organoid and in silico models. However, in the current state of the art these non-animal models are not full-fledged alternatives that provide the same level of insights as animal studies, and this is unlikely to change in the foreseeable future. There were sufficient safeguards in the original Commission texts, and the additional wording introduced by the European Parliament on animal testing, for instance in recital 31 of the directive 2023/0132(COD) and recital 46 of regulation 2023/0131(COD) are repetitive and will not help scientific progress.

Paediatric medicines

Access to medicines for children is still more limited than for adults, as they need to be specifically developed and adapted to ensure safety and optimised quality treatment for their health needs. The Paediatric Committee (PDCO) within the European Medicine Agency (EMA) has significantly increased the availability of paediatric medicines across Europe, which is why we strongly advocate for the continuation of the expert advisory group.

Children often have more limited or delayed access to medicines compared to adults, with potential lifelong consequences for physical and mental development. This vulnerable target group must be protected, by including the impact of the long-term age-appropriate development, growth and maturation of paediatric patients, and of retaining their normal daily activities, in the definition of Unmet Medical Needs.

Furthermore, support for patient registries and other sources for real-world evidence should receive adequate funding and support as they provide valuable information on the safety and efficacy of medicines. They are of particular benefit for paediatric medicines, that are often associated with a more limited availability of data, complicating their market authorisation and surveillance.

Conclusion

While supportive of the overall goals of the legislation, there are still crucial issues in the EU health sector that need to be addressed. A balanced approach of push and pull incentives, and support for academic research and translation, are needed to generate better outcomes for patients. Ultimately, the BioMed Alliance calls for specific enhancements to address existing gaps and fully realise the potential benefits of the reform for patients and the medical community. Equitable access should be at the heart of the legislation, and its main outcome.