EPF proposal for a patient-centred framework for defining Unmet Medical Needs

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BACKGROUND AND KEY CONSIDERATIONS

Article 83 of the European Commission proposal for a Directive on the Union code relating to medicinal products for human use proposes a definition of "unmet medical need". The definition will drive pharmaceutical companies' eligibility for an additional 6-month regulatory data protection, conditional marketing authorisation, and enhanced scientific and regulatory support. Its implications will also go beyond Research & Development (R&D) and will likely affect the post-authorisation phase and health technology assessment (HTA). While there is a need for a coherent approach to the definition of unmet medical need throughout the medicines' lifecycle, making sure it reflects patients' needs is all the more important.

EPF believes that the definition included in the Commission proposal is unnecessarily restrictive and inadequate to achieve better health innovation in the EU. In addition, we regret the lack of patient involvement in the development of this definition which will be enshrined in EU law and of mandatory patient participation in the adoption of scientific guidelines for its application (art. 83(3)). By only considering morbidity and mortality as indicators of unmet medical need, the EU would ignore other important life-changing indicators for which significant therapeutic improvements or even breakthroughs may be needed from a patient perspective, such as the appropriateness of existing treatments or impacts on quality of life.

The term "unmet **medical** need" itself seems inappropriate, when in fact **"**unmet **patients'** needs**" –** defined *by* and *with* patients **– should be the main driver of pharmaceutical R&D.** This legislative review is **a unique opportunity to encourage more needs-driven and patient-centred healthcare systems.**

It is important to highlight the nuance between the concepts of "*high* unmet medical need" and "unmet medical need". Firstly, it remains vital to stimulate innovation in rare diseases and drive investment into areas that companies would not consider commercially viable otherwise. Currently, an estimated 95% of rare disease patients do not have an approved therapy[i]; many rare diseases licensed treatments are not curative and only manage symptoms or slow down disease progression, leading to continued high morbidity and mortality rates. This constitutes, unquestionably, a *high* unmet medical need. At the same time, medicinal products that provide exceptional additional therapeutic benefits in non-orphan diseases that currently cannot be cured and are associated with high morbidity or mortality should also be considered as addressing high unmet medical needs.

The concept of "unmet medical need" however should aim **at distinguishing innovative medicines that bring real and significant added therapeutic value to patients from other "new" medicines**. EPF supports efforts to avoid misleading, non-patient centred "unmet medical need" claims that advertise niche benefits without actual impacts on patients' lives. The use of the concept should in fact contribute to better patient and healthcare stakeholder information about the true value of a product. At the same time, prioritising promising *medicinal products* should not translate into deprioritising certain *diseases*, with some patients considered as less "worthy" of innovation despite identified therapeutic needs.

Ultimately, a **framework for identifying "unmet medical needs" is a step towards defining "innovation" and this definition exercise cannot take place without patients' involvement.** EPF warns against a simplistic definition whose sole objective would be to justify short-term cost-containment measures at the expense of

patients' access to products that can truly improve their health outcomes and daily lives. Assessing whether a medicine fulfils an unmet medical need will require **case-by-case assessments** and **additional guidance** by the European Medicines Agency (EMA) to specify key criteria. In view of their unique expertise and experience of their condition and needs, we call **for meaningful patient involvement in this process**, including patient participation in decision making, as well as consultation of patient representatives in each specific disease area.

EPF PROPOSAL FOR AN INCLUSIVE FRAMEWORK FOR IDENTIFYING UNMET MEDICAL NEED

To develop this framework, EPF has consulted its members and relied on ongoing work in the field[ii]. EPF encourages the exchange of knowledge and best practices among member states, EU bodies, and patient organisations to support further reflections on the concept of unmet medical need and build on relevant, patient-centred initiatives. EPF believes that a future framework for the characterisation of "unmet medical need" in the context of the EU pharmaceutical legislation should:

- Apply to medicinal products intended to treat, but also medicinal products intended to prevent or diagnose a disease, or to restore, correct or modify physiological functions as per the **definition of medicinal products** in the EU[iii].
- Address **patients' immediate therapeutic needs**, defined as needs perceived by the patients that are not met by currently available treatments[iv], **and longer-term societal needs**, including impacts on carers and future public health threats.
- **Be inclusive** – many lifelong, chronic diseases are not or no longer considered as "life-threatening" but still represent a **significant burden** on patients, carers, and healthcare systems. At the individual level, the burden of disease refers to the impact of a disease on physical and psychosocial health[v]. It encompasses not only the mortality and morbidity of the disease, but takes into account the impacts of the disease on patients' lives and experience more broadly, including health related quality of life, which is very important for the patient community.
- Specifically, when assessing patients' therapeutic needs, the following criteria should be considered:
    - Impact of the condition on **life expectancy** after providing standard of care treatment
    - **Appropriateness of the current standard of care** for the patient, including considerations of:
        - Impact of the new product on one or several **serious outcome(s)** of the condition that is/are not or insufficiently addressed by the current standard of care
        - Impact of the new product on **disease onset, progression, and duration**, as well as **disease severity,** compared with the current standard of care
        - Impact of the new product on a specific **patient subpopulation** who fails to tolerate or to respond to the current standard of care
        - Inconvenience of current standard of care and **major improvements to patient care**, including when the new product addresses treatment compliance issues, leading in turn to significantly improved outcomes
        - **Lack of availability** of the current standard of care in one or several EU member states. Certain highly innovative medicines, such as gene therapies, are sometimes only available in very few countries due to their high price, leading to unacceptable inequalities. As a result, a product that has a clear potential to provide a more broadly available alternative to an existing product would also address an unmet medical need.

- Impact of the condition on patients as it relates to their experience and everyday lives after receiving standard of care treatment. This includes considerations of **quality of life,** recognising the multifactorial nature and current evolutions of the concept (e.g., mental health impacts).
    - In this context, EPF recalls **the importance of including patient experience data (PED),** beyond "traditional" clinical endpoints, **at all stages of medicines' development and regulatory decision-making**. PED are data collected to describe patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care[vi]. There is consensus among regulatory authorities and other healthcare stakeholders on the value of PED to inform the benefit/risk assessment and drive medicines' development[vii]. PED can play a crucial role in assessing whether and how a product addresses patients' unmet needs.

Listening to patients, through consideration of the impacts of their disease, related treatment or therapy on their lives and systematic patient involvement in decision-making, is a basic step in any attempt at developing a relevant and workable framework for the definition of unmet medical need.

About EPF

The European Patients' Forum (EPF) is an umbrella organisation of patient organisations across Europe and across disease areas. Our 79 members include disease-specific patient groups active at the EU level and national coalitions of patients representing 19 countries and an estimated 150 million patients across Europe. www.eu-patient.eu

[i] European Commission
[ii] In particular, the Belgian health insurance institute came up with a definition of unmet medical needs in the context of early access procedures, which takes into account a patient-centered approach. Belgium is also conducting a larger study to determine and measure unmet medical needs, the NEED project. INAMI/RIZIV « Besoin Médical non rencontré »
[iii] EMA glossary
[iv] Belgian Healthcare Knowledge Centre, "Identifying patient needs: Methodological Approach and Application", KCE report 348.
[v] Sciensano, Burden of disease. https://www.sciensano.be/en/health-topics/burden-disease#how-to-estimate-the-burden-of-disease
[vi] Executive summary - Patient experience data in EU medicines development and regulatory decision-making workshop (europa.eu)
[vii] See reference VIII and FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making | FDA