Cancer Patients Europe – Defining Unmet Medical Needs (UMN)

September 2025

On 4 June 2025, the Council of the EU agreed its position on the revision of the EU pharmaceutical legislation, clearing the way for final trilogue talks with the European Parliament and the European Commission. A central element of this reform is the creation of a new EU-wide definition of Unmet Medical Need (UMN). This definition will shape how incentives are granted, how research is prioritised, and how patients, especially those with cancer, gain timely access to innovation.

The idea of fixing a single legal definition of UMN raises concerns among the patient community. Needs are not static, they evolve with advances in science and technology, changes in disease progression, and patient experience. In order to address these concerns, the definition of UMN must be broad, flexible and inclusive of patient, medical and societal perspectives. This is critical for cancer patients: the definition will shape the entire lifecycle of medicines from research priorities and drug development to pricing and reimbursement decisions.

The Proposed Definitions of UMN falls short: A proper definition of UMN plays a fundamental role in ensuring innovation and access. While the goal is to encourage development in areas of high need, current proposals risk the opposite—limiting innovation and delaying access for patients.

- **European Commission** - Very narrow scope: restricted to life-threatening or seriously debilitating diseases, with new products required to show a meaningful reduction in mortality/morbidity over existing treatments. This increases uncertainty, deterring R&D investment in the EU.
- **European Council** - also narrow, focusing on life-threatening or seriously debilitating diseases, with UMN limited to situations where no authorised product exists or where the product must demonstrate clinically relevant improvements. However, the Council's text makes an important step by clarifying that the conditions to meet UMN are alternative rather than cumulative. This approach is more workable for patients. At the same time, the Council's reference to comparative clinical trials is problematic, as such trials are not always feasible for scientific, ethical, logistical, or economic reasons.
- **European Parliament** - Broader: includes morbidity, patient quality of life, high burden of disease/treatment, and inability to perform daily activities. It also recognises the role of patient experience data. However, this definition applies

Rue de l'Industrie 24
1000 Brussels, Belgium
Registration number: 0783762176
www.cancerpatientseurope.org
info@cancerpatientseurope.org

conditions cumulatively rather than alternatively, which risks excluding important innovations.

CPE's Call for a Patient-Centric, Future-Oriented Definition: UMNs must be understood as **dynamic and evolving**, not static or narrowly defined. The current legislative proposals risk undermining innovation, restricting access, and failing to reflect the complexity of medical and patient realities—particularly in cancer care.

CPE therefore calls on trilogue negotiators to move towards a **definition that captures elements from both the Parliament and the Council**: broad in scope to include morbidity and patient experience, but also pragmatic in retaining the Council's approach of alternative rather than cumulative conditions. At the same time, unnecessary requirements such as mandatory comparative clinical trials must be avoided.

CPE proposed definition:

*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, severely debilitating, or substantially quality-of-life-impairing disease**, and at least one of the following conditions is met:*

- *There is **no medicinal product authorised** in the Union for such disease, or, where medicinal products are authorised, the **disease remains associated with high morbidity or mortality**;*
- *The use of the medicinal product results in a **meaningful reduction in disease morbidity or mortality** for the relevant patient population;*
- *It offers **clinically meaningful progress** and addresses **patient-centred care gaps**, such as improved safety, tolerability, access, adherence, acceptability, convenience, or individual patient experience.*
- *It responds to **societal needs**, including improvements in disease management, healthcare system sustainability or resource allocation.*

Why This Matters for Cancer Patients: Cancer care often progresses incrementally, with innovation achieved step by step rather than solely through breakthrough therapies. Treatments frequently move from third- or second-line to first-line use over time or are combined for greater benefit. A rigid, narrow UMN definition risks disqualifying many such therapies from support or incentives, despite their real-world impact on survival and quality of life.

UMN is Not One-Size-Fits-All: The definition of UMN depends on context and perspective. From a **medical** standpoint, it is determined by clinicians based on disease severity and the

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availability of effective treatments. From a **patient** perspective, it is shaped by lived experience, including the impact on quality of life, daily functioning, and treatment burden. From a **societal** viewpoint, it is linked to healthcare system priorities, resource allocation, and cost-effectiveness considerations.

CPE emphasises the importance of distinguishing between unmet medical needs, unmet patient needs, and unmet societal needs, and ensuring that all three are taken into account.

Risks of a Too Narrow Definition: A restrictive UMN definition could exclude impactful incremental innovations in cancer care, influence national HTA, pricing, and reimbursement decisions, increase disparities in cancer care between Member States, and undermine 20 years of progress towards patient-centric innovation.

CPE urges EU co-legislators to adopt a **definition of UMN that is broad, flexible, and patient-centred**—one that captures both breakthrough and incremental innovation, and reflects the diverse realities of people living with cancer.

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Signatories

This position paper is endorsed by the following organisations:

- European Association of Urology - EAU
- European Liver Patient's Association - ELPA
- Pancreatic Cancer Europe - PCE
- Anticancer Fund
- European Federation of Crohn's & Ulcerative Colitis Association - EFCCA
- Parkinson's Europe
- European Society of Radiotherapy & Oncology - ESTRO
- European Network of Gynaecological Cancer Advocacy Groups - ENGAGe
- Cittadinanzattiva - Active Citizenship Network
- Digestive Cancer Europe - DiCE
- European Society of Gynaecological Oncology - ESGO