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Unmet medical need: a comprehensive definition ensures innovative patient care

Currently, the EU is revising its pharmaceutical regulations. The EU Commission aims to encourage the development of new therapies that address unmet medical needs, and proposes to do so by creating a regulatory framework that imposes a narrower definition and includes an additional “high unmet medical need” category and offers enhanced regulatory protection rights. These provisions have negative consequences for patients as they reduce incentives for research and development, thereby hindering important innovations – incremental innovations in particular. The definition of unmet medical need must remain comprehensive and unrestricted by additional gradations in order to address the diverse range of patients' unmet medical needs. Rather than imposing restrictions, there should be more incentives for research and development to encourage innovation in Europe.

Research-based pharmaceutical companies primarily focus on developing innovative drugs that address previously unmet medical needs, particularly for people with life-threatening or severely debilitating diseases where effective treatment options are limited or unavailable. A prime example is the field of rare diseases, which has seen over 200 new therapies approved in approximately 20 years. Similarly, significant advancements have been made in oncology with the development of a tremendous number of new treatment options.

Patients have very different individual needs

Patients have varying perspectives on their conditions and their unmet medical needs. People with chronic diseases define their needs differently from people faced with a life-threatening condition. Patients with chronic conditions prioritize aspects such as integrating medicines into their daily lives and reducing side effects. Conversely, many people with terminal illnesses consider prolongation of life more important, while others may prioritize pain relief and overall quality of life. Consequently, patients exhibit a broad spectrum of unmet medical needs that can only be adequately addressed through a comprehensive definition.

The proposed changes restrict unmet need as an incentive tool

The concept of unmet medical need is supposed to help identify and prioritize urgent patient needs. The classification is intended to provide incentives for research and development, such as preferential access to accelerated or special approval pathways, including for rare diseases. The existing broad definition of unmet medical need has been successful in driving progress. However,

the EU Commission is proposing to change those incentives by introducing a narrower definition into European legislation and an additional "high" unmet medical need category that is linked to regulatory protection rights.

Narrow definition discourages research, development and innovation

Incentives based on a narrow definition may slow down innovation by stifling investment in high-risk research areas that do not fit neatly into the narrow definition, potentially preventing the development of effective new treatments for a number of diseases and conditions. Patients whose medical needs fall outside the narrow definition may suffer as a result. Furthermore, the narrowed definition reduces predictability in terms of incentives versus the old definition, further complicating investment decisions. This narrowing threatens to impede rather than accelerate the development of new drugs, adversely affecting future patient care.

Additional classification of high unmet need is a fatal mistake

The introduction of an additional gradation for high unmet need, aimed at achieving "exceptional progress," is fundamentally flawed. Such an approach disregards incremental, step-by-step innovations, which represent a significant portion of advancements and are crucial for sustained progress and breakthrough innovations. Patients who anticipate gradual improvements in the treatment of their medical needs may be left disappointed in the future. In addition, hopes for breakthrough innovations could also be diminished. Additionally, the gradation of high unmet need raises concerns about the arbitrary nature of prioritization practices, leading to ethical considerations.

A comprehensive definition ensures innovation

To ensure innovation and effectively address unmet medical needs, a comprehensive definition without additional gradation that encompasses the fundamental characteristics of medical need is necessary, in order to do justice to the wide range of unmet medical needs specific to diseases and patients. Incremental innovations should be accorded a place in that definition, in the best interest of patients.

Research-based pharmaceutical companies recommend the following definition:

> An unmet medical need is a condition that existing approved medications and methods do not adequately prevent, treat, or diagnose.

The latter definition aligns with the European regulatory authorities' understanding of accelerated and special marketing authorization pathways (PRIME, conditional marketing authorization), by capturing and acknowledging disease-specific and patient-specific unmet needs. This definition offers effective incentives to develop innovative medicines to treat unmet need and can help ensure future innovation.

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