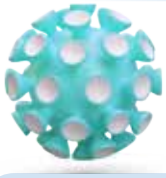
Unmet Medical Need: A Patient-Centered Definition that Promotes the Development of Treatments

Understanding the scientific progress is critical to address unmet needs and improve patients' outcomes.

The development of medicines is always driven by an ambition to address unmet medical needs (UMN). However, **making large jumps in how we understand disease is not linear,** and predicting where science will lead is often impossible.

Research may reach a number of dead ends before leading to a new medicine, **and efforts aimed at addressing a certain disease can lead to significant advances in other areas.**

How we define and incentivise medical innovation will shape future developments in medical progress. To achieve this, we need **to foster, incentivise and support a broad research ecosystem** that allow science to explore new pathways. Addressing UMN effectively requires tools that align with the realities of scientific progress and R&D investment.



The mRNA technology, initially explored as a potential therapeutic approach in oncology, was studied for decades with few or no clinical applications. However, this long-term research provided us with a tool that was utilized to protect citizens from the coronavirus.

Right objective, wrong approach

The EU's proposal to revise European pharmaceutical legislation includes a narrow definition of unmet medical need.This **would disincentivise investment in some areas, limiting future innovation.**

According to the European Commission's proposal, only **18%** **of medicines are very likely to be evaluated as addressing an unmet medical need, having a significant impact on specific disease categories, such as cardiovascular diseases (CVD), diabetes, migraines, and certain indications in oncology[1].**

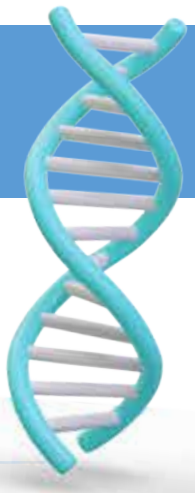
A broad, patient-centered definition of unmet medical need is necessary.

Defining UMN is personal. Patients with long-term conditions may define their unmet needs differently from those facing a life-threatening disease. Limiting UMN to life-threatening or severely debilitating diseases risks overlooking conditions that, while not meeting these criteria, still have significant negative impacts on patients, their caregivers, and society. **A more patient-centered definition of UMN would encourage research into chronically debilitating conditions that significantly impact quality of life and disease remission while emphasizing improvements in disease management, as well as the inability to perform daily activities.** This broader definition should also reflect patient needs by promoting treatments, prevention measures, and vaccines that address these important concerns.

If a narrow definition had been in place for the past 20 years, the paradigm shift in the management of type 2 diabetes, and therapies that have had major benefits in the areas of neurodegenerative diseases and CVD would simply not have been possible.

1. EFPIA, The Commission's criteria to define unmet medical need and high unmet medical need. Implications of a proposed incentive framework, https: https://efpia.eu/media/4aep340e/criteria-to-define-umn-and-humn-implications-of-a-proposed-incentive-framework.pdf

Scientific progress occurs gradually. Since investments to address a disease may depend on the existence of appropriate incentives, the European legislative framework should be designed to support both breakthrough and incremental innovations.



A narrow definition of UMN would disregard incremental innovations, such as pain reduction, slowing disease progression, or improving treatment adherence, while taking into account patients' preferences regarding how therapies are administered.

It is unlikely that for any patient population with an unmet medical need, a single medicine will solve that need entirely. I**t is more probable that multiple medications will address different aspects of unmet medical need,** offering complementary effectiveness.

Addressing UMN through innovation is at the heart of everything the Pharmaceutical Industry does, driving advancements in patient care. Each treatment that is made available to patients along the way **contributes to advance research, eventually making it possible to develop new innovative treatments.**

Cervical Cancer	Hepatitis C	Cystic Fibrosis
In 2020, 13,437 women across Europe lost their lives to cervical cancer. However, due to the HPV vaccine, the risk of developing cervical cancer is reduced by 90%, saving 90 million women[1].	Approximately 15 million Europeans live with hepatitis C. Due to innovative medications, the cure rate reaches 95%[2].	A transformative treatment for cystic fibrosis was approved by the European Medicine Agency in 2020, 30 years after the gene mutation causing cystic fibrosis was identified. This came as the ninth treatment option. Since 2012, six medicines brought treatment options for patients, paving the way for the ninth treatment to be developed
HIV	**Psoriasis**	**Advanced Melanoma**
The first antiretroviral to treat HIV was approved in 1986; several more followed in the 1990s, as well as new classes of drugs which acted much faster than its predecessors. Over the past 30 years, innovative treatments have transformed HIV from a fatal disease into a manageable condition.	In the 2000s, biologics originally developed for rheumatoid arthritis were found to reduce skin lesions in patients with psoriatic arthritis. These treatments improved psoriasis symptoms by 75% in approximately half of the patients. Building on this breakthrough, subsequent waves of discovery increased the number of patients with no detectable psoriasis from 0.1% with placebo to 4.2% with the first biologic to 40% with current treatments.	Survival rates for advanced melanoma have gradually increased in recent years—and there is still room for improvement. Ten years ago, only 10-15% of patients survived five years after diagnosis. Immunotherapy delivered some progress, but the first waves of treatments were expensive and had strong side effects. Today, thanks to incremental innovation,survival rates have increased to 60%.

KEY POINTS

1. The definitions of UMN and high unmet medical need (HUMN) aim to create a framework that encourages innovation directed at areas of highest need. It plays a crucial role in investment and priority-setting decisions among stakeholders such as regulators, HTA agencies, payers, academic community, and the pharmaceutical industry.
2. Identifying a condition as a UMN highlights its health policy importance, encourages research activities, and promotes the development of innovative treatments and health technologies.
3. The definition should recognize patients' need for therapies which allow improvements beyond morbidity and mortality outcomes.
4. Actively engaging all relevant stakeholders is essential to identify (UMN) from different perspectives. These multi-stakeholder collaborations should involve representatives from diverse patient groups, as well as broader societal and health care system stakeholders. For this purpose, clear rules of engagement should be developed.
5. Developing a patient-centred, more inclusive definition of UMN. By acknowledging the value of innovation and encouraging advances in prevention, treatments and care, Europe can ensure that no patient is left behind.