

UNMET MEDICAL NEED IN EUROPE

Health innovation is focused on **developing therapies** that use cutting edge science. Innovation meets the unmet medical need by **treating serious illnesses** and improving outcomes for patients. Over the last couple of decades, the debate on unmet medical need has evolved due to critical advancement in scientific and technological innovation.

1

Data and data science have **transformed drug discovery and development at an unprecedented scale** resulting in a paradigm shift to biomarker led medicine and personalised medicines

2

Innovation offers **possibility of treatments** for patients with **limited treatment options**

3

New and better medicines are being developed that may **offer significant benefits over current alternatives**

This new reality has sparked a debate on what constitutes “unmet medical need”. With the upcoming revisions to the General Pharmaceutical Legislation and the Orphan Medicinal Products Regulation, Amgen supports the development of a legal framework for unmet medical need that encourages a pro-innovation environment, ensuring that treatments are developed to help patients who need them.

THE ISSUE

Unmet medical need is a complex concept that is subject to **different interpretations**, with more than 15¹ definitions **depending on the stakeholder perspective**: patients, industry, healthcare professionals, society, regulators or payers¹

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The upcoming revision to the General Pharmaceutical legislation is expected to **agree on a common understanding** of unmet need in light of different interpretations.

Identifying a particular medical need as "unmet" is intended to encourage innovation in that area



We risk having too narrow a definition



A narrow definition may de-incentivize investment for innovation in Europe

Getting a **harmonised understanding on a definition** will be essential to **continue encouraging innovation** in the European Union.

1. EFPIA: Sounds of Science – Episode 7 on Unmet Medical Needs (UMN) Sounds of Science – Episode 7 on Unmet Medical Needs (UMN) (eucope.org)
 2. European Commission (2020). Pharmaceutical Strategy for Europe. Available at: <https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:52020DC0761&from=EN>.
 3. OECD (2018). Pharmaceutical Innovation and Access to Medicines. Available at: <https://www.oecd.org/health/health-systems/Pharmaceutical-Innovation-and-Access-to-Medicines-Executive-Summary-ENGLISH.pdf>.
 4. EFPIA (2022) The Pharmaceutical Industry in Figures 2022. <https://efpia.eu/media/637143/the-pharmaceutical-industry-in-figures-2022.pdf>
 5. Europe accounted for a 19.3% share of global clinical trials activity in 2020, a decrease of 6.3% compared with a 25.6% average over the last ten-years, according to GlobalData. See: <https://www.clinicaltrialsarena.com/clinical-trials-analysis/europe-accounts-for-19-3-share-of-global-clinical-trial-activity-in-2020/#:~:text=Europe%20accounted%20for%20a%2019.3,%20year%20average%20of%2025.6%25>

THE SOLUTION

We call for a **predictable set of criteria** for the concept of **Unmet Medical Need** in which **addressing disease severity and burden on patients, families and caregivers** is attributed to the concept of **major therapeutic advantage** (e.g., **Conditional Marketing Authorization & Accelerated Assessment**).

Where treatment already exists, an **innovative treatment** could be considered as **addressing one or more** of the following examples:

IMPROVING EFFICACY AND/OR SAFETY OF A TREATMENT

TARGETING PATIENTS UNABLE TO BE TREATED, TOLERATE, OR WHO FAIL TO RESPOND TO EXISTING TREATMENTS

IMPROVING THE QUALITY OF PATIENTS' EXPERIENCE, SUCH AS THE MODE OF ADMINISTRATION OR DELIVERY SYSTEMS

IMPROVING PATIENT COMPLIANCE WITH RESPECT TO THE TIMING, DOSAGE, AND FREQUENCY

THE IMPACT

Attracting drug developers to invest in underserved disease areas is often a challenge – but also a key priority of the Pharmaceutical Strategy for Europe². The **EU's system of incentives** to stimulate innovation across a wide range of therapy areas **will depend on what constitutes an Unmet Medical Need. Getting this right is** therefore **essential to:**

Provide a **predictable and stable framework** that rewards innovators for taking on the considerable **risk of developing medicines and therapeutics** as well as manufacturing at risk before approval. **Drug development** takes an average of **10-15 years**³, it involves significant capital investments and has high failure rates.

Attract innovation back to Europe.

Current trends show how this region's global **R&D investment** has decreased in comparison to the **United States and China**.^{4,5}