Cardiovascular disease (CVD) and unmet medical need (UMN)

Addressing unmet medical need (UMN) is a cornerstone of pharmaceutical innovation as the industry aims to develop treatments that improve and extend peoples' lives.

What is unmet medical need?

- → Identifying a particular condition or disease area as an UMN is intended to signal its health policy significance, stimulate research activities and incentivise the development of innovative treatments, diagnoses or health technologies aiming to serve patients who need them.
- → Taking the patient needs and the evolution of science as a starting point, EFPIA understands an UMN as a condition that is not adequately prevented, treated or diagnosed by authorised interventions. A broad understanding of UMN should recognise and tackle the many ways in which UMNs manifest.

What is needed to spur innovation in CVD?

- → To incentivise continued CVD research in Europe and deliver novel treatments to patients, policymakers need to ensure that the right financial and regulatory frameworks are in place.
- → Given the long and risky development timelines, predictable incentives are needed to stimulate companies to invest in R&D for relevant treatments to meet patients' needs.
- → With appropriate investment into CVD and a more flexible regulatory framework, the potential of CVD R&D can be unlocked to address the remaining unmet needs of patients.

What are the risks of a narrow definition of UMN?

- → Any definition of UMN that is based on narrow criteria such as disease severity, the presence/absence of current treatment options, or the potential of new treatments to reduce morbidity or mortality can pose great risk to innovation as it disregards the chronic nature of many CVDs and the importance of patient-reported outcomes and experiences.
- → A narrow definition disregards incremental innovation which can offer important improvements to patients such as improving quality of life, reducing pain, or extending lives and slowing the progression of disease.
- → Narrowing the definition of UMN would reduce predictability and disincentivise companies to invest in R&D and decide to start complex trials where the outcome in comparison to incentives and regulatory pathways is uncertain. Narrow criteria defining UMN would put most innovation in CVD at great disadvantage, while there is continued patient needs in most CVD areas, such as high LDL-C, heart failure, etc.

CVD is the number one killer in Europe and costs the EU €210 billion each year.[1]

If the EU wants to remain innovation-driven and competitive in the CVD area and ensure its strategic open autonomy, better promotion of R&D and consideration for patient access even in cases of incremental advances in innovation are key. The EU should put in place a system of incentives that rewards results in the CVD field that includes appropriate valuation, consideration of surrogate endpoints, patient-reported outcomes and quality of life indicators.

[1] Wilkins, E. et al. European Cardiovascular Disease Statistics 2017, European Heart Network (2017)