Supporting patient-centred innovation: the value of patient experience data

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One key priority for EPF is to ensure that **patients have access to the medicines they need**. For many patients with chronic diseases, medicines are an essential part of their treatment. Medicines offer the promise of a significant improvement in health or quality of life, or even a cure.

Access to medicines is not only a question of availability, affordability and accessibility, but also of **adequacy** and **appropriateness**. Medicines must be safe, of high quality and effective, and they must also address patients' needs, as defined by patients themselves.

The inclusion of patient experience data (PED) at all stages of medicine development and regulatory decision-making ensures that new medicines address the outcomes and preferences that matter to patients. These data are collected to describe patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care[1]. Examples of such data include patient preference studies, large patient surveys, or impacts captured in registries. They ensure that the views and experiences of patients living with a particular condition are taken into account in the development and approval of medicines, ultimately leading to more relevant decisions for patients. The EMA's ongoing work on the generation, collection and use of patient experience data for regulatory purposes, together with the support of the European Medicines Regulatory Network[2], is a crucial step forward. These advancements need to be reflected in the revision of EU pharmaceutical legislation to encourage patient-centred and needs-driven healthcare innovation.

- **Patient experience data are crucial to obtain a full picture of the impacts of a disease on patients and define unmet medical needs.**

The concept of unmet medical need should aim to distinguish innovative medicines that provide tangible and significant added therapeutic value to patients from other new medicines. From the patient's perspective, a medicine that addresses an unmet medical need goes beyond mortality or morbidity considerations. It encompasses the broader impact of the disease, treatment or therapy on the patient's life, and addresses key questions, for example: will this medicine significantly enhance quality of life through e.g. less time spent at the hospital, decreased side effects, reduced symptoms, increased productivity? Will it significantly slow down the progression of a disease? Will it provide a cheaper alternative where existing treatments do not reach patients?

Many lifelong chronic diseases, while not necessarily or no longer life-threatening, impose a significant burden on individuals, caregivers, and healthcare systems. This reality underscores the importance of collecting patient experience data, which play a key role in assessing whether and how a product meets patients' unmet needs. By integrating this data, we can ensure that healthcare innovation is truly patient-centred and addresses their unmet medical needs, while avoiding misleading claims that promote minor benefits with no real impacts on patients' lives.

The definition of unmet medical need in the pharmaceutical legislation is a critical issue for the patient community as it has far-reaching implications beyond research and development. Not only will it form the

[1] Executive summary – patient experience data in EU medicines development and regulatory decision-making workshop (Europa.eu)
[2] European Medicine Agencies Network Strategy to 2025 (Europa.eu) ; Regulatory Science Strategy to 2025 (Europa.eu)

basis for companies' eligibility for data protection, conditional marketing authorisations and enhanced regulatory and scientific support, but it will also have an impact throughout the post-authorisation phase and during the health technology assessment (HTA) process. In this context, it must reflect criteria that matter to patients, which patient experience data help measure.

- **Companies should be encouraged to include patient experience data in the marketing authorisation application.**

While there is growing consensus about the value of patient experience data in the medicines' development process, more efforts are needed to ensure that clinical research addresses the parameters that matter most to patients, such as quality-of-life indicators. Without systematic patient involvement from the outset, across the research and regulatory continuum, the assessment of the risk-benefit balance and the real added value of a new product remains incomplete. Requirements under the pharmaceutical legislation to include, whenever possible, patient experience data in the marketing authorisation dossier would provide a clear signal to manufacturers. The IMI PARADIGM project already provides tools and practices to mainstream patient perspectives and experiences, while enhancing mutual trust between stakeholders in the patient engagement process. Additional guidance from the EMA is needed on the collection and integration of this data into the regulatory decision-making process.

In addition, the inclusion of patient experience data in the marketing authorisation dossier is particularly relevant to the HTA process, which informs subsequent decisions by payers. As part of the implementation of the EU HTA Regulation, the inclusion of patient experience data in the marketing authorisation dossier could help shorten the time between marketing authorisation and actual patient access. In fact, the scientific evidence submitted to obtain marketing authorisation is often deemed insufficient by HTA body assessors, leading to delays and inefficiencies[3].

Ultimately, the inclusion of patient experience data across the regulatory lifecycle is critical to promoting patient access to the medicines they need and achieving better health outcomes for patients, while supporting more sustainable healthcare systems.

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[3] KCE (2021). "Evidence gaps for drugs and medical devices at market entry in Europe and potential solutions"v