AFCROs

LES ENTREPRISES DE LA RECHERCHE CLINIQUE

PATIENTS CONTRIBUTIONS IN MEDICAL PRODUCT LIFE CYCLE

Article written byr

Céline Fabre, HORIANA
Lise Radoszycki, CARENITY
Mathieu Rosé, ALIRA HEALTH
Membres du groupe de travail RWD de l'AFCROs

02/2024

Patient experience data is increasingly being collected and considered at every stage of the medical product lifecycle. By integrating the voice of patients, important concepts such as shared medical decision-making, patient quality of life, as well as the enhancement of care quality can be considered in the evaluation and monitoring of these products. In addition to health authority recommendations advocating for the systematic integration of patient experience in healthcare product evaluation, this data, being rich and multidimensional, can also serve as a tool for communication and differentiation in a competitive market. It also enables the regulation of the healthcare system with devices and systems that integrate indicators of the quality of the care pathway.

Patients, and especially patient associations, are major players in the healthcare research ecosystem. Their involvement, highly valued and recommended by health authorities, brings essential knowledge to the execution of studies, particularly concerning the patient's experience. In France, in numerous conditions, such as HIV or diabetes, for instance, deeply committed patient associations have become indispensable in evaluating healthcare products.

Patient-Reported Outcomes (PROs) enable the evaluation of outcomes perceived by the patient, without interpretation by the physician or a third party. This approach generates pertinent, reliable, and robust data, particularly in the life cycle and evaluation of healthcare products. Two main categories are identified: Patient-Reported Outcome Measures (PROMs), which measure care outcomes, and Patient-Reported Experience Measures (PREMs), which measure the patient's experience regarding their care pathway. PROs can be utilized in all stages of developing a healthcare product (especially to anticipate market access) and also in post-market surveillance studies.

Implementing studies incorporating PROMs and PREMs requires an appropriate statistical methodology depending on the study design and the type of questionnaire: generic, specific, or even created from scratch. For the latter, collaboration between sociologists, patients, and methodologists is crucial in their development and study implementation. This collaboration allows for considering the patient's feelings comprehensively and proposing an adapted methodology.

Patients play a crucial role in research implementation by acting as intermediaries and promoting studies on healthcare products (patient information, recruitment, support, etc.). They can intervene at various levels: during study design, providing strong expertise on the disease and the patient's experience with the healthcare product, which can guide the study's design; in patient information and protection of patients' rights. Patient associations can alert health authorities and intervene in the evaluation and negotiation of healthcare product prices.

Early patient involvement in healthcare product design is an effective approach to ensure the alignment of treatments, services, and devices under development with patient expectations. By describing their unmet medical needs and expectations, patients contribute significantly to defining the profiles of products to be developed (Target Product Profile). Preference studies such as Discrete Choice Experiments (DCE), Best-Worst Scaling (BWS), or Vignettes prove to be valuable tools in identifying expectations related to new treatments, covering preferences regarding administration modalities, dosage form, and providing a comprehensive understanding of the benefit-risk balance from the patient's perspective.

In the field of medical devices and digital therapies, early patient involvement offers the opportunity to test solutions under development beforehand, thus promoting optimal device usage in real life and encouraging its long-term adoption.

Understanding patients' experiences prior to clinical trials allows for optimizing research design and protocol. Indeed, patients can help validate the evaluation criteria (endpoints/outcomes) initially proposed by clinicians by sharing their opinions on quality-of-life scales (PROs) assessed during the trial and identifying evaluation criteria that are most important from the patients' perspective.

When consulted on the protocol, patients can highlight constraints and obstacles related to their participation and contribute to identifying solutions to promote patient inclusion and retention throughout the clinical trial. They can also contribute to reviewing documents intended for participants, especially the informed consent form (ICF). This approach aims to ensure a clear understanding of the study design, participants' rights, and potential risks associated with the study.

Early collaboration with patients enhances the ethics of clinical research and promotes the development of healthcare products that align with the needs and expectations of end-users.

Patient involvement at the time of launching a healthcare product can be crucial to evaluate if the clinical value of an innovation aligns well with patient needs and expectations. Conducting interviews with early adopters of innovations allows for understanding their emotional perspective and measuring the healthcare product's benefit on patients' quality of life in real-life conditions. The insights collected enable healthcare industries to make necessary adjustments for optimizing the access and usage of their new healthcare product.

Patients also directly engage with health authorities. In France, since 2015, patient associations have joined the HAS-Haute Autorité de Santé (French Health Authority) healthcare product evaluation committees*. They sit as full members with deliberative votes and participate in scoring each healthcare product for reimbursement. They express how they experience their diseases, their current treatments, and their expectations for the healthcare product (medicine or medical device) under evaluation.

*Haute Autorité de Santé - Arrivée des usagers à la CT et à la CNEDiMTS (has-sante.fr)

Once on the market, a healthcare product may undergo evaluation to assess its real-world use, clinical benefit, and side effects. To answer these questions, a real-world study conducted within the context of usual patient care (as opposed to clinical trials conducted in an experimental setting) can be implemented.

In France, the HAS encourages using patient-reported data as judgment criteria, whether as secondary or primary criteria, that notably documents quality of life. In these studies, it is recommended to incorporate PROs that collect data for analyzing patients' quality of life and/or any other relevant measures for patients.

Patient centricity is a comprehensive approach aiming to position the patient at the heart of healthcare stakeholders' concerns to better anticipate and address their needs.

Patients possess specific and valuable knowledge. Their experience of illness, existing treatments, or the care pathway, along with their expectations, are elements that can enrich the evaluation of healthcare products.

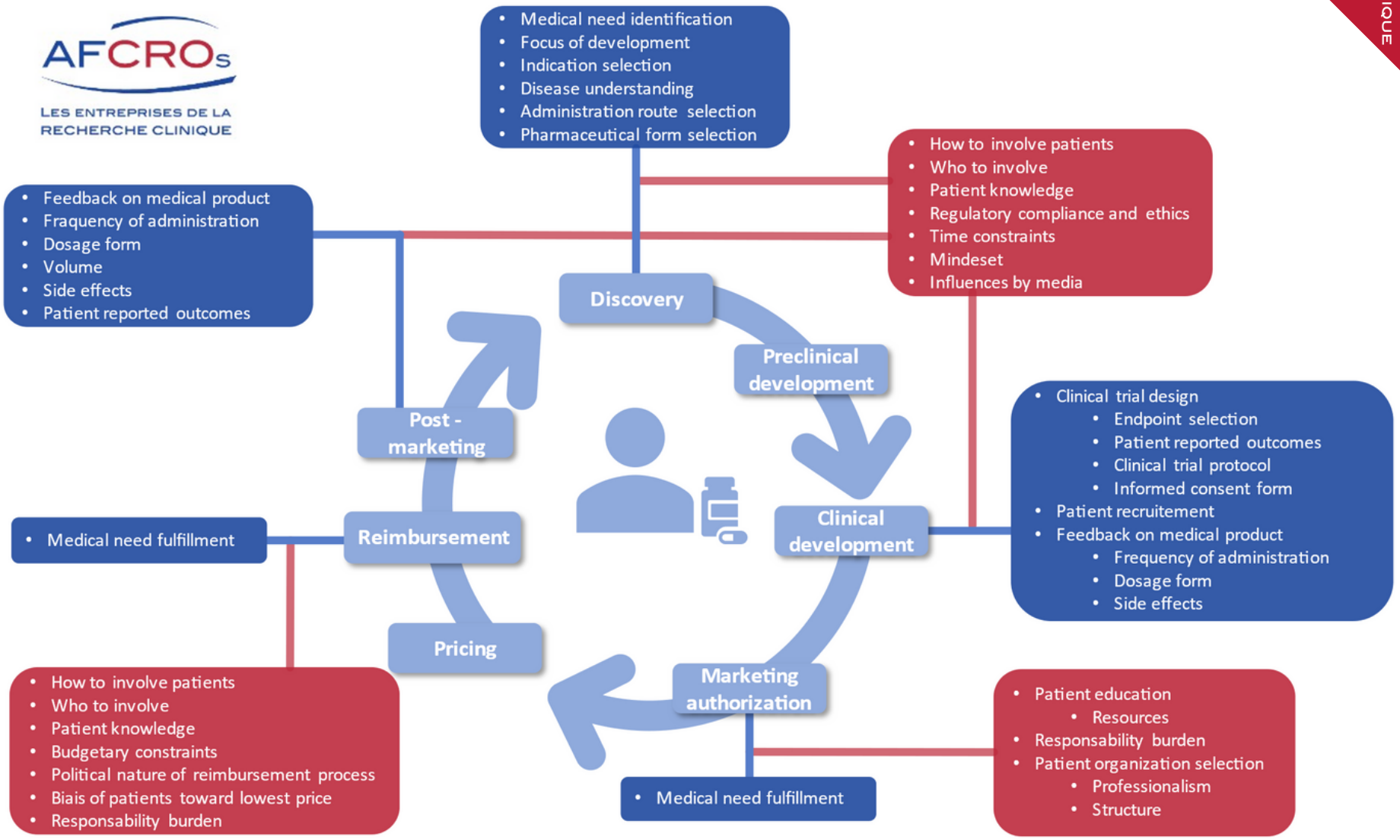
As genuine partners and stakeholders in their health, involving patients at every stage of the medical product life cycle, from conception to market entry and commercialization, is valuable. In this context, health authorities in France and abroad are increasingly paying attention to patient-reported data.

Contributions from patients or the associations representing them can be made through qualitative interviews or quantitative studies aiming to measure PREMs/PROMs indicators or assess patient preferences. Whichever approach is chosen, it is necessary to employ rigorous methodology to design the study, select the indicators to measure, and minimize study biases (cognitive biases, selection biases, etc.).

Therefore, conducting such studies requires essential scientific, methodological, regulatory, and ethical expertise so that patients' contributions can be considered by Health Technology Assessment (HTA) bodies. In France, HAS has issued recommendations, especially for using PREMs and PROMs in routine clinical practice, as well as for using real-world data in the evaluation of drugs and medical devices**.

The widespread use of tools and methods to collect patients' real needs and experiences appears efficiently and regularly as a key element in improving the perceived quality of care by healthcare system users.

*https://www.has-sante.fr/jcms/p_3325627/fr/aide-a-l-utilisation-des-proms-en-pratique-clinique-courante
**https://www.has-sante.fr/upload/docs/application/pdf/2021-06/guide_etude_en_vie_reelle_medicaments__dm.pdf



Patients contributions in medical product life cycle