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Merging Science and Patient in Future Drug Development to Enhance Safety and Effectiveness

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1. Introduction

Increasing longevity is the result of growing wealth and advances in healthcare provision including effective pharmacotherapy. The number of patients with high or very high age and multimorbidity are becoming the new reality in healthcare provision challenging our one-disease and one-treatment model (1). As healthcare professionals we are focusing on the treatment of the disease led by diagnosable disease parameter. Poor effectiveness, non-adherence, medication errors and increasing hospitalization due to adverse drug reactions have put more focus on the patient as a user of the drug products and important factor in therapeutic outcomes. As a result, regulations are emerging to include the patient perspective into the drug development programs (2, 3). The objective of this abstract to provide insight into patients behavior, expectation and capabilities related to the management of the drug therapy.

2. Patient behavior and expectation

In contrast to the rational treatment, which is based on diagnostics and clinical evidence, human behavior is much less rationally driven. Bodily sensations, health beliefs, personal expectations and psychosocial wellbeing are not necessarily in sync with evidence-based health behaviors. The diagnose of a disease has a significant impact on the self-identity and the life plans of an individual. Acceptance of the disease is the first important step towards "evaluation" of a prescribed drug therapy to manage the disease. The expectation of patients in a treatment focuses on the elimination of the negative disease symptoms, wellbeing and quality of life. When these expectations are not met or adverse drug reaction occurs patients tend to take a consent decision to not pursue the drug therapy. In case of acceptance of the therapy, the required dosing moments must be incorporated without disrupting the daily routine and social activities (4).

3. Patients capabilities

Self-preparation and administration of the therapy according to a defined schedule and defined instructions for use requires a certain degree of cognitive, sensory and functional capabilities (5). Multimorbidity is associated with increasing disabilities and limitations in managing complex therapeutic schedules. As multimorbidity correlates with the number of drugs (polypharmacy) the workload of therapeutic complexity increases, while the functional capacity of the patient declines until patients are no longer in the position to self-manage the therapy. Patients tend to overestimate their own capabilities and develop different pathways to reduce the complexity (6). This might lead to unintended hospital admissions due to medication errors and adverse drug reaction, which correlate with the number of drug products, of which the majority seem to be preventable (7, 8).

4. Patient drug product interface

The interface with a drug product starts with a visual and tangible investigation in order to understand the object and its functioning. This assessment is being done based on prior experience with similar drug products to reduce the level of cognitive demand. The intention is to identify a few dominant visual or sensory cues that fit into any known use model. The use than follows the previous product uses, which is an effortless and intuitive user process. This intuitive user approach bears the high risk for errors or incorrect use that remains undetected by the patient.

The medication preparation process requires an increasingly high level of organizational, temporal, operational, cognitive and emotional input from the patients. Successful performance requires that both cognitive demand and level of focused attention increases with the number of medicines, which might overload the patient capabilities. To reduce the level of demand and complexity patients develop routines and their own procedures (6). Patients experience issues with preparing the medication schedules of which manual issues (e.g. open packaging, splitting tablets), recognition issues (e.g. not knowing medication, confusion about products, identification) and complexity issues (e.g. omitting medicines, wrong dose) are the most prominent ones. These issues are related to elements of the pharmaceutical drug product design which are not in accordance with the capabilities and needs of the patient.

5. Conclusions

Pharmaceutical and medical sciences are based on scientific data and clinical evidence. The focus during the drug development is on the safety, efficacy and quality of pharmaceutical product. Despite these data, there is growing awareness of the gap between efficacy and effectiveness that is achieved in real-world patients. Including the patient perspective into the drug development as well as into the design of the pharmaceutical drug product is being considered to enhance drug product quality and effectiveness. This assumption is being made based on the experience with human factor design principles for medical devices. While implementation into drug product development remains a challenge, ongoing patient research and regulatory initiatives will continue to provide further guidance towards patient centric product design without compromising on the fast access of innovative drug product to patients.

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