The Increasing Importance of Patient Reported Outcomes and the Patient Voice in Drug Safety

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Over the past few years there has been a paradigm shift in the overall approach to pharmacovigilance from that of pure safety analysis to overall benefit-risk evaluation of products. Furthermore, patient centeredness and patient safety have emerged as core elements in today’s interactive and responsive health care systems. This has been coupled with a growing awareness to obtain more real world data directly from patients.

Studies show that patient reports are more valuable compared to healthcare professional (HCP) reports as patients, 1) often report earlier, 2) perceive the impact and severity of reactions differently and, 3) give more detailed descriptions—including specific circumstances like prescribing errors, off-label use, and ADRs (adverse drug reactions) of an intimate nature. Patient-reported outcomes of adverse events (PRO-AEs) offer one way to improve the quantity and/or quality of safety information. This brings real world data forward, thereby enabling improved benefit-risk assessment of pharmaceutical products.

PRO-AEs: ENABLING BETTER SAFETY EVALUATION AND DECISIONS

Patient-reported outcomes (PROs) are data elements directly reported by patients about experiences with healthcare, symptoms, functional status, or quality of life. This includes data collected via structured and validated questionnaires, as well as free-text spontaneous patient submissions. A PRO-AE is any untoward medical occurrence—whether or not considered treatment/intervention related—that is reported or transmitted directly by the patient without interpretation by a clinician, or by anyone else.

The benefits of PRO-AEs are applicable across various stakeholders. Patients can know expected potential AEs due to prescribed medications, and medication preferences, based on prior experiences of other patients (“patients like me”). PRO-AEs can help drug developers understand how well patients will tolerate a product by identifying tolerated dose levels in early-phase research, and then comparing tolerability between products from the patient perspective in pivotal trials.

Furthermore, the systematic, patient-reported approach based on detailed, complete reports would increase confidence of regulators in the fidelity of reported safety information for benefit-risk assessment. Payers would better understand the AEs due to specific treatments for prediction of utilization of healthcare services, and then determine benefit-

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risk balance (value) of medicines. HCPs would get a better understanding of the patient’s symptoms based on the individual patient’s experience combined with the physician perspective (based on experience/training). This can help to improve the measurement of symptoms in both clinical trials and practice.

**PATIENT-CENTRIC REGULATIONS, PRO-AE TOOLS, AND TECHNOLOGY ENABLERS**

Recent pharmacovigilance (PV) legislation from EMA and US FDA clearly call for inclusion of patient-reported information for safety monitoring to better define the safety profile, and benefit-risk balance, of new and existing medicines. Effective involvement of patients in the safety reporting process is possible via novel adverse drug reporting tools and processes like ADR online forms, mobile applications, and through local pharmacies.

Patient involvement in the set-up and user-testing ensures fit-for-purpose and user-friendly reporting systems. It also allows for the development of follow-up procedure guidelines for reports in consultation with patients’/HCP organizations, and enables the collection of high-quality data. Patient-centred systematic data collection can be achieved in limited populations via patient registries, or in a wider population using existing infrastructure, e.g. community pharmacies/pharmacists. The Patient-Reported Outcomes Safety Event Reporting (PROSPER) Consortium was convened to improve safety reporting by ensuring that the patient voice and perspective feed into safety data collection to enable better definition of safety profiles and benefit-risk balance of new and existing medicines. The guidance covers a minimum core dataset for use by industry or regulators to structure PRO-AEs, and explains how data, once collected, can be evaluated to drive safe and effective use of medicinal products.

Today, technological advances like simplified internet search capabilities, electronic health records, digital mobile devices, and patient online communities, offer unprecedented multidirectional communication between patients, clinicians, the private sector, industry, and regulators.

Social media is a promising tool for early detection of potential new emergent safety signals obtained directly from patients. But social media data is unstructured data, fraught with technical challenges like incomplete, incorrect fragmented reporting information, non-standard terminologies, and risk of identifying false safety concerns. Company managed PRO-AE-enabled websites, and online patient forums allow a more structured approach, where pre-emptively designed datasets provide higher quality of data.

**References**

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