Regulating Off-label Use of Medicines in Europe

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What is off-label use?

Off-label refers to the use of an authorised pharmaceutical product outside the terms of its marketing authorisation; therefore, not in accordance with the information in the Summary of Product Characteristics. Off-label occurs when a medicine is used for a therapeutic indication other than that approved by a regulatory authority, or in a different dosage, frequency, method of administration, or is used in a group of patients for which it has not been authorised.

Off-label use happens in all patient groups, but it is more common among certain patient populations. According to the ´Study on off-label use of medicinal products in the European Union´, such uses are particularly widespread in paediatrics—where children are often excluded from clinical trials—and in the area of rare diseases. Pregnant women and the elderly are also groups of concern. Oncology, hematology, psychiatry and rheumatology are frequently mentioned in the literature as clinical areas in which off-label use occurs. Off-label uses have not undergone the type of benefit-risk harm assessment required in the procedure for medicines' marketing authorisation. Yet, doctors might consider prescribing a medicine outside its approved label when there are is no available treatment for the patient (or if an available treatment has not been effective). Off-label prescription is part of medical practice and may be informed by reliable scientific evidence. However, it has been reported that healthcare professionals regularly prescribe medicines off-label with levels of evidence considered to be low. This is particularly problematic because off-label use lacking strong scientific evidence is has been associated with higher rates of adverse drug reactions events(ADR). While the promotion by pharmaceutical companies of off-label uses is illegal, it does happen. The commercial interests at stake, the lack of standard regulatory review, and uncertainties about liability and patients' rights are all factors that mean off-label use should be approached with caution.

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