Children's Hospital of Philadelphia PolicyLab

Beyond the Label: Steering the Focus Toward Safe and Effective Prescribing

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Each year, hundreds of millions of prescription medications are dispensed to pediatric patients. A significant proportion of prescriptions are used in an off-label manner, outside the specifications approved by the US Food and Drug Administration (FDA), rendering off-label prescribing a “public health issue for infants, children and adolescents,” as described by the Committee on Drugs for the American Academy of Pediatrics. The committee also explicitly states that off-label use “does not imply an improper, illegal, contraindicated or investigational use.” Yet, when used in research, clinical practice, or even the lay media, the term off-label commonly carries a negative connotation. This interpretation probably reflects the sense of uncertainty in understanding the risk–benefit balance of a medication without FDA review and approval. However, prescribing according to the package insert does not necessarily translate to the safe and effective use of a medication. The clinical trial data required for FDA approval often represent highly select populations in controlled settings with limited follow-up. These data may not translate well to real-world use, hence the need for postmarketing surveillance and research. Conversely, lack of pediatric labeling for a medication does not necessarily indicate a lack of evidence. It may simply mean the pharmaceutical company has not submitted an application for FDA approval to add a new indication or population.

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