Off-label drug use common, but patients may not know they're taking them, study finds

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Many people have probably heard of off-label drug use, but they may not know when that applies to prescriptions they are taking, a Mayo Clinic analysis found. Off-label drug use occurs when a physician prescribes medication to treat a condition before that use has been approved by the Food and Drug Administration. In a newly published article in *Mayo Clinic Proceedings*, researchers pose and answer 10 questions about off-label drug use.

"Since the Food and Drug Administration does not regulate the practice of medicine, off-label drug use has become very common," says lead author Christopher Wittich, M.D., internal medicine physician at Mayo Clinic. "Health care providers and patients should educate themselves about off-label drugs to weigh the risks and benefits before a physician prescribes one or a patient takes one."

Some highlights from the article:

- Off-label drug use is common. Within a group of commonly used medications, roughly 1 in 5 prescriptions were for an off-label use, a 2006 report found. Another study found that about 79 percent of children discharged from pediatric hospitals were taking at least one off-label medication.
- Patients may not know when drugs they have been prescribed are being used off-label. No court decision has required that physicians must disclose, through informed consent, the off-label use of a drug, the authors say. The FDA makes clear that it doesn't regulate the practice of medicine and that the federal Food, Drug, and Cosmetic Act of 1938 doesn't make physicians liable for off-label drug use, they note.
- Off-label drug use can become the predominant treatment for a condition. For example, some antidepressants are not approved by the FDA as a treatment for neuropathic pain, yet some drugs in this class are considered a first-line treatment option.
- Examples of widely practiced off-label drug use include morphine, used extensively to treat pain in hospitalized pediatric patients. Many inhaled bronchodilators, antimicrobials, anticonvulsants, and proton pump inhibitors also are used in children without formal FDA approval.
- Obtaining new FDA approval for a medication can be costly and time-consuming. To add additional indications for an already approved medication requires a supplemental drug application; if eventually approved, revenue from it may not offset the expense and effort for obtaining approval.
- Generic medications may not have the requisite funding resources needed to pursue FDA-approval studies. For these financial reasons, drug proprietors may never seek FDA approval for a new drug indication.
- Pharmaceutical manufacturers are not allowed to promote off-label uses of medications. However, they can respond to unsolicited questions from health care providers and distribute peer-reviewed publications about off-label use. Just this year, GlaxoSmithKline agreed to pay a record $3 billion to settle a Justice Department case involving alleged off-label drug use marketing, and Merck Sharp & Dohme was fined $322 million over its alleged promotion of the painkiller Vioxx for

an off-label use.

Provided by Mayo Clinic