Off-label Drug Use

Atty. Rodel V. Capule M.D.*

Off-label drug use is the practice of prescribing drugs outside the scope of the drug's approved label—this could be prescribing drugs beyond their intended use, duration, population or dosage."[1] "A physician prescribing a drug exactly as approved by FDA (Food and Drug Administration) is doing so on-label. When a physician veers from that path, he or she is prescribing a drug off-label."[2]

Take note that (FDA) approval "doesn't play a part in defining the drug's standard of care or how it is prescribed"[3] and FDA even conceded that some use of off-label drugs does not fall within the current standards of medical care or treatment.[4] Furthermore, when the FDA approves a drug or device for sale and marketing, it does so only with respect to the indicated uses.[5] This is not to say that other FDA labeling must be ignored. "The FDA-approved label may also include 'contraindications' and 'warnings and precautions.' A 'contraindication' is a 'situation() in which the drug should not be used because the risk of use . . . clearly outweighs any possible therapeutic benefit.' 'Warnings and precautions' are descriptions of 'clinically significant adverse reactions . . , other potential safety hazards . . . , limitations in use imposed by them . . . , and steps that should be taken if they occur,' as well as any other information regarding any special care to be exercised by the practitioner for safe and effective use of the drug..."[6]

Notwithstanding, off-label prescribing is common and in most cases legally and ethically acceptable. In third world countries, engaging in off-label use of drugs is even more compelling for economic reasons. There are even occasions that the first drug of choice is not available in the local market or prohibitively expensive, prompting physicians to resort to innovation justifying off-label use of drugs readily available.

Although the FDA approves drugs for only the uses indicated in the product labeling, drugs may have other, off-label uses that are beneficial.[7] However, "The fact that FDA has not approved labeling of a drug for a particular use does not necessarily bear on those uses of the drug that are established within the medical and scientific community as medically appropriate."[8] Emphatically, "the FDA does not regulate the practice of medicine," and "the decision whether to use a drug for an off-label purpose is a matter of medical judgment, not of regulatory approval."[9]

"It is also important to differentiate between experimentation and therapy. The difference lies mainly in intent. If it benefits the patient the off-label usage of a drug is deemed therapy, but if the purpose is to gain general knowledge for a broader population then it is considered human experimentation. Furthermore, "the interests of the researcher and the subject may conflict" in human experimentation. Therapeutic misconception arises from this conflict when patients believe that the research is going to benefit them directly, even though the intent and purpose of the study is to gain general knowledge and not necessarily benefit the patient. Thus, the ultimate purpose of research is discovery and knowledge, unlike therapy which promotes the best interest of the patient. Medical practice also recognizes "innovative therapy" that combines "research and practice"; here the "physician may wish to give a patient the best-known medication and simultaneously evaluate the medicine's efficacy." However, legally defining innovative therapy comes down to the physician's intent."[10]

"Physicians' freedom to prescribe drugs off-label carries important advantages. It permits innovation in clinical practice, particularly when approved treatments have failed. It offers patients and physicians earlier access to potentially valuable medications and allows physicians to adopt new practices based on emerging evidence. And it can provide the only available treatments for "orphan" conditions. The spectrum of off-label use includes guideline-recommended practice, last-resort therapy, and first-line therapy."[11] On the flip side, off-label prescribing is often done in the absence of adequate supporting data. Off-label uses have not been formally evaluated, and evidence provided for one clinical situation may not apply to others.[12] It undercuts expectations that drug safety and efficacy have been fully evaluated. When newer, more expensive drugs are used off-label, it increases health care costs.[13] In other words, "off-label drug use presents a unique paradox: it can both aid and harm the patient."[14]

Ultimately, off-label use of drugs should be made known to a patient. The fiduciary nature of a physician-patient relations demands securing an informed consent for its use. The information must not be limited to a bare "off-label" use of the drug, but should also include its reasonable use within the concept of standard of care for a particular illness.

References

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Dr. R.V. Capule is an attorney specializing in medical malpractice, physical injuries and food torts. He is a law professor of Legal Medicine at Arellano University School of Law and a consultant in Legal Medicine at Adventist Medical Center-Manila and Makati Medical Center.

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