

Ask the Ethicist: Off-Label Meds

May 2011

Q: *Almost every week I hear about promising new treatments for various eye diseases. While I want to offer my patients the latest developments, I hesitate when learning that many of these treatments are considered "off-label." What are the ethical issues with off-label treatments?*

A: The FDA approves drugs and devices for specific indications. The use of any medication "prescribed to treat a condition for which it has not been approved by the FDA" is therefore defined as off-label. While a drug's labeled use is specified by a regulatory body, its indications and incorporation into standard of care are determined by clinical trials and consensus practices. Thus, there is an inherent disconnect. An estimated 50 percent of medications used routinely in ophthalmic practice are used off-label. And many of these uses, ironically, have been validated by robust clinical trials.

The central tenet of the Academy's Code of Ethics is that clinical practice should be guided by the best interest of the patient. Advisability of any treatment for an individual requires an objective appraisal of the disease's natural history compared with known clinical effectiveness of available therapies. Physicians should discuss realistic treatment expectations, the reasonable odds of achieving these goals and incidence of potential complications with the patient. While one individual may choose a treatment with only a 10 percent chance of success, another may decline to use any treatment with less than a 90 percent chance of success. The decision to proceed should be made by the patient after appropriate informed consent.

Ophthalmologists must be aware of potential conflicts of interest with the use of off-label medications, including financial gain, notoriety or recognition, advancement of a personal research program or promotion of a third party interest, and carefully assess whether these interests are affecting treatment recommendations. Lastly, since the charge for unapproved medication is often discretionary, fees for drugs or services should be reasonable and not exploit patient vulnerability.

In sum, the off-label use of medications is not inherently unethical. In many instances, off-label treatments may be the best, or the only, available treatment, and withholding treatment would be unethical. An honest and complete discussion of the proposed treatment, its known and potential risks, realistic expectations of treatment, alternative treatments and potential financial burden is mandatory. Ultimately, the patient's best interest remains the primary motivation for any treatment.

To submit a question, contact the Ethics Committee staff at ethics@aaao.org.