

APA Official Actions

Position Statement on Off-Label Treatments

Approved by the Board of Trustees, December 2021

Approved by the Board of Trustees, July 2016

Approved by the Assembly, November 2021

Approved by the Assembly, May 2016

“Policy documents are approved by the APA Assembly and Board of Trustees. . . These are . . . position statements that define APA official policy on specific subjects. . .” – *APA Operations Manual*

Issue:

Once the FDA approves a drug for marketing, “a physician may prescribe it for uses or in treatment regimens or in patient populations that are not included in approved labeling.”¹ New uses for these drugs may have been found, and often medical evidence supports the new use. But the makers of the drugs have not put them through the formal, lengthy, and often costly studies required by FDA to officially approve the new uses.

For example, the drug is:

- Used for a different disease or medical condition.
- Given in a different way (such as by a different route).
- Given in a different dose.
- Given for a different patient population (e.g., age, gender).
- Given to patients with conditions for which the drug is contraindicated (e.g. specific medical conditions, pregnancy).
- Given in combination with another drug or drugs that are contraindicated in the label.

The questioning of off-label treatments by third party payers (both public and private) in the management of a patient’s clinical care infringes on physicians’ autonomy and clinical decision-making authority. Administrative hurdles, including additional documentation requirements and financial penalties for patients, can result in sub-optimal care.

APA Position:

The APA supports the autonomous clinical decision-making authority of a physician, including a physician’s lawful use of an FDA-approved drug product or medical device for an off-label indication. When the off-label prescription of a drug or use of a device represents safe and effective therapy, it should be:

- 1. Reimbursed by third-party payers, public and private, as reasonable and necessary medical care;**
- 2. Covered in fulfillment of payor responsibility to the beneficiary; and**
- 3. Authorized without exception when the appropriate off-label use is for a drug on formulary.**

Authors: Joseph Mawhinney, MD and Susan McLeer MD (primary); Council on Healthcare

¹ FDA Drug Bulletin Volume 12, Number 1, page 4. http://www.circare.org/fda/fdadrugbulletin_041982.pdf

Systems and Financing

Collaborators: Dr. Mark Russ, Dr. Ole Thienhaus, Dr. Laurence Miller; Council on Healthcare Systems and Financing and Dr. Steven Starks; Council on Quality Care