

Position Statement on Off-Label Treatments

Approved by the Board of Trustees, July 2016

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:

Management of a patient’s clinical care by third party payers (both public and private) has infringed on physician autonomy and clinical decision-making authority. This is especially problematic when prescribing drugs for off-label use. Management techniques, including additional administrative hurdles and financial penalties for patients, open the door for sub-optimal care.

POSITION:

The APA affirms strong support for the autonomous clinical decision-making authority of a physician and for a physician’s lawful use of an FDA-approved drug product or medical device for an off-label^[i] indication when such use is based upon sound scientific evidence in conjunction with sound medical judgment; APA encourages the use of the current drug compendia recognized by the Centers for Medicare and Medicaid Services (American Hospital Formulary Service-Drug Information, Gold Standard Inc. Clinical Pharmacology Compendium, National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium, Thomson Micromedex DrugDex[®] Compendium, Thomson Healthcare DrugPoints[®] Compendium) in conjunction with the peer-reviewed literature for determining the medical acceptability of unlabeled uses; APA further affirms that when the prescription of a drug or use of a device represents safe and effective therapy, third-party payers, including Medicare, should consider the intervention as reasonable and necessary medical care, should fulfill their obligation to their beneficiaries by covering such therapy, and should be required to cover appropriate off-label uses of drugs on their formularies.

[Footnote] [i] The FDA describes off-label use of approved drugs as “when a drug is used in a way that is different from that described in the FDA-approved drug label...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 drug for 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

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