

Off-Label Use of Pharmaceuticals

The Academy of Managed Care Pharmacy (AMCP) supports off-label use of FDA-approved drugs when medically appropriate and necessary, but opposes government-mandated coverage of specific pharmaceuticals, whether for FDA-approved or off-label uses.

Off-label use means the use of a drug for clinical indications other than those stated in the labeling approved by the U.S. Food and Drug Administration (FDA). An example would be using a drug to treat one form of cancer (e.g., pancreatic) that had been approved originally for treating other types of cancer (e.g., ovarian, bladder, breast and lung).

AMCP believes each pharmaceutical agent on the market should be used only in accordance with generally accepted standards of medical practices. AMCP also recognizes that pharmacotherapy is a rapidly changing field and that FDA-approved indications for particular drug products do not always keep pace with changing clinical protocols and medical practices. Therefore, AMCP supports having managed care organizations and third party payors consider the following criteria before deciding whether to provide coverage of FDA-approved drugs for certain off-label uses:

- Whether the drug has been proven effective and accepted for the treatment of the specific medical condition for which it has been prescribed according to the current edition of American Hospital Formulary Service - Drug Information® (AHFS-DI®), Thomson Micromedex DRUGDEX®, Clinical Pharmacology, or the National Comprehensive Cancer Network (NCCN) Drug and Biologics Compendium™.
- Whether the drug is recommended for the particular condition involved and has been proven to be safe and effective for that condition according to reproducible formal clinical studies, the results of which have been published in peer-reviewed evidence-based medical literature. Randomized, controlled trials are preferred over observational research or case studies.

AMCP does not believe government should mandate coverage of specific pharmaceuticals. Benefit design, including coverage determination, must be the responsibility of managed care organizations so they may ensure the provision of appropriate services for their members. To achieve this goal, managed care organizations must have authority to determine when to cover drugs for certain off-label uses, and in making these determinations, should take into account medical appropriateness, fiscal responsibility and principles of compassionate use.

See also AMCP's *Where We Stand on Formularies; Prescription Drug Coverage; and Direct-to-Consumer Advertising*, available online at www.amcp.org/positionstatements.

Revised by the AMCP Board of Directors, December 2008

Revised by the AMCP Board of Directors, July 1997

Adopted by the AMCP Board of Directors, February 1994