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Off-label Use: Safeguards for Drugs and Medical Devices

Pharmaceuticals and medical devices are approved or cleared by the U.S. Food and Drug Administration (FDA) for specific clinical indications. On occasion, physicians determine that, in their professional judgment, a drug or medical device may be beneficial for conditions other than those approved or cleared by the FDA. This practice, known as "off-label use," may involve:

- Prescribing a drug or using a medical device for a clinical indication or therapeutic purpose that has not been officially approved or cleared by the FDA.
- Selecting a drug dose, duration of use or mode of administration, or a medical device setting or treatment length, that differs from what the FDA has approved or cleared.
- Combining, compounding or mixing drugs into a product not approved or cleared by the FDA.
- Prescribing a drug for patients of a gender or age cohort other than the population for which it was approved or cleared by the FDA.

State practice regulations and acts generally do not address off-label use of drugs or devices by physicians or other licensed independent healthcare professionals. Nor is off-label use prohibited by the FDA, which does not regulate the practice of medicine. Nevertheless, off-label use can present significant risks to patients, providers and healthcare organizations, as non-approved applications of drugs and medical devices do not undergo the rigors of formal FDA evaluation.

Off-label use by physicians should be approved by healthcare organizations on a careful, case-by-case basis, involving analysis of relevant scientific evidence; thorough documentation of the product's proposed application, potential hazards and desired patient outcome; and adherence to established operating and quality protocols. The checklist of organizational safeguards on the following two pages is designed to help providers and administrators manage the most common professional liability risks associated with off-label product use.

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RISK CONTROL POLICIES / PROCEDURES	(Y / N)	COMMENTS
POLICY FRAMEWORK		
Is an organizational policy in place addressing off-label use of drugs and medical devices?		
Are physicians required to notify organizational leadership prior to using a drug or medical device in an off-label manner?		
Is the organization's governing body made aware that permitting off-label use of a drug or device may create potential liability for the facility itself, as well as providers?		
Do physicians rely on evidence-based, peer-reviewed medical journal articles when determining the appropriateness of off-label use?		
Do physicians and organizations accept medical and scientific data only from manufacturers that adhere to the FDA's recommended "Good Reprint Practices"? (See Additional FDA Resources, below, for the URL.)		
Is a protocol in place regarding documentation of off-label drug / medical device use and reporting of observed problems to manufacturers and appropriate authorities?		
Does the healthcare facility mandate, evaluate and act upon reports of adverse patient occurrences associated with off-label use?		
Has the organization evaluated the advantages and disadvantages of voluntarily reporting off-label drug- and device-related mishaps to the FDA via MedWatch, as well as to designated patient safety organizations and oversight programs? (See Additional FDA Resources, below, for the MedWatch URL.)		
Does the organization have a written procedure for responding to the recall of off-label drugs and medical devices, and does this procedure afford timely notice to affected patients?		
INTERNAL REVIEW		
Is the current FDA-approved label for drugs and devices – including boxed warnings, contraindications, drug-drug interactions and adverse effects listing – thoroughly reviewed prior to proposed off-label use?		
Are specific patient risk factors and characteristics – such as underlying chronic diseases, concomitant medications, gender and age – considered when deciding whether to use a product off-label?		
Is consideration given to the length of time the approved drug or device has been on the market and the frequency of its off-label use?		
Is a written report of the scientific and medical evidence supporting the off-label use submitted beforehand to a peer review committee, internal review board, or pharmacy and therapeutics committee?		
If off-label-use drugs are prepared by a compounding pharmacy, is the pharmacy appropriately credentialed and licensed in the dispensing state?		
Are records regarding off-label use of drugs and devices stored in a centralized database?		
Do records of off-label drug or device use include patient and provider identifiers, dates of usage and serial / lot numbers?		
Is professional liability insurance coverage verified before the organization authorizes off-label use of a drug or device?		
Does the organization determine in advance if off-label use of a medical device would void its warranty?		

RISK CONTROL POLICIES / PROCEDURES	(Y / N)	COMMENTS
PATIENT SELECTION AND DOCUMENTATION		
Is a thorough patient history and examination conducted prior to treatment, in order to rule out any underlying condition or existing therapy that may make a specific off-label treatment inadvisable?		
Are "on-label" (i.e., approved) medical treatments considered prior to off-label options, when clinically indicated?		
Does the patient healthcare information record contain all relevant information, including: - The intended off-label use? - Potential risks, including side effects and adverse interactions, as well as contraindications?		
Is the decision-making process for off-label use carefully documented, including diagnostic findings, results of earlier treatment attempts and rationale?		
Does the informed consent process reflect a shared-decision model, whereby practitioners educate patients and fully answer their questions about the medical indicators for off-label use, as well as discussing risks, benefits and on-label alternatives?		
Does the patient sign a standardized informed consent form for off-label use, including: - Details of the treatment, rendered in easily understood terms? - A list of the known risks and complications associated with the off-label application? - A warning that, as off-label use is inherently experimental, unforeseen complications may occur and a successful outcome cannot be guaranteed? - A clear statement that the patient is not obligated to consent to the off-label treatment?		
Are discharge and follow-up instructions provided in writing, including appropriate responses to potentially dangerous side effects or other adverse outcomes?		
COMMUNICATION		
Are physicians required to disclose financial arrangements with product manufacturers in grand rounds, educational programs, and other activities that may involve discussing the off-label use of drugs or medical devices?		
Are providers instructed to beware of aggressive sales and marketing tactics – including misleading claims with regard to product safety and efficacy, or suppression of adverse clinical data – which may compromise the credible exchange of scientific information on new product uses?		
Are providers who intend to use a product off-label required to evaluate and report to management the potential hazards and alternatives prior to use?		
Does the provider's evaluation include a thorough review of the product manufacturer's marketing materials and warnings?		
Are products purchased only from companies that appear to refrain from aggressive marketing techniques, such as openly supporting off-label uses, offering incentives to physicians for off-label use or guaranteeing positive outcomes in promotional materials?		

ADDITIONAL FDA RESOURCES:

- Center for Drug Evaluation and Research, at **http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/default.htm**.
- Guidance for Industry: "Distributing Scientific and Medical Publications on Unapproved New Uses: Recommended Practices" (revised draft guidance), February 2014, at **http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM387652.pdf**.
- Guidance for Industry: "Good Reprint Practices for the Distribution of Medical Journal Articles and Medical or Scientific Reference Publications on Unapproved New Uses of Approved Drugs and Approved or Cleared Medical Devices," January 2009, at **http://www.fda.gov/RegulatoryInformation/Guidances/ucm125126.htm**.
- Guidance for Industry: "Responding to Unsolicited Requests for Off-Label Information about Prescription Drugs and Medical Devices" (draft guidance), December 2011, at **http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM285145.pdf**.
- Guidance for Institutional Review Boards and Clinical Investigators: "'Off-Label' and Investigational Use Of Marketed Drugs, Biologics, and Medical Devices – Information Sheet," updated August 2011, at **http://www.fda.gov/RegulatoryInformation/Guidances/ucm126486.htm**.
- Manufacturer and User Facility Device Experience database (MAUDE), at **http://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfMAUDE/Search.cfm**.
- MedWatch: The FDA Safety Information and Adverse Event Reporting Program, at **http://www.fda.gov/Safety/MedWatch/default.htm**.

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