

Medication Policy Manual

Policy No: dru031

Topic: Off-Label Use of FDA-Approved Drugs

Date of Origin: September 1997

Revised/Effective Date: August 18, 2009

Next Review Date: August 2010

IMPORTANT REMINDER

This Medical Policy has been developed through consideration of medical necessity, generally accepted standards of medical practice, and review of medical literature and government approval status.

Benefit determinations should be based in all cases on the applicable contract language. To the extent there are any conflicts between these guidelines and the contract language, the contract language will control.

The purpose of medical policy is to provide a guide to coverage. Medical Policy is not intended to dictate to providers how to practice medicine. Providers are expected to exercise their medical judgment in providing the most appropriate care.

Description

This policy provides guidance to medication policy developers and clinical reviewers to determine whether a particular medication may need to be reviewed for medical necessity or may be covered without review.

For FDA-approved indications, also known as labeled indications, the FDA has reviewed and approved the drug for the specified indications for final marketing based on adequate, well-controlled clinical trials which have documented safety and effectiveness. The use of a medication for conditions or indications other than those approved by the FDA is known as “off-label use”.

Under the Federal Food, Drug and Cosmetic (FD&C) Act, pharmaceutical manufacturers can only promote, advertise, and include information on the package insert for FDA approved indications. However, the FD&C Act does not limit the indications or manner in which a physician prescribes the drug. Such off-label use may be appropriate and rational in certain circumstances.

Regence considers vaccines to be medically necessary as of the effective date of a recommendation made by any of the following:

- U.S. Preventive Services Task Force
- American Academy of Pediatrics
- Advisory Committee on Immunization Practices

Vaccines are reimbursed according to the member's available benefits.

Policy/Criteria

- I.** Medications that have an IND designation (Investigational New Drug) have not received final approval from the FDA and may not be marketed. (10)

An approved NDA or ANDA (New Drug Application or Abbreviated New Drug Application) is considered final FDA-marketing approval for the purposes of this policy. A medication's NDA or ANDA may be located at:
www.accessdata.fda.gov/scripts/cder/drugsatfda/

The FDA may require post marketing surveillance for certain medications that have received final marketing approval.

The term "orphan drug" refers to a product that treats a rare disease affecting fewer than 200,000 Americans. Orphan drugs may be in either an IND or NDA status. For the purposes of this policy, orphan drug status has no significance in the evaluation of off label treatments.

- II.** Off-label use of medications and vaccines that have received final FDA approval for marketing may be reviewed for medical necessity and / or investigational uses when any of the following criteria are met:

- A.** There is an existing medication policy for the prescribed medication and Regence requires prior authorization for the medication.

OR

- B.** The member's prescription benefit includes a closed formulary and the medication is non-formulary/not preferred.

OR

- C.** The member's prescription benefit includes an option for copayment tier exceptions and the medication is non-formulary/not preferred.

OR

- D.** The medication is prescribed by a practitioner who is subject to audit by Regence.

III. Requests for coverage of off-label uses of medications and vaccines not meeting any of the criteria I A through I D above will not be routinely reviewed for medical necessity.

IV. Medications and vaccines that have not received final FDA marketing approval for any indication are considered investigational.

Cross References
Medication Policy Manual Introduction dru150
Non-Formulary Medications, Medical Exception Criteria for Closed Benefit Designs dru116
Non-Preferred Medications, Medical Exception Criteria for Tiered Benefit Designs with Tier Copay Exceptions dru125
Research Urgent Treatments med74.htm, TRG Medical Policy Manual, TRGMPPM - Medicine

Codes	Number	Description
N/A		

References:

1. http://www.fda.gov/cder/regulatory/applications/ind_page_1.htm#Introduction (Verified 3/13/09)
2. <http://www.fda.gov/cder/regulatory/applications/NDA.htm> (Verified 3/13/09)
3. <http://www.fda.gov/cder/handbook/orphan.htm> (Verified 3/13/09)