Off-Label Drug Use

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

Off-label drug use is the use of a drug approved by the U.S. Food and Drug Administration (FDA) for other uses or in treatment regimens or patient populations that are not included in approved labeling.

The FDA approves drugs for specific indications that are included in the drug's labeling. When a drug is used for an indication other than those specifically included in the labeling, it is referred to as an off-label use. Many off-label uses are effective, well-documented in the literature, and widely used.

Unapproved uses of drugs include a variety of situations ranging from completely unstudied to thoroughly investigated drug uses where the FDA has not been asked for approval, whereas approved uses of drugs have been proven to be safe and effective by the FDA after the review of adequate and controlled clinical trials that have documented their use.

POLICY

Criteria for Initial Approval

The off-label use of a drug may be considered **medically necessary** when the following criteria are met:

I. The requested drug has NOT been excluded from coverage by Wellmark due to lack of demonstrated safety or efficacy, clinical benefit, or administrative program (i.e. exclusion at launch while awaiting P&T review, plan exclusions).
II. The patient has a documented history of a trial and failure, contraindication, or intolerance to established FDA approved and/or clinical guideline recommended therapies (if applicable) used to treat or manage the disease or condition.

III. The requested dosing frequency and duration of use falls within the manufacturer's published dosing guidelines or within dosing guidelines found in the compendia of current literature (examples: package insert, Drug Facts & Comparisons, current accepted consensus-based guidelines).

IV. The diagnosis is clinically supported as a use by at least one of the following:

A. One of the following compendia:

- Drug Facts & Comparisons® Level of Evidence A
- National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium™ Category of Evidence and Consensus 1 or 2A

OR

B. Scientific evidence demonstrating efficacy and safety for the requested use. The evidence must:

- Consist of an adequate number of well-designed studies with sufficient numbers of patients in relation to the incidence of the disease.
- Be published in major peer-reviewed journals that publish original manuscripts only after the manuscripts have been critically reviewed by unbiased independent experts for scientific accuracy, validity, and reliability.
- Demonstrate consistent results across all studies.
- Establish appropriate dosing frequency and duration of use.
- Document positive health outcomes and demonstrate that the drug is as effective as or more effective than established alternatives.
- Document improvements that are attainable outside the investigational setting.

If criterion I, II, III and IV have not been satisfied, the requirements of the off-label drug use policy have not been met.

Approval will be for the **duration of prescription/treatment course up to 12 months.**

Continuation of Therapy

The request for continuation of therapy may be considered medically necessary when ALL initial authorization criteria are met AND clinical documentation is provided showing the patient has achieved and/or maintained a positive clinical response with the requested therapy.

Approval will be for the **duration of prescription/treatment course up to 12 months.**

Note: Prescribers must submit clinical documentation supporting the drug's safety and effectiveness in treating the intended indication.

Dosage and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

CLINICAL RATIONALE

Facts & Comparisons® eAnswers is a drug compendia that provides up-to-date, comprehensive drug information, including off-label uses. An editorial review panel consisting of practicing physicians and pharmacists assign a documentation level to each monograph with level A being good and level G being poor. According to Facts and Comparisons, this panel critically evaluates monograph data and provides an

authoritative consensus about whether the reviewed published information is considered sufficient to warrant prescribing in appropriate populations. For the purpose of this policy, indications listed in Facts and Comparisons® eAnswers is determined medically necessary when the Level of Evidence is A.

The National Comprehensive Cancer Network (NCCN) Drug & Biologics Compendium is a listing of agents with both FDA-approved indications and NCCN designated off-label indications derived from the NCCN Clinical Practice Guidelines in Oncology. As stated by NCCN, these off-label uses are based upon evaluation of evidence from literature combined with expert judgment in an evidence-based method. Each recommendation is designated with a Category of Evidence that reflects the quality of evidence and consensus on which the recommendation is based. For the purpose of this policy, indications listed in NCCN Drug and Biologics Compendium with a Category of Evidence and Consensus 1 or 2A are considered medically necessary.

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- Code(s), if applicable.

REFERENCES

- U.S. Food and Drug Administration (FDA). Off-label and investigational use of marketed drugs, biologics, and medical devices. Available at:
- http://www.fda.gov/RegulatoryInformation/Guidances/ucm126486.htm.
- DrugDex® System (database online). Greenwood Village, CO: Thomson Micromedex. Available at: http://www.micromedexsolutions.com.
- Facts and Comparisons® (database online). St. Louis, MO: Wolters Kluwer Health, Inc. Available at: http://www.factsandcomparisons.com.
- National Comprehensive Cancer Network®. NCCN Drugs & Biologic Compendium™ (database online). Available at: http://www.nccn.org.

POLICY HISTORY

Policy #: 05.01.09
Policy Creation: July 2005
Reviewed: October 2021
Revised: October 2020
Effective: October 28, 2020