

Off-Label Use

Policy PHARM-049

Origination Date: 06/02/2015

Reviewed/Revised Date: 05/20/2020

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Current Effective Date: 06/01/2020

Disclaimer:

1. Policies are subject to change in accordance with Federal and State notice requirements.
2. Policies outline coverage determinations for all members and clients of University of Utah Health Plans. Refer to the “Policy” and “Lines of Business” section for more information.

Purpose

To define the conditions under which off-label drug use may be covered.

Definitions

1. Off-label drug use is the use of an approved drug by the U.S. Food and Drug Administration (FDA) for uses in treatment that have not been included in the drug information labeling.
2. The FDA approves drugs for specific indicated use(s) that are listed in the drug labeling. When a drug is being used in a non-approved indication, this treatment is considered an off label use. Off label uses of the drug may be considered effective and are well documented in literature.
3. Unapproved treatment uses of the drugs are used in a variety of situations from being completely un- or understudied to having been investigated and the FDA hasn't been asked to include this in the approval. Approved uses have proven to be safe and effective by the FDA after review showing that the studies are adequate and have also gone through the clinical trials process.

Policy/Coverage

1. Off-Label Use Criteria

A. Authorization Criteria

- i. Rare or Orphan diseases will be reviewed case by case and with Medical or Pharmacy Director Review. A rare disease is defined as a condition that affects fewer than 200,000 people in the US and is recognized by NORD (National Organization for Rare Disorders). See www.raredisease.org
- ii. All of the following criteria must be met for off-label drug use to be considered medically necessary. Documentation must be provided.

- a. The drug is approved by the FDA.
- b. The patient has tried and failed or has a contraindication to one of the following:
 - 1) FDA approved drugs
 - 2) Clinical guidelines recommended therapy.
- c. The requested off-label use is supported by at least one of the following:
 - 1) Thomson Micromedex DrugDex[®] meeting each of following:
 - A) Strength of Recommendation Class I or IIa and
 - B) Strength of Evidence Category A or B and
 - C) Efficacy Class I or IIa
 - 2) Facts & Comparisons[®] documentation level 1 or 2
 - 3) Established off-label use per Lexicomp
 - 4) National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium[™] Category of Evidence and Consensus 1,2A, or 2B
 - 5) Two articles from major scientific or medical peer-review journals (this excludes case reports, letters, poster or abstracts, with the exception of Orphan Diseases), or one major multi-site study or three smaller studies that have validating and uncontested data, which supports the proposed use of the drug and is shown to be safe and effective for the condition to be treated.
 - A) Examples of accepted journals include, but are not limited to, American Journal of Medicine, Clinical Cancer Research, Journal of American Medical Association, Journal of Clinical Oncology, and New England Journal of Medicine.

2. Clinical Rationale

- A. Thomson Micromedex DrugDex[®] is a drug compendia that includes the approved FDA uses and off-label indications. The off-label indications are identified through monitoring literature and information provided by the FDA, NIH, and CDC. DrugDex[®] uses a rating system of Strength of Recommendation, Strength of Evidence, and Efficacy. For this policy, the indication will be determined as medically necessary when the Strength of Recommendation is class I or IIa, the Strength of Evidence is category A or B, and the Efficacy class is I or II.²
- B. Facts & Comparisons[®] is drug compendia that provides up-to-date information of all FDA approved indications and off-label uses. A review panel, consisting of practicing physicians and pharmacists, rate document monographs from level 1 (good) to level 5 (poor). For this policy, an off-label indication will be determined as medically necessary when the documentation is a level 1 (good) or level 2 (fair).³
- C. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium[™] is a listing of approved treatments as well as NCCN off-label indications taken from the clinical practice guidelines in oncology. The off-label indications have been selected with expert judgment using the evidence based method. For the purpose of this policy, the indications listed with the NCCN

Category of Evidence and Consensus 1 or 2A, or 2B will be determined as medically necessary.⁴

3. Exclusions/Contraindications

- A. The prior use of samples will not be considered in the determination of a member's eligibility for coverage for this medication.

Lines of Business

1. University of Utah Health Plans

- A. Healthy U
- B. Commercial Groups
- C. Individual Exchange

2. Mountain Health Co-Op

- A. Commercial Groups
- B. Individual Exchange

3. Medicare Advantage

- A. Advantage U

References:

1. Anthem Blue Cross Blue Shield. Off-Label Drug and Approved Orphan Drug Use. Available at: http://www.anthem.com/ca/medicalpolicies/guidelines/gl_pw_a048553.htm. Updated February 5, 2015, Accessed on May 29, 2015.
2. DrugDex® System [online database]. Truven Health Analytics, Greenwood Village, Colorado, USA. Available at: <http://www.micromedexsolutions.com/>. Accessed on May 29, 2015.
3. Facts & Comparisons® (electronic version). St. Louis, MO: Wolters Kluwer Health. [Updated 2015; Accessed on May 29, 2015]. Available at: <http://online.factsandcomparisons.com/Viewer.aspx?book=OLF&monoID=fandc-olf5195>.
4. National Comprehensive Cancer Network®. NCCN Drugs & Biologic Compendium™. Available at: <http://www.nccn.org>. Accessed on May 29, 2015.
5. U.S Food and Drug Administration. 'Off-Label' and Investigational Use of Marketed Drugs, Biologics, and Medical Devices. Available at:
<http://www.fda.gov/RegulatoryInformation/Guidances/ucm126486.htm>. Accessed on May 29, 2015.
7. Wellmark Blue Cross Blue Shield. Off-Label Drug Use. Available at: https://www.wellmark.com/Provider/MedPoliciesAndAuthorizations/MedicalPolicies/policies/Off-label_Drug_Use.aspx. Accessed on May 29, 2015.
8. The National Organization for Rare Disorders <https://rarediseases.org/>
9. Lexicomp® [online database]. © 2020 Wolters Kluwer Clinical Drug Information, Inc. and its affiliates and/or licensors. All Rights Reserved.

Revision Date	Revision
06/02/2015	Policy created.
04/06/2016	Policy reviewed and approved by P&T Committee.
01/25/2017	Policy reviewed and approved by P&T Committee.
04/18/2018	Policy reviewed and approved by P&T Committee.
05/06/2019	Policy sent to P&T Committee for review.
05/09/2019	Policy approved by P&T Committee.
05/12/2020	Added Lexicomp as a reference Added the definition to orphan/rare disease and NORD as a reference
06/01/2020	Policy reviewed and approved by P&T Committee.

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The codes for treatments and procedures applicable to this policy are included for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

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