

## Off-Label Use

**Policy:** PHARM-049

**Origination Date:** 06/02/2015

**Reviewed/Revised Date:** 03/27/2024

**Next Review Date:** 03/27/2025

**Current Effective Date:** 04/01/2024

### Disclaimer:

1. Policies are subject to change in accordance with Federal and State notice requirements.
2. Policies outline coverage determinations University of Utah Health Plans Commercial. Refer to the "Policy" and "Lines of Business" section for more information.
3. Services requiring prior-authorization may not be covered, if the prior-authorization is not obtained.
4. This Pharmacy Policy does not guarantee coverage or payment of the service. The service must be a benefit in the member's plan and the member must be eligible for coverage at the time of service. Additional payment guidelines may be applied that are not included in this policy.

### 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 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 with the Medical or Pharmacy Director. 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 <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>

- ii. All of the following criteria must be met for off-label drug use to be considered medically necessary. Documentation must be provided.
  - a. Coverage options such as clinical trials and expanded use access have been exhausted
    - 1) See <https://www.fda.gov/news-events/public-health-focus/expanded-access>; [ClinicalTrials.gov](http://ClinicalTrials.gov)
  - b. The drug is approved by the FDA.
  - c. The patient has tried and failed, or has a contraindication to ALL of the following if applicable:
    - 1) Drugs labeled by the FDA for requested condition
    - 2) Recommended therapies per Clinical Guidelines
  - d. The requested off-label use is supported by at least one of the following:
    - 1) Thomson Micromedex DrugDex<sup>®</sup> 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) Lexicomp
    - 3) National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium™ Category of Evidence and Consensus 1, 2A, or 2B
    - 4) Qualified articles from major scientific or medical peer-review journals. Qualified articles include at least one large, multi-centered and prospective, double blinded and randomized trial OR at least three small high-quality trials with appropriate controls when a large multi-center study is not possible. Articles must include validating and uncontested data supporting the proposed safety and efficacy for the use of the drug in the requested disease state.
      - 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. 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.
- B. Requests that do not meet above criteria are considered experimental/investigational and are not a covered benefit (See plan benefit coverage documents)

## Lines of Business

### 1. University of Utah Health Insurance Plans

- A. Commercial
- B. MHC

#### References:

1. Anthem Blue Cross Blue Shield. Off-Label Drug and Approved Orphan Drug Use. Available at: <https://www.bcbsks.com/medical-policies/label-approved-orphan-and-expanded-access-compassionate-use-drugsUpdated08/8/2023.> , Accessed 03/13/2024.
2. DrugDex® System [online database]. Truven Health Analytics, Greenwood Village, Colorado, USA. Available at: [http://www.micromedexsolutions.com/.](http://www.micromedexsolutions.com/) Accessed 03/13/2024.
3. Lexi-Drugs Off-Label Uses Policy <https://www.wolterskluwer.com/en/solutions/uptodate/about/notices/lexidrug-off-label-uses-policy> Accessed 03/13/2024
4. National Comprehensive Cancer Network®. NCCN Drugs & Biologic Compendium™. Available at: <http://www.nccn.org>. Accessed 03/13/2024.
5. U.S Food and Drug Administration. ‘Off-Label’ and Investigational Use of Marketed Drugs, Biologics, and Medical Devices. Available at: <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/label-and-investigational-use-marketed-drugs-biologics-and-medical-devices>. Accessed 03/13/2024
6. Wellmark Blue Cross Blue Shield. Off-Label Drug Use. Available at: [https://www.wellmark.com/-/media/sites/public/files/medical-policies/off-label-drug-use.pdf?sc\\_lang=en&hash=FDFO58C93DE30C25B8A5B0C2847E56D0](https://www.wellmark.com/-/media/sites/public/files/medical-policies/off-label-drug-use.pdf?sc_lang=en&hash=FDFO58C93DE30C25B8A5B0C2847E56D0) . Accessed 03/13/2024.
7. The National Organization for Rare Disorders <https://rarediseases.org/>
8. 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.
01/28/2021	Updated Lines of Business
05/11/2021	<ul style="list-style-type: none"><li>• Updated Rare diseases website</li><li>• Removed Facts and Comparisons as a reference to guide use as there is no access to Facts and Comparisons with current U of U subscriptions.</li><li>• Added Lexicomp as reference</li><li>• Clarified language defining the requirements for medical literature that may be considered to allow off-label use</li></ul>
05/19/2021	Policy reviewed and approved by P&T Committee. Effective 06.01.2021
01/01/2022	Removed University of Utah Health Plans (Healthy U, Healthy U Integrated) from Lines of Business to create Healthy U specific policy.

	Removed Medicare Advantage from University of Utah Health Insurance Plans to create Medicare Advantage specific policy.
05/11/2022	Completed annual review of policy. Removed: Case reports, case series without control cohort, letters, posters, and abstracts are not qualified articles but may be considered for rare or orphan diseases as indicated above Added: Requests that do not meet above criteria are considered experimental/investigational and are not a covered benefit (See plan benefit coverage documents)
05/18/2022	Policy reviewed and approved by the P&T Committee. Policy effective 06.01.2022
03/13/2024	Completed annual review of policy.
03/27/2024	Policy reviewed and approved by the P&T Committee. Policy effective 04.01.2024

**Disclaimer:**

This document is for informational purposes only and should not be relied on in the diagnosis and care of individual patients. Medical and Coding/Reimbursement policies do not constitute medical advice, plan preauthorization, certification, an explanation of benefits, or a contract. Members should consult with appropriate health care providers to obtain needed medical advice, care, and treatment. Benefits and eligibility are determined before medical guidelines and payment guidelines are applied. Benefits are determined by the member’s individual benefit plan that is in effect at the time services are rendered.

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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