Off-Label Use

Policy: PHARM-HU-049

Origination Date: 01/01/2022

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 Healthy U (Medicaid). 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
   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® 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 Plans**
    A. Healthy U
    B. Healthy U Integrated

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-drugsUpdated 08/8/2023. , Accessed 03/13/2024.
2. DrugDex® System [online database]. Truven Health Analytics, Greenwood Village, Colorado, USA. Available at: 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=FDF058C93DE30C25B8A5B0C2847E56D0 . 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
01/01/2022	Healthy U specific policy created. Separated out from PHARM-049
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

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.

U of U Health Plans makes no representations and accepts no liability with respect to the content of any external information cited or relied upon in this policy. U of U Health Plans updates its Coverage Policies regularly, and reserves the right to amend these policies and give notice in accordance with State and Federal requirements.