Off-Label Use

Policy: PHARM-049

Origination Date: 06/02/2015

Reviewed/Revised Date: 05/19/2021

Next Review Date: 05/19/2022

Current Effective Date: 06/01/2021

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 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). Medical necessity may also be reviewed on a case by case basis with Medical or Pharmacy Director as

needed. 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. 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) 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 trials OR at least three small high-quality trials with appropriate controls when a large multi-center study is not possible. 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. 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.

Lines of Business

1. University of Utah Health Insurance Plans

A. Medicare Advantage
B. Commercial
C. MHC

2. University of Utah Health Plans

A. Healthy U

B. Healthy U Integrated

References:

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2. DrugDex® System [online database]. Truven Health Analytics, Greenwood Village, Colorado, USA. Available at: http://www.micromedexsolutions.com/. Accessed on May 29, 2015.
3. Lexi-Drugs Off-Label Uses Policy https://www.wolterskluwer.com/en/solutions/lexicomp/resources/clinical-notices/lexi-drugs-off-label-uses-policy accessed 04/26/2021
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:
6. 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.
01/28/2021	Updated Lines of Business
05/11/2021	• Updated Rare diseases website • 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. • Added Lexicomp as reference • Clarified language defining the requirements for medical literature that may be considered to allow off-label use
05/19/2021	Policy reviewed and approved by P&T Committee. Effective 06.01.2021