ADMINISTRATIVE POLICY STATEMENT
INDIANA MEDICAID

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06/06/2013	06/15/2019		06/15/2018
Policy Name			**Policy Number**
Medical Necessity – Off Label, Approved Orphan and Compassionate Use Drugs			AD-0060
Policy Type			
Medical	**ADMINISTRATIVE**	Pharmacy	Reimbursement

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A. SUBJECT

Medical Necessity – Off Label, Approved Orphan and Compassionate Use Drugs

B. BACKGROUND

The U.S. Food and Drug Administration (FDA) approves drugs for specific indications included in the drug's product information label. Off-label or "unlabeled" drug use is the utilization of an FDA approved drug for uses other than those listed in the FDA approved labeling or in treatment regimens or populations that are not included in approved labeling. Many off-label uses are effective, well documented in the peer-reviewed literature and widely used even though the manufacturer has not pursued the additional indications.

The FDA advises physician's use of off-label or "unlabeled" drugs must be done in a well-informed manner in conjunction with firm scientific rationale and medical evidence. Physicians must also maintain records of the product's use and effects.

CareSource will employ, at its discretion, drug utilization management programs (i.e., prior authorization) to ensure appropriate and safe use of medications.

NOTE: The Introduction section is for your general knowledge and is not to be construed as policy coverage criteria. The rest of the policy uses specific words and concepts familiar to medical professionals and is intended for providers. A provider can be a person, such as a doctor, nurse, psychologist, or dentist. A provider can also be a place where medical care is given, like a hospital, clinic or lab. This policy informs providers about when a service may be covered.

C. DEFINITIONS

- **FDA Approved medication:** Is the official description of a drug product which includes indication; who should take it; adverse events; instructions for uses in pregnancy; children, and other populations; and safety information for the patient. Labels are often found inside drug product packaging.
- **Off-label or "unlabeled" drug use:** Is the use of a drug approved by the U.S. Food and Drug Administration (FDA) for other uses 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.
- **Orphan Drug:** Is a drug or biologic (i.e., blood or vaccine product) that treats a rare disease (e.g., affecting fewer than 200,000 Americans). Products have FDA orphan drug approval when they meet the orphan drug criteria established by the FDA. The intent of the Orphan Drug Act (ODA) is to stimulate the research, development, and approval of products that treat rare diseases. Orphan designation can be obtained prior to submission of a marketing application. The safely and efficacy of the drug must be established through clinical studies. If the designated product meets the standard FDA regulatory requirements and process for obtaining marketing approval, it is given an FDA approved orphan drug designation status (i.e., "Designated/Approved"). Over 1700 drugs and biologics have been designated as orphan drugs and over 300 have been approved for marketing.
- **Expanded Access:** Refers to the use of an investigational new drug (IND) outside of a clinical trial by patients with serious or life-threatening conditions who do not meet the enrollment criteria for the clinical trial in progress. This type of access may be available may be available, in accordance with United States Food and Drug

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Administration (FDA) regulations, when it is clear that patients may benefit from the treatment, the therapy can be given safely outside the clinical trial setting, no other alternative therapy is available, and the drug developer agrees to provide access to the drug. The FDA refers to such a program as an expanded access program (EAP). EAPs can be used in a wide range of therapeutic areas including HIV/AIDS and other infectious diseases, cancer, rare diseases, and cardiovascular diseases. There are several types of EAPs allowed in the United States. Treatment protocols and treatment INDs provide large numbers of patient's access to investigational drugs. A single-patient IND is a request from a physician to the FDA that an individual patient be allowed access to an investigational drug on an emergency or compassionate use basis.

D. POLICY

CareSource will review prior authorization requests for the use of medications and consider the use to be medically necessary when the following criteria have been met for situations as listed below. This policy will not supersede drug-specific criteria developed and approved by the CareSource Pharmacy and therapeutics Committee (P&T). CareSource Pharmacy department will keep track of all off-label, approved orphan and compassionate use requests submitted to use for analysis and trending for potential recommendations for changes in the formulary

I. Experimental or Investigational

CareSource will review available scientific data and seek opinions of experts in a particular field and opinions and assessments of nationally recognized review organizations may also be considered by the Plan but are not determinative or conclusive.

A. Off-Label Drug Use

1. Off-label use of cancer drugs:

1.1 Off-labeled use of an FDA approved prescription drug for cancer treatment **is covered** if the prescription drug is recognized for treatment of the indication in **ONE** of the following:

a. National Comprehensive Cancer Network (NCCN) Drug & Biologics Compendium Category of Evidence and Consensus 1

b. In TWO substantially accepted peer-reviewed medical literature documents

1.2 For experimental / investigational chemotherapy drugs (not FDA approved), deny as **experimental / investigational**

2. Off-label drug use for non-cancer drugs is considered **medically necessary** when **ALL** the following conditions are met:

2.1 The drug is approved by the U.S. Food and Drug Administration

2.2 The prescribed drug use is supported in any **ONE OR MORE** of the following:

a. American Hospital Formulary Service Drug Information (AHFS) or Clinical Pharmacology or Micromedex:

1. Strength of Recommendation Class I
2. Strength of Evidence Category A or B
3. Efficacy Class I

2.3 Evidence from **TWO** published studies from major scientific or medical peer reviewed journals that are < 5 years old preferred and < 10 years required to support the proposed use for the specific medical condition as safe and effective in persons aged 18 and over. (Accepted study designs

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include, but are not limited to, randomized, double blind, placebo controlled trials). For persons less than 18, to protect vulnerable minors according to US standards, studies must be approved by a United States (US) institutional review board (IRB) accredited by the Association for the Accreditation of Human Research Protection Programs, Inc. (AAHRPP).

II. Orphan Drug Use

A. Use of an orphan drug is considered **medically necessary** when it receives FDA Orphan Drug designation and marketing approval ("Designated/Approved")
B. A product may have an orphan drug designation but fail to meet the criteria to have FDA marketing approval. Use of a product with orphan drug designation alone without FDC marketing approval is considered **not medically necessary**.

III. Expanded Access (Compassionate Use) Drugs

A. Expanded Access (Compassionate Use) Drugs (e.g. when a single patient IND (investigational new drug) requests is approved by the FDA on a compassionate use basis) are considered **experimental / investigational** but may be covered if Research Urgent or Off-label Drug use requirements are met.

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.

E. CONDITIONS OF COVERAGE

HCPCS
CPT

AUTHORIZATION PERIOD
Approved authorizations are designated an appropriate authorization period. Continued treatment may be considered when the member has shown biological response to treatment. **ALL** authorizations are subject to continued eligibility.

F. RELATED POLICIES/RULES

N/A

G. REVIEW/REVISION HISTORY

DATES		ACTION
Date Issued	06/06/2013	
Date Revised	10/30/2014	Added definition to excluded indications
	05/05/2015	Removed indications in reference of plan specific member handbooks, EOC, etc. Removed specialty and subspecialty associations and combined with no determinations policy
	12/15/2015	Revised class/category and defined evidence criteria for article submissions
	01/11/2018	Updated format
Date Effective	06/15/2018	

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H. REFERENCES

1. 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
2. U.S. Food and Drug Administration (FDA). Orphan Product Designations and Approval Search. Available at: http://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm.
3. U.S. Food and Drug Administration (FDA). Developing Orphan Products: FDA and Rare Disease Day. Last updated February 16, 2016. Available at: http://www.fda.gov/ForConsumers/Consumer Updates/ucm107293.htm.
4. National Comprehensive Cancer Network®. NCCN Drugs & Biologic Compendium™ (electronic version). Available at: http://www.nccn.org.

The Administrative Policy Statement detailed above has received due consideration as defined in the Administrative Policy Statement Policy and is approved.

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