Administrative Policy Statement
GEORGIA MARKETPLACE PLANS

Policy Name		Policy Number	Date Effective
Medical Necessity – Off Label		PAD-0026-GA-MPP	01/01/2022
Policy Type			
Medical	**ADMINISTRATIVE**	Pharmacy	Reimbursement

Administrative Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) are derived from literature based on and supported by clinical guidelines, nationally recognized utilization and technology assessment guidelines, other medical management industry standards, and published MCO clinical policy guidelines. Medically necessary services include, but are not limited to, those health care services or supplies that are proper and necessary for the diagnosis or treatment of disease, illness, or injury and without which the patient can be expected to suffer prolonged, increased or new morbidity, impairment of function, dysfunction of a body organ or part, or significant pain and discomfort. These services meet the standards of good medical practice in the local area, are the lowest cost alternative, and are not provided mainly for the convenience of the member or provider. Medically necessary services also include those services defined in any Evidence of Coverage documents, Medical Policy Statements, Provider Manuals, Member Handbooks, and/or other policies and procedures.

Administrative Policy Statements prepared by CSMG Co. and its affiliates (including CareSource) do not ensure an authorization or payment of services. Please refer to the plan contract (often referred to as the Evidence of Coverage) for the service(s) referenced in the Administrative Policy Statement. If there is a conflict between the Administrative Policy Statement and the plan contract (i.e., Evidence of Coverage), then the plan contract (i.e., Evidence of Coverage) will be the controlling document used to make the determination.

Table of Contents

A. Subject

Medical Necessity – Off Label, Approved Orphan and Compassionate 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. 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.

D. Policy

CareSource will review prior authorization requests for coverage based on medical necessity. This policy will not supersede drug-specific criteria developed and approved by the CareSource Pharmacy and Therapeutics Committee (P&T).

Requests for off-label uses of a drug will be considered for approval according to the following criteria:

I. Documentation must be submitted showing the member has tried and failed the existing FDA approved and/or clinical guideline recommended therapies unless contraindicated or not tolerated; AND

II. The prescribed use must be supported by one or more of the following:
   a. Narrative information from American Hospital Formulary Service Drug Information (AHFS) or Clinical Pharmacology
   b. Lexicomp: Evidence level A

c. Micromedex: Recommendation class I, IIa, or IIb
d. Evidence from at least two published studies from major scientific or medical peer reviewed journals demonstrates safety and efficacy for the specified condition in a comparable population (i.e. age group, level of disease severity, etc.)
If applicable clinical trial is yet to be published but interim results are supportive, this may be taken into consideration by the clinician reviewer.

NOTE: For off-label use of oncology drugs, please refer to the policy titled "Oncology Treatment Regimen Review" accessible from the CareSource website.

E. Conditions of Coverage

AUTHORIZATION PERIOD

Approved authorizations are designated an appropriate authorization period. Continued treatment may be considered when the member has shown tolerability and a positive clinical response.

F. Related Policies/Rules

Oncology Treatment Regimen Review

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
	06/12/2020	Policy moved to a new template
	11/30/2021	General edits for clarity. Added note about clinical trials in progress. Removed content related to orphan drugs and compassionate use. Removed cancer drug section and refer to separate policy. Added component that member must try and fail available FDA approved on label drugs first. Amended list of acceptable compendia to include Lexicomp. Updated reference section.
Date Effective	**01/01/2022**	
Date Archived		

H. References

1. U.S. Food and Drug Administration (FDA). Off-label and investigational use of marketed drugs, biologics, and medical devices. Available at: www.fda.gov
2. U.S. Food and Drug Administration (FDA). Orphan Product Designations and Approval Search. Available at: www.accessdata.fda.gov
3. U.S. Food and Drug Administration (FDA). Developing Orphan Products: FDA and Rare Disease Day. Last updated February 16, 2016. Available at: www.fda.gov
4. National Comprehensive Cancer Network®. NCCN Drugs & Biologic Compendium™ (electronic version). Available at: www.nccn.org.

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