Sentara Health Plans

Medicaid

Off-Label Drug Use, Pharmacy 12

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Effective Date	2/1/2026
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All requests for authorization for the services described by this medical policy will be reviewed per Early and Periodic Screening, Diagnostic and Treatment (EPSDT) guidelines. These services may be authorized under individual consideration for Medicaid members under the age of 21-years if the services are judged to by medically necessary to correct or ameliorate the member's condition. Department of Medical Assistance Services (DMAS), Supplement B - EPSDT (Early and Periodic Screening, Diagnosis and Treatment) Manual.*.

Description & Definitions:

The United States Food and Drug Administration (FDA) authorizes medications for certain purposes, which are listed on the label (including indication, dose, age and/or duration). Off-label refers to the use of a medication other than what is explicitly included on the label. There is well-documented, off-label use for numerous therapies recorded in peer-reviewed research, and effective in many cases.

The requirements outlined in this policy are superseded by those in drug-specific Sentara Health Plans policies. Therefore, before using the following criteria, drug-specific policies need to be evaluated. In the scenario when a drug-specific policy does not outline off-label use of an FDA-approved drug, the criteria outlined in this document should be applied.

Criteria:

Off-label drug use and off-label dosing are considered medically necessary for **one or more of the following**:

- **For initial therapy authorization** off-label drug use and off-label dosing are considered medically necessary when **all of the following** are met:
  - The member has a documented history of failure, intolerance, or contraindication to standard, conventional therapies meeting the following:
    - Approved and labeled by the FDA for the treatment of the member's diagnosis
    - Having strong support for use in the requested condition per clinical guidelines
    - Note: this requirement is not applicable to requests for oncology drugs prescribed for the treatment of cancer [refer to SHP Chemotherapy and Supportive Services, Medical 316]
  - The drug is currently approved by the United States Food and Drug Administration (FDA) for another indication
  - The drug is being prescribed to treat a medical condition that is not listed in the product's label and for which medical treatment is medically necessary
  - Approval has been obtained by a Sentara Health Plan Medical Director or Pharmacist
  - A drug will be considered as being used for an off-label indication when it meets one of the following:

        - The use of the drug for the ordered indication is listed in **one or more the following** compendia:
            - American Hospital Formulary Service Drug Information® (AHFS®)
            - American Society of Health-System Pharmacists Drug Information [AHFS Drug Information]
            - Clinical Pharmacology (Elsevier/Gold Standard, Inc.)
            - Lexi-Drugs (Wolters Kluwer)
            - Merative Micromedex ®with all of the following: Evidence favors efficacy; Strength of Recommendation Class I, or IIa, or IIb; Strength of Evidence Category A or B;
            - National Comprehensive Cancer Network® (NCCN®) Drug & Biologics Compendium ® Category of Evidence and Consensus 1 or [refer to SHP Chemotherapy and Supportive Services, Medical 316]
        - Two or more adequate and well-controlled studies (preferably at different institutions) performed by experts qualified by scientific training and experience can be identified using the drug for the ordered indication, appropriate dose, and dosing frequency. This excludes case reports, letters, posters, and abstracts. To discern the level of evidence used to support safety and efficacy of a therapy used off-label, **all of the following** will be assessed:
            - Disease prevalence and statistical validity relative to the study population
            - Demographic characteristics of the study population and its representation in the published evidence
            - The impact on the patient's health and clinical response to other therapies that indicate effectiveness (i.e., amelioration in disease severity, disease stability, and reduction in mortality)
            - Clinically meaningful study outcomes
            - The quality of study design such as, but not limited to, randomization, placebo-controlled, statistical analysis, and population inclusionary/exclusionary criteria
- **Continuation of therapy authorization** when **all of the following** are met:
    - All indication-specific and dosing conditions outlined above must be met
    - The member is not experiencing unacceptable toxicity to the requested drug
    - The member has been observed to have a positive clinical response since the beginning of therapy evidenced by disease stability, or mild progression

Off label drug use is considered **not medically necessary** for any use other than those indicated in clinical criteria, to include but not limited to:

- The member has failed a previous course or trial of the requested drug
- The member is currently part of a clinical trial utilizing this medication

This policy shall not be interpreted to require coverage for any drug or biological agent when the FDA has determined its use to be contraindicated.

The prior use of samples will not be considered in the determination of a member's eligibility for coverage of the requested therapy.

Document History:

Revised Dates:

- 2025: October – Implementation date of February 1, 2026. Updated definition. Updated criteria to include reference to SHP Chemotherapy and Supportive Services, Medical 316, and further defined adequate study language.
- 2024: October – Added criteria relating to the member's need for off-label drug utilization, updated description of service and criteria. Added criteria for continued use.
- 2020: December
- 2019: November
- 2014: November
- 2013: October
- 2012: November
- 2011: March

Reviewed Dates:

- 2023: October
- 2022: October
- 2021: December
- 2019: February
- 2018: February
- 2017: January
- 2015: September
- 2010: December
- 2009: November

Origination Date: November 2008

Coding:

Medically necessary with criteria:

Coding	Description
	None

Considered Not Medically Necessary:

Coding	Description
	None

The preceding codes are included above for informational purposes only and may not be all inclusive. Additionally, inclusion or exclusion of a treatment, procedure, or device code(s) does not constitute or imply member coverage or provider reimbursement.

Special Notes: *

- Coverage: See the appropriate benefit document for specific coverage determination. Member specific benefits take precedence over medical policy.
- Application to Products: Policy is applicable to Sentara Health Plan Medicaid products.
- Authorization Requirements: Pre-certification by the Plan is required.
    - https://vamedicaid.dmas.virginia.gov/bulletin/continuous-glucose-monitoring-cgm-coverage-update
- Special Notes:
    - Medicaid
        - This medical policy expresses Sentara Health Plan's determination of medically necessity of services, and they are based upon a review of currently available clinical information. These policies are used when no specific guidelines for coverage are provided by the Department of Medical Assistance Services of Virginia (DMAS). Medical Policies may be superseded by state Medicaid Plan guidelines. Medical policies are not a substitute for clinical judgment or for any prior authorization requirements of the health plan. These policies are not an explanation of benefits.
        - Medical policies can be highly technical and complex and are provided here for informational purposes. These medical policies are intended for use by health care professionals. The medical policies do not constitute medical advice or medical care. Treating health care professionals are solely responsible for diagnosis, treatment and medical advice. Sentara Health Plan members should discuss the information in the medical policies with their treating health care professionals. Medical technology is constantly evolving, and these medical policies are subject to change without notice, although Sentara Health Plan will notify providers as required in advance of changes that could have a negative impact on benefits.

- The Early and Periodic Screening, Diagnostic and Treatment (EPSDT) covers services, products, or procedures for children, if those items are determined to be medically necessary to "correct or ameliorate" (make better) a defect, physical or mental illness, or condition (health problem) identified through routine medical screening or examination, regardless of whether coverage for the same service or support is an optional or limited service under the state plan. Children enrolled in the FAMIS Program are not eligible for all EPSDT treatment services. All requests for authorization for the services described by this medical policy will be reviewed per EPSDT guidelines. These services may be authorized under individual consideration for Medicaid members under the age of 21-years if the services are judged to by medically necessary to correct or ameliorate the member's condition. Department of Medical Assistance Services (DMAS), Supplement B - EPSDT (Early and Periodic Screening, Diagnosis and Treatment) Manual.

References:

Including but not limited to: Specialty Association Guidelines; Government Regulations; Winifred S. Hayes, Inc; UpToDate; Literature Review; Specialty Advisors; National Coverage Determination (NCD); Local Coverage Determination (LCD).

Commonwealth of Virgina Law: CHAPTER 656: An Act to amend and reenact § 2.1-20.1, as it is currently effective and as it may become effective, and § 38.2-3407.5 of the Code of Virginia, relating to off-label drug use. [S 1164] Approved March 21, 1997. 15 SB 1164. Retrieved 8.20.2025. https://www.congress.gov/crs-product/R45792#:~:text=Prescribing%20for%20so%2Dcalled%20off,%25%20of%20doctor%2Doffice%20prescriptions.

U.S. Code of Federal Regulations. Title 42, Chapter IV, Subchapter B, Part 414, Subpart K. § 414.930 Compendia for determination of medically-accepted indications for off-label uses of drugs and biologicals in an anti-cancer chemotherapeutic regimen. Last amended 8.5.2025. https://www.ecfr.gov/current/title-42/chapter-IV/subchapter-B/part-414/subpart-K/section-414.930

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American Society of Health-System Pharmacists (ASHP). ASHP Guidelines on the Evaluation of Off-Label Medication Use in the Inpatient Setting. 7.17.2025. Retrieved 8.21.2025. https://www.ashp.org/-/media/assets/policy-guidelines/docs/guidelines/off-label-med-use-inpatient-setting.pdf

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Keywords:

SHP Off-Label Drug Use, SHP Pharmacy 12, Food and Drug Administration, FDA