BlueCross BlueShield Arizona

An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINE

DIACOMIT® (stiripentol)
Generic Equivalent (if available)

This Pharmacy Coverage Guideline (PCG):
- Provides information about the reasons, basis, and information sources we use for coverage decisions
- Is not an opinion that a drug (collectively "Service") is clinically appropriate or inappropriate for a patient
- Is not a substitute for a provider's judgment (Provider and patient are responsible for all decisions about appropriateness of care)
- Is subject to all provisions e.g. (benefit coverage, limits, and exclusions) in the member's benefit plan; and
- Is subject to change as new information becomes available.

Scope
- This PCG applies to Commercial and Marketplace plans
- This PCG does not apply to the Federal Employee Program, Medicare Advantage, Medicaid or members of out-of-state Blue Cross and/or Blue Shield Plans

Instructions & Guidance
- To determine whether a member is eligible for the Service, read the entire PCG.
- This PCG is used for FDA approved indications including, but not limited to, a diagnosis and/or treatment with dosing, frequency, and duration.
- Use of a drug outside the FDA approved guidelines, refer to the appropriate Off-Label Use policy.
- The "Criteria" section outlines the factors and information we use to decide if the Service is medically necessary as defined in the Member's benefit plan.
- The "Description" section describes the Service.
- The "Definition" section defines certain words, terms or items within the policy and may include tables and charts.
- The "Resources" section lists the information and materials we considered in developing this PCG
- **We do not accept patient use of samples as evidence of an initial course of treatment, justification for continuation of therapy, or evidence of adequate trial and failure.**
- Information about medications that require prior authorization is available at www.azblue.com/pharmacy. You must fully complete the request form and provide chart notes, lab workup and any other supporting documentation. The prescribing provider must sign the form. Fax the form to BCBSAZ Pharmacy Management at (602) 864-3126 or email it to Pharmacyprecert@azblue.com.

Criteria:

- **Criteria for initial therapy:** Diacomit (stiripentol) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

  1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Neurologist

  2. Individual is 6 months of age or older and weighs at least 7kg

  3. Individual has a confirmed diagnosis of seizures associated with Dravet syndrome (DS) and is having at least **four** generalized clonic or tonic-clonic seizures per month despite taking clobazam and valproate

  4. Individual has received and completed a **baseline complete blood count test** before initiation of treatment and with continued monitoring of the individual as clinically appropriate

5. **If available**: Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

6. If approved, it will not be used as monotherapy, it will be added to treatment with clobazam and valproate

7. Individual does not have moderate or severe renal impairment

8. Individual does not have moderate or severe hepatic impairment

Initial approval duration: 6 months

- **Criteria for continuation of coverage (renewal request):** Diacomit (stiripentol) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy)**:

1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Neurologist

2. Individual's condition has responded while on therapy with response defined as **ALL** of the following:
    a. There is no evidence of disease progression
    b. Achieved and maintains at least a 50% decrease in frequency (per 30 days) of generalized clonic or tonic-clonic seizures compared to baseline
    c. Achieved and maintains a longer interval between seizures over baseline

3. Individual has been adherent with the medication

4. **If available**: Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

5. Individual has not developed any significant adverse drug effects that may exclude continued use such as:
    a. Emergence or worsening of depression, suicidal thoughts, behavior, or thoughts of self-harm and/or any unusual changes in mood or behavior
    b. Phenylketonuria from use of powder for suspension formulation
    c. Neutropenia
    d. Thrombocytopenia

6. If approved, it will not be used as monotherapy, treatment regimen includes clobazam and valproate

7. Individual does not have moderate or severe renal impairment

8. Individual does not have moderate or severe hepatic impairment

Renewal duration: 12 months

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- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

  1. **Off-Label Use of Non-Cancer Medications**

  2. **Off-Label Use of Cancer Medications**

Description:

Diacomit (stiripentol) is indicated for the treatment of seizures associated with Dravet syndrome (DS) in patients taking clobazam who are 6 months of age and older and weighing 7 kilograms or more. There are no clinical data to support the use of Diacomit (stiripentol) as monotherapy in DS. The mechanism by which Diacomit (stiripentol) exerts its anticonvulsant effect in humans is unknown. Possible mechanisms of action include direct effects mediated through the gamma-aminobutyric acid (GABA) receptor and indirect effects involving inhibition of cytochrome P450 activity with resulting increase in blood levels of clobazam and its active metabolite.

DS, previously known as severe myoclonic epilepsy of infancy, is a rare early-onset epileptic encephalopathy characterized by refractory epilepsy and neurodevelopmental problems beginning in infancy. Patients present in the first year of life with a prolonged, often febrile, clonic seizure in the setting of normal cognitive and motor development prior to seizure onset. In most, febrile, and afebrile seizures, including episodes of status epilepticus, recur repeatedly in the weeks to months after the initial event, and psychomotor impairment begins thereafter. Myoclonus, both epileptic and non-epileptic, occurs frequently. The majority of older children and young adults with DS have motor system dysfunction, gait and postural abnormalities, and cognitive and behavioral impairment.

DS seizures tend to be refractory to most anti-seizure drugs, and some patients derive benefit from a ketogenic diet and vagus nerve stimulation. The most commonly used anti-seizure drugs include valproate, clobazam, topiramate, levetiracetam, stiripentol, and cannabidiol. Most patients require two or more agents to achieve reasonable seizure control.

A recent international consensus panel in 2022 ranked maintenance therapy as follows: first line: valproate; second line: clobazam, fenfluramine, and stiripentol; third line: cannabidiol; fourth line: topiramate, ketogenic diet. However, the separation of the categories was not universally agreed upon. Some argued that clobazam, fenfluramine, and stiripentol should be first line. Others argued that cannabidiol and fenfluramine should be considered at any point. It was agreed upon that carbamazepine, oxcarbazepine, lamotrigine, and phenytoin should be avoided.

Definitions:

U.S. Food and Drug Administration (FDA) MedWatch Forms for FDA Safety Reporting
MedWatch Forms for FDA Safety Reporting | FDA

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Resources:

Diacomit (stiripentol) cap & powder for oral solution product information, revised by Biocodex, Inc. 06-2024. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed on February 18, 2025.

Andrade DM, Nascimento FA. Dravet syndrome: Genetics, clinical features, and diagnosis. In: UpToDate, Nordil DR, Dashe JF (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at http://uptodate.com. Literature current through January 2025. Topic last updated June 19, 2024. Accessed on February 25, 2025.

Andrade DM, Nascimento FA. Dravet syndrome: Management and prognosis. In: UpToDate, Nordil DR, Dashe JF (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at http://uptodate.com. Literature current through January 2025. Topic last updated June 14, 2024. Accessed on February 25, 2025.

Wirrell EC, Hood V, Knupp KG, et al.: International consensus on diagnosis and management of Dravet syndrome. Epilepsia 2022;63:1761-1777. Available at https://doi.org/10.1111/epi.17274. Accessed on March 16, 2023. Re-evaluated February 25, 2025.