Draft New Policy: Do Not Implement

Garetosmab-grts (Pasatru™)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Pasatru is indicated to reduce formation of new heterotopic ossification (HO) lesions and clinician-assessed flare-ups in adults with fibrodysplasia ossificans progressiva (FOP).

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
Initial Requests:

- Genetic test results confirming diagnosis of fibrodysplasia ossificans progressiva (FOP) with documented activin receptor type 1 (ACVR1) variant (e.g., R206H).
- Chart notes or medical record documentation supporting signs and symptoms of FOP (i.e., congenital malformation of the great toe AND episodic soft-tissue swelling and/or ectopic ossification in ligament or muscle tissue).
- Chart notes or medical record documentation supporting signs and symptoms of active disease within the past 12 months (e.g., FOP flare-ups, worsening of joint function, radiographic progression of heterotopic ossification lesions).

Continuation Requests:
Chart notes or medical record documentation supporting benefit from therapy.

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a physician who is experienced in the treatment of fibrodysplasia ossificans progressiva (FOP) (e.g., endocrinologist, orthopedist, rheumatologist, neurologist).

COVERAGE CRITERIA

Fibrodysplasia Ossificans Progressiva (FOP)

Authorization of 12 months may be granted for reduction in the volume of new heterotopic ossification (HO) lesions and clinician-assessed flare-ups of FOP in adult members (18 years of age and older) when all of the following criteria are met:

- Member has a confirmed diagnosis of FOP by genetic test indicating the member has a pathogenic activin receptor type 1 (ACVR1) FOP-causing variant (e.g., R206H).
- Member has signs and symptoms of FOP (i.e., congenital malformation of the great toe AND episodic soft-tissue swelling and/or ectopic ossification in ligament or muscle tissue).
- Member has evidence of active disease within the past 12 months (e.g., FOP flare-ups, worsening of joint function, radiographic progression of HO lesions).
- Member has a Cumulative Analog Joint Involvement Scale (CAJIS) score less than or equal to 19 at baseline.
- Member has no active or recent malignancy.
- Member has no significant uncontrolled comorbid illness (e.g., cardiac, neurologic, rheumatologic).
- Females of reproductive potential should have a negative pregnancy test.
- Member is not currently breastfeeding, pregnant, or planning pregnancy.
- The requested medication will not be used in combination with palovarotene (Sohonos).

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in adult members (18 years of age and older) requesting reauthorization for an indication listed in the coverage criteria when all of the following criteria are met:

- Member is experiencing benefit from therapy as evidenced by disease improvement or disease stability (e.g., stability or reduction in the volume of new heterotopic ossification, reduced frequency, severity of clinician-assessed flare-ups).
- Member has had no serious adverse event that would contraindicate continued therapy (e.g., serious uncontrolled epistaxis, serious skin/soft-tissue infection).
- Females of reproductive potential should have a negative pregnancy test.
- Member is not currently breastfeeding, pregnant, or planning pregnancy.
- The requested medication will not be used in combination with palovarotene (Sohonos).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Pasatru [package insert]. Tarrytown, NY: Regeneron Pharmaceuticals, Inc.; August 2026.
2. Genetic and Rare Diseases Information Center (GARD). Fibrodysplasia Ossificans Progressiva. Rare Disease Database. Last updated June 2026. Accessed August 26, 2026. https://rarediseases.info.nih.gov

This document has been classified as public information

3. A Study to Assess Safety, Tolerability and Efficacy of Garetosmab Versus Placebo Administered Intravenously (IV) in Adult Participants With Fibrodysplasia Ossificans Progressiva (FOP) (OPTIMA). ClinicalTrials.gov identifier: NCT05394116. Updated July 17, 2026. Accessed August 31, 2026. https://clinicaltrials.gov/study/NCT05394116
4. Kaplan FS, Al Mukaddam M, Baujat G, et al. The medical management of fibrodysplasia ossificans progressiva: current treatment considerations. Proc Intl Clin Council FOP. 2019;1:1-111.

EFFECTIVE DATE

ID_CHS_75902026_1226