Pharmacy Medical Necessity Guidelines:
Carglumic acid (Carbaglu®)

Effective: January 13, 2026

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM

These pharmacy medical necessity guidelines apply to the following:
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:
RXUM: 617.673.0939

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION (FDA)-APPROVED INDICATIONS

Carbaglu® (carglumic acid) is a Carbamoyl Phosphate Synthetase 1 activator indicated as:

- Adjunctive therapy to the standard of care for the treatment of acute hyperammonemia due to the deficiency of the hepatic enzyme N-acetylglutamate synthase (NAGS) and
- Maintenance therapy for the treatment of chronic hyperammonemia due to NAGS deficiency.
- Adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to propionic acidemia (PA) or methylmalonic academia (MMA)

COVERAGE GUIDELINES

The plan may authorize coverage of carglumic acid for Members when the following criterion is met:

1. There is a documented diagnosis of hyperammonemia due to one of the following:
   a. N-acetylglutamate synthase (NAGS) deficiency
   b. Propionic acidemia (PA)
   c. Methylmalonic acidemia (MMA)

LIMITATIONS

1. Requests for brand-name products, which have AB-rated generics, will be reviewed according to the Brand Name criteria.

CODES

None

REFERENCES

1. Batshaw ML, MacArthur RB, Tuchman M. Alternative pathway therapy for urea cycle disorders: Twenty years later. *J Pediatr*. 2001 Jan;138(1):S46-55.
2. Carbaglu (carglumic acid) [prescribing information]. Lebanon, NJ: Recordati Rare Diseases Inc; January 2024.
3. Enns GM, Berry SA, Berry GT, et al. Survival after Treatment with Phenylacetate and Benzoate for Urea-Cycle Disorders. *N Engl J Med.* 2007 May 31;356:2282-92.
4. Shih, V. Alternative-Pathway therapy for Hyperammonemia. *N Engl J Med.* 2007 May 31;356(22): 2321-2.
5. Summar ML, Dobbelaere D, Brusilow S, et al. Diagnosis, symptoms, frequency and mortality of 260 patients with urea cycle disorders from a 21-year, multicentre study of acute hyperammonaemic episodes. *Acta Paediatr*. 2008 Oct;97(10):1420-5.
6. Tuchman M, Lee B, Litchter-Konecki U, et al. Cross-sectional multi-center study of patients with urea cycle disorders in the United States. *Mol Genet Metab.* 2008 Aug;94(4):397-402.
7. Urea Cycle Disorders Conference group. Consensus statement from a conference for the management of patients with urea cycle disorders. *J Pediatr*. 2001 Jan;138:S1-5.

APPROVAL HISTORY

October 1, 2022: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. May 9, 2023: Effective June 1, 2023, administrative update: Requests for brand-name products, which have AB-rated generics, will be reviewed according to the Brand Name criteria.
2. January 9, 2024: Effective February 1, 2024, updated the fax number for RxUM.
3. January 14, 2025: No changes
4. January 13, 2026: No changes.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services