Pharmacy Medical Necessity Guidelines: Carbaglu® (carglumic acid)

Effective: April 14, 2020

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:

Commercial Products

☐ Tufts Health Plan Commercial products – large group plans
☐ Tufts Health Plan Commercial products – small group and individual plans
☐ Tufts Health Freedom Plan products – large group plans
☐ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☐ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☐ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM:
617.673.0988

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 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 the deficiency of the hepatic enzyme NAGS.

COVERAGE GUIDELINES

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

1. There is a documented diagnosis of hyperammonemia due to N-acetylglutamate synthase (NAGS) deficiency.

LIMITATIONS

None

CODES

None

REFERENCES

1. 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):1420-25.
2. Batshaw ML, MacArthur RB, Tuchman M. Alternative pathway therapy for urea cycle disorders: Twenty years later. *J Pediatr*. 2001 Jan;138(1):S46-55.
3. Carbaglu (carglumic acid) [prescribing information]. Paris, France: Orphan Europe SARL; 2019 December.
4. 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.
5. Shih, V. Alternative-Pathway therapy for Hyperammonemia. *N Engl J Med.* 2007 May 31;356(22): 2321-2.
6. 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.
7. 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.

8. 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

March 10, 2009: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
1. February 14, 2012: No changes
2. February 12, 2013: No changes
3. January 14, 2014: No changes
4. December 9, 2014: No changes
5. November 10, 2015: No changes
6. January 1, 2016: Administrative change to rebranded template.
7. November 15, 2016: No changes. Effective November 15, 2016, Medical Necessity Guidelines applies to Tufts Health Together.
8. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
9. November 14, 2017: Administrative update. Edited the overview section.
10. November 13, 2018: No changes.
11. November 12, 2019: No changes.
12. April 14, 2020: Effective March 30, 2020, PA no longer required for Commercial and Direct Plans. MNG only applies to MA and RITogether plans.

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.