Effective Date:12/01/2020
Reviewed Date: 9/2020, 4/2021 Scope:Medicaid

CARBAGLU (carglumic acid)

POLICY

I. 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

1. Acute hyperammonemia in patients with NAGS deficiency
   Carbaglu is indicated as an adjunctive therapy in pediatric and adult patients for the treatment of acute hyperammonemia due to the deficiency of the hepatic enzyme N-acetylglutamate synthase (NAGS). During acute hyperammonemic episodes, concomitant administration of Carbaglu with other ammonia lowering therapies such as alternate pathway medications, hemodialysis, and dietary protein restriction is recommended.

2. Maintenance therapy for chronic hyperammonemia in patients with NAGS deficiency
   Carbaglu is indicated for maintenance therapy in pediatric and adult patients for chronic hyperammonemia due to the deficiency of the hepatic enzyme NAGS. During maintenance therapy, the concomitant use of other ammonia lowering therapies and protein restriction may be needed based on plasma ammonia levels.

3. Indicated in pediatric and adult patients as adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to Propionic Acidemia (PA) or Methylmalonic Acidemia (MMA).

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR INITIAL APPROVAL

Treatment of acute hyperammonemia due to the deficiency of the hepatic enzyme N-acetylglutamate synthase (NAGS)

A. An authorization may be granted for 6 months when the following criteria are met:
   a. The patient has a confirmed diagnosis of N-acetylglutamate synthase (NAGS) deficiency
   b. The prescriber is a physician specializing in the patient's diagnosis or is in consultation with a physician experienced in metabolic disorders

c. The initial and maintenance doses do not exceed United States Food and Drug Administration (FDA) approved labeling.
d. Documentation of baseline ammonia levels

Treatment of Propionic Acidemia (PA) or Methylmalonic Acidemia (MMA)

A. An authorization may be granted for 6 months when the following criteria are met:
a. The patient has a diagnosis of Methylmalonic Acidemia (MMA) or Propionic Acidemia (PA)
b. The prescriber is a physician specializing in the patient's diagnosis of MMA or PA
c. The dosing does not exceed United States Food and Drug Administration (FDA) approved labeling

III. CRITERIA FOR CONTINUATION OF THERAPY

Acute hyperammonemia due to the deficiency of the hepatic enzyme N-acetylglutamate synthase (NAGS)

An Authorization of 6 months may be granted to the member when the patient meets all initial criteria, is tolerating treatment and has documentation of positive clinical response, as evidenced by a decrease in ammonia levels from baseline.

Treatment of Propionic Acidemia (PA) or Methylmalonic Acidemia (MMA)

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for methylmalonic acidemia or propionic academia when the member meets all initial criteria, is tolerating treatment and are experiencing benefit from therapy as evidenced by disease stability or disease improvement.

IV. REFERENCES

1. Carbaglu. Package Insert. Revised by manufacturer January 2021. Available on UpToDate