CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Georgia Medicaid

DRUG NAME	Carbaglu (carglumic acid)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Carbaglu is a carbamoyl phosphate synthetase 1 (CPS 1) activator initially approved by the FDA in 2010. It indicated in pediatric and adult patients as adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to N-acetylglutamate synthase (NAGS) deficiency. Carbaglu is also indicated for maintenance therapy for the treatment of chronic hyperammonemia due to NAGS deficiency. Adults and pediatric patients may also use Carbaglu for adjunctive therapy to standard of care for the treatment of acute hyperammonemia due to propionic acidemia (PA) or methylmalonic acidemia (MMA).

Carbaglu (carglumic acid) will be considered for coverage when the following criteria are met:

N-acetylglutamate Synthase (NAGS) Deficiency with Hyperammonemia

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a geneticist, hepatologist or a metabolic disorder specialist; AND
2. Member has confirmed hepatic N-acetylglutamate synthase deficiency confirmed by enzyme analysis or DNA mutation analysis; AND
3. Member has an elevated plasma ammonia level based on the patient's age; AND
4. Member has a normal anion gap and a normal blood glucose level; AND
5. Member will be taking Carbaglu as adjunctive therapy with a protein-restricted diet; AND
6. **Dosage allowed/Quantity limit:**
   Acute Hyperammonemia due to NAGS deficiency:100 mg/kg/day to 250 mg/kg/day divided into 2 to 4 doses and rounded to the nearest 100 mg.
   Chronic Hyperammonemia due to NAGS deficiency:10 mg/kg/day to 100 mg/kg/day divided into 2 to 4 doses and rounded to the nearest 100 mg.

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease, demonstrated by a normal or improved ammonia level.

If all the above requirements are met, the medication will be approved for an additional 12 months.

Propionic Acidemia (PA) or Methylmalonic Acidemia (MMA), Acute Treatment

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a geneticist, nephrologist or a metabolic disorder specialist; AND
2. Member has a diagnosis of Propionic Acidemia (PA) or Methylmalonic Acidemia (MMA), confirmed by genetic, urine or enzymatic analysis;
3. Member has a documented plasma ammonia level of ≥ 50 micromol/L; AND
4. Member will be taking Carbaglu as adjunctive therapy with other ammonia-lowering therapies, such as intravenous glucose, insulin, L-carnitine, protein restriction, and dialysis; AND
5. **Dosage allowed/Quantity limit:**
   Weight ≤ 15kg: 150mg/kg/day
   Weight > 15kg: 3.3g/m^2/day

If all the above requirements are met, the medication will be approved for 7 days.

For **reauthorization**:
1. Medication will not be reauthorized.

CareSource considers Carbaglu (carglumic acid) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/03/2022	New policy for Carbaglu created.

References:

1. Carbaglu tablets [prescribing information]. Lebanon, NJ: Recordati Rare Diseases; August 2021.
2. Kenneson A, Singh RH. Presentation and management of N-acetylglutamate synthase deficiency: a review of the literature. Orphanet J Rare Dis. 2020;15(1):279.
3. Forny P, Hörster F, Ballhausen D, et al. Guidelines for the diagnosis and management of methylmalonic acidaemia and propionic acidaemia: First revision. J Inherit Metab Dis. 2021 May;44(3):566-592.
4. Haijes HA, van Hasselt PM, Jans JJM, Verhoeven-Duif NM. Pathophysiology of propionic and methylmalonic acidemias. Part 2: Treatment strategies. J Inherit Metab Dis. 2019 Sep;42(5):745-761.
5. Baumgartner MR, Hörster F, Dionisi-Vici C, et al. Proposed guidelines for the diagnosis and management of methylmalonic and propionic acidemia. *Orphanet J Rare Dis*. 2014;9:130.

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