BlueCross BlueShield Federal Employee Program

CARBAGLU
(carglumic acid)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Carbaglu (carglumic acid) is used to treat certain conditions that result in hyperammonemia (excess ammonia) in the blood. Carbaglu treats hyperammonemia by acting as a replacement for N-acetylglutamate (NAG) in the urea cycle, whose role is the disposition of ammonia. NAG acts as the activator of carbamoyl phosphate synthetase 1 (CPS 1), a mitochondrial liver enzyme which catalyzes the first reaction of the urea cycle. The urea cycle includes a series of biochemical reactions in the liver resulting in the conversion of ammonia into urea, which is then excreted through the urine. Carbaglu acts as a CPS1 activator, improves or restores the function of the urea cycle, and facilitates ammonia detoxification and urea production (1).

Regulatory Status

FDA-approved indications: Carbaglu (carglumic acid) is a carbamoyl phosphate synthetase 1 (CPS 1) activator indicated in pediatric and adult patients as: (1)

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

Management of hyperammonemia due to NAGS deficiency, propionic acidemia or methylmalonic acidemia should be done in coordination with medical personnel experienced in metabolic disorders. Ongoing monitoring of plasma ammonia levels, neurological status, laboratory tests and clinical responses in patients receiving Carbaglu is crucial to assess patient response to treatment (1).

The safety and effectiveness of Carbaglu for the treatment of pediatric patients aged birth to 17 years of age have been established (1).

Summary

Carbaglu (carglumic acid) is used to treat certain conditions that result in hyperammonemia (excess ammonia) in the blood. Carbaglu treats hyperammonemia by acting as a replacement for

N-acetylglutamate (NAG) in the urea cycle, whose role is the disposition of ammonia. NAG acts as the activator of carbamoyl phosphate synthetase 1 (CPS 1), a mitochondrial liver enzyme which catalyzes the first reaction of the urea cycle. The urea cycle includes a series of biochemical reactions in the liver resulting in the conversion of ammonia into urea, which is then excreted through the urine. Carbaglu acts as a CPS1 activator, improves or restores the function of the urea cycle, and facilitates ammonia detoxification and urea production (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Carbaglu while maintaining optimal therapeutic outcomes.

References

1. Carbaglu [package insert]. Lebanon, NJ: Recordati Rare Diseases, Inc.; January 2024.