FIRST TIME GENERIC APPROVAL

Brand Name	Carglumic acid
Generic Name	carglumic acid
Drug Manufacturer	Novitium Pharma LLC

New Drug Approval

TYPE OF CLINICAL UPDATE

First Time Generic

FDA APPROVAL DATE

October 13, 2021

LAUNCH DATE

December 1, 2021

REVIEW DESIGNATION

Abbreviated New Drug Application (ANDA): 213729

TYPE OF REVIEW

Standard

DISPENSING RESTRICTIONS

N/A

Overview

INDICATION FOR USE

Carglumic acid tablets for oral suspension is a carbamoyl phosphate synthetase 1 (CPS 1) activator 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.
- Maintenance therapy for the treatment of chronic hyperammonemia due to NAGS deficiency.

MECHANISMS OF ACTION

Carglumic acid is a synthetic structural analogue of N-acetylglutamate (NAG) which is produced from glutamate and acetyl-CoA in a reaction catalyzed by N-acetylglutamate synthase (NAGS), a mitochondrial liver enzyme. NAG acts as the essential allosteric activator of carbamoyl phosphate synthetase 1 (CPS 1), a mitochondrial liver enzyme which catalyzes the first reaction of the urea cycle. The urea cycle, whose role is the disposition of ammonia, includes a series of biochemical reactions in the liver resulting in the conversion of ammonia into urea, which is then excreted through the urine. Carglumic acid acts as a CPS1 activator, improves or restores the function of the urea cycle, and facilitates ammonia detoxification and urea production.

DOSE FORM AND STRENGTH

Tablets for oral suspension: 200 mg, functionally scored.

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DOSE & ADMINISTRATION

Acute Hyperammonemia due to NAGS deficiency

- The recommended pediatric and adult dosage is 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

- The recommended pediatric and adult dosage is 10 mg/kg/day to 100 mg/kg/day divided into 2 to 4 doses and rounded to the nearest 100 mg.

Therapeutic Monitoring for NAGS Deficiency

- Closely monitor plasma ammonia and titrate dosage to maintain the ammonia level within normal range for the patient's age, taking into consideration their clinical condition.