

Clinical Policy: Carglumic Acid (Carbaglu)

Reference Number: PA.CP.PHAR.206

Effective Date: 01/18

Last Review Date: 07/18

[Coding Implications](#)

[Revision Log](#)

Description

The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness[®] clinical policy for carglumic acid (Carbaglu[®])

FDA Approved Indication(s)

Carbaglu is indicated for:

- Acute hyperammonemia in patients with NAGS deficiency
 - Adjunctive therapy in pediatric and adult patients for the treatment of acute hyperammonemia due to the deficiency of the hepatic enzyme NAGS. During acute hyperammonemic episodes concomitant administration of Carbaglu with other ammonia lowering therapies such as alternate pathway medications, hemodialysis, and dietary protein restriction are recommended.
- Maintenance therapy for chronic hyperammonemia in patients with NAGS deficiency
 - 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 reduced or discontinued based on plasma ammonia levels.

Policy/Criteria

It is the policy of Pennsylvania Health and Wellness that carglumic acid is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Urea Cycle Disorder: N-acetylglutamate synthase deficiency (must meet all):

1. Prescribed by or in consultation with a physician experienced in metabolic disorders;
2. Diagnosis of a urea cycle disorder (UCD) caused by deficiency of the enzyme N-acetylglutamate synthase (NAGS);
3. NAGS deficiency is confirmed by enzymatic or genetic analysis;

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

A. Urea Cycle Disorder: N-acetylglutamate synthase deficiency (must meet all):

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Member is responding positively to therapy.

Approval duration: 12 months

B. Other diagnoses/indications (1 or 2):

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;

Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to PA.CP.PMN.53

Background

Description/Mechanism of Action:

Carglumic acid is a synthetic structural analogue of N-acetylglutamate (NAG), which is an essential allosteric activator of carbamoyl phosphate synthetase 1 (CPS 1) in liver mitochondria. CPS 1 is the first enzyme of the urea cycle, which converts ammonia into urea. NAG is the product of N-acetylglutamate synthase (NAGS), a mitochondrial enzyme. Carglumic acid acts as a replacement for NAG in NAGS deficiency patients by activating CPS 1.

Formulations:

Carbaglu is supplied as scored tablets containing 200 mg of carglumic acid.

Appendices

Appendix A: Abbreviation Key

NAGS: N-acetyl glutamate synthetase

UCD: urea cycle disorder

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date
Removed requirement for confirmation that Carbaglu is prescribed to treat acute or chronic hyperammonemia as this is characteristic of the condition itself. References reviewed and updated.	02/18	

References

Carbaglu Prescribing Information. Lebanon, NJ: Recordati Rare Diseases, Inc.; November 2015. Available at <https://www.carbaglu.net/wp-content/uploads/2016/04/carbaglu-pi-nov-2015-final.pdf>. Accessed November 14, 2017.