

Pharmacy Policy Bulletin

Title: Carglumic acid (Carbaglu®)

Policy #: Rx.01.10

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

▸ Intent:

The intent of this policy is to communicate the medical necessity criteria for carglumic acid (Carbaglu®) as provided under the member's prescription drug benefit.

▸ Description:

N-acetylglutamate synthetase (NAGS) deficiency is a rare, autosomal, recessive genetic disorder in which lack of NAGS enzyme leads to hyperammonemia (excess ammonia). NAGS deficiency is one of several urea cycle disorders.

Carglumic acid (Carbaglu®) 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 NAGS, a mitochondrial enzyme. Carglumic acid acts as a replacement for NAG in NAGS deficiency patients by activating CPS 1.

Carglumic Acid (Carbaglu®) is indicated for adjunctive therapy in the treatment of acute hyperammonemia and maintenance therapy of chronic hyperammonemia due to the deficiency of the hepatic enzyme NAGS.

▸ Policy:

Carglumic Acid (Carbaglu®) is approved when there is a diagnosis of N-acetylglutamate synthase (NAGS) deficiency.

▸ Black Box Warning:

N/A

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION


Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Carbaglu® [package insert]. Lebanon NJ. Recordati Rare Diseases, Inc. November 2015. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2010/022562lbl.pdf. Accessed July 20, 2018.

N-acetylglutamate synthetase deficiency. National organization for rare disorders. Available at: <http://rarediseases.org/rare-diseases/n-acetylglutamate-synthetase-deficiency/>. Accessed July 20, 2018.

Applicable Drugs:

 Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Carbaglu	Carglumic Acid

Cross References:

N/A

Policy Version Number:	9.00
P&T Approval Date:	July 12, 2018
Policy Effective Date:	October 01, 2018
Next Required Review Date:	July 12, 2019

The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment. Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.