

Carbaglu (carglumic acid)
Effective 3/01/2025

Plan	<input type="checkbox"/> MassHealth UPPL <input checked="" type="checkbox"/> Commercial/Exchange	Program Type	<input checked="" type="checkbox"/> Prior Authorization <input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
Benefit	<input checked="" type="checkbox"/> Pharmacy Benefit <input type="checkbox"/> Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	Medical and Specialty Medications		
	All Plans	Phone: 877-519-1908	Fax: 855-540-3693
Exceptions	Non-Specialty Medications		
	All Plans	Phone: 800-711-4555	Fax: 844-403-1029
Exceptions	N/A		

Overview

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

Coverage Guidelines

Authorization may be granted for members new to the plan within the past 90 days who are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs

OR

Authorization may be granted all of the following criteria are met:

1. Member has one of the following diagnoses:
 - Hyperammonemia due to the N-acetylglutamate synthetase (NAGS) deficiency
 - Propionic acidemia
 - Methylmalonic acidemia (MMA)

Continuation of Therapy

Requests for reauthorization will be approved when the following criteria are met:

1. Documentation is submitted demonstrating improvement and/or normalization of blood ammonia levels

Limitations

1. Initial approvals will be granted for 12 months.
2. Reauthorizations will be granted for 36 months.

References

1. Carbaglu (carglumic acid) [prescribing information]. Bridgewater, NJ, NJ: Recordati Rare Diseases Inc; January 2024.
2. Daniotti M, la Marca G, Fiorini P, Filippi L. New developments in the treatment of hyperammonemia: emerging use of carglumic acid. *Int J Gen Med* 2011; 4:21
3. Gessler P, Buchal P, Schwenk HU, Wermuth B. Favourable long-term outcome after immediate treatment of neonatal hyperammonemia due to N-acetylglutamate synthase deficiency. *Eur J Pediatr*. 2010;169:197-199.
4. Lee B, Diaz GA, Rhead W, et al. Glutamine and hyperammonemic crises in patients with urea cycle disorders. *Mol Genet Metab* 2016; 117:27
5. NAGS deficiency [press release on the Internet]. Paris (France): Orphan Europe SARL; 2007 Mar 22. Available from: <http://www.orphan-europe.com/Data/ModuleGestionDeContenu/03-Diseases/Hyperammonaemia/16.asp>.
6. N-acetylglutamate synthetase deficiency. National Organization of Rare Diseases (NORD). 2014. Available at: <http://www.rarediseases.org/rare-disease-information/rare-diseases/byID/313/viewFullReport>

Review History

06/25/2012 – Reviewed

06/24/2013 – Reviewed

06/23/2014 – Reviewed

06/22/2015 – Reviewed

06/27/2016 – Reviewed

06/26/2017 – Reviewed

06/25/2018 – Reviewed

06/19/2019 – Reviewed

07/22/2020 – Reviewed and updated July P&T Mtg; added started and stabilized statement

09/16/2020 – Reviewed and updated Sept P&T Mtg; removed specialist requirement; references updated.

Effective 12/01/2020.

12/11/2024 – Reviewed and updated at December P&T. Updated verbiage for new members. Updated initial criteria to include supplemental indications of MMA and propionic acidemia. Removed Appendix. Effective 3/1/2025.

