

Key points	Carbaglu is the only Australian product.
Indication	Hyperammonaemia due to N-acetylglutamate synthase (NAGS) deficiency or organic acidurias (isovaleric aciduria (IVA), methylmalonic aciduria (MMA), or propionic aciduria (PA)). ⁶
Action	Carglumatic acid is a synthetic analog of N-acetylglutamate (NAG) that activates carbamoyl-phosphate synthase I (CPS-I), the first enzyme of the urea cycle enhancing ureagenesis.
Contraindications	Hypersensitivity to carglumatic acid or any component of the formulation ⁶
Precautions	Dose adjustment is recommended in kidney impairment ⁶
Dose	<p>Initial dose 100-250 mg/kg/day orally, divided into 2-4 doses.⁵ Titrate the dose according to plasma ammonia concentration and clinical response. Concomitant ammonia-lowering therapies are recommended during acute decompensation.⁶</p> <p>Maintenance dose Should be individually adjusted to maintain normal plasma ammonia for age and adequate metabolic control.⁶</p> <p>Renal impairment: Moderate impairment (GFR 30-59 mL/min): Initial dose - 50-125 mg/kg/day Severe impairment (GFR ≤29 mL/min): Initial dose - 15-40 mg/kg/day. Lower maintenance doses are recommended.⁶ Discuss with paediatric metabolic specialist.</p>
Route	ORAL
Presentation	Carglumatic acid (Carbaglu) 200mg dispersible tablet ⁵
Preparation	Disperse tablet in a minimum of 5 – 10 mL of water ¹ Tablets can be halved and quartered, round dose to nearest quarter or half. ¹
IV Compatibility	Not applicable.
IV Incompatibility	Not applicable.
Administration	Administer immediately prior to feeds.
Monitoring	Regular monitoring of liver, renal, cardiac functions, ammonia, plasma amino acids, and haematological parameters.
Drug Interactions	No specific clinical drug-interaction studies have been performed. Clinically significant CYP-mediated interactions are not anticipated based on in-vitro data. ⁶
Adverse Reactions	Following are reported only with large doses: ² tachycardia, sweating, increased bronchial secretions, hyperthermia, restlessness, and raised transaminases have been reported in NAGS deficiency.
Overdose	<p>A patient receiving 750 mg/kg/day developed tachycardia, profuse sweating, increased bronchial secretions, increased body temperature, and restlessness; symptoms resolved after dose reduction.⁵</p> <p>Symptomatic treatment.</p> <p>AUSTRALIA: Contact the Poisons Information Centre on 13 11 26 for information on management.</p> <p>NEW ZEALAND: Contact the National Poisons Centre on 0800 764 766 for information on management.</p>
Stability	Prepare immediately before each dose and discard any unused dispersion. ⁶
Storage	<p>Unopened container: refrigerate at 2-8°C. Do not freeze. Protect from light.⁶</p> <p>After opening: store below 30°C (do not refrigerate), keep tightly closed to protect from moisture and use within 3 months.⁶</p>
Excipients	Microcrystalline cellulose, sodium lauryl sulfate, hypromellose, croscarmellose sodium, anhydrous colloidal silica, and sodium stearyl fumarate. ⁶
Evidence	<p>Overview</p> <p>Carglumatic acid treatment</p> <p>In Australia, carglumatic acid is indicated for hyperammonaemia due to NAGS deficiency and for hyperammonaemia due to organic acidurias including IVA, MMA and PA.⁶ In the USA, carglumatic acid is approved for the treatment of acute and chronic hyperammonemia due to NAGS deficiency. In the EU, it is approved for both acute and long-term maintenance therapy for NAGS deficiency. For IVA, PA, or MMA, the formal EU authorisation covers hyperammonemia linked to these organic acidurias. Carglumatic acid has been effective in the management of acute hyperammonemia in PA, IVA and MMA. The recommended initial dose of carglumatic acid in Europe is 100–250 mg/kg/day, divided into 2-4 oral doses and titrated to plasma ammonia and clinical response.³</p>

	<p>In a multicentre, prospective, randomized controlled trial, patients aged ≤ 15 years with confirmed PA and MMA were given carglumic acid (50 mg/kg/day in divided doses, twice daily) added to standard treatment in the study. Patients were followed up for 2 years. Carglumic acid in addition to standard treatment over the long term significantly reduces the number of Emergency admissions.⁴</p> <p>Safety</p> <p>In newborns and paediatric patients, it is usually well tolerated without significant adverse effects. A sympathomimetic toxicity syndrome with tachycardia, sweating, increased bronchial secretion, hyperthermia, and restlessness has been reported at 750 mg/kg/day, and resolved after dose reduction.²</p>
References	<ol style="list-style-type: none"> 1. British Inherited Metabolic Diseases Group (BIMDG). https://bimdg.org.uk/emergency/paediatric-guidelines/. Accessed on 11 August 2026. 2. Daniotti M, La Marca G, Fiorini P, Filippi L. New developments in the treatment of hyperammonemia: emerging use of carglumic acid. <i>International journal of general medicine</i>. 2011;21–8. 3. Valayannopoulos V, Baruteau J, Delgado MB, Cano A, Couce ML, Del Toro M, et al. Carglumic acid enhances rapid ammonia detoxification in classical organic acidurias with a favourable risk-benefit profile: a retrospective observational study. <i>Orphanet journal of rare diseases</i>. 2016;11(1):32. 4. Alfadhel M, Nashabat M, Saleh M, Elamin M, Alfares A, Al Othaim A, et al. Long-term effectiveness of carglumic acid in patients with propionic acidemia (PA) and methylmalonic acidemia (MMA): a randomized clinical trial. <i>Orphanet journal of rare diseases</i>. 2021;16(1):422. 5. Product information for AusPAR Carbaglu carglumic acid Emerge Health Pty Ltd. Accessed 2 September 2015. 6. Recordati Rare Diseases Australia Pty Ltd. Carbaglu (carglumic acid) 200 mg dispersible tablets. Australian Product Information. Revised 28 January 2026. Accessed 20 September 2026.

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