



# Summary of Investigation Results

## Carglumic acid

June 11, 2024

### Non-proprietary name

Carglumic acid

### Brand name (marketing authorization holder)

Carbaglu dispersible tablets 200 mg (Recordati Rare Diseases Japan K.K.)

### Japanese market launch

December 2016

### Indications

Hyperammonaemia due to the following diseases:

- N-acetylglutamate synthase deficiency
- Isovaleric acidaemia
- Methylmalonic acidaemia
- Propionic acidaemia

### Summary of revisions

1. A statement should be added to the 7. PRECAUTIONS CONCERNING DOSAGE AND ADMINISTRATION section that the starting dose of this drug should be reduced in patients with moderate or severe renal impairment.
2. The 9.2 Patients with Renal Impairment section should be newly added to the 9. PRECAUTIONS CONCERNING PATIENTS WITH SPECIFIC BACKGROUNDS section, and “patients with moderate or severe renal impairment (eGFR less than 60 mL/min/1.73 m<sup>2</sup>)” should be added.

### Investigation results and background of the revision

The results of a clinical pharmacology study of this drug in subjects with renal impairment

Pharmaceuticals and Medical Devices Agency



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were evaluated. The study results showed an increased exposure to this drug in patients with moderate or severe renal impairment compared to those with normal renal function. As a result of consultation with expert advisors, the MHLW/PMDA concluded that revision of PRECAUTIONS was necessary.

The expert advisors present at the Expert Discussion regarding the current investigation were nominated based on their conflict of interest declarations concerning the relevant products, pursuant to the “Rules for Convening Expert Discussions, etc., by the Pharmaceuticals and Medical Devices Agency” (PMDA Administrative Rule No. 20-8, dated December 25, 2008).