Summary of risk management plan for Carglumic Acid Waymade (carglumic acid)

This is a summary of the risk management plan (RMP) for Carglumic Acid Waymade (Carglumic acid). The RMP details important risks of Carglumic acid, how these risks can be minimised, and how more information will be obtained about Carglumic acid's risks and uncertainties (missing information).

Carglumic acid's summary of product characteristics (SmPC) and its package leaflet give essential information to healthcare professionals and patients on how Carglumic acid should be used.

Important new concerns or changes to the current ones will be included in updates of Carglumic Acid Waymade.

I. The medicine and what it is used for

Carglumic acid is authorised for treatment of hyperammonaemia due to N-acetylglutamate synthase primary deficiency, isovaleric acidaemia, methylmalonic acidaemia or propionic acidaemia. It contains carglumic acid as the active substance and it is given orally by ingestion or via a nasogastric tube using a syringe, if necessary.

II. Risks associated with the medicine and activities to minimise or further characterise the risks

Important risks of Carglumic acid, together with measures to minimise such risks and the proposed studies for learning more about Carglumic acid's risks, are outlined below.

Measures to minimise the risks identified for medicinal products can be:

- Specific information, such as warnings, precautions, and advice on correct use, in the package leaflet and SmPC addressed to patients and healthcare professionals;
- Important advice on the medicine's packaging;
- The authorised pack size — the amount of medicine in a pack is chosen so to ensure that the medicine is used correctly;
- The medicine's legal status — the way a medicine is supplied to the patient (e.g. with or without prescription) can help to minimise its risks.

Together, these measures constitute *routine risk minimisation* measures.

If important information that may affect the safe use of Carglumic acid is not yet available, it is listed under 'missing information' below.

II.A List of important risks and missing information

Important risks of Carglumic acid are risks that need special risk management activities to further investigate or minimise the risk, so that the medicinal product can be safely administered. Important risks can be regarded as identified or potential. Identified risks are concerns for which there is sufficient proof of a link with the use of Carglumic acid. Potential risks are concerns for which an association with

the use of this medicine is possible based on available data, but this association has not been established yet and needs further evaluation. Missing information refers to information on the safety of the medicinal product that is currently missing and needs to be collected (e.g. on the long-term use of the medicine);

List of important risks and missing information	
Important identified risks	None
Important potential risks	Medication error (including incorrect route of administration) Off label use
Missing information	Use in pregnant women Patients with cardiac diseases/renal and hepatic impairment

II.B **Summary of important risks**

The safety information in the proposed Product Information is aligned to the reference medicinal product.

II.C **Post-authorisation development plan**

II.C.1 Studies which are conditions of the marketing authorisation

There are no studies which are conditions of the marketing authorisation or specific obligation of Carglumic acid.

II.C.2 Other studies in post-authorisation development plan

There are no studies required for Carglumic acid.