

Summary of risk management plan for Miglustat Waymade 100 mg capsules (miglustat)

This is a summary of the risk management plan (RMP) for Miglustat Waymade 100 mg capsules. The RMP details important risks of Miglustat Waymade, how these risks can be minimised, and how more information will be obtained about Miglustat Waymade's risks and uncertainties (missing information).

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

Important new concerns or changes to the current ones will be included in updates of Miglustat Waymade's RMP.

I. The medicine and what it is used for

Miglustat Waymade is authorised for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease. Miglustat Waymade may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable.

Miglustat Waymade is authorised for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease treatment of (see SmPC for the full indication). It contains miglustat as the active substance and is given by orally.

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

Important risks of Miglustat Waymade, together with measures to minimise such risks and the proposed studies for learning more about Miglustat Wyamde'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 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.

In addition to these measures, information about adverse reactions is collected continuously and regularly analysed so that immediate action can be taken as necessary. These measures constitute routine pharmacovigilance activities.

II.A List of important risks and missing information

Important risks of Miglustat Waymade are risks that need special risk management activities to further investigate or minimise the risk, so that the medicinal product can be safely taken. 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 Miglustat Waymade. 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).

Summary of safety concerns	
Important identified risks	<ul style="list-style-type: none">• None
Important potential risks	<ul style="list-style-type: none">• None
Missing information	<ul style="list-style-type: none">• None

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 Miglustat Waymade.

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

There are no studies required for Miglustat Waymade.