Summary of the risk management plan

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

Yargesa 100 mg hard capsules summary of product characteristics (SmPC) and its package leaflet give essential information to healthcare professionals and patients on how Yargesa 100 mg hard capsules should be used.

This summary of the RMP for Yargesa 100 mg hard capsules should be read in the context of all this information including the assessment report of the evaluation and its plain-language summary, all which is part of the European Public Assessment Report (EPAR).

Important new concerns or changes to the current ones will be included in updates of Yargesa 100 mg hard capsules RMP.

I. The medicine and what it is used for

Yargesa 100 mg hard capsules is authorised for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease. Yargesa may be used only in the treatment of patients for whom enzyme replacement therapy is unsuitable. It is also indicated for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease.

It contains 100 mg of miglustat as the active substance and it is given by oral route of administration.

Further information about the evaluation of Yargesa 100 mg hard capsules benefits can be found in Yargesa 100 mg hard capsules EPAR, including in its plain-language summary, available on the EMA website, under the medicine's webpage.

https://www.ema.europa.eu/en/medicines/human/EPAR/yargesa link to product's EPAR summary landing page on the EMA webpage.

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

Important risks of Yargesa 100 mg hard capsules, together with measures to minimise such risks and the proposed studies for learning more about Yargesa 100 mg hard capsules 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.

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

If important information that may affect the safe use of Yargesa 100 mg hard capsules is not yet available, it is listed under 'missing information' below.

II.A List of important risks and missing information

Important risks of Yargesa 100 mg hard capsules 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 Yargesa 100 mg hard capsules. 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	• Peripheral neuropathy • Growth disturbance in paediatric patients with NP-C disease
Important potential risks	• Adverse effects on spermatogenesis and sperm parameters and reducing fertility • Reproductive toxicity, including dystocia • Increased incidence of large intestinal inflammation, adenoma and adenocarcinoma • Crohn's disease
Missing information	• Use in elderly patients with GD-1 • Patients with a history of significant gastrointestinal disease, including inflammatory bowel disease

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 Yargesa 100 mg hard capsules.

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

There are no studies required for Yargesa 100 mg hard capsules.