Part VI: Summary of the Risk Management Plan

Summary of Risk Management Plan for DEFERASIROX 90 mg, 180 mg, 360 mg film-coated tablets

This is a summary of the risk management plan (RMP) for DEFERASIROX 90 mg, 180 mg, 360 mg film-coated tablets (hereinafter referred to as Deferasirox). The RMP details important risks of Deferasirox, how these risks can be minimised, and how more information will be obtained about Deferasirox's risks and uncertainties (missing information).

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

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

I. The Medicine and What It is used for

Deferasirox is authorised for the treatment of chronic iron overload (see SmPC for the full indication). It contains deferasirox as the active substance and it is given orally.

II. Risks Associated with the Medicine and Activities to Minimise or Further Characterise the Risks

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

In the case of Deferasirox, these measures are supplemented with *additional risk minimisation measures* mentioned under relevant important risks, below.

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.*

If important information that may affect the safe use of deferasirox is not yet available, it is listed under ‘missing information’ below.

II.A List of Important Risks and Missing Information

Important risks of Deferasirox 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 Deferasirox. 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).

Table 9: Summary of Safety Concerns

List of important risks and missing information	
Important identified risks	• Renal disorders (increased serum creatinine, acute renal failure, renal tubular disorders (acquired Fanconi's syndrome)) • Increased liver transaminases/hepatic failure • Gastrointestinal haemorrhage and ulcers; oesophagitis • Hearing loss • Lens opacities, retinal changes, and optic neuritis • Severe cutaneous adverse reactions (SCARs) (including Stevens-Johnson syndrome [SJS], Toxic epidermal necrolysis [TEN] and Drug reaction with eosinophilia and systemic symptoms [DRESS])
Important potential risks	• Compliance with posology and biological monitoring • Medication errors
Missing information	• Long term safety in paediatric NTDT patients aged 10 to 17 years • Safety of new formulation

II.B Summary of Important Risks

Table 10: Summary of Pharmacovigilance Activities and Risk Minimisation Activities by Safety Concern

Important identified risk: Renal disorders (increased serum creatinine, acute renal failure, renal tubular disorders (acquired Fanconi's syndrome))	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.2, 4.3, 4.4, and 4.8. SmPC section 4.4 where advice is given on monitoring the renal function PL sections 2 and 4. Prescription only medicine.

	Additional risk minimisation measures None
Important identified risk: Increased liver transaminases/hepatic failure	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.2, 4.4, and 4.8. SmPC section 4.4 where advice is given on monitoring the liver function PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures None
Important identified risk: Gastrointestinal haemorrhage and ulcers; oesophagitis	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.4, 4.5, and 4.8. PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures None
Important identified risk: Hearing loss	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.4, and 4.8. SmPC section 4.4 where advice is given on auditory testing. PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures None
Important identified risk: Lens opacities, retinal changes, and optic neuritis	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.4, and 4.8. SmPC section 4.4 where advice is given on ophthalmic testing. PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures None
Important identified risk: Severe cutaneous adverse reactions (SCARs) (including Stevens-Johnson syndrome [SJS], Toxic epidermal necrolysis [TEN] and Drug reaction with eosinophilia and systemic symptoms [DRESS])	
Risk minimisation measures	Routine risk minimisation measures SmPC sections 4.4 and 4.8. PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures None

Important potential risk: Compliance with posology and biological monitoring	
Risk minimisation measures	Routine risk minimisation measures SmPC section 4.4. PL sections 2 and 4. Prescription only medicine. Additional risk minimisation measures Healthcare Professional Guide Patient guide
Important potential risk: Medication errors	
Risk minimisation measures	Routine risk minimisation measures SmPC section 4.2. PL section 3 Prescription only medicine. Additional risk minimisation measures Healthcare Professional Guide Patient guide
Missing information: Long term safety in paediatric NTDT patients aged 10 to 17	
Risk minimisation measures	Routine risk minimisation measures SmPC section 4.4. Prescription only medicine. Additional risk minimisation measures None
Missing information: Safety of new formulation	
Risk minimisation measures	Routine risk minimisation measures Prescription only medicine Additional risk minimisation measures None

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 Deferasirox.

II.C.2 Other Studies in Post-Authorisation Development Plan

There are no studies required for Deferasirox.