Medicines & Healthcare products
Regulatory Agency

Public Assessment Report

National Procedure

Pedmarqsi 80 mg/mL solution for infusion

sodium thiosulfate

PLGB 20011/0078

NORGINE PHARMACEUTICALS LIMITED

LAY SUMMARY

Pedmarqsi 80 mg/mL solution for infusion
sodium thiosulfate

This is a summary of the Public Assessment Report (PAR) for Pedmarqsi 80 mg/mL solution for infusion. It explains how this product was assessed and its authorisation recommended, as well as its conditions of use. It is not intended to provide practical advice on how to use this product.

This product has been authorised by the Medicines and Healthcare products Regulatory Agency (MHRA) for Great Britain (consisting of England, Scotland and Wales). In coming to its decision, MHRA has relied on a European Commission (EC) decision on 26 May 2023 (EMEA/H/C/005130/0000), in accordance with the advice from the Committee for Medicinal Products for Human Use (CHMP). This is known as the EC Decision Reliance Procedure.

This application was approved under Regulation 50 of the Human Medicines Regulation 2012, as amended (previously Article 8(3) of Directive 2001/83/EC, as amended).

For practical information about using Pedmarqsi 80 mg/mL solution for infusion, patients should read the Patient Information Leaflet (PIL) or contact their doctor or pharmacist.

What is Pedmarqsi 80 mg/mL solution for infusion and what is it used for?

This application was a full-dossier application. This means that the results of pharmaceutical, non-clinical and clinical tests have been submitted to show that this medicine is suitable for treating the specified indications.

Pedmarqsi 80 mg/mL solution for infusion is used to reduce the risk of hearing loss from the cancer medicine cisplatin. It is given to children and adolescents aged 1 month to 18 years who are being treated with cisplatin for solid tumours that have not spread to other areas of the body.

How does Pedmarqsi 80 mg/mL solution for infusion work?

This medicine contains the active substance sodium thiosulfate. The way that Pedmarqsi works to reduce the risk of hearing loss from cisplatin is not fully understood but may include increasing levels of antioxidants, inhibiting a type of cellular stress or effects that occur after direct interactions between sodium thiosulfate and cisplatin.

How is Pedmarqsi 80 mg/mL solution for infusion used?

The pharmaceutical form of this medicine is solution for infusion and the route of administration is intravenous (into a vein). This means that the medicine is given as an infusion (drip) into a vein by a doctor or nurse. This is usually done via a tube inserted into a vein in the chest, known as a central line.

The infusion is given over 15 minutes. Treatment is started 6 hours after the dose of cisplatin has finished. Before the patient receives this medicine, they will be given anti-sickness medicines to help prevent vomiting.

The dose of this medicine is worked out based on the patient's size (body surface area) in m^2, which is calculated from height and weight. The recommended dose for those weighing 10 kg or more is 12.8 g per m^2 and lower doses are given to those weighing less than 10 kg. The patient's doctor will work out the dose that is right for the patient.

For further information on how Pedmarqsi 80 mg/mL solution for infusion is used, refer to the PIL and Summary of Product Characteristics (SmPC) available on the Medicines and Healthcare products Regulatory Agency (MHRA) website.

This medicine can only be obtained with a prescription.

The patient or their parent or caregiver should ask the administering healthcare practitioner if they have any questions concerning their medicine.

What benefits of Pedmarqsi 80 mg/mL solution for infusion have been shown in studies?
Two studies found that Pedmarqsi reduced the risk of hearing loss in children aged 1 month to 18 years who were receiving cisplatin to treat solid tumours.

The first study involved 114 children with hepatoblastoma (a cancer of the liver), with an average age of about 19 months. The results showed that 35% (20 out of 57) of children who received Pedmarqsi 6 hours after each dose of cisplatin developed hearing loss compared with 67% (35 out of 52) of children who only received cisplatin.

The second study involved 125 children aged 1 month to 18 years with different types of cancer, including hepatoblastoma, neuroblastoma (a cancer of immature nerve cells) and tumours of the central nervous system. The study found that hearing loss was experienced by 29% (14 out of 49) of children who received Pedmarqsi after each cisplatin dose compared with 56% (31 out of 55) of those who received only cisplatin.

What measures are being taken to ensure the safe and effective use of Pedmarqsi 80 mg/mL solution for infusion?
As for all newly-authorised medicines, a Risk Management Plan (RMP) has been developed for Pedmarqsi 80 mg/mL solution for infusion. The RMP details the important risks of Pedmarqsi 80 mg/mL solution for infusion, how these risks can be minimised, any uncertainties about Pedmarqsi 80 mg/mL solution for infusion (missing information), and how more information will be obtained about the important risks and uncertainties.

The following safety concerns have been recognised for Pedmarqsi 80 mg/mL solution for infusion:

Summary of safety concerns	
Important identified risks	None
Important potential risks	• Anaphylactic reactions • Medication errors
Missing information	• Long term safety

The information included in the SmPC and the PIL is compiled based on the available quality, non-clinical and clinical data, and includes appropriate precautions to be followed by healthcare professionals and patients. Side effects of Pedmarqsi 80 mg/mL solution for infusion are continuously monitored and reviewed including all reports of suspected side-effects from patients, their carers, and healthcare professionals.

An RMP and a summary of the pharmacovigilance system have been provided with this application and are satisfactory.

Other information about Pedmarqsi 80 mg/mL solution for infusion

A marketing authorisation was granted in Great Britain for Pedmarqsi 80 mg/mL solution for infusion on 11 October 2023 to Fennec Pharmaceuticals (EU) Limited as PLGB 57848/0001.

A subsequent change of ownership procedure took place on 18 April 2024 from the original marketing authorisation to the current marketing authorisation holder Norgine Pharmaceuticals Limited as PLGB 20011/0078.

The full PAR for Pedmarqsi 80 mg/mL solution for infusion follows this summary.

This summary was last updated in February 2025.

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I. INTRODUCTION

Based on the review of the data on quality, safety and efficacy, the Medicines and Healthcare products Regulatory Agency (MHRA) considered that the application for Pedmarqsi 80 mg/mL solution for infusion (PLGB 20011/0078) could be approved.

The product is approved for the prevention of ototoxicity induced by cisplatin chemotherapy in patients 1 month to <18 years of age with localised, non-metastatic, solid tumours.

The active substance in Pedmarqsi 80 mg/mL solution for infusion is sodium thiosulfate. The mechanism of sodium thiosulfate protection against ototoxicity is not fully understood, but may include increasing levels of endogenous antioxidants, inhibition of intracellular oxidative stress, and direct interaction between cisplatin and the thiol group in sodium thiosulfate to produce inactive platinum species. Concurrent incubation of sodium thiosulfate with cisplatin decreased the in vitro cytotoxicity of cisplatin to tumour cells; delaying the addition of sodium thiosulfate to these cultures prevented the protective effect.

This product has been authorised by MHRA for Great Britain (consisting of England, Scotland and Wales). In coming to its decision, MHRA has relied on a European Commission (EC) decision on 26 May 2023 (EMEA/H/C/005130/0000), in accordance with the advice from the Committee for Medicinal Products for Human Use (CHMP).

For the scientific discussion of the quality, non-clinical and clinical assessment conducted by the European Medicines Agency (EMA), please refer to the European Public Assessment Report, available on the EMA website.

This application was approved under Regulation 50 of the Human Medicines Regulation 2012, as amended (previously Article 8(3) of Directive 2001/83/EC, as amended).

In line with the legal requirements for children's medicines, the application included a licensing authority decision on the agreement of a paediatric investigation plan (PIP) P/0368/2019. At the time of the submission of the application, the PIP was completed and the PCDO issued an opinion on compliance of the PIP.

The MHRA has been assured that acceptable standards of Good Manufacturing Practice (GMP) are in place for this product at all sites responsible for the manufacture, assembly and batch release of this product.

A Risk Management Plan (RMP) and a summary of the pharmacovigilance system have been provided with this application and are satisfactory.

A marketing authorisation was granted in Great Britain for Pedmarqsi 80 mg/mL solution for infusion on 11 October 2023 to Fennec Pharmaceuticals (EU) Limited as PLGB 57848/0001. A subsequent change of ownership procedure took place on 18 April 2024 from the original marketing authorisation to the current marketing authorisation holder Norgine Pharmaceuticals Limited as PLGB 20011/0078.

II. PRODUCT INFORMATION

SUMMARY OF PRODUCT CHARACTERISTICS (SmPC)

The SmPC is in line with current guidelines and is satisfactory.

PATIENT INFORMATION LEAFLET (PIL)

The PIL is in line with current guidelines and is satisfactory.

LABEL
The labelling is in line with current guidelines and is satisfactory.

III. QUALITY ASPECTS
MHRA considered that the quality data submitted for this application is satisfactory. The grant of a marketing authorisation was recommended.

IV. NON-CLINICAL ASPECTS
MHRA considered that the non-clinical data submitted for this application is satisfactory. The grant of a marketing authorisation was recommended.

V. CLINICAL ASPECTS
MHRA considered that the clinical data submitted for this application is satisfactory. The grant of a marketing authorisation was recommended.

VI. RISK MANAGEMENT PLAN (RMP)
The applicant has submitted an RMP, in accordance with the requirements of Regulation 182 of The Human Medicines Regulation 2012, as amended. The applicant proposes only routine pharmacovigilance and routine risk minimisation measures for all safety concerns. This is acceptable.

VII. USER CONSULTATION
A full colour mock-up of the Patient Information Leaflet (PIL) was provided with the application in accordance with legal requirements, including user consultation.

VIII. OVERALL CONCLUSION, BENEFIT/RISK AND RECOMMENDATION
The quality of the product is acceptable. The non-clinical and clinical data submitted have shown the positive benefit/risk of this product in the treatment of prevention of ototoxicity induced by cisplatin chemotherapy in patients 1 month to <18 years of age with localised, non-metastatic, solid tumours.

The Summary of Product Characteristics (SmPC), Patient Information Leaflet (PIL) and labelling are satisfactory.

In accordance with legal requirements, the current approved GB version of the SmPC and PIL for this product are available on the MHRA website.

IX. TABLE OF CONTENT OF THE PAR UPDATE

Steps taken after the initial procedure with an influence on the Public Assessment Report (non-safety variations of clinical significance).

Please note that only non-safety variations of clinical significance are recorded below and in the annexes to this PAR. The assessment of safety variations, where significant changes are made, are recorded on the MHRA website or European Medicines Agency (EMA) website. Minor changes to the marketing authorisation are recorded in the current SmPCs and/or PIL available on the MHRA website.

Application type	**Scope**	**Product information affected**	**Date of grant**	**Outcome**	**Assessment report attached Y/N**