EMA/201917/2023
EMEA/H/C/005130

Pedmarqsi (*sodium thiosulfate*)

An overview of Pedmarqsi and why it is authorised in the EU

What is Pedmarqsi and what is it used for?

Pedmarqsi is a medicine used in children aged 1 month to less than 18 years old to reduce the risk of hearing loss caused by the cancer medicine cisplatin when used to treat solid tumours that have not spread.

Pedmarqsi contains the active substance sodium thiosulfate.

How is Pedmarqsi used?

Pedmarqsi can only be obtained with a prescription and must be given in a hospital under the supervision of an appropriately qualified doctor. It is given as an infusion (drip) into a vein lasting 15 minutes, exactly 6 hours after the patient has received cisplatin.

For more information about using this medicine, see the package leaflet or contact you or your child's doctor or pharmacist.

How does Pedmarqsi work?

The way Pedmarqsi works is not fully understood, but the active substance, sodium thiosulfate, is thought to act by binding to and blocking the action of cisplatin that has not been taken up by cells and preventing damage to cells caused by molecules known as 'oxygen free radicals'. These combined actions are expected to help protect the ear against hearing loss caused by cisplatin.

What benefits of Pedmarqsi 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.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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 are the risks associated with Pedmarqsi?

For the full list of side effects and restrictions with Pedmarqsi, see the package leaflet.

The most common side effects with Pedmarqsi (which may affect more than 1 in 10 people) include vomiting, nausea (feeling sick), hypernatraemia (high blood levels of sodium), hypophosphataemia (low blood levels of phosphate) and hypokalaemia (low blood levels of potassium).

The most common serious side effects with Pedmarqsi (which may affect more than 1 in 10 people) include hypersensitivity (allergic reactions).

Pedmarqsi must not be used in infants under the age of 1 month.

Why is Pedmarqsi authorised in the EU?

Hearing loss due to cisplatin is an important clinical issue for which there were no treatment options available at the time of the authorisation of Pedmarqsi. Pedmarqsi has been shown to prevent hearing loss in children and adolescents caused by cisplatin treatment for certain cancers. In addition, the safety profile of Pedmarqsi is in line with that known for sodium thiosulfate when given for other uses and is considered acceptable. The European Medicines Agency therefore decided that Pedmarqsi's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Pedmarqsi?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Pedmarqsi have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Pedmarqsi are continuously monitored. Suspected side effects reported with Pedmarqsi are carefully evaluated and any necessary action taken to protect patients.

Other information about Pedmarqsi

Pedmarqsi received a marketing authorisation valid throughout the EU on 26 May 2023.

Further information on Pedmarqsi can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/pedmarqsi.

This overview was last updated in 05-2023.