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EPAR summary for the public

Procysbi

mercaptamine

This is a summary of the European public assessment report (EPAR) for Procysbi. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Procysbi.

For practical information about using Procysbi, patients should read the package leaflet or contact their doctor or pharmacist.

What is Procysbi and what is it used for?

Procysbi is a medicine that contains the active substance mercaptamine (also known as cysteamine). It is used in patients with nephropathic (kidney) cystinosis. Cystinosis is an inherited disease in which excess amounts of cystine, an amino acid naturally found in the body, build up within cells, especially in the kidneys and the eyes, damaging them.

Because the number of patients with cystinosis is low, the disease is considered 'rare', and Procysbi was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 September 2010.

Procysbi is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but Procysbi is available in a formulation that allows for a delayed release of the active substance in the body. The reference medicine for Procysbi is Cystagon.

How is Procysbi used?

Procysbi can only be obtained with a prescription and treatment should be started under the supervision of a doctor who has experience in the treatment of cystinosis.

Procysbi is available as gastroresistant capsules (25 and 75 mg). Gastroresistant means that the capsules' contents pass through the stomach without being broken down until they reach the intestine. The recommended daily dose is calculated according to body surface area, as 1.30 g per m^2 divided

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into 2 doses given every 12 hours. Cystine levels in white blood cells (which are measured as nmol hemicystine per mg white blood cell protein), or alternatively mercaptamine concentration in the blood, should be monitored and used to adjust the dose, which should never exceed 1.95 g per m^2 per day. For further information, see the package leaflet.

How does Procysbi work?

The active substance in Procysbi, mercaptamine, reacts with cystine to form another amino acid, called cysteine, and a compound called a cysteine-cysteamine salt. The body is able to remove this salt from the cells. The amount of cystine in the organs is therefore reduced, and this limits the damage to these organs.

What benefits of Procysbi have been shown in studies?

Procysbi given every 12 hours has been shown to be at least as effective as Cystagon given every 6 hours at maintaining the amount of cystine in white blood cells at acceptable levels (less than 1 nmol hemicystine per mg of white blood cell protein). In a main study involving 43 patients with nephropathic cystinosis, there was no meaningful difference between the average levels of cystine in white blood cells during a 3-week treatment with the two medicines. Levels were 0.51 nmol/mg with Procysbi, compared with 0.44 nmol/mg with Cystagon.

What are the risks associated with Procysbi?

The most common side effects with Procysbi (which may affect more than 1 in 10 people) are loss of appetite, vomiting, nausea (feeling sick), diarrhoea, lethargy (lack of energy) and pyrexia (fever). For the full list of all side effects reported with Procysbi, see the package leaflet.

Procysbi must not be used in people who are hypersensitive (allergic) to any form of mercaptamine or any of the other ingredients, or to penicillamine. It must also not be used in women who are breastfeeding.

Why is Procysbi approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Procysbi's benefits are greater than its risks and recommended that it be approved for use in the EU. The CHMP noted that Procysbi was shown to be at least as effective as Cystagon at maintaining the amount of cystine in white blood cells at acceptable levels. The Committee also considered that the gastroresistant formulation, due to its less frequent administration, is expected to increase compliance with treatment and the quality of life of patients with cystinosis. Regarding its safety, the CHMP considered that the safety profile of mercaptamine is well established and the safety of Procysbi is expected to be similar to that of the reference medicine.

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

A risk management plan has been developed to ensure that Procysbi is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Procysbi, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Procysbi will provide educational material to all doctors expected to prescribe the medicine, containing important safety information including the risk that the medicine may be harmful to the unborn child.

Other information about Procysbi

The European Commission granted a marketing authorisation valid throughout the European Union for Procysbi on 06.09.2013.

The full EPAR for Procysbi can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Procysbi, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Procysbi can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 09-2013.