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EPAR summary for the public

Cystagon

mercaptamine

This is a summary of the European public assessment report (EPAR) for Cystagon. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Cystagon.

What is Cystagon?

Cystagon is a medicine containing the active substance mercaptamine (also known as cysteamine). It is available as capsules (50 and 150 mg).

What is Cystagon used for?

Cystagon is used in patients who have nephropathic (kidney) cystinosis. Cystinosis is a rare 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.

The medicine can only be obtained with a prescription.

How is Cystagon used?

Cystagon treatment should be started by a doctor who has experience in the treatment of cystinosis.

Cystine levels in white blood cells should be monitored and used to adjust the dose.

For children up to the age of 12 years, the recommended daily dose is calculated according to body surface area (which is calculated from the patient's height and weight), as 1.30 g per m^2 divided into 4 doses. For patients over the age of 12 and over 50 kg in weight, the recommended daily dose is 2 g divided into 4 doses. Starting doses should be one quarter to one sixth of the final expected doses. The starting dose should be increased gradually over 4 to 6 weeks. The maximum dose should never exceed 1.95 g per m^2 per day. For further information, see the package leaflet.

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How does Cystagon work?

The active substance in Cystagon, 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.

How has Cystagon been studied?

Cystagon has been investigated in three main studies including 234 patients over a 12-year period. These studies included children and newly recruited patients in whom two different doses were tested. Because the disease is very serious, it was not possible for ethical reasons to compare Cystagon directly to placebo (a dummy treatment). The comparison was done instead with a group of patients who had been treated with a dummy treatment as part of another, unrelated trial. The studies looked at the kidney function, the survival and the growth rate of the patients.

What benefit has Cystagon shown during the studies?

The three studies showed that Cystagon delays kidney problems and the need for dialysis or renal transplantation, when treatment is started at an early age. It also improves survival and growth rate in the children treated.

What is the risk associated with Cystagon?

The most common side effects with Cystagon (seen in more than 1 patient in 10) 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 Cystagon, see the package leaflet.

Cystagon must not be used in people who are hypersensitive (allergic) to cysteamine or any of the other ingredients, or to another medicine called penicillamine. It should also not be used in women who are breastfeeding, or who are pregnant (particularly during the first three months) unless it is clearly necessary.

Why has Cystagon been approved?

Cystinosis is a rare, fatal disease, and Cystagon is considered a useful medicine for this disease. The CHMP decided that Cystagon's benefits are greater than its risks and recommended that it be given marketing authorisation.

Other information about Cystagon

The European Commission granted a marketing authorisation valid throughout the European Union for Cystagon on 23 June 1997.

The full EPAR for Cystagon 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 Cystagon, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 07-2013.