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EPAR summary for the public

Cystadane

betaine anhydrous

This is a summary of the European public assessment report (EPAR) for Cystadane. 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 Cystadane.

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

What is Cystadane and what is it used for?

Cystadane is a medicine used to treat homocystinuria, an inherited disease where the amino acid homocysteine cannot be broken down and therefore builds up in the body. This causes a wide range of symptoms, including impaired vision, weak bones and circulatory problems.

It is used with other treatments, such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), folate and a special diet.

Cystadane contains the active substance betaine anhydrous.

Because the number of patients with homocystinuria is low, the disease is considered ‘rare’, and Cystadane was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 9 July 2001.

How is Cystadane used?

Treatment with Cystadane should be supervised by a doctor who has experience in the treatment of patients with homocystinuria.

Cystadane is available as a powder to be taken by mouth. The standard dose of Cystadane is 100 mg per kilogram of body weight a day, divided into two equal doses. The dose can be adjusted depending on the response to treatment (monitored by measuring the level of homocysteine in the blood). The aim of the treatment is to keep blood levels of homocysteine below 15 micromoles or as low as possible. This is usually achieved within a month.

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Cystadane is supplied with 3 measuring spoons to measure 100 mg, 150 mg and 1 g of the powder. It should be dissolved fully in water, juice, milk, formula or food immediately before being taken.

How does Cystadane work?

Betaine is a natural substance that is extracted from sugar beet. In homocystinuria, betaine reduces homocysteine levels in the blood by transforming homocysteine into the amino acid methionine. This helps to improve the symptoms of the disease.

What benefits of Cystadane have been shown in studies?

The company presented information on Cystadane from the scientific literature. This included 202 reports that described the effects of Cystadane, given at a variety of doses, on homocysteine levels in homocystinuria patients of various ages. For 140 patients, information was also provided on their symptoms, the dose and duration of treatment, and other medicines being taken. Most patients were also taking vitamins B6 or B12, or folate. The information from these studies was compared to published reports describing the outcome of untreated patients with the disease.

Patients taking Cystadane appeared to have greater reductions in homocysteine levels than untreated patients. This was associated with an improvement in symptoms affecting the cardiovascular system (heart and blood vessels) as well as an improvement in developmental problems in around three quarters of the patients taking Cystadane. The medicine was effective in patients with all three types of homocystinuria.

What are the risks associated with Cystadane?

The most common side effect when taking Cystadane (seen in more than 1 patient in 10) is elevated levels of methionine in the blood. Methionine levels should be monitored in patients taking Cystadane, as it might lead to cerebral oedema (swelling in the brain). Patients with symptoms of cerebral oedema, such as morning headaches with vomiting, or changes in vision, should speak to their doctor, as treatment with Cystadane may have to be interrupted.

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

Why is Cystadane approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that, although there were few systematic studies of Cystadane, the medicine is useful when added to existing treatments for homocystinuria, such as vitamin supplementation and using a special diet. The Committee noted that Cystadane is not a substitute for other treatments.

The Committee decided that Cystadane's benefits outweighed its risks in the adjunctive treatment of homocystinuria, when it is used according to its indication. It recommended that Cystadane be given marketing authorisation.

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

The company that makes Cystadane will set up a register of patients taking the medicine to monitor its safety. In particular, the company will monitor cases of cerebral oedema, which was seen in a small number of patients during the testing of the medicine.

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

Other information about Cystadane

The European Commission granted a marketing authorisation valid throughout the European Union for Cystadane on 15 February 2007.

The full EPAR for Cystadane 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 Cystadane, 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 Cystadane can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 10-2016