

Package leaflet: Information for the patient

Cystadane 1 g oral powder (betaine anhydrous)

Read all of this leaflet carefully before you start taking this medicine because it contains important information for you.

- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor or your pharmacist.
- This medicine has been prescribed for you only. Do not pass it on to others. It may harm them, even if their signs of illness are the same as yours.
- If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

1. What Cystadane is and what it is used for
2. What do you need to know before you take Cystadane
3. How to take Cystadane
4. Possible side effects
5. How to store Cystadane
6. Contents of the pack and other information

1. What Cystadane is and what it is used for

Cystadane contains betaine anhydrous which is intended to be an adjunctive treatment of homocystinuria, an inherited (genetic) disease where the amino acid methionine cannot be broken down completely by the body.

Methionine is present in regular food protein (e.g. meat, fish, milk, cheese, eggs). It is converted into homocysteine which is then normally converted into cysteine during digestion. Homocystinuria is a disease caused by the accumulation of homocysteine which is not converted to cysteine and is characterised by formation of clots in the veins, bone weakness, and skeletal and crystalline lens abnormalities. The use of Cystadane together with other treatments such as vitamin B6, vitamin B12, folate and a specific diet aims to reduce the elevated homocysteine levels in your body.

2. What you need to know before you take Cystadane

Do not take Cystadane

If you are allergic to betaine anhydrous.

Warnings and precautions

Talk to your doctor or pharmacist before taking Cystadane.

If you notice side effects like headaches, vomiting or a change in your vision and you are of the homocystinuria subtype called CBS (cystathionine beta-synthase deficiency), please contact your doctor immediately, they could be signs of a swelling in the brain (cerebral oedema). In that case your doctor will monitor your methionine level in your body and may review your diet. Your treatment with Cystadane may need to be interrupted.

If you are treated with Cystadane and with an amino-acid mixture and if you need to take other medicines at the same time, leave 30 minutes between the intake (see “section “Other medicines and Cystadane”).

Other medicines and Cystadane

Tell your doctor if you are taking, have recently taken or might take any other medicines.

If you are taking amino-acid mixture or medicines such as vigabatrin or Gaba analogues (medicine used to treat epilepsy), please tell your doctor as they might interact with your treatment with Cystadane.

Pregnancy and breast-feeding

If you are pregnant or breast-feeding, think you may be pregnant or are planning to have a baby, ask your doctor for advice before taking this medicine. Your doctor will decide if you can use this medicine during pregnancy and breast-feeding.

Driving and using machines

Cystadane has no or negligible influence on the ability to drive and use machines.

3. How to take Cystadane

The use of this medicine will be supervised by a doctor experienced in the treatment of patients with homocystinuria.

Always take this medicine exactly as your doctor or pharmacist has told you. Check with your doctor or pharmacist if you are not sure.

The recommended dose in children and adults is 100mg/kg/day divided in 2 doses per day. In some patients doses above 200 mg/ kg/day were needed to reach therapeutic goals. Your doctor may adapt the dose depending on your laboratory values.

You will therefore need regular blood tests to determine the correct daily dose.

You should take Cystadane orally (by mouth).

To measure the dose:

- shake the bottle lightly before opening
- take the correct measuring spoon:
 - the small green spoon measures 100 mg of betaine anhydrous powder;
 - the middle size blue spoon measures 150 mg of betaine anhydrous powder ;
 - the large pink spoon measures 1 g of betaine anhydrous powder.
- take a heaped spoonful of powder out of the bottle
- pass the flat back of a knife over the top of the spoon
- the powder left in the spoon is one spoonful
- take the correct number of spoonfuls of powder from the bottle

Mix the measured dose of powder with water, juice, milk, formula or food until completely dissolved and ingest immediately after mixing.

If you take more Cystadane than you should

If you accidentally take too much Cystadane, talk to a doctor or pharmacist immediately.

If you forget to take Cystadane

Do not take a double dose to make up for forgotten doses. If you forget to take a dose take it as soon as you remember it and continue the next dose as planned.

If you stop taking Cystadane

Do not stop the treatment without consulting your doctor. Contact your doctor or pharmacist before stopping.

If you have any further questions on the use of this medicine, ask your doctor or pharmacist.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

The most commonly side effect when taking Cystadane which may affect more than 1 in 10 people (frequency very common) is elevated levels of methionine in the blood.

Methionine level can be related to swelling in the brain (cerebral swelling), which may affect up to 1 in 100 people (frequency uncommon). If you experience morning headaches with vomiting and/or visual changes, contact immediately your doctor (they could be signs of a swelling in the brain).

Gastrointestinal disorders like diarrhoea, nausea, vomiting, stomach discomfort and inflammation of the tongue may occur uncommonly (may affect up to 1 in 100 people).

Other uncommon side effects (may affect up to 1 in 100 people) may include decreased appetite (anorexia), agitation, irritability, hair loss, hives, skin odour abnormal, lack of control over passing urine (urinary incontinence).

Reporting of side effects

If you get any side effects, talk to your doctor, nurse or pharmacist. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via

United Kingdom

Yellow Card Scheme

Website: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store

Ireland

HPRA Pharmacovigilance

Website: www.hpra.ie

By reporting side effects you can help provide more information on the safety of this medicine.

5. How to store Cystadane

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date which is stated on the bottle label and the carton after EXP. The expiry date refers to the last day of that month.

Do not store above 25°C.

Keep the bottle tightly closed in order to protect from moisture.

After the first opening of the bottle, the medicine should be used within 3 months.

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What Cystadane contains

- The active substance is betaine anhydrous. 1 g of oral powder contains 1 g of betaine anhydrous.
- There is no other ingredient.

What Cystadane looks like and contents of the pack

Cystadane is a white crystalline free flowing powder. It is presented in bottles with child resistant closures. Each bottle contains 180 g of powder. Each carton contains one bottle and three measuring spoons.

Marketing Authorisation Holder

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For any information about this medicine, please contact the local representative of the Marketing Authorisation Holder.

United Kingdom

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This leaflet was last revised in April 2019

Detailed information on this medicine is available on the European Medicines Agency web site: <http://www.ema.europa.eu/>. There are also links to other websites about rare diseases and treatments.