



EUROPEAN MEDICINES AGENCY  
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## Cystadrops (*mercaptamine*)

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

### What is Cystadrops and what is it used for?

Cystadrops is an eye medicine used in patients from 6 months of age who have cystinosis. Cystinosis is an inherited disease where a natural substance called cystine builds up in the body forming damaging crystals, particularly in the kidneys and cornea (the transparent layer in front of the eye). Cystadrops is used to reduce the build-up of cystine crystals in the cornea.

Because the number of patients with cystinosis is low, the disease is considered 'rare', and Cystadrops was designated an 'orphan medicine' (a medicine used in rare diseases) on 7 November 2008.

Cystadrops contains the active substance mercaptamine (also known as cysteamine).

### How is Cystadrops used?

Cystadrops can only be obtained with a prescription and treatment should be started under the supervision of a doctor experienced in the management of cystinosis.

Cystadrops is available as eye drops. The recommended dose is 1 drop in each eye 4 times a day during waking hours. The doctor may decrease the dose progressively to 1 drop a day depending on the results of eye examinations. Treatment should be continued long-term.

For further information, see the package leaflet.

### How does Cystadrops work?

Damage from the build-up of cystine crystals inside the cells of the cornea can cause serious vision problems. The active substance in Cystadrops, mercaptamine, reacts with cystine to dissolve it and to form substances that can be removed from the cells. When it is applied to the eye, the amount of cystine in the cells of the cornea decreases, limiting damage to the eye.

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## **What benefits of Cystadrops have been shown in studies?**

Cystadrops has been compared with another less concentrated mercaptamine eye drop solution in one main study involving 32 patients from 2 years of age with cystinosis. The main measure of effectiveness was based on the reduction in corneal cystine crystals as seen under the microscope and quantified using a score called the IVCN score. The score ranges from 0 to 28, with 0 corresponding to no crystals. At the start of the study patients in both groups had an average score of 10. Cystadrops was shown to be more effective than the comparator at decreasing corneal cystine crystals after 3 months of treatment: in patients using Cystadrops the IVCN score decreased by 4.6 points compared with a decrease of 0.5 points in patients using the comparator. Treatment with Cystadrops also led to reductions in photophobia (eye discomfort in bright light).

Additional data from five children younger than 2 years of age indicated that Cystadrops can reduce corneal cystine crystal deposits in children from 6 months of age. In addition, the safety of Cystadrops in children was shown to be similar to that in adults.

## **What are the risks associated with Cystadrops?**

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

The most common side effects with Cystadrops (which may affect more than 1 in 10 people) are related to the eye and include pain, itching and irritation in the eye, increased lacrimation (watering eyes), blurred vision and ocular hyperaemia (red eye). These effects are usually mild or moderate and do not last.

## **Why is Cystadrops authorised in the EU?**

Mercaptamine eye drop solutions made up locally in pharmacies or hospitals have been used for many years for the management of eye symptoms of cystinosis. In addition, Cystadrops has been shown in a study to be effective at decreasing corneal cystine crystals. The medicine also improved other symptoms of cystinosis such as photophobia. Regarding safety, although side effects related to the eye are very common, they are usually manageable.

The European Medicines Agency therefore decided that Cystadrops's benefits are greater than its risks and recommended that it be approved for use in the EU.

## **What measures are being taken to ensure the safe and effective use of Cystadrops?**

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

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

## **Other information about Cystadrops**

Cystadrops received a marketing authorisation valid throughout the EU on 19 January 2017.

Further information on Cystadrops can be found on the Agency's website:

[ema.europa.eu/medicines/human/EPAR/cystadrops](http://ema.europa.eu/medicines/human/EPAR/cystadrops) This overview was last updated in 06-2025.