



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## EPAR summary for the public

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# Chenodeoxycholic acid sigma-tau

## chenodeoxycholic acid

This is a summary of the European public assessment report (EPAR) for Chenodeoxycholic acid sigma-tau. 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 Chenodeoxycholic acid sigma-tau.

For practical information about using Chenodeoxycholic acid sigma-tau, patients should read the package leaflet or contact their doctor or pharmacist.

### What is Chenodeoxycholic acid sigma-tau and what is it used for?

Chenodeoxycholic acid sigma-tau is a medicine that contains the active substance chenodeoxycholic acid. This is a 'primary bile acid', which is a main component of bile (a fluid produced by the liver that helps to digest fats).

Chenodeoxycholic acid sigma-tau is used to treat adults and children from one month of age with cerebrotendinous xanthomatosis. These patients cannot produce enough of the primary bile acid chenodeoxycholic acid due to genetic abnormalities that result in a lack of the liver enzyme sterol 27 hydroxylase. When primary bile acids are lacking, the body produces abnormal bile acids and other substances instead which accumulate throughout the body, causing damage.

Because the number of patients with this condition is low, the disease is considered 'rare', and Chenodeoxycholic acid sigma-tau was designated an 'orphan medicine' (a medicine used in rare diseases) on 16 December 2014.

Chenodeoxycholic acid sigma-tau is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance. The reference medicine for Chenodeoxycholic acid sigma-tau is Xenbilox. However, Xenbilox differs from Chenodeoxycholic acid sigma-tau in that it is authorised for a different use (to dissolve cholesterol gallstones).



## **How is Chenodeoxycholic acid sigma-tau used?**

Chenodeoxycholic acid sigma-tau can only be obtained with a prescription and treatment should be started and supervised by a doctor experienced in treating cerebrotendinous xanthomatosis or other diseases of primary bile acid production.

Chenodeoxycholic acid sigma-tau is available as capsules (250 mg) which are taken 3 times a day at approximately the same time every day. The daily dose is chosen and adjusted during treatment for each patient depending on the patient's age, liver function and levels of bile acids in their blood and urine. For small children and children who cannot swallow capsules, the contents of the capsules can be mixed with sodium bicarbonate solution 8.4% to make a liquid.

For further information, see the package leaflet.

## **How does Chenodeoxycholic acid sigma-tau work?**

Chenodeoxycholic acid is one of the main primary bile acids produced by the liver. The chenodeoxycholic acid contained in this medicine replaces the patient's missing chenodeoxycholic acid. This helps to reduce the production of abnormal substances and contributes to the normal activity of bile in the digestive system, thereby relieving the symptoms of the disease.

## **What benefits of Chenodeoxycholic acid sigma-tau have been shown in studies?**

Chenodeoxycholic acid sigma-tau has been investigated in a study which looked at the records of 35 patients with cerebrotendinous xanthomatosis who received chenodeoxycholic acid for around 9 years. Among 23 patients for whom data on blood levels of bile acids were available, all had reductions in their levels (average reduction of 56–69  $\mu\text{mol/l}$ ). Among 14 patients for whom data on urine levels of bile acids were available, 79% (11 out of 14) had reduced levels at their most recent test. Most patients also had improvements in symptoms of the disease: all reported an improvement in diarrhoea, 89% of patients showed an improvement in their mental ability, 60% of patients showed improvement in mobility, and 85% and 77% of patients showed psychiatric improvement based on two different scores.

## **What are the risks associated with Chenodeoxycholic acid sigma-tau?**

The side effects seen with Chenodeoxycholic acid sigma-tau are constipation and abnormal liver test values; however their frequency could not be reliably estimated from the available limited data. They were mild or moderate in severity and did not last.

For the list of restrictions, see the package leaflet.

## **Why is Chenodeoxycholic acid sigma-tau approved?**

Chenodeoxycholic acid has been used to treat cerebrotendinous xanthomatosis for about 40 years, although it was not licensed for this use. However, due to the rarity of the disease, there are still limited data available on the use of the medicine. Nevertheless, studies have shown that the medicine benefits patients and has no significant side effects. The Agency's Committee for Medicinal Products for Human Use (CHMP) therefore decided that Chenodeoxycholic acid sigma-tau's benefits are greater than its risks and recommended that it be approved for use in the EU.

Chenodeoxycholic acid sigma-tau has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Chenodeoxycholic acid sigma-

tau due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

### **What information is still awaited for Chenodeoxycholic acid sigma-tau?**

Since Chenodeoxycholic acid sigma-tau has been approved under exceptional circumstances, the company that markets this medicine will set up a registry to monitor the benefits and safety of the medicine.

### **What measures are being taken to ensure the safe and effective use of Chenodeoxycholic acid sigma-tau?**

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

### **Other information about Chenodeoxycholic acid sigma-tau**

The European Commission granted a marketing authorisation valid throughout the European Union for Chenodeoxycholic acid sigma-tau on 10 April 2017.

The full EPAR for Chenodeoxycholic acid sigma-tau can be found on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/European public assessment reports](http://ema.europa.eu/Find%20medicine/Human%20medicines/European%20public%20assessment%20reports). For more information about treatment with Chenodeoxycholic acid sigma-tau, 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 Chenodeoxycholic acid sigma-tau can be found on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/Rare disease designation](http://ema.europa.eu/Find%20medicine/Human%20medicines/Rare%20disease%20designation).

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