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# Emcitate (*tiratricol*)

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

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

Emcitate is a medicine used to treat peripheral thyrotoxicosis (excess blood levels of certain thyroid hormones) in people with monocarboxylate transporter 8 (MCT8) deficiency. MCT8 deficiency is also known as Allan-Hernon-Dudley syndrome, a disorder affecting brain development.

Emcitate contains the active substance tiratricol and is a hybrid medicine. This means that it is similar to a reference medicine containing the same active substance, but that there are certain differences between the two. Emcitate is available as dispersible tablets (to be mixed with water before being taken), while the reference medicine was available as tablets to be taken by mouth. The reference medicine for Emcitate is Téatrois, which was marketed in France for the treatment of thyroid hormone resistance syndrome.

MCT8 deficiency is rare, and Emcitate was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 November 2017. Further information on the orphan designation can be found on the EMA website.

## How is Emcitate used?

Emcitate can only be obtained with a prescription, and treatment should be started and supervised by a doctor who is experienced in managing patients with rare genetic disorders.

The medicine is available as dispersible tablets to be taken 1 to 3 times a day. The tablets should be dispersed (mixed) in a small amount of water and then given by syringe into the mouth or through a feeding tube. The starting dose depends on the patient's body weight. The dose is increased gradually every two weeks until the blood levels of the thyroid hormone triiodothyronine (T3) are sufficiently lowered.

For more information about using Emcitate, see the package leaflet or contact your doctor or pharmacist.

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
**Send us a question** Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

## How does Emcitate work?

Thyrotoxicosis in MCT8 deficiency is caused by a mutation (change) in the gene that provides instructions for making a protein called MCT8. This protein normally transports the thyroid hormone T3 into cells of various tissues and organs, including those of the brain. Due to the gene mutation, MCT8 does not work properly and so T3 cannot enter the brain cells, causing problems with brain and muscle development. It also leads to a buildup of T3 in other parts of the body, causing problems due to too much thyroid hormone (hyperthyroidism). The active substance in Emcitate, tiratricol, is very similar to T3 but it does not need MCT8 to move in and out of cells. The medicine is therefore expected to enter the body's cells in patients with MCT8 deficiency and replace the missing T3 hormone. This helps restore normal thyroid hormone activity in the body and reduce symptoms of peripheral thyrotoxicosis.

## What benefits of Emcitate have been shown in studies?

A main study involving 46 children and adults with MCT8 deficiency that causes disease symptoms found that treatment with Emcitate reduced the average T3 blood level from 4.97 nmol/l to 1.82 nmol/l after 12 months of treatment. Supportive data also showed small improvements in signs of thyrotoxicosis such as increased heart rate, high blood pressure and premature atrial contractions (extra heartbeats). In the study, Emcitate was not compared with another medicine or placebo (a dummy treatment).

## What are the risks associated with Emcitate?

For the complete list of side effects and restrictions of Emcitate, see the package leaflet.

The most common side effects with Emcitate (which may affect up to 1 in 10 people) include hyperhidrosis (excessive sweating), diarrhoea, irritability, anxiety and nightmares. These side effects usually happen at the start of treatment, or when the dose is increased, and tend to get better within a few days.

Emcitate must not be used to treat other causes of hyperthyroidism. It must also not be taken during pregnancy.

## Why is Emcitate authorised in the EU?

At the time of approval, there was no authorised treatment in the EU for peripheral thyrotoxicosis caused by MCT8 deficiency, which is a very rare and serious condition. A study involving patients with MCT8 deficiency has shown that Emcitate reduces the average levels of the thyroid hormone T3 and leads to small improvements in signs of peripheral thyrotoxicosis. The safety of Emcitate is comparable to that of the reference medicine and is considered acceptable with appropriate dosing and monitoring.

Therefore, the Agency's view was that the benefits of Emcitate are greater than its risks and it can be authorised for use in the EU.

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

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

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

## Other information about Emcitate

Emcitate received a marketing authorisation valid throughout the EU on 12/02/2025.

Further information on Emcitate can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/emcitate.

This overview was last updated in 01-2025.