



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Trecondi (*treosulfan*)

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

What is Trecondi and what is it used for?

Trecondi is a medicine given to patients before they have a bone marrow transplant from a donor known as 'allogeneic haematopoietic stem cell transplantation'. It is used as a 'conditioning' treatment to clear the patient's bone marrow and make room for the transplanted bone marrow cells, which can then produce healthy blood cells.

Trecondi is used together with a medicine called fludarabine in adults and children from 1 month of age with blood cancers or other severe disorders requiring a bone marrow transplant.

The active substance in Trecondi is treosulfan.

Haematopoietic stem cell transplantation is rare, and Trecondi was designated an 'orphan medicine' (a medicine used in rare diseases) on 23 February 2004. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu304186.

How is Trecondi used?

Trecondi is given as a two-hour infusion (drip) into a vein. The patient receives Trecondi once a day for 3 days before the transplantation. Fludarabine is given once a day for 5 days before transplantation.

The medicine can only be obtained with a prescription and the use of Trecondi must be supervised by a doctor experienced in conditioning treatment before allogeneic haematopoietic stem cell transplantation.

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

How does Trecondi work?

The active substance in Trecondi, treosulfan, belongs to a group of medicines called alkylating agents. In the body, treosulfan is converted into other compounds called epoxides which kill cells, especially cells that develop rapidly such as bone marrow cells. Epoxides act by attaching to the DNA of cells

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while they are dividing. By doing so, Trecondi can kill cells in the patient's bone marrow and make room for the new cells from a donor.

What benefits of Trecondi have been shown in studies?

Three main studies showed that Trecondi is at least as effective as busulfan, another medicine used to prepare patients for haematopoietic stem cell transplantation.

In one of the studies, involving 570 adults with acute myeloid leukaemia (a blood cancer) or myelodysplastic syndromes (conditions in which large numbers of abnormal blood cells are produced), 64% of patients given Trecondi (with fludarabine) had a successful transplant and were alive and disease-free after 2 years, compared with 51% of patients given busulfan (with fludarabine).

In another study involving 70 children with blood cancers, 99% of the children given Trecondi (with fludarabine) were alive 3 months after their transplant; 91% of the children were alive after one year and 84% after three years.

In an additional study involving 101 children with other severe disorders requiring a bone marrow transplant, none of the 51 children given Trecondi had died from causes related to transplantation in the 3 months after the procedure, compared with 5 out of 50 children given busulfan (with fludarabine). One year after the transplant, 96% of the children given Trecondi and 88% of those given busulfan were alive.

What are the risks associated with Trecondi?

The most common side effects in adults and children with Trecondi (which may affect more than 1 in 10 people) are infections, nausea (feeling sick), stomatitis (inflammation of the lining of the mouth), vomiting, and diarrhoea. Tiredness, febrile neutropenia (low white blood cell counts with fever) and high blood levels of bilirubin (a breakdown product of red blood cells) are also seen in more than 1 in 10 adults, and liver toxicity and an increase in blood levels of certain liver enzymes, abdominal pain (belly ache), itching and fever may also affect more than 1 in 10 children.

Common side effects in adults (which may affect more than 1 in 100 people) are abdominal pain, decreased appetite, rash, itching, hair loss, fever, oedema (swelling due to fluid retention), and an increase in blood levels of certain liver enzymes. Common side effects in children (which may affect more than 1 in 100 people) are tiredness, febrile neutropenia, rash, hair loss, and high blood levels of bilirubin.

Trecondi must not be used in patients with an active, uncontrolled infection, with severe heart, lung, liver or kidney problems, and in patients with Fanconi anaemia and other DNA repair disorders. Pregnant women must not use Trecondi and live vaccines must not be given to patients receiving Trecondi.

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

Why is Trecondi authorised in the EU?

Trecondi is effective at preparing adults and children for haematopoietic stem cell transplantation.

Side effects with Trecondi are manageable and comparable to those seen with busulfan. Like busulfan, Trecondi is considered as a conditioning treatment of 'reduced-intensity': this means that it is less toxic than standard conditioning treatments which are based on chemotherapy with or without radiation.

The European Medicines Agency therefore decided that Trecondi's benefits 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 Trecondi?

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

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

Other information about Trecondi

Trecondi received a marketing authorisation valid throughout the EU on 20 June 2019.

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

This overview was last updated in 02-2023.