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EPAR summary for the public

Exjade

deferasirox

This is a summary of the European public assessment report (EPAR) for Exjade. 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 Exjade.

For practical information about using Exjade, patients should read the package leaflet or contact their doctor or pharmacist.

What is Exjade and what is it used for?

Exjade is a medicine used to treat chronic iron overload (an excess of iron in the body) in:

- patients from 6 years of age who have beta thalassaemia major (an inherited blood disorder in which patients do not have enough normal haemoglobin in the blood) and who receive frequent blood transfusions;
- children aged 2 to 5 years with beta thalassaemia major who receive frequent blood transfusions, when deferoxamine (another medicine used to treat iron overload) cannot be used or is inadequate;
- patients from 2 years of age with beta thalassaemia major who receive infrequent blood transfusions, when deferoxamine cannot be used or is inadequate;
- patients from 2 years of age who suffer from other types of anaemia (low levels of haemoglobin in the blood) and who receive blood transfusions, when deferoxamine cannot be used or is inadequate;
- patients from 10 years of age with non-transfusion-dependent thalassaemia syndromes, when deferoxamine cannot be used or is inadequate. Non-transfusion-dependent thalassaemia syndromes are blood disorders similar to beta thalassaemia major but which do not require blood transfusions. In these patients iron overload is caused by excess absorption of iron from the gut.

Exjade contains the active substance deferasirox.

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Because the number of patients with chronic iron overload is low, the disease is considered ‘rare’, and Exjade was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 13 March 2002.

How is Exjade used?

Exjade can only be obtained with a prescription and treatment should be started and supervised by a doctor who is experienced in the treatment of chronic iron overload.

It is available as film-coated tablets, dispersible tablets and granules. The film-coated tablets are to be swallowed with water while the dispersible tablets are to be mixed with a liquid to make up a suspension that the patient can drink. The granules are sprinkled on soft food such as yogurt or apple sauce that the patient can eat.

The starting dose of Exjade depends on the patient’s body weight, on which form of the medicine is being taken, what the medicine is used for, and on the level of iron overload. The dose is then adjusted as needed, every 3 to 6 months, according to the iron levels in the blood.

Exjade is taken once a day at around the same time. The dispersible tablets should be taken on an empty stomach (at least 30 minutes before food), and the film-coated tablets and granules can be taken on an empty stomach or with a light meal. For further information, see the package leaflet.

How does Exjade work?

The body cannot remove iron effectively and excess iron can cause damage. The active substance in Exjade, deferasirox, is an ‘iron chelator’. It attaches to excess iron in the body to form a compound called a ‘chelate’ that can be removed by the body, mainly in the stool. This helps to correct the iron overload and prevent damage to organs such as the heart or liver from excess iron.

What benefits of Exjade have been shown in studies?

In chronic iron overload due to blood transfusions, one main study compared Exjade with deferoxamine in 591 patients with beta thalassaemia major. About half of the patients were under the age of 16 years, and 56 were less than 6 years old. Effectiveness was determined by the level of iron in the liver before and after one year of treatment. The iron level was reduced satisfactorily in 53% of the patients receiving Exjade, compared with 66% of the patients receiving deferoxamine. Exjade was not as effective overall as the comparator medicine. However, in the 381 patients who had particularly high levels of iron at the beginning of the study and who received comparable amounts of Exjade and deferoxamine, the two medicines were as effective as each other.

Another study involved 184 patients who could not be treated with deferoxamine, including patients with beta thalassaemia major and with other types of anaemia. In more than half of these patients the iron level was reduced satisfactorily after a year of treatment with Exjade, including patients aged between 2 and 5 years.

In a further main study involving 166 patients from 10 years of age (including 21 patients aged 10 to 18 years) with non-transfusion-dependent thalassaemia syndromes and iron overload, Exjade was more effective than placebo (a dummy treatment). The main measure of effectiveness was the change in iron levels in the liver after 12 months of treatment. In patients treated with Exjade, liver iron levels decreased on average by 3.8 mg per gram of liver compared with an average increase of 0.4 mg per gram of liver in patients treated with placebo.

What are the risks associated with Exjade?

The most common side effect with Exjade (which may affect more than 1 in 10 people) is increased blood creatinine (a marker of kidney problems). Other common side effects (in up to 1 patient in 10) include nausea (feeling sick), vomiting, diarrhoea, indigestion, abdominal (belly) pain, constipation, headache, rash, itching, blood tests showing raised transaminases (which may suggest liver damage) and protein in urine. For the full list of all side effects and restrictions with Exjade, see the package leaflet.

Exjade must not be used in people whose creatinine clearance (a measure of how well the kidney is working) is less than 60 ml per minute. It must not be used in combination with other iron chelators.

Why is Exjade approved?

The European Medicines Agency decided that Exjade's benefits are greater than its risks and recommended that it be given marketing authorisation.

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

The company that makes Exjade must prepare an education pack for healthcare professionals. This pack aims to inform them about the treatment recommendations with Exjade, including choosing the right dose, that the doses are different for dispersible tablets compared to film-coated tablets or granules, and the need to monitor the patient's health, especially kidney function. The company will also prepare a similar pack for patients.

The company will also perform the following studies: a study on the long-term effects of the film-coated tablets and dispersible tablets in children aged over 10 years with non-transfusion-dependent thalassaemia; and a study to assess the safety of the film-coated tablets (particularly when they are crushed) in children.

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

Other information about Exjade

The European Commission granted a marketing authorisation valid throughout the European Union for Exjade on 28 August 2006.

The summary of opinion of the Committee for Orphan Medicinal Products for Exjade can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designations.

The full EPAR for Exjade can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Exjade, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 10-2017.