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EPAR summary for the public

Lojuxta

lomitapide

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

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

What is Lojuxta and what is it used for?

Lojuxta is a medicine that contains the active substance lomitapide. It is used to treat adult patients with homozygous familial hypercholesterolaemia, an inherited disease causing high blood levels of cholesterol (a type of fat). It is used together with a low fat diet and other medicines to reduce the level of fats in the blood. The patient's disease should be confirmed by genetic testing whenever possible.

How is Lojuxta used?

Lojuxta can only be obtained with a prescription. It is available as capsules (5, 10, 20, 30, 40 and 60 mg) to be taken by mouth on an empty stomach, at least two hours after the evening meal. Treatment should be started and monitored by a doctor experienced in treating conditions involving high levels of fats in the blood. Treatment should begin at a dose of 5 mg once per day, and if well tolerated may be steadily increased to a maximum dose of 60 mg per day. Patients with moderately or severely reduced liver function must not take Lojuxta. Patients on kidney dialysis may need to take a reduced dose. Patients taking certain other medicines may also need to take a reduced dose or take Lojuxta and their other medication at different times. Patients should avoid drinking grapefruit juice while taking Lojuxta. For further information, see the package leaflet.

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How does Lojuxta work?

The active substance in Lojuxta, lomitapide, blocks the action of a substance in the body called 'microsomal triglyceride transfer protein', which is present in the cells of the liver and the gut. Microsomal triglyceride transfer protein is involved in assembling fatty substances such as cholesterol and triglyceride into larger particles called lipoproteins, which are then released into the blood stream. By blocking this protein, Lojuxta decreases the level of fats released into the blood, thereby helping to reduce the level of cholesterol in hypercholesterolaemia.

What benefits of Lojuxta have been shown in studies?

The benefits of Lojuxta in reducing blood cholesterol were assessed in a main study involving 29 patients with homozygous familial hypercholesterolaemia. All patients were given Lojuxta together with other medicines for reducing the level of fats in the blood. Lojuxta was not compared with any other treatment. The main measure of effectiveness was the change in the patients' blood levels of 'low density lipoprotein' (LDL) cholesterol, commonly known as 'bad cholesterol', after 26 weeks of treatment. On average, the patients' LDL cholesterol levels were reduced by 40%.

What are the risks associated with Lojuxta?

The most serious side effect seen in some patients treated with Lojuxta is abnormally raised liver enzyme levels. The most common side effects are problems with the gut, which may affect as many as 9 out of 10 people: diarrhoea and nausea (feeling sick) were each seen in around 7 out of 10 people, dyspepsia (heartburn) and vomiting were each seen in more than 3 out of 10 people, while pain, discomfort and bloating of the abdomen (belly), constipation and flatulence were each seen in at least 2 out of 10 people. For the full list of side effects, see the package leaflet.

Lojuxta must not be used in women who are pregnant. It must also not be used by patients with moderately or severely reduced liver function or with unexplained, abnormal liver test results, or by patients with significant or long-term bowel problems. Lojuxta must not be used together with over 40 mg per day of simvastatin (another medicine used to lower blood cholesterol levels) or with certain other medicines that affect the way lomitapide is broken down in the body. For the full list of restrictions, see the package leaflet.

Why is Lojuxta approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Lojuxta's benefits are greater than its risks and recommended that it be approved for use in the EU. The CHMP considered that the medicine's effect in reducing LDL cholesterol levels was a benefit for patients with homozygous familial hypercholesterolaemia, who have an unmet medical need. However, the CHMP noted that the long-term benefit for the heart and circulatory system still needed to be confirmed. The Committee also noted that Lojuxta had side effects in the gut in most patients, which caused some patients to stop treatment, and that it led to increased liver enzyme levels whose long-term consequences are not known. Therefore, the Committee considered that these effects need to be closely monitored and managed.

Lojuxta has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Lojuxta to date 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 Lojuxta?

Since Lojuxta has been approved under exceptional circumstances, the company that markets the medicine is carrying out a long-term study in patients taking Lojuxta to provide further data on its safety and effectiveness, including its side effects on the liver, stomach, gut, and cardiovascular system. The study will also provide data on pregnancies in women taking the medicine, and on healthcare professionals' compliance with the recommendations to screen and monitor patients before and during treatment.

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

A risk management plan has been developed to ensure that Lojuxta is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Lojuxta, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Lojuxta will provide educational materials to all doctors expected to prescribe Lojuxta, containing information on how to select suitable patients as well as key safety information, including side effects, interactions with other medicines and use in women who could potentially have children. It will also contain educational materials to be given to patients, including a brochure and an alert card.

Other information about Lojuxta

The European Commission granted a marketing authorisation valid throughout the European Union for Lojuxta on 31 July 2013.

The full EPAR for Lojuxta 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 Lojuxta, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 01-2016.