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Waylivra (*volanesorsen*)

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

What is Waylivra and what is it used for?

Waylivra is a medicine used to treat familial chylomicronaemia syndrome (FCS), a genetic condition that gives rise to high levels of fats called triglycerides in the blood. Excess fat builds up in various parts of the body and leads to symptoms including abdominal pain (belly ache), deposits of fat under the skin and pancreatitis (inflammation of the pancreas).

Waylivra, along with a low fat diet, is used to reduce triglyceride blood levels in patients with FCS that has been confirmed by genetic testing. It is only given to patients in whom other medicines to reduce triglycerides have not worked and who are at high risk of developing pancreatitis.

FCS is rare, and Waylivra was designated an 'orphan medicine' (a medicine used in rare diseases) on 19 February 2014. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3141249.

Waylivra contains the active substance volanesorsen.

How is Waylivra used?

Waylivra is available as a solution for injection under the skin, which patients or their carers can inject themselves once they have been trained.

At the start of treatment, Waylivra is given once a week. After 3 months, patients who have had a sufficient reduction in the level of triglycerides can continue to receive the medicine once every 2 weeks. The frequency of injections is adjusted again after 6 and 9 months depending on how well the medicine is working.

Because Waylivra can reduce blood levels of platelets (components that help the blood to clot), patients' blood platelet levels should be checked regularly, in addition to regular checks of triglycerides to see how well the medicine is working. Depending on the results, frequency of injections may be adjusted, or treatment paused or discontinued.

Waylivra can only be obtained with a prescription and treatment should be supervised by a doctor who has experience in treating FCS. For more information about using Waylivra, 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 Waylivra work?

The active substance in Waylivra, volanesorsen, is an 'antisense oligonucleotide,' a very short piece of synthetic RNA (a type of genetic material). It has been designed to block the production of a protein that slows down the breakdown of fats called apolipoprotein C-III. By blocking the production of this protein, the medicine reduces the level of triglycerides in the blood and, as a result, fat accumulation in the body, which is expected to reduce the risk of pancreatitis.

What benefits of Waylivra have been shown in studies?

Waylivra was effective in reducing triglycerides in the blood in a study of 67 patients with FCS. After 3 months, patients given Waylivra had an average 77% reduction in the level of triglycerides compared with an average 18% reduction in patients given placebo (a dummy treatment). All patients in the study were on a low fat diet in addition to receiving Waylivra or placebo.

What are the risks associated with Waylivra?

The most common side effects with Waylivra are reduced platelet levels (which may affect 4 in 10 people) and reactions at the site of the injection such as pain, swelling, itching, or bruising (which may affect 8 in 10 people).

Waylivra must not be used in patients who have long-term or unexplained thrombocytopenia (low blood platelet levels).

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

Why is Waylivra authorised in the EU?

Waylivra effectively reduces triglyceride levels and is expected to reduce the risk of pancreatitis in patients with FCS. The main risk with this medicine is thrombocytopenia which, when severe, can lead to bleeding. Patients must therefore be monitored closely and the frequency of injections adjusted if needed.

The European Medicines Agency decided that, for patients at high risk of pancreatitis and for whom other medicines and a low-fat diet have not worked well enough, Waylivra's benefits are greater than its risks and that it can be authorised for use in the EU.

Waylivra has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Waylivra?

Since Waylivra has been given conditional authorisation, the company that markets Waylivra will provide results of a study based on a registry of patients to investigate how blood checks and adjustments to frequency of injections are carried out in practice and how well they work to prevent thrombocytopenia and bleeding.

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

The company that markets Waylivra will provide educational materials for patients and healthcare professionals with information on the risk of thrombocytopenia and bleeding with treatment and the importance of regularly checking platelet levels in patients.

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

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

Other information about Waylivra

Waylivra received a conditional marketing authorisation valid throughout the EU on 3 May 2019.

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

This overview was last updated in 04-2019.