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Evkeeza (*evinacumab*)

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

What is Evkeeza and what is it used for?

Evkeeza is a medicine used together with low-fat diet and other medicines to reduce levels of cholesterol in the blood. It is used in adults, adolescents and children aged 5 years and older with homozygous familial hypercholesterolaemia. This is an inherited disease that increases the levels of low-density lipoprotein cholesterol (LDL cholesterol or 'bad cholesterol') in the blood, which is a known risk factor for cardiovascular disease.

Evkeeza contains the active substance evinacumab.

How is Evkeeza used?

Evkeeza can only be obtained with a prescription, and treatment should be started and supervised by a doctor experienced in the treatment of lipid disorders (altered fat levels in the blood). Patients should be on stable doses of other cholesterol-lowering treatments before they can start treatment with Evkeeza.

Evkeeza is given as an infusion (drip) into a vein for 60 minutes every four weeks.

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

How does Evkeeza work?

The active substance in Evkeeza, evinacumab, is a monoclonal antibody (a type of protein) that has been designed to attach to ANGPTL3, a protein that blocks certain lipases (enzymes that break down fats) in the body. Once evinacumab attaches to ANGPTL3, the lipases can function again, which lowers the blood levels of fats and reduces cholesterol.

What benefits of Evkeeza have been shown in studies?

A main study showed that Evkeeza effectively reduced LDL cholesterol levels in adults and adolescents aged 12 years and older with homozygous familial hypercholesterolaemia. The participants received Evkeeza or placebo (a dummy treatment) while also taking other cholesterol-lowering therapies.

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The study involved 65 patients who received either Evkeeza or placebo once every four weeks. After 24 weeks, the average LDL cholesterol levels in the blood of patients receiving Evkeeza had reduced by around 47% from the start of the treatment, compared with about a 2% increase in patients receiving placebo. The improvement in LDL cholesterol levels with Evkeeza was maintained when treatment was given for 24 additional weeks.

An additional main study looked at the effectiveness of Evkeeza in 14 children aged 5 to 11 years with homozygous familial hypercholesterolaemia. In this study, Evkeeza was not compared with another treatment or placebo. After 24 weeks of treatment, Evkeeza lowered LDL cholesterol levels by 48%.

A study in 6 children aged 5 to 11 years showed that Evkeeza acts in the same way in the body of younger children as in that of adolescents and adults.

What are the risks associated with Evkeeza?

The most common side effect with Evkeeza (which may affect more than 1 in 10 people) is inflammation of the nose and throat. Other side effects (which may affect up to 1 in 10 people) are flu-like illness, dizziness, back pain and nausea (feeling sick). The most frequent serious side effect (which may affect up to 1 in 100 people) is anaphylaxis (sudden severe allergic reaction).

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

Why is Evkeeza authorised in the EU?

Two studies showed that adding Evkeeza to other cholesterol-lowering treatments effectively reduced LDL cholesterol levels in the blood of adults and adolescents with homozygous familial hypercholesterolaemia. A third study in children aged 5 to 11 years showed comparable results. However, the long-term benefits for the heart and circulatory system still need to be studied. Side effects with Evkeeza were acceptable, and most patients could receive prolonged treatment (at least one year) without needing to stop.

Although uncertainties remain, the European Medicines Agency therefore decided that Evkeeza's benefits are greater than its risks, and it can be authorised for use in the EU.

Evkeeza has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Evkeeza due to the rarity of the disease. 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 Evkeeza?

Since Evkeeza has been authorised under exceptional circumstances, the company that markets Evkeeza will provide results every year, highlighting the medicine's long-term safety, outcomes of any pregnancies that occur, and the effect on fatty deposits in the arteries (atherosclerosis). The company that markets Evkeeza will collect these results from an ongoing registry (a collection of information) of patients with homozygous familial hypercholesterolaemia.

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

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

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

Other information about Evkeeza

Evkeeza received a marketing authorisation valid throughout the EU on 17 June 2021.

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

This overview was last updated in 12-2023.