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Adakveo (*crizanlizumab*)

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

What is Adakveo and what is it used for?

Adakveo is a medicine for preventing painful crises in patients with sickle cell disease aged 16 years and older.

Sickle cell disease is a genetic condition in which the red blood cells become rigid and sticky and change from being disc-shaped to being crescent-shaped (like a sickle). They can block the flow of blood in blood vessels, causing painful crises that affect the chest, abdomen and other parts of the body.

Adakveo can be used as an add-on treatment with hydroxycarbamide (also known as hydroxyurea) or on its own in patients for whom hydroxycarbamide does not work well enough or causes too many side effects. Adakveo contains the active substance crizanlizumab.

Sickle cell disease is rare, and Adakveo was designated an 'orphan medicine' (a medicine used in rare diseases) on 9 August 2012. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3121034.

How is Adakveo used?

Adakveo is given by infusion (drip) into a vein lasting 30 minutes and the dose depends on the patient's weight. The first two infusions are given 2 weeks apart. Subsequent infusions are given every 4 weeks.

The medicine can only be obtained with a prescription. Treatment should be started by a doctor experienced in treating sickle cell disease.

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

How does Adakveo work?

The active substance in Adakveo, crizanlizumab, is a monoclonal antibody (a type of protein) designed to attach to a substance, P-selectin, present on the surface of the cells lining blood vessels. P-selectin helps cells stick to the blood vessels and plays a role in the clogging up of vessels during painful crises

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

in sickle cell disease. By attaching to and blocking the action of P-selectin, the medicine helps prevent painful crises.

What benefits of Adakveo have been shown in studies?

A main study in 198 patients with sickle cell disease showed that Adakveo was effective at reducing the number of painful crises. In this study, patients given Adakveo had on average 1.6 crises per year, while patients given placebo (a dummy treatment) had on average 3 crises per year.

The study also showed that Adakveo reduced the yearly number of crises by almost a third in patients already taking hydroxycarbamide (2.4 versus 3.6) and by half in patients not taking hydroxycarbamide (1 versus 2).

What are the risks associated with Adakveo?

The most common side effects with Adakveo (which may affect more than 1 in 10 people) are joint pain, nausea, back pain, fever and abdominal (belly) pain. Severe joint pain or fever may occur in around 1 in 100 people.

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

Why is Adakveo authorised in the EU?

The main study showed that Adakveo was effective at reducing the number of painful crises in patients with sickle cell disease. There was some uncertainty about the size of Adakveo's effect because of how the study was designed, but results showed consistent improvements with Adakveo, including a reduction in hospitalisations.

The side effects of Adakveo were relatively mild and considered manageable. The European Medicines Agency concluded that Adakveo's benefits are greater than its risks and it can be authorised for use in the EU.

Adakveo 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 Adakveo?

Since Adakveo has been given conditional authorisation, the company that markets Adakveo will provide further data on the safety and effectiveness of the medicine from two additional studies.

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

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

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

Other information about Adakveo

Adakveo received a conditional marketing authorisation valid throughout the EU on 28 October 2020.

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

This overview was last updated in 10-2020.