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Cablivi (*caplacizumab*)

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

What is Cablivi and what is it used for?

Cablivi is a medicine for use in adults and children from 12 years of age weighing at least 40 kg and who have an episode of acquired thrombotic thrombocytopenic purpura (aTTP), a blood clotting disorder. During an episode of aTTP, blood clots form in small blood vessels and the patient has a low count of platelets (components that help the blood to clot).

Cablivi is used together with plasma exchange (a procedure that removes certain antibodies from the blood) and treatments to reduce the activity of the immune system (the body's defences).

Cablivi contains the active substance caplacizumab.

aTTP is rare, and Cablivi was designated an 'orphan medicine' (a medicine used in rare diseases) on 30 April 2009. Further information on the orphan designation can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation

How is Cablivi used?

Cablivi can only be obtained with a prescription. Treatment should be started and supervised by doctors experienced in managing patients who have clotting disorders affecting small blood vessels.

Cablivi is started with a dose of 10 mg given by injection into a vein before plasma exchange. Treatment is continued with 10 mg daily given by injection under the skin in the belly after daily plasma exchange and continued for 30 days after daily plasma exchange is stopped. Treatment with Cablivi may continue for longer if necessary. Patients also receive treatments to reduce the immune system's activity.

Patients or their carers may be able to inject Cablivi themselves after appropriate training.

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

How does Cablivi work?

In patients with aTTP, levels of a substance called von Willebrand factor are increased. Von Willebrand factor acts on platelets to cause them to stick together and form blood clots. Caplacizumab, the active substance in Cablivi, is a nanobody (a small antibody) which has been designed to attach to von

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Willebrand factor in a way that stops it acting on platelets. This reduces platelets sticking together and forming clots in blood vessels and, as a result, platelet levels in the blood rise because they are no longer taken up to form clots.

What benefits of Cablivi have been shown in studies?

Two main studies have investigated the effectiveness of Cablivi in patients with aTTP who required plasma exchange to treat their condition. All patients were receiving standard treatment.

In the first study, involving 75 patients, platelet counts returned to the normal range after 3 days on average in patients treated with Cablivi, compared with nearly 5 days in those receiving placebo (a dummy treatment).

The second study, involving 145 patients, measured how long it took for the platelet count to return to the normal range and for sufficient improvement in the condition to allow daily plasma exchange to be stopped within 5 days. The study found that platelet count was likely to return to the normal range more quickly in patients treated with Cablivi compared with those receiving placebo.

Although the main studies only involved adults, the company provided additional modelling data indicating that the medicine will be as effective in children from 12 years of age who weigh at least 40 kg.

What are the risks associated with Cablivi?

The most common side effects with Cablivi (which may affect more than 1 in 10 people) are nosebleeds, headache and bleeding from gums. For the full list of side effects and restrictions with Cablivi, see the package leaflet.

Why is Cablivi authorised in the EU?

The European Medicines Agency decided that Cablivi's benefits are greater than its risks and it can be authorised for use in the EU. The Agency considered that, in patients with aTTP, Cablivi combined with plasma exchange and immunosuppression can reduce the time it takes for platelet counts to return to the normal range, which is associated with shorter duration of plasma exchange treatment and shorter stay in an intensive care facility. The most important side effect of treatment is bleeding but it is considered manageable. The company is expected to provide results of a study on Cablivi's safety and effectiveness over a longer period.

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

The company that markets Cablivi will provide materials, including a patient alert card, on the risk of serious bleeding and how the risk should be managed.

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

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

Other information about Cablivi

Cablivi received a marketing authorisation valid throughout the EU on 31 August 2018.

Further information on Cablivi can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.

This overview was last updated in 05-2020.