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Questions and answers

Withdrawal of the application for a change to the marketing authorisation for Ceprotin (human protein C)

On 22 October 2014, Baxter AG officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a new indication for Ceprotin to treat acquired protein C deficiency.

What is Ceprotin?

Ceprotin is a medicine that is used to treat patients with severe congenital (hereditary) protein C deficiency and for the short-term prevention of blood clotting in patients with severe congenital protein C deficiency. Detailed information on the approved use of Ceprotin in the European Union (EU) can be found here.

Ceprotin contains the active substance human protein C. It has been authorised in the EU since July 2001.

What was Ceprotin expected to be used for?

Ceprotin was also expected to be used in patients with 'acquired' protein C deficiency. 'Acquired' means that the disease is not inherited but develops during the patient's life and may occur for different reasons such as severe infection, liver disease or vitamin K deficiency.

How is Ceprotin expected to work?

Ceprotin contains human protein C, extracted and purified from human plasma (the liquid part of the blood). In the body, protein C controls the generation of thrombin, one of the proteins involved in blood clotting. Protein C slows down the production of thrombin, and therefore slows down clotting. An injection of Ceprotin produces an immediate but temporary increase in levels of protein C, controlling clotting problems in protein C-deficient patients.

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What did the company present to support its application?

The applicant presented data from 2 main studies involving a total of 52 children with protein C deficiency due to severe infection, where Ceprotin was compared with placebo (a dummy treatment). Results from a retrospective study based on medical records of 94 children with congenital or acquired protein C deficiency were also provided.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn while CHMP was still evaluating the initial documentation provided by the company.

What was the recommendation of the CHMP at that time?

As the CHMP was evaluating the initial documentation provided by the company, it had not yet made any recommendations.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of application, the company stated that it withdrew the application because in its preliminary assessment the CHMP was requesting additional clinical data to support the claim that Ceprotin is also effective in patients with acquired protein C deficiency.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that there are no consequences for patients in clinical trials or compassionate use programmes.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, contact the doctor who is giving it to you.

What is happening with Ceprotin for the treatment of patients with severe congenital protein C deficiency?

There are no consequences on the use of Ceprotin in its authorised indication.

The full European Public Assessment Report for Ceprotin can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.