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Questions and answers

Withdrawal of the marketing authorisation application for Begedina (begelomab)

On 4 July 2016, Adienne S.r.l. S.U. officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Begedina, for the treatment of graft-versus-host disease.

What is Begedina?

Begedina is a medicine that contains the active substance begelomab. It was to be available as a concentrate to be made into a solution for infusion (drip) into a vein.

What was Begedina expected to be used for?

Begedina was expected to be used for the treatment of acute graft-versus-host disease (a condition in which transplanted cells attack the patient's body) in adults who have had haematopoietic progenitor cell transplantation (transplant of cells that can develop into different types of blood cells) from a donor. The medicine was for use in patients whose disease did not respond to treatment with steroids.

Begedina was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 26 November 2010 for treatment of graft-versus-host disease. Further information on the orphan designation can be found here.

How was Begedina expected to work?

The active substance in Begedina, begelomab, is a monoclonal antibody (a type of protein) that has been designed to attach itself to a receptor called CD26, which is present on T cells. These are a type of white blood cells that play a role in graft-versus-host disease. By attaching to CD26, this medicine is expected to reduce the rate at which the T cells multiply. This is expected to reduce the number and

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activity of T cells from the transplant that attack the patient's organs, thereby helping to control graft-versus-host disease.

What did the company present to support its application?

The company presented the results of two studies with Begedina involving a total of 29 adults with acute graft-versus-host disease that did not respond to steroid treatment. In the studies, Begedina was not compared with any other treatment.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the CHMP had evaluated the initial documentation provided by the company and formulated a list of questions. The company had not yet responded to the questions at the time of the withdrawal.

What was the recommendation of the CHMP at that time?

Based on the review of the available data, at the time of the withdrawal, the CHMP had some concerns and was of the provisional opinion that Begedina could not have been approved for the treatment of acute graft-versus-host disease that did not respond to steroid treatment.

The CHMP considered that the data provided were insufficient to demonstrate a beneficial effect of Begedina. In addition, the safety profile and the way the medicine is expected to work in the body had not been sufficiently characterised. There were also deficiencies identified in the manufacturing process of the medicine.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the company had not provided enough data to support the application for Begedina.

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 acknowledges the need to obtain additional data from a confirmatory study.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials with Begedina.

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