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Questions and answers

Withdrawal of the application for a change to the marketing authorisation for Arzerra (ofatumumab)

On 8 November 2016, Novartis Europharm Ltd officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for Arzerra to be used in a new combination with bendamustine for the treatment of relapsed chronic lymphocytic leukaemia (CLL).

What is Arzerra?

Arzerra is a cancer medicine used to treat adults with chronic lymphocytic leukaemia (CLL), a cancer of a type of white blood cells called lymphocytes. It is used together with the cancer medicines chlorambucil or bendamustine in previously untreated patients who cannot be given treatment based on another medicine, fludarabine. It can also be used in patients whose disease has not responded to previous treatment with fludarabine and a medicine called alemtuzumab[1].

Arzerra has been authorised since April 2010. It contains the active substance ofatumumab and is available as a concentrate that is made up into a solution for infusion (drip) into a vein.

Arzerra was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 7 November 2008 for CLL. Further information on the orphan designation can be found here.

What was Arzerra expected to be used for?

Arzerra was also expected to be used in combination with bendamustine to treat adults with relapsed CLL (CLL that came back after previous treatment).

[1] On 10 November 2016, the CHMP adopted a positive opinion recommending an extension to this indication: http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/medicines/001131/smops/Positive/human_smop_001049.jsp&mid=WC0b01ac058001d127

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How does Arzerra work?

The active substance in Arzerra, ofatumumab, is a monoclonal antibody, a protein that has been designed to recognise and attach to another protein called CD20 on the surface of lymphocytes, including the cancerous lymphocytes seen in CLL. By attaching to CD20, ofatumumab stimulates the body's immune system to attack the cancerous cells, helping to control the disease.

What did the company present to support its application?

The company presented the results from a study involving 53 patients with relapsed CLL. All patients received Arzerra plus bendamustine; Arzerra was not compared with any other treatment in this study. The main measure of effectiveness was based on the number of patients who showed a partial or complete response to treatment.

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

The application was withdrawn after the CHMP had evaluated the documentation provided by the company and formulated a list of questions. After the CHMP had assessed the company's responses to the questions, there were still some unresolved issues.

What was the recommendation of the CHMP at that time?

Based on the review of the data, at the time of the withdrawal the CHMP had some concerns and was of the provisional opinion that Arzerra could not have been approved for the treatment of relapsed CLL in combination with bendamustine.

The CHMP was concerned that the study did not compare Arzerra with any other medicine and only included 53 patients with relapsed CLL. Additionally, although some patients (39 out of 53) responded to the combination Arzerra plus bendamustine, only a few patients (6 out of 53) had a complete response, and these data were not supported by further study results.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the results of the study were not considered to be robust and the CHMP concluded that the medicine could not have been approved based on the data presented by the company.

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 its decision was based on the objections raised by the CHMP with regard to the design of the study and its patient population.

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 or who may be joining clinical trials using Arzerra. If you are in a clinical trial and need more information about your treatment, contact the doctor who is giving it to you.

What is happening with Arzerra for the treatment of other diseases?

There are no consequences on the use of Arzerra in its authorised indications.

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