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Withdrawal of application for the marketing authorisation of Aliqopa (copanlisib)

Bayer AG withdrew its application for a marketing authorisation of Aliqopa for the treatment of adult patients with previously treated marginal zone lymphoma (MZL), a cancer of a type of white blood cells called B lymphocytes or B cells.

The company withdrew the application on 20 December 2021.

What is Aliqopa and what was it intended to be used for?

Aliqopa was developed as a medicine to treat adults with MZL. It was intended to be used in combination with rituximab (another cancer treatment) for previously treated MZL or on its own in adults who previously received at least two prior therapies.

Aliqopa contains the active substance copanlisib and was to be given as an infusion (drip) into a vein.

This medicine was designated an 'orphan medicine' (a medicine used in rare diseases) on 24 August 2018 for the treatment of MZL. Further information on the orphan designation can be found on the Agency's website: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182064.

How does Aliqopa work?

The active substance in this medicine, copanlisib, is expected to block the effects of an enzyme called PI3K. PI3K plays a role in the growth and survival of white blood cells and is overactive in these cells in patients with MZL. By targeting this enzyme and blocking its effects, copanlisib is expected to cause the death of the cancer cells, thereby delaying or stopping the progression of MZL.

What did the company present to support its application?

The company presented results from two main studies which looked at the effectiveness of Aliqopa in patients with indolent non-Hodgkin's lymphoma (iNHL), of whom a subset had MZL. The first study compared Aliqopa with placebo (a dummy treatment), both taken in combination with rituximab, in 95 patients with previously treated MZL and looked at how long patients lived without their disease getting worse (progression free survival). The second study evaluated the effect of Aliqopa on its own

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in 23 patients with MZL who had received at least two prior therapies. In this study Aliqopa was not compared with any other treatment and the main measure of effectiveness was the proportion of patients who showed a response to treatment (partial or complete response).

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

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and prepared questions for the company. The company had not responded to the list of questions at the time of the withdrawal.

What did the Agency recommend at that time?

Based on the review of the available information, at the time of the withdrawal, the Agency had concerns and its provisional opinion was that Aliqopa could not have been authorised for the monotherapy treatment of previously treated MZL.

In particular, the Agency had concerns about the design of the monotherapy study and raised questions about the robustness of the results because of the lack of a comparator. The Agency considered that the number of patients with MZL in the monotherapy study was too limited to draw conclusions on the medicine's benefits and safety at the time of the withdrawal.

Therefore, at the time of the withdrawal, the Agency was not able to draw conclusions on the effectiveness of Aliqopa in treating MZL and its opinion was that the benefits of Aliqopa in the monotherapy setting did not outweigh its risks.

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

In its letter notifying the Agency of the withdrawal of the application, the company stated that the decision was based on the need to await further analyses/data to further characterize the benefits and the risks, in particular for the combination treatment.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials using Aliqopa.

If you are in a clinical trial and need more information about your treatment, speak with your clinical trial doctor.