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Withdrawal of application for the marketing authorisation of Oportuzumab monatox DLRC Pharma Services (oportuzumab monatox)

DLRC Pharma Services withdrew its application for a marketing authorisation of Oportuzumab monatox DLRC Pharma Services for the treatment and prevention of recurrence of cancer of the bladder and the prevention of recurrence of papillary tumours.

The company withdrew the application on 20 August 2021.

What is Oportuzumab monatox DLRC Pharma Services and what was it intended to be used for?

Oportuzumab monatox DLRC Pharma Services was developed as a medicine for two types of bladder cancer. It was to be used for the treatment and prevention of recurrence of carcinoma-in-situ (CIS) of the urinary bladder and for the prevention of recurrence of high-grade Ta and/or T1 papillary tumours. It was to be used in patients who had undergone surgery to remove the cancer (transurethral resection) and whose cancer had not responded to BCG immunotherapy (a type of cancer treatment).

Oportuzumab monatox DLRC Pharma Services contains the active substance oportuzumab monatox and was to be injected directly into the bladder.

How does Oportuzumab monatox DLRC Pharma Services work?

Oportuzumab monatox DLRC Pharma Services consists of a fragment of an antibody (a type of protein) which is attached to a cytotoxic (cell-killing) substance. The antibody has been designed to attach to a target found on cancer cells (EpCAM), allowing the medicine to enter the cancer cell. Once the medicine is inside, the cytotoxic substance is expected to kill the cell. The medicine was also expected to trigger an immune response against the cancer cells.

What did the company present to support its application?

The company presented results from a study in 133 patients with CIS of the urinary bladder or papillary tumours (high-grade Ta or any grade T1) whose cancer did not respond to BCG

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immunotherapy. The main measures of effectiveness were the absence of signs of cancer cells (complete response) after 3 months and the duration of this response. Oportuzumab monatox DLRC Pharma Services was not compared with other medicines or placebo (a dummy treatment).

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the initial information from the company and had prepared questions for the company.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

What did the Agency recommend at that time?

Based on the review of the data, at the time of the withdrawal, the Agency had major concerns and its provisional opinion was that Oportuzumab monatox DLRC Pharma Services could not have been authorised for the treatment and prevention of recurrence of carcinoma-in-situ of the urinary bladder or for the prevention of recurrence of papillary tumours.

The Agency had concerns about the quality, safety and effectiveness of the medicine. In terms of quality, several processes were used for manufacturing the medicine, which raised questions about the measure of the medicine activity across the various processes used. Further information was also needed about compliance with good manufacturing practices and the presence of potential impurities. In terms of effectiveness, the Agency considered that the patient population involved in the main study was not compatible with the targeted indication. Also, major changes to the study design were carried out while the study was ongoing, raising issues for the interpretation of the results, and the choice of the main measures of effectiveness was not deemed appropriate. Further to that, there were doubts about the clinical relevance of the reported results on effectiveness. Finally, in terms of safety, some data seemed contradictory and had to be further explained.

Therefore, at the time of the withdrawal, the Agency had major concerns about the reliability of the data and concluded that the medicine could not have been authorised based on the data from the company.

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 they withdrew their application following feedback received from the US FDA on an application for oportuzumab monatox submitted in the United States.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that this withdrawal will have no impact on patients in clinical trials using Oportuzumab monatox DLRC Pharma Services.

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