European Medicines Agency

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QUESTIONS AND ANSWERS ON THE WITHDRAWAL OF THE MARKETING APPLICATION
for
RIQUENT

International non-proprietary name (INN): ***abetimus***

On 13 October 2006, La Jolla Limited officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Riquent, for the treatment of lupus nephritis.

What is Riquent?
Riquent is a solution for injection that contains 50 mg/ml of the active substance abetimus.

What was Riquent expected to be used for?
Riquent was expected to be used to treat lupus nephritis, an inflammation of the kidneys in patients with systemic lupus erythematosus (SLE, an auto-immune disease caused by the body's own defence system attacking normal tissue) who have a history of kidney disease and have been tested to check that they will benefit from the medicine. Riquent was expected to delay and to reduce the incidence of 'flares' (increased signs of kidney disease).
Because the number of patients with lupus nephritis is low, the disease is rare, and Riquent was designated an orphan medicine (a medicine used in rare diseases) on 20 November 2001.

How is Riquent expected to work?
Abetimus, the active substance in Riquent, is a selective immunosuppressive agent (a compound that selectively dampens down the immune system). Most patients with SLE have antibodies in their blood that are directed against double-stranded DNA (deoxyribonucleic acid). These antibodies are thought to be linked to the development of lupus kidney disease. Abetimus is a small piece of double-stranded DNA that has been designed to reduce circulating levels of these antibodies. When Riquent is injected, the levels of these antibodies in the blood are lowered and this is expected to help reduce the patient's likelihood of experiencing a 'flare'.

What documentation did the company present to support its application to the CHMP?
The effects of Riquent were first tested in experimental models before being studied in humans. There were two main studies involving a total of 529 patients with SLE, in which Riquent was compared with a placebo (a dummy treatment). The studies measured how many patients developed 'flares' and the time to the development of the flares by measuring the levels of protein in the urine and of creatinine (an enzyme that is a marker of kidney function) in the blood.

How far into the evaluation was the application when it was withdrawn?
The application was at day 114 when the company withdrew. The CHMP was evaluating the initial documentation provided by the company.

The CHMP normally takes up to 210 days to evaluate a new application. Based on the review of the initial documentation, the CHMP prepares a list of questions at day 120, which is sent to the company. Once the company has supplied responses to the questions, the CHMP reviews them and may, before giving an opinion, ask any remaining questions at day 180. Following the CHMP's opinion, it usually takes around 2 months for the European Commission to grant a licence.

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What was the recommendation of the CHMP at that time?
The CHMP was evaluating the initial documentation provided by the Company and had not yet made any recommendations.

What were the reasons given by the company to withdraw the application?
The letter from the company notifying the EMEA of the withdrawal of the application is available here.

What are the consequences of the withdrawal for patients undergoing clinical trials / compassionate use programmes with Riquent?
The company informed the CHMP that it plans to continue the ongoing clinical trials in the treatment of lupus nephritis. The Company has no compassionate use programme. A compassionate use programme is a programme through which doctors can request a medicine for a specific disease for one of their patients before the medicine is fully authorised.
If you are in a clinical trial and need more information about your treatment, contact the doctor who is giving it to you.