European Medicines Agency

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**QUESTIONS AND ANSWERS ON THE WITHDRAWAL OF THE MARKETING APPLICATION**
**for**
**KIACTA**

International non-proprietary name (INN): ***eprodisate sodium***

On 13 March 2008, Neurochem Luxco II SARL officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Kiacta for the treatment of amyloid A amyloidosis. Kiacta was designated an orphan medicinal product on 31 July 2001.

**What is Kiacta?**
Kiacta is a medicine containing the active substance eprodisate disodium. It was to be available as capsules.

**What was Kiacta expected to be used for?**
Kiacta was expected to be used to treat amyloid A amyloidosis. This is a rare, life-threatening disease that occurs in patients with long-lasting inflammation, most commonly due to rheumatoid arthritis. Amyloid A amyloidosis is caused by the build-up of insoluble 'fibrils' (fine threads) of a protein called 'amyloid A' (AA) in the organs of the body. AA is produced in the body from a protein called 'serum amyloid A', which is released by liver cells in response to inflammation. The most serious symptoms of the disease are caused by AA deposits building up in the kidneys and damaging them.

**How is Kiacta expected to work?**
The active substance in Kiacta, eprodisate disodium, is expected to work by interfering with the formation of fibrils of AA, preventing the deposits from building up in the organs. This is expected to help to prevent organ damage.

**What documentation did the company present to support its application to the CHMP?**
The effects of Kiacta were first tested in experimental models before being studied in humans.
The effectiveness of Kiacta was studied in one main study involving 183 patients with AA amyloidosis, in which Kiacta was compared with placebo (a dummy treatment). The main measure of effectiveness was the number of patients whose kidney function got significantly worse or who died over two years of treatment.

**How far into the evaluation was the application when it was withdrawn?**
The evaluation had finished and the CHMP had given a negative opinion. The company had requested a re-examination of the negative opinion, but this had not yet finished when the company withdrew.

**What was the recommendation of the CHMP at that time?**
Based on the review of the data and the company's response to the CHMP list of questions at the time of the withdrawal, the CHMP had given a negative opinion and did not recommend a marketing authorisation for Kiacta for the treatment of AA amyloidosis.

**What were the main concerns of the CHMP?**
The CHMP was concerned that the effectiveness of Kiacta in treating AA amyloidosis had not been demonstrated sufficiently in the single main study. Although there was a suggestion that Kiacta may

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be active, the Committee concluded that another study would be needed to demonstrate the medicine's effectiveness.
In addition, following an inspection of the site where the data from the study were analysed, the CHMP had concerns over the reliability of the study's findings because of the way the analysis was carried out.
Therefore, at the time of the withdrawal, the CHMP's view was that a benefit of Kiacta had not been sufficiently demonstrated and any benefits did not outweigh the identified risks.

**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 in clinical trials with Kiacta?**
The company informed the CHMP that the ongoing clinical trial with Kiacta is being stopped, so that it can carry out a second study to compare Kiacta with placebo. If you are in a clinical trial and need more information about your treatment, contact the doctor who is giving it to you.