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Withdrawal of the marketing authorisation application for Epjevy (pacritinib citrate)

On 7 February 2019, CTI Life Sciences Limited officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Epjevy, for treating symptoms of myelofibrosis in patients with severe thrombocytopenia.

What is Epjevy?

Epjevy is a medicine that contains the active substance pacritinib citrate. It was to be available as capsules to be taken by mouth.

What was Epjevy expected to be used for?

Epjevy was expected to be used to treat enlarged spleen and other symptoms of myelofibrosis, a disorder in which scar tissue builds up in the bone marrow where blood cells are produced. Epjevy was for use in patients with very low blood platelet counts (severe thrombocytopenia).

Epjevy was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 25 August 2010 for myelofibrosis (primary myelofibrosis, post-essential thrombocythaemia myelofibrosis, and post-polycythaemia vera myelofibrosis).

How does Epjevy work?

The active substance in Epjevy, pacritinib citrate, blocks the action of JAK2 and FLT3, two proteins involved in the production and growth of blood cells. Patients with myelofibrosis have too much of these proteins which cause the production of immature blood cells leading to long-term inflammation and scarring of the bone marrow and spleen enlargement. By blocking these enzymes, this medicine is expected to slow down the abnormal growth of blood cells, reducing the symptoms of the disease.

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What did the company present to support its application?

The company presented data from 2 studies in a total of 638 patients with myelofibrosis, around half of whom had thrombocytopenia. The study compared Epjevy with best available therapy. It looked at the number of patients whose spleen size reduced by at least 35% and the number of patients having at least a 50% improvement in a score measuring symptoms within 24 weeks of 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How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the CHMP had evaluated the documentation provided by the company and formulated lists of questions.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

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 lists of questions, at the time of the withdrawal, the CHMP had some concerns and was of the provisional opinion that Epjevy could not have been approved to treat myelofibrosis in patients with severe thrombocytopenia.

The CHMP considered that while the medicine was shown to be effective at reducing patients' spleen size, there was insufficient evidence that it reduced patients' symptoms, which was one of the main objectives of the treatment. In addition, the most appropriate dose of Epjevy had not yet been determined.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that, because of lack of proven effectiveness, the benefits of Epjevy 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 it could not generate the additional data required within the procedural timeframe.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials or compassionate use programmes using Epjevy.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, contact the doctor who is giving it to you.