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Questions and answers

Withdrawal of the marketing authorisation application for Infinia (alpha-1-antitrypsin)

On 21 June 2017, Kamada BioPharma Limited officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Infinia, for the treatment of adults with lung disease due to congenital deficiency of alpha-1-antitrypsin.

What is Infinia?

Infinia is a medicine containing the active substance alpha-1-antitrypsin (also known as alpha$_1$-proteinase inhibitor). It was to be available as a solution to be inhaled.

What was Infinia expected to be used for?

The medicine was expected to be used for treating lung disease in adults with congenital (inborn) deficiency of alpha-1-antitrypsin. A lack of this enzyme can cause damage to the lungs (such as emphysema and airway obstruction) which leads to breathing problems.

Infinia was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 16 November 2004 for the treatment of emphysema due to congenital alpha-1-antitrypsin deficiency. Further information on the orphan designation can be found here.

Other medicines containing alpha-1-antitrypsin are authorised in the EU for the same disease but are given as an infusion (drip) into a vein.

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How does Infinia work?

The active substance in Infinia, alpha-1-antitrypsin, is a protein in the blood which protects lung tissue from damage. It is obtained from human blood and was intended to replace the missing protein in the lungs of patients with alpha-1-antitrypsin deficiency.

What did the company present to support its application?

The company presented results of a main study in 168 patients looking at the effects of the medicine on exacerbations (sudden worsening of symptoms) of the disease. The medicine was compared with placebo (a dummy treatment) and the main measure of effectiveness was the length of time patients went without having an exacerbation.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the CHMP had evaluated the documentation provided by the company and formulated lists of questions.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, at the time of the withdrawal, the CHMP had some concerns and was of the provisional opinion that Infinia could not have been approved for the treatment of adults with lung disease due to congenital deficiency of alpha-1-antitrypsin.

The committee considered that the study failed to show beneficial effects in the population studied. In addition, there were concerns about the tolerability and safety profile of the medicine, since patients who were taking Infinia had to stop treatment due to side effects more often than patients taking placebo. There were also concerns that patients taking Infinia may produce antibodies against it which could reduce its effects or make patients more prone to allergic reactions.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the benefits of Infinia 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 needed more time to collect additional information that the CHMP requested in relation to this application.

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 Infinia.

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.