24 June 2016
EMA/430267/2016
EMEA/H/C/003936

Questions and answers

Withdrawal of the marketing authorisation application for Arikayce (amikacin)

On 8 June 2016, Insmed Limited officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Arikayce intended for the treatment of *Mycobacterium avium* complex (MAC) lung disease.

What is Arikayce?

Arikayce is an antibiotic containing the active substance amikacin. It was to be available as a suspension for inhalation.

What was Arikayce expected to be used for?

Arikayce was expected to be used to treat adults with a lung infection caused by *Mycobacterium avium* complex (MAC), a group of bacteria commonly found in the environment, such as in soil and water. It was to be used in patients whose infection has persisted despite previous treatment.

Arikayce was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 8 April 2014 for treating infections caused by MAC and similar bacteria. Further information on the orphan designation can be found here.

How is Arikayce expected to work?

The active substance in Arikayce, amikacin, is a well-established antibiotic of the group 'aminoglycosides' that works by disrupting bacterial production of vital proteins. In this medicine, amikacin is contained within microscopic fat capsules known as liposomes. These liposomes help reduce the rate at which the active substance is broken down, allowing it to remain in the body for longer. Because the medicine is breathed in, it also reaches the lungs more directly than amikacin given by injection.

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What did the company present to support its application?

The company submitted results of an early (phase 2) study in 89 adult patients with lung infections caused by MAC or similar bacteria. This study compared Arikayce with placebo (a dummy treatment) and looked mainly at how effectively the medicine cleared bacteria from patients' sputum (phlegm) after about 3 months 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How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the CHMP had evaluated the documentation provided by the company and formulated lists of questions.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, the CHMP was of the provisional opinion that Arikayce could not be approved for the treatment of MAC lung infection. The Committee's main concern was that the study submitted in the application did not provide enough evidence that Arikayce can permanently clear the bacteria from patients' sputum.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the benefits of Arikayce 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, the company stated that its decision is based on the understanding that CHMP was not going to recommend approval of the medicine on the basis of current data. The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials for Arikayce. If you are in a clinical trial and need more information about your treatment, contact the doctor who is treating you.