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Withdrawal of application for the marketing authorisation of Upkanz (deferiprone)

Apotex B.V. withdrew its application for a marketing authorisation of Upkanz for the treatment of pantothenate kinase-associated neurodegeneration.

The company withdrew the application on 10 August 2020.

What is Upkanz and what was it intended to be used for?

Upkanz was developed as a medicine for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), a rare inherited disease that causes increasing damage in the brain and can lead to conditions such as dystonia (uncontrolled muscle movement), parkinsonism and dementia. The first signs of the disease develop in childhood.

Upkanz contains the active substance deferiprone and was to be available as a liquid to take by mouth.

Upkanz was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 June 2018 for the treatment of neurodegeneration with brain iron accumulation. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3182034.

How does Upkanz work?

In patients with PKAN, there is a build-up of iron in parts of the brain, including those involved in controlling movement. This excessive amount of iron leads to damage. Deferiprone, the active ingredient in Upkanz, is an iron chelator, which means that it attaches to iron in the body. This stops the iron from causing damage and allows it to be removed from the body, mainly in the urine. Deferiprone was expected to enter the brain and help to prevent the build-up of iron in brain cells and so reduce brain damage.

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What did the company present to support its application?

The company presented results of a main study involving 89 patients from 4 years of age with PKAN. The study compared changes in the severity of dystonia in patients receiving Upkanz with those receiving placebo (a dummy treatment).

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and prepared questions for the company. The company had not responded to the last round of questions at the time of the withdrawal.

What did the Agency recommend at that time?

Based on the review of the data and the company's response to the Agency's questions, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that Upkanz could not have been authorised for the treatment of PKAN. Notably, the Agency was concerned that the main study had not clearly shown that the medicine was effective.

Therefore, at the time of the withdrawal, the Agency's opinion was that, because effectiveness was not proven, the benefits of Upkanz 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 following transfer of the medicine to another company, its development and marketing were being reassessed.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Upkanz.