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Kayfanda (*odevixibat*)

An overview of Kayfanda and why it is authorised in the EU

What is Kayfanda and what is it used for?

Kayfanda is a medicine used for treating cholestatic pruritus (intense itching due to a build-up of bile) caused by Alagille syndrome in patients aged 6 months and older.

Alagille syndrome is an inherited disease in which bile (a fluid produced in the liver that helps to break down fats) cannot drain properly from the liver, resulting in a build-up of bile acid in the liver and blood. One of the symptoms of this build-up is cholestatic pruritus.

Kayfanda contains the active substance odevixibat.

How is Kayfanda used?

Kayfanda can only be obtained with a prescription and treatment must be started and supervised by a doctor experienced in treating Alagille syndrome.

Kayfanda is available as capsules to be taken by mouth, or alternatively, the capsule can be opened, and its contents sprinkled on food or liquid, once daily. The dose depends on the patients' body weight.

If treatment causes severe diarrhoea lasting longer than three days, or if intravenous (IV) fluids are required to manage dehydration caused by the diarrhoea, the dose of Kayfanda may be reduced. If this resolves, then the dose can be increased.

For more information about using Kayfanda, see the package leaflet or contact your doctor or pharmacist.

How does Kayfanda work?

The active substance in Kayfanda, odevixibat, blocks the action of a protein known as ileal bile acid transporter (IBAT) that helps to transport bile acid from the bowel back to the blood and liver. By blocking IBAT, odevixibat facilitates removal of bile acid through the bowel and lowers the amount of bile acid in the blood, relieving the symptoms of cholestatic pruritis.

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What benefits of Kayfanda have been shown in studies?

Kayfanda was shown to reduce cholestatic pruritis in a main study involving 52 children with Alagille syndrome. In this study, patients were treated with either Kayfanda or placebo (a dummy treatment). After 24 weeks of treatment, children given Kayfanda had on average, a reduction of around 1.7 in morning and evening scratching as measured by the Albireo ObsRO caregiver questionnaire (a tool that helps caregivers record severity of the itching, using a scale from 0 to 4, with higher results indicating more severe itching) while children given placebo had on average, a reduction of around 0.8.

Treatment with Kayfanda also resulted in an improvement in sleep disturbances caused by cholestatic pruritis. After 21 to 24 weeks of treatment, children given Kayfanda had a reduction of around 43% in the days they needed help to fall asleep and a reduction of around 47% in the days they needed soothing to fall asleep, compared with a reduction of around 10% and 6% in children treated with placebo, respectively. After 20 and 24 weeks of treatment, children given Kayfanda had a reduction in their blood levels of bile acids of around 88 µmol/L compared with an increase of around 25 µmol/L in children given placebo.

What are the risks associated with Kayfanda?

For the full list of side effects and restrictions with Kayfanda, see the package leaflet.

The most common side effect with Kayfanda (which may affect more than 1 in 10 people) is diarrhoea.

Why is Kayfanda authorised in the EU?

Alagille syndrome is a rare and life-threatening condition. Severe and persistent itching affects between 45% to 88% of people with this syndrome. At the time of approval of Kayfanda, there were limited treatment options for cholestatic pruritus associated with Alagille syndrome.

Although there were some limitations with the main study, including the small number of patients and the short duration of follow-up of patients in the study, treatment with Kayfanda led to meaningful improvements in symptoms of Alagille syndrome, such as itching and associated sleep disturbances. Kayfanda was also effective in reducing blood levels of bile acids, which is the main cause of cholestatic pruritis.

While the data on the safety of Kayfanda are limited and further information needs to be gathered, the main side effects, such as those affecting the stomach and gut, are considered manageable. Based on how the medicine works, the efficacy and safety profile in adults is considered to be the same as that in children.

The European Medicines Agency therefore decided that Kayfanda's benefits are greater than its risks and that it can be authorised for use in the EU.

Kayfanda has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Kayfanda due to the rarity of the disease. The company must submit the results of a study on the long-term safety of Kayfanda for the treatment of cholestatic pruritis in patients aged 6 months and older with Alagille syndrome. Every year, the European Medicines Agency will review any new information regarding the safety and efficacy of Kayfanda.

What measures are being taken to ensure the safe and effective use of Kayfanda?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Kayfanda have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Kayfanda are continuously monitored. Suspected side effects reported with Kayfanda are carefully evaluated and any necessary action taken to protect patients.

Other information about Kayfanda

Kayfanda received a marketing authorisation under exceptional circumstances valid throughout the EU on 19 September 2024.

Further information on Kayfanda can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/kayfanda.

This overview was last updated in 08-2024.