13 November 2023
EMA/492792/2023
EMEA/H/C/004691/II/0011

Withdrawal of application to change the marketing authorisation for Bylvay (odevixibat)

Albireo withdrew its application for the use of Bylvay to treat cholestatic pruritus (intense itching due to a build-up of bile in body tissues) in patients with Alagille syndrome (ALGS).

The company withdrew the application on 22 October 2023.

What is Bylvay and what is it used for?

Bylvay is a medicine for treating patients from the age of 6 months with progressive familial intrahepatic cholestasis (PFIC), a rare type of liver disease in which bile acids build up in the liver.

Bylvay has been authorised in the EU since July 2021.

It contains the active substance odevixibat and is available as capsules.

What change had the company applied for?

The company applied to extend the use of Bylvay to treat intense itching in patients with Alagille syndrome aged 6 months or older. Alagille syndrome is an inherited disease in which bile cannot drain properly from the liver, resulting in a build-up of bile acids in the liver and blood.

Bylvay was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 July 2012 for PFIC and on 9 August 2012 for Alagille syndrome.

How does Bylvay work?

The active substance in Bylvay, odevixibat, blocks the actions of IBAT, a protein in the intestine that transports bile acid from the intestines into the liver. By blocking the actions of IBAT, odevixibat reduces the amount of bile acid that is transported from the intestines into the liver. This reduces the build-up of bile acids and prevents damage to the liver tissue.

Bylvay works in the same way in people with PFIC and Alagille syndrome.

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What did the company present to support its application?

The company presented results from a main study comparing Bylvay with placebo (a dummy treatment) in 52 patients aged from 6 months to 15.5 years with Alagille syndrome. The study measured the improvements in itching symptoms measured using a standard scoring system.

How far into the evaluation was the application when it was withdrawn?

The European Medicines Agency had completed its evaluation when the application was withdrawn by the company.

What did the Agency recommend at that time?

Based on the review of the information and the company's response to the Agency's questions, at the time of the withdrawal, the Agency had recommended authorising the use of Bylvay for treating cholestatic pruritus in patients with Alagille syndrome.

What were the reasons given by the company for withdrawing the application?

The company withdrew the application after EMA's orphan medicines committee (COMP) indicated that it was going to issue a recommendation not to maintain the orphan designation for Alagille syndrome.

However, Bylvay has an orphan designation for PFIC and under EU law a medicine cannot be authorised for an orphan condition and a non-orphan one at the same time. More information about why the company withdrew is available in the company's withdrawal letter.

There are no concerns about the balance of the benefits and risk of Bylvay in patients with Alagille syndrome. The company is working on an application for a separate marketing authorisation for this condition.

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 the ongoing clinical trial or compassionate use programme.

What is happening with Bylvay for the treatment of PFIC?

There are no consequences for the use of Bylvay to treat PFIC.