



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Bylvay (*odevixibat*)

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

### 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. Bile acids are a component of bile, a fluid produced in the liver that helps to absorb fats from the gut.

Bylvay contains the active substance odevixibat.

### How is Bylvay used?

Bylvay is available as capsules. The recommended dose is 40 micrograms per kilogram body weight. The capsules should be taken once a day in the morning. They can be taken whole or they can be opened and sprinkled on food. If the medicine is not working well enough after three months, the treating physician may increase the dose up to 120 microgram per kilogram body weight.

The medicine can only be obtained with a prescription and treatment should be started and supervised by a doctor who has experience in the management of PFIC. For more information about using Bylvay, see the package leaflet or contact your doctor or pharmacist.

### How does Bylvay work?

The active substance in Bylvay, odevixibat, blocks the actions of a protein in the intestine (known as IBAT) 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 will prevent the build-up of bile acids and damage to the liver tissue.

### What benefits of Bylvay have been shown in studies?

Bylvay was more effective than a placebo (dummy treatment) at reducing the severity of PFIC in one main study, which involved 62 patients aged between 6 months and 18 years. The main measure of effectiveness was based on the number of patients whose bile acid level in the blood decreased by at least 70% after 24 weeks of treatment.

Bylvay treatment led to the required reduction in around 44% (10 out of 23) of patients who received the standard dose (40 micrograms per kilogram body weight per day) and in around 21% (4 out of 19)

**Official address** Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands

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of patients who received the maximum daily dose (120 micrograms per kilogram body weight per day), compared with 0% (0 out of 20) of those receiving placebo. The study also showed that Bylvay can improve symptoms such as itching and prevent delayed growth.

### **What are the risks associated with Bylvay?**

The most common side effects with Bylvay (which may affect up to 1 in 10 people) are soft stools, diarrhoea, belly pain, and an enlarged liver. For the full list of side effects of Bylvay, see the package leaflet.

### **Why is Bylvay authorised in the EU?**

One main study has shown that Bylvay is effective at reducing the amount of bile acid in the blood of patients with PFIC. Bylvay was also effective at reducing signs and symptoms of PFIC such as itching. Because PFIC is a very rare disease, the study was small but the short-term data available indicated that Bylvay could delay disease progression and the need for surgery and/or liver transplantation. The side effects seen to date are considered manageable. Given the seriousness of the condition and the lack of existing treatments, the European Medicines Agency decided that Bylvay's benefits are greater than its risk and it can be authorised for use in the EU.

Bylvay has been authorised under 'exceptional circumstances'. This means that, because the indication is encountered so rarely, it has not been possible to obtain full information about the medicine. Every year, the European Medicines Agency will review any new information that may become available and this summary will be updated as necessary.

### **What information is still awaited for Bylvay?**

Since Bylvay has been authorised under exceptional circumstances, the company that markets Bylvay will conduct a study to provide data on the long-term effectiveness of Bylvay. The study will also focus on whether Bylvay treatment delays liver-related surgery and/or liver transplantation for PFIC patients.

### **What measures are being taken to ensure the safe and effective use of Bylvay?**

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

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

### **Other information about Bylvay**

Bylvay received a marketing authorisation valid throughout the EU on 16 July 2021.

Further information on Bylvay can be found on the Agency's website:  
[ema.europa.eu/medicines/human/EPAR/bylvay](https://ema.europa.eu/medicines/human/EPAR/bylvay)

This overview was last updated in 07-2021.