

# Medicine: odevixibat (brand name: Bylvay®) for progressive familial intrahepatic cholestasis

Albireo AB

Odevixibat meets the Scottish Medicines Consortium (SMC) definition of an ultra-orphan medicine, which is a medicine to treat an extremely rare condition. This document summarises the initial SMC assessment of odevixibat for treating progressive familial intrahepatic cholestasis (PFIC). It is used in patients who are six months old or older.

## What does this mean for patients?

If your healthcare professional thinks that odevixibat is the right medicine for you or your child, you or they should be able to have the treatment on the NHS in Scotland within the **ultra-orphan pathway** (see next page). This is provided the company submits a plan to the Scottish Government describing how further data, including on the patient and carer lived experience, will be collected over the next 3 years. After this, SMC will reassess the medicine and make a decision on routine availability.



## What is odevixibat used for?

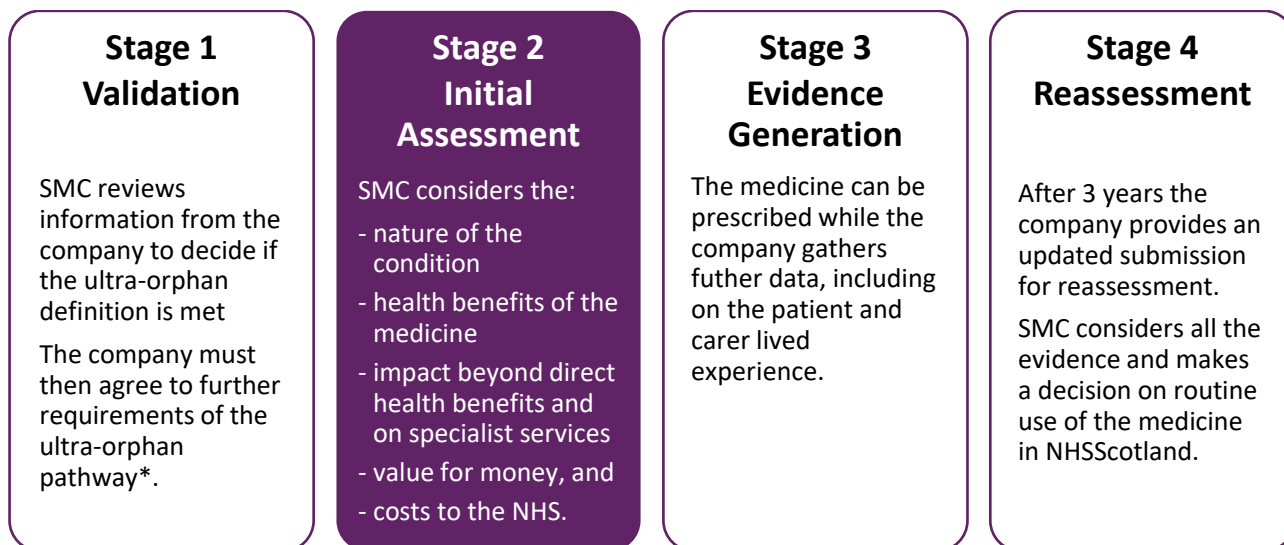
Odevixibat is used to treat patients with PFIC. PFIC is a rare group of conditions that cause bile acids (which are essential for the digestion and absorption of fats), to build up in the liver causing damage, with many patients progressing to end stage liver failure and needing a liver transplant. The severe itchiness caused by the condition has a big impact on quality of life, leading to skin damage, sleep deprivation, irritability, poor attention and difficulties at school. It also causes fats to not be absorbed properly, which affects growth and weight, and can lead to vitamin deficiency.

## How does odevixibat work?

Odevixibat works by blocking a protein called IBAT. This protein is present in the intestines and transports bile acids into the liver. By blocking IBAT odevixibat can reduce the amount of bile acids that build up in the liver and may help limit liver damage.

## How do we assess ultra-orphan medicines?

SMC uses a broad assessment framework for ultra-orphan medicines. This is part of the ultra-orphan pathway in NHSScotland which has **four stages**:



\*provide a confidential discount known as a Patient Access Scheme (PAS) to increase the cost-effectiveness of the medicine, and provide a data collection plan.

### What have we said in this assessment?

- Odevixibat reduced serum bile acids and itchiness compared with placebo (a dummy treatment), however there is still uncertainty about whether it delays the need for liver transplant and further data are awaited.
- The clinical trial showed that there was a general improvement in quality of life with odevixibat compared with placebo.
- Despite the confidential discount offered by the company, the cost in relation to the health benefits of odevixibat remains high.

For further information please see the SMC ultra-orphan medicine initial assessment report (SMC2411).



### More information

The organisation below can provide more information and support for people with PFIC and their families. SMC is not responsible for the content of any information provided by external organisations.

#### Children's Liver Disease Foundation (CLDF)



<https://childliverdisease.org>



0121 212 3839

You can find out more about odevixibat (Bylvay®) in the Patient Leaflet by searching for the medicine name on the electronic medicines compendium (EMC) website.



<https://www.medicines.org.uk/emc/>