



NCPE Plain English Summary

Drug name: Odevixibat (*pronounced: Odev - ixibat*) is a treatment for progressive familial intrahepatic cholestasis (PFIC) in patients aged six months or older.

Brand name: Bylvay®

What is the NCPE?

The National Centre for Pharmacoeconomics (NCPE) is a team of experts who look at the health benefits and costs of medicines. The HSE asks us to advise on whether or not a new medicine is good value for money. We give unbiased advice to help the HSE provide the most effective, safe and cost-effective (value for money) treatments for patients.

How do we make our recommendations?

Our main focus is on the health benefits and cost effectiveness of a medicine. We look at the wider costs and health benefits associated with a new medicine, for example:

- Does the new medicine work better than other treatments available in Ireland?
- Is the new medicine easier to give or easier to take compared with other treatments available in Ireland?
- Does the new medicine reduce the need for patients to be hospitalised?
- Does the new medicine improve the quality of a patient's life over other treatments available in Ireland?
- Will the new medicine save resources elsewhere within the health system?

We review the information from clinical trials along with the cost and value for money data presented by the pharmaceutical company. We ask doctors and other healthcare professionals for advice about any health benefits of the new medicine compared with current treatments. We also ask patient organisations to send us their views on how the new drug may improve patients' day-to-day experience of living with a disease.

What is odevixibat used for?

Odevixibat is used to treat progressive familial intrahepatic cholestasis which is a rare, inherited liver disorder associated with raised serum bile acid levels and severe pruritis (itching). Progression to severe liver disease occurs and frequently requires surgical intervention, including liver transplantation. Odevixibat capsules are administered orally once a day and the number of capsules to be taken depends on the patients weight, with a

greater number of capsules required with increasing weight. The benefits of odevixibat include a reduction in serum bile acids and reduced severity of itching. Patients quality of life is also improved. Odevixibat may reduce the requirement for surgical intervention. Patients continue to receive symptom-based therapies as required.

What recommendation has the NCPE made to the HSE?

Odevixibat is expensive and so we have recommended that the HSE should consider not funding Odevixibat for the treatment of progressive familial intrahepatic cholestasis in patients 6 months or older unless the cost-effectiveness (value for money) can be improved when compared with current treatments. The HSE will consider our recommendation and make the final decision about reimbursement (funding). When making the funding decision, the HSE will also consider the additional criteria outlined in the Health (Pricing and Supply of Medical Goods) Act 2013.

Why did we make this recommendation?

After reviewing the data presented by the pharmaceutical company, we recommend that the HSE consider not providing this medicine unless the HSE can agree a suitable price reduction with the pharmaceutical company. This is because we believe the medicine may work as well or better than other ways to manage this condition. However, the price of the medicine too high compared with other ways to manage this condition, and we believe that the medicine is very poor value for money.

Next steps

When the HSE receives our recommendation, it will look at all the relevant data about Odevixibat. The HSE makes the final decision on reimbursement.

Where can I get more information?

You can get more information about Odevixibat from the following online options:

- the NCPE Technical Summary Document
- Searching for Odevixibat on our website (www.ncpe.ie);
- searching for Odevixibat on the European Medicines Agency (EMA) website (www.ema.europa.eu).

Please refer to the NCPE website for updated information on the reimbursement status of this medicine.

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