

Odevixibat: Adis Evaluation

Clinical Considerations

- Approved in the USA for pruritus in progressive familial intrahepatic cholestasis (PFIC) and in the EU and UK for PFIC
- Significantly reduces serum bile acids and improves pruritus symptoms relative to placebo
- Improves growth and sleep parameters, as well as quality of life outcomes
- Clinical benefits maintained for up to 72 weeks of treatment, according to an interim analysis of ongoing phase 3 study data
- Generally well tolerated

Plain Language Summary

Background and rationale

- Progressive familial intrahepatic cholestasis (PFIC) is a rare inherited cholestatic liver disease that is characterized by disturbed bile homeostasis, pruritus, accumulation of biliary components and potentially fatal liver disease
- Recently, ileal bile acid transporter (IBAT) inhibitors have emerged as a novel non-surgical approach for the treatment of PFIC
- Odevixibat (Bylvay™; Bylvay®), a potent IBAT inhibitor, has been approved in the USA for the treatment of pruritus in PFIC for patients aged ≥ 3 months and in the EU and UK for patients aged ≥ 6 months with PFIC

Clinical findings

- In patients with PFIC, odevixibat significantly decreased serum bile acids and improved pruritus symptoms compared with placebo
- Improvements in growth and sleep parameters, as well as measures of quality of life were also seen with odevixibat versus placebo
- Odevixibat is generally well tolerated, with most adverse events being mild to moderate in severity

Conclusion

Odevixibat is a valuable, effective and generally well-tolerated treatment option for patients with PFIC

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