



Bylvay™ (odevixibat) – New orphan drug approval

- On July 20, 2021, [Albireo Pharma announced](#) the [FDA approval](#) of [Bylvay \(odevixibat\)](#), for the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
 - Bylvay may not be effective in PFIC type 2 patients with ABCB11 variants resulting in nonfunctional or complete absence of bile salt export pump protein.
- PFIC is a rare disorder that causes progressive, life-threatening liver disease. Patients have impaired bile flow, or cholestasis, caused by genetic mutations. The most prominent manifestation of the disease is pruritus, or intense itching. Other symptoms include jaundice, poor weight gain and slowed growth. In some cases, PFIC leads to cirrhosis and liver failure.
- Bylvay is a non-systemic ileal bile acid transport inhibitor and the first drug approved for the treatment of pruritus in all subtypes of PFIC.
- The efficacy of Bylvay was established in a 24-week, randomized, double-blind, placebo-controlled study in 62 pediatric patients, aged 6 months to 17 years, with a confirmed molecular diagnosis of PFIC type 1 or type 2, and presence of pruritus at baseline. Patients were randomized to placebo, Bylvay 40 mcg/kg, or Bylvay 120 mcg/kg. The primary endpoint was the patients' percentage of observer-reported outcome (ObsRO) assessments over the 24-week treatment period that were scored as 0 (no scratching) or 1 (a little scratching).
 - The mean percentage of assessments scored as 0 (no scratching) or 1 (a little scratching) was 13.2, 35.4, and 30.1 for placebo, Bylvay 40 mcg/kg/day (mean difference vs. placebo: 22.2; 95% CI: 4.7, 39.6), and Bylvay 120 mcg/kg/day (mean difference vs. placebo: 16.9; 95% CI -2.0, 35.7), respectively.
- Warnings and precautions for Bylvay include liver test abnormalities, diarrhea, and fat-soluble vitamin (FSV) deficiency.
- The most common adverse reactions (> 2%) with Bylvay use were liver test abnormalities, diarrhea, abdominal pain, vomiting, and FSV deficiency.
- The recommended dosage of Bylvay is 40 mcg/kg once daily in the morning with a meal. If there is no improvement in pruritus after 3 months, the dosage may be increased in 40 mcg/kg increments up to 120 mcg/kg once daily not to exceed a total daily dose of 6 mg.
 - Bylvay oral pellets are intended for use by patients weighing less than 19.5 kilograms.
 - Bylvay capsules are intended for use by patients weighing 19.5 kilograms or above.
- Albireo Pharma plans to launch Bylvay immediately. Bylvay will be available as 200 mcg and 600 mcg oral pellets and 400 mcg and 1200 mcg oral capsules.



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