

## Bylvay<sup>®</sup> (odevixibat) – New orphan indication

- On June 13, 2023, [Ipsen announced](#) the FDA approval of [Bylvay \(odevixibat\)](#), for the treatment of cholestatic pruritus in patients 12 months of age and older with Alagille syndrome (ALGS).
- Bylvay is also approved for the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
- ALGS is a rare genetic disorder that can affect multiple organ systems in the body. Liver damage may result from having fewer than normal, narrowed, or malformed bile ducts, which leads to toxic bile acid build-up, which in turn can cause scarring and progressive liver disease.
  - Most patients with ALGS present with chronic cholestasis and severe intractable pruritus.
  - Currently in the U.S., it is estimated that there are 1,300 patients who may be eligible for treatment.
- The approval of Bylvay for the new indication was based on a randomized, double-blind, placebo-controlled study in 52 pediatric patients, aged 6 months to 15 years, with a confirmed diagnosis of ALGS and presence of pruritus at baseline. Patients were randomized to Bylvay or placebo. A single-item observer-reported outcome (ObsRO) was used to measure patients' scratching severity as observed by their caregiver twice daily (once in the morning and once in the evening). Scratching severity was assessed on a 5-point ordinal response scale, with scores ranging from 0 (no scratching) to 4 (worst possible scratching).
  - The mean change from baseline in scratching score at month 6 was -0.8 with placebo vs. -1.7 with Bylvay (treatment difference -0.9, 95% CI: -1.4, -0.3; p = 0.002).
- The most common adverse reactions (> 5%) with Bylvay use for ALGS were diarrhea, abdominal pain, hematoma, and decreased weight.
- The recommended dosage of Bylvay for ALGS is 120 mcg/kg taken orally once daily in the morning with a meal. The table below shows the recommended once daily dosage by body weight.

Body weight (kg)	Once daily dosage (mcg)
7.4 and below	600
7.5 to 12.4	1,200
12.5 to 17.4	1,800
17.5 to 25.4	2,400
25.5 to 35.4	3,600
35.5 to 45.4	4,800
45.5 to 55.4	6,000
55.5 and above	7,200

- 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.
- Refer to the Bylvay drug label for dosing for PFIC.