CareSource

PHARMACY POLICY STATEMENT
North Carolina Marketplace

DRUG NAME	Bylvay (odevixibat)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Bylvay, approved by the FDA in July 2021, is indicated for the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC). It is the first drug approved for PFIC and is available as oral pellets or capsules, taken once daily.
PFIC is an ultra-rare group of genetic disorders that disrupt bile formation in the liver. It usually presents during infancy and is characterized by cholestasis, jaundice, and intense itching. Most patients will eventually require biliary diversion surgery or liver transplant. PFIC types 1, 2, and 3 are the most common and types 1 and 2 are the most severe. PFIC1 involves extrahepatic manifestations while PFIC2 does not. However, PFIC2 can be complicated by hepatocellular carcinoma. Less is known about the other PFIC subtypes.
In cholestatic liver disease, biliary substances aren't eliminated from the liver, thus they re-enter circulation. Cholestatic itch is thought to be related to the accumulation of bile acids in the skin. Bylvay is a reversible inhibitor of the ileal bile acid transporter (IBAT). Inhibiting IBAT decreases reuptake of bile salts, as observed by a decrease in serum bile acids, which helps improve pruritis. The pivotal clinical trials PEDFIC1 and PEDFIC2 met both primary endpoints of improving pruritis and reducing serum bile acids (sBA).

Bylvay (odevixibat) will be considered for coverage when the following criteria are met:

Progressive familial intrahepatic cholestasis (PFIC)

For **initial** authorization:

1. Member is at least 3 months of age; AND
2. Medication must be prescribed by or in consultation with a gastroenterologist or hepatologist; AND
3. Member has a diagnosis of PFIC type 1 or 2 confirmed by genetic testing identifying pathogenic mutations of the ATP8B1 or ABCB11 genes (results must be provided for review); AND
4. Member has significant pruritis not attributed to another cause; AND
5. Documentation of serum bile acid level ≥ 100 µmol/L; AND
6. Documentation of baseline liver tests (e.g., ALT, AST, bilirubin, INR); AND
7. Trial and failure of ursodiol (may also continue concurrently); AND
8. Member does NOT have any of the following:
   a) Decompensated cirrhosis
   b) Variants of the ABCB11 gene (PFIC type 2) that code for non-functional or complete absence of the bile salt export pump (BSEP-3) protein (per submitted genetic test result)
   c) Biliary diversion surgery in the past 6 months
   d) Liver transplant
9. **Dosage allowed/Quantity limit:** 40 mcg/kg orally once daily. If no improvement after 3 months, may increase in 40 mcg/kg increments up to 120 mcg/kg. Max dose per day 6 mg.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must show improvement of pruritis compared to baseline; AND
2. Chart notes must show reduced serum bile acid from baseline; AND
3. Member has NOT experienced portal hypertension or a hepatic decompensation event (e.g., variceal hemorrhage, ascites, hepatic encephalopathy) while being treated with Bylvay.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Bylvay (odevixibat) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
11/17/2021	New policy for created for Bylvay.

References:

1. Bylvay [prescribing information]. Albireo Pharma, Inc.; 2021.
2. Baumann U, Sturm E, Lacaille F, et al. Effects of odevixibat on pruritus and bile acids in children with cholestatic liver disease: Phase 2 study. *Clin Res Hepatol Gastroenterol*. 2021;45(5):101751. doi:10.1016/j.clinre.2021.101751
3. Deeks ED. Odevixibat: First Approval [published correction appears in Drugs. 2021 Sep 23;:]. *Drugs*. 2021;81(15):1781-1786. doi:10.1007/s40265-021-01594-y
4. Kamath BM, Stein P, Houwen RHJ, Verkade HJ. Potential of ileal bile acid transporter inhibition as a therapeutic target in Alagille syndrome and progressive familial intrahepatic cholestasis. *Liver Int*. 2020;40(8):1812-1822. doi:10.1111/liv.14553
5. Gunaydin M, Bozkurter Cil AT. Progressive familial intrahepatic cholestasis: diagnosis, management, and treatment. *Hepat Med*. 2018;10:95-104. Published 2018 Sep 10. doi:10.2147/HMER.S137209
6. Siddiqi I, Tadi P. Progressive Familial Intrahepatic Cholestasis. [Updated 2021 Sep 29]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2021 Jan-. Available from: https://www.ncbi.nlm.nih.gov/books/NBK559317/
7. Baker A, Kerkar N, Todorova L, Kamath BM, Houwen RHJ. Systematic review of progressive familial intrahepatic cholestasis [published correction appears in Clin Res Hepatol Gastroenterol. 2020 Feb;44(1):115]. *Clin Res Hepatol Gastroenterol*. 2019;43(1):20-36. doi:10.1016/j.clinre.2018.07.010
8. Srivastava A. Progressive familial intrahepatic cholestasis. *J Clin Exp Hepatol*. 2014;4(1):25-36. doi:10.1016/j.jceh.2013.10.005
9. Squires RH, Ng V, Romero R, et al. Evaluation of the pediatric patient for liver transplantation: 2014 practice guideline by the American Association for the Study of Liver Diseases, American Society of Transplantation and the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition. *Hepatology*. 2014;60(1):362-398. doi:10.1002/hep.27191

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