Protocol for Bylvay® (odevixibat)

Approved July 2022

Background: *Progressive familial intrahepatic cholestasis (PFIC) is a disorder that causes progressive liver disease, which typically leads to liver failure.*

Bylvay *is an ileal bile acid transporter (IBAT) inhibitor indicated for the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis.*

Criteria for approval:

1. Patient has a diagnosis of PFIC confirmed by genetic testing.
2. Patient is ≥ 3 months or older
3. Patient has significant pruritus if able to report
4. For PFIC type 2 patients, genetic testing does NOT indicate pathologic variations of the ABCB11 gene that predict non-function or complete absence of the bile salt export pump (BSEP) protein (see exclusion of therapy)
5. Medication is prescribed by or in consultation with a hepatologist or gastroenterologist
6. Patient has tried and has inadequate response, intolerance, or contraindication to treatment with ursodeoxycholic acid, or other agents used for symptomatic relief of pruritus (e.g., antihistamine, rifampicin, cholestyramine)
7. Patient's weight should be monitored
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer- reviewed evidence

Exclusion (Limitation of Use):
Bylvay may not be effective in PFIC type 2 patients with ABCB11 variants resulting in non-functional or complete absence of bile salt export pump protein (BSEP-3)

Continuation of therapy:

1. Patient is responding positively to therapy as evidenced by improvement in any of the following:
    a. Improvement in pruritus if able to report
    b. Reduction of serum bile acids from baseline
2. Patient's weight should be monitored
3. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary

Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer- reviewed evidence

Initial Approval Duration: 3 months

Renewal Approval Duration: 6 months

References:

1. Bylvay [prescribing information]. Albireo Pharma Inc. Boston, MA 02109. July 2021
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
3. Gunaydin M and Cil ATB. Progressive familial intrahepatic cholestasis: diagnosis, management, and treatment. Hepat Med. 2018;10:95-104
4. Düll, M.M., Kremer, A.E. Newer Approaches to the Management of Pruritus in Cholestatic Liver Disease. Curr Hepatology Rep 19, 86–95 (2020). https://doi.org/10.1007/s11901-020-00517-x
5. Davit-Spraul A et al. Progressive familial intrahepatic cholestasis. Orphanet Journal of Rare Diseases 2009, 4:1;10.1186/1750-1172-4-1. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2647530/ Accessed online on April 29, 2022