



5.50.031

Section:	Prescription Drugs	Effective Date:	April 1, 2025
Subsection:	Gastrointestinal Agents	Original Policy Date:	August 13, 2021
Subject:	Bylvay	Page:	1 of 5

Last Review Date: March 7, 2025

Bylvay

Description

Bylvay (odevixibat)

Background

Bylvay (odevixibat) is an inhibitor of the ileal bile acid transporter (IBAT). IBAT is almost completely responsible for the reabsorption of bile acid from the ileum, returning biliary products to systemic circulation. Inhibition of this process promotes elimination of bile acid and reduces pruritus associated with cholestatic disease (1).

Regulatory Status

FDA-approved indications: Bylvay is an ileal bile acid transporter (IBAT) inhibitor indicated for: (1)

- Progressive Familial Intrahepatic Cholestasis (PFIC)
 - the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
 - Limitation of Use: Bylvay may not be effective in a subgroup of PFIC type 2 patients with ABCB11 variants resulting in non-functional or complete absence of the bile salt export pump protein.
- Alagille Syndrome (ALGS)
 - the treatment of cholestatic pruritus in patients 12 months of age and older with Alagille syndrome (ALGS).

Patients with PFIC and ALGS may have impaired hepatic function at baseline. The efficacy and safety in PFIC and ALGS patients with clinically significant portal hypertension, and in patients with decompensated cirrhosis have not been established (1).

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Bylvay has warnings regarding the following: diarrhea, liver test abnormalities, and Fat-Soluble Vitamin (FSV) deficiency. Patients should obtain baseline levels of liver function and fat-soluble vitamins and be monitored for abnormalities in liver function and for FSV deficiency throughout treatment (1).

The Rare Disease Database includes diagnostic criteria for Alagille syndrome, including characteristic symptoms, bile duct paucity, and genetic testing (2).

The safety and effectiveness of Bylvay in pediatric patients less than 3 months of age with PFIC have not been established. The safety and effectiveness of Bylvay in pediatric patients less than 12 months of age with ALGS have not been established (1).

Related policies

Livmarli

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Bylvay may be considered **medically necessary** if the conditions indicated below are met.

Bylvay may be considered **investigational** for all other indications.

Prior-Approval Requirements

Diagnoses

Patient must have **ONE** of the following:

1. Pruritus associated with Progressive Familial Intrahepatic Cholestasis (PFIC)
 - a. 3 months of age or older
 - b. **NO** PFIC type 2 with ABCB11 variants causing non-functional or complete absence of bile salt export pump protein
2. Cholestatic pruritus associated with Alagille syndrome (ALGS)
 - a. 12 months of age or older

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- b. Diagnosis has been confirmed by **ONE** of the following:
 - i. Genetic testing (e.g., JAGGED1 mutation)
 - ii. Patient has bile duct paucity **AND** at least 3 major clinical features of ALGS (e.g., cholestasis, cardiac defect, skeletal abnormality, ophthalmic abnormality, or characteristic facial features)

AND ALL of the following:

- 1. **NO** cirrhosis, clinically significant portal hypertension, or hepatic decompensation
- 2. Inadequate treatment response, intolerance, or contraindication to **ONE** of the following:
 - a. Cholestyramine
 - b. Rifampin
 - c. Ursodeoxycholic acid (UDCA)
- 3. Patient has had baseline liver function tests (LFTs) and serum fat-soluble vitamin (FSV) levels performed
- 4. Prescriber agrees to monitor LFTs and serum FSV levels during treatment

Prior-Approval *Renewal* Requirements

Diagnoses

Patient must have **ONE** of the following:

- 1. Pruritus associated with Progressive Familial Intrahepatic Cholestasis (PFIC)
 - a. 3 months of age or older
- 2. Cholestatic pruritus associated with Alagille syndrome (ALGS)
 - a. 12 months of age or older

AND ALL of the following:

- 1. Improvement in pruritus symptoms, or observed improvement in scratching
- 2. **NO** cirrhosis, clinically significant portal hypertension, or hepatic decompensation
- 3. Prescriber agrees to monitor LFTs and serum FSV levels during treatment

Policy Guidelines

Pre-PA Allowance

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None

Prior – Approval Limits

Quantity 7,200 mcg (7.2 mg) per day

Duration 12 months

Prior–Approval *Renewal* Limits

Same as above

Rationale

Summary

Bylvay is an ileal bile acid transport (IBAT) inhibitor indicated for the treatment of pruritus associated with progressive familial intrahepatic cholestasis (PFIC) or cholestatic pruritus associated with Alagille syndrome (ALGS). Bylvay may not be effective in PFIC type 2 patients with ABCB11 variants resulting in absence or non-function of bile salt export pump protein. Current warnings include diarrhea, liver test abnormalities, and fat-soluble vitamin deficiency. Bylvay was not evaluated in patients with cirrhosis and treatment should be discontinued permanently if patient progresses to portal hypertension or has a hepatic decompensation event (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Bylvay while maintaining optimal therapeutic outcomes.

References

1. Bylvay [package insert]. Cambridge, MA: Ipsen Biopharmaceuticals, Inc.; February 2024.
2. National Organization for Rare Disorders (NORD). Alagille syndrome. Rare Disease Database. <https://rarediseases.org>. Published 2023.

Policy History

Date	Action
August 2021	Addition to PA

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September 2021	Annual review
December 2021	Annual review. Per FEP: added rifampicin to list of medication options, and added requirement that patient must not have cirrhosis, clinically significant portal hypertension, or hepatic decompensation for approval and renewal requirements
March 2022	Annual review
March 2023	Annual review and reference update. Changed policy number to 5.50.031
June 2023	Annual review and reference update
July 2023	Per PI update, added indication of cholestatic pruritus associated with Alagille syndrome (ALGS). Increased quantity limit to 7.2 mg per day
September 2023	Annual review
March 2024	Annual review and reference update
June 2024	Annual review and reference update
August 2024	Revised rifampicin to rifampin
December 2024	Annual review
March 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.