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# 5.50.031

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| **Section:** | Prescription Drugs | **Effective Date:** | July 1, 2024 |
| **Subsection:** | Gastrointestinal Agents | **Original Policy Date:** | August 13, 2021 |
| **Subject:** | Bylvay | **Page:** | 1 of 5 |

***Last Review Date:*** June 13, 2024

# 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 indication: 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).

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
    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. Rifampicin
    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

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 |
| September 2021 | Annual review |

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| 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 |

## Keywords

**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 13, 2024 and is effective on July 1, 2024.**