

Effective Date: 04/01/2022
Reviewed: 01/2022, 01/2023, 05/2023, 8/2023, 01/2024, 02/2025, 02/2026
Scope: Medicaid

BYLVAY (odevixibat)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met, and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

- A. Bylvay is indicated for the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC).

Limitations of Use: Bylvay may not be effective in PFIC type 2 patients with specific ABCB11 variants resulting in nonfunctional or complete absence of bile salt export pump protein (BSEP-3).

- B. Bylvay is indicated for the treatment of cholestatic pruritus in patients 12 months of age and older with Alagille syndrome (ALGS).

All other indications are considered experimental/investigational and not medically necessary.

II. EXCLUSIONS

Coverage will not be provided for members who have PFIC type 2 with variants in the ABCB11 gene that predict non-functional or complete absence of bile salt export pump protein (BSEP-3).

III. CRITERIA FOR INITIAL APPROVAL

Pruritus in progressive familial intrahepatic cholestasis (PFIC)

Authorization of 6 months may be granted for treatment of pruritus in progressive familial intrahepatic cholestasis (PFIC) when all of the following criteria are met:

- A. Member is 3 months of age or older
- B. This medication must be prescribed by or in consultation with a hepatologist or gastroenterologist or a physician that specializes in pruritus in progressive familial intrahepatic cholestasis (PFIC)
- C. Documentation that the member has moderate to severe pruritus and drug-induced pruritus has been ruled out
- D. Member has a confirmed molecular diagnosis of PFIC type (e.g., mutations in *ATP8B1*, *ABCB11*, *TJP2*, *MYO5B*, and *ABCB4*).
- E. Documentation that the member has serum bile acid level ≥ 100 $\mu\text{mol/L}$
- F. Documentation that the member does not have any other concomitant liver disease (e.g., cirrhosis, biliary atresia, benign recurrent intrahepatic cholestasis [BRIC], liver cancer, alternate non-PFIC related etiology of cholestasis) or history of a hepatic decompensation event (e.g., variceal hemorrhage, ascites, hepatic encephalopathy, portal hypertension)
- G. Documentation that the member has not received a liver transplant or surgical interruption of the enterohepatic circulation (e.g., partial external biliary diversion surgery)

- H. Documentation that the member experienced an inadequate treatment response or intolerance to at least two systemic medications for PFIC-related pruritus (e.g., ursodiol at a dose of 20-30 mg/kg/day, rifampin, cholestyramine)
- I. Documentation that the member's dose will not exceed 40 mcg/kg/day. Member's current weight and prescribed dose must be provided.

Cholestatic pruritis in Alagille syndrome (ALGS)

Authorization of 6 months may be granted for treatment of cholestatic pruritis in Alagille syndrome (ALGS) when all of the following criteria are met:

- A. Member is 12 months of age or older
- B. This medication must be prescribed by or in consultation with a hepatologist or gastroenterologist or a physician that specializes in Alagille Syndrome.
- C. Documentation that the member has moderate to severe pruritus and drug-induced pruritus has been ruled out
- D. Documentation that the member has a diagnosis of ALGS established by one of the following (see Appendix A for major clinical features of ALGS):
 - i. Genetic testing (i.e., mutations in the *JAG1* or *NOTCH2* gene)
 - ii. Family history of a ALGS and one or more major clinical features of ALGS
 - iii. Bile duct paucity and three or more major clinical features of ALGS
 - iv. Four or more major clinical features of ALGS
- E. Documentation that the member has evidence of cholestasis defined as the presence of one or more of the following:
 - i. Total serum bile acid greater than 3 times the upper limit of normal (ULN) for age
 - ii. Conjugated bilirubin greater than 1 mg/dL
 - iii. Fat soluble vitamin deficiency otherwise unexplainable
 - iv. Gamma-glutamyl transferase (GGT) greater than 3 times ULN for age
 - v. Intractable pruritis explainable only by liver disease
- F. Documentation that the member does not have any other concomitant liver disease (e.g., cirrhosis, liver cancer) or history of a hepatic decompensation event (e.g., variceal hemorrhage, ascites, hepatic encephalopathy, portal hypertension)
- G. Documentation that the member has not received a liver transplant or surgical interruption of the enterohepatic circulation (e.g., partial external biliary diversion surgery)
- H. Documentation that the member experienced an inadequate treatment response, intolerance or contraindication to at least two systemic medications for ALGS-related pruritus (e.g., ursodiol at a dose of 20-30 mg/kg/day, rifampin, cholestyramine, naltrexone)
- I. Documentation that the member experienced an inadequate treatment response, intolerance, or contraindication to Livmarli (maralixibat)
- J. Documentation that the member's dose will not exceed 120 mcg/kg/day. Member's current weight and prescribed dose must be provided

IV. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for all members (including new members) with documentation requesting continuation of therapy when the member meets all of the following:

- A. The member meets all initial criteria
- B. The member is experiencing benefit from therapy (e.g., improvement in pruritis and reduction in serum bile acid).
- C. Member's dose will not exceed 120 mcg/kg/day and if requesting dose increase for PFIC, documentation supports no improvement in pruritis after at least 3 months at each dose of 40 mcg/kg/day and 80 mcg/kg/day, if applicable.

V. QUANTITY LIMIT

- A. Bylvay oral pellets 200 mcg – 360 per 30 days, daily dose of 12
- B. Bylvay oral pellets 600 mcg – 120 per 30 days, daily dose of 4
- C. Bylvay capsules 400 mcg – 540 per 30 days, daily dose of 18
- D. Bylvay capsules 1200 mcg – 180 per 30 days, daily dose of 6

Indication	Dosing Regimen	Maximum Dose																		
PFIC	<p>The recommended dosage of Bylvay is 40 mcg/kg once daily in the morning with a meal. If there is no improvement in pruritus after 3 months, the dosage may be increased in 40 mcg/kg increments up to 120 mcg/kg once daily, not to exceed a total daily dose of 6 mg.</p> <p>Bylvay oral pellets are intended for use by patients weighing < 19.5 kg, while Bylvay capsules are intended for use by patients weighing ≥ 19.5 kg. The table below shows the recommended weight-based total daily dosage needed for the recommended dosage at 40 mcg/kg once daily.</p> <table border="1" data-bbox="477 884 1159 1192"> <thead> <tr> <th>Body Weight (kg)</th> <th>Total Daily Dose (mcg)</th> </tr> </thead> <tbody> <tr> <td>≤ 7.4</td> <td>200</td> </tr> <tr> <td>7.5 – 12.4</td> <td>400</td> </tr> <tr> <td>12.5 – 17.4</td> <td>600</td> </tr> <tr> <td>17.5 – 25.4</td> <td>800</td> </tr> <tr> <td>25.5 – 35.4</td> <td>1200</td> </tr> <tr> <td>35.5 – 45.4</td> <td>1600</td> </tr> <tr> <td>45.5 – 55.4</td> <td>2000</td> </tr> <tr> <td>≥55.5</td> <td>2400</td> </tr> </tbody> </table>	Body Weight (kg)	Total Daily Dose (mcg)	≤ 7.4	200	7.5 – 12.4	400	12.5 – 17.4	600	17.5 – 25.4	800	25.5 – 35.4	1200	35.5 – 45.4	1600	45.5 – 55.4	2000	≥55.5	2400	6 mg/day
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ALGS	<p>The recommended dosage of Bylvay is 120 mcg/kg once daily in the morning with a meal.</p> <p>The table below shows the recommended weight-based total daily dosage needed for the recommended dosage at 120 mcg/kg once daily.</p> <table border="1" data-bbox="355 1451 1281 1759"> <thead> <tr> <th>Body Weight (kg)</th> <th>Total Daily Dose (mcg)</th> </tr> </thead> <tbody> <tr> <td>≤ 7.4</td> <td>600</td> </tr> <tr> <td>7.5 – 12.4</td> <td>1200</td> </tr> <tr> <td>12.5 – 17.4</td> <td>1800</td> </tr> <tr> <td>17.5 – 25.4</td> <td>2400</td> </tr> <tr> <td>25.5 – 35.4</td> <td>3600</td> </tr> <tr> <td>35.5 – 45.4</td> <td>4800</td> </tr> <tr> <td>45.5 – 55.4</td> <td>6000</td> </tr> <tr> <td>≥55.5</td> <td>7200</td> </tr> </tbody> </table>	Body Weight (kg)	Total Daily Dose (mcg)	≤ 7.4	600	7.5 – 12.4	1200	12.5 – 17.4	1800	17.5 – 25.4	2400	25.5 – 35.4	3600	35.5 – 45.4	4800	45.5 – 55.4	6000	≥55.5	7200	120mcg/kg/day
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VI. APPENDIX A

Major Clinical Features of ALGS

- Hepatic abnormality (e.g., cholestasis)
- Cardiac abnormality (e.g., stenosis of the peripheral pulmonary artery and its branches)
- Skeletal abnormality (e.g., butterfly vertebrae)
- Ophthalmologic abnormality (e.g., posterior embryotoxon)
- Characteristic facial features (e.g., triangular-shaped face with a broad forehead and a pointed chin, bulbous tip of the nose, deeply set eyes, and hypertelorism)
- Vascular abnormalities (e.g., intracranial bleeds, systemic vascular anomalies)
- Renal structural or functional abnormality (e.g., abnormally small size, cysts)

VII. REFERENCES

1. Blyvay [package insert]. Boston, MA: Albireo Pharma, Inc.; March 2025. Accessed February 2026.
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5. The Childhood Liver Disease Research Network. Alagille syndrome. <https://childrennetwork.org/For-Physicians/Alagille-Syndrome-Information-for-Physicians>. Accessed August 27, 2004..