For pruritus in your patients with progressive familial intrahepatic cholestasis (PFIC)

Grow up with BYLVAY®*

with flexible administration options[1]

RAPID REDUCTION IN PRURITUS AS EARLY AS WEEKS 1-4[1,2]

PEDFIC 1 Key Efficacy Endpoint:
Mean percentage of pruritus assessments scored as little to no scratching (BYLVAY 40 mcg/kg/day 35%, BYLVAY 120 mcg/kg/day 30% vs placebo 13%)[1]

PEDFIC 1 Primary Endpoint:
Proportion of positive pruritus assessments over 24 weeks (All BYLVAY 55% vs placebo 30%)[3]

The mean of patients' worst weekly average scratching scores in each treatment group for each month, where the weekly average utilized the worst score from each day (morning or evening score) was assessed in PEDFIC 1.[1]

*BYLVAY can be taken 3 ways: swallow capsules whole; open capsules or oral pellets and mix contents with soft food or liquid.[1]

All images are actor portrayals.

INDICATION

BYLVAY (odevixibat) is an ileal bile acid transporter (IBAT) inhibitor indicated for the treatment of pruritus in patients ≥3 months of age with progressive familial intrahepatic cholestasis (PFIC).

Limitation of Use:

BYLVAY is not recommended in a subgroup of PFIC type 2 patients with specific *ABCB11* variants resulting in non-functional or complete absence of the bile salt export pump protein.

IMPORTANT SAFETY INFORMATION

Contraindications

IBAT inhibitors, including BYLVAY, are contraindicated in patients with prior or active hepatic decompensation events (e.g., variceal hemorrhage, ascites, hepatic encephalopathy).

Please see additional Important Safety Information throughout and the full Prescribing Information.

Bylvay® (odevixibat)
200 | 400 | 600 | 1200 mcg capsules

Approved to treat pruritus in all PFIC types, including newly detected subtypes[1]

The genetic understanding of PFIC is rapidly evolving[4]

- PFIC refers to a group of disorders of bile acid secretion and transport with a genetic cause[4]
- Phenotypes are highly variable, but all PFIC types are characterized by impaired bile flow, causing cholestasis[4,5]
- In many patients, the most impactful symptom of cholestasis is cholestatic pruritus[4,5]

PFIC type[1,4]	Affected gene[1,4]
PFIC1*	*ATP8B1*
PFIC2*	*ABCB11*
PFIC3*	*ABCB4*
PFIC4*	*TJP2*
PFIC5	*NR1H4*
PFIC6	*SLC51A*
PFIC7	*USP53*
PFIC8	*KIF12*
PFIC9	*ZFYVE19*
PFIC10*	*MYO5B*
PFIC11	*SEMA7A*
PFIC12	*VPS33B*
PFIC13	*PSKH1*

Genetic testing in PFIC

- PFIC can be diagnosed clinically; this may involve physical exam, lab, imaging, and histological assessments, plus a review of medical and family history[4,6]
- Genetic testing can help support a clinical diagnosis and may be confirmatory; however, it is often inconclusive[6-8]
- Recent expert opinion recommends IBATi treatment initiation concurrently with genetic testing for patients with a clinical suspicion of PFIC experiencing pruritus[6]
- Potential variants in other genetic loci have also been identified[4,6]

Limitation of Use: BYLVAY is not recommended in a subgroup of PFIC type 2 patients with specific *ABCB11* variants resulting in non-functional or complete absence of the bile salt export pump protein.[1]

*PFIC types studied in the PEDFIC clinical trial program.[1]
IBATi=ileal bile acid transporter inhibitor.

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Hepatotoxicity

BYLVAY treatment is associated with a potential for drug-induced liver injury (DILI). In the PFIC trials, treatment-emergent elevations or worsening of liver tests occurred. Of the three PFIC patients who experienced DILI, two underwent liver transplant.

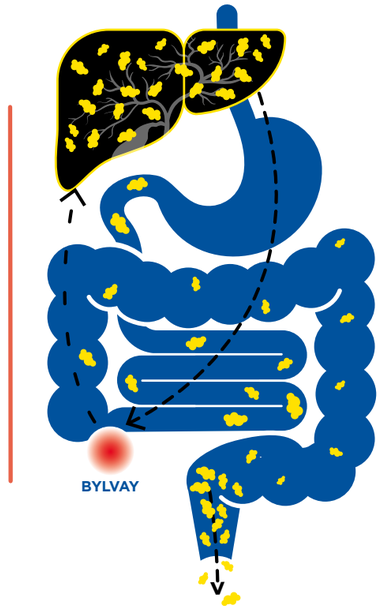
BYLVAY decreases the reabsorption of bile acids[1]

PFIC is characterized by cholestasis[4]

- Disruption to normal bile flow leads to bile acid accumulation in the liver[4,5,9]
- Excess bile acid "spills over" into the bloodstream, elevating serum bile acid (sBA) levels.[5,9] Elevated sBA may play a role in pruritus[10,11]
- The ileal bile acid transporter (IBAT) helps regulate the bile acid (BA) pool, reabsorbing up to 95% of secreted BA[5]

BYLVAY reversibly inhibits the ileal bile acid transporter (IBAT)[1,5]

1. **Reversibly inhibits the IBAT, decreasing bile acid reabsorption in the terminal ileum**
2. **Decreasing reabsorption in the terminal ileum increases excretion**
3. **Decreased reabsorption is observed as a decrease in serum bile acid levels**
4. **The mechanism by which BYLVAY improves pruritus is not fully understood**



BYLVAY was minimally absorbed and no accumulation was observed following once-daily dosing[1]

Please see additional Important Safety Information throughout and the full Prescribing Information.

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PEDFIC 1 is the FIRST COMPLETED GLOBAL Phase 3 clinical trial in PFIC[3,12]

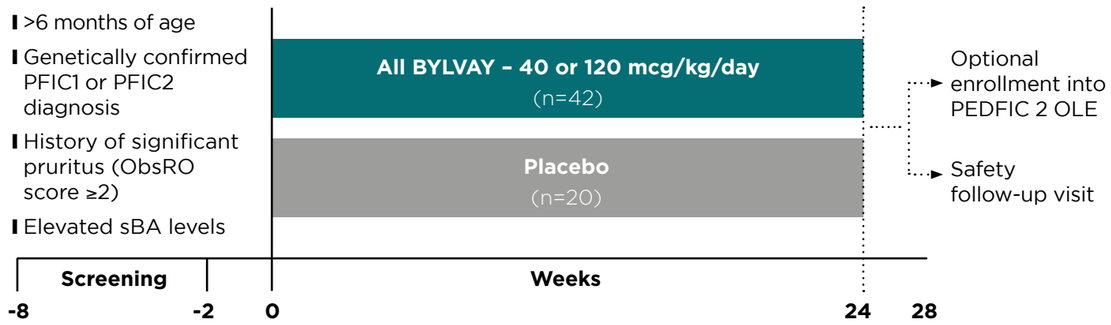
PEDFIC 1[3]

24-week randomized, double-blind Phase 3 study

All BYLVAY doses were administered once daily

Enrolled participants[3]:

- >6 months of age
- Genetically confirmed PFIC1 or PFIC2 diagnosis
- History of significant pruritus (ObsRO score ≥2)
- Elevated sBA levels



Primary Endpoint[3]:

- Proportion of participant's positive pruritus assessments over 24 weeks*

Secondary Endpoints included[3]:

- Proportion of participants with sBA response at Week 24†
- Change in sleep parameters through Week 24‡

Concomitant ursodeoxycholic acid (UDCA) and other antipruritic medications were permitted provided they were at a stable dose at least 4 weeks before enrollment with no dosage changes planned during the study period.[3]

89% of participants were receiving UDCA or rifampicin at PEDFIC 1 baseline.[13]

*Defined as a scratching score of ≤1 or at least a 1-point reduction from baseline on the PRUCISION™ ObsRO instrument.[3]

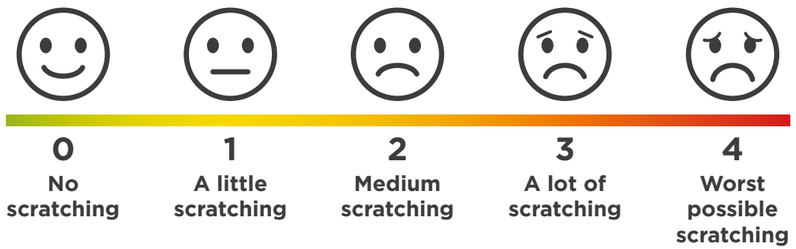
†sBA response was defined as at least a 70% decrease in sBA or a final sBA level of ≤70 µmol/L.[3]

‡Data reported using the PRUCISION™ ObsRO instrument; caregivers answered up to 9 questions about the effects that itch had on the participant's sleep and sleep-related parameters daily.[13]

IBATi=ileal bile acid transporter inhibitor; ObsRO=observer-reported outcomes; OLE=open-label extension.

Cholestatic pruritus was measured using the PRUCISION™ ObsRO scale[3]

PRUCISION™ ObsRO Scale[14]



A **validated** instrument that defines a ≥1-point reduction as a clinically meaningful change in pruritus score.[3,14]

Caregivers recorded scratching scores twice daily based on the following questions[14]:

- AM score: How bad was your child's worst scratching since he/she went to bed last night?
- PM score: How bad was your child's worst scratching since he/she woke up this morning?

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Hepatotoxicity (cont'd)

Obtain baseline liver tests because some PFIC patients have abnormal liver tests at baseline and monitor patients frequently for the first 6 to 8 months, and as clinically needed thereafter, for elevations in liver tests, for the development of liver-related adverse reactions, and for physical signs of hepatic decompensation.

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PEDFIC 1

Rapid and durable reduction in cholestatic pruritus as early as Weeks 1-4[1-3]

RAPID ITCH REDUCTION

Pruritus reduction data at Weeks 1-4[16]

Little to no scratching achieved with BYLVAY[1]

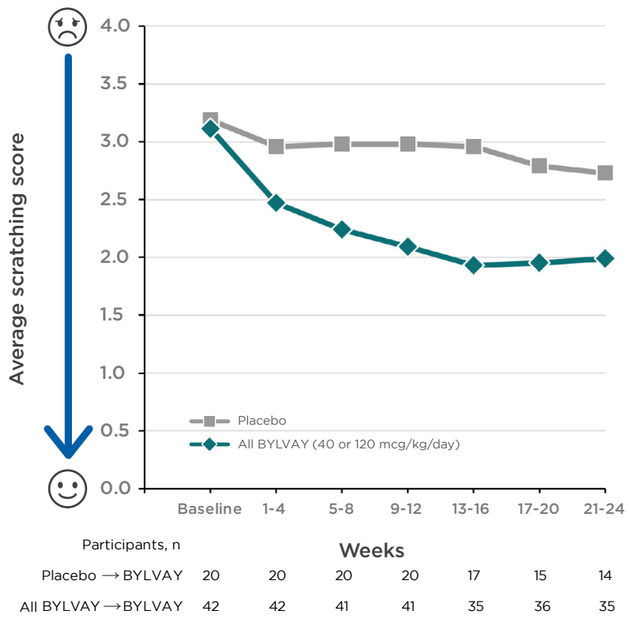
Key Efficacy Endpoint: Proportion of pruritus assessments achieving little or no scratching (score ≤1) through Week 24[1]*

35% on BYLVAY 40 mcg/kg/day (n=23)[1]

30% on BYLVAY 120 mcg/kg/day (n=19)[1]

vs **13%** with placebo (n=20)[1]

Worst average scratching score through Week 24[1,3]



Participants, n							
Placebo → BYLVAY	20	20	20	20	17	15	14
All BYLVAY → BYLVAY	42	42	41	41	35	36	35

47% **of participants** treated with BYLVAY experienced a clinically meaningful improvement† through Week 24[15]

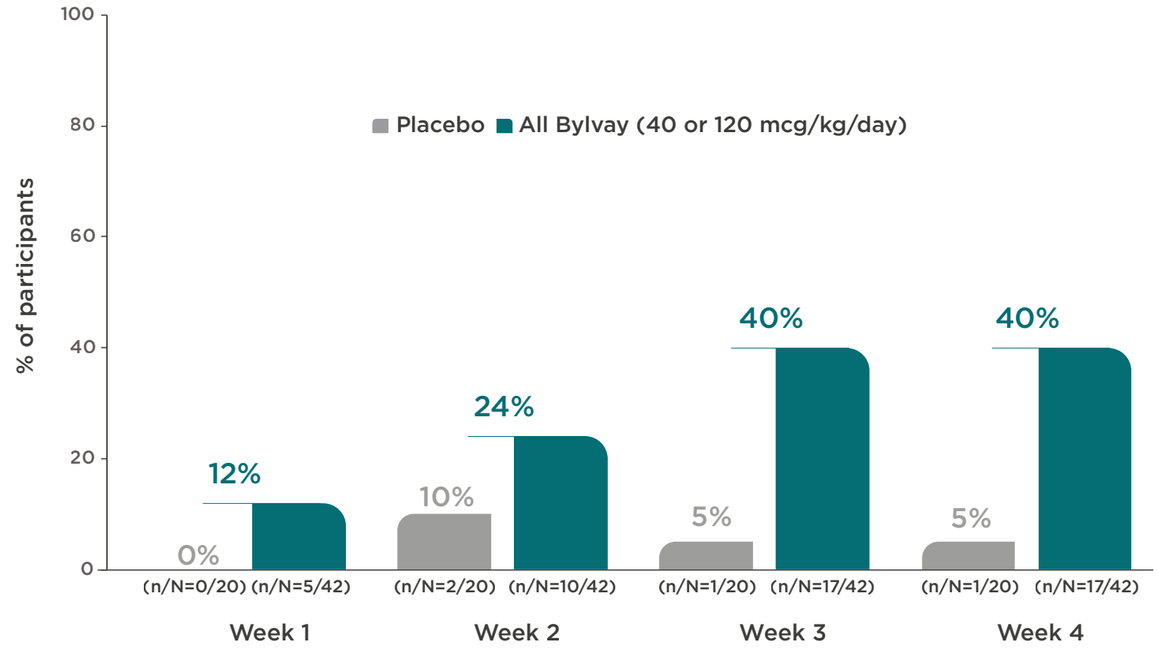
*Mean difference vs placebo (95% CI): BYLVAY 40 mcg/kg/day: 22.2 (4.7, 39.6); BYLVAY 120 mcg/kg/day: 16.9 (-2.0, 35.7). N=62.[1]

†Defined as a ≥1-point improvement in ObsRO score.[3]

ObsRO=observer-reported outcomes; OLE=open-label extension; sBA=serum bile acid.

Please see additional Important Safety Information throughout and the full Prescribing Information.

Proportion of participants experiencing a clinically meaningful* reduction in average scratching score at Weeks 1 to 4[16]



LIMITATION: Data are descriptive and not powered to determine statistical significance; interpret with caution.

*Defined as a ≥1-point reduction in ObsRO score.[3]

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Hepatotoxicity (cont'd)

If liver test abnormalities or signs of clinical hepatitis occur in the absence of other causes, consider dose reduction or treatment interruption. Permanently discontinue BYLVAY if a patient experiences the following: persistent or recurrent liver test abnormalities, or upon rechallenge, signs and symptoms consistent with clinical hepatitis, or a hepatic decompensation event.

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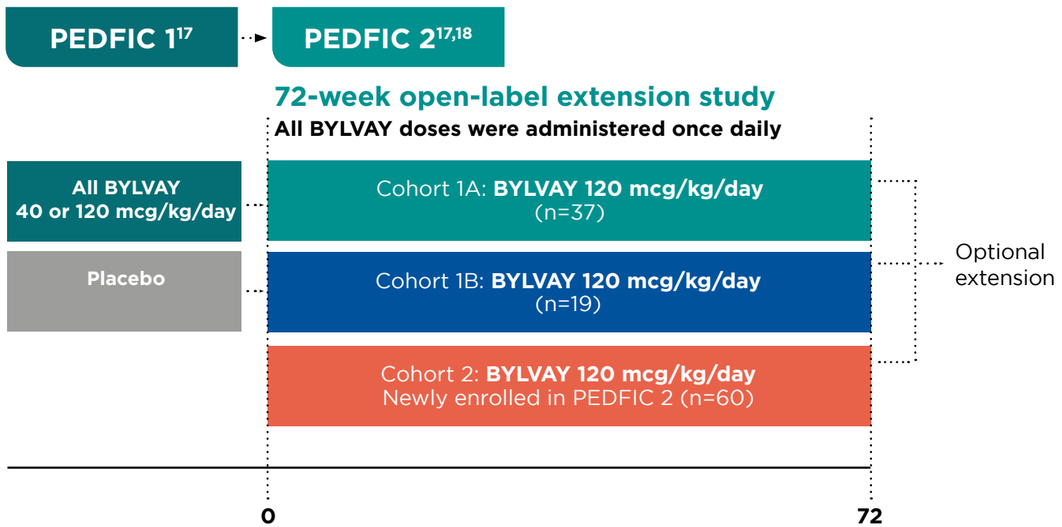
PEDFIC 2 is an open-label extension study to assess long-term efficacy and safety[17]



Eligibility criteria[17]:

- Any age
- Any PFIC type
- History of significant pruritus
- Elevated sBA levels

Primary Endpoint:

- Proportion of participant's positive pruritus assessments over 72 weeks[17]*

Secondary Endpoints included:

- Change from baseline in sBA at Week 72[17]

Exploratory Endpoint:

- Change in sleep parameters through Week 72[17]†

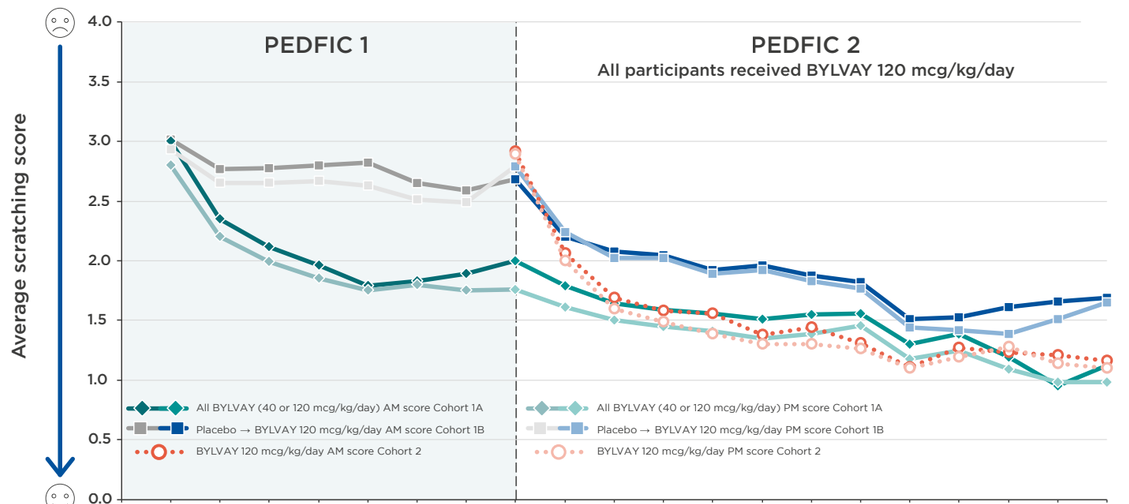
*Defined as a scratching score of ≤1 or at least a 1-point reduction from baseline on the PRUCISION™ ObsRO instrument.[17]

†Data reported using the PRUCISION™ ObsRO instrument; caregivers answered up to 9 questions about the effects that itch had on the participant's sleep and sleep-related parameters daily.[18]

PEDFIC 1 / PEDFIC 2

Impact of once-daily BYLVAY on pruritus throughout the day and night[19]

Average day and night scratching scores through Week 96[19]*



Participants, n (AM / PM)																				
Placebo → BYLVAY	20/20	20/20	20/20	20/20	17/17	15/15	14/14	19/19	19/19	19/19	19/19	18/18	18/18	17/17	16/16	14/14	14/14	13/13	13/13	12/12
All BYLVAY → BYLVAY	42/42	42/42	41/41	41/41	35/35	36/36	35/35	36/37	37/37	37/37	37/37	37/37	36/37	36/36	30/31	28/28	29/29	31/31	22/21	28/26
BYLVAY Cohort 2	—	—	—	—	—	—	—	53/53	53/53	51/51	49/50	48/50	47/48	45/46	43/43	36/37	41/41	33/33	28/28	30/32

LIMITATION: Data are descriptive and not powered to determine statistical significance; interpret with caution.

Reductions were observed through 96 weeks[19]

PEDFIC 2 is an open-label extension of the PEDFIC 1 trial.[17]

*AM score reflects nighttime scratching; PM score reflects daytime scratching.[20]

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Hepatotoxicity (cont'd)

The safety and effectiveness of BYLVAY have not been established in patients with decompensated cirrhosis. Monitor patients with compensated cirrhosis or portal hypertension more frequently and discontinue if hepatic decompensation occurs. IBAT inhibitors, including BYLVAY, are contraindicated in patients with prior or active hepatic decompensation events.

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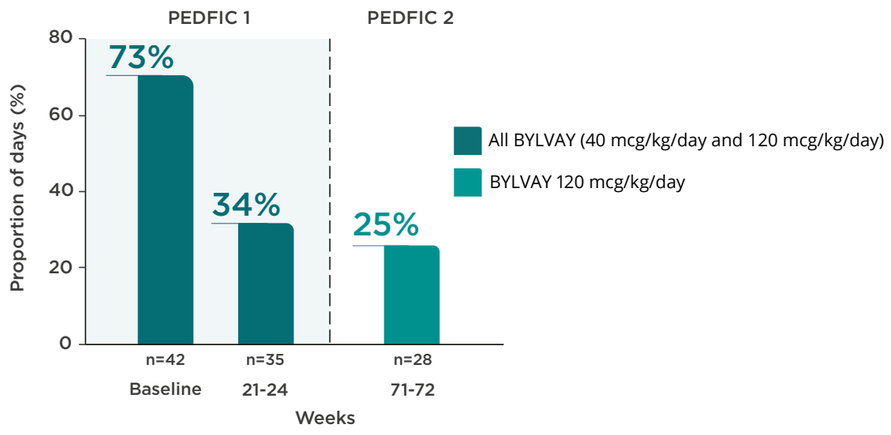
PEDFIC 1 / PEDFIC 2

Long-term BYLVAY data on itch-related sleep disruptions[21]

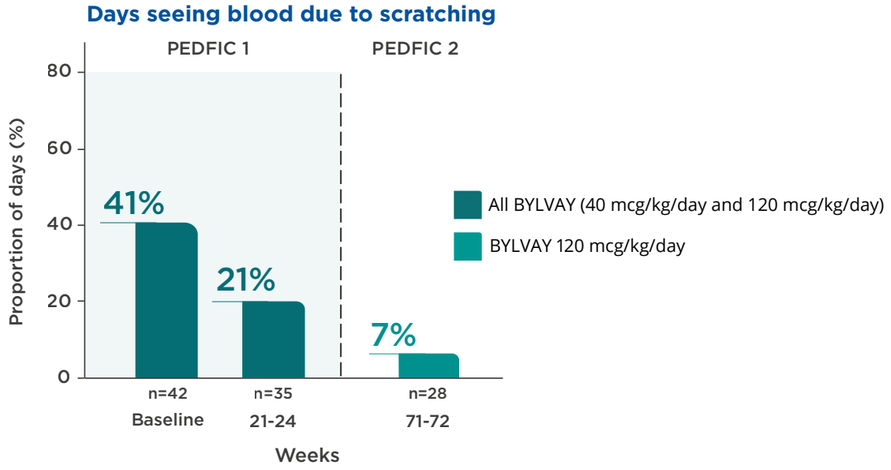
Data on reduction in pruritus-associated sleep disruption at 96 weeks

24 weeks in PEDFIC 1 (n=42) and 72 weeks in PEDFIC 2 (n=37)[21]

Days needing to sleep with a caregiver



Days seeing blood due to scratching



LIMITATION: Data are descriptive and not powered to determine statistical significance; interpret with caution.

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Diarrhea

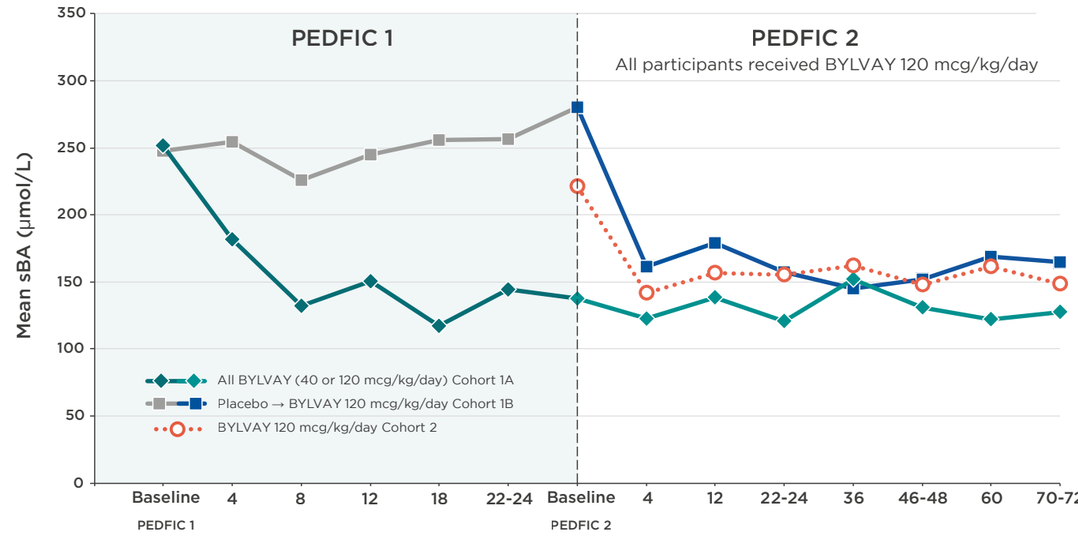
In the PFIC clinical trials, diarrhea was reported more frequently in BYLVAY-treated patients compared to placebo. In the PFIC clinical trials, treatment interruption due to diarrhea occurred in 2 patients with 3 events. Treatment interruption due to diarrhea ranged between 3 to 7 days. One patient withdrew from the trial.

PEDFIC 1 / PEDFIC 2

Data on reduction in serum bile acids through 96 weeks[17,22]

RAPID sBA REDUCTION

Mean sBA levels through Week 96[17,22]



Participants, n														
Placebo → BYLVAY	20	20	18	17	16	15	19	17	18	17	17	15	13	15
All BYLVAY → BYLVAY	42	40	37	36	26	34	37	34	35	32	29	29	27	28
BYLVAY Cohort 2	—	—	—	—	—	—	60	53	54	55	49	49	47	43

LIMITATIONS: The clinical trial was not designed to make conclusions regarding the clinical benefit of sBA reduction. The correlation between sBA and clinical outcomes is not known. sBA levels do not accurately predict bile acids in the liver. It is unknown whether the sBA assay was qualified to assess sBA levels during the analysis.

Reductions in sBA through **Week 96[22]**

Please see additional Important Safety Information throughout and the full Prescribing Information.

Bylvay® (odevixibat)
200 | 400 | 600 | 1200 mcg capsules

Through Week 24

Safety profile established in PEDFIC 1[3]

PEDFIC 1: Serious TEAEs[3]

Placebo (n=20) n (%)	BYLVAY 40 mcg/kg/day (n=23) n (%)	BYLVAY 120 mcg/kg/day (n=19) n (%)
5 (25)	0	3 (16)

- All SAEs were unrelated to treatment, and none led to discontinuation[23]
  - Treatment-emergent SAEs in the BYLVAY 120 mcg/kg/day group included supraventricular tachycardia, urinary tract infection, dehydration, and elevated liver function tests[23]
- No deaths were reported[3]

PEDFIC 1: Common Adverse Reactions (≥2%)[1]

Adverse reaction	Placebo (n=20) n (%)	BYLVAY 40 mcg/kg/day (n=23) n (%)	BYLVAY 120 mcg/kg/day (n=19) n (%)
Diarrhea	2 (10)	9 (39)	4 (21)
Transaminases increased (ALT, AST)	1 (5)	3 (13)	4 (21)
Vomiting	0	4 (17)	3 (16)
Abdominal pain	0	3 (13)	3 (16)
Blood bilirubin increased	2 (10)	3 (13)	2 (11)
Fat-soluble vitamin deficiency (A, D, E)	1 (5)	0	3 (16)
Splenomegaly	0	0	2 (11)
Cholelithiasis	0	0	1 (5)
Dehydration	0	0	1 (5)
Fracture	0	1 (4)	0

- Most TEAEs were mild to moderate in severity[3]
- Most common adverse reactions (≥2%) were liver test abnormalities, diarrhea, abdominal pain, vomiting, and fat-soluble vitamin deficiency[1]
- 3 participants in the BYLVAY 120 mcg/kg/day group discontinued, with 1 due to TEAEs[3]

Through Week 72

Long-term safety profile established in PEDFIC 2[24]

Rates of SAEs[24]

Participants with any	Cohort 1A (n=37) n (%)	Cohort 1B (n=19) n (%)	Cohort 2 (n=60) n (%)
Treatment-emergent SAEs	7 (19)	5 (26)	23 (38)
Drug-related treatment-emergent SAEs	1 (3)	0	1 (2)

Common TEAEs (≥10%)[24]

Adverse reaction	Cohort 1A (n=37) n (%)	Cohort 1B (n=19) n (%)	Cohort 2 (n=60) n (%)
Cough	13 (35)	4 (21)	10 (17)
Pyrexia	12 (32)	7 (37)	16 (27)
Blood bilirubin increased	10 (27)	4 (21)	14 (23)
URTI	10 (27)	7 (37)	12 (20)
Diarrhea	9 (24)	2 (11)	17 (28)
Nasopharyngitis	9 (24)	3 (16)	9 (15)
Vomiting	2 (5)	2 (11)	10 (17)
Influenza	7 (19)	0	8 (13)
Pruritus	7 (19)	3 (16)	7 (12)
Gastroenteritis	6 (16)	1 (5)	6 (10)
INR increased	5 (14)	3 (16)	11 (18)
Hepatomegaly	4 (11)	1 (5)	1 (2)
Viral infection	4 (11)	1 (5)	7 (12)
ALT increased	3 (8)	2 (11)	6 (10)
Constipation	3 (8)	2 (11)	4 (7)
COVID-19	3 (8)	3 (16)	16 (27)
Epistaxis	3 (8)	1 (5)	6 (10)
Otitis media	3 (8)	2 (11)	2 (3)
Splenomegaly	3 (8)	2 (11)	4 (7)
Tonsilitis	3 (8)	3 (16)	1 (2)
Ear infection	2 (5)	2 (11)	3 (5)
Varicella	2 (5)	3 (16)	3 (5)
Vitamin D deficiency	2 (5)	1 (5)	8 (13)

- 35 (30%) of 116 participants experienced treatment-emergent SAEs; the majority of SAEs were assessed as unrelated to treatment with BYLVAY[24]
- The most commonly reported types of SAEs overall were infections and infestations, gastrointestinal disorders, and hepatobiliary disorders. 5 participants discontinued treatment due to SAEs[24]
- No deaths were reported[24]
- The most common reason for treatment interruption was blood bilirubin increase[24]
- Overall, 10 (9%) participants reported TEAEs that led to treatment discontinuation[24]

Additional TEAEs reported in ≥5% of participants in any cohort included abdominal pain, jaundice, rhinorrhea, AST increased, and anemia.[24]

PEDFIC 2 is an open-label extension of the PEDFIC 1 trial.[17]

ALT=alanine aminotransferase; AST=aspartate aminotransferase; INR=international normalized ratio; SAE=serious adverse event; TEAE=treatment-emergent adverse event; URTI=upper respiratory tract infection.

Please see additional Important Safety Information throughout and the full Prescribing Information.

BYLVAY offers once-daily dosing and flexible administration[1]

The **ONLY IBAT inhibitor** that:

- ✔ **OFFERS** once-daily dosing[25]
- ✔ **DOES NOT** require fasting or delaying of meals[25]
- ✔ **DOES NOT** require dose titration for standard treatment initiation[25]

1 **daily dose** taken with a morning meal[1]

2 **oral formulation options:** oral pellets, capsules[1]

3 **ways to take:** swallow capsules whole; mix contents* with soft food or liquid[1]

*Pellets or capsules.

Oral Pellets[1]

For patients weighing <19.5 kg

Capsules[1]

For patients weighing ≥19.5 kg

Do not crush or chew capsules

After mixing BYLVAY with food or liquids, discard the empty shells.[1]

†Add 1 teaspoon (5 mL of liquid) of an age-appropriate liquid (for example, breast milk, infant formula, or water).

Dosing for pruritus in PFIC[1]

- Recommended starting dose: **40 mcg/kg/day**
- 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
- Dose should not exceed 6 mg/day

IMPORTANT SAFETY INFORMATION (CONT'D)

WARNINGS AND PRECAUTIONS

Diarrhea (cont'd)

If diarrhea occurs, monitor for dehydration and treat promptly. Interrupt dosing if a patient experiences persistent diarrhea. Restart BYLVAY at 40 mcg/kg/day when diarrhea resolves and increase the dose as tolerated if appropriate. If diarrhea persists and no alternate etiology is identified, stop treatment.

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Indication and Important Safety Information

INDICATION

BYLVAY is an ileal bile acid transporter (IBAT) inhibitor indicated for the treatment of pruritus in patients ≥3 months of age with progressive familial intrahepatic cholestasis (PFIC).

Limitation of Use:

BYLVAY is not recommended in a subgroup of PFIC type 2 patients with specific *ABCB11* variants resulting in non-functional or complete absence of the bile salt export pump protein.

IMPORTANT SAFETY INFORMATION

Contraindications

IBAT inhibitors, including BYLVAY, are contraindicated in patients with prior or active hepatic decompensation events (e.g., variceal hemorrhage, ascites, hepatic encephalopathy).

WARNINGS AND PRECAUTIONS

Hepatotoxicity

BYLVAY treatment is associated with a potential for drug-induced liver injury (DILI). In the PFIC trials, treatment-emergent elevations or worsening of liver tests occurred. Of the three PFIC patients who experienced DILI, two underwent liver transplant.

Obtain baseline liver tests because some PFIC patients have abnormal liver tests at baseline and monitor patients frequently for the first 6 to 8 months, and as clinically needed thereafter, for elevations in liver tests, for the development of liver-related adverse reactions, and for physical signs of hepatic decompensation. If liver test abnormalities or signs of clinical hepatitis occur in the absence of other causes, consider dose reduction or treatment interruption. Permanently discontinue BYLVAY if a patient experiences the following: persistent or recurrent liver test abnormalities, or upon rechallenge, signs and symptoms consistent with clinical hepatitis, or a hepatic decompensation event.

The safety and effectiveness of BYLVAY have not been established in patients with decompensated cirrhosis. Monitor patients with compensated cirrhosis or portal hypertension more frequently and discontinue if hepatic decompensation occurs. IBAT inhibitors, including BYLVAY, are contraindicated in patients with prior or active hepatic decompensation events.

Diarrhea

In the PFIC clinical trials, diarrhea was reported more frequently in BYLVAY-treated patients compared to placebo. In the PFIC clinical trials, treatment interruption due to diarrhea occurred in 2 patients with 3 events. Treatment interruption due to diarrhea ranged between 3 to 7 days. One patient withdrew from the trial.

If diarrhea occurs, monitor for dehydration and treat promptly. Interrupt dosing if a patient experiences persistent diarrhea. Restart BYLVAY at 40 mcg/kg/day when diarrhea resolves and increase the dose as tolerated if appropriate. If diarrhea persists and no alternate etiology is identified, stop treatment.

Fat-Soluble Vitamin (FSV) Deficiency

Fat-soluble vitamins (FSV) include vitamin A, D, E, and K. PFIC patients can have FSV deficiency at baseline. BYLVAY may adversely affect absorption of FSVs. In clinical trials, new onset or worsening of existing FSV deficiency was reported more frequently in BYLVAY-treated patients compared to placebo.

Obtain baseline INR (International Normalized Ratio) and FSV levels and monitor during treatment along with any clinical manifestations. If FSV deficiency is diagnosed, supplement with FSV. Discontinue BYLVAY if FSV deficiency persists, worsens, or complications occur despite adequate FSV supplementation.

If bone fracture occurs, consider interrupting BYLVAY treatment and supplement with FSV if indicated.

If bleeding occurs, interrupt treatment with BYLVAY. Optimize treatment of FSV deficiency and consider restarting BYLVAY once the patient is clinically stable.

Adverse Reactions

The most common adverse reactions for BYLVAY in patients with PFIC are diarrhea, liver test abnormalities, vomiting, abdominal pain, and FSV deficiency.

Drug Interactions

For patients taking bile acid binding resins, take BYLVAY at least 4 hours before or 4 hours after taking a bile acid binding resin.

Use in Specific Populations

Limited human data on BYLVAY use in pregnant persons are insufficient to establish a drug-associated risk of major birth defects, miscarriage, or adverse developmental outcomes. Based on findings from animal reproduction studies, BYLVAY may cause cardiac malformations when a fetus is exposed during pregnancy. As BYLVAY may inhibit the absorption of fat-soluble vitamins, which are essential for normal fetal growth and development, monitor pregnant patients for FSV deficiency and increase supplementation as needed. Consider the woman's need for BYLVAY, the potential drug-related risks to the fetus, and the potential adverse outcomes from untreated maternal PFIC.

There is a pregnancy exposure registry that monitors pregnancy outcomes in persons exposed to BYLVAY during pregnancy. Pregnant women exposed to BYLVAY, or their healthcare providers, should report BYLVAY exposure by calling 1-855-463-5127.

To report SUSPECTED ADVERSE REACTIONS, contact Ipsen Biopharmaceuticals, Inc. at 1-855-463-5127 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see the full Prescribing Information.

REFERENCES

1. BYLVAY. Prescribing Information. Ipsen Biopharmaceuticals, Inc.; 2025. **2.** Thompson RJ, Amell H, Artan R, et al. Odevixibat treatment in progressive familial intrahepatic cholestasis: a randomised, placebo-controlled, phase 3 trial (suppl). *Lancet Gastroenterol Hepatol.* 2022;7(9):830-842. doi:10.1016/S2468-1253(22)00093-0 **3.** Thompson RJ, Amell H, Artan R, et al. Odevixibat treatment in progressive familial intrahepatic cholestasis: a randomised, placebo-controlled, phase 3 trial. *Lancet Gastroenterol Hepatol.* 2022;7(9):830-842. doi:10.1016/S2468-1253(22)00093-0 **4.** Vitale G, Sciveres M, Mandato C, Pio d'Adamo A, Di Giorgio A. Genotypes and different clinical variants between children and adults in progressive familial intrahepatic cholestasis: a state-of-the-art review. *Orphanet J Rare Dis.* 2025;20(1):80. doi:10.1186/s13023-025-03599-2 **5.** 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 **6.** McKiernan P, Bernabeu JQ, Girard M, et al. Opinion paper on the diagnosis and treatment of progressive familial intrahepatic cholestasis. *JHEP Rep.* 2023;6(1):100949. doi:10.1016/j.jhepr.2023.100949 Erratum in: *JHEP Rep.* 2024;6(6):101058. doi:10.1016/j.jhepr.2o24.101058 **7.** Nayagam JS, Foskett P, Stautnieks S, et al. Clinical phenotypes of adult-onset liver disease in patients with variants in ABCB4, ABCB11, and ATP8B1. *Hepatol Commun.* 2022;6(10):2654-2664. doi:10.1002/hep4.2051 **8.** Bakir A, Topçu V, Çavdarli B. The molecular landscape of progressive familial intrahepatic cholestasis in Turkey: Defining the molecular profiles and expanding the variant spectrum. *Ann Hum Genet.* 2022:86(3):119-126. doi:10.1111/ahg.12456 **9.** Gunaydin M, Bozkurter Cil AT. Progressive familial intrahepatic cholestasis: diagnosis, management, and treatment. *Hepat Med.* 2018;10:95-104. doi:10.2147/HMER.S137209 **10.** Cholestasis. Cleveland Clinic. Updated December 19, 2022. Accessed January 15, 2026. https://my.clevelandclinic.org/health/diseases/24554-cholestasis **11.** Ibrahim SH, Kamath BM, Loomes KM, Karpen SJ. Cholestatic liver diseases of genetic etiology: advances and controversies. *Hepatology.* 2022;75(6):1627-1646. doi:10.1002/hep.32437 **12.** Data on file. Ipsen US. [NON-US-004498]. **13.** Data on file. Ipsen US. [NON-US-004379]. **14.** Gwaltney C, Ivanescu C, Karlsson L, Warholic N, Kjems L, Horn P. Validation of the PRUCISION instruments in pediatric patients with progressive familial intrahepatic cholestasis. *Adv Ther.* 2022;39(11):5105-5125. doi:10.1007/s12325-022-02262-7 **15.** Data on file. Ipsen US. [BYL-US-002794]. **16.** Data on file. Ipsen US. [NON-US-004201]. **17.** Thompson RJ, Artan R, Baumann U, et al. Interim results from an ongoing, open-label, single-arm trial of odevixibat in progressive familial intrahepatic cholestasis. *JHEP Rep.* 2023;5(8):100782. doi:10.1016/j.jhepr.2023.10078 **18.** Data on file. Ipsen US. [NON-US-004380]. **19.** Data on file. Ipsen US. [NON-US-004202]. **20.** Data on file. Ipsen US. [NON-US-004421]. **21.** Data on file. Ipsen US. [NON-US-004424]. **22.** Data on file. Ipsen US. [NON-US-004203]. **23.** Data on file. Ipsen US. [NON-US-004422]. **24.** Data on file. Ipsen US. [NON-US-004423]. **25.** Data on file. Ipsen US. [NON-US-004496].

Bylvay® (odevixibat)
200 | 400 | 600 | 1200 mcg capsules

Grow up with BYLVAY*

with flexible administration options[1]

RAPID REDUCTION IN PRURITUS AS EARLY AS WEEKS 1-4[1,2]

Rapid and durable reduction in pruritus[1-3]

Once-daily dosing with flexible administration options[1]

The **ONLY IBAT inhibitor** that:

- ✔ Offers once-daily dosing[25]
- ✔ Does not require fasting or delaying of meals[25]
- ✔ Does not require dose titration for standard treatment initiation[25]

IPSEN CARES® offers coverage, access, reimbursement, and education support for patients prescribed BYLVAY

Scan to learn more

PEDFIC 1 Key Efficacy Endpoint:
Mean percentage of pruritus assessments scored as little to no scratching (BYLVAY 40 mcg/kg/day 35%, BYLVAY 120 mcg/kg/day 30% vs placebo 13%)[1]

PEDFIC 1 Primary Endpoint:
Proportion of positive pruritus assessments over 24 weeks (All BYLVAY 55% vs placebo 30%)[3]

The mean of patients' worst weekly average scratching scores in each treatment group for each month, where the weekly average utilized the worst score from each day (morning or evening score), was assessed in PEDFIC 1.[1]

*BYLVAY can be taken 3 ways: swallow capsules whole; open capsules or oral pellets and mix contents with soft food or liquid.[1]

INDICATION

BYLVAY (odevixibat) is an ileal bile acid transporter (IBAT) inhibitor indicated for the treatment of pruritus in patients ≥3 months of age with progressive familial intrahepatic cholestasis (PFIC).

Limitation of Use:

BYLVAY is not recommended in a subgroup of PFIC type 2 patients with specific *ABCB11* variants resulting in non-functional or complete absence of the bile salt export pump protein.

IMPORTANT SAFETY INFORMATION

Contraindications

IBAT inhibitors, including BYLVAY, are contraindicated in patients with prior or active hepatic decompensation events (e.g., variceal hemorrhage, ascites, hepatic encephalopathy).

Please see additional Important Safety Information throughout and the full Prescribing Information.

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