

Summary report on authorisation dated 23 March 2026

Bylvay[®] (active substance: odevixibat)

Authorisation in Switzerland: 4 December 2025

Oral hard capsules for the treatment of cholestatic pruritus (itching) in patients aged 6 months and older with progressive familial intrahepatic cholestasis (PFIC) or patients aged 12 months and older with Alagille syndrome (ALGS)

About the medicinal product

Bylvay[®] contains the active substance odevixibat.

The medicinal product Bylvay is used for the treatment of intense itching caused by a build-up of bile in patients aged 6 months and older with PFIC or patients aged 12 months and older with ALGS.

PFIC and ALGS are rare genetic diseases in which bile may be prevented from draining away normally, which causes cholestasis. This leads to an increase in bile acids and other

components of bile in the blood. As a result, affected patients often suffer from severe itching.

The medicinal product Bylvay helps reduce the accumulation of bile acids in the body, thus alleviating the itching.

Since PFIC and ALGS are rare, life-threatening diseases, Bylvay has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

Odevixibat, the active substance in the medicinal product Bylvay, blocks the specific bile acid transporter in the intestine. This decreases the reabsorption of bile acids into

the body and reduces the accumulation of bile acids in the blood. This mechanism of action can alleviate itching.

Administration

Bylvay is a prescription-only medicine and available as oral hard capsules in dosages of 200 µg, 400 µg, 600 µg and 1200 µg odevixibat per hard capsule.

The usual daily starting dose is 40 µg/kg body weight in PFIC and 120 µg/kg body weight in ALGS.

The medicinal product is taken in the morning with or without food. The hard capsules

can be swallowed whole or opened and sprinkled on soft food or added to an age-appropriate fluid.

If added to fluid, an oral syringe must be used for administration.

Efficacy

The efficacy of Bylvay was investigated in patients with PFIC and ALGS who were experiencing pronounced cholestatic itching.

In a 24-week study (A4250-005) involving 62 children and adolescents with PFIC, treatment with Bylvay reduced itching significantly compared to placebo (dummy drug).

58.3% of patients treated with 40 µg/kg Bylvay and 47.7% of patients treated with 120 µg/kg Bylvay achieved a clinically relevant response (≤ 1 or ≥ 1 point improvement in pruritus assessment¹) compared to 28.7% of patients who received placebo.

43.5% of patients who received 40 µg/kg Bylvay and 21.1% of patients who received 120 µg/kg Bylvay achieved at least a 70% reduction in serum bile acids or a serum bile acid concentration of 70 µmol/L² or less. None of the patients in the placebo group achieved this endpoint.

Patients with PFIC maintained the improvements in itching and serum bile acid concentrations throughout long-term treatment lasting up to 96 weeks. However, no disease-modifying effect (e.g. delaying severe liver complications) was demonstrated. In view of this, authorisation has been restricted to the treatment of cholestatic pruritus³.

Bylvay also significantly reduced itching in a 24-week study (A4250-012) involving 52 patients with ALGS.

Serum bile acid concentrations also fell significantly.

A stable effect was sustained for up to 96 weeks in long-term treatment of ALGS. However, no beneficial effect on the course of the liver disease was demonstrated.

Precautions, undesirable effects, & risks

Bylvay may not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects are diarrhoea, abdominal pain and vomiting.

Increases in liver enzymes (e.g. ALT, AST, bilirubin) and decreases in fat-soluble vitamins (Vitamin D and E) have also been described.

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

The clinical data submitted show that odevixibat brings about a clinically relevant reduction in the itching triggered by bile

acid accumulation in patients with PFIC and ALGS.

¹ The pruritus assessment is a standardised metric – in this case a numeric rating scale – that objectifies the subjective sensation of itching on a scale of 0 (no itching) to 4 (severest possible itching) before and after treatment.

² Normal range: 0–10 µmol/L

³ Itching caused by disrupted bile drainage in the liver or bile ducts and thus by high concentrations of bile acids in the blood.

The improvement in quality of life is substantial, while the safety profile has been assessed as acceptable, despite possible hepatotoxic⁴ events and vitamin deficiencies.

No effect on the progression of the underlying disease was demonstrated.

The medicinal product has therefore been authorised for the treatment of cholestatic pruritus in these rare diseases.

Taking the limited treatment options for PFIC- and ALGS-related itching, the available data, and all risks and precautions into account, the benefits of Bylvay outweigh the risks. Swissmedic has therefore authorised the medicinal product Bylvay, containing the active substance odeixibat, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: [Information for healthcare professionals Bylvay®](#)

Information for patients (package leaflet): [Information for patients Bylvay®](#)
Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.

⁴ Hepatotoxic = damaging to the liver