swissmedic

Summary report on authorisation dated 3 February 2025

Livmarli® (active substance: maralixibat)

Authorisation in Switzerland: 18.07.2024

Oral solution for the treatment of cholestatic pruritus (itching) in patients aged 3 months and older with Alagille syndrome (ALGS)

About the medicinal product

Livmarli contains the active substance maralixibat.

The medicinal product Livmarli is used for the treatment of intense itching due to a build-up of bile in patients aged 3 months and older with ALGS.

ALGS is a rare hereditary disease that can lead, among other things, to a decrease in bile ducts in the liver, preventing normal bile drainage and causing 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 and fatigue. In addition, the skin may turn yellow.

The medicinal product Livmarli helps reduce the accumulation of bile acids in the body, thus relieving patients' symptoms.

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

Livmarli has been authorised by Swissmedic under Article 13 of the Therapeutic Products Act (TPA) for patients aged 12 months and older. This means that the medicinal product is already authorised in another country with comparable medicinal product control. In this case, Swissmedic takes into consideration the results of checks carried out by foreign regulatory agencies, provided certain requirements are fulfilled. These involve checks on the quality, efficacy, and safety of the medicinal product, and the extent to which the results can be accepted for Switzerland. The consideration of the results of foreign authorisation procedures is intended to help ensure that medicines that are already authorised abroad can be made available to patients in Switzerland as quickly as possible.

In deciding whether to authorise Livmarli in Switzerland, Swissmedic accepted the assessment findings of the US Food and Drug Administration (FDA; Reference ID: 4863362) and has carried out only a limited independent scientific assessment for infants aged 3 to 12 months.

Accordingly, in the SwissPAR (Swiss Public Assessment Report) and the resulting Summary report on authorisation, Swissmedic refers to the publicly available Assessment Report issued by the reference authority, the FDA: www.fda.gov

Mode of action

Maralixibat, the active substance in the medicinal product Livmarli, 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 liver and blood. This mechanism of action can alleviate itching.

Use

Livmarli is a prescription-only medicine and available as an oral solution at a dose of 9.5 mg maralixibat per mL.

The recommended dose is 380 µg/kg body weight once daily, taken 30 minutes before a morning meal.

Dosing starts at 190 µg/kg once daily, with the dose increasing after 1 week to 380 µg/kg once daily. The maximum daily dose for patients over 70 kg is 28.5 mg. If a dose is missed, it should be compensated for as soon as possible, within 12 hours of the usual dosing time.

The physician should check the patient's liver parameters before starting treatment and continue to monitor them.

Efficacy

The efficacy of Livmarli was investigated in a study involving a total of 31 patients with ALGS suffering from itching. The study participants were treated with the medicinal product Livmarli for 18 weeks. This was followed by a 4-week, placebo-controlled[1] phase. In this phase, some patients continued treatment with Livmarli, while others received a placebo.

The results of the study showed that itching was significantly reduced in patients receiving Livmarli, compared to the placebo group.

Precautions, undesirable effects, & risks

Livmarli must 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, vomiting, cough, infections of the nasal and throat mucosa, ear infections, headache, and fat-soluble vitamin deficiency.

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

Why the medicinal product has been authorised

At present, there are only limited drug treatment options for patients with ALGS suffering from cholestatic pruritus.

In the study conducted, it was shown that Livmarli, containing the active substance maralixibat, can reduce itching and thereby improve patients' quality of life.

Taking all the risks and precautions into account, and based on the available data, the benefits of Livmarli outweigh the risks.

[1] Placebo: dummy drug

Swissmedic has therefore authorised the medicinal product Livmarli, containing the active substance maralixibat, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Livmarli®

Information for patients (package leaflet): Information for patients Livmarli®

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.