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EPAR summary for the public

Kolbam

cholic acid

This is a summary of the European public assessment report (EPAR) for Kolbam. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Kolbam.

For practical information about using Kolbam, patients should read the package leaflet or contact their doctor or pharmacist.

What is Kolbam and what is it used for?

Kolbam is a medicine that contains the active substance cholic acid. This is a 'primary bile acid', which is a main component of the bile (a fluid produced by the liver that helps to digest fats).

Kolbam is authorised for the life-long treatment of adults and children from one month of age who cannot produce enough primary bile acids such as cholic acid due to certain genetic abnormalities that result in a lack of the following liver enzymes: sterol 27-hydroxylase, 2-methylacyl-CoA racemase; or cholesterol 7α-hydroxylase.

When primary bile acids are lacking, the body produces abnormal bile acids instead, which can damage the liver potentially leading to life-threatening liver failure. The condition is known as 'inborn errors in primary bile acid synthesis'.

Because the number of patients with inborn errors in primary bile acid synthesis is low, the condition is considered 'rare', and Kolbam was designated an 'orphan medicine' (a medicine used in rare diseases) on 28 October 2009.

How is Kolbam used?

Kolbam can only be obtained with a prescription and treatment should be started and supervised by a doctor specialised in the diseases Kolbam is used to treat.

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Kolbam is available as capsules (50 and 250 mg). The daily dose is chosen and adjusted during treatment for each patient depending on the patient's levels of bile acids in their blood and urine and their liver function. The maximum daily dose should not exceed 15 mg per kilogram body weight.

Kolbam should be taken every day at approximately the same time, with a meal. For small children who cannot swallow capsules, the contents can be mixed in with infant formula, expressed breast milk, mashed potatoes or fruit puree.

For more information, see the package leaflet.

How does Kolbam work?

Cholic acid is one of the main primary bile acids produced by the liver. The cholic acid contained in Kolbam replaces the patient's missing cholic acid. This helps to reduce the production of abnormal bile acids and contributes to the normal activity of bile in the digestive system, thereby relieving the symptoms of the condition.

What benefits of Kolbam have been shown in studies?

Kolbam was investigated in one main study involving 52 patients with inborn errors in primary bile acid synthesis, including 7 patients who lack either sterol 27-hydroxylase, 2-methylacyl-CoA racemase or cholesterol 7α-hydroxylase. The main measures of effectiveness were changes in bile acid levels and liver function before and after treatment with Kolbam. The efficacy of Kolbam for the authorised indications was established based on the results of this study. This was consistent with clinical expectations and literature data.

What are the risks associated with Kolbam?

Side effects with Kolbam are generally mild to moderate in severity and transitory. The most common side effects (which may affect up to 1 in 10 people) are peripheral neuropathy (nerve damage in the hands and feet), diarrhoea, nausea (feeling sick), acid reflux (stomach acid flowing up into the mouth), oesophagitis (inflammation of the food pipe), jaundice (yellowing of the skin and eyes), skin problems (lesions) and malaise (feeling unwell).

Kolbam must not be used in combination with phenobarbital (a medicine for epilepsy).

For the full list of all side effects and restrictions, see the package leaflet.

Why is Kolbam approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Kolbam's benefits are greater than its risks and recommended that it be approved for use in the EU. The CHMP concluded that Kolbam has beneficial effects in patients with deficiencies in the following liver enzymes: sterol 27-hydroxylase; 2-methylacyl-CoA racemase; and cholesterol 7α-hydroxylase. Regarding its safety, the side effects appeared to be non-serious and reversible.

Kolbam has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Kolbam due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

What information is still awaited for Kolbam?

Since Kolbam has been approved under exceptional circumstances, the company that markets Kolbam will monitor the benefits and safety of Kolbam from a patient registry and provide yearly updates.

What measures are being taken to ensure the safe and effective use of Kolbam?

A risk management plan has been developed to ensure that Kolbam is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Kolbam, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Kolbam will provide educational material with information on the correct and safe use of Kolbam to all doctors expected to prescribe this medicine.

Further information can be found in the summary of the risk management plan.

Other information about Kolbam

The European Commission granted a marketing authorisation valid throughout the European Union for Kolbam on 20 November 2015.

The full EPAR and risk management plan summary for Kolbam can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Kolbam, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Kolbam can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 02-2016.