EMA/4952/2022
EMEA/H/C/005646

Sapropterin Dipharma (*sapropterin*)

An overview of Sapropterin Dipharma and why it is authorised in the EU

What is Sapropterin Dipharma and what is it used for?

Sapropterin Dipharma is a medicine that is used to treat high blood levels of phenylalanine in adults and children of all ages with the genetic disorders phenylketonuria (PKU) or tetrahydrobiopterin (BH4) deficiency.

Patients with these disorders cannot process the amino acid phenylalanine from dietary protein. As a result, phenylalanine builds up in the blood to abnormally high levels, causing problems in the nervous system.

Sapropterin Dipharma contains the active substance sapropterin and is a 'generic medicine'. This means that Sapropterin Dipharma contains the same active substance and works in the same way as a 'reference medicine' already authorised in the EU called Kuvan. For more information on generic medicines, see the question-and-answer document here.

How is Sapropterin Dipharma used?

Sapropterin Dipharma is available as soluble tablets or as a powder, to be dissolved in water and drunk. The medicine can only be obtained with a prescription and treatment must be started and supervised by a doctor who has experience in treating PKU and BH4 deficiency. It is important that patients continue with a diet low in phenylalanine and protein when taking Sapropterin Dipharma, and intake of phenylalanine and protein must be monitored and adjusted to make sure that blood phenylalanine levels and nutritional balance are controlled. Sapropterin Dipharma is intended for long-term use.

The starting dose of Sapropterin Dipharma depends on the patient's weight. The dose is then adjusted depending on blood levels of amino acids, including phenylalanine. Sapropterin Dipharma is taken with a meal at the same time every day, preferably in the morning. For some patients with BH4 deficiency, the dose may need to be divided into 2 or 3 doses over the course of the day to get the best effect.

A satisfactory response is defined as a reduction in blood phenylalanine levels of at least 30% or to a level determined by the doctor. If this has been achieved after one month, the patient is classified as a 'responder' and can continue taking Sapropterin Dipharma.

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For more information about using Sapropterin Dipharma, see the package leaflet or contact your doctor or pharmacist.

How does Sapropterin Dipharma work?

The high levels of phenylalanine in the blood are due to a problem with the breakdown of phenylalanine through the enzyme 'phenylalanine hydroxylase'. Patients with PKU have defective versions of the enzyme, and patients with BH4 deficiency have low levels of BH4, a 'cofactor' that this enzyme needs to work properly.

The active substance in Sapropterin Dipharma, sapropterin, is a synthetic copy of BH4. In patients with PKU, it works by enhancing the activity of the defective enzyme, while in patients with BH4 deficiency it replaces the missing cofactor. These actions help restore the ability of the enzyme to convert phenylalanine into tyrosine, thereby reducing phenylalanine levels in the blood.

How has Sapropterin Dipharma been studied?

Studies on the benefits and risks of the active substance in the authorised uses have already been carried out with the reference medicine, Kuvan, and do not need to be repeated for Sapropterin Dipharma.

As for every medicine, the company provided data on the quality of Sapropterin Dipharma. The company also carried out a study that showed that it is 'bioequivalent' to the reference medicine. Two medicines are bioequivalent when they produce the same levels of the active substance in the body and are therefore expected to have the same effect.

What are the benefits and risks of Sapropterin Dipharma?

Because Sapropterin Dipharma is a generic medicine and is bioequivalent to the reference medicine, its benefits and risks are taken as being the same as the reference medicine's.

Why is Sapropterin Dipharma authorised in the EU?

The European Medicines Agency concluded that, in accordance with EU requirements, Sapropterin Dipharma has been shown to have comparable quality and to be bioequivalent to Kuvan. Therefore, the Agency's view was that, as for Kuvan, the benefits of Sapropterin Dipharma outweigh the identified risks and it can be authorised for use in the EU.

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

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Sapropterin Dipharma have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Sapropterin Dipharma are continuously monitored. Suspected side effects reported with Sapropterin Dipharma are carefully evaluated and any necessary action taken to protect patients.

Other information about Sapropterin Dipharma

Sapropterin Dipharma received a marketing authorisation valid throughout the EU on 16 February 2022.

Further information on Sapropterin Dipharma can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/sapropterin-dipharma. Information on the reference medicine can also be found on the Agency's website.

This overview was last updated in 02-2022.