

KUVAN[®]

(sapropterin dihydrochloride) Tablets



What are KUVAN[®] (sapropterin dihydrochloride) Tablets?

KUVAN (sapropterin dihydrochloride) Tablets are a form of the cofactor (or helper) BH4 that already naturally exists in your body. In people who respond to treatment with KUVAN, it works in the body just like BH4 does to help stimulate residual PAH enzyme activity to convert Phe to tyrosine and lower blood Phe levels. KUVAN has been prescribed along with a low-Phe diet to lower blood Phe levels in many people with PKU of all ages and types—even classic!



KUVAN[®] (sapropterin dihydrochloride) Tablets Facts

- Scientists around the world have studied the safety of BH4, the active ingredient in KUVAN (sapropterin dihydrochloride) Tablets, for over **30** years.
- The safety of KUVAN has been tested in over **600** people with PKU of all ages in BioMarin clinical trials.
 - During the clinical trials, the most common side effects reported were: headache, diarrhea, abdominal or throat pain, upper respiratory tract infections, vomiting and nausea. These same side effects were seen in patients taking placebo (sugar pill) tablets.
- BioMarin maintains patient registries of over **1,000** patients that collect data related to the safety of KUVAN.
- Over **3,000** people with PKU have been treated with KUVAN in the United States since its FDA approval in 2007.

Phenylketonuria (PKU) is caused by a defect in an enzyme called phenylalanine hydroxylase (PAH). The PAH enzyme uses tetrahydrobiopterin (BH4), a naturally occurring cofactor (or helper) in the body, to break down phenylalanine (Phe) into tyrosine. Tyrosine is a building block for many proteins and important chemicals called neurotransmitters that help brain function which can influence the way someone feels, thinks, and acts.

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In PKU, the PAH enzyme is not working properly and is unable to break down Phe in your body. When tyrosine is not produced and Phe builds up in the blood it can cause a variety of signs and symptoms, including mental, behavioral, neurological, and physical problems.

Could KUVAN[®] (sapropterin dihydrochloride) Tablets be right for you?

Talk with your doctor and together, *You* can decide!

KUVAN (sapropterin dihydrochloride) Tablets are approved to reduce blood Phe levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). KUVAN is to be used with a Phe-restricted diet.

Important Safety Information

High blood Phe levels are toxic to the brain and can lead to lower intelligence and decrease in the ability to focus, remember and organize information. Any change you make to your diet may impact your blood Phe level. Follow your doctor's instructions carefully. Your doctor and dietitian will continue to monitor and may adjust your diet throughout your treatment with KUVAN (sapropterin dihydrochloride) Tablets.

If you have a fever, or if you are sick, your Phe level may go up. Tell your doctor or dietitian as soon as possible so they can see if they have to adjust your treatment to help keep your blood Phe levels in the desired range.

KUVAN is a prescription medicine and should not be taken by people who are allergic to any of its ingredients. Tell your doctor if you have ever had liver problems, are nursing or pregnant or may become pregnant, have poor nutrition or are anorexic. Your doctor will decide if KUVAN is right for you. Tell your doctor about all the medicines you take.

The most common side effects reported when using KUVAN are headache, diarrhea, abdominal pain, upper respiratory tract infection (like cold), throat pain, vomiting, and nausea.

To report SUSPECTED ADVERSE REACTIONS, contact BioMarin Pharmaceutical Inc. at 1-866-906-6100, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. Please see attached prescribing information.



Indications & ISI



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