

# An Introduction to BioMarin RareConnections™



BioMarin is committed to the PKU community and to ensuring that patients who need access to KUVAN® (sapropterin dihydrochloride) Tablets or Powder for Oral Solution are supported. Through BioMarin RareConnections™ you will be connected with a personal case manager who will be available to assist you every step of the way!

## Getting Started with KUVAN

Once you and your doctor have determined KUVAN is right for you, you will be asked to complete and sign a Patient Authorization Form. Your clinic will provide this form, along with your KUVAN prescription directly to BioMarin RareConnections. You will then be contacted directly by a BioMarin RareConnections Case Manager. Your case manager will work with you every step of the way during your KUVAN treatment to ensure you have the support you need to be successful.

## Personalized Reimbursement Support

BioMarin RareConnections will contact your insurance provider to determine co-pay and/or deductible amounts for your KUVAN insurance coverage. Your case manager will then review this information with you and, if needed, advise you of financial assistance options that may be available through the National Organization for Rare Disorders (NORD) or BioMarin.

- If you cannot afford your insurance co-payment or require additional financial assistance, your BioMarin RareConnections™ Case Manager is available to assist you with a referral to NORD.
- If you do not have insurance, or if your insurance does not cover KUVAN, BioMarin RareConnections will assist you to identify alternative sources of coverage for KUVAN.
- If an alternative source of coverage is not available, the BioMarin Patient Assistance Program may be able to provide you with KUVAN at no cost. Patients must first enroll in the program and meet eligibility criteria in order to receive free medication.

## Connecting You with a Specialty Pharmacy

Once BioMarin RareConnections has identified and confirmed your insurance benefits you will be contacted by a Specialty Pharmacy to schedule your KUVAN shipments at a date and time convenient for you.

## Reimbursement Facts

- KUVAN (sapropterin dihydrochloride) is covered by most insurance plans.
- Most patients have a co-payment of \$50 or less for their KUVAN prescription.
- NORD offers financial assistance to eligible patients to assist with the costs associated with PKU treatment.
- The BioMarin Patient Assistance Program provides KUVAN at no charge to eligible patients without insurance.

### Phone

1.877.MY.KUVAN  
(1.877.695.8826)

### Fax

1.888.863.3361

### Email

support@biomarin-  
rareconnections.com

### Hours of Operation

M-F, 6AM-5PM (PT)

## Indication

KUVAN® (sapropterin dihydrochloride) Tablets for Oral Use and Powder for Oral Solution are approved to reduce blood Phe levels in people with a certain type of Phenylketonuria (PKU). KUVAN is to be used with a Phe-restricted diet.

## Important Safety Information

It is not possible to know if KUVAN will work for you without a trial of the medicine. Your doctor will check your blood Phe levels when you start taking KUVAN to see if the medicine is working.

Starting KUVAN does not eliminate the need for ongoing dietary management. Any change to your diet may impact your blood Phe level. Follow your doctor's instructions carefully. Your doctor and dietitian will continue to monitor your diet and blood Phe levels throughout your treatment with KUVAN **to make sure your blood Phe levels are not too high or too low.**

If you have a fever, or if you are sick, your Phe level may go up. Tell your doctor and dietitian as soon as possible so they can make any necessary changes to your treatment.

Children younger than 7 years old treated with KUVAN doses of 20 mg/kg per day are at an increased risk for low levels of blood Phe compared with children 7 years and older. Frequent blood monitoring is recommended in this population to ensure that blood Phe levels do not fall too low.

Tell your doctor if you have ever had liver or kidney problems, have poor nutrition or have a loss of appetite, are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

KUVAN is a prescription medicine and should not be taken by people who are allergic to any of its ingredients. KUVAN and other medicines may interact with each other. Tell your doctor about **all the medicines you take**, including prescription and over-the-counter medicines, vitamins, herbal and dietary supplements.

If you forget to take your dose of KUVAN, take it as soon as you remember that day. Do not take 2 doses in a day. If you take too much KUVAN, call your doctor for advice.

The most common side effects reported when using KUVAN are headache, runny nose and nasal congestion, sore throat, diarrhea, vomiting, and cough. Additional adverse reactions reported in connection with worldwide marketing include sore throat, heartburn or pain in the esophagus, inflammation of the lining of the stomach, indigestion, stomach pain, and nausea. These are not all the possible side effects seen with KUVAN. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

KUVAN can cause serious side effects, including:

- **Severe allergic reactions.** Stop taking KUVAN and **get medical help right away** if you develop any of these symptoms of a severe allergic reaction:
  - Wheezing or trouble breathing
  - Nausea
  - Flushing
  - Lightheadedness or fainting
  - Coughing
  - Rash
- **Inflammation of the lining of the stomach (gastritis).** Gastritis can happen with KUVAN and may be severe. **Call your doctor right away if you have any:**
  - Severe upper stomach-area discomfort or pain
  - Blood in your vomit or stool
  - Black, tarry stools
  - Nausea and vomiting
- **Too much or constant activity (hyperactivity) can happen with KUVAN.** Tell your doctor if you have any signs of hyperactivity, including fidgeting, moving around or talking too much.

For more information, call BioMarin RareConnections™ at 1-888-863-3361.

**Please read the attached full Patient Information.**

**References:** 1. Muntau AC et al. J Inherit Metab Dis. 2014;37(suppl 1):S69. Abstract P-096. 2. Hoeksma M, et al. J Inherit Metab Dis. 2005;28(6):845-854. 3. Huemer M et al. J Inherit Metab Dis. 2007;30(5):694-699. 4. Enns GM et al. Mol Genet Metab. 2010;101(2-3):99-109.

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**KUVAN**®  
(sapropterin dihydrochloride)  
Tablets or Powder for Oral Solution