

Your KUVAN treatment starts with BPPS. Our priority is to see that your KUVAN treatment begins promptly and is not interrupted.

To reach a BPPS case manager:

Call toll-free: **1-877-MY-KUVAN (1-877-695-8826)**

E-mail: **bpps@BMRN.com**

**Personalized support
for all KUVAN patients.**



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Patient Support and Assistance for KUVAN

A comprehensive guide to access programs
available for patients starting KUVAN treatment



Gina, age 40,
takes KUVAN



BioMarin Is Committed to Helping PKU Patients Obtain Access to KUVAN

Because KUVAN is the first treatment option of its kind for phenylketonuria, or PKU, you might find it challenging to navigate the insurance process on your own. That's why BioMarin Patient and Physician Support (BPPS) is here—to work with you, your doctor's office, and your health insurance company. BPPS does the majority of the work and handles most of the details for you.

At BPPS, our priority is to help you begin treatment with KUVAN as promptly as possible.

This booklet explains the programs that are in place to help you obtain coverage and access to KUVAN. Most insurance companies cover KUVAN with a reasonable co-payment (the average co-payment is \$50 per month, but this varies from plan to plan). BPPS can work with your insurance

company to help obtain coverage for KUVAN, and help you research insurance coverage if you don't already have it. BPPS is also your gateway to other patient services, such as:

- Specialty pharmacies that can deliver KUVAN directly to your home.
- The KUVAN Patient Assistance Program (K-PAP), which can provide KUVAN at no charge for eligible patients without insurance coverage.
- The National Organization for Rare Disorders (NORD), which can provide premium and co-payment assistance for KUVAN and other assistance with costs directly related to your PKU treatment.

Please direct any questions on these topics to BPPS at **1-877-MY-KUVAN (1-877-695-8826)**. Additional information on all of these topics can also be found at www.KUVAN.com.

KUVAN® (sapropterin dihydrochloride) Tablets is 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.

Please see Important Safety Information on pages 14–15.



BPPS Is Your Gateway to Receiving KUVAN

5 simple steps for reimbursement

- 1** **Sign** the BPPS Patient Authorization form. Your clinic will complete the prescription form.
- 2** **Ask** your doctor's/dietitian's office to fax the forms to BPPS.
- 3** **Expect** a call from your personal BPPS case manager, who will research insurance coverage and alternative benefit options to get you started on KUVAN as soon as possible, while keeping you updated every step of the way.
- 4** **Schedule** your first home delivery of KUVAN with the specialty pharmacy, which will confirm benefits and co-payment expense, and make payment arrangements with you.
- 5** **Call** your BPPS case manager with any questions or problems about your KUVAN shipment or insurance coverage.

The process

After you and your doctor and/or dietitian complete the BPPS forms and fax them in, the BPPS case manager assigned to you will call you within one business day. Your BPPS case manager works with your insurance plan to submit any required paperwork, investigates your benefits for prescription drugs, and identifies any additional insurance plan requirements for coverage. If you do not have insurance, BPPS will help to research an insurance policy for you that provides KUVAN coverage.

During the process, your BPPS case manager will keep you, your doctor, and your dietitian updated as the process moves forward.

Please see Important Safety Information on pages 14–15.



Once approved by your insurance plan, your BPPS case manager will refer your case to a specialty pharmacy that sends regular shipments of KUVAN directly to your home on an ongoing basis. The specialty pharmacy will verify your benefits and the exact out-of-pocket expense and make payment arrangements with you. However, your BPPS case manager is available for any future questions or issues that may arise as your treatment continues.

Your BPPS case manager will keep you,
your doctor, and your dietitian updated
every step of the way.

Timing

It generally takes BPPS 2–4 weeks to complete the insurance evaluation for an individual case. Each step in the insurance evaluation requires approval by you, your doctor, or insurance plan to move forward, so it is very important that you respond as soon as possible to any questions that BPPS may have.

Contacting BPPS

You can reach BPPS by phone Monday through Friday between 7 AM and 4 PM (Pacific Time) at **1-877-MY-KUVAN (1-877-695-8826)** or by e-mail at **bpps@BMRN.com**. Although BPPS is there to answer questions about reimbursement, BPPS refers all clinical questions about taking KUVAN back to your doctor.

More information about BPPS can be found at **www.KUVAN.com** under the FAQs link.

Please see Important Safety
Information on pages 14–15.



Specialty Pharmacies

KUVAN is not available at your neighborhood pharmacy. Instead, it is conveniently dispensed by a specialty pharmacy. BPPS will arrange for the specialty pharmacy to ship KUVAN to your home. Most shipments take 2–3 days to arrive, but in emergency cases, specialty pharmacies can ship KUVAN via next-day delivery.

The process

Once BPPS has completed the insurance authorization process for you, BPPS will notify you that the case has been referred to a specialty pharmacy for dispensing. The specialty pharmacy will call you to confirm your insurance coverage and the co-payment amount and arrange for KUVAN delivery. The specialty pharmacy will also call 7–10 days before the next refill date to schedule future product shipments. Therefore, be sure to return any phone calls from the specialty pharmacy promptly.

Contacting the specialty pharmacy

Contact BPPS by telephone at **1-877-MY-KUVAN (1-877-695-8826)** with any questions about the specialty pharmacy. The phone number of the specific specialty pharmacy assigned to you will be provided by your BPPS case manager.

More information about specialty pharmacies can be found at **www.KUVAN.com** under the FAQs link.



Please see Important Safety Information on pages 14–15.

KUVAN[®]
(sapropterin dihydrochloride) Tablets

KUVAN Patient Assistance Program (K-PAP)

If BPPS discovers that you do not have insurance coverage for KUVAN and is not successful in locating insurance coverage for KUVAN, BPPS will refer you to K-PAP. K-PAP provides KUVAN at no charge to qualified patients, on a case-by-case basis, who do not have insurance coverage for KUVAN.

The process

BPPS will already have the forms you and your doctor and/or dietitian completed, but you will need to complete an additional application packet. K-PAP will work with your doctor to obtain a prescription for the initial 30-day shipment. Once the application and prescription are received, the K-PAP specialty pharmacy will contact you to arrange for product shipment. If you respond to treatment, K-PAP will work with you and your doctor to obtain a follow-up prescription.

Please note that K-PAP is a program of last resort. Therefore, patients who are referred to K-PAP must meet general eligibility requirements and must be willing to work with BPPS to identify and secure alternative sources for prescription drug coverage.

Timing

If you remain compliant with treatment and work with BPPS to attempt to obtain other kinds of coverage for KUVAN, you are eligible for K-PAP for up to 12 months. You must reapply annually to K-PAP.

Contacting K-PAP

You can call BPPS case managers who provide K-PAP assistance to patients Monday through Friday from 7AM to 4 PM (PT) at **1-877-MY-KUVAN (1-877-695-8826)**. The BPPS case managers can mail the K-PAP application packet to you to get you started on this program.

More information about K-PAP can be found at www.KUVAN.com under the FAQs link.

Please see Important Safety Information on pages 14–15.



National Organization for Rare Disorders (NORD)

Some patients may be unable to afford their KUVAN co-payment. If this applies to you, BPPS can refer you to NORD. NORD is an independent nonprofit agency dedicated to the treatment and cure of rare diseases. NORD has set up a fund that provides premium and co-payment assistance for patients receiving prescription drug treatment for PKU. Other costs necessary for remaining on drug therapy, such as lab tests, may also be covered by NORD.

The process

After referral to NORD by BPPS, NORD will call you and send an application packet upon request. All patients are eligible to apply for the program. There is no preset income threshold for applying to the program; however, NORD examines household income and available assets versus household and medical expenses in determining eligibility.

NORD will notify you by mail of acceptance into the program or denial. After acceptance into the program, you must remain compliant with treatment, defined as taking $\geq 90\%$ of prescribed PKU medications.

If for some reason access to the program is denied, NORD encourages you to resubmit your application along with additional information explaining why your case should be reconsidered.

Timing

All fully completed applications are reviewed within 10–12 business days. Eligibility for NORD assistance is guaranteed for 12 months, then you must reapply.

Contacting NORD

After referral by BPPS, you can reach NORD patient representatives by calling **1-877-798-8716** Monday through Friday from 9 AM to 5 PM (Eastern Time) or by accessing their Web site at www.rarediseases.org.

More information about NORD can be found at www.KUVAN.com under the FAQs link.

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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.

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 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 a 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.

For more information, please read the attached Patient Package Insert.

