



With BioMarin by your side, you'll have access to comprehensive resources and individualized support

Look inside to learn more



Jennifer, Living With PKU



Bailey, Living With PKU

Learn more about comprehensive patient support from BioMarin

BioMarin is committed to providing personalized support services to help PKU patients start and stay on therapy, including:



BioMarin RareConnections™

Learn how a Case Manager will work with your clinic team and support you by confirming insurance benefits, providing financial assistance options, and coordinating shipment of medication.

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BioMarin Clinical Coordinators

Review details of the one-on-one product support and education you'll receive from a Clinical Coordinator.

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Co-pay assistance programs

Explore options to help you pay the out-of-pocket costs for PALYNZIQ® (pegvaliase-pqpz) Injection and KUVAN® (sapropterin dihydrochloride) Tablets or Powder for Oral Solution.

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Free 30-day trial of KUVAN

Find out about an opportunity to try KUVAN for free to see if it helps lower blood Phe levels.

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What is PALYNZIQ?

PALYNZIQ® (Pal-lin-zeek) (pegvaliase-pqpz) Injection is a prescription medication used to lower blood levels of phenylalanine (Phe) in adults with PKU (phenylketonuria) who have uncontrolled blood Phe levels above 600 micromol/L (10 mg/dL) on their current treatment. You should discuss the potential benefits and risks of PALYNZIQ with your healthcare provider.

BOXED WARNING: RISK OF ANAPHYLAXIS

- Anaphylaxis has been reported after administration of PALYNZIQ and may occur at any time during treatment with PALYNZIQ

What is KUVAN?

KUVAN® (sapropterin dihydrochloride) Tablets for Oral Use and Powder for Oral Solution are prescription medicines used to lower blood Phe levels in adults and children over one month of age with a certain type of Phenylketonuria (PKU). KUVAN is to be used along with a Phe-restricted diet.

Please see Important Safety Information for KUVAN throughout and full [Prescribing Information](#). Please see Important Safety Information for PALYNZIQ throughout, including important warning for risk of anaphylaxis, and full [Prescribing Information](#) and [Medication Guide](#).



BioMarin RareConnections™ Case Managers are here to support you

Whether you're living with a rare disease or caring for someone who is, it's normal to have a lot on your mind. The last thing you need to worry about is making sure the treatment is covered by your insurance, or figuring out the logistics of getting your treatment.

That's where BioMarin RareConnections Case Managers come in. Experienced Case Managers provide one-on-one support to address unique product needs, including:



Personalized financial support

With the help of your Case Manager, you'll have support throughout the process of securing coverage for your medication, which starts with identifying your insurance coverage. If needed, he or she will provide information about financial assistance options that may help you pay for your treatment.



Specialty pharmacy coordination

Your Case Manager will work directly with a specialty pharmacy to coordinate delivery of your medication at a date and time that is convenient for you. They can also provide updates on shipment status.



Ongoing support

BioMarin RareConnections Case Managers work closely with your clinic and other BioMarin teams to ensure you have product support throughout treatment.

To take advantage of BioMarin support services, contact BioMarin RareConnections. Get started by visiting [BioMarin-RareConnections.com](https://www.biopharm.com/rareconnections), or by calling 1-866-906-6100.

Please see Important Safety Information for KUVAN throughout and full [Prescribing Information](#). Please see Important Safety Information for PALYNZIQ throughout, including important warning for risk of anaphylaxis, and full [Prescribing Information](#) and [Medication Guide](#).



Experience personal product support with a BioMarin Clinical Coordinator

When you're living with a rare disease like PKU, it's important to have a trusted team that is ready to answer questions about your therapy. Your BioMarin Clinical Coordinator will be there to support your treatment journey by helping you fit your BioMarin treatment into your lifestyle.

BioMarin Clinical Coordinators will provide ongoing product support and education in person, by phone or text, or via email. More specifically, they will provide:



Product support throughout therapy

As you get started on the treatment plan your clinic sets up, your Clinical Coordinator will be there to answer questions you have about your therapy. They are also available for face-to-face or virtual visits.



Education

Your Clinical Coordinator will partner with you to provide information about PKU, your BioMarin therapy, how to take your medication, and dietary options. They also offer grocery store tours to show you foods that meet your dietary needs.



Help staying on track

We're here to support you throughout your treatment journey. Your Clinical Coordinator will help provide reminders to stay on track with the treatment plan your clinic team sets up.

For adults on PALYNZIQ® (pegvaliase-pqpz) Injection: In addition to the services listed above, Clinical Coordinators can provide injection training and ongoing reinforcement of how to properly inject PALYNZIQ.

Please see Important Safety Information for KUVAN throughout and full [Prescribing Information](#). Please see Important Safety Information for PALYNZIQ throughout, including important warning for risk of anaphylaxis, and full [Prescribing Information](#) and [Medication Guide](#).



Eligible patients pay as little as \$0 for BioMarin therapies with co-pay assistance programs*

We understand the challenges that come with PKU, and we want to help with co-pay or out-of-pocket costs for you and your family. Contact us today to find out if you qualify to receive assistance with out-of-pocket costs for BioMarin therapies.

With the PALYNZIQ Co-Pay Assistance Program...

99%

of participating patients paid
\$0 OUT-OF-POCKET
for their prescription†

- The PALYNZIQ Co-Pay Assistance Program will cover all co-pay costs related to both your PALYNZIQ® (pegvaliase-pqpz) Injection and auto-injectable epinephrine prescriptions, up to the annual maximum, for as long as you remain on therapy*
- Eligible patients will receive 3 fills of auto-injectable epinephrine per year at no additional cost*

With the KUVAN Co-Pay Assistance Program...

98%

of participating patients paid
\$0 OUT-OF-POCKET
for their prescription†

- The program covers all co-pay costs related to your KUVAN® (sapropterin dihydrochloride) Tablets for Oral Use and Powder for Oral Solution prescription, up to the annual maximum, for as long as you remain on therapy and eligible*

In order to qualify for BioMarin's Co-Pay Assistance Programs, you must:

- Have a PALYNZIQ or KUVAN prescription for an FDA-approved indication
- Currently have commercial (private) insurance
- Ensure your prescription is not covered in whole or in part by any federal or state healthcare programs
- Live in the United States or Puerto Rico

To take advantage of BioMarin support services, contact BioMarin RareConnections™. Get started by visiting [BioMarin-RareConnections.com](https://www.biopharm-rareconnections.com), or by calling 1-866-906-6100.

*Valid only for those with commercial insurance. Offer not valid for prescriptions eligible to be reimbursed, in whole or in part, by Medicare, Medicaid, or any other federal or state program (including any state prescription drug assistance programs) (eg, VA, DoD, TRICARE), for cash-paying patients, where product is not covered by patient's commercial insurance, or where plan reimburses you for entire cost of your prescription drug. No claim for reimbursement of the out-of-pocket expense amount covered by the program shall be submitted to any third-party payer, whether public or private. Offer is not valid where prohibited by law. Residents of CA and MA are not eligible for co-pay assistance for auto-injectable epinephrine or the KUVAN Co-Pay Assistance Program. Valid only in the United States and Puerto Rico. This program is not health insurance. Offer may not be combined with any other rebate, coupon, or offer. Co-payment assistance under the program is not transferable. BioMarin Pharmaceutical Inc. reserves the right to rescind, revoke, or amend the program without notice. Patient certifies responsibility for complying with applicable limitations, if any, of any commercial insurance and reporting receipt of program rewards, if necessary, to any commercial insurer. This program is subject to termination or modification at any time. The PALYNZIQ Co-Pay Assistance Program will cover up to \$16,750 in assistance per calendar year for eligible patients and the KUVAN Co-Pay Assistance Program will cover up to \$15,750 in assistance per calendar year for eligible patients. Some restrictions apply.

†Based on co-pay program data between January 1, 2018 and November 30, 2020.

Please see Important Safety Information for KUVAN throughout and full [Prescribing Information](#). Please see Important Safety Information for PALYNZIQ throughout, including important warning for risk of anaphylaxis, and full [Prescribing Information](#) and [Medication Guide](#).



Free 30-day trial of KUVAN

While KUVAN® (sapropterin dihydrochloride) works for many, not every person with PKU responds to KUVAN. You can try KUVAN free of charge to see if it will help you or your loved one lower blood Phe levels in combination with a low-Phe diet.

Try
KUVAN
for **FREE** to see if it can
work for you

- BioMarin provides a free 30-day trial of KUVAN for eligible PKU patients
- During the trial period, you will have the support of a BioMarin Clinical Coordinator to help you stay on track with the treatment plan you and your clinic create

For more information about the BioMarin free 30-day trial program, visit www.KUVAN.com.

Program details:

- The free trial of KUVAN for up to 30 days is available only to individuals in the United States who have not tried KUVAN, are affiliated with a participating clinic, and are diagnosed with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin (BH4)-responsive PKU
- This program is offered through BioMarin Pharmaceutical Inc. and is managed by BioMarin RareConnections™. If you and your doctor determine that KUVAN is right for you or your child and if you have a valid prescription for KUVAN, your free trial for up to 30 days will be arranged by BioMarin RareConnections
- Upon receipt of your completed form, you will be contacted by a representative from BioMarin to complete a Patient Authorization to Share Health Information form. You must be 18 years of age or older to complete this form. A parent or guardian can submit the form on behalf of his/her child
- Before the free trial period ends, you will be evaluated by your clinic to see if you should continue taking KUVAN

Please see Important Safety Information for KUVAN throughout and full [Prescribing Information](#). Please see Important Safety Information for PALYNZIQ throughout, including important warning for risk of anaphylaxis, and full [Prescribing Information](#) and [Medication Guide](#).



Important Safety Information

What is KUVAN?


KUVAN® (sapropterin dihydrochloride) Tablets for Oral Use and Powder for Oral Solution are prescription medicines used to lower blood Phe levels in adults and children over one month of age with a certain type of Phenylketonuria (PKU). KUVAN is to be used along with a Phe-restricted diet.

What is the most important information I should know about KUVAN? KUVAN can cause serious side effects, including:

- **Severe allergic reactions including anaphylaxis.** Stop taking KUVAN and get medical help right away if you develop any of these signs or symptoms of a severe allergic reaction:
 - Wheezing, coughing, or trouble breathing
 - Feeling flushed, nauseous, lightheaded, or you faint
 - Rash
- **Inflammation of the lining of the esophagus or stomach (esophagitis and gastritis).** Your doctor will monitor you for symptoms of inflammation in your upper gastrointestinal tract, including your stomach and esophagus. If untreated, that inflammation can lead to serious side effects including narrowing of the esophagus, ulcers, and bleeding. Call your doctor right away if you have any of these signs or symptoms:
 - Pain in the upper abdomen (stomach area), esophagus, or throat
 - Nausea, trouble swallowing, loss of appetite, or vomiting
 - Blood in your vomit or stool
 - Black, tarry stools
- **Phe levels that are too low.** Patients have experienced low blood Phe during treatment with KUVAN. Low blood Phe is more common in children under the age of 7 who take high doses of KUVAN each day.
- **Too much or constant activity (hyperactivity) can happen with KUVAN.** Tell your doctor if you have any signs of hyperactivity, including:
 - Fidgeting or moving around too much
 - Talking too much

What should I tell my doctor before I take KUVAN? Before you take KUVAN, tell your doctor about all your medical conditions, including if you:

- Are allergic to sapropterin dihydrochloride or any of the ingredients in KUVAN
- Have poor nutrition or have a loss of appetite
- Are pregnant or plan to become pregnant
- Are breastfeeding or plan to breastfeed. It is not known if KUVAN passes into your breast milk. Talk to your doctor about the best way to feed your baby if you take KUVAN.



Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal and dietary supplements. KUVAN and other medicines may interact with each other. Especially tell your doctor if you take:

- A medicine that contains levodopa
- An antifolate medicine such as methotrexate, valproic acid, phenobarbital, trimethoprim
- Sildenafil (Revatio, Viagra), tadalafil (Adcirca, Cialis), vardenafil (Staxyn, Levitra)

Tell your doctor if you are not sure if your medicine is one that is listed above. Know the medicines you take. Keep a list of them to show your doctor and pharmacist when you get a new medicine.

How should I take KUVAN?

See the detailed “Instructions for Use” that comes with KUVAN for information about the correct way to dissolve and take a dose of KUVAN tablets or KUVAN powder for oral solution.

- KUVAN does not work for everyone. It is not possible to know if KUVAN will work for you until you start taking KUVAN. Your doctor will check your blood Phe levels when you start taking KUVAN to see if the medicine is working.
- Take KUVAN exactly as your doctor tells you
- You can swallow KUVAN tablets whole or dissolve the tablets in water or apple juice
- KUVAN powder for oral solution should be dissolved in water or apple juice
- KUVAN should be taken with a PKU-specific diet. Any change you make to your diet may affect your blood Phe level. Follow your doctor’s instructions carefully and do not make any changes to your dietary Phe intake without first talking with your doctor. Even if you take KUVAN, if your blood Phe levels are not well controlled, you can develop severe neurologic problems.
- Your doctor should continue to monitor your blood Phe levels often during your treatment with KUVAN **to make sure that your blood Phe levels are not too high or too low and frequent monitoring for pediatric patients is recommended. Both high and low Phe can result in serious problems. Active management of dietary Phe intake while taking KUVAN is required.**
- If you have a fever, or if you are sick, your blood Phe level may go up. Tell your doctor as soon as possible so he or she can change your dose of KUVAN to help keep your blood Phe levels in the desired range.
- 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 of KUVAN are: headache, runny nose and nasal congestion, sore throat, diarrhea, vomiting, and cough.

These are not all the possible side effects of KUVAN. For more information, ask your doctor or pharmacist. Call your doctor for medical advice about side effects.

You may report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to BioMarin Pharmaceutical Inc. at 1-866-906-6100.

Please read the full Prescribing Information by clicking [here](#).

IMPORTANT SAFETY INFORMATION

What is PALYNZIQ?

PALYNZIQ® (Pal-lin-zeek) (pegvaliase-pqpz) is a prescription medication used to lower blood levels of phenylalanine (Phe) in adults with PKU (phenylketonuria) who have uncontrolled blood Phe levels above 600 micromol/L (10 mg/dL) on their current treatment. You should discuss the potential benefits and risks of PALYNZIQ with your healthcare provider.

What is the most important information I should know about PALYNZIQ?

PALYNZIQ can cause a severe allergic reaction (anaphylaxis) that may be life threatening and can happen any time during treatment with PALYNZIQ.

Severe allergic reactions are a serious but common side effect of PALYNZIQ.

- You will receive your first injection of PALYNZIQ in a healthcare setting where you will be closely watched for at least 1 hour after your injection for a severe allergic reaction
- Your healthcare provider will prescribe auto-injectable epinephrine for you, and will teach you (or your caregiver) and your observer, if needed, when and how to use it if you have a severe allergic reaction
- If you have a severe allergic reaction during treatment with PALYNZIQ, you will need to receive an injection of epinephrine immediately and get emergency medical help right away
- Your healthcare provider will decide if you (or your caregiver) are able to give the PALYNZIQ injections, recognize the signs and symptoms of a severe allergic reaction, give an injection of epinephrine, and call for emergency help, if needed
- Your healthcare provider may recommend that an adult observer (or your caregiver) be with you when you give your PALYNZIQ injection and for at least 1 hour after your injection to watch you for signs and symptoms of a severe allergic reaction and, if needed, give you an injection of epinephrine and call for emergency medical help

Stop injecting PALYNZIQ and get emergency medical care right away if you have any of the following symptoms:

- | | |
|---------------------------------------|--|
| • Fainting (passing out) | • Swelling of your face, lips, eyes, or tongue |
| • Dizziness or lightheadedness | • Throat tightness |
| • Sudden confusion | • Flushed skin |
| • Trouble breathing or wheezing | • Skin rash, itching, or raised bumps on skin |
| • Chest discomfort or chest tightness | • Nausea, vomiting, or diarrhea |
| • Fast heart rate | • Losing control of urine or stools |

Keep the auto-injectable epinephrine with you at all times during treatment with PALYNZIQ.

Read the Patient Information that comes with the auto-injectable epinephrine that your healthcare provider prescribes for you for more information.

If you have a severe allergic reaction, do not continue to take PALYNZIQ until you talk with your healthcare provider. Your healthcare provider will tell you if you can continue treatment with PALYNZIQ.

People taking PALYNZIQ have also experienced **allergic reactions other than anaphylaxis**. Talk to your healthcare provider if you experience any allergic reactions when taking PALYNZIQ.

PALYNZIQ REMS: PALYNZIQ is available only through a restricted program called the PALYNZIQ REMS (Risk Evaluation and Mitigation Strategy). Talk to your healthcare provider about the PALYNZIQ REMS and how to enroll.

What should I tell my healthcare provider BEFORE starting PALYNZIQ?

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

Before injecting PALYNZIQ, talk to your healthcare provider right away if you cannot or will not use auto-injectable epinephrine to treat a severe allergic reaction. If you are pregnant or plan to become pregnant while taking PALYNZIQ, talk to your healthcare provider to discuss the risks and benefits of taking PALYNZIQ during pregnancy to you and your unborn baby. If you are breastfeeding or plan to breastfeed, talk to your healthcare provider about the best way to feed your baby if you take PALYNZIQ.

Before injecting PALYNZIQ, read the Medication Guide and Instructions for Use that come with your PALYNZIQ injection.

What should I watch for AFTER starting PALYNZIQ?

PALYNZIQ may cause serious side effects, including:

- Severe allergic reactions (anaphylaxis)
- Other allergic reactions to PALYNZIQ can happen during treatment with PALYNZIQ. Contact your healthcare provider right away if you have any of the following symptoms of an allergic reaction including: rash, itching, or swelling of the face, lips, eyes, or tongue. Your healthcare provider may change your dose of PALYNZIQ, stop your treatment with PALYNZIQ for a period of time, or prescribe medicine for you to take before your PALYNZIQ injection to help reduce the symptoms of an allergic reaction

The most common side effects of PALYNZIQ include injection site reactions (such as redness, itching, pain, bruising, rash, swelling, or tenderness), joint pain, headache, skin reactions that spread and last at least 14 days (such as itching, rash, or redness), nausea, stomach pain, vomiting, cough, mouth and throat pain, itching, diarrhea, stuffy nose, feeling very tired, dizziness, anxiety, and low levels of Phe in your blood.

These are not all of the possible side effects of PALYNZIQ. Speak with your healthcare provider right away about any side effects.

Important notes

Blood Phe testing and diet

- Your healthcare provider will monitor your blood Phe levels during PALYNZIQ treatment
- Monitor the amount of protein and Phe that you eat or drink. Your healthcare provider may change the amount of protein and Phe you should have in your diet during treatment with PALYNZIQ, depending on the levels of Phe in your blood. Follow your healthcare provider's instructions about the amount of protein and Phe you should have in your diet

Missed dose

- If a dose is missed, take your next dose as scheduled and do not take 2 doses of PALYNZIQ to make up for the missed dose

Pregnancy Surveillance Program

- There is a pregnancy surveillance program for females who take PALYNZIQ during pregnancy, or who become pregnant while receiving PALYNZIQ or within 1 month after their last dose of PALYNZIQ. The purpose of this program is to collect information about the health of you and your baby while taking PALYNZIQ. Talk to your healthcare provider about how you can take part in this program or call BioMarin at 1-866-906-6100

You may report side effects to BioMarin at 1-866-906-6100.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see full [Prescribing Information](#), including an important warning for risk of anaphylaxis, and the [Medication Guide](#).

A continued commitment to PKU

BioMarin has partnered with the PKU community for over 15 years. Together, we have advanced knowledge of care. Inspired every day by the courage of individuals with PKU and their families, we are committed to providing personalized support and innovating to meet the needs of the PKU community.

Take the first step to getting dedicated one-on-one support—reach out to BioMarin RareConnections™ today!



Email
support@BioMarin-RareConnections.com

Visit
BioMarin-RareConnections.com

Call
1-866-906-6100

