

Available PKU Therapies



Medication Name	How It Works	Administration	Who Can Take It	Possible Side Effects
Sapropterin Kuvan® (BioMarin Pharmaceutical) Javygtor™ (Cycle Pharmaceuticals)	This is a source of BH4 that is provided to help the PAH enzyme work better and lower Phe levels	Oral (powder or pill) Should be taken with food, preferably a higher fat meal	People with PKU who are 1 month and older. To be effective, individuals must have a form of PKU that is responsive to BH4	• Stomach pain, nausea or vomiting • Sore throat • Diarrhea
Sepiapterin Sephience™ (PTC Therapeutics)	Helps the body to make BH4 to help the PAH enzyme work better and lower blood Phe levels	Oral (powder) Should be taken with food, preferably a higher fat meal	People with PKU who are 1 month and older who are responsive to this medication	• Diarrhea • Headache • Stomach pain • Sore throat • Unusual colored feces
Pegvaliase-pqpz Palynziq™ (BioMarin Pharmaceutical)	Provides phenylamine ammonia lyase (PAL) which is an enzyme that can break down Phe so that it can be reduced in the body, as a substitute for the non-working PAH enzyme	Injection administered just under the skin	People with PKU who are 12 years and older and have uncontrolled blood Phe levels above 600 µmol/L (10 mg/dL) on their current treatment	• Severe allergic reaction (anaphylaxis) • Injection site reactions: redness, itching, pain, bruising, rash, swelling and tenderness • Phe levels that are too low

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About PKU

- People with PKU are missing an enzyme called the phenylalanine hydroxylase (PAH) enzyme, which is responsible for breaking down phenylalanine (Phe) into tyrosine (Tyr) in the body. This means that people with PKU cannot break down Phe, which can lead to a toxic build-up in the body of elevated blood levels.
- The PAH enzyme needs the cofactor, BH₄, which helps the PAH enzyme work properly.

In addition to managing PKU with diet and formula, there are now three approved medications for people with PKU and many more in development.

Approved medications have been extensively tested in clinical trials and then reviewed by the United States Food and Drug Administration (FDA) for safety and effectiveness and approved to help lower blood Phe levels in people with PKU. You can learn more about these treatments on the back page of this flyer.



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