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[Home](#) > FDA approves new treatment for PKU

Generic Name:

Pegvaliase-pqpz

Trade Name:

Palynziq

Company:

BioMarin Pharmaceutical

Notes:

FDA approved [pegvaliase-pqpz](#) for adults with the rare and serious genetic disease phenylketonuria (PKU), in which patients are born with an inability to break down the amino acid phenylalanine (Phe). Phe is present in protein-containing foods and high-intensity sweeteners used in a variety of foods and beverages.

The drug is a novel enzyme therapy for adult patients with PKU who have uncontrolled blood Phe concentrations on current treatment.

Safety and efficacy of the new drug were studied in two clinical trials in adult patients with PKU with blood Phe concentrations greater than 600 $\mu\text{mol/L}$ on existing management. Most of the participants were on an unrestricted diet before and during the trials.

In the first study, a randomized, open-label trial, patients were treated with increasing doses of pegvaliase-pqpz administered as an S.C. injection up to a target dose of either 20 mg/d or 40 mg/d. The second study was an 8-week, placebo-controlled, randomized withdrawal trial of patients who were previously treated with pegvaliase-pqpz. Patients treated with pegvaliase-pqpz achieved statistically significant reductions in blood phenylalanine concentrations from their pretreatment baseline blood Phe concentrations.

The most common adverse events included injection-site reactions, joint pain, hypersensitivity reactions, headache, generalized skin reactions lasting at least 14 days, pruritus, nausea, dizziness, abdominal pain, throat pain, fatigue, vomiting, cough, and diarrhea. Hypersensitivity reactions occurred in most patients, likely because of formation of antibodies to the product.

The most serious adverse reaction was anaphylaxis, which occurred most frequently during upward titration of the dose within the first year of treatment. Because of this serious risk, the labeling for pegvaliase-pqpz includes a boxed warning, and the product is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Prescribers must be certified by enrolling in the REMS program and completing training.

Requirements include the following:

- Prescribers must prescribe auto-injectable epinephrine with pegvaliase-pqpz.
- Pharmacies must be certified with the program and must dispense only to patients who are authorized to receive pegvaliase-pqpz.
- Patients must enroll in the program and be educated about the risk of anaphylaxis by a certified prescriber to ensure they understand the risks and benefits of treatment.

- Patients must have auto-injectable epinephrine available at all times while taking pegvaliase-pqpz.

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