MedicalXpress

New treatment approved for rare disease PKU

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(HealthDay)—Palynziq (pegvaliase-pqpz) has been approved by the U.S. Food and Drug Administration to treat phenylketonuria, commonly called PKU.

People with the rare yet serious inherited disorder can't break down an amino acid called phenylalanine, which is found in many sweeteners and protein-containing foods.

The disorder affects about 1 in 10,000 to 15,000 people in the United States. Left untreated, it can lead to serious learning, developmental and psychiatric disabilities, the FDA said in a news release.

Palynziq's safety and effectiveness were evaluated in clinical studies involving people with high concentrations of blood phenylalanine. The most common side effects of the enzyme substitution therapy were injection-site reactions, allergic-like hypersensitivity reactions, joint pain, headache, itchy skin, nausea, dizziness and abdominal pain.

The drug's label warns of a less-common risk of life-threatening anaphylaxis, which the agency said most often occurred when the dosage was increased during the first year of treatment.

Palynziq is produced by BioMarin Pharmaceutical, based in Novato, Calif.

More information: The FDA has more about this approval.