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ASIA EDITION

FDA approves BioMarin's metabolic disorder treatment

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The FDA approved Palynziq for adults who have unontrolled concentrations of phenylalanine above a specific threshold despite other treatments.



Singapore- U.S. regulators approved BioMarin Pharmaceutical Inc's Palynziq for adults with phenylketonuria, a rare metabolic disorder.

Phenylketonuria, or PKU, is a rare genetic disease that manifests at birth and results in a variety of cumulative toxic effects on the brain.

The drug, an injected enzyme replacement therapy, will have a list price of $267,000 a year, but after discounts mandated by government health plans and other concessions the net price will be $192,000.

Palynziq is the first approved enzyme substitution therapy to target the underlying cause of phenylketonuria, or PKU, by helping the body to break down phenylalanine, an amino acid found in protein-rich foods such as meat, dairy and eggs.

The FDA approved Palynziq for adults who have uncontrolled concentrations of phenylalanine above a specific threshold despite other treatments.