

USFDA approves Cerdelga for a rare genetic disorder

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Gaucher disease occurs in people who do not produce enough of an enzyme called glucocerebrosidase.

The enzyme deficiency causes fatty materials to collect in the spleen, liver and bone marrow.

The major signs of Gaucher disease include liver and spleen enlargement, low red blood cell counts (anemia), low blood platelet counts and bone problems.

Cerdelga is said to be a hard gelatin capsule containing eliglustat that is taken orally.

In patients with Gaucher disease Type-1, the drug slows down the production of the fatty materials by inhibiting the metabolic process that forms them.