

FDA approves new drug for rare genetic disease

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The Food and Drug Administration has approved a new capsule-based drug to treat Gaucher's disease.

Regulators approved the new drug, Cerdelga, for patients with the Type 1 form of the [rare genetic disorder](#), which causes excess fat to build up in the spleen, liver and [bone marrow](#).

Cerdelga is from Genzyme, the specialty drugmaker that introduced the first drug for Gaucher's disease 20 years ago. The oral treatment could serve as an alternative to Genzyme's best-selling drug Cerezyme, which is given intravenously.

Cerezyme had sales of \$935 million last year. The drug costs \$300,000 for a year's supply, on average. Genzyme says it will soon release pricing for its new drug.

Type 1 is the most common form of Gaucher's disease and affects an estimated 6,000 people in the U.S.

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