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On Drugs and Therapeutics

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IN BRIEF

Velaglucerase (*Vpriv*) for Gaucher's Disease

The FDA has approved velaglucerase alfa (*Vpriv* – Shire), a new formulation of glucocerebrosidase prepared from human fibroblasts, for treatment of the non-neurologic form of Gaucher's disease (Type 1). Patients with Gaucher's disease have a congenital deficiency of glucocerebrosidase that leads to accumulation of glucosylceramide, the end-product of sphingolipid catabolism, in the lysosomes of reticuloendothelial cells in the liver, spleen and bone marrow. Velaglucerase is the second form of the enzyme now available in the US; imiglucerase (*Cerezyme* – Genzyme), which is produced by recombinant DNA technology from Chinese hamster ovary cells, was marketed earlier but has recently been in short supply.¹ These agents are usually given as an IV infusion every 2 weeks. Both velaglucerase and imiglucerase have been shown to increase serum hemoglobin concentrations and platelet counts and decrease the size of the spleen. Velaglucerase contains the exact amino acid sequence of the human enzyme, while imiglucerase has one amino acid difference, but their activities are similar.² The main difference between them may be in their cost. *Cerezyme* costs about \$200,000 per year, while *Vpriv* is expected to cost about 15% less.²

1. Differentiation will be key challenge for Shire's Gaucher disease drug *Vpriv*. *The Pink Sheet*. March 8, 2010; 72: 27.
2. B Brumshtein et al. Characterization of gene-activated human acid-beta-glucosidase: crystal structure, glycan composition, and internalization into macrophages. *Glycobiology* 2010; 20:24.

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