VPRIV (Velaglucerase Alfa for Injection; Shire Human Genetic Therapies Inc.) for type 1 gaucher disease

Record Status
This is a bibliographic record of a published health technology assessment. No evaluation of the quality of this assessment has been made for the HTA database.

Citation
VPRIV (Velaglucerase Alfa for Injection; Shire Human Genetic Therapies Inc.) for type 1 gaucher disease. Lansdale: HAYES, Inc.. Health Technology Brief Publication. 2013

Authors' conclusions
Gaucher disease is an autosomal recessive disease and the most prevalent lysosomal storage disorder. This inborn disorder of metabolism results from a variant(s) of the glucocerebrosidase (GBA) gene, which leads to a deficiency of the enzyme beta-glucocerebrosidase. The defective beta-glucocerebrosidase activity results in the accumulation of glucosyl ceramide in cells of monocyte/macrophage origin, which are called Gaucher cells. Gaucher cells accumulate in the liver, spleen, cortical bone and bone marrow, lymph nodes, and lungs. This causes hepatic and splenic enlargement, anemia and thrombocytopenia, destructive bone disease, lymphadenopathy and, occasionally, pulmonary dysfunction. Considered an ultra-orphan disease, Gaucher disease is very rare and affects 1 in 40,000 to 1 in 60,000 people in the general population. There are 3 types of Gaucher disease with type 1 Gaucher disease being the most common, affecting approximately 90% of these patients. It primarily affects the visceral organs in children and adults. The most common treatment for type 1 Gaucher disease is enzyme replacement therapy (ERT). The goal of ERT is to replace or supplement the patient's glucocerebrosidase enzyme. This therapy is given by intravenous infusion. Imiglucerase (Cerezyme, Genzyme Corp.), a recombinant human glucocerebrosidase, was the first ERT approved by the Food and Drug Administration (FDA) in 1991, for symptomatic type 1 Gaucher disease. In February 2010, the FDA approved a new ERT, velaglucerase alfa (VPRIV, Shire HGT), for long-term therapy for patients with type 1 Gaucher disease.

Final publication URL
The report may be purchased from: http://www.hayesinc.com/hayes/crd/?crd=15158

Indexing Status
Subject indexing assigned by CRD

MeSH
Humans; Gaucher Disease; Glucosylceramidase; Recombinant Proteins

Language Published
English

Country of organisation
United States

English summary
An English language summary is available.

Address for correspondence
HAYES, Inc., 157 S. Broad Street, Suite 200, Lansdale, PA 19446, USA. Tel: 215 855 0615; Fax: 215 855 5218 Email: hayesinfo@hayesinc.com

AccessionNumber
32013000588
Date abstract record published
29/07/2013