24 June 2010
EMA/CHMP/384050/2010
Committee for medicinal products for human use (CHMP)

Summary of opinion[1] (initial authorisation)

VPRIV

velaglucerase alfa

On 24 June 2010 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product VPRIV, 200 U, 400 U, powder for solution for infusion, intended for the treatment for long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease. VPRIV was designated as an orphan medicinal product on 9 June 2010. The applicant for this medicinal product is Shire Pharmaceuticals Ireland Ltd.

They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of VPRIV is velaglucerase alfa, Other alimentary tract and metabolism products – enzymes (ATC Code: A16AB10) is a purified recombinant form of the naturally occurring human lysosomal enzyme glucocerebrosidase that cleaves glucocerebroside to glucose and ceramide. It was developed as a long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.

The benefits with VPRIV are its ability to manage the symptoms of Gaucher disease, such as that it increases haemoglobin and platelet count, and decreases in liver and spleen volumes in patients with type 1 Gaucher disease. The most common side effects are infusion related adverse events, e.g. headache, dizziness and nausea.

A pharmacovigilance plan for VPRIV will be implemented as part of the marketing authorisation.

The approved indication is: “VPRIV is indicated for long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease”. It is proposed that VPRIV treatment should be supervised by a physician experienced in the management of patients with Gaucher disease. Home administration under the supervision of a healthcare professional may be considered only for patients who have received at least three infusions and were tolerating their infusions well.

1 Summaries of positive opinion are published without prejudice to the commission decision, which will normally be issued 67 days from adoption of the opinion.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7<sector fax>
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SPC), which will be published in the European public assessment report (EPAR), and will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit to risk balance for VPRIV and therefore recommends the granting of the marketing authorisation.