Study of Velaglucerase Alfa Enzyme Replacement Therapy in Japanese Patients With Gaucher Disease

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Gaucher disease is an inherited deficiency of the lysosomal enzyme glucocerebrosidase (GCB) that leads to progressive accumulation of glucocerebroside within macrophages and subsequent tissue and organ damage; typically of the liver, spleen, bone marrow, and brain. The disease has been classified into 3 clinical subtypes based on the presence or absence of neurological symptoms and severity of neurological disease. Type 1 Gaucher disease affects an estimated 30,000 persons worldwide and is the most common. Type 1 Gaucher disease does not involve the central nervous system. Patients with type 2 Gaucher disease present with acute neurological deterioration, which leads to early death. Those with type 3 disease typically display a more sub-acute neurological course, with later onset and slower progression.

The primary objective of this study is to evaluate the safety of every other week dosing of velaglucerase alfa in Japanese patients with Gaucher disease.

Velaglucerase alfa has been developed and approved as an enzyme replacement therapy for Type 1 Gaucher disease.