A Study of Gene-Activated® Human Glucocerebrosidase (GA-GCB) Enzyme Replacement Therapy in Gaucher Disease

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Gaucher disease is a rare lysosomal storage disorder caused by the deficiency of the enzyme glucocerebrosidase (GCB). Due to this deficiency of functional GCB, glucocerebroside accumulates within macrophages leading to cellular engorgement, organomegaly, and organ system dysfunction. The purpose of this study is to evaluate the efficacy of every other week dosing of Gene-Activated® Human Glucocerebrosidase (GA-GCB, velaglucerase alfa) at doses of 45 and 60 U/kg in treatment-naïve patients with type 1 Gaucher disease.