

**Title: A Multicenter Open-Label Treatment Protocol to Observe the Safety of Gene-Activated® Human Glucocerebrosidase (GA-GCB, velaglucerase alfa) Enzyme Replacement Therapy in Newly Diagnosed or Previously Treated (with imiglucerase) Patients with Type 1 Gaucher Disease**

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**Synopsis:**

Gaucher disease is a rare lysosomal storage disorder caused by the deficiency of the enzyme glucocerebrosidase (GCB). Due to the deficiency of functional GCB, glucocerebroside accumulates within macrophages leading to cellular engorgement, organomegaly, and organ system dysfunction.

ERT, has been the cornerstone of treatment for Gaucher disease since the early 1990s and is highly effective in improving many of the clinical manifestations [2]. Data from the International Collaborative Gaucher Group demonstrate that approximately 90% of all patients with Gaucher disease should achieve normal hemoglobin concentration within 2 years of initiation of treatment [6]. Enzyme replacement therapy reduces organomegaly, improves hematological parameters and positively impacts health-related quality of life [6,7].

The purpose of this multi-center study is to observe the safety of velaglucerase alfa in patients with type 1 Gaucher disease who are either treatment naive (newly diagnosed) or who are currently being treated with the Enzyme Replacement Therapy (ERT) imiglucerase.

Up to 500 subjects (age 2 years and older) with a documented diagnosis of type 1 Gaucher disease who are currently treated with imiglucerase as well as treatment naive patients. There is no gender specificity for the disease.