Study of GA-GCB Enzyme Replacement Therapy in Type 1 Gaucher Disease Patients Previously Treated With Imiglucerase

Regional Metabolic Center, Los Angeles, California, United States Children's Hospital Oakland, Oakland, California, United States Emory University, Decatur, Georgia, United States Feinberg School of Medicine, Chicago, Illinois, United States Children's of Minnesota, Minneapolis, Minnesota, United States Children's Mercy Hospital and Clinic, Kansas City, Missouri, United States NYU School of Medicine, New York, New York, United States Cincinatti Children's Hospital, Cincinnati, Ohio, United States Texas Children's Hospital, Houston, Texas, United States Medical Genetics/Pediatrics, Salt Lake City, Utah, United States Children's Hospital of Wisconsin, Milwaukee, Wisconsin, United States Shaare Zedek Medical Center, Jerusalem, , Israel Children's Memorial Health Institute, Warszawa, , Poland Hospital Universitario Miguel Servet, Zaragoza, , Spain The Royal Free Hospital, London, , United Kingdom

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. The purpose of this study is to evaluate the safety and efficacy of every other week dosing of GA-GCB (velaglucerase alfa) in participants with type 1 Gaucher disease who were previously treated with imiglucerase.