

Gaucher disease is an inherited condition affecting fewer than 10,000 people worldwide. People with Gaucher disease do not have enough of an enzyme, acid β -glucosidase (glucocerebrosidase) that breaks down a certain type of fat molecule. As a

result, lipid engorged cells (called Gaucher cells) amass in different parts of the body, primarily the spleen, liver and bone marrow.

Accumulation of Gaucher cells may cause spleen and liver enlargement, anemia, excessive bleeding and bruising, bone disease and a number of other signs and symptoms. The most common form of Gaucher disease, type 1, generally does not affect the brain.