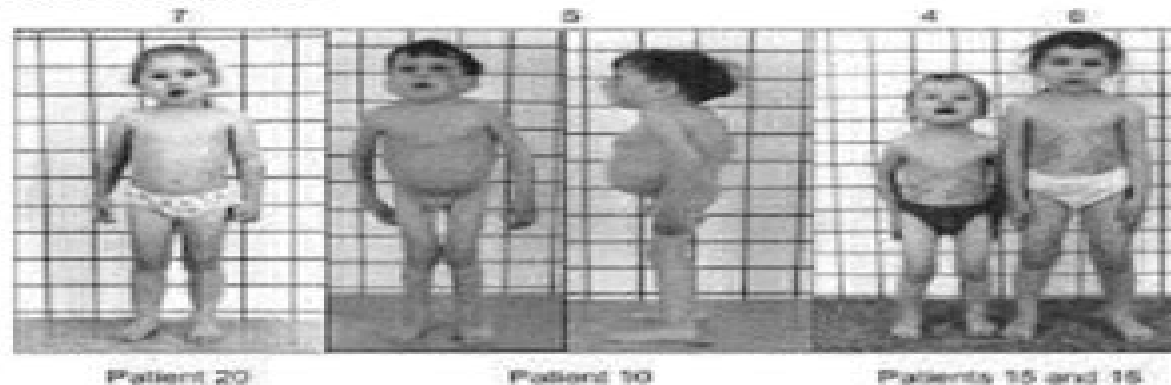


Gaucher Disease



1. What is Gaucher disease?

Gaucher disease is a rare pan-ethnic, lysosomal storage disorder resulting due to beta-Glucosidase (*GBA1*) gene defect. This leads to the glucocerebrosidase enzyme deficiency and an increased accumulation of undegraded glycolipid glucocerebroside inside the cells' lysosomes. To date, nearly 460 mutations have been described in the *GBA1* gene. There is no cure for Gaucher disease, but treatments can relieve symptoms and greatly improve quality of life.

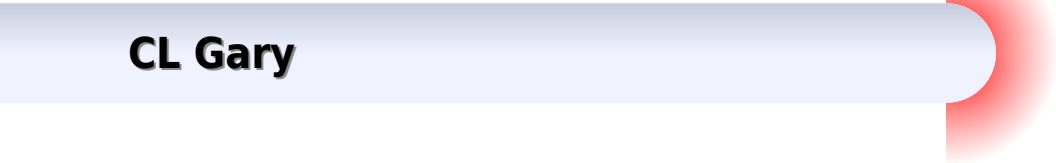
2. Types of Gaucher disease:

There are three types of Gaucher disease (pronounced go-shay). All cause similar symptoms in the organs and bones. Some forms of the disease also affect the brain. The types of Gaucher disease are:

- **Type 1 (Non-neurological):** The most common type in the U.S. Gaucher disease type 1 affects the spleen, liver, blood and bones. It does not affect the brain or spinal cord. Gaucher disease type 1 is treatable, but there is no cure. For some people, symptoms are mild. Other people experience severe bruising,

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