Gene Therapy Approaches for Treatment of Fabry Disease

About Fabry Disease

Fabry disease is a rare, genetic condition which is estimated to affect around 1 in 100,000 people.

In Fabry, an enzyme called α-galactosidase A (α-Gal A) is missing or there is a reduced amount. This means the body cannot break down a certain type of fat called globotriaosylceramide (Gb3). Gb3 continues to build up in body cells causing damage to tissues and organs. Gradually, this leads to a range of physical symptoms and complications, which vary from one person to another.¹

Genes therapy Approaches for Fabry Disease

Liver-Targeted Adeno-Associated Virus (AAV) Gene Therapy

Cardiomyocyte-Targeted Adeno-Associated Virus (AAV) Gene Therapy

Hematopoietic Stem Cell Therapy

New approaches to the treatment of Fabry disease are ongoing. Visit this resource to learn more about clinical trials or support for people living with Fabry disease:

Fabry International Network | fabrynetwork.org

References