

Thalassemia

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Scientists recently tested a gene-editing method called adenine base editing to fix two severe mutations that cause - thalassemia, a genetic blood disease.

About Thalassemia

It is an inherited blood disorder

It affects your body's ability to produce normal hemoglobin

Hemoglobin is a protein in red blood cells (RBCs). It allows your RBCs to transport oxygen throughout your body.

If yohave thalassemia, your body produces fewer healthy hemoglobin proteins, and your bone marrow produces fewer healthy RBCs

The condition of having fewer RBCs is called anemia. That can make yofeel tired and weak.

As RBCs serve the vital role of delivering oxygen to tissues in your body, not having enough healthy RBCs can deprive your body's cells of the oxygen they need to make energy and thrive.

Thalassaemia is caused by inheriting a gene mutation (change in the normal DNA) from one or both parents.

Traits for thalassemia are more common in people from Mediterranean countries, like Greece and Turkey, and in people from Asia, Africa, and the Middle East

There are different types of thalassemia. The type someone has depends on which gene mutation they inherit.

Symptoms: Thalassemia can cause mild or severe anemia and other complications over time (such as iron overload)

Symptoms of Anemia Include:

1Fatigue.

2Trouble breathing.

3Feeling cold.

4Dizziness.

5Pale skin.

6Severe thalassemia may cause death.

Treatments:

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Plot A P.127, AF block, 6 th street, 11th Main Rd, Shanthi Colony, Anna Nagar, Chennai, Tamil Nadu 600040 Phone: **044 4353 9988 / 98403 94477** / Whatsapp : **09710729833**

Blood transfusions – regular blood transfusions treat and prevent anaemia; in severe cases these are needed around once a month.

Chelation therapy – treatment with medicine to remove the excess iron from the body that builds up as a result of having regular blood transfusions.

The only possible cure for thalassemia is a stem cell or bone marrow transplant, but this is not done very often because of the risks involved

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