

# Casgevy

By IASToppers | 2023-11-28 15:25:00



## Casgevy

The UK drug regulator recently approved Casgevy, a groundbreaking gene therapy for <u>sickle cell disease</u> and thalassaemia using Crispr-Cas9 technology.



[Ref: pharmaceutical-technology]

# What is Casgevy?

- Casgevy therapy represents the first licensed use of Crispr-Cas9, a gene editing technology awarded the Nobel Prize in 2020.
- The technology, often referred to as **"genetic scissors,"** has revolutionized biotechnology since its emergence in 2012.

#### **How Does it Work?**

- Casgevy works by editing the faulty gene responsible for these blood disorders.
- It targets the **BCL11A gene**, key in the transition from foetal to adult haemoglobin.
- It increases **production of foetal haemoglobin**, which doesn't have the abnormalities of adult haemoglobin, thus alleviating symptoms.

#### **Procedure**

- Casgevy involves a one-time treatment.
- Blood stem cells are collected and edited over six months, followed by a transplant.

#### **Side Effects:**

 Similar to those associated with autologous stem cell transplants, including nausea, fatigue, fever, and infection risk.

#### **Challenges:**

- High Cost
- Lack of local manufacturing facilities



### About Sickle Cell Disease:

- It causes red blood cells to become **crescent-shaped**, leading to **blocked blood flow, severe** pain, infections, anaemia, or stroke.
- An estimated **30,000-40,000 children** are born with this disorder annually in India.
- Inheritance pattern: Symptoms manifest in individuals inheriting a pair of damaged genes; carriers with one gene generally lead a normal life.

### About Thalassaemia:

- Thalassaemia results in **low haemoglobin levels**, causing fatigue, breathlessness, and irregular heartbeats.
- Continuous blood transfusions are required, leading to excess iron accumulation in the body.
- India has the largest number of children with thalassaemia major globally, **approximately 1-1.5** lakh.