Casgevy

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

Casgevy

The UK drug regulator recently approved Casgevy, a groundbreaking gene therapy for sickle cell disease 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.**

