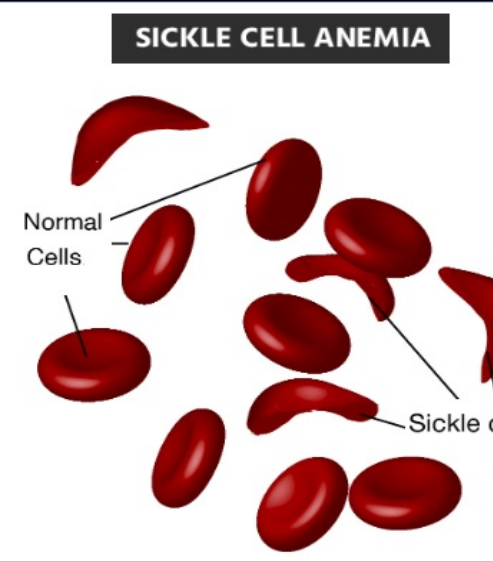
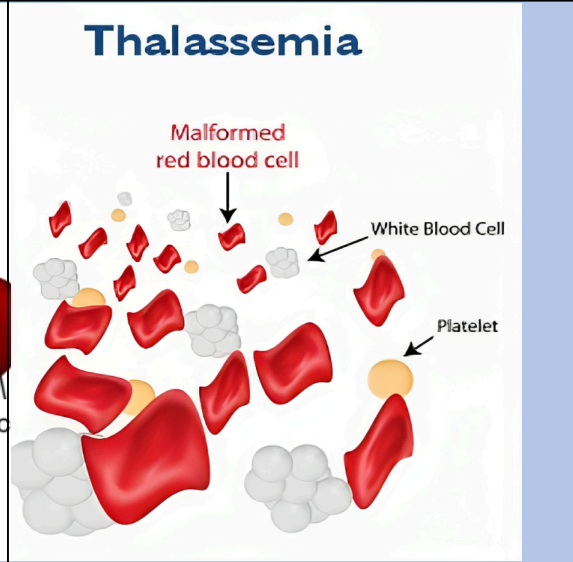


## Gene Therapy for Sickle Cell Anaemia

### Why in news?

The first therapy based on gene editing technology Crispr-Cas9 for [sickle cell disease](#) and thalassaemia has been approved in UK.

### What is sickle cell anaemia and thalassaemia?

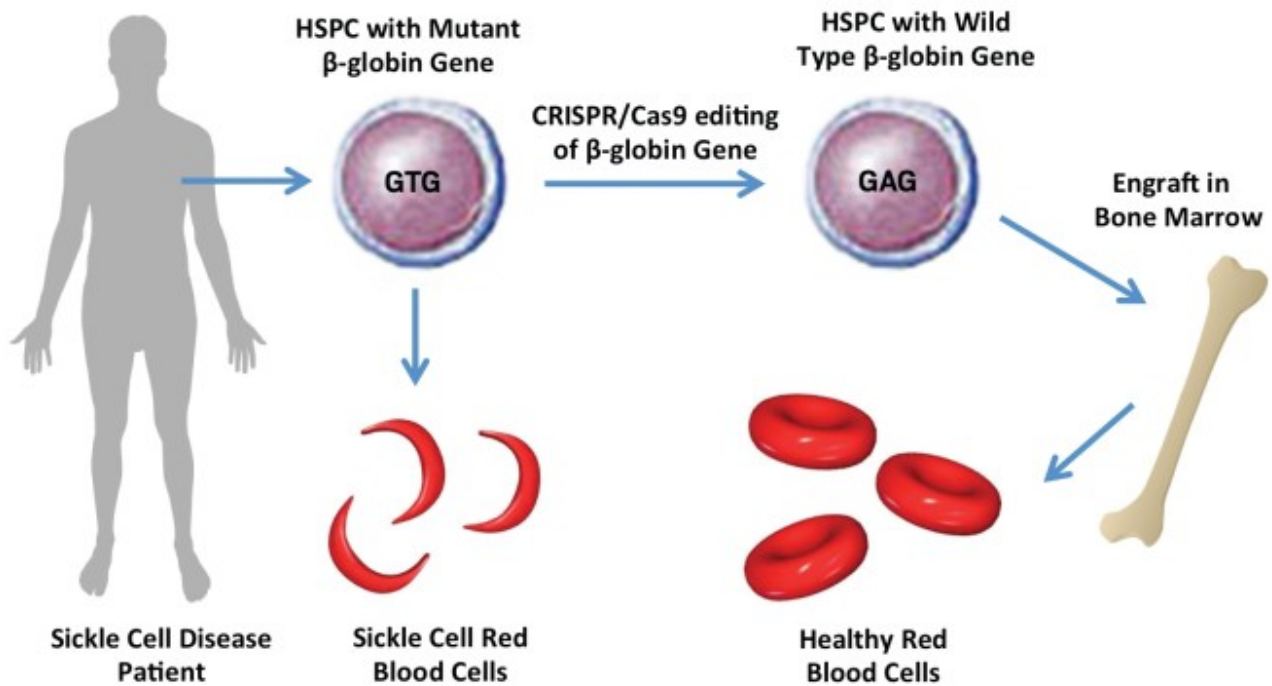
About	Sickle Cell Anaemia	Thalassaemia
<b>Disease</b>	An inherited blood disorder where people who inherit a pair of genes from both parents experience symptoms like severe anaemia.	
<b>Effect on haemoglobin chain</b>	Caused by a mutation in the haemoglobin- $\beta$ gene found on <b>Chromosome 11</b> affecting <i>only the beta chain</i>	Production of <i>either the alpha or beta chains</i> is reduced resulting in either alpha-thalassaemia or beta-thalassaemia
<b>Haemoglobin production</b>	Mutation in haemoglobin chains makes them into a <i>crenate shape</i> under low oxygen level	Caused by reduced production of haemoglobin chains
<b>Effects</b>	Pain, fever, infection, stroke and organ damage	Fatigue, shortness of breath, irregular heartbeats and need blood transfusions throughout their life
<b>Status in India</b>	An estimated 30,000-40,000 children in India are born with the disorder every year.	India has the largest number of children with thalassaemia (about 1-1.5 lakh).
	 <p><b>SICKLE CELL ANEMIA</b></p> <p>Normal Cells</p> <p>Sickle c</p>	 <p><b>Thalassaemia</b></p> <p>Malformed red blood cell</p> <p>White Blood Cell</p> <p>Platelet</p>
<b>Treatment</b>	Treated by blood transfusions, iron supplements, or stem cell transplants.	

## GLOBAL BURDEN OF SICKLE CELL DISEASE



### What is Casgevy?

- Casgevy is the 1<sup>st</sup> licensed therapy in the world based on the gene editing technology [Crispr-Cas9](#).
- **Apheresis** - It is a *one-time treatment* for which the doctor has to first collect blood stem cells from the bone marrow using a process called **apheresis** (filtering out the blood for different components).
- The cells are then sent to the manufacturing site where it takes about 6 months for them to be edited and tested.
- **Gene editing** - The therapy uses the patient's own blood stem cells, which are precisely edited using **Crispr-Cas9**.
  - So far, the only permanent treatment has been a bone marrow transplant, for which a closely matched donor is needed.
- A gene called **BCL11A**, which is crucial for switching from foetal to adult haemoglobin, is targeted by the therapy.
  - Foetal haemoglobin (naturally present in everyone at birth), does not carry the same abnormalities as adult haemoglobin.
- The therapy uses the body's own mechanisms to start producing more of foetal haemoglobin, alleviating the symptoms of the two conditions.
- **Side effects**- They are similar to those associated with autologous stem cell transplants, including nausea, fatigue, fever and increased risk of infection.



### What are the pros and cons of this treatment?

Significance	Challenges
<b>Efficacy-</b> It restores haemoglobin production and alleviates symptoms in most patients.	<b>Limited authorization-</b> It is currently approved in the UK only and is being reviewed by other regulatory bodies.
<b>Pain reliever-</b> It reduces the need for blood transfusions and pain crises in the patients.	<b>Health inequity-</b> It is expensive, thereby limiting the accessibility in poor countries.
<b>Reliable-</b> No serious safety concerns were reported, but long-term effects are still being monitored.	<b>Inaccuracy-</b> There are concerns with potential off targets effects of CRISPR editing, which could cause unwanted changes in other parts of the genome.

### References

1. [Indian Express- Sickle cell breakthrough](#)
2. [Live Science- World's first CRISPR therapy has been approved](#)