



Casgevy Therapy for Sickle Cell Disease and Thalassaemia

[Source: IE](#)

Why in News?

Recently, the **UK Drug Regulator** sanctioned a [gene therapy](#) called **Casgevy** heralded as a significant breakthrough for **treating [sickle cell disease](#) and [thalassaemia](#)**.

- Notably, this marks the **world's inaugural licensed therapy leveraging the CRISPR-Cas9 gene editing technology** that earned its innovators a [Nobel Prize in Chemistry 2020](#).

How does the Casgevy Therapy Work?

- Both **sickle cell disease and thalassaemia** are caused by **errors in the gene for [haemoglobin\(Hb\)](#)**, a protein in the red blood cells that carry oxygen to organs and tissues.
 - The therapy **uses the patient's own blood stem cells**, which are precisely **edited using [CRISPR-Cas9](#)**.
 - A gene called **BCL11A**, which is crucial for switching from **foetal to adult haemoglobin**, is targeted by the therapy.
- **Foetal haemoglobin**, which is 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 this foetal haemoglobin**, alleviating the symptoms of the two conditions.
- Casgevy involves a single treatment wherein **blood stem cells are extracted via apheresis** and then edited over approximately six months before being reintroduced into the patient.
 - Apheresis is a medical procedure that involves removing specific components from blood and returning the rest to the body.

What are Sickle Cell Disease and Thalassaemia?

- **Sickle Cell Disease:**
 - **About:** Sickle cell disease is a genetic blood disorder characterized by an abnormality in hemoglobin, the **protein responsible for carrying oxygen in red blood cells**.
 - It causes **red blood cells to adopt a sickle or crescent shape**, hindering their movement through vessels, leading to potential complications like **severe pain, infections, anaemia, and strokes**.
 - In India alone, an estimated **30,000-40,000 children** are born with sickle cell disease annually.
 - **Types:** It encompasses various types, each dependent on the **inherited genes from parents**, all encoding abnormal hemoglobin. The most prevalent forms of SCD include:
 - **HbSS (Sickle Cell Anemia):** Individuals **inherit two "S" genes**, one from each parent, resulting in **abnormal hemoglobin "S"**.
 - This type often leads to severe manifestations characterized by **rigid, sickle-shaped red blood cells**.
 - **HbSC:** Inheriting an **"S" gene from one parent and a different abnormal hemoglobin, "C,"** from the other, leads to this milder variant of SCD.
 - **HbS Beta Thalassaemia:** This form arises from **inheriting an "S" gene from one parent and a beta thalassaemia gene from the other**.

- The severity varies based on the type of beta thalassaemia inherited either "**zero**" (**HbS beta0**) or "**plus**" (**HbS beta+**), with the former typically resulting in a severe form and the latter in a milder manifestation.
- **Thalassaemia:** Similar to **sickle cell disease**, individuals with **thalassaemia experience severe anaemia due to low haemoglobin levels**, necessitating lifelong blood transfusions and **chelation therapy** to manage iron accumulation.
- Major symptoms include **fatigue, paleness or jaundice, shortness of breath, delayed growth, facial bone deformities (in severe cases)** among others.

Note

Chelation therapy is a proven treatment for **heavy metal poisoning**. It uses substances that bind to **heavy metals and help clear them from the body**.

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GLOBAL BURDEN OF SICKLE CELL DISEASE



INHERITANCE OF SICKLE CELL

PARENTS

Sickle Cell Trait (Carrier father)



Sickle Cell Trait (Carrier mother)

CHILDREN

No Sickle Cell



Sickle Cell Trait (Carrier children)



Sickle Cell Anaemia

Normal haemoglobin A gene

Sickle haemoglobin S gene

ABOVE, each parent has one normal haemoglobin A gene and one haemoglobin S gene. This means each of their children has:

25% CHANCE of inheriting two normal A genes. This child will not have either sickle cell trait or sickle cell disease

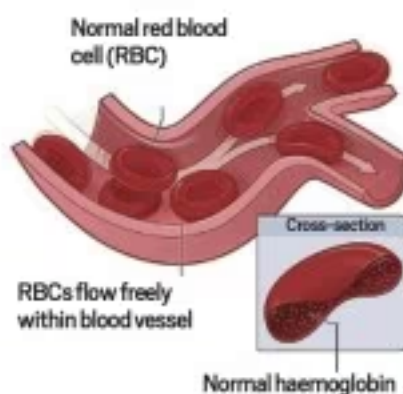
50% CHANCE of inheriting one normal A gene and one S gene. This child will have sickle cell trait, and be a carrier

25% CHANCE of inheriting two S genes. This child will have sickle cell disease

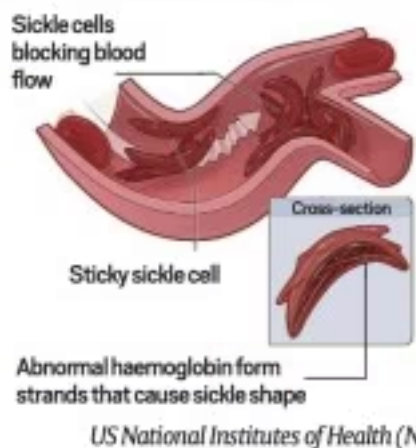
EACH TIME the couple has a child, chances of the child having sickle cell disease remain same

IMPACT ON RED BLOOD CELLS

Normal red blood cells



Abnormal, sickled, RBC (sickle cells)



Note

The [National Sickle Cell Anemia Eradication Mission](#) in India targets the elimination of sickle cell anemia by 2047.

What is CRISPR-Cas9 Technology?

- **CRISPR-Cas9** is a groundbreaking technology that empowers geneticists and medical researchers to modify specific portions of the genome.



- This is achieved through the **precise removal, addition, or modification of segments within the [DNA sequence](#)**.
- It involves two essential parts for editing DNA. First, there is **Cas9**, which acts like **molecular scissors**, cutting DNA at specific spots.
 - Then, there is **guide RNA (gRNA)**, containing a designed sequence. This sequence guides Cas9 to the exact spot in the genome to make the cut.
 - This **precise guidance ensures Cas9 works accurately where needed**, allowing for specific changes in the DNA.

UPSC Civil Services Examination, Previous Year Question (PYQ)

Prelims

Q. What is Cas9 protein that is often mentioned in news? (2019)

- (a) A molecular scissors used in targeted gene editing
- (b) A biosensor used in the accurate detection of pathogens in patients
- (c) A gene that makes plants pest-resistant
- (d) A herbicidal substance synthesized in genetically modified crops

Ans: (a)

Mains

Q. What are the research and developmental achievements in applied biotechnology? How will these achievements help to uplift the poorer sections of society? (2021)

Global Energy Monitor's Global Coal Plant Tracker

For Prelims: [Global Energy Monitor](#), GEM's Global Coal Plant Tracker, Status of coal power projects worldwide.

For Mains: Global Energy Monitor, Distribution of key natural resources across the world (including South Asia and the Indian subcontinent).

Source: [DTE](#)

Why in News?

Recently, [Global Energy Monitor \(GEM\)](#), a not-for-profit cataloging coal projects worldwide, has released its quarterly update of **GEM's Global Coal Plant Tracker**, highlighting several key findings regarding the **status of coal power projects worldwide**.

What are the Key Findings of the GEM Report?

- **Global Trends in Coal Construction:**
 - More than 95% of coal plant capacity beginning construction in 2023 is in China, showcasing a dominance in new coal projects.
 - A decline is observed in new coal power capacity construction for the **second consecutive year**, signaling a shift away from coal in many regions.
- **Coal Capacity Under Consideration:**
 - 110 GW of coal power capacity is under consideration in 32 countries, indicating a significant amount of [Coal](#) projects are still being deliberated.
 - India, Bangladesh, and Indonesia lead, **comprising 83% of the proposed coal capacity outside China.**
- **Trends in Project Status:**
 - 18.3 GW of coal capacity **moved from proposed to shelved or canceled status** in the first nine months of 2023 across several countries.
 - Despite cancellations, 15.3 GW of entirely new **proposals emerged in India**, Indonesia, Kazakhstan, and Mongolia.
 - India, Indonesia, Bangladesh, and Vietnam represent 84% of the 67 GW of coal power capacity under construction outside China as of July 2023.
- **Indian Scenario:**
 - India plans to increase coal-fired power plant capacity significantly by 2032, aiming for **80 GW compared to the previously stated 27 GW in the National Electricity Plan 2022-32 (NEP).**
 - Specific states in India have seen advancements in coal plant projects, with permits granted and progress reported in states like Chhattisgarh, Gujarat, Jharkhand, Madhya Pradesh, Odisha, and Uttar Pradesh.
- **Recommendations:**
 - Amidst global efforts to combat climate change, the report emphasizes the urgent need to cease the construction of new unabated coal power plants to limit global warming effectively.

What is Global Energy Monitor (GEM)?

- **About:**
 - GEM develops and shares **information in support of the worldwide movement for clean energy.**
 - By studying the evolving international energy landscape and creating databases, reports, and interactive tools that enhance understanding, **GEM seeks to build an open guide to the world's energy system.**
 - Users of GEM's data and reports include the [International Energy Agency](#), [United Nations Environment Programme](#), the [World Bank](#), and the Bloomberg Global Coal Countdown.
- **Global Coal Plant Tracker:**
 - It is an online database that identifies and **maps every known coal-fired generating unit** and every new unit proposed since 2010 (30 megawatts and larger).
 - Developed by GEM the tracker uses footnoted wiki pages to document each plant and is updated biannually, around January and July.

What is Coal?

- **About:**
 - It is a type of fossil fuel found in a form of **sedimentary rocks and is often known as 'Black Gold'.**
 - It is a conventional source of energy and is widely available. It is used as a **domestic fuel**, in industries such as iron and steel, steam engines and to generate electricity. Electricity from coal is called thermal power.
 - The leading coal producers of the world include China, US, Australia, Indonesia, India.
- **Distribution of Coal in India:**

- **Gondwana Coal Fields (250 million years old):**
 - Gondwana coal makes **up to 98 % of the total reserves and 99 %** of the production of coal in India.
 - Gondwana coal forms India's metallurgical grade as well as superior quality coal.
 - It is found in Damodar (Jharkand-West Bengal), Mahanadi (Chhattisgarh-Odisha), Godavari (Maharashtra), and Narmada valleys.
- **Tertiary Coal Fields (15 - 60 million years old):**
 - Carbon content is very low but is rich in moisture and sulphur.
 - Tertiary coalfields are **mainly confined to extra-peninsular regions**
 - Important areas include Assam, Meghalaya, Nagaland, Arunachal Pradesh, Jammu and Kashmir, Himalayan foothills of Darjeeling in West Bengal, Rajasthan, Uttar Pradesh, and Kerala.
- **Classification:**
 - **Anthracite** (80 - 95% carbon content, found in small quantities in J&K).
 - **Bituminous** (60 - 80% of carbon content and is found in Jharkhand, West Bengal, Odisha, Chhattisgarh and Madhya Pradesh).
 - **Lignite** (40 to 55% carbon content, high moisture content and is found in Rajasthan, Lakhimpur (Assam) and Tamil Nadu).
 - **Peat** (less than 40% carbon content and it is in the first stage of transformation from organic matter (wood) to coal).

UPSC Civil Services Examination Previous Year Question (PYQ)

Prelims

Q1. Consider the following statements: (2019)

1. Coal sector was nationalized by the Government of India under Indira Gandhi.
2. Now, coal blocks are allocated on lottery basis.
3. Till recently, India imported coal to meet the shortages of domestic supply, but now India is self-sufficient in coal production.

Which of the statements given above is/are correct?

- (a) 1 only
- (b) 2 and 3 only
- (c) 3 only
- (d) 1, 2 and 3

Ans: (a)

Exp:

- Coal sector was nationalised in two phases under Indira Gandhi Government in 1972. Hence, statement 1 is correct.
- The coal blocks are allocated through auctions and not on a lottery basis. Hence, statement 2 is not correct.
- The coal sector is the monopolistic sector in India. India holds 5th biggest coal reserves in the world, but due to the incapacity of coal production by monopolistic firms, it imports coal to meet the shortages of domestic supply. Hence, statement 3 is not correct.
- Therefore, option (a) is the correct answer.

Q2. Which of the following is/are the characteristic/characteristics of Indian coal? (2013)

1. High ash content
2. Low sulphur content
3. Low ash fusion temperature

Select the correct answer using the codes given below:

- (a) 1 and 2 only
- (b) 2 only
- (c) 1 and 3 only
- (d) 1, 2 and 3

Ans: (a)

Mains

Q. Despite India being one of the countries of Gondwanaland, its mining industry contributes much less to its Gross Domestic Product (GDP) in percentage. Discuss. **(2021)**

Q. “In spite of adverse environmental impact, coal mining is still inevitable for development”. Discuss. **(2017)**

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