



## Gene Therapy for Hemophilia A

**For Prelims:** [National Science Day](#) , [Hemophilia A](#), [DNA technology](#), [Raman Effect](#), [Global Innovation Index](#), [Maya OS](#) , [Chandrayaan-3 Mission](#), [Gaganyaan mission](#).

**For Mains:** Gene Therapy for hemophilia A, India's Achievements in Science and Technology.

[Source:PIB](#)

### Why in News?

Recently, the Union Minister of Science & Technology addressing the [National Science Day 2024](#) programme stated that India conducted its first human clinical trial of gene therapy for [hemophilia A \(FVIII deficiency\)](#) at Christian Medical College (CMC) Vellore.

- India's progress in [science and technology \(S&T\)](#) was also highlighted at the event.

### What is Hemophilia A?

- About:** Hemophilia is a group of **rare bleeding disorders** caused by a congenital deficiency in specific clotting factors. The most prevalent form is **Hemophilia A**.
  - Hemophilia A** results from a deficiency in a crucial blood clotting protein known as **factor VIII**.
  - Due to this deficiency, individuals experience prolonged bleeding after injuries, as their blood takes longer to clot than usual.
- Causes:** It is primarily inherited ([genetic](#)) and follows an **X-linked recessive pattern**, meaning the gene responsible for **factor VIII production** is located on the X chromosome.
  - Males have **one X and one Y chromosome**, while females have two X chromosomes.
    - If a male inherits an X chromosome with the defective gene from his mother, he will have hemophilia A.
    - Females with one defective copy typically do not experience symptoms because the other X chromosome usually provides enough factor VIII.
    - However, females can have hemophilia A if they **inherit two defective copies**, one from each parent (much less common).
- Symptoms:** The severity of hemophilia A varies depending on the level of factor VIII activity in the blood. Common symptoms can include:
  - Easy bruising** and excessive bleeding from minor injuries (cuts, scrapes)
  - Bleeding in the joints (especially knees, elbows, and ankles), causing pain, swelling, and stiffness
  - Bleeding after surgery or dental procedures.
- Treatment:** The treatment involves replacing the missing blood clotting factor so that the blood can clot properly. This is typically done by injecting treatment products, called **clotting factor concentrates**, into a person's vein. The two main types of clotting factor concentrates available are:
  - Plasma-derived Factor Concentrates:** Derived from human plasma, which is the liquid

component of blood containing various proteins, including clotting factors.

- **Recombinant Factor Concentrates:** Introduced in 1992, recombinant factor concentrates are **genetically engineered using DNA technology** and do not rely on human plasma.
  - They are free from plasma or albumin, eliminating the risk of transmitting bloodborne viruses.
- However, gene therapy is now gaining prominence.
  - In recent trials, they used a new method that involves using a special type of virus called a **lentiviral vector to insert a gene that produces FVIII** into the patient's own stem cells.
  - These modified stem cells then produce FVIII when they develop into specific types of blood cells.
- **Acquired Hemophilia A:** While Hemophilia A is typically inherited, it can also be acquired later in life as a **result of auto-antibodies targeting factor VIII**.
  - This condition, known as acquired hemophilia A, is **rare** and differs from the congenital form in its onset and progression.

## Note

**World Haemophilia day** is celebrated on **17<sup>th</sup> April every year**, aiming to increase awareness about hemophilia and other inherited bleeding disorders. The day is celebrated in the honor of **Frank Schnabel**, founder of the World Federation of Haemophilia (WHF).

## What is National Science Day?

- The National Science Day is celebrated on **28<sup>th</sup> February** every year to commemorate the discovery of '**Raman Effect**' in 1928 by **Sir Chandrasekhara Venkata Raman**, which led to the **Nobel Prize being awarded to him in 1930**.
  - **Raman effect** is a method for identifying materials **based on how they scatter light**.
  - By shining light on a substance, scientists can analyze the unique way it interacts with molecules, revealing its chemical composition and structure.
- The purpose of celebrating this day is to **enhance scientific temper, popularization of science** and encouraging innovative activities by infusing scientific temperament in the masses and creating a positive scientific research culture.
  - Theme for National Science Day 2024: '**Indigenous Technologies for Viksit Bharat.**'

## What are India's Recent Advancements in Science and Technology?

- India has emerged as the **world's third-largest startup ecosystem**, boasting over 100 unicorns, showcasing remarkable entrepreneurial growth.
- The **bio-economy sector** has witnessed an extraordinary **13-fold increase** in the past decade, reaching a staggering USD 130 billion in 2024.
- India ranks among the **top five countries for scientific research publications** and holds the **40th position in the Global Innovation Index (GII)**, highlighting its commitment to innovation.
- Groundbreaking initiatives like the **Aroma Mission** and **Purple Revolution** have revolutionized agriculture, fostering a thriving community of agri-startups.
- The development of **Maya OS** by the **Indian Defence Research and Development Organisation** has bolstered cybersecurity measures, protecting against online threats from foreign entities.
- India's intellectual property landscape is experiencing a surge, with **patent filings surpassing 90,000**, marking the highest in two decades.
- The successful **Chandrayaan-3 Mission** has showcased India's prowess in space exploration, paving the way for the historic **Gaganyaan mission**.
- **Related Government Initiatives:**
  - **India Semiconductor Mission**

- [Artificial Intelligence Mission](#)
- [Unified Payment Interface](#)
- [INS Vikrant](#)
- [Bharat 6G project](#)
- [Initiative on Critical and Emerging Technology \(India and US\)](#)

### UPSC Civil Services Examination, Previous Year Question (PYQ)

**Q.** How is science interwoven deeply with our lives? What are the striking changes in agriculture triggered by science-based technologies? **(2020)**

PDF Reference URL: <https://www.drishtiias.com/printpdf/gene-therapy-for-hemophilia-a>

