Gene Therapy Treatment for Haemophilia

Source: TH

Indian scientists have developed a novel **gene therapy** **for haemophilia A**, offering a **one-time treatment to replace frequent clotting factor injections**.

- In a trial at Christian Medical College, Vellore, five patients have been free of bleeding episodes for over a year.
- Haemophilia A is a **genetic disorder** caused by **insufficient Factor VIII,** preventing **proper blood clotting.** India, with 40,000 to 100,000 affected patients, has the second-largest haemophilia population globally.
    - Haemophilia A is inherited in an **X-linked recessive pattern.** Males with a **defective X chromosome have hemophilia,** while females **need two defective X chromosomes to be affected.**
    - Current treatments are lifelong and expensive, costing up to Rs 2.54 crore over ten years, making gene therapy a cost-effective alternative.
- **Gene therapy replaces defective genes in a patient's cells with healthy ones.**
- **Roctavian,** the only USFood and Drug Administration approved gene therapy, uses an **adenovirus vector to deliver a gene for Factor VIII production** in the liver but is **not approved for children.**
- The Vellore trial used a **lentivirus vector,** considered **safer and potentially suitable for children,** offering new possibilities for gene therapy in resource-constrained settings.

Read more: World Haemophilia Day