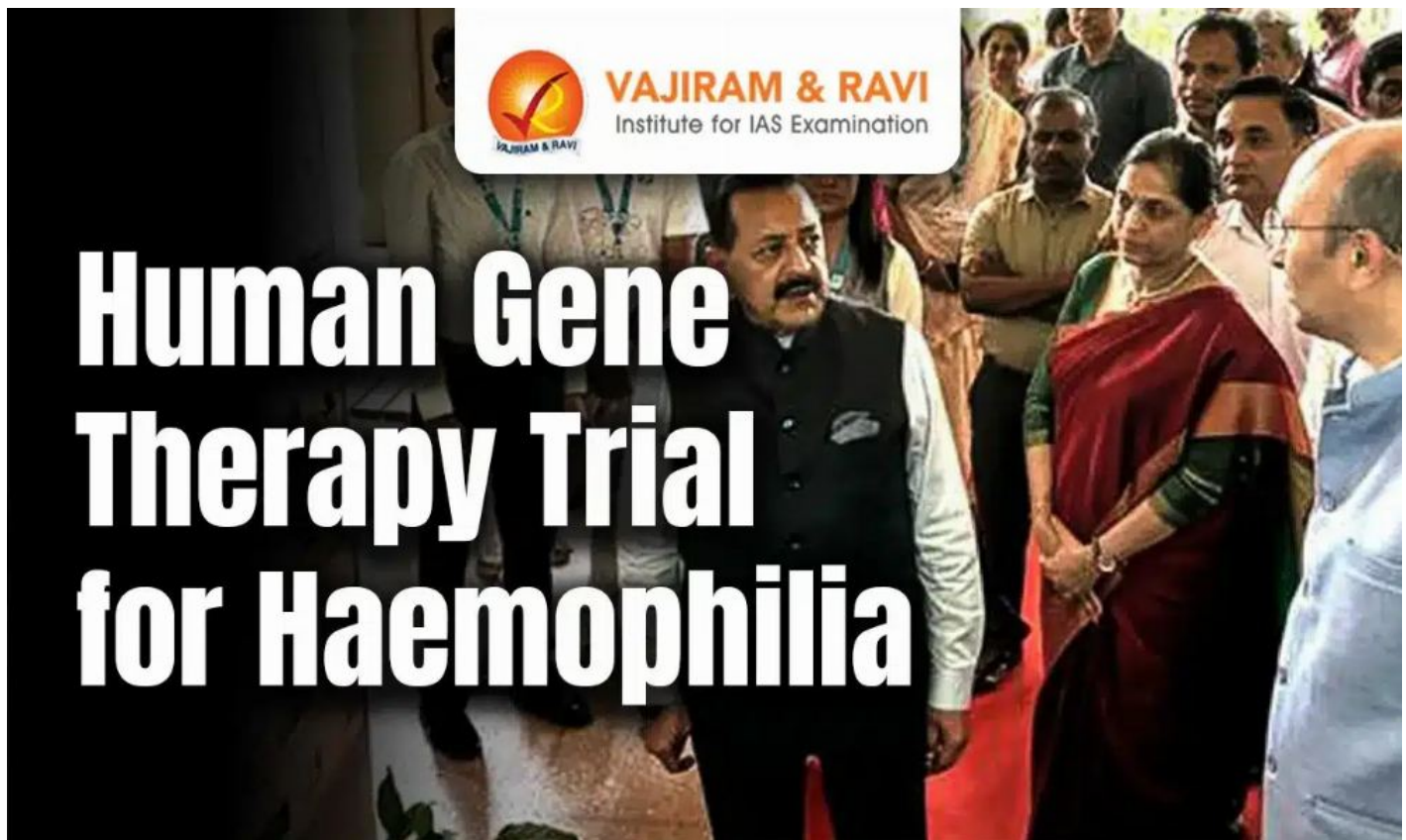


Human Gene Therapy Trial for Haemophilia

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Human Gene Therapy Trial for Haemophilia Latest News

BRIC-inStem, Bengaluru, has successfully conducted India's first-in-human gene therapy trial for Haemophilia in collaboration with CMC Vellore.

About Gene Therapy

- **Gene therapy** is a **biomedical technique** that involves **modifying or replacing faulty genes** in a person's cells to **treat or prevent diseases**.
- The primary objective is to **introduce a functional gene** to compensate for a **mutated or disease-causing gene**.
- Gene therapy approaches include:
 - **Replacing** a mutated gene with a **healthy copy**.
 - **Inactivating** a malfunctioning gene.
 - **Introducing** a completely new gene into the body.

- Unlike conventional medicines, **gene therapy targets the root genetic causes inside cells**, rather than just addressing symptoms.
- **Current clinical trials** focus on both **inherited and acquired disorders**, using methods such as:
 - **Ex vivo** modification of **hematopoietic stem cells** and **T-lymphocytes**.
 - **In vivo** gene delivery or use of **gene-editing reagents** directly into the patient's body.

About Haemophilia

- **Haemophilia** is a **rare genetic bleeding disorder** where **blood clotting is impaired** due to **mutations in genes encoding clotting proteins**.
- These genes are located on the **X chromosome**, making **males more susceptible**.
- It affects **about 1 in 10,000 people**, with **India** having a significant patient load.

About BRIC-inStem

- **BRIC-inStem**, part of the **Biotechnology Research and Innovation Council (BRIC)**, integrates 14 autonomous research institutions under one national umbrella.
- It has pioneered **translational and regenerative research**, including:
 - **Gene therapy**
 - **Anti-viral germicidal masks** (developed during COVID-19)
 - **'Kisan Kavach' pesticide shield** for farmers
- **Biosafety Level III Lab** at BRIC-inStem is essential for studying **high-risk pathogens** under the **One Health Mission**.

Source: **PIB**

Source: <https://vajiramandravi.com/current-affairs/human-gene-therapy-trial-for-haemophilia/>

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