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 - About Hemophilia A:
 - Here's a breakdown:
 - QuizTime:
 - Are you Ready!
- Read the Below Instructions Carefully:
 - Please Rate!
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 - Question 2:
 - Model Answer:
 - Relevance to the UPSC Prelims and Mains syllabus under the following topics:

What's new?

- Union Minister Dr. Jitendra Singh addressing the “National Science Day 2024” programme at Vigyan Bhawan, New Delhi.
- **India has conducted the first human clinical trial of gene therapy for haemophilia A (FVIII deficiency) at Christian Medical College (CMC) Vellore.**
- This was disclosed by Union Science & Technology Minister (Independent Charge) Dr. Jitendra Singh while addressing the “National Science Day 2024” programme in Vigyan Bhavan.

Where is it done?

- Vellore, Christian Medical College.

An important milestone:

- With the completion of the first human gene therapy study for hemophilia A, India made progress in the fight. This innovative research at Christian Medical College (CMC) Vellore gives those with this crippling blood clotting problem great hope.

Understanding Hemophilia A:

- Hemophilia A is a hereditary condition that lacks Factor VIII, a blood-clotting protein. This defect causes uncontrolled bleeding, putting patients at risk. Traditional treatment requires repeated protein infusions, which can stress patients and lower quality of life.

Gene Therapy: A Potential Cure:

- Gene therapy could cure hemophilia A by inserting a functioning Factor VIII gene into stem cells. Modified stem cells can manufacture clotting factor throughout the body, removing the need for infusions. This CMC Vellore experiment used lentiviral vectors to deliver the therapeutic gene, allowing Factor VIII expression to last.

Significance and Next Steps:

- This initial trial's success is critical. The therapy's long-term safety and efficacy need further study with bigger patient groups. For future affordability and accessibility, indigenous lentiviral vector production is essential.

A Collaboration Testament:

- This milestone shows India's medical research strength and the value of partnership. The Department of Biotechnology, Centre for Stem Cell Research, and Emory University collaborated on the trial. Collaborations accelerate gene therapy advances and provide global patient access.

A Better Future:

- This hemophilia A gene treatment could be transformative. It empowers patients to live more active and fulfilling lives by eliminating the possibility of bleeding. This trial gives hope to hemophilia A patients and the gene therapy profession, paving the path for therapies for additional genetic illnesses.

About Hemophilia A:

- Hemophilia A, also known as Factor VIII deficiency, is a genetic disorder that affects the body's ability to clot blood properly.



Here's a breakdown:

- Cause: Factor VIII, a blood-clotting protein, gene mutations cause hemophilia A. The X chromosome contains this gene.
- Disease inheritance: Hemophilia A is X-linked recessive. Females have two X chromosomes, and if one has the faulty gene, the other normally provides enough Factor VIII to prevent clotting. Males have one X chromosome, thus if it has the faulty gene, they develop hemophilia A. Women can carry the disease but rarely have symptoms.
- Symptoms: Hemophilia A causes prolonged bleeding after small injury. Internal bleeding in joints and muscles is also a problem. Patients with hemophilia A may bruise easily and bleed excessively after surgery or dental procedures. How much Factor VIII activity is left affects

symptom severity.

This table summarizes the main points:

Feature	Description
Cause	Mutation in Factor VIII gene
Inheritance	X-linked recessive
Who is affected?	Primarily males
Symptoms	Prolonged bleeding, easy bruising, internal bleeding (joints, muscles)

Treatment: Hemophilia A has no cure, however therapies can reduce bleeding. This includes:

- Factor VIII replacement therapy: This involves intravenous infusions of the missing clotting factor to help control bleeding.
- Medications: Medications like antifibrinolytics can help prevent blood clots from breaking down too quickly.
- Physical therapy: Regular physical therapy can help maintain joint health and function, especially after bleeding episodes.
- Hope for the future: Gene therapy trials, like the one conducted in India, hold promise for a potential cure by introducing a functional Factor

VIII gene into a patient's own cells. This could allow the body to produce the clotting factor on its own, eliminating the need for regular infusions.



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Are you Ready!

Thank you, Time Out !

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General Studies
CURRENT AFFAIRS QUIZ

Read the Below Instructions Carefully:

- Click on - Start Quiz
 - Attempt all questions (You can attempt or leave)
 - After Attempting Last Question.
 - Enter Name & Email
 - Click on - Check Result
 - Scroll down - Check out Solutions too.
- Thank you.

Loading ...



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Category: **General Studies**

Lentiviral vectors are a key component used in the Indian gene therapy trial for Hemophilia A. What is the PRIMARY function of these vectors?

- To stimulate the production of new blood cells.
- To safely deliver the therapeutic gene into the patient's cells.
- To directly replace the missing Factor VIII protein.
- To suppress the immune system's response to the introduced gene.

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Category: **General Studies**

The recent successful human clinical trial for gene therapy in India targeted which of the following diseases?

- Sickle Cell Anemia
- Thalassemia
- Hemophilia A
- Cystic Fibrosis

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Category: General Studies

For wider accessibility of gene therapy for Hemophilia A in India, what is a crucial next step?

- Conducting further trials with larger patient groups.
- Establishing collaborations with international pharmaceutical companies.
- Developing cost-effective methods for producing lentiviral vectors.
- All of the above

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Category: General Studies

Consider the following statements about Hemophilia A:

- I. It is a blood clotting disorder caused by a deficiency in Factor IX.
- II. It is primarily an X-linked recessive genetic disorder.
- III. Individuals with Hemophilia A experience excessive bleeding after minor injuries.

IV. Traditional treatment relies solely on pain management medication.

Which of the statements given above are correct?

- I and II only
- II and III only
- I, II and III only
- I, II, III and IV

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Category: **General Studies**

Which of the following statements BEST describes the significance of the Indian gene therapy trial for Hemophilia A?

- It offers a definitive cure for all types of hemophilia.
- It eliminates the need for blood transfusions in all blood clotting disorders.
- It represents a potential cure for Hemophilia A by enabling the body to produce its own Factor VIII.
- It provides a readily available and affordable treatment option for Hemophilia A patients globally.

Prev

Finish

Check Rank, Result Now and enter correct email as you will get Solutions in the email as well for future use!

Check the Result

Your score is

0%

Restart quiz

Please Rate!

Send feedback

Mains Questions:



**Below Mains
Question**

Write in Comment Section

Question 1:

Explain Hemophilia A, its causes, symptoms, and traditional treatment methods. Discuss the significance of the successful human clinical trial for gene therapy targeting Hemophilia A conducted in India. (250 words)

Model Answer:

Hemophilia A:

- Cause: A genetic mutation in the X chromosome that reduces or eliminates the production of Factor VIII, a protein crucial for blood clotting.
- Symptoms: Prolonged bleeding after injuries, easy bruising, internal bleeding (joints, muscles).

Traditional Treatment:

- Factor VIII replacement therapy via intravenous infusions.
- Medications to prevent blood clot breakdown.
- Physical therapy to maintain joint health.

Significance of the Gene Therapy Trial:

- Offers a potential cure by introducing a functional Factor VIII gene into the patient's own stem cells.
- This allows the body to produce the clotting factor on its own, potentially eliminating the need for regular infusions.
- Represents a significant step forward for hemophilia A treatment and highlights India's growing strength in medical research.

Question 2:

Discuss the ethical considerations and challenges associated with gene therapy,

particularly in the context of the recent Indian trial for Hemophilia A. (250 words)

Model Answer:

Ethical Considerations:

- **Informed consent:** Ensuring participants understand the risks and benefits of a novel therapy like gene therapy.
- **Long-term effects:** The long-term safety and potential side effects of gene therapy are still under investigation.
- **Accessibility and equity:** Ensuring affordability and equitable access to this potentially expensive treatment for all patients who need it.

Challenges:

- **Technical challenges:** Optimizing the delivery and expression of the therapeutic gene.
- **Regulatory hurdles:** Obtaining necessary approvals for clinical trials and widespread use of gene therapy.
- **Cost and infrastructure:** Establishing efficient and affordable production methods for gene therapy vectors.

Remember: These are just sample answers. It's important to further research and refine your responses based on your own understanding and perspective.

Relevance to the UPSC Prelims and Mains syllabus under the following topics:



UPSC Prelims:

▪ **General Science (Paper-I):**

Human body and its health: Diseases and their causes. (This section could potentially touch upon blood-related disorders in a broader sense)

UPSC Mains:

▪ **General Studies III (GS-III):**

Science and Technology – developments and their applications and impact on society. (This section might ask about advancements in medical research and their implications, without specifically mentioning Hemophilia A)

*Click here to read in
Hindi.*

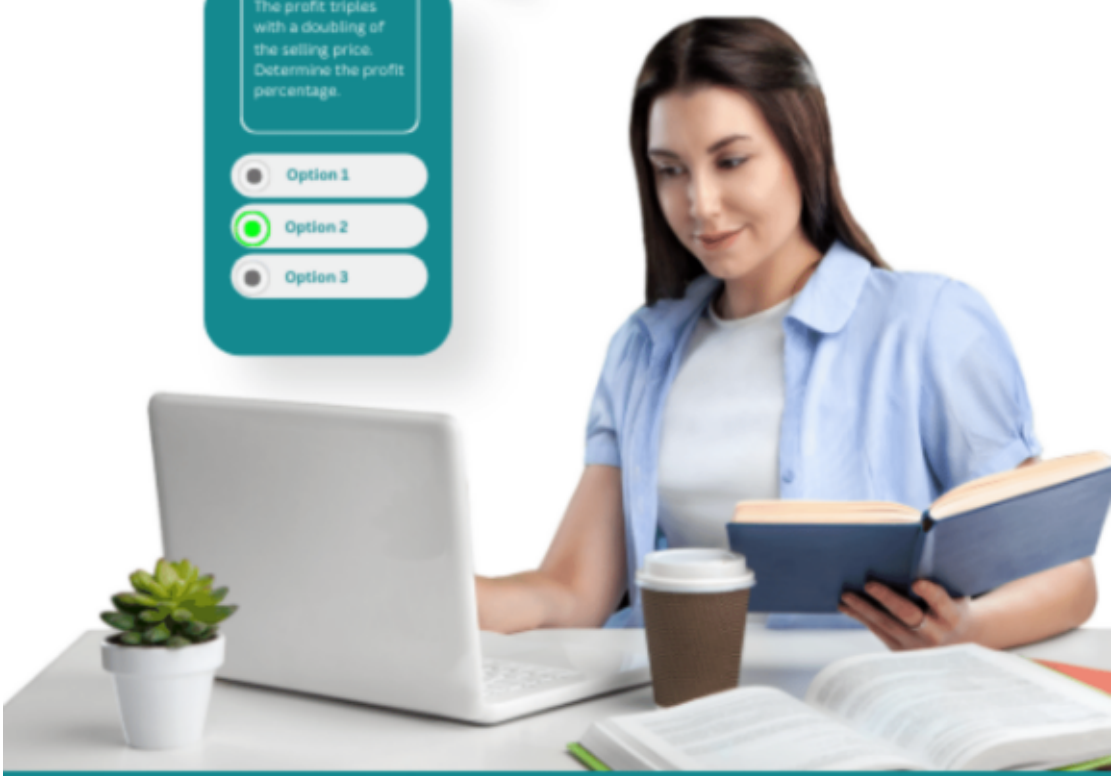
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Quiz Time

The profit triples with a doubling of the selling price. Determine the profit percentage.

- Option 1
- Option 2
- Option 3



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