

MCQ Question · mcqquestion.com

Genetic Medicine: How Gene Therapy Works

Free · Share

Genetic Medicine: How Gene Therapy Works

In November 2025, PIB reported the launch of BIRSA 101, India's first home-made CRISPR gene therapy for sickle cell disease. That news sits inside a bigger idea, genetic medicine.

It treats disease by correcting the faulty gene behind it. It does not rewrite all the DNA. UPSC tested this idea in its 2026 preliminary paper. The provisional answer key gives statements 1 and 2.

At a glance

Four key facts on this topic

01 **Exam answer**Statements 1 and 2, from UPSC CSP 2026, Question 41 (provisional key).

02 **Two carriers**Engineered viruses and lipid nanoparticles.

03 **Two routes**Ex vivo, outside the body. In vivo, inside the body.

04 **India's news**BIRSA 101, reported by PIB on 19 November 2025.

Must Know · Understand the words

1 What genetic medicine is

1.1 What is genetic medicine?

Gene therapy is a medical approach that treats or prevents disease by correcting the underlying genetic problem. MedlinePlus, a US National Library of Medicine (NLM) service, defines it this way. The UPSC question uses the words **genetic medicine**. Treating the two terms as one idea is our own reading.

The NLM site says the instructions for making proteins are carried in a person's genetic code. A variant, or mutation, in this code can change how a protein is made or how it works. Fixing or compensating for the change may let the protein do its job again.

1.2 What are the main ways to fix a gene?

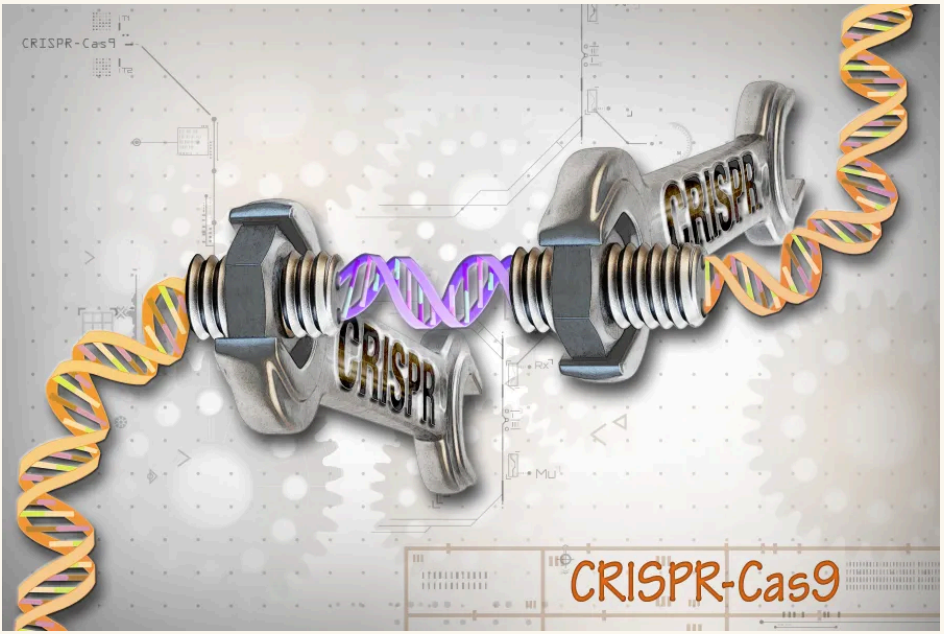
Bulcha and colleagues, in a 2021 review, list four basic approaches.

- **Gene replacement:** delivering a functional gene to replace a non-working gene.
- **Gene silencing:** inactivating a mutated gene that has become toxic to cells.
- **Gene addition:** over-expressing a "foreign" gene to affect how cells work.
- **Gene editing:** a permanent manipulation of a gene in a patient's genome.

The NLM site calls genome editing a newer technique. CRISPR-Cas9 is a well-known type. The NLM site says genome editing is being studied for four jobs.

- Fix a genetic alteration, so the gene can function properly.

- Turn on a gene to help fight a disease.
- Turn off a gene that is functioning improperly.
- Remove a piece of DNA that is impairing gene function and causing disease.



CRISPR-Cas9 Editing of the Genome. The Commons description says CRISPR-Cas9 is a customizable tool. It lets scientists cut and insert small pieces of DNA at precise areas along a DNA strand. Credit given on Commons: Ernesto del Aguila III, NHGRI. Image by the National Human Genome Research Institute via Wikimedia Commons. Licensed CC BY 2.0.

1.3 Does genetic medicine rewrite the whole genome?

No. This is the trap in the UPSC statement. The NLM site says genome editing allows genetic material to be added, removed or altered. This happens at precise locations in the genome. The target is a specific place. It is not the entire DNA sequence.

Here is our own analogy. A faulty gene is like a typing error on one page of a long book. Genetic medicine corrects that page. It does not reprint the whole book.

> 7. **Think it through:** A tool changes one faulty spot in a gene and leaves the rest of the DNA alone. Is that genetic medicine, or is it altering the entire DNA sequence? See answer ↓

> **Quick check 1.** True or false: Genetic medicine rewrites a patient's entire DNA sequence. *(True or False?)* See answer ↓

Sources: medlineplus.gov; pmc.ncbi.nlm.nih.gov

2 Engineered viruses as carriers

2.1 Why does a gene need a carrier?

The NLM site says genetic material or gene-editing tools usually do not work if placed straight into a cell. So a carrier called a **vector** is built to carry and deliver the material.

2.2 Why use a virus as the carrier?

Certain viruses are used as vectors. They can deliver material by infecting the cell. The NLM site says these viruses are modified. They can’t cause disease when used in people. That matches the words **engineered viruses** in the UPSC statement.

In their 2021 review, Bulcha and colleagues call adenoviruses, adeno-associated viruses (**AAV**) and **lentiviruses** the three key vector strategies.

Three viral vectors, as the sources describe them

Vector	What the sources say	Example in the sources
Adenovirus	MedlinePlus: introduces its DNA into the cell nucleus, but the DNA is not integrated into a chromosome.	Bulcha and colleagues name it as one of the three key vector strategies.
AAV	Bulcha and colleagues: its genome forms a stable, persistent episomal form. They report it detected beyond 10 years after administration in non-dividing cells. They say AAVs are accepted as the least immunogenic of the current viral vectors.	Luxturna. The prescribing information calls it an adeno-associated virus vector-based gene therapy.
Lentivirus	Bulcha and colleagues: lentiviral vectors are integrating vectors that permit long-term transgene expression. They list lentiviruses among the retroviruses.	Bulcha and colleagues describe lentiviral vectors used to modify a patient's T cells for cancer therapy, and a patient's CD34+ cells in a beta-thalassaemia study.

The table covers only three vectors. Bulcha and colleagues name them as key. It leaves out other viral vectors and the non-viral carriers in card 3. Episomal means a form that stays apart from the chromosomes; that plain gloss is our own.

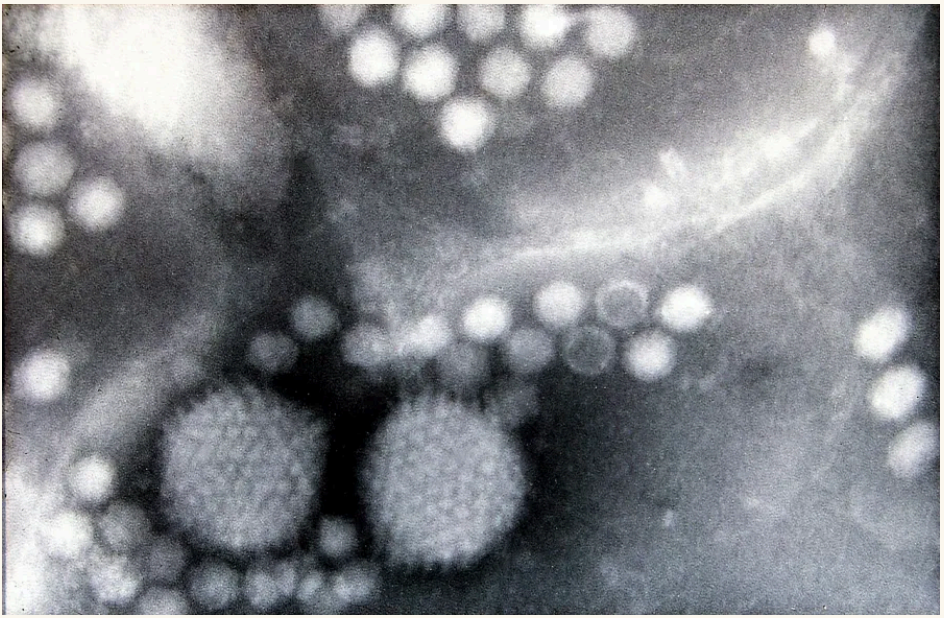
2.3 What are the risks of viral carriers?

The NLM site says gene therapy with viral vectors has been successful. It does carry some risk.

- Sometimes the virus triggers a dangerous immune response.

- Vectors that integrate genetic material into a chromosome can cause errors that lead to cancer.

The NLM site adds that some viruses, such as retroviruses, integrate their genetic material into a chromosome. Others, such as adenoviruses, do not.



Electron micrograph of two adenovirus particles surrounded by numerous, smaller adeno-associated viruses, as the Commons description puts it. Photo by Graham Beards, 20 October 2019, via Wikimedia Commons. Licensed CC BY-SA 4.0.

> **Quick check 2.** True or false: Viruses used as gene-therapy vectors are modified so they cannot cause disease in people. *(True or False?)* See answer ↓

Sources: dailymed.nlm.nih.gov

3 Lipid nanoparticles and mRNA vaccines

3.1 What is a nanoparticle carrier?

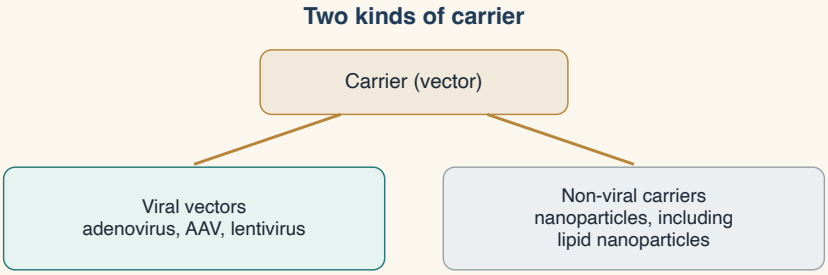
The NLM site says researchers are developing newer technologies. They deliver genetic material or gene-editing tools without using viruses. One technique uses special structures called **nanoparticles** as vectors. The NLM site describes nanoparticles as incredibly small structures.

Duan and colleagues list **lipid nanoparticles (LNPs)** among the nano-delivery systems for CRISPR/Cas9. A lipid is a fatty molecule. That plain gloss is our own.

3.2 Why do researchers like them?

The NLM site says nanoparticles are less likely than viral vectors to cause immune reactions. It also says they are easier to design and modify for specific purposes.

Those are the NLM site's comparisons. It also says gene therapy with viral vectors has been successful.



Original diagram. Groups follow MedlinePlus and Bulcha et al.

Original diagram of the carrier families named in this lesson.

3.3 Are COVID-19 mRNA vaccines the same as genetic medicine?

Verbeke and colleagues write that the COVID-19 mRNA vaccines are made of synthetic mRNA strands. Lipid nanoparticles pack them and deliver them to cells. So LNPs are a carrier used in those vaccines.

But the NLM site says mRNA from vaccines does not enter the nucleus and does not alter DNA. Sharing a carrier is not the same as editing DNA. That link between the two sources is our own reading.

> 8. **Think it through:** A friend says COVID-19 mRNA vaccines edit your DNA because they use lipid nanoparticles. What is wrong with that claim? See answer ↓

> **Quick check 3.** True or false: mRNA from COVID-19 vaccines enters the cell nucleus and alters DNA. *(True or False?)* See answer ↓

Sources: pmc.ncbi.nlm.nih.gov; medlineplus.gov

4 Ex vivo versus in vivo

4.1 What does ex vivo mean?

Ex vivo therapy works outside the body. Bulcha and colleagues describe the steps. A patient's cells are taken out. A vector carrying a therapeutic gene changes them. They are grown in culture. Then they are infused back into the patient.

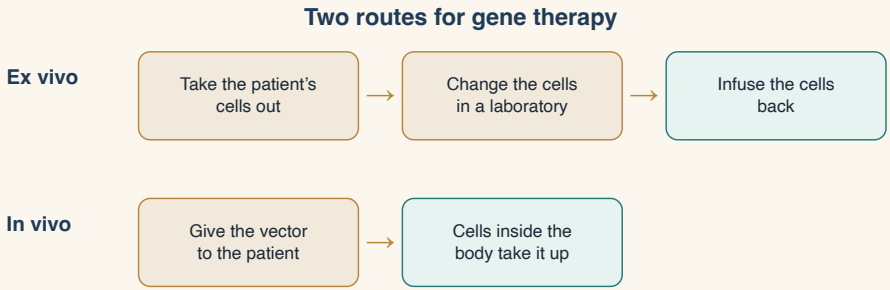
The NLM site describes the same idea. A sample of the patient's cells is removed. In a laboratory, it is exposed to the vector. The cells containing the vector are then returned to the patient.

4.2 What does in vivo mean?

In vivo therapy works inside the body. Bulcha and colleagues say it means giving the vector, which carries a healing gene, straight to the patient.

The NLM site says the vector can be injected, or given by IV, straight into one tissue. There it is taken up by individual cells.

The ex vivo and in vivo labels for named products in this lesson are our own reading of these definitions.



Original diagram of the two routes described in card 4.

4.3 Where does cancer CAR-T therapy fit?

Bulcha and colleagues describe a trial. A patient's own T cells were transduced with lentiviral vectors. The vectors carried a transgene. It makes a chimeric antigen receptor (CAR) that binds CD19. They say this treatment was later sold as Kymriah. The FDA approved it in 2017

as the first cell-based gene therapy, for certain children and young adults with B-cell acute lymphoblastic leukaemia (ALL). It was not the first FDA-approved drug for childhood leukaemia.

The two routes side by side

Point	Ex vivo	In vivo
Where the vector meets the cells	In a laboratory, after cells are removed	Inside the body, after the vector is given
What goes back to the patient	The changed cells	Nothing is returned, because the vector is given directly
Example in this lesson	Casgevy and CAR-T cells (our reading)	Luxturna, given by subretinal injection (our reading)

The table covers only the two routes in the sources above. The last row is our own reading of the definitions. The sources do not use those labels for these products.

> **Quick check 4.** True or false: In ex vivo therapy, a patient's cells are taken out, changed in a laboratory, and returned to the patient. *(True or False?)* See answer ↓

5 Somatic and germline cells

5.1 Which cells do doctors change?

The NLM site says current gene therapy research has focused on body (**somatic**) cells. Examples are bone marrow or blood cells. It says this type of change cannot be passed to a person’s children.

This is the NLM site's wording as of its page update on 28 February 2022.

5.2 What is germline therapy?

Gene therapy could be aimed at egg and sperm cells. The NLM site calls these germ cells. This would let the changes pass to future generations. It calls this **germline gene therapy** and says the idea is controversial.

The National Human Genome Research Institute says germline editing changes would pass to future generations.

5.3 Why is it controversial?

The NLM site lists these concerns.

- Germline changes might affect the development of a fetus in unexpected ways.
- Long-term side effects are not yet known.
- People who would be affected are not yet born, so they cannot choose the treatment.

The NLM site also says the US Government does not allow federal funds for germline gene therapy research in people. The National Human Genome Research Institute says about 40 countries discouraged or banned germline editing research as of 2014. Neither source says germline therapy is banned everywhere today.

> **Quick check 5.** True or false: Most gene therapy research so far has aimed at egg and sperm cells. *(True or False?)* See answer ↓

Sources: medlineplus.gov; genome.gov

Good to Know · Follow the milestones

6 Landmark approvals: Luxturna and Casgevy

6.1 What is Luxturna?

The prescribing information calls Luxturna an **adeno-associated virus vector-based gene therapy**. It is indicated for patients with a confirmed biallelic RPE65 mutation. The mutation causes retinal dystrophy. The label says it is for subretinal injection only. The label gives its initial US approval as 2017.

Bulcha and colleagues call Luxturna the second AAV drug to gain commercial approval. They say Glybera was the first AAV-based gene therapy approved for sale. The European Medicines Agency approved it in 2012.

They also say Luxturna delivers RPE65, the gene affected in Leber's congenital amaurosis. It launched at $425,000 per eye.

6.2 What is Casgevy?

The UK regulator, the MHRA, authorised Casgevy on 16 November 2023. It said Casgevy was the first medicine licensed that uses CRISPR. The MHRA licence covers sickle-cell disease and transfusion-dependent beta-thalassaemia. It is for patients aged 12 and over.

The US FDA approved it on 8 December 2023. The FDA calls Casgevy the first FDA-approved therapy using CRISPR/Cas9. This is a type of genome editing technology. It says Casgevy and Lyfgenia are the first cell-based gene therapies for sickle cell disease.

The MHRA says stem cells are taken out of bone marrow and edited in a laboratory. They are then put back into the patient.

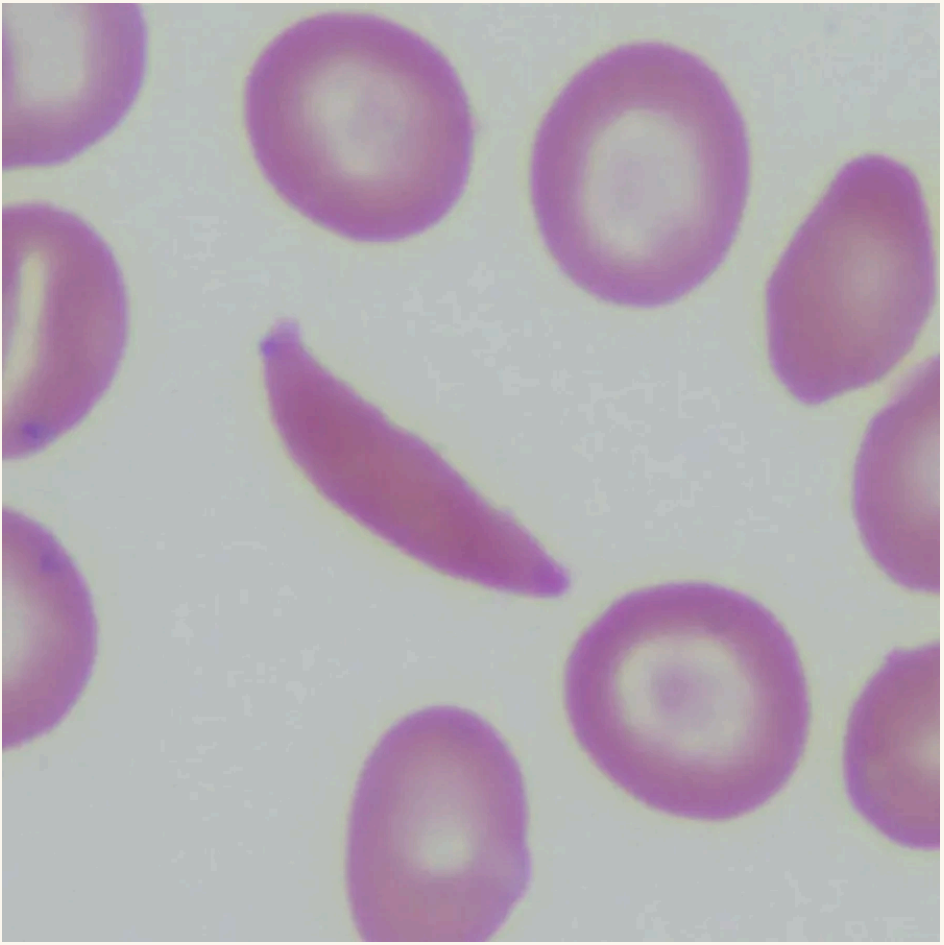
The FDA says both products are made from the patient's own blood stem cells. They are given back as a one-time, single-dose infusion.

For Lyfgenia, the FDA says the blood stem cells are genetically modified to produce HbAT87Q.

6.3 What is sickle cell disease?

The MHRA says sickle-cell disease and beta-thalassaemia are genetic conditions. They are caused by errors in the genes for haemoglobin. Red blood cells use haemoglobin to carry oxygen around the body.

The MHRA says sickle-cell disease can lead to attacks of very severe pain. It can also cause serious infections and anaemia.



Photomicrograph of normal-shaped and sickle-shape red blood cells from a patient with sickle cell disease, as the Commons description puts it. Photo by Gregory Kato, 15 March 2007, via Wikimedia Commons. Licensed CC BY-SA 4.0.

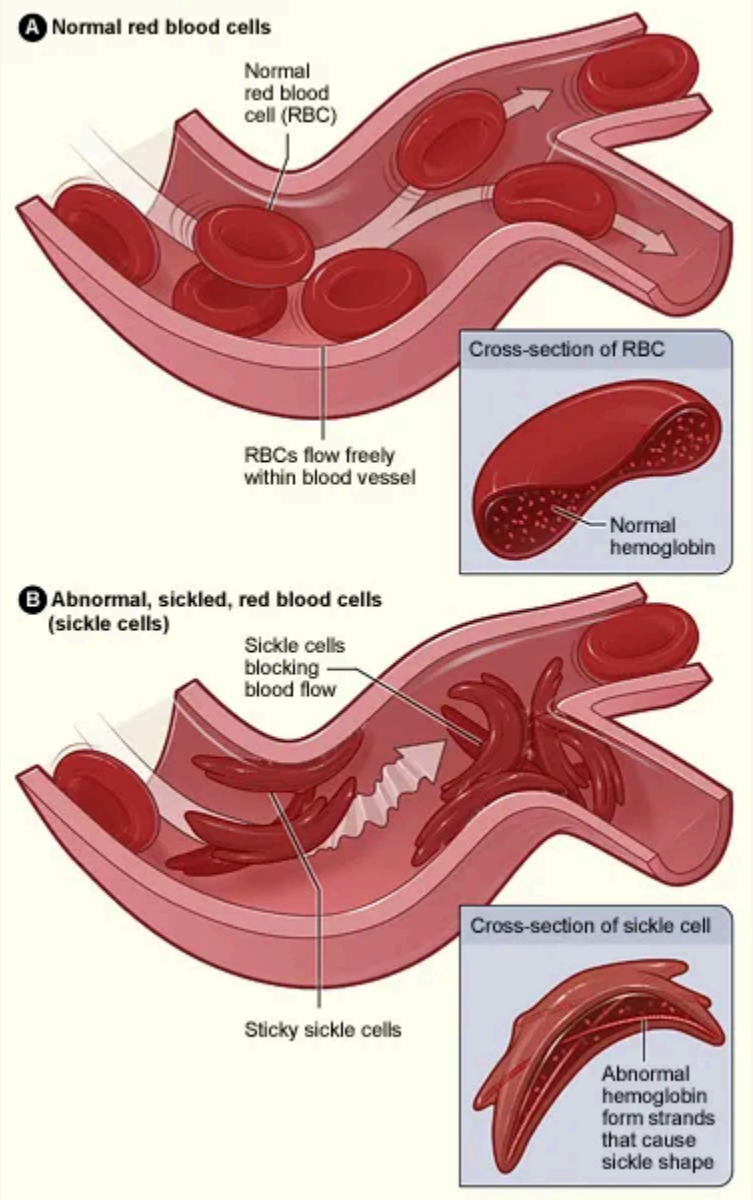


Figure A shows normal red blood cells flowing freely in a blood vessel. Figure B shows abnormal, sickled red blood cells blocking blood flow, as the Commons description puts it. Diagram by the National Heart, Lung, and Blood Institute (NHLBI), public domain, via Wikimedia Commons.

Two approved therapies named in this lesson

Point	Luxturna	Casgevy
Condition	RPE65 mutation-associated retinal dystrophy (prescribing information)	Sickle cell disease (FDA); also transfusion-dependent beta-thalassaemia (MHRA)
Carrier or tool	Adeno-associated virus vector (prescribing information)	CRISPR/Cas9 genome editing (FDA)
How it is given	Subretinal injection (prescribing information)	Patient's own blood stem cells, edited, infused back (FDA, MHRA)
Approval dates in these sources	Label: initial U.S. approval 2017	MHRA: 16 November 2023. FDA: 8 December 2023

The table covers only these two products. It leaves out Lyfgenia and other approved therapies.

> **Quick check 6.** True or false: Casgevy is made from the patient's own blood stem cells. *(True or False?)* See answer ↓

Sources: gov.uk; fda.gov

Current Affairs 2025-26 · India’s own therapy

7 BIRSA 101: India's CRISPR therapy

7.1 What happened on 14 May 2026?

The Ministry of Tribal Affairs, CSIR and CSIR-IGIB held a workshop on BIRSA 101 at CSIR-IGIB, New Delhi, on 14 May 2026. PIB says the Ministry of Tribal Affairs has supported the project with financial assistance of approximately Rs. 3.75 crores.

PIB says participants were briefed on the phased progress of trial facilities. They also heard about rules and partnerships. It says the workshop highlighted the transfer of the technology framework to Serum Institute of India Pvt. Ltd. for collaborative clinical and manufacturing development. The release does not report trial results.

7.2 What was announced on 19 November 2025?

PIB reported that Union Minister Dr Jitendra Singh launched India’s first home-made CRISPR gene therapy for sickle cell disease. The therapy is named **BIRSA 101**. It says it is dedicated to Bhagwan Birsa Munda, remembered as a great tribal freedom fighter.

PIB says the work was developed at CSIR-Institute of Genomics and Integrative Biology (IGIB). CSIR-IGIB and the Serum Institute of India exchanged a technology transfer and collaboration agreement. It covers

IGIB's engineered enFnCas9 CRISPR platform.

The Minister described the approach as a "precise genetic surgery". He tied it to the Prime Minister's vision of a Sickle Cell-Free India by 2047.

7.3 What did the releases say about cost?

PIB says the Minister spoke of the potential to replace treatments priced at Rs. 20-25 crore overseas. Dr Umesh Shaligram of the Serum Institute is quoted as saying that gene therapies cost over three million dollars worldwide.

Neither release gives a price for BIRSA 101 itself. PIB's account of the 14 May 2026 workshop gives no trial results. Treat both as launch-stage news as of those dates.

Why it matters for the exam: BIRSA 101 is described by PIB as India's first indigenous CRISPR-based therapy. It shows how genome editing links to public health and tribal welfare. This is our own note on why the item may be asked.

Sources: pib.gov.in

Exam Practice

8 How UPSC tested it

8.1 What exactly did UPSC ask?

This appeared in the Civil Services (Preliminary) Examination 2026. It was Question 41 in the Series A booklet of General Studies Paper I. The official words are below.

UPSC's provisional answer key for Series A gives option (c), 1 and 2 only, for Question 41. The key is marked provisional. No final key had been published.

See UPSC CSP 2026 GS Paper I, full question paper.

8.2 Which statements were right and why?

The three statements, shortened

Statement	What it says	Correct?	Why
1	Genetic medicines correct or compensate for faulty genes	Yes	MedlinePlus: gene therapy treats disease by correcting the underlying genetic problem
2	Engineered viruses and lipid nanoparticles are carriers	Yes	MedlinePlus: modified viruses and nanoparticles are used as vectors; Duan and colleagues list LNPs
3	The whole DNA sequence is altered	No	MedlinePlus: genome editing changes precise locations in the genome

Statement wording is shortened here. The full official wording is above.

8.3 How can you eliminate options step by step?

- Statement 3 is wrong, so any option that includes statement 3 is out.
- That removes the options "2 and 3 only" and "1, 2 and 3".

- Statement 2 is correct, so “1 only” is incomplete.
- The option “1 and 2 only” is left, and it matches the provisional key.

8.4 Quick Q&A

Which statements were correct?
Statements 1 and 2.

Which statement was the trap?
Statement 3. Genetic medicine targets specific faulty genes.

Which two carriers did the question name?
Engineered viruses and lipid nanoparticles.

Which two routes does this lesson add?
Ex vivo and in vivo.

Sources: upsc.gov.in

Remember this

- **Genetic medicine:** corrects or compensates for specific faulty genes. It does not alter the entire DNA sequence.
- **Carriers:** engineered viruses (AAV, lentivirus, adenovirus) and nanoparticles, including lipid nanoparticles.
- **Routes:** ex vivo means cells are changed outside the body. In vivo means the vector is given directly.
- **Somatic cells:** body cells. Changes in them cannot be passed to children. Germline changes would pass on.
- **Casgevy:** MHRA authorised it on 16 November 2023. The FDA approved it on 8 December 2023.
- **BIRSA 101:** PIB reported its launch on 19 November 2025, with CSIR-IGIB and the Serum Institute.
- **Official answer:** statements 1 and 2, UPSC CSP 2026 GS Paper I, Question 41 (provisional key).

Explain it back: Say in your own words why fixing one faulty gene is not the same as changing the whole DNA. Then name the two carriers in the UPSC statement.

Practice MCQs

1. Consider the following statements:

1. Genetic medicines can use engineered viruses as carriers.

2. Genetic medicine typically replaces a patients entire DNA sequence.

3. Ex vivo gene therapy modifies cells outside the body before returning them to the patient.

Which of the statements given above is/are correct?

A. 1 and 3 only

B. 1 and 2 only

C. 2 and 3 only

D. 1, 2 and 3

See answer ↓

2. How is the gene therapy Casgevy made for each patient, according to the UK regulator and the US FDA?

A. Stem cells from a matched donor are transplanted

B. A lipid nanoparticle carries the edit to the liver

C. A viral vector is injected straight into the eye

D. The patient's own blood stem cells are edited in a laboratory and infused back

See answer ↓

3. In gene therapy, what is a vector?

A. A patient's own blood stem cell

B. A carrier engineered to deliver genetic material or gene-editing tools into cells

C. A piece of DNA that is cut out and thrown away

D. A medicine that switches off the immune system

See answer ↓

4. Which statement about COVID-19 mRNA vaccines is supported by the sources?

A. The mRNA enters the nucleus and rewrites the DNA

B. The mRNA is carried inside adeno-associated viruses

C. The mRNA is delivered by an injection into the retina

D. The mRNA is packaged in lipid nanoparticles and does not alter DNA

See answer ↓

5. Which genome-editing technology does the US FDA say Casgevy uses?

A. CRISPR/Cas9

B. RNA interference

C. Zinc finger nucleases

D. TALEN

See answer ↓

6. The National Human Genome Research Institute says most ethical debate on genome editing centres on the germline. What reason does it give?

A. Editing changes made in the germline would be passed down to future generations

B. Germline editing is always cheaper than body-cell editing

C. Germline edits are made only in bone marrow

D. Germline edits cannot be done with CRISPR

See answer ↓

7. Consider the following statements about Luxturna.

1. It is an adeno-associated virus vector-based gene therapy.

2. It is for subretinal injection.

3. It is approved to treat sickle cell disease.

Which statements are correct?

A. 1 only

B. 1 and 2 only

C. 2 and 3 only

D. 1, 2 and 3

See answer ↓

Answer Key

1. **A.** Statements 1 and 3 are correct. MedlinePlus says viruses modified so they can't cause disease are used as vectors, so statement 1 holds. Bulcha and colleagues describe ex vivo therapy as taking out a patient's cells, changing them with a vector and infusing them back. MedlinePlus describes the same steps, so statement 3 holds. MedlinePlus says genome editing alters genetic material at precise locations in the genome. So statement 2, about replacing a patient's entire DNA sequence, is wrong. ↑ Q1
2. **D.** The MHRA says stem cells are taken out of bone marrow, edited in a laboratory and infused back into the patient. The FDA says Casgevy is made from the patient's own blood stem cells, which are modified and given back as a one-time infusion. The other three options describe a different route. None of them appears in either source for Casgevy. ↑ Q2
3. **B.** MedlinePlus says a carrier called a vector is genetically engineered to carry and deliver genetic material or gene-editing tools. Bulcha and colleagues say delivery is carried out by gene delivery vehicles called vectors. Neither source describes a vector as a stem cell, a discarded DNA piece or an immune-system drug. ↑ Q3
4. **D.** Verbeke and colleagues write that the COVID-19 mRNA vaccines consist of synthetic mRNA packaged in lipid nanoparticles to deliver mRNA to cells. MedlinePlus says mRNA from vaccines does not enter the nucleus and does not alter DNA. Neither source mentions viral carriers or retinal injection for these vaccines. ↑ Q4
5. **A.** The FDA says Casgevy is the first FDA-approved therapy using CRISPR/Cas9, a type of genome editing technology. The MHRA likewise says Casgevy is based on the gene-editing tool CRISPR. Duan and colleagues name zinc finger nucleases and TALEN as other gene-editing technologies, not the one used in Casgevy. ↑ Q5
6. **A.** The National Human Genome Research Institute says most ethical discussions centre on the human germline. Editing changes made there would be passed down to future generations. MedlinePlus says the same contrast. Changes in body (somatic) cells such as bone marrow or blood cells cannot be passed to a person's children. ↑ Q6

7. **B.** The prescribing information calls Luxturna an adeno-associated virus vector-based gene therapy. It says the product is for subretinal injection only. So statements 1 and 2 are correct. The label gives its indication as RPE65 mutation-associated retinal dystrophy. Bulcha and colleagues link RPE65 to Leber's congenital amaurosis. Sickle cell disease is the condition for Casgevy, so statement 3 is wrong. ↑ Q7

Quick-check answers

1. **False.** MedlinePlus says genome editing adds, removes or alters genetic material at precise locations in the genome. ↑ 1
2. **True.** MedlinePlus says the viruses are modified so they can't cause disease when used in people. ↑ 2
3. **False.** MedlinePlus says mRNA from vaccines does not enter the nucleus and does not alter DNA. ↑ 3
4. **True.** Bulcha and colleagues and MedlinePlus both describe removing the patient's cells, treating them with the vector outside the body, and returning them. ↑ 4
5. **False.** MedlinePlus says current research has focused on body (somatic) cells such as bone marrow or blood cells. ↑ 5
6. **True.** The FDA says both Casgevy and Lyfgenia are made from the patients' own blood stem cells, which are modified and given back. ↑ 6
7. **Think it through.** It is genetic medicine. The NLM site describes changes at precise locations in the genome. Altering the entire sequence is the wrong statement in the UPSC question. ↑ 7
8. **Think it through.** The NLM site says mRNA from vaccines does not enter the nucleus and does not alter DNA. The carrier is shared, but the cargo and the result are different. ↑ 8

Curated by Arsalan Zuberi · mcqquestion.com



Scan to read online

https://mcqquestion.com/articles/scitech0134-genetic-medicine-how-gene-therapy-works/