

Biotechnology and its Application

Level - 1

CORE SUBJECTIVE QUESTIONS

MULTIPLE CHOICE QUESTIONS (MCQs)

(1 Mark)

- Option (D) is correct
Explanation: Transgenic mice have been used in research to test the safety and efficacy of the polio vaccine.
- Option (D) is correct
Explanation: Golden rice is genetically engineered to synthesise beta-carotene, which the body can convert into vitamin A, helping to combat vitamin A deficiency in certain populations.
- Option (A) is correct
Explanation: The specific Bt toxin genes Cry-I Ac and Cry-II Ab, derived from *Bacillus thuringiensis* (Bt), produce proteins that are toxic to cotton bollworm larvae. When ingested, these proteins bind to gut receptors, causing cell lysis and death of the insects. Incorporating these genes into cotton plants enhances pest resistance, reduces reliance on chemical insecticides, and improves crop yields sustainably.
- Option (D) is correct
Explanation: Approximately 95% of the transgenic animals developed for research purposes are mice, making them the most commonly used model organism in genetic studies.
- Option (C) is correct
Explanation: The crystals of Bt toxin produced by bacteria, such as *Bacillus thuringiensis*, are in an inactive form and are only activated in the alkaline environment of the insect gut. This inactivity prevents the bacteria from being harmed by the toxin they produce.
- Option (D) is correct
Explanation: Dr. R.A. Mashelkar challenged the patent right granted to the University of Mississippi Medical Centre for the use of turmeric in wound healing, arguing that turmeric is a traditional knowledge and should not be patented.
- Option (B) is correct
Explanation: The product produced as a result of DNA manipulation in the first transgenic cow 'Rosie' is α -lactalbumin.
- Option (B) is correct
Explanation: The vector of T-DNA is typically the Ti plasmid from *Agrobacterium tumefaciens*.
- Option (C) is correct
Explanation: Nematode-resistant tobacco plants have been developed by introducing DNA that produces sense and antisense RNA, which leads to the suppression of specific genes in nematodes. This technique, known as RNA interference (RNAi), disrupts the expression of genes essential for nematode survival, providing the plants with enhanced resistance against these pests.

ASSERTION-REASON QUESTIONS

(1 Mark)

- Option (D) is correct
Explanation: *Agrobacterium tumefaciens* primarily infects dicot plants, not monocots. It transfers T-DNA into the host plant cells, causing them to proliferate and form tumours.
- Option (C) is correct
Explanation: Cells in the embryonic stage are not considered "immortal." While they can divide and proliferate, they are generally undifferentiated, meaning they have not yet developed into specialised cell types.
- Option (B) is correct
Explanation: Transgenic microorganisms, such as bacteria, are commonly used to produce proteins efficiently and in large quantities. For example, the gene for human insulin can be inserted into bacteria, allowing them to synthesise insulin, which is essential for treating diabetes.
- Option (C) is correct
Explanation: Lymphocytes are not immortal. While some lymphocytes can proliferate in response to an immune challenge, they have a limited lifespan and do not divide indefinitely.
- Option (C) is correct
Explanation: Bt toxin does not kill the bacterium that produces it, but kill the insect that ingests it because the endotoxin that accumulates in the bacterium is an inactive precursor. It gets activated only in the alkaline gut of insect.

VERY SHORT ANSWER TYPE QUESTIONS

(2 Marks)

- Applications of stem cell technology are:
 - Stem cell technology is useful in treatment of diseases like diabetes, spinal cord injury, heart disease etc.
 - Genetic disease e.g. cystic fibrosis can be treated by this.
 - Through tissue repair, different disease can be treated by this technology.
 - Adult bone marrow is injected in heart arteries to improve cardiac functions.
 - Injection of stem cells reduces pancreatic cancer.
 - Stem cells are also helpful in rheumatoid arthritis.
 - These are also used in restoration of vision.

(Any Two)
- A single stranded DNA or RNA tagged with a radioactive molecule (probe), is allowed to hybridise with its complementary DNA in a clone of cells, followed by detection using autoradiography, the clone having the mutated gene will hence not appear on the photographic film.

Polymerase Chain Reaction (PCR) is used to detect a disease even before any clinical symptoms appears, involves denaturation, annealing, to amplify DNA of the pathogen using pathogen specific primers.
- Transgenic animals are designed to allow the study of gene regulation and effects of gene on the normal functions of the body. The result obtained by introducing gene from other species that alter the formation of any growth like factors (such as insulin like growth factor) is used to know its biological role.
 - Transgenic animals are made sensitive to toxic substances and when exposed to these substances the effects is studied to get results in lesser time.
- Made crops more tolerant to abiotic stresses (cold, drought, salt, heat.)
 - Reduced reliance on chemical pesticides (pest-resistant crops).
 - Helped to reduce post-harvest losses.
 - Increased efficiency of mineral usage by plants (this prevents early exhaustion of fertility of soil).
 - Enhanced nutritional value of food, e.g., golden rice, i.e., Vitamin 'A' enriched rice.
 - In addition to these uses, GM has been used to create tailor-made plants to supply alternative resources to industries, in the form of starches, fuels and pharmaceuticals. (Any four applications)
- Basmati Rice
As Indian Basmati was crossed with semi-dwarf varieties (therefore claimed as an invention).

SHORT ANSWER TYPE QUESTIONS

(3 Marks)

- Transgenic animals are those which have their DNA manipulated to possess and express a foreign gene.
 - First transgenic Cow – Rosie
It produced human protein enriched milk (2.4 g/litre). Cow milk containing human alpha lactalbumin protein is nutritionally more balanced product for human babies than natural cow milk.
- Introduction of nematode specific gene using *Agrobacterium* vector. Production of both sense and anti-sense RNA in host cell, production of dsRNA, RNAi/RNA interference is initiated, silencing of specific mRNA of the nematode. Parasite could not survive in the transgenic host and transgenic plant is protected from the parasite.
- It is 'pro-insulin' produced, in an inactive state.
 - A – A peptide, B – B peptide, C – C peptide, D – disulphide bonds.
- Bacillus thuringiensis*
 - GM cotton crop contains Bt toxin protein which exists as inactive protoxins, but once bollworm ingest the inactive toxin it is converted into an active form due to the alkaline pH of the gut of the worm, which solubilises the crystals of the prototoxin. Activated toxin binds to the surface of the midgut epithelial cells of the worm and creates pores that cause swelling and lysis and death of the insect caterpillar.
- Gene Therapy is collection of methods that allows correction of a gene defect that has been diagnosed in a child/embryo.
 - A normal gene is isolated from bone marrow cells, and introduced into the cells at early embryonic stage
ADA deficiency (adenosine deaminase deficiency)/SCID (severe combined immunodeficiency disease)
- They have the ability of self-renewal, to divide, and differentiate into any kind of cell/tissue/organ.
 - Inner cell mass of blastocyst/umbilical cord/Bone marrow. (Any one)
 - (1) Diabetes treatment via forming islets of Langerhans.
(2) Restoration of vision by injecting stem cells,
(3) To treat rheumatoid arthritis,
(4) Reduces pancreatic cancer,
(5) To treat genetic disorder like cystic fibrosis, spinal cord injuries, heart disease. (Any two)
- Meloidegryne incognitia*
Roots
 - By using *Agrobacterium* vector, Nematode specific genes were introduced into host plant. Introduction of DNA produced both sense and anti-sense RNA in the host cells. This two RNAs being complementary to each other form a double stranded RNA (ds RNA), that initiated RNAi and thus silenced the specific mRNA of the nematode. Hence, parasite could not survive in transgenic host.
- In bacteria, toxin is produced in inactive form/protoxin, insect ingests it, protoxin/inactive protein becomes active due to alkaline pH of gut (solubilise), toxin binds

- to epithelial cells (of midgut) and creates pores, causes swelling, lysis and death.
9. *Agrobacterium tumefaciens*— (plants pathogen). The disarmed Ti Plasmid is used as a vector to introduce the gene of interest in the variety of plants.
Retrovirus—(animals), Disarmed Retroviruses are used to transfer genes of interest into mammalian host cells.
 10. (i) Recombinant DNA technology, Polymerase Chain Reaction, ELISA
(ii) Effective treatment of diseases, early diagnosis, understanding path physiology of disease, identification of genetic disorders. (any three)
 11. *Bacillus thuringiensis*
Bacillus thuringiensis forms protein crystals which exist as inactive toxin/protoxin, therefore do not kill it. The ingested inactive toxin in insect gets solubilised and become active, due to alkaline pH of insect's gut thereby killing the insect.
 12. Cry proteins are toxic insecticidal proteins/pro-toxin/crystal proteins produced by cry genes of *Bacillus thuringiensis*.
E.g., in Bt Cotton/Bt corn/rice/tomato/ soyabean.
Bt toxin protein exists as inactive protoxins, which get activated inside the alkaline pH of the gut of insect, causes perforations/swelling/lysis of midgut ultimately killing the insect.
 13. (i) Enzyme-Linked Immunosorbent Assay
It is based on the principle of antigen-antibody interaction/HIV infection can be detected by the presence of antigen or by detecting the antibody synthesised against the pathogen.
Polymerase chain reaction
Early detection and amplification of genetic material of pathogen/virus.
(ii) RNA
- 14. Insulin production in human body:**
- (i) Synthesised naturally in the form of proinsulin consisting of polypeptide chain A and polypeptide chain B, linked together by disulphide bonds and an extra stretch called C-peptide.
 - (ii) The C-peptide is removed during processing and proinsulin matures into functional insulin.
- Insulin production by rDNA technology:**
- (i) Two DNA sequences corresponding to chain A and chain B of human insulin are synthesised.
 - (ii) They are introduced into two different plasmids of *E. coli*.
 - (iii) Chain A and chain B are produced separately,
 - (iv) Extracted and combined by disulphide bond to form human insulin.

LONG ANSWER TYPE QUESTIONS

(5 Marks)

1.
 - Several nematodes parasitize a wide variety of plants and animals including human beings.
 - A nematode *Meloidogyne incognitia* infects the roots of tobacco plants and causes a great reduction in yield.
 - A novel strategy was adopted to prevent this infestation which was based on the process of RNA interference (RNAi).
 - RNAi takes place in all eukaryotic organisms as a method of cellular defense.
 - This method involves silencing of a specific mRNA due to a complementary dsRNA molecule that binds to and prevents translation of the mRNA (silencing).
 - The source of this complementary RNA could be from an infection by viruses having RNA genomes or mobile genetic elements (transposons) that replicate via an RNA intermediate.
 - Using *Agrobacterium* vectors, nematode-specific genes were introduced into the host plant.
 - The introduction of DNA was such that it produced both sense and anti-sense RNA in the host cells.
 - These two RNA's being complementary to each other formed a double stranded (dsRNA) that initiated RNAi and thus, silenced the specific mRNA of the nematode.
 - The consequence was that the parasite could not survive in a transgenic host expressing specific interfering RNA. The transgenic plant therefore got itself protected from the parasite.
2.
 - Gene therapy is a collection of methods that allows correction of a gene defect that has been diagnosed in a child/embryo. Here genes are inserted into a person's cells and tissues to treat a disease.
 - Correction of a genetic defect involves delivery of a normal gene into the individual or embryo to take over the function of and compensate for the non-functional gene.
 - The first clinical gene therapy was given in 1990 to a 4-year old girl with adenosine deaminase (ADA) deficiency. This enzyme is crucial for the immune system to function.
 - The disorder is caused due to the deletion of the gene for adenosine deaminase.
 - In some children ADA deficiency can be cured by bone marrow transplantation; in others it can be treated by enzyme replacement therapy, in which functional ADA is given to the patient by injection.
 - But the problem with both of these approaches is that they are not completely curative.
 - As a first step towards gene therapy, lymphocytes from the blood of the patient are grown in a culture outside the body.
 - A functional ADA cDNA (using a retroviral vector) is then introduced into these lymphocytes, which are subsequently returned to the patient.
 - However, as these cells are not immortal, the patient requires periodic infusion of such genetically engineered lymphocytes.
 - However, if the gene isolate from marrow cells producing ADA is introduced into cells at early embryonic stages, it could be a permanent cure.

3. (i) Specific Bt toxin gene cry IAc/cry II Ab, isolated from *Bacillus thuringiensis* bacteria and incorporated into the cotton plant to provide resistance to bollworm.
- (ii) *Bacillus thuringiensis* forms toxic insecticidal protein or Bt toxin protein during a particular growth phase, Bt toxin protein exist as inactive pro-toxin. On ingestion by the bollworm inactive toxin is converted into active form due to alkaline pH of the gut, activated toxin binds to the surface of the mid-gut epithelial cells, create pores and causes cell swelling, lysis and death of the insect.
4. (i) Bt cotton crop is pest resistant/insect/with increase productivity.
- (ii) Cotton bollworms/corn borer/tobacco budworm/army worm/coleopterans (beetles)/dipterans (flies, mosquitoes) (Any two)
- (iii) BT toxin protein exists as an inactive protoxin in the bacterium, but once the insect this toxin it is converted into an active form, due to the alkaline pH of the gut, which solubilises inactive crystals of toxic insecticide. The activated toxin binds to the surface of the midgut epithelial cells of the insect, creates pores, that cause cell swelling and lysis and eventually the death of the insect.
5. (i) Bone marrow transplantation
- (ii) Enzyme replacement therapy/ functional ADA is given to the patient by injection.
- (iii) Gene therapy/lymphocytes from the blood of the patient are grown in a culture outside the body, a functional ADA cDNA (using a retroviral vector) is then introduced into these lymphocytes, which are subsequently returned to the patient. These cells are not immortal, the patient requires periodic infusion of such genetically engineered lymphocytes. However if the gene isolate from marrow cells producing ADA is introduced into cells at early embryonic stages.
6. (i) When monkeys are treated with saline solution, serum cholesterol level increases from 24 hours to 264 hours.
- When monkeys are treated with 2.5mg/kg SiRNAs, level of serum cholesterol decreases from 24 hours to 264 hours.
- (ii) Using *Agrobacterium* vectors, nematode specific genes are introduced into the host plant, introduced DNA forms both sense and anti-sense RNA in the host cell, these two RNAs being complementary to each other, form a double stranded RNA, that initiates RNAi and thus silencing the specific mRNA of the nematode, nematode is unable to survive in the transgenic plant.
7. (i)
 - Species A
 - The leaf area damaged by species - A in Bt-corn is the least.
- (ii) Species-B
- (iii) Not to grow B variety as seeds are expensive and of not much benefit (productivity wise)/advise to grow Bt corn with its proper justification.
- (iv) Cry IAb.
8. (i) Adenosine Deaminase (ADA) Deficiency.
- (ii) Firstly, lymphocytes from the blood of the patient are grown in a culture outside the body. Then a functional ADA cDNA is introduced into the lymphocytes using a retroviral vector. Lastly, the lymphocytes subsequently returned to the patient. Gene therapy using lymphocytes is not a permanent cure as the patient required periodic infusion of such genetically engineered lymphocytes.
- (iii) The permanent cure of the disease is introduction of gene producing ADA isolated from marrow cells into cells at early embryonic stages.

Level - 2

ADVANCED COMPETENCY FOCUSED QUESTIONS

MULTIPLE CHOICE QUESTIONS (MCQs)

(1 Mark)

1. Option (B) is correct
Explanation: A major aim of biotechnology in agriculture is to improve crop quality and yield, enhance resistance to pests, diseases, and environmental stresses, and reduce reliance on chemical pesticides.
2. Option (C) is correct
Explanation: Golden Rice is a genetically modified variety of rice enriched with β -carotene, a precursor of Vitamin A. When consumed, β -carotene is converted into Vitamin A in the human body.
3. Option (B) is correct
Explanation: In Bt cotton, the Cry gene from *Bacillus thuringiensis* is introduced into the plant genome. The plant produces an inactive *protoxin*. When an insect (e.g., bollworm) feeds on the cotton plant, the *protoxin* reaches the insect's alkaline gut. There, it gets activated into a toxic form. The active toxin binds to receptors in the gut lining, forming pores and causing cell *lysis* and death of the insect.
4. Option (B) is correct
Explanation: *Humulin* is the first recombinant therapeutic protein approved for human use. It is recombinant human insulin produced using genetically engineered *E. coli* through recombinant DNA technology. It was developed to treat diabetes mellitus. It mimics natural human insulin and has fewer side effects compared to animal-derived insulin.
5. Option (D) is correct
Explanation:
- (i) **Therapeutics:** biotechnology is used to produce recombinant drugs like insulin, growth hormone, and monoclonal antibodies.
- (ii) **GM crops for agriculture:** genetic engineering is used to create pest-resistant, nutrient-enriched crops (e.g., Bt cotton, Golden Rice).
- (iii) **Diagnostics:** biotech tools like PCR, ELISA, and DNA fingerprinting help in disease detection and forensic science.
- (iv) **Stone masonry:** Not related to biotechnology; it is a construction technique.

ASSERTION-REASON QUESTIONS

(1 Mark)

1. Option (A) is correct

Explanation: Assertion is true. Bt cotton is a genetically modified crop that produces insecticidal proteins, protecting the plant from pests like bollworms without needing chemical pesticides.

Reason is also true. The Bt gene inserted into the cotton plant produces an inactive *protoxin*. When insect larvae consume the plant, the alkaline pH of their gut activates the toxin, which then binds to the gut lining, causing cell *lysis* and death of the insect.

2. Option (D) is correct

Explanation: Assertion is false. Golden Rice is not genetically modified for iron content. It is developed to combat vitamin A deficiency, not iron deficiency.

Reason is true. Golden Rice is genetically engineered to produce β -carotene, a precursor of vitamin A, which helps prevent night blindness and other disorders related to vitamin A deficiency.

3. Option (D) is correct

Explanation: Assertion is false. Gene therapy is not yet a permanent solution to all genetic disorders. It shows promise but faces challenges like: temporary effects in somatic cell gene therapy, limited success in complex or *multigenic* disorders, and risks like immune response or insertional mutations.

Reason is true. Gene therapy does involve inserting a healthy gene into a patient's cells to correct

or compensate for a faulty gene—this is a valid mechanism.

4. Option (A) is correct

Explanation: Assertion is true. Recombinant vaccines are generally considered safer because they do not use live or weakened pathogens, reducing the risk of infection or side effects.

Reason is also true. These vaccines are produced by inserting a gene coding for the antigenic protein (like a viral surface protein) into another organism (e.g., yeast). Only the purified antigen is used, not the whole pathogen.

Since the safety of recombinant vaccines is due to the absence of the entire pathogen, the reason correctly explains the assertion.

5. Option (B) is correct

Explanation: Assertion is true. Biopiracy is the unethical or illegal use of biological resources (like medicinal plants or genetic material) or traditional knowledge from one country or community, often without permission or benefit sharing.

Reason is also true.

Both assertion and reason are true but reason refers to developed countries (not developing) that exploit resources from developing nations. So while the fact is true, it does not correctly explain the definition in the assertion — it's an example or consequence, not a definition.

VERY SHORT ANSWER TYPE QUESTIONS

(2 Marks)

1. They produce insecticidal proteins (from *Bacillus thuringiensis*) within the plant, reducing the need for chemical pesticides. This lowers environmental pollution and protects non-target organisms.
2. Biotechnology led to the development of Golden Rice, a genetically modified crop enriched with β -carotene, a precursor of vitamin A, helping combat blindness and malnutrition.
3. Recombinant insulin (*Humulin*) is identical to human insulin, reducing the risk of allergic reactions. It is produced in large quantities using genetically engineered *E. coli*, making it ethical and efficient.

4. (i) **Medicine:** Transgenic animals are used to produce therapeutic proteins like antithrombin in milk (e.g., transgenic goats).

(ii) **Agriculture:** They help improve traits like milk yield or meat quality (e.g., transgenic cows or pigs).

5. Biopiracy is the unauthorised commercial use of biological resources or traditional knowledge.

Example: Patenting of basmati rice varieties by a US company without acknowledging Indian farmers' contributions.

SHORT ANSWER TYPE QUESTIONS

(3 Marks)

1. (1) The Bt gene from *Bacillus thuringiensis* is inserted into crop plants (e.g., Bt cotton).
(2) The plants produce Cry proteins that are inactive in plant tissue.
(3) When insect larvae (like bollworms) consume the plant, the alkaline pH in their gut activates the Cry toxin.
(4) The toxin creates pores in the gut lining, causing cell *lysis* and death of the insect.
2. (1) GMOs like Bt crops reduce reliance on chemical pesticides, lowering soil and water pollution.
(2) GM bacteria (e.g., *Pseudomonas putida*) can degrade oil spills and industrial pollutants (bioremediation).
(3) GMOs help produce recombinant vaccines and medicines (e.g., insulin, hepatitis B vaccine), making healthcare safer and more accessible.

3. Gene therapy is the introduction of a functional gene to correct a genetic defect.

In ADA (Adenosine Deaminase) deficiency, a functional ADA gene was inserted into the patient's T-lymphocytes using a retroviral vector. The corrected cells were reintroduced into the patient, partially restoring immune function. This process was repeated periodically; later approaches involved bone marrow gene therapy.

4. Transgenic animals are those that have foreign genes deliberately inserted into their genome.

Uses:

(1) **Pharming:** e.g., producing therapeutic proteins like *antithrombin III* in goat's milk.

(2) **Disease models:** used to study human diseases like cancer or Alzheimer's for research and drug testing.

5. Recombinant vaccines are made by inserting genes encoding antigenic proteins of pathogens into microbes like yeast. These microbes then produce the antigen, which is purified and used as a vaccine.

Example: Hepatitis B vaccine produced in genetically modified yeast.

Advantages: Safer, no live pathogens, less risk of side effects, and easier to store.

CASE BASED QUESTIONS

(4 Mark)

1. (i) Option (C) is correct
Explanation: The most commonly used eukaryotic microorganism in biotechnology is *Saccharomyces cerevisiae*, commonly known as baker's yeast.
- (ii) Option (C) is correct
Explanation: The correct option for household products made with yeast is Dosa, Idli, Bread. These foods are produced through fermentation, where yeast helps in leavening and flavour enhancement.
- (iii) Option (A) is correct
Explanation: The most common product made by certain bacteria that significantly impacts human health is antibiotics.
- (iv) Option (B) is correct
Explanation: *E. coli* widely used in biotechnology due to its rapid growth, well-characterised genetics, and ability to efficiently replicate plasmids, making it ideal for gene cloning and protein expression.
2. (i) Hormone - insulin
Disease - diabetes
- (ii) Insulin is a protein hormone. If taken orally, they will get digested by pepsin and trypsin in the stomach and small intestine into their simpler forms, thereby losing their function.
- (iii) (a) False
Correct statement - The structure in the image is a protein and is the final/active/mature form of the hormone.
The C-peptide which is part of the precursor form is missing in the structure shown, indicating that it is the final form.
- OR**
- (b) Challenge - getting insulin assembled into a mature form without c-peptide sequence.
Overcome - chains A and B were produced separately by rDNA technology in *E. coli* cells, extracted and combined by creating disulphide bonds to form human insulin.

LONG ANSWER TYPE QUESTIONS

(5 Marks)

1. (i) Golden Rice is a genetically modified variety of rice enriched with β -carotene, a precursor of Vitamin A, developed by inserting genes from daffodil and a bacterium.
- (ii) When consumed, β -carotene is converted to Vitamin A in the human body, helping to prevent night blindness and other deficiency disorders.
- (iii) **Advantages:**
- (1) Nutritional improvement without changing dietary habits.
 - (2) Can reach remote populations through staple food.
- Limitation:** Ethical and environmental concerns regarding GM crop use.
2. (i) Animal insulin often caused allergic reactions and ethical concerns; human recombinant insulin is identical to natural human insulin.
- (ii) **Chains:** A and B chains
The genes for both chains are inserted into separate plasmids and introduced into *E. coli*. The bacteria produce the chains, which are purified and combined using disulfide bonds.
- (iii) (1) Consistent, safe, and large-scale production.
(2) No ethical issues from using animals.
3. (i) Bt crops reduce the need for chemical pesticides, thus lowering soil and water pollution and protecting beneficial insects.
- (ii) *Pseudomonas putida* - used in bioremediation of oil spills and detoxifying industrial waste.
- (iii) Bio-pesticides are biologically derived agents used to control pests. Example: *Bacillus thuringiensis* (Bt) - produces toxins that kill insect larvae when ingested.
4. (i) **Biopiracy** is the unauthorised use of biological resources or traditional knowledge by companies or researchers without compensation.
- (ii) **Example:** Patenting of basmati rice varieties by a U.S. company based on Indian traditional varieties.
- (iii) (1) Enforcing international treaties and biodiversity laws.
(2) Documenting traditional knowledge and providing community rights.
- (iv) Patents encourage innovation by offering exclusivity, but they can exploit indigenous communities and limit access to essential resources.
5. (i) Gene therapy is the technique of introducing a normal gene into cells to correct a genetic defect.
Example: ADA (Adenosine Deaminase) deficiency
- (ii) (1) T-cells are removed from the patient.
(2) A functional ADA gene is inserted using a retroviral vector.
(3) Modified cells are cultured and reintroduced into the patient's bloodstream.
- (iii) (1) Temporary effects requiring repeated treatments.
(2) Risk of immune response or insertional mutations.

