Gene Therapy Quiz Questions and Answers PDF

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Which regulatory body oversees gene therapy in the United States?

- ◯ WHO
- ◯ EMA
- ◯ **FDA ✓**
- ◯ CDC

> The Food and Drug Administration (FDA) is the regulatory body responsible for overseeing gene therapy in the United States. This includes evaluating the safety and efficacy of gene therapy products before they can be approved for public use.

What are some challenges faced by gene therapy? (Select all that apply)

- ☐ **Immune response ✓**
- ☐ **Delivery issues ✓**
- ☐ High success rate
- ☐ **Ethical concerns ✓**

> Gene therapy faces several challenges including immune responses to the therapy, difficulties in delivering the therapeutic genes effectively, potential off-target effects, and ethical concerns regarding genetic modifications.

What is the term for a gene that is transferred from one organism to another?

- ◯ **Transgene ✓**
- ◯ Genome
- ◯ Chromosome
- ◯ Alleles

> The term for a gene that is transferred from one organism to another is 'transgene.' This process is often utilized in genetic engineering to introduce new traits into an organism.

Which of the following are ethical considerations in gene therapy? (Select all that apply)

- [ ] Cost of treatment
- [ ] **Long-term effects ✓**
- [ ] **Accessibility to all patients ✓**
- [ ] **Germline modifications ✓**

> Ethical considerations in gene therapy include issues such as informed consent, potential long-term effects on patients and future generations, and the risk of genetic discrimination. These factors must be carefully evaluated to ensure responsible application of gene therapy technologies.

What are the main challenges in delivering genes to target cells in gene therapy?

> **The main challenges in delivering genes to target cells in gene therapy are efficient delivery mechanisms, immune system evasion, and precise targeting to avoid off-target effects.**

Discuss the ethical implications of using CRISPR technology in gene therapy.

> **The use of CRISPR technology in gene therapy raises ethical issues such as the risk of off-target effects, the potential for creating genetic inequalities, and the debate over the appropriateness of germline editing.**

How does the use of viral vectors in gene therapy work, and what are the associated risks?

Viral vectors work by infectiously delivering genetic material into target cells, allowing for the expression of therapeutic genes. However, associated risks include immune responses that can lead to inflammation, the possibility of inserting the therapeutic gene into unintended locations in the genome (insertional mutagenesis), and the chance of the viral vector replicating uncontrollably.

Which diseases have been successfully treated using gene therapy? (Select all that apply)

- [x] **Certain types of blindness ✓**
- [x] **Severe Combined Immunodeficiency (SCID) ✓**
- [ ] Influenza
- [x] **HIV (in investigational treatments) ✓**

Gene therapy has been successfully used to treat a variety of diseases, including certain types of inherited genetic disorders, some cancers, and specific viral infections such as HIV. Notable examples include treatments for spinal muscular atrophy and inherited retinal diseases.

Which of the following is a genetic disorder that gene therapy aims to treat?

- ○ Diabetes
- ○ **Cystic Fibrosis ✓**
- ○ Hypertension
- ○ Asthma

Gene therapy is designed to treat various genetic disorders by correcting or replacing defective genes. Common examples of genetic disorders targeted by gene therapy include cystic fibrosis, hemophilia, and muscular dystrophy.

What year was the first successful gene therapy performed?

- ○ 1980
- ○ **1990 ✓**
- ○ 2000
- ○ 2010

> The first successful gene therapy was performed in 1990, marking a significant milestone in medical science. This therapy involved treating a young girl with a genetic disorder by inserting a functional copy of the gene she was missing.

What is the primary goal of gene therapy?

- ◯ To enhance athletic performance
- ◯ **To alter genetic material to treat or prevent disease ✓**
- ◯ To create genetically modified organisms for agriculture
- ◯ To clone animals

> The primary goal of gene therapy is to treat or prevent diseases by correcting or replacing defective genes in a patient's cells. This innovative approach aims to address the underlying genetic causes of various disorders.

What is a major ethical concern associated with germline gene therapy?

- ◯ Cost of treatment
- ◯ Long-term environmental impact
- ◯ **Changes can be inherited by future generations ✓**
- ◯ Difficulty in administering treatment

> A major ethical concern associated with germline gene therapy is the potential for unintended consequences, such as off-target effects or the introduction of new genetic disorders, which could affect not only the individual but also future generations.

Which type of gene therapy targets non-reproductive cells?

- ◯ Germline Gene Therapy
- ◯ **Somatic Gene Therapy ✓**
- ◯ Viral Gene Therapy
- ◯ CRISPR Gene Therapy

> Gene therapy that targets non-reproductive cells is known as somatic gene therapy. This approach focuses on treating or preventing diseases by modifying the genes in somatic (body) cells rather than germline (reproductive) cells.

Which vector is commonly used in gene therapy for delivering genes?

- ◯ Bacteria
- ◯ Fungi

- ◯ **Viral Vectors ✓**
- ◯ Plant Cells

> Adenoviruses are commonly used as vectors in gene therapy due to their ability to efficiently deliver genetic material into host cells. They can infect both dividing and non-dividing cells, making them versatile tools for therapeutic applications.

What are potential side effects of gene therapy? (Select all that apply)

- ☐ **Immune reactions ✓**
- ☐ **Insertional mutagenesis ✓**
- ☐ Weight gain
- ☐ **Off-target effects in gene editing ✓**

> Gene therapy can lead to various side effects, including immune reactions, insertional mutagenesis, and off-target effects. These potential risks highlight the need for careful monitoring and evaluation in clinical applications.

Which of the following are methods of gene delivery in gene therapy? (Select all that apply)

- ☐ **Viral Vectors ✓**
- ☐ **Liposomes ✓**
- ☐ **Direct injection of naked DNA ✓**
- ☐ Antibiotics

> Gene delivery methods in gene therapy include viral vectors, liposomes, electroporation, and microinjection. These techniques are essential for effectively introducing therapeutic genes into target cells.

Describe a case study where gene therapy was successfully used to treat a genetic disorder.

> **In 2019, Zolgensma was approved for treating spinal muscular atrophy (SMA), a genetic disorder caused by a deficiency of the SMN1 gene. This gene therapy has shown remarkable success in improving motor function and prolongation of life in affected infants.**

Explain the difference between somatic and germline gene therapy.

> **The main difference between somatic and germline gene therapy is that somatic gene therapy alters genes in non-reproductive cells, affecting only the individual, whereas germline gene therapy modifies genes in reproductive cells, allowing changes to be passed on to offspring.**

Gene therapy can be used to treat which of the following conditions? (Select all that apply)

- [ ] **Hemophilia ✓**
- [ ] **Muscular Dystrophy ✓**
- [ ] Common Cold
- [ ] **Certain types of blindness ✓**

> Gene therapy can be used to treat a variety of genetic disorders, certain types of cancer, and some viral infections by correcting or replacing defective genes. Conditions such as cystic fibrosis, hemophilia, and muscular dystrophy are examples where gene therapy has shown potential.

In your opinion, what is the future potential of gene therapy in medicine, and what advancements are needed to realize this potential?

The future potential of gene therapy in medicine is significant, particularly for treating genetic disorders and cancers. To realize this potential, advancements are needed in targeted delivery systems, improved safety profiles, and streamlined regulatory processes.