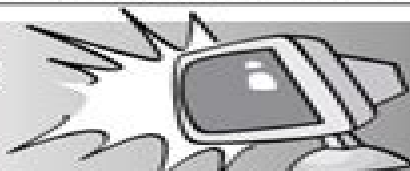




# Exploring Gene Therapy - Answer Key



Log on to: <http://gslc.genetics.utah.edu/units/genetherapy> and explore this module to find the answers to the questions below.

*Hint: the Search feature on this website may or may not help you find what you are looking for; it is best to go through the module to find the answers.*

## Questions:

1. What is gene therapy?

*Adding a normally functioning copy of a mutated gene to help affected cells, tissues and organs work properly.*

2. What are the five criteria for a gene therapy candidate disease?

- 1. The condition must result from mutations in one or more genes.*
- 2. You must know which gene is involved and have an available DNA copy of that gene.*
- 3. You must know the biology of the disorder - which tissue is affected, the role of the protein encoded by the gene, and how mutations in the gene affect the protein product.*
- 4. Adding a normal copy of the gene must fix the problem.*
- 5. It must be possible to deliver the gene to cells of the affected tissue.*

3. Case Study: Is cystic fibrosis a good candidate for gene therapy? Why or why not?

*Yes, cystic fibrosis meets all of the criteria above.*

4. What are the hallmarks of successful gene delivery in gene therapy trials?

*Targeting the right cells, activating the gene, integrating the gene into the cells, avoiding harmful side effects.*

5. Explain the *in vivo* technique of delivering genes to a patient's cells.

*The vector carrying the gene is injected directly into the body. The *in vivo* approach happens inside the body.*

6. Explain the *ex vivo* technique of delivering genes to a patient's cells.

*Cells from the affected tissue are isolated and cultured in a Petri dish. The vector carrying the gene is introduced to the cells in the Petri dish. The *ex vivo* approach happens outside the body.*

# Part 1 Exploring Gene Therapy Answers

**Michael Hoelscher**



## **Part 1 Exploring Gene Therapy Answers:**

## **Part 1 Exploring Gene Therapy Answers** Book Review: Unveiling the Power of Words

In a world driven by information and connectivity, the energy of words has become more evident than ever. They have the ability to inspire, provoke, and ignite change. Such may be the essence of the book **Part 1 Exploring Gene Therapy Answers**, a literary masterpiece that delves deep into the significance of words and their effect on our lives. Written by a renowned author, this captivating work takes readers on a transformative journey, unraveling the secrets and potential behind every word. In this review, we shall explore the book's key themes, examine its writing style, and analyze its overall impact on readers.

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